FORM S-1/A

General form of registration statement for all companies including face-amount certificate companies [amend]

Filing Date: **2019-06-10**
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SIC: **2836** Biological products, (no disgnostic substances)

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As filed with the Securities and Exchange Commission on June 10, 2019

Registration No. 333-231754

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

Amendment No. 1 to

FORM S-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

Prevail Therapeutics Inc.

(Exact name of registrant as specified in its charter)

Delaware	2836	82-2129632
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification No.)

430 East 29th Street, Suite 940
New York, New York 10016
(917) 336-9310
(Address, including zip code, and telephone number, including area code, of registrant' s principal executive offices)

Asa Abeliovich, M.D., Ph.D.
President and Chief Executive Officer
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Approximate date of commencement of proposed sale to the public: As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. ☐

If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act. ☒

CALCULATION OF REGISTRATION FEE

Title of Each Class of Securities To Be Registered	Amount to be Registered(1)	Proposed Maximum Offering Price Per Share(2)	Proposed Maximum Aggregate Offering Price(2)	Amount of Registration Fee(3)
Common Stock, par value $0.0001 per share	8,455,950	$18.00	$152,207,100	$18,448

(1) Includes an additional 1,102,950 shares that the underwriters have the option to purchase.
(2) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(a) under the Securities Act of 1933, as amended.
(3) $12,120 of this amount was previously paid.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

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The information in this prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these securities and it is not soliciting an offer to buy these securities in any state where the offer or sale is not permitted.

SUBJECT TO COMPLETION, DATED JUNE 10, 2019
PRELIMINARY PROSPECTUS

7,353,000 Shares

Common Stock

We are offering 7,353,000 shares of our common stock. This is our initial public offering and no public market currently exists for our common stock. We anticipate that the initial public offering price will be between $16.00 and $18.00 per share. We have applied to list our common stock on the Nasdaq Global Market under the symbol "PRVL."

We are an "emerging growth company" as defined under the U.S. federal securities laws and, as such, may elect to comply with reduced public company reporting requirements for this and future filings. See "Prospectus Summary–Implications of Being an Emerging Growth Company."

Investing in our common stock involves a high degree of risk. See "Risk Factors" beginning on page 10 of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

	PER SHARE	***TOTAL***
Initial public offering price	*$*	*$*
Underwriting discounts and commissions (1)	*$*	*$*
Proceeds, before expenses, to us	*$*	*$*

(1) See "Underwriting" for a description of all compensation payable to the underwriters.

We have granted the underwriters an option for a period of 30 days to purchase up to 1,102,950 additional shares of common stock.

The underwriters expect to deliver the shares of common stock against payment in New York, New York on or about , 2019.

MORGAN STANLEY *BofA MERRILL LYNCH* *COWEN*

WEDBUSH PACGROW

Prospectus dated , 2019.

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We and the underwriters have not authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectuses we have prepared. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may provide you. We are offering to sell, and seeking offers to buy, shares of common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of the common stock.

For investors outside the United States: Neither we nor the underwriters have done anything that would permit this offering or the possession or distribution of this prospectus in any jurisdiction where action for those purposes is required, other than in the United States. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, this offering of common stock and the distribution of this prospectus outside the United States.

Until , 2019 (25 days after the date of this prospectus), all dealers that buy, sell or trade our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

PROSPECTUS SUMMARY

This summary highlights information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, especially the section titled "Risk factors" and our financial statements and the related notes appearing elsewhere in this prospectus, before making an investment decision. As used in this prospectus, unless the context otherwise requires, references to "we," "us," "our," "the company" and "Prevail Therapeutics" refer to Prevail Therapeutics Inc.

Overview

We are a gene therapy company leveraging breakthroughs in human genetics with the goal of developing and commercializing disease-modifying AAV-based gene therapies for patients with devastating neurodegenerative diseases. We are applying a precision medicine approach to neurodegeneration by studying our gene therapies in genetically defined patient populations. We believe this will increase the probability of creating disease-modifying therapies that improve patients' lives. Our lead program is PR001 for the treatment of Parkinson's disease with *GBA1* mutation, or PD-GBA, and neuronopathic Gaucher disease. We are focused on developing a broad pipeline of gene therapies for a range of neurodegenerative diseases, including PR006 for the treatment of frontotemporal dementia with *GRN* mutation and PR004 for the treatment of synucleinopathies.

Our differentiated approach to developing gene therapies for neurodegenerative diseases is designed to mitigate challenges faced by others in the development of therapeutics for the central nervous system, or CNS. We select targets for diseases that correspond to patient populations with particular genetic mutations whom we believe can be treated by increasing or decreasing the expression of a particular gene, which makes them well-suited for gene therapy. We apply our deep understanding of human genetics to design our gene therapy product candidates, each of which is intended to be a one-time treatment to address the key underlying genetic mutation that we believe drives disease progression.

We are developing our lead program, PR001, to treat patients with PD-GBA and neuronopathic Gaucher disease. PD-GBA affects 7% to 10% of the total Parkinson's disease population worldwide and an estimated 90,000 individuals in the United States alone. Gaucher disease is among the most common lysosomal storage disorders, with an estimated global prevalence of one per 30,000 to one per 100,000. Neuronopathic Gaucher disease patients exhibit neurological manifestations in addition to the non-CNS manifestations of Gaucher disease, and represent approximately 6% of all Gaucher disease cases in the United States.

PD-GBA and Gaucher disease share the same underlying genetic mechanism, and we believe they represent a continuum of pathology. The symptoms and severity of the CNS disease in PD-GBA and Gaucher disease depend on the level of enzyme deficiency, which is driven by both the severity and number of *GBA1* mutations. *GBA1* encodes the lysosomal enzyme, beta-glucocerebrosidase, or GCase. PD-GBA patients have a mutation in one chromosomal copy of *GBA1* and Gaucher disease patients have mutations in both chromosomal copies of *GBA1*. These mutations lead to a deficiency of GCase, resulting in the non-CNS manifestations of Gaucher disease as well as lysosomal dysfunction in CNS cells, which we believe leads to the inflammation and neurodegeneration present in PD-GBA and neuronopathic Gaucher disease patients. Approved enzyme replacement therapies, or ERTs, which restore GCase, are effective for the treatment of the non-CNS manifestations of Gaucher disease, but ERTs cannot cross the blood-brain barrier to treat neurodegeneration. Based on the common genetically driven mechanism of PD-GBA and Gaucher disease, we have designed PR001 to express *GBA1* in patients' CNS cells. We believe that restoring *GBA1* in the CNS will slow or stop disease progression in PD-GBA and neuronopathic Gaucher disease patients.

In our comprehensive preclinical program in both mouse models and non-human primates, PR001 was observed to be well tolerated and demonstrated robust and widespread biodistribution. Additionally, in mouse

models, we observed significant increases in enzyme activity, reductions in lipid accumulation and improvements in motor function. We submitted an investigational new drug application, or IND, to the U.S. Food and Drug Administration, or FDA, for PR001 for the treatment of PD-GBA in May 2019, and the FDA has notified us that our trial may proceed. We intend to initiate our Phase 1/2 clinical trial for PR001 in PD-GBA in 2019. We plan to submit an IND to the FDA for PR001 for the treatment of neuronopathic Gaucher disease in mid-2019 and, subject to feedback from the FDA, we intend to initiate a Phase 1/2 clinical trial for PR001 in neuronopathic Gaucher disease in 2019. Our Phase 1/2 clinical trials in these patients will investigate the safety and tolerability of PR001, and will also measure key biomarkers and exploratory efficacy endpoints.

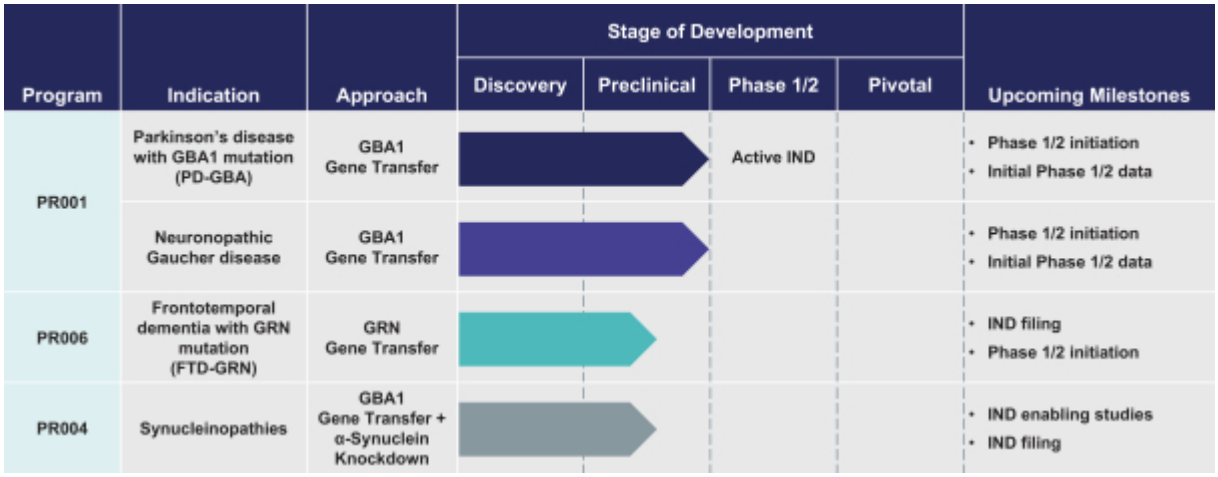
In addition to our lead program, we are developing PR006 for the treatment of frontotemporal dementia with *GRN* mutation, or FTD-GRN. We intend to submit an IND to the FDA for PR006 for the treatment of FTD-GRN in 2019. We are also currently conducting preclinical studies of PR004 for the treatment of synucleinopathies.

All of our current programs utilize adeno-associated virus, or AAV, gene therapy technology, which we believe is particularly well-suited for the treatment of CNS diseases. AAV-based viral vectors have been observed in third-party clinical trials to be well-tolerated and to have promise in delivering stable, long-lasting transgene expression in a range of tissues, including the CNS. We have initially chosen to use AAV9 based on its transformational biological properties and track record, which we believe will translate into a positive clinical effect in our initial indications. In a third-party Phase 1 clinical trial in Type 1 spinal muscular atrophy, AAV9 was observed to enable gene delivery in the CNS and broad brain-wide biodistribution with a single administration. We have entered into license agreements with REGENXBIO Inc., or REGENXBIO, pursuant to which they granted us an exclusive, worldwide license to use AAV9 delivering the gene encoding for GBA1 for the treatment of disease, as well as three distinct exclusive options for specified genes for the treatment of disease. In April 2019, we exercised all three options, including for AAV9 delivering the genes encoding for progranulin and α-Synuclein. For our initial programs, we plan to deliver directly to the cerebrospinal fluid via a minimally invasive non-surgical procedure.

Our company was founded through a collaborative effort by Asa Abeliovich, M.D., Ph.D., our Chief Executive Officer, OrbiMed and The Silverstein Foundation for Parkinson’ s with GBA, who shared a common vision: to cure Parkinson’ s disease and other neurodegenerative disorders. Dr. Abeliovich and our other scientific leaders bring a deep understanding of the human genetics of neurodegenerative diseases, the underlying molecular mechanisms by which genetic mutations cause these diseases, and how to optimally design potential therapies to restore the function impaired by these genetic mutations. We intend to apply our expertise to developing therapies that have potential to slow or stop disease progression for neurodegenerative disease patients.

Our Pipeline

Our initial AAV9 gene therapy programs are summarized in the table below. We hold worldwide commercial rights to all of our programs.



We are focused on developing a broad pipeline of disease-modifying AAV gene therapies for the treatment of a range of neurodegenerative diseases with high unmet medical need, including Parkinson' s disease, frontotemporal dementia, or FTD, Alzheimer' s disease, amyotrophic lateral sclerosis, or ALS, dementia with Lewy bodies, or DLB, and related lysosomal disorders. Our goal is to use the capabilities we have established for our other product candidates to rapidly advance these programs towards clinical testing.

Our Strategy

We are leveraging breakthroughs in human genetics with the goal of developing and commercializing disease-modifying gene therapies for patients with neurodegenerative diseases with high unmet medical need. Key elements of our strategy to achieve this goal include:

Build a patient-focused gene therapy company.

Apply precision medicine to developing gene therapies for the treatment of neurodegenerative diseases.

Leverage the transformational potential of AAV gene therapy technology.

Rapidly advance PR001 through clinical trials.

Continue to develop our innovative pipeline of gene therapies.

Continue to develop our manufacturing processes to meet clinical and commercial needs.

Private Financings

To date, we have raised approximately $129.0 million in aggregate gross proceeds, primarily through convertible preferred stock financings. Our investors include OrbiMed, The Silverstein Foundation for Parkinson' s with GBA, Pontifax, RA Capital Management, EcoR1 Capital, Omega Funds, BVF Partners, Boxer Capital, Adage Capital Management, Alexandria Venture Investments, and Surveyor Capital (a Citadel company).

Risks Affecting Our Business

Our business is subject to a number of risks. These risks are discussed more fully in the section titled "Risk Factors" immediately following this prospectus summary. You should read these risks before you invest in our common stock. In particular, risks associated with our business include, but are not limited to, the following:

We have a limited operating history, have incurred significant losses since inception and anticipate that we will incur continued losses for the foreseeable future. We may never achieve or maintain profitability.

We will require additional capital to fund our operations, which may not be available on acceptable terms, if at all.

We are heavily dependent on the success of PR001, which is still in early development, and if we are unable to progress PR001 through clinical testing, receive regulatory approval for PR001 or successfully commercialize PR001, our business may be harmed.

We intend to identify and develop product candidates based on our novel approach to gene therapy, which makes it difficult to predict the time, cost and potential success of product candidate development.

Gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.

Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.

Clinical trials are expensive, time-consuming, difficult to design and implement, and involve an uncertain outcome. Further, we may encounter substantial delays in our clinical trials.

Our gene therapy product candidates rely on AAV to efficiently transmit a therapeutic gene, and the mechanism of action by which AAV targets particular tissues is novel and still not well understood. We cannot assure you that our viral vectors will be able to meet safety and efficacy levels needed to be therapeutic in humans or that they will not cause significant adverse events or toxicity, including the risk of immunogenicity attendant to any gene therapy product based on viral vectors.

Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact the development or commercial success of our current and future product candidates.

Compassionate use or expanded access of our unapproved therapies could negatively affect our reputation, our development timelines and our business, including as a result of the existence of clinical holds, such as the clinical hold placed on the investigator-sponsored IND initiated by the University of Florida for PR001 for the treatment of Type 2 Gaucher disease.

The affected populations for our other product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.

We and our contract manufacturers for plasmids and viruses are subject to significant regulation. The third-party manufacturing facilities on which we rely, and any manufacturing facility that we may have in the future, may have limited capacity or fail to continue to meet the applicable stringent regulatory requirements.

We depend on proprietary technology licensed from others. If we lose our existing licenses or are unable to acquire or license additional proprietary rights from third parties, we may not be able to continue developing our product candidates.

We may be subject to third party claims arising from consulting agreements entered into by our officers, employees, independent contractors and/or consultants, including for example, the letter we received in June 2019 on behalf of Alector, Inc., or Alector, alleging, among other things, that certain

confidential information of Alector was used by our company and that Alector has certain rights to our patents and patent applications.

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.

Our Corporate Information

We were incorporated under the laws of the State of Delaware in July 2017. Our principal executive offices are located at 430 East 29th Street, Suite 940, New York, New York 10016, and our telephone number is (917) 336-9310. Our website address is www.prevailtherapeutics.com. The information contained on, or accessible through, our website is not incorporated by reference into this prospectus, and you should not consider any information contained in, or that can be accessed through, our website as part of this prospectus or in deciding whether to purchase our common stock.

We own a U.S. federal trademark application for PREVAIL THERAPEUTICS. All other trademarks or trade names referred to in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus are referred to without the symbols ® and ™, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

Implications of Being an Emerging Growth Company

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We will remain an emerging growth company until the earliest to occur of: (1) the last day of the fiscal year in which we have more than $1.07 billion in annual revenue; (2) the date we qualify as a "large accelerated filer," with at least $700 million of equity securities held by non-affiliates as of the prior June 30th; (3) the issuance, in any three-year period, by us of more than $1 billion in non-convertible debt securities; and (4) December 31, 2024. An emerging growth company may take advantage of reduced reporting requirements that are otherwise applicable to public companies. These provisions include, but are not limited to:

reduced obligations with respect to financial data, including presenting only two years of audited financial statements and only two years of selected financial data in this prospectus;

an exception from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act;

reduced disclosure about our executive compensation arrangements in our periodic reports, proxy statements and registration statements; and

exemptions from the requirements of holding non-binding advisory votes on executive compensation or golden parachute arrangements.

We have elected to take advantage of certain of the reduced disclosure obligations in this prospectus and may elect to take advantage of other reduced reporting requirements in our future filings with the U.S. Securities and Exchange Commission, or the SEC. As a result, the information that we provide to our stockholders may be different than you might receive from other public reporting companies in which you hold equity interests.

In addition, under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company," which would allow us to take advantage of many of the same exemptions from disclosure requirements including reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements.

THE OFFERING

Common stock offered by us	7,353,000 shares.
Option to purchase additional shares	We have granted the underwriters an option for a period of 30 days to purchase up to 1,102,950 additional shares of common stock.
Common stock to be outstanding after this offering	33,997,131 shares (or 35,100,081 shares if the underwriters exercise their option to purchase additional shares in full).
Use of proceeds	We estimate that the net proceeds to us from this offering will be approximately $113.4 million, or approximately $130.8 million if the underwriters exercise their option to purchase additional shares in full, assuming an initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering, together with our existing cash and cash equivalents: to advance the development of PR001, including to fund our planned Phase 1/2 trials for PR001 for the treatment of PD-GBA and neuronopathic Gaucher disease; to advance the development of PR006, including our ongoing preclinical studies for PR006 for the treatment of FTD-GRN; to advance the development of PR004, including our ongoing preclinical studies for PR004 for the treatment of synucleinopathies; and for working capital and general corporate purposes. See the section titled "Use of Proceeds."
Risk factors	See the section titled "Risk Factors" and the other information included in this prospectus for a discussion of factors you should consider carefully before deciding to invest in our common stock.
Proposed Nasdaq Global Market symbol	"PRVL"

The number of shares of our common stock to be outstanding after this offering is based on 26,644,131 shares of our common stock outstanding as of March 31, 2019, and excludes:

- 4,376,161 shares of common stock issuable upon the exercise of options outstanding as of March 31, 2019, with a weighted-average exercise price of $1.00 per share;
- 485,862 shares of common stock reserved for future issuance pursuant to our 2017 Equity Incentive Plan, or the 2017 Plan;
- 2,773,562 shares of common stock reserved for future issuance pursuant to our 2019 Equity Incentive Plan, or the 2019 Plan, as well as any automatic increases in the number of common shares reserved for future issuance under the 2019 Plan; and

330,000 shares of common stock reserved for future issuance pursuant to our 2019 Employee Stock Purchase Plan, or 2019 ESPP, as well as any automatic increases in the number of common shares reserved for future issuance under the 2019 ESPP.

Unless otherwise indicated, this prospectus reflects and assumes the following:

a 1.62-for-one stock split of our common stock, effected June 7, 2019;

the automatic conversion of all outstanding shares of our convertible preferred stock into an aggregate of 19,435,131 shares of our common stock immediately prior to the closing of this offering;

no exercise of the outstanding options described above;

no exercise by the underwriters of their option to purchase up to 1,102,950 additional shares of our common stock; and

the filing and effectiveness of our amended and restated certificate of incorporation and the adoption of our amended and restated bylaws immediately prior the closing of this offering.

SUMMARY FINANCIAL DATA

The following tables summarize our financial data for the periods indicated. We have derived the summary statements of operations data for the period from July 6, 2017 (date of inception) through December 31, 2017 and the year ended December 31, 2018 from our audited financial statements included elsewhere in this prospectus. We have derived our balance sheet data as of December 31, 2018 from our audited financial statements included elsewhere in this prospectus. The summary statements of operations data for the three months ended March 31, 2018 and 2019 and the summary balance sheet data as of March 31, 2019 have been derived from our unaudited interim financial statements included elsewhere in this prospectus. The unaudited financial statements have been prepared on the same basis as the audited financial statements, and in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly our financial position and results of operations.

Our interim and historical results are not necessarily indicative of the results that may be expected for the full year or any other future period. The following summary financial data should be read in conjunction with the section titled "Management' s Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes included elsewhere in this prospectus.

	Period from July 6, 2017 (Date of Inception) through December 31, 2017	Year Ended December 31, 2018	Three Months Ended March 31, 2018	Three Months Ended March 31, 2019
			(unaudited)	
	(in thousands, except share and per share data)			
Statement of Operations Data:				
Operating expenses:				
Research and development	$ 995	$ 14,127	$1,304	$8,411
General and administrative	804	4,682	641	1,885
Total operating expenses	1,799	18,809	1,945	10,296
Operating loss	(1,799)	(18,809)	(1,945)	(10,296)
Change in fair value of derivative liabilities	–	(781)	(781)	–
Other income	–	87	–	–
Interest (expense) income, net	(27)	416	(471)	351
Net loss	$ (1,826)	$ (19,088)	$(3,197)	$(9,945)
Net loss per share attributable to common stockholders, basic and diluted(1)	$ (0.45)	$ (3.71)	$(0.66)	$(1.73)
Weighted-average shares outstanding, basic and diluted(1)	4,050,000	5,145,469	4,860,000	5,740,874
Pro forma net loss per share attributable to common stockholders, basic and diluted(1)		$ (1.21)		$(0.51)
Pro forma weighted average shares outstanding, basic and diluted(1)		15,748,039		19,957,958

(1) See note 14 to our financial statements included elsewhere in this prospectus for an explanation of the method used to calculate basic and diluted net loss per share.

	As of March 31, 2019		
	Actual	Pro Forma(1)	Pro Forma As Adjusted(2)(3)
		(in thousands)	
Balance Sheet Data:			
Cash and cash equivalents	$ 100,268	$100,268	$213,619
Working capital(4)	98,894	98,894	212,245
Total assets	115,194	115,194	228,545
Convertible preferred stock	129,544	–	–
Accumulated deficit	(30,859)	(30,859)	(30,859)
Total stockholders' (deficit) equity	(27,751)	101,793	215,144

(1) Pro forma balance sheet data reflects (a) the automatic conversion of all outstanding shares of convertible preferred stock into common stock as if such conversion had occurred on March 31, 2019 and (b) the filing and effectiveness of our amended and restated certificate of incorporation, each of which will occur immediately prior to the closing of this offering.

(2) Pro forma as adjusted balance sheet data reflects the pro forma items described immediately above and our sale of 7,353,000 shares of common stock in this offering at an assumed initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

(3) Pro forma as adjusted balance sheet data is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. Each $1.00 increase or decrease in the assumed initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease pro forma as adjusted cash and cash equivalents, working capital, total assets and total stockholders' (deficit) equity by approximately $6.8 million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting estimated underwriting discounts and commissions. We may also increase or decrease the number of shares we are offering. A 1,000,000 share increase or decrease in the number of shares offered by us would increase or decrease pro forma as adjusted cash and cash equivalents, working capital, total assets and total stockholders' (deficit) equity by approximately $15.8 million, assuming that the assumed initial offering price to the public remains the same, and after deducting estimated underwriting discounts and commissions.

(4) We define working capital as current assets less current liabilities. See our financial statements and the related notes included elsewhere in this prospectus for further details regarding our current assets and current liabilities.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the following risks and uncertainties, together with all of the other information contained in this prospectus, including our financial statements and related notes included elsewhere in this prospectus, before making an investment decision. The risks described below are not the only ones facing us. The occurrence of any of the following risks, or of additional risks and uncertainties not presently known to us or that we currently believe to be immaterial, could materially and adversely affect our business, financial condition or results of operations. In such case, the trading price of our common stock could decline, and you may lose part or all of your investment.

Risks Related to Our Financial Position and Need for Additional Capital

We have a limited operating history, have incurred significant losses since inception and anticipate that we will incur continued losses for the foreseeable future. We may never achieve or maintain profitability.

Since our inception in 2017, we have incurred significant operating losses. Our net losses were $1.8 million for the period from July 6, 2017 (date of inception) through December 31, 2017, $19.1 million for the year ended December 31, 2018 and $9.9 million for the three months ended March 31, 2019. As of December 31, 2018 and March 31, 2019, we had an accumulated deficit of $20.9 million and $30.9 million, respectively. Since our inception, we have focused primarily on organizing and staffing our company, raising capital, establishing and protecting our intellectual property portfolio, in-licensing AAV9 in particular fields, developing and progressing our gene therapy product candidates through preclinical studies and preparing for clinical trials, and establishing our manufacturing platform. Consequently, we have no meaningful operations upon which to evaluate our business, and predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing gene therapies.

We have no products approved for commercial sale. We have never been profitable and do not expect to be profitable in the foreseeable future. Our ability to generate revenue and become profitable depends upon our ability to successfully complete the development of our product candidates and to obtain the necessary regulatory approvals for their commercialization. We are developing our lead product candidate, PR001, an AAV9 vector delivering the *GBA1* gene, for several related indications, including PD-GBA and neuronopathic Gaucher disease. We submitted an IND to the FDA for PR001 for the treatment of PD-GBA in May 2019, and the FDA has notified us that our trial may proceed. We intend to initiate our Phase 1/2 clinical trial for PR001 in PD-GBA in 2019. We plan to submit an IND to the FDA for PR001 for the treatment of neuronopathic Gaucher disease in mid-2019 and, subject to feedback from the FDA, we intend to initiate a Phase 1/2 clinical trial for PR001 in neuronopathic Gaucher disease in 2019. We have not yet demonstrated an ability to successfully complete a clinical program, including large-scale, pivotal clinical trials, obtain marketing approval, manufacture product at a commercial scale, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization.

We expect to continue to incur significant expenses and additional operating losses for the foreseeable future as we seek to advance our product candidates through preclinical and clinical development, expand our research and development activities, develop new product candidates, complete clinical trials, seek regulatory approval and, if we receive regulatory approval, commercialize our products. Furthermore, the costs of advancing product candidates into each succeeding clinical phase tend to increase substantially over time. The total costs to advance any of our product candidates to marketing approval in even a single jurisdiction would be substantial. Because of the numerous risks and uncertainties associated with gene therapy product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to begin generating revenue from the commercialization of products or achieve or maintain profitability. Our expenses will also increase substantially if and as we operate as a public company and add clinical, scientific, operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts, as well as to support our transition to a public reporting company.

Before we generate any revenue from product sales, each of our programs and product candidates will require additional preclinical and/or clinical development, potential regulatory approval in multiple jurisdictions, manufacturing, building of a commercial organization, substantial investment and significant marketing efforts. Our expenses could increase beyond expectations if we are required by the FDA, European Medicines Agency, or EMA, or other regulatory authorities to perform preclinical studies and clinical trials in addition to those that we currently anticipate. These risks are further described under "−Risks Related to Discovery, Development, Clinical Testing, Manufacturing and Regulatory Approval" and "−Risks Related to Commercialization." As a result, we expect to continue to incur net losses and negative cash flows for the foreseeable future. These net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital.

As we continue to build our business, we expect our financial condition and operating results may fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any particular quarterly or annual period as indications of future operating performance. If we are unable to develop and commercialize one or more of our product candidates either alone or with collaborators, or if revenues from any product candidate that receives marketing approval are insufficient, we will not achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability. If we are unable to achieve and then maintain profitability, the value of our equity securities will be adversely affected.

We will require additional capital to fund our operations, which may not be available on acceptable terms, if at all.

We expect to spend substantial amounts to advance our product candidates into clinical development and to complete the clinical development of, seek regulatory approvals for and commercialize our product candidates, if approved. We will require additional capital beyond the proceeds of this offering, which we may raise through equity offerings, debt financings, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or other sources to enable us to complete the development and potential commercialization of our product candidates. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative effect on our financial condition and our ability to pursue our business strategy. In addition, attempting to secure additional financing may divert the time and attention of our management from day-to-day activities and harm our product candidate development efforts. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate certain of our research and development programs.

Our operations have consumed significant amounts of cash since inception. As of December 31, 2018, our cash and cash equivalents were $63.0 million. In March 2019, we raised an aggregate of $50.0 million of gross proceeds from our Series B convertible preferred stock financing. Based on our planned use of the net proceeds of this offering and our current cash and cash equivalents, we estimate that such funds will be sufficient to enable us to fund our operating expenses and capital expenditure requirements at least through the first half of 2021. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Changing circumstances could cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more than currently expected because of circumstances beyond our control. Because the length of time and activities associated with successful development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of our planned clinical trials for PR001;
- continuing our current research programs and our preclinical development of product candidates, including PR001, PR006 and PR004, from our current research programs;

seeking to identify, assess, acquire and/or develop additional research programs and additional product candidates;

the preclinical testing and clinical trials for any product candidates we identify and develop;

the cost of establishing a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval;

the outcome, timing and cost of meeting regulatory requirements established by the FDA, EMA and other regulatory authorities;

the cost of expanding and protecting our intellectual property portfolio, including filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;

the cost of defending potential intellectual property disputes, including patent infringement actions brought by third parties against us or any of our product candidates;

the effect of competing technological and market developments;

the cost of further developing and scaling our potential manufacturing facility and processes;

the cost and timing of completion of commercial-scale manufacturing activities;

the cost of making royalty, milestone or other payments under current and any future in-license agreements;

the extent to which we in-license or acquire other products and technologies; and

the initiation, progress, timing and results of our commercialization of our product candidates, if approved for commercial sale.

To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a holder of common stock. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

We are heavily dependent on the success of our most advanced product candidate, PR001, which is still in early development. If PR001 does not progress to the next phase of clinical testing or receive regulatory approval, or if we are unable to successfully commercialize PR001, our business may be harmed.

To date, we have invested a significant portion of our efforts and financial resources in the development of PR001. Our future success and ability to generate product revenue is substantially dependent on our ability to successfully develop, obtain regulatory approval for and successfully commercialize this product candidate.

We currently have no products that are approved for commercial sale and may never be able to develop marketable products. We expect to invest a meaningful portion of our efforts and expenditures over the next few years in PR001, which will require clinical development, management of clinical and manufacturing activities, regulatory approval in multiple jurisdictions, manufacturing sufficient supply, building of a commercial organization, substantial investment and significant marketing efforts before we can generate any revenues from any commercial sales. Accordingly, our business currently depends heavily on the successful development,

regulatory approval and commercialization of PR001, which may never occur. We cannot be certain that PR001 will be successful in clinical trials or receive regulatory approval. Even if we receive regulatory approval to market PR001 from the FDA, EMA or other regulatory bodies, we cannot be certain that our product candidate will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available therapies. We also cannot be certain that third-party payors will adequately reimburse for treatments involving our product candidate. Additionally, the research, testing, manufacturing, labeling, approval, sale, marketing and distribution of gene therapy products are and will remain subject to extensive and evolving regulation by the FDA, EMA and other regulatory authorities. We are not permitted to market PR001 in the United States until it receives approval of a biologics license application, or BLA, from the FDA, and we cannot market it in the European Union until we receive approval for a Marketing Authorization Application, or MAA, from the EMA, or other required regulatory approval in other countries.

PR001 is our most advanced product candidate, and because some of our other product candidates are based on similar technology, if PR001 shows unexpected adverse events or a lack of efficacy in the indications we intend to treat, or if we experience other regulatory or developmental issues, our development plans and business could be significantly harmed. Further, competitors may be developing products with similar technology and may experience problems with their products that could identify problems that would potentially harm our business.

We may not be successful in our efforts to identify additional product candidates.

Part of our strategy involves identifying novel product candidates based on our deep understanding of human genetics. The process by which we identify product candidates may fail to yield product candidates for clinical development for a number of reasons, including those discussed in these risk factors and also:

- we may be unable to develop programs that have a clearly defined and genetically driven disease mechanism, are well suited to gene therapy, have compelling preclinical data, are in genetically defined populations, have biomarkers that may provide early clinical proof-of-mechanism or that have a large potential market opportunity;
- we may not be able to assemble sufficient resources to acquire or discover additional product candidates;
- competitors may develop alternatives that render our potential product candidates obsolete or less attractive;
- potential product candidates we develop may nevertheless be covered by third parties' patents or other exclusive rights;
- potential product candidates may, on further study, be shown to have harmful side effects, toxicities or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance;
- potential product candidates may not be effective in treating their targeted diseases;
- the market for a potential product candidate may change so that the continued development of that product candidate is no longer reasonable;
- a potential product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or
- the regulatory pathway for a potential product candidate may be too complex and difficult to navigate successfully or economically.

In addition, we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. As a result, we may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product

candidates or other diseases that may later prove to have greater commercial potential, or relinquish valuable rights to such product candidates through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to retain sole development and commercialization rights. If we are unable to identify additional suitable product candidates for clinical development, this would adversely impact our business strategy, our financial position and share price and could potentially cause us to cease operations.

Risks Related to Discovery, Development, Clinical Testing, Manufacturing and Regulatory Approval

We intend to identify and develop product candidates based on our novel approach to gene therapy, which makes it difficult to predict the time, cost and potential success of product candidate development.

We have concentrated our research and development efforts on our gene therapy product candidates. Our future success depends on the successful development of these novel therapeutic approaches. To date, very few products that utilize gene transfer have been approved in the United States or Europe. There have been a limited number of clinical trials of gene transduction technologies, with only two product candidates ever approved by the FDA.

Our gene therapy product candidates are based on a viral vector which we can deploy with gene therapy constructs, which relies on the ability of AAV to efficiently transmit a therapeutic gene to certain kinds of cells. The mechanism of action by which this vector targets particular tissues is still not completely understood. Therefore, it is difficult for us to determine that our vectors will be able to properly deliver gene transfer constructs to enough tissue cells to reach therapeutic levels. We cannot be certain that our viral vectors will be able to meet safety and efficacy levels needed to be therapeutic in humans or that they will not cause significant adverse events or toxicities. We cannot be certain that we will be able to avoid triggering toxicities in our future preclinical studies or clinical trials. Any such results could impact our ability to develop a product candidate, including our ability to enroll patients in our clinical trials. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our approach to gene therapy, or any similar or competitive programs, will result in the identification, development, and regulatory approval of any product candidates, or that other gene therapy programs will not be considered better or more attractive. There can be no assurance that any development problems we experience in the future related to our current gene therapy product candidates or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays and challenges in achieving sustainable, reproducible, and scalable production. Any of these factors may prevent us from completing our preclinical studies or clinical trials or commercializing any product candidates we may develop on a timely or profitable basis, if at all.

Gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.

The manufacture of gene therapy products is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies.

We presently contract with third parties for the manufacturing of our program materials and are working to develop commercial-scale manufacturing capabilities with these third parties. We currently have no plans to build our own clinical or commercial-scale manufacturing facilities. The use of contracted manufacturing and reliance on collaboration partners is relatively cost efficient and has eliminated the need for our direct investment in manufacturing facilities and additional staff early in development. Although we rely on contract manufacturers, we have personnel with manufacturing and quality experience to oversee our contract manufacturers.

To date, our third-party manufacturers have met our manufacturing requirements for our program materials. We expect third-party manufacturers to be capable of providing sufficient quantities of our program materials to meet anticipated clinical trial scale demands. To meet our projected needs for additional clinical trials and commercial manufacturing, we will need to secure multiple suppliers. We believe that there are alternate sources of supply for our program materials that can satisfy our clinical and commercial requirements, although we cannot be certain that identifying and establishing relationships with such sources, if necessary, would not result in significant delay or material additional costs.

To date, our third-party manufacturers have met our quality standards for our program materials. The manufacturers of pharmaceutical products must comply with strictly enforced current good manufacturing practices, or cGMP, requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure of us or our contract manufacturing organizations to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our program materials for clinical study or enforcement action from the FDA or foreign regulatory authorities. If we or our manufacturers were to fail to comply with the FDA, EMA, or other regulatory authority, it could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates. Our potential future dependence upon others for the manufacture of our product candidates may also adversely affect our future profit margins and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

Biological products are inherently difficult to manufacture. Our program materials are manufactured using technically complex processes requiring specialized equipment and facilities, highly specific raw materials, cells, and reagents, and other production constraints. Our production process requires a number of highly specific raw materials, cells and reagents with limited suppliers. Even though we aim to have backup supplies of raw materials, cells and reagents whenever possible, we cannot be certain they will be sufficient if our primary sources are unavailable. A shortage of a critical raw material, cell line, or reagent, or a technical issue during manufacturing may lead to delays in clinical development or commercialization plans. Any changes in the manufacturing of components of the raw materials we use could result in unanticipated or unfavorable effects in our manufacturing processes, resulting in delays.

We are developing a scalable baculovirus production system for our gene therapy pipeline. If we are not able to successfully develop or transition to this new manufacturing process, we may be unable to meet our supply needs, which could adversely affect our future profit margins and our ability to progress our clinical development programs or commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.

The regulatory requirements that will govern any novel gene therapy product candidates we develop are not entirely clear and are subject to change. Within the broader genetic medicine field, very few therapeutic products have received marketing authorization from the FDA or EMA. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products and cell therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of existing gene therapy products and cell therapy products. For example, in the United States, the FDA has established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Our product

candidates will need to meet safety and efficacy standards applicable to any new biologic under the regulatory framework administered by the FDA. In addition to FDA oversight and oversight by institutional review boards, or IRBs, under guidelines promulgated by the National Institutes of Health, or NIH, gene therapy clinical trials are also subject to review and oversight by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment. While the NIH guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation.

The same applies in the European Union. The EMA' s Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety, and efficacy of advanced-therapy medicinal products. Advanced-therapy medicinal products include gene therapy medicines, somatic-cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any gene therapy product candidate we may develop, but that remains uncertain at this point.

Adverse developments in preclinical studies or clinical trials conducted by others in the field of gene therapy and gene regulation products may cause the FDA, the EMA, and other regulatory bodies to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing gene regulation technologies, either of which could harm our business. In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. The regulatory approval process for product candidates such as ours can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Further, as we are developing novel potential treatments for diseases in which, in some cases, there is little clinical experience with potential new endpoints and methodologies, there is heightened risk that the FDA, the EMA or other regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. Any natural history studies that we may conduct or rely upon in our clinical development may not be accepted by the FDA, EMA or other regulatory authorities. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene regulation technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the commercialization of resulting products.

The regulatory review committees and advisory groups described above and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop.

Clinical trials are expensive, time-consuming, difficult to design and implement, and involve an uncertain outcome. Further, we may encounter substantial delays in our clinical trials.

The clinical trials and manufacturing of our product candidates are, and the manufacturing and marketing of our products, if approved, will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we intend to test and market our product candidates. Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target indication. In particular, because our product candidates are subject to regulation as biological drug products, we will need to demonstrate that they are safe, pure and potent for use in their target indications. Each product candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive and can take many years to complete, and is subject to uncertainty. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. Failure can occur at any time during the clinical trial process. Even if our future clinical trials are completed as planned, we cannot be certain that their results will support the safety and effectiveness of our product candidates for their targeted indications or support continued clinical development of such product candidates. Our future clinical trial results may not be successful.

In addition, even if such trials are successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. To the extent that the results of the trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates.

To date, we have not completed any clinical trials required for the approval of our product candidates. We may experience delays in conducting any clinical trials and we do not know whether our clinical trials will begin on time, need to be redesigned, recruit and enroll patients on time or be completed on schedule, or at all. Events that may prevent successful or timely completion of clinical development include:

- inability to generate sufficient preclinical, toxicology, or other in vivo or in vitro data to support the initiation of clinical trials;
- delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for advanced clinical trials;
- delays in developing suitable assays for screening patients for eligibility for trials with respect to certain product candidates;
- delays in reaching agreement with the FDA, EMA or other regulatory authorities as to the design or implementation of our clinical trials;
- obtaining regulatory approval to commence a clinical trial;
- reaching an agreement on acceptable terms with clinical trial sites or prospective contract research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites;
- obtaining IRB approval at each site;
- recruiting suitable patients to participate in a clinical trial;
- developing and validating the companion diagnostic to be used in a clinical trial, if applicable;
- having patients complete a clinical trial or return for post-treatment follow-up;

clinical sites, CROs or other third parties deviating from trial protocol or dropping out of a trial;

failure to perform in accordance with the FDA' s good clinical practice, or GCP, requirements, or applicable regulatory guidelines in other countries;

addressing patient safety concerns that arise during the course of a trial, including occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;

adding a sufficient number of clinical trial sites; or

manufacturing sufficient quantities of product candidate for use in clinical trials.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates or significantly increase the cost of such trials, including:

we may experience changes in regulatory requirements or guidance, or receive feedback from regulatory authorities that requires us to modify the design of our clinical trials;

clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon development programs;

the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;

our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;

we or our investigators might have to suspend or terminate clinical trials of our product candidates for various reasons, including non-compliance with regulatory requirements, a finding that our product candidates have undesirable side effects or other unexpected characteristics, or a finding that the participants are being exposed to unacceptable health risks;

the cost of clinical trials of our product candidates may be greater than we anticipate and we may not have funds to cover the costs;

the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;

regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate; and

any future collaborators that conduct clinical trials may face any of the above issues, and may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

incur unplanned costs;

be delayed in obtaining marketing approval for our product candidates or not obtain marketing approval at all;

obtain marketing approval in some countries and not in others;

obtain marketing approval for indications or patient populations that are not as broad as intended or desired;

obtain marketing approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;

be subject to additional post-marketing testing requirements; or

have the product removed from the market after obtaining marketing approval.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board, or DSMB, for such trial or by the FDA, EMA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Our most advanced product candidates, PR001, PR006 and PR004, will require extensive clinical testing before we are prepared to submit a BLA or MAA for regulatory approval. We cannot predict with any certainty if or when we might complete the clinical development for our product candidates and submit a BLA or MAA for regulatory approval of any of our product candidates or whether any such BLA or MAA will be approved. We may also seek feedback from the FDA, EMA or other regulatory authorities on our clinical development program, and the FDA, EMA or such regulatory authorities may not provide such feedback on a timely basis, or such feedback may not be favorable, which could further delay our development programs.

We cannot predict with any certainty whether or when we might complete a given clinical trial. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be harmed, and our ability to generate revenues from our product candidates may be delayed. In addition, any delays in our clinical trials could increase our costs, slow down the development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and results of operations. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

The affected populations for our other product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.

We select the targets for development of our product candidates based on genetically defined patient populations where we believe there is a large addressable market opportunity. However, our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Incidence and prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The process we have used in developing an estimated incidence and prevalence range for the indications we are targeting has involved collating limited data from multiple sources. Accordingly, the incidence and prevalence estimates included in this prospectus should be viewed with caution. Further, the data and statistical information used in this prospectus, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

The use of such data involves risks and uncertainties and is subject to change based on various factors. Our estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of the diseases we seek to address. The number of patients with the diseases we are targeting in the United States, the European Union, Israel and elsewhere may turn out to be lower than expected or may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or access, all of which would harm our results of operations and our business.

Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact the development or commercial success of our current and future product candidates.

Our potential therapeutic products involve introducing genetic material into a patient' s cells. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene therapy and gene regulation for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy and gene regulation are unsafe, unethical or immoral, and consequently, our products may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products once approved. For example, in 2003, trials using early versions of murine gamma-retroviral vectors, which integrate with, and thereby alter, the host cell' s DNA, have led to several well-publicized adverse events, including reported cases of leukemia. Although none of our current product candidates utilize murine gamma-retroviral vectors, our product candidates use AAV viral vectors. Among the risks in any gene therapy product based on viral vectors are the risks of immunogenicity, elevated liver enzymes, and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation. If our vectors demonstrate a similar effect we may decide or be required to halt or delay further clinical development of PR001. Adverse events in our or others' clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. The risk of cancer remains a concern for gene therapy and we cannot assure that it will not occur in any of our planned or future clinical trials or in any clinical trials conducted by other companies. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. If any such adverse events occur, commercialization of our product candidates or further advancement of our clinical trials could be halted or delayed, which would have a negative impact on our business and operations.

Compassionate use or expanded access of our unapproved therapies could negatively affect our reputation, our development timelines and our business.

Expanded access, sometimes called "compassionate use," is the use of investigational new drug products outside of clinical trials to treat patients with serious or immediately life-threatening diseases or conditions when there are no comparable or satisfactory alternative treatment options. In April 2019, University of Florida submitted an investigator-sponsored IND to the FDA requesting an expanded access study in which infants with Type 2 Gaucher disease could receive PR001. This IND has been placed on clinical hold by the FDA. We do not have control over the University of Florida' s communications with the FDA or the timing of any resolution of

this clinical hold. We do not expect this clinical hold to affect the development timeline for our currently active IND for PR001 for the treatment of PD-GBA or our anticipated IND submission for PR001 for the treatment of neuronopathic Gaucher disease, however, the existence of this clinical hold could negatively impact public opinion of PR001.

In addition, patients who receive access to unapproved drugs through compassionate use or expanded access programs have life-threatening illnesses and often have exhausted all other available therapies. The risk for serious adverse events in this patient population is high which could have a negative impact on the safety profile of our product candidates, including PR001, which could cause significant delays or an inability to successfully commercialize our product candidates, which could materially harm our business. Further, we may in the future need to restructure or pause ongoing compassionate use and/or expanded access programs, which could prompt adverse publicity or other disruptions related to current or potential participants in such programs.

Other companies have been the target of disruptive social media campaigns related to a request for access to unapproved drugs for patients with significant unmet medical need. If we experience a similar social media campaign regarding our decision to provide or not provide our product candidates under an expanded access corporate policy, our reputation may be negatively affected and our business may be harmed.

Recent media attention to individual patients' expanded access requests has resulted in the introduction of legislation at the local and national level referred to as "Right to Try" laws, such as the Right to Try Act, which are intended to give patients access to unapproved therapies. New and emerging legislation regarding expanded access to unapproved drugs for life-threatening illnesses could negatively impact our business in the future.

A possible consequence of both activism and legislation in this area is the need for us to initiate an unanticipated expanded access program or to make our product candidates more widely available sooner than anticipated. We are a small company with limited resources and unanticipated trials or access programs could result in diversion of resources from our primary goals.

We and our contract manufacturers for plasmids and viruses are subject to significant regulation with respect to manufacturing our products. The third-party manufacturing facilities on which we rely, and any manufacturing facility that we may have in the future, may have limited capacity or fail to meet the applicable stringent regulatory requirements.

We currently have relationships with a limited number of suppliers for the manufacturing of plasmids and viruses, components of our product candidates. However, if we experience slowdowns or problems with our facility or those of our manufacturing partners and are unable to establish or scale our internal manufacturing capabilities, we will need to continue to contract with manufacturers that can produce the preclinical, clinical and commercial supply of our products. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain and we may be unable to license such intellectual property rights on reasonable commercial terms or to transfer or sublicense the intellectual property rights we may have with respect to such activities.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for components our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials in the European Union must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA or MAA on a timely basis. Our facilities and quality systems and the facilities and quality systems of some or all of our third-

party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted, and they could put a hold on one or more of our clinical trials if the facilities of our contract development and manufacturing organizations, or CDMOs, do not pass such audit or inspections. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, inspect or audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could harm our business. If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be harmed. Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. An alternative manufacturer would need to be qualified through a BLA and/or MAA supplement which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully, if approved. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

Any contamination or interruption in our manufacturing process, shortages of raw materials or failure of our suppliers of plasmids and viruses to deliver necessary components could result in delays in our clinical development or marketing schedules.

Given the nature of gene therapy manufacturing, there is a risk of contamination. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage. Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects.

If we or our collaborators encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the study until its conclusion. Any natural history studies that we may conduct may fail to provide us with patients for our clinical trials because patients enrolled in the natural history studies may not be good candidates for our clinical trials, or may choose to not

enroll in our clinical trials. We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials, and even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. The enrollment of patients depends on many factors, including:

- perceived risks and benefits of AAV-based gene therapy approaches for the potential treatment of neurological diseases;
- perceived risks of the delivery procedures;
- the size and nature of the patient population, and the severity and difficulty of diagnosing the disease under investigation;
- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial' s primary endpoints;
- the proximity of patients to study sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new products that may be approved for the indications we are investigating;
- patients with preexisting antibodies to the gene therapy vector that preclude their participation in the trial;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will drop out of the trials before completion.

In addition, our clinical trials may compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates or approved products for the same clinical indications, and this competition may reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which may reduce the number of patients who are available for our clinical trials in such clinical trial site.

Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop our product candidates, or could render further development impossible. If we or our collaborators have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which may have an adverse effect on our results of operations and prospects.

Our product candidates may cause serious adverse events or undesirable side effects or have other properties which may delay or prevent their regulatory approval, cause us to suspend or discontinue clinical trials, limit the commercial profile of an approved label, or, result in significant negative consequences following marketing approval, if any.

Serious adverse events or undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA, EMA or other authorities. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects, toxicities or unexpected characteristics, including death. Among the risks in any gene therapy product based on viral vectors are the risks of immunogenicity, elevated liver enzymes, and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation.

If unacceptable side effects or deaths arise in the development of our product candidates, we, the FDA, the IRBs at the institutions in which our studies are conducted, DSMB, EMA or CAT could suspend or terminate our clinical trials or the FDA, EMA or other regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. Undesirable side effects or deaths in clinical trials with our product candidates may cause the FDA or comparable foreign regulatory authorities to place a clinical hold on the associated clinical trials, to require additional studies, or otherwise to delay or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

If any of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by any such product, including during any long-term follow-up observation period recommended or required for patients who receive treatment using our products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may suspend, limit or withdraw approvals of such product, or seek an injunction against its manufacture and distribution;
- we may be required to recall a product or change the way such product is administered to patients;
- additional restrictions may be imposed on the marketing of the particular product or the manufacturing processes for the product;
- regulatory authorities may require additional warnings on the label, such as a boxed warning or contraindication, or issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- we may be required to implement a Risk Evaluation and Mitigation Strategy, or REMS, or create a medication guide outlining the risks of such side effects for distribution to patients;
- the products could become less competitive;
- we may be subject to fines, injunctions, or the imposition of civil or criminal penalties;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

Potential product liability lawsuits against us could cause us to incur substantial liabilities and limit commercialization of any products that we may develop.

The use of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, health care providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. On occasion, large judgments have been awarded in class action lawsuits based on products that had unanticipated adverse effects. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- impairment of our business reputation and significant negative media attention;

withdrawal of participants from our clinical trials;

significant costs to defend the related litigation and related litigation;

distraction of management' s attention from our primary business;

substantial monetary awards to patients or other claimants;

inability to commercialize our product candidates;

product recalls, withdrawals or labeling, marketing or promotional restrictions;

decreased demand for our product candidates, if approved for commercial sale; and

loss of revenue.

Positive results, if any, obtained in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials or other studies, and failure to replicate positive results from early studies may inhibit our ability to progress our clinical programs and develop and commercialize product candidates.

Results from previous preclinical studies or clinical trials are not necessarily predictive of future clinical trial results, and interim results of a clinical trial are not necessarily indicative of final results. Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials.

Positive results in preclinical testing and early clinical trials do not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. Frequently, product candidates that have shown promising results in early clinical trials have subsequently suffered significant setbacks in later clinical trials. In addition, the design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We have limited experience designing clinical trials and may be unable to design and execute a clinical trial to support regulatory approval. There is a high failure rate for drugs and biologic products proceeding through clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. These setbacks have been caused by, among other things, preclinical findings made while clinical trials were underway or safety or efficacy observations made in clinical trials, including previously unreported adverse events.

Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. Any such delays could negatively impact our business, financial condition, results of operations and prospects.

The regulatory approval processes of the FDA, EMA and other regulatory authorities are lengthy, time consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, on a timely basis or at all, our business will be substantially harmed.

The time required to obtain approval by the FDA, EMA and other regulatory authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate' s clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate and it is possible that none of our product candidates in clinical programs or any other product candidates we may seek to develop in the future will ever obtain regulatory approval. Neither we nor any

future collaborator is permitted to market any of our product candidates in the United States or the European Union until we receive regulatory approval of a BLA from the FDA or a MAA from the EMA, respectively. It is possible that the FDA may refuse to accept for substantive review any BLAs or the EMA any of our MAAs, that we submit for our product candidates or may conclude after review of our data that our application is insufficient to obtain marketing approval of our product candidates.

Our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate' s clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of a BLA or other submission or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies;
- the FDA or comparable foreign regulatory authorities may fail to approve the companion diagnostics we contemplate developing with partners; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Prior to obtaining approval to commercialize a product candidate in the United States, the European Union or elsewhere, we or our collaborators must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA, EMA or foreign regulatory agencies, that such product candidates are safe and effective for their intended uses. Results from nonclinical studies and clinical trials can be interpreted in different ways. Even if we believe the nonclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA, EMA or other regulatory authorities. The FDA or EMA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or post-approval, or it may object to elements of our clinical development program. Depending on the extent of these or any other FDA or EMA required studies, approval of any regulatory approval applications that we submit may be delayed by several years, or may require us to expend significantly more resources than we have available.

Of the large number of potential products in development, only a small percentage successfully complete the FDA, EMA or other foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, results of operations and prospects.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may impose significant limitations in the form

of narrow indications, warnings, or a REMS. Regulatory authorities may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

Even if we obtain FDA or EMA approval for PR001 or any other product candidates in the United States or European Union, we may never obtain approval for or commercialize any of them in any other jurisdiction, which would limit our ability to realize their full market potential.

In order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy.

Approval by the FDA in the United States or the EMA in the European Union does not ensure approval by regulatory authorities in other countries or jurisdictions. However, the failure to obtain approval in one jurisdiction may negatively impact our ability to obtain approval elsewhere. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country.

Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and increased costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including in international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized.

Even if we receive regulatory approval of our product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.

Any product candidate for which we obtain marketing approval will be subject to ongoing regulatory requirements for, among other things, manufacturing processes, submission of post-approval clinical data and safety information, labeling, packaging, distribution, adverse event reporting, storage, recordkeeping, export, import, advertising and promotional activities. These requirements include submissions of safety and other post-marketing information and reports, establishment registration and drug listing requirements, continued compliance with cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping and GCP requirements for any clinical trials that we conduct post-approval.

The FDA and EMA closely regulate the post-approval marketing and promotion of genetic therapy medicines to ensure they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. However, companies may share truthful and not misleading information that is otherwise consistent with a product' s FDA approved labeling. The FDA and EMA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we market our products for uses beyond their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the U.S. federal Food, Drug, and Cosmetic Act, or FDCA, relating to the promotion of prescription drugs for unapproved uses may lead to enforcement actions and investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on manufacturing such products;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters, or holds on clinical trials;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure or detention; or
- injunctions or the imposition of civil or criminal penalties.

The FDA' s policies, and the policies of foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. For example, in December 2016, the 21st Century Cures Act, or Cures Act, was signed into law. The Cures Act, among other things, is intended to modernize the regulation of biologics and spur innovation, but its ultimate implementation is unclear.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the Trump administration may impact our business and industry. Namely, the Trump administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, FDA' s ability to engage in routine oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. It is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA' s ability to exercise its regulatory authority. If these executive actions impose restrictions on FDA' s ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained which would adversely affect our business, prospects and ability to achieve or sustain profitability.

Interim "top-line" and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim "top-line" or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or

"top-line" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects.

Changes in funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel, accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. Our business depends upon the ability of the FDA to accept and review our potential regulatory filings. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our ability to advance clinical development of our product candidates.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to timely capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Risks Related to Healthcare Laws and Other Legal Compliance Matters

Enacted and future healthcare legislation may increase the difficulty and cost for us to progress our clinical programs and obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

In the United States, the European Union and other jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the ACA, was enacted, which substantially

changed the way healthcare is financed by both governmental and private insurers. Among the provisions of the ACA, those of greatest importance to the pharmaceutical and biotechnology industries include the following:

- an annual, non-deductible fee payable by any entity that manufactures or imports certain branded prescription drugs and biologic agents (other than those designated as orphan drugs), which is apportioned among these entities according to their market share in certain government healthcare programs;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer' s Medicaid rebate liability;
- a licensure framework for follow on biologic products;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and
- establishment of a Center for Medicare & Medicaid Innovation at the Centers for Medicare & Medicaid Services, or CMS, to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Since its enactment, there have been judicial, Congressional and executive branch challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. While Congress has not passed comprehensive repeal legislation, it has enacted laws that modify certain provisions of the ACA such as removing penalties, starting January 1, 2019, for not complying with the ACA' s individual mandate to carry health insurance, delaying the implementation of certain ACA-mandated fees, and increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D. On December 14, 2018, a U.S. District Court Judge in Texas ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, or the Tax Act. While such U.S. District Court Judge, as well as the Trump administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA and our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, led to aggregate reductions of Medicare payments to providers of 2% per fiscal year. These reductions went into effect in April 2013 and, due to subsequent legislative amendments to the statute will remain in effect through 2027 unless additional action is taken by Congress. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws or any other similar laws introduced in the future may result in additional reductions in Medicare and other health care funding, which could negatively affect our customers and accordingly, our financial operations.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, and review the relationship between pricing and manufacturer patient programs. The Trump administration' s budget proposal for fiscal year 2019 contains further drug price

control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Additionally, on May 11, 2018, President Trump laid out his administration' s "Blueprint" to lower drug prices and reduce out-of-pocket costs of prescription drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. The Department of Health and Human Services, or HHS, has already started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. On January 31, 2019, the HHS Office of Inspector General proposed modifications to U.S. federal Anti-Kickback Statute safe harbors which, among other things, will affect rebates paid by manufacturers to Medicare Part D plan sponsors, Medicaid managed care organizations and those entities' pharmacy benefit managers, the purpose of which is to further reduce the cost of drug products to consumers. Although some of these, and other, proposals may require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the EU or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved.

In markets outside of the United States and the European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our

product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers may be subject to applicable healthcare regulatory laws, which could expose us to penalties.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers, may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. Such laws include:

the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or providing any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under U.S. federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

the U.S. federal civil and criminal false claims and civil monetary penalties laws, including the civil False Claims Act, which can be enforced by private individuals on behalf of the government through civil whistleblower or qui tam actions, prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act;

the U.S. federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal civil and criminal liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 and their implementing regulations, which impose certain obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information without appropriate authorization by covered entities subject to the rule, such as health plans, healthcare clearinghouses and certain healthcare providers as well as their business associates, independent contractors of a covered entity that perform certain services involving the use or disclosure of individually identifiable health information;

federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;

the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;

the U.S. Public Health Service Act, which prohibits, among other things, the introduction into interstate commerce of a biological product unless a biologics license is in effect for that product;

the U.S. Physician Payments Sunshine Act and its implementing regulations, which require certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children' s Health Insurance Program, with specific exceptions, to report annually to CMS information related to certain payments and other transfers of value to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;

analogous U.S. state laws and regulations, including: state anti-kickback and false claims laws, which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry' s voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to drug pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities; state and local laws that require the registration of pharmaceutical sales and medical representatives; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts; and

similar healthcare laws and regulations in the European Union and other jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers and laws governing the privacy and security of personal data, including the General Data Protection Regulation, which imposes obligations and restrictions on the collection and use of personal data relating to individuals located in the E.U. and E.E.A. (including with regard to health data).

Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment, which could affect our ability to operate our business. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations. We can face serious consequences for violations.

U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations, or collectively, Trade Laws, prohibit, among other things, companies and their employees, agents, clinical research organizations, legal counsel, accountants, consultants, contractors, and other partners from

authorizing, promising, offering, providing, soliciting, or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of Trade Laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We also expect our non-U.S. activities to increase over time. We expect to rely on third parties for research, preclinical studies, and clinical trials and/or to obtain necessary permits, licenses, patent registrations, and other marketing approvals. We can be held liable for the corrupt or other illegal activities of our personnel, agents, or partners, even if we do not explicitly authorize or have prior knowledge of such activities.

We are subject to environmental, health and safety laws and regulations, and we may become exposed to liability and substantial expenses in connection with environmental compliance or remediation activities.

Our operations, including our development, testing and manufacturing activities, are subject to numerous environmental, health and safety laws and regulations. These laws and regulations govern, among other things, the controlled use, handling, release and disposal of and the maintenance of a registry for, hazardous materials and biological materials, such as chemical solvents, human cells, carcinogenic compounds, mutagenic compounds and compounds that have a toxic effect on reproduction, laboratory procedures and exposure to blood-borne pathogens. If we fail to comply with such laws and regulations, we could be subject to fines or other sanctions.

As with other companies engaged in activities similar to ours, we face a risk of environmental liability inherent in our current and historical activities, including liability relating to releases of or exposure to hazardous or biological materials. Environmental, health and safety laws and regulations are becoming more stringent. We may be required to incur substantial expenses in connection with future environmental compliance or remediation activities, in which case, the production efforts of our third-party manufacturers or our development efforts may be interrupted or delayed.

Risks Related to Commercialization

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.

Drug development, particularly in the gene therapy field, is highly competitive and subject to rapid and significant technological advancements. As a significant unmet medical need exists in the neurology field, particularly for the treatment of Parkinson’ s disease and other neurodegenerative diseases, there are several large and small pharmaceutical companies focused on delivering therapeutics for the treatment of these diseases. Further, it is likely that additional drugs will become available in the future for the treatment of our target indications.

We consider our most direct competitors with respect to PR001 to be companies developing GCase pathway-targeting therapies, including Sanofi Genzyme, a unit of Sanofi S.A., and Lysosomal Therapeutics, Inc. Sanofi Genzyme is developing SAR402671, a small molecule GluCer synthase inhibitor for the treatment of Parkinson’ s disease with a GBA mutation and for the treatment of Type 3 Gaucher disease in adult patients. In addition, Lysosomal Therapeutics, Inc. is developing LTI-291, a small molecule activator of the GCase enzyme, for the treatment of Parkinson’ s patients with a heterozygous mutation in the GBA gene. In addition to these investigational programs, there are several products targeting the GCase pathway that are approved or in development for Type 1 Gaucher disease, including approved enzyme replacement therapies, or ERTs, and substrate reduction therapies, or SRTs, but these ERTs and SRTs are not approved for neuronopathic Gaucher disease in the United States. There are other gene therapy companies that are attempting to use both AAV and lentiviral gene therapy approaches to treat Gaucher disease, but to our knowledge, none of those companies has

disclosed plans to pursue PD-GBA. Several companies are also developing therapies designed to prevent the progression of Parkinson' s disease and FTD. Examples include therapies in development by Alector, Biogen Inc., Denali Therapeutics Inc., Prothena Corporation plc and Roche Holding AG.

Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries. Our current and potential future competitors may also have significantly more experience commercializing drugs, particularly gene therapy and other biological products, that have been approved for marketing. Mergers and acquisitions in the pharmaceutical and biotechnology industries could result in even more resources being concentrated among a small number of our competitors.

We will face competition from other drugs or from other non-drug products currently approved or that will be approved in the future in the neurology field, including for the treatment of Parkinson' s disease. Therefore, our ability to compete successfully will depend largely on our ability to:

develop and commercialize drugs that are superior to other products in the market;

demonstrate through our clinical trials that our product candidates are differentiated from existing and future therapies;

attract qualified scientific, product development and commercial personnel;

obtain patent or other proprietary protection for our medicines;

obtain required regulatory approvals;

obtain coverage and adequate reimbursement from, and negotiate competitive pricing with, third-party payors; and

successfully collaborate with pharmaceutical companies in the discovery, development and commercialization of new medicines.

The availability of our competitors' products could limit the demand, and the price we are able to charge, for any product candidate we develop. The inability to compete with existing or subsequently introduced drugs would have an adverse impact on our business, financial condition and prospects. In addition, the reimbursement structure of approved gene therapies by other companies could impact the anticipated reimbursement structure of our gene therapies, if approved, and our business, financial condition, results of operations and prospects.

Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing, receiving regulatory and marketing approval for or commercializing drugs before we do, which would have an adverse impact on our business and results of operations.

The successful commercialization of our product candidates will depend in part on the extent to which governmental authorities and health insurers establish coverage, adequate reimbursement levels and pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

The availability of coverage and adequacy of reimbursement by governmental healthcare programs such as Medicare and Medicaid, private health insurers and other third-party payors are essential for most patients to be able to afford medical services and pharmaceutical products such as our product candidates, assuming FDA

approval. Our ability to achieve acceptable levels of coverage and reimbursement for our products or procedures using our products by governmental authorities, private health insurers and other organizations will have an effect on our ability to successfully commercialize our product candidates. Obtaining coverage and adequate reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. Separate reimbursement for the product itself or the treatment or procedure in which our product is used may not be available. A decision by a third-party payor not to cover or separately reimburse for our products or procedures using our products, could reduce physician utilization of our products once approved. Assuming there is coverage for our product candidates or procedures using our product candidates by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. We cannot be sure that coverage and reimbursement in the United States, the European Union or elsewhere will be available for our product candidates or any product that we may develop, and any reimbursement that may become available may not be adequate or may be decreased or eliminated in the future.

Third-party payors increasingly are challenging prices charged for pharmaceutical products and services, and many third-party payors may refuse to provide coverage and reimbursement for particular drugs or biologics when an equivalent generic drug, biosimilar or a less expensive therapy is available. It is possible that a third-party payor may consider our product candidates as substitutable and only offer to reimburse patients for the less expensive product. Even if we show improved efficacy or improved convenience of administration with our product candidates, pricing of existing third-party therapeutics may limit the amount we will be able to charge for our product candidates. These payors may deny or revoke the reimbursement status of a given product or establish prices for new or existing marketed products at levels that are too low to enable us to realize an appropriate return on our investment in our product candidates. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize our product candidates, and may not be able to obtain a satisfactory financial return on our product candidates.

There is significant uncertainty related to the insurance coverage and reimbursement of newly-approved products. In the United States, third-party payors, including private and governmental payors, such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered. The Medicare and Medicaid programs increasingly are used as models in the United States for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and biologics. Some third-party payors may require pre-approval of coverage for new or innovative devices or drug therapies before they will reimburse healthcare providers who use such therapies. We cannot predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

No uniform policy for coverage and reimbursement for products exists among third-party payors in the United States. Therefore, coverage and reimbursement for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Furthermore, rules and regulations regarding reimbursement change frequently, in some cases on short notice, and we believe that changes in these rules and regulations are likely.

Our product candidates and other gene therapies are designed to be single-dose treatments. Historically, chronic conditions such as Parkinson’ s disease and Gaucher disease have not had single-dose treatment options. Given the novelty of this treatment approach, significant uncertainty exists with respect to the pricing structure of gene therapies and the business model of pharmaceutical companies that do not have product candidates that require recurring purchases. If other companies establish a new pricing structure or business model, including payment based on demonstration of long term efficacy, our ability to price or obtain reimbursement for our products may be adversely affected. If such pricing structure or business model do not adequately fund the costs of our research and development, manufacturing and commercialization efforts, our business may be adversely affected.

Additionally, we or our collaborators may develop companion diagnostic tests for use with our product candidates as we are targeting certain genetically defined populations for our treatments. We, or our collaborators, will be required to obtain coverage and reimbursement for these tests separate and apart from the coverage and reimbursement we seek for our product candidates, once approved. While we, or our collaborators, have not yet developed any companion diagnostic test for our product candidates, if we do, there is significant uncertainty regarding our ability to obtain coverage and adequate reimbursement for the same reasons applicable to our product candidates.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe and other countries have and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially-reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of our product candidates due to the trend toward managed health care, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and biologics and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products.

Even if our product candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.

If our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If they do not achieve an adequate level of acceptance, we may not generate significant product revenues or become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including but not limited to:

- the efficacy and potential advantages compared to alternative treatments;
- effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments, including any similar generic treatments;
- our ability to offer our products for sale at competitive prices;
- the convenience and ease of administration;
- the availability of centers and medical professionals that can and will perform the applicable procedure;
- the frequency of genotyping in medical practice;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of third-party payor coverage and adequate reimbursement;

product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product' s approved labeling;

the incidence and severity of any side effects; and

any restrictions on the use of our product together with other medications.

Because we expect sales of our product candidates, if approved, to generate substantially all of our product revenues, the failure of our product candidates to find market acceptance would harm our business and could require us to seek additional financing.

If we are unable to establish sales, marketing and distribution capabilities either on our own or in collaboration with third parties, we may not be successful in commercializing our product candidates or realizing the synergies in the target indications of our programs, even if they are approved.

We do not have any infrastructure for the sales, marketing or distribution of our products, and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. We expect to build a focused sales, distribution and marketing infrastructure to market our product candidates in the United States and European Union, if approved. There are significant expenses and risks involved with establishing our own sales, marketing and distribution capabilities, including our ability to hire, retain and appropriately incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities could delay any product launch, which would adversely impact the commercialization of our product candidates. Additionally, if the commercial launch of our product candidates for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

We may not have the resources in the foreseeable future to allocate to the sales and marketing of our product candidates in certain international markets. Therefore, our future sales in these markets will largely depend on our ability to enter into and maintain collaborative relationships for such capabilities, the collaborator' s strategic interest in the product and such collaborator' s ability to successfully market and sell the product. We may pursue collaborative arrangements regarding the sale and marketing of PR001, PR006 and PR004, if approved, for certain markets overseas; however, we cannot assure that we will be able to establish or maintain such collaborative arrangements, or if able to do so, that they will have effective sales forces.

If we are unable to build our own sales force or negotiate a collaborative relationship for the commercialization of PR001 or any of our other product candidates, if approved, we may be forced to delay the potential commercialization of PR001 or any of our other product candidatesor reduce the scope of our sales or marketing activities for PR001 or any of our other product candidates. If we elect to increase our expenditures to fund commercialization activities internationally, we will need to obtain additional capital, which may not be available to us on acceptable terms, or at all. We could enter into arrangements with collaborative partners at an earlier stage than otherwise would be ideal and we may be required to relinquish rights to PR001 or any of our other product candidates or otherwise agree to terms unfavorable to us, any of which may have an adverse effect on our business, operating results and prospects.

If we are unable to establish adequate sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing PR001 or any of our other product candidates, if approved, and may not become profitable and may incur significant additional losses. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

If we obtain approval to commercialize any products outside of the United States or the European Union, a variety of risks associated with international operations could adversely affect our business.

If PR001 or any of our other product candidates are approved for commercialization, we may seek to enter into agreements with third parties to market them in certain jurisdictions outside the United States and the European Union. We expect that we would be subject to additional risks related to international pharmaceutical operations, including:

- different regulatory requirements for drug and biologic approvals and rules governing drug and biologic commercialization in foreign countries;
- reduced protection for intellectual property rights;
- foreign reimbursement, pricing and insurance regimes;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires, or from economic or political instability;
- greater difficulty with enforcing our contracts;
- potential noncompliance with the U.S. Foreign Corrupt Practices Act, the U.K. Bribery Act 2010 and similar anti-bribery and anticorruption laws in other jurisdictions; and
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad.

We have no prior experience in these areas. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by individual countries in Europe with which we will need to comply. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.

The ACA includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor' s own pre-clinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have an adverse effect on the future commercial prospects for our biological products.

There is a risk that any of our product candidates approved as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to congressional

action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. If competitors are able to obtain marketing approval for biosimilars referencing our candidates, if approved, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and potential adverse consequences.

Risks Related to Our Dependence on Third Parties

We currently contract with third parties for the manufacture of plasmids and viruses used in producing our product candidates. Relying on third parties increases the risk that we will not have sufficient quantities of such materials, product candidates, or any therapeutics that we may develop and commercialize, or that such supply will not be available to us at an acceptable cost, which could delay, prevent, or impair our clinical development or commercialization efforts.

We currently rely on third-party manufacturers for the manufacture of plasmids and viruses used in the production of our product candidates. We do not have a long term supply agreement with any of the third-party manufacturers, and we purchase our required supply on a purchase order basis.

We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

the possible breach of the manufacturing agreement by the third party;

the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us;

delays due to limited supply or capacity of production facilities and/or failure to meet standards for quality; and

reliance on the third party for regulatory compliance, quality assurance, safety, and pharmacovigilance and related reporting.

Third-party manufacturers may not be able to comply with cGMP regulations or other regulatory requirements that might be required by the FDA or EMA. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocations, seizures or recalls of product candidates or medicines, operating restrictions, and criminal prosecutions, any of which could adversely affect supplies of our candidates and harm our business, financial condition, results of operations and prospects.

Any therapies that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval.

Our current and anticipated future dependence upon others for the manufacture of any product candidates we may develop or any components required for the manufacture of our product candidates may adversely affect our ability to meet our clinical timelines, our future profit margins and our ability to commercialize any product candidates that receive marketing approval on a timely and competitive basis.

We may collaborate with third parties for the development and commercialization of our product candidates. We may not succeed in establishing and maintaining collaborative relationships, and our collaborators may fail to perform satisfactorily, which may significantly limit our ability to develop and commercialize our product candidates successfully, if at all.

We may seek collaborative relationships for the development and commercialization of our product candidates. Failure to establish collaborative relationships for our product candidates may significantly impair their commercial potential. We also may need to enter into collaborative relationships to provide funding to support our other research and development programs. The process of establishing and maintaining collaborative relationships is difficult, time-consuming and involves significant uncertainty, such as:

- a collaboration partner may shift its priorities and resources away from our product candidates due to a change in business strategies, or a merger, acquisition, sale or downsizing;
- a collaboration partner may seek to renegotiate or terminate their relationships with us due to unsatisfactory clinical results, manufacturing issues, a change in business strategy, a change of control or other reasons;
- a collaboration partner may cease development in therapeutic areas which are the subject of our strategic collaboration;
- a collaboration partner may not devote sufficient capital or resources towards our product candidates;
- a collaboration partner may change the success criteria for a product candidate thereby delaying or ceasing development of such candidate;
- a significant delay in initiation of certain development activities by a collaboration partner will also delay payment of milestones tied to such activities, thereby impacting our ability to fund our own activities;
- a collaboration partner may fail to comply with applicable regulatory requirements, thereby jeopardizing our ability to successfully develop and seek approval for our product candidates, on a timely basis or at all, or otherwise exposing us to potential liability;
- a collaboration partner could develop a product that competes, either directly or indirectly, with our product candidate;
- a collaboration partner with commercialization obligations may not commit sufficient financial or human resources to the marketing, distribution or sale of a product;
- a collaboration partner with manufacturing responsibilities may encounter regulatory, resource or quality issues and be unable to meet demand requirements;
- a collaboration partner may terminate a strategic alliance;
- a dispute may arise between us and a partner concerning the research, development or commercialization of a product candidate resulting in a delay in milestones, royalty payments or termination of an alliance and possibly resulting in costly litigation or arbitration which may divert management attention and resources; and
- a partner may use our products or technology in such a way as to make us subject to litigation with a third party.

If any collaborator fails to fulfill its responsibilities in a timely manner, or at all, our research, clinical development, manufacturing or commercialization efforts related to that collaboration could be delayed or terminated, or it may be necessary for us to assume responsibility for expenses or activities that would otherwise have been the responsibility of our collaborator. If we are unable to establish and maintain collaborative relationships on acceptable terms or to successfully transition terminated collaborative agreements, we may have to delay or discontinue further development of one or more of our product candidates, undertake development

and commercialization activities at our own expense or find alternative sources of capital. Moreover, any collaborative partners we enter into agreements with in the future may shift their priorities and resources away from our product candidates or seek to renegotiate or terminate their relationships with us.

We have relied, and we expect to continue to rely on third parties to conduct, supervise and monitor our clinical trials, and if these third parties perform in an unsatisfactory manner or are unable to perform in a timely manner, our business could be harmed.

We expect to rely on CROs and clinical trial sites to ensure our clinical trials are conducted properly and on time. We may also engage third parties such as clinical data management organizations, medical institutions and clinical investigators to conduct or assist in our clinical trials or other clinical development work. While we will have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our third-party service providers' activities. Nevertheless, we will be responsible for ensuring that each of our clinical studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards. Our reliance on these third-parties does not relieve us of our regulatory responsibilities. If any locations terminate the clinical trial, we would be required to find another party to conduct any new trials. We may be unable to find a new party to conduct new trials of our product candidates or obtain clinical supply of our product candidates or AAV vectors for such trials.

We and our third-party service providers are required to comply with the FDA' s GCPs for conducting, recording and reporting the results of IND-enabling studies and clinical studies to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. We are also required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. The FDA enforces these GCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites at which the FDA may determine that our clinical trials did not comply with GCPs. If we or our third-party service providers fail to comply with applicable GCPs, the clinical data generated in our future clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving any marketing applications. In addition, our future clinical trials will require a sufficient number of patients to evaluate the safety and effectiveness of our product candidates. Accordingly, if we or our third-party service providers fail to comply with these regulations or fail to recruit a sufficient number of patients, we may be required to repeat such clinical trials, which would delay the regulatory approval process. Failure to comply can also result in fines, adverse publicity, and civil and criminal sanctions.

Our third-party service providers are not our employees, and we are therefore unable to directly monitor whether or not they devote sufficient time and resources to our clinical and nonclinical programs. These third-party service providers may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If our third-party service providers do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines or comply with applicable regulatory requirements, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

Risks Related to Intellectual Property

We depend on proprietary technology licensed from others. If we lose our existing licenses or are unable to acquire or license additional proprietary rights from third parties, we may not be able to continue developing our product candidates.

We currently in-license certain intellectual property from REGENXBIO. We are a party to agreements with REGENXBIO for certain technology and AAV9 vector-related patents, and we may enter into additional agreements, including license agreements, with other parties in the future that impose diligence, development and commercialization timelines, milestone payments, royalties, insurance and other obligations on us. For example, in exchange for the rights granted to us by REGENXBIO, we are obligated to pay an annual fee, certain royalty percentages on net sales of licensed products, and certain percentages of proceeds on sublicensing fees. We are also obligated to achieve certain development milestones with respect to licensed products in our fields of use within specified time periods. If we fail to comply with our obligations to REGENXBIO or any of our other current or future collaborators, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market any product candidate that is covered by these agreements, which could adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in us having to negotiate new or reinstated agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

We may rely on third parties from whom we license proprietary technology to file and prosecute patent applications and maintain patents and otherwise protect the intellectual property we license from them. We may have limited control over these activities or any other intellectual property that may be related to our in-licensed intellectual property. For example, we cannot be certain that such activities by these licensors will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. We may have limited control over the manner in which our licensors initiate an infringement proceeding against a third-party infringer of the intellectual property rights, or defend certain of the intellectual property that may be licensed to us. It is possible that the licensors' infringement proceeding or defense activities may be less vigorous than if we conduct them ourselves. The licensing and acquisition of third-party intellectual property rights is a competitive practice, and companies that may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their larger size and cash resources or greater clinical development and commercialization capabilities. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire. Furthermore, we may be unable to in-license any compositions, methods of use, processes, or other third-party intellectual property rights from third parties, which we identify as necessary for our product candidates.

If we are unable to obtain and maintain patent protection for our technology and product candidates or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

We rely, and will continue to rely, upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our proprietary technologies, product candidate development programs and product candidates. Our success depends in large part on our ability to secure and maintain patent protection in the United States and other countries with respect to our current product candidates and any future product candidates we may develop. We seek to protect our proprietary position by filing or collaborating with our licensors to file patent applications in the United States and abroad related to our proprietary technologies, development programs and product candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent

applications at a reasonable cost or in a timely manner. Moreover, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing, and prosecution of patent applications, or to maintain the rights to patents licensed to third parties. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our proprietary products and technology, including current product candidates, any future product candidates we may develop, and our gene regulation technology in the United States or in other foreign countries, in whole or in part. Alternately, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing products and technologies. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can prevent a patent from issuing from a pending patent application or later invalidate or narrow the scope of an issued patent. For example, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Even if patents do successfully issue and even if such patents cover our current product candidates, any future product candidates we may develop and our gene regulation technology, third parties may challenge their validity, ownership, enforceability or scope thereof, which may result in such patents being narrowed, invalidated, or held unenforceable or circumvented. Any successful challenge to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any of our product candidates or gene regulation technology. In addition, the issuance of a patent does not give us the right to practice the patented invention. Third parties may have blocking patents that could prevent us from marketing our product candidate, if approved, or practicing our own patented technology. Our competitors may be able to circumvent our patents by developing similar or alternative product candidates in a non-infringing manner. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate and our gene regulation technology under patent protection could be reduced. If any of our patents expire or are challenged, invalidated, circumvented or otherwise limited by third parties prior to the commercialization of our product candidate, and if we do not own or have exclusive rights to other enforceable patents protecting our product candidate or technologies, competitors and other third parties could market products and use processes that are substantially similar, or superior, to ours and our business would suffer.

If the patent applications we hold or have in-licensed with respect to our development programs and product candidates fail to issue, if their validity, breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for any of our current or future product candidates or technology, it could dissuade companies from collaborating with us to develop product candidates, encourage competitors to develop competing products or technologies and threaten our ability to commercialize future product candidates. Any such outcome could harm our business.

We are a party to intellectual property license agreements with REGENXBIO which are important to our business, and we expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, royalties and other obligations on us. See “Business−Licensing Agreements.” If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, or, in some cases, under other circumstances, the licensor may have the right to terminate the license, in which event we would not be able to market product candidate(s) covered by the license. In addition, certain of these license agreements are not assignable by us without the consent of the respective licensor, which may have an adverse effect on our ability to engage in certain transactions.

The patent position of biotechnology and pharmaceutical companies is generally highly uncertain, involves complex legal, scientific and factual questions, and is characterized by the existence of large numbers of patents and frequent litigation based on allegations of patent or other intellectual property infringement or violation. The standards that the U.S. Patent and Trademarks Office, or the USPTO, and its foreign counterparts use to grant patents are not always applied predictably or uniformly. In addition, the laws of jurisdictions outside the United States may not protect our rights to the same extent as the laws of the United States, and many companies have encountered significant problems in protecting and defending such rights in foreign jurisdictions. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. Since patent applications in the United States and other jurisdictions are confidential for a period of time after filing, we cannot be certain that we were the first to file for patents covering our inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in the issuance of patents, or may result in the issuance of patents which fail to protect our technology or products, in whole or in part, or which fail to effectively prevent others from commercializing competitive technologies and products.

The issuance of a patent is not conclusive as to its inventorship, ownership, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. We may become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our owned or licensed patent rights. For example, with respect to our licensed patents and patent applications from REGENXBIO, competitors may claim that they invented the inventions claimed in our issued patents or patent applications prior to the inventors of our licensed patents, or may have filed patent applications before the Trustees of the University of Pennsylvania, or UPenn, as owner of the patent rights licensed by us from REGENXBIO. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. Thus, even if our patent applications issue as patents, they may not issue in a form that will provide us with meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however the life of a patent, and the protection it affords, is limited. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Third parties may assert claims against us alleging infringement of their patents and proprietary rights, or we may need to become involved in lawsuits to defend or enforce our patents, either of which could result in substantial costs or loss of productivity, delay or prevent the development and commercialization of our product candidates, prohibit our use of proprietary technology or sale of products or put our patents and other proprietary rights at risk.

Our commercial success depends, in part, upon our ability to develop, manufacture, market and sell our product candidates without alleged or actual infringement, misappropriation or other violation of the patents and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Litigation relating to infringement or misappropriation of patent and other intellectual

property rights in the pharmaceutical and biotechnology industries is common, including patent infringement lawsuits, interferences, derivation and administrative law proceedings, inter partes review and post-grant review before the USPTO, as well as oppositions and similar processes in foreign jurisdictions. The various markets in which we plan to operate are subject to frequent and extensive litigation regarding patents and other intellectual property rights. Our competitors in both the United States and abroad, many of which have substantially greater resources and have made substantial investments in patent portfolios and competing technologies, may have applied for or obtained or may in the future apply for or obtain, patents that will prevent, limit or otherwise interfere with our ability to make, use and sell, if approved, our product candidates. In addition, many companies in intellectual property-dependent industries, including the biotechnology and pharmaceutical industries, have employed intellectual property litigation as a means to gain an advantage over their competitors. Numerous U.S., EU and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing product candidates, and as the biotechnology and pharmaceutical industries expand and more patents are issued, and as we gain greater visibility and market exposure as a public company, the risk increases that our product candidates may be subject to claims of infringement of the intellectual property rights of third parties. Some claimants may have substantially greater resources than we do and may be able to sustain the costs of complex intellectual property litigation to a greater degree and for longer periods of time than we could. In addition, patent holding companies that focus solely on extracting royalties and settlements by enforcing patent rights may target us.

We may be subject to third-party claims including patent infringement, interference or derivation proceedings, post-grant review and inter partes review before the USPTO or similar adversarial proceedings or litigation in other jurisdictions. Even if such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, and the holders of any such patents may be able to block our ability to commercialize the applicable product candidate unless we obtained a license under the applicable patents, or until such patents expire or are finally determined to be invalid or unenforceable. There may be third-party patents or patent applications with claims to compositions, formulations, or methods of treatment, prevention use, or manufacture of our product candidates or technologies. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover aspects of our compositions, formulations, or methods of treatment, prevention or use, the holders of any such patents may be able to prohibit our use of those compositions, formulations, methods of treatment, prevention or use or other technologies, effectively blocking our ability to progress the clinical development of or commercialize the applicable product candidate until such patent expires or is finally determined to be invalid or unenforceable or unless we obtained a license.

We also may be subject to third party claims arising from consulting agreements entered into by our officers, employees, independent contractors and/or consultants. Claims may include breach of nondisclosure, nonuse, noncompetition and non-solicitation provisions, intellectual property assignment and ownership, and misuse or misappropriation of intellectual property, trade secrets and other confidential information, among others. If a court of competent jurisdiction finds that we breached the provisions of third party consulting agreements, we may be prohibited from using certain intellectual property, trade secrets and confidential information, effectively blocking our ability to seek patent protection for our inventions and halting the progress of our clinical development and commercialization efforts.

For example, on June 7, 2019, we received a letter on behalf of Alector, a biopharmaceutical company employing antibodies for the treatment of neurodegeneration, stating concerns regarding whether confidential information of Alector was used in connection with work on behalf of our company and patents and patent applications filed on behalf of our company, as well as alleging that Alector has certain rights to our patents and patent applications. We believe these allegations of wrongdoing and Alector’ s claims of rights to any of our intellectual property are without basis or merit, as our gene therapy programs and underlying patents and patent applications were based on work done by Dr. Abeliovich derived from publicly available information or from work outside of and wholly separate from any matters on which he consulted for Alector or information he

received while consulting for Alector. We intend to vigorously defend any claim or lawsuit making allegations relating to these matters, however, there can be no assurance regarding any resolution or the outcome of these matters.

In addition, defending such claims would cause us to incur substantial expenses and, if successful, could cause us to pay substantial damages if we are found to be infringing a third party' s intellectual property rights. These damages potentially include increased damages and attorneys' fees if we are found to have infringed such rights willfully. Further, if a patent infringement suit is brought against us or our third-party service providers, our development, manufacturing or sales activities relating to the product or product candidate that is the subject of the suit may be delayed or terminated, as parties making claims against us may obtain injunctive or other equitable relief. As a result of patent infringement claims, or in order to avoid potential infringement claims, we may choose to seek, or be required to seek, a license from the third party, which may require payment of substantial royalties or fees, or require us to grant a cross-license under our intellectual property rights. These licenses may not be available on reasonable terms or at all. Even if a license can be obtained on reasonable terms, the rights may be nonexclusive, which would give our competitors access to the same intellectual property rights. If we are unable to enter into a license on acceptable terms, we could be prevented from commercializing one or more of our product candidates, or forced to modify such product candidates, or to cease some aspect of our business operations, which could harm our business significantly. We might also be forced to redesign or modify our product candidates so that we no longer infringe the third-party intellectual property rights, which may result in significant cost or delay to us, or which redesign or modification could be impossible or technically infeasible. Even if we were ultimately to prevail, any of these events could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business. Intellectual property litigation, regardless of its outcome, may cause negative publicity, adversely impact prospective customers, cause product shipment delays, or prohibit us from manufacturing, importing, marketing or otherwise commercializing our products, services and technology. In addition, if the breadth or strength of protection provided the patents and patent applications we own or in-license is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Competitors may infringe our patents or other intellectual property. If we or one of our licensors were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that our patent is invalid or unenforceable. In patent litigation in the United States and in Europe, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, obviousness lack of written description, or non-enablement. Third parties might allege unenforceability of our patents because during prosecution of the patent an individual connected with such prosecution withheld relevant information, or made a misleading statement. Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications. The outcome of proceedings involving assertions of invalidity and unenforceability during patent litigation is unpredictable. With respect to the validity of patents, for example, we cannot be certain that there is no invalidating prior art of which we and the patent examiner were unaware during prosecution, but that an adverse third party may identify and submit in support of such assertions of invalidity. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Our patents and other intellectual property rights also will not protect our technology if competitors design around our protected technology without infringing our patents or other intellectual property rights.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments, and if securities analysts or investors view these announcements in a negative light, the price of our common stock could be adversely affected. Such litigation or proceedings could substantially increase our operating losses and reduce our resources available for development activities. We may not have sufficient financial or

other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have an adverse effect on our ability to compete in the marketplace.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop, manufacture and market our product candidates.

We cannot guarantee that any of our or our licensors' patent searches or analyses, including but not limited to the identification of relevant patents, analysis of the scope of relevant patent claims or determination of the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States, Europe and elsewhere that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction. For example, in the United States, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Patent applications in the United States, the European Union and elsewhere are published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Therefore, patent applications covering our product candidates could be filed by others without our knowledge. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our product candidates or the use of our product candidates. After issuance, the scope of patent claims remains subject to construction as determined by an interpretation of the law, the written disclosure in a patent and the patent' s prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect, which may negatively impact our ability to market our product candidates. We may incorrectly determine that our product candidates are not covered by a third-party patent or may incorrectly predict whether a third party' s pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States, the European Union or elsewhere that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our product candidates. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our product candidates, if approved.

If we fail to identify or correctly interpret relevant patents, we may be subject to infringement claims. We cannot guarantee that we will be able to successfully settle or otherwise resolve such infringement claims. If we fail in any such dispute, in addition to being forced to pay monetary damages, we may be temporarily or permanently prohibited from commercializing our product candidates. We might, if possible, also be forced to redesign our product candidates in a manner that no longer infringes third-party intellectual property rights. Any of these events, even if we were ultimately to prevail, could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.

We currently depend, and will continue to depend, on our license agreements, including our agreements with REGENXBIO, whereby we obtain rights in certain patents and patent applications owned by UPenn. Further development and commercialization of our current or any future product candidates may require us to enter into additional license or collaboration agreements, including, potentially, additional agreements with REGENXBIO or any of our other licensors. The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

If any of our licenses or material relationships or any in-licenses upon which our licenses are based including the underlying agreements between REGENXBIO and UPenn are terminated or breached, we may:

lose our rights to develop and market our products;

lose patent protection for our products;

experience significant delays in the development or commercialization of our products;

not be able to obtain any other licenses on acceptable terms, if at all; or

incur liability for damages.

These risks apply to any agreements that we may enter into in the future for our products or for any future product candidates. If we experience any of the foregoing, it could have a material adverse effect on our business, financial condition, results or operations and prospects.

Changes in patent laws or patent jurisprudence could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology and genetic medicine industries involve both technological and legal complexity. Therefore, obtaining and enforcing biotechnology and genetic medicine patents is costly, time-consuming and inherently uncertain. In addition, the Leahy-Smith America Invents Act, or the AIA, which was passed in September 2011, resulted in significant changes to the U.S. patent system.

An important change introduced by the AIA is that, as of March 16, 2013, the United States transitioned from a "first-to-invent" to a "first-to-file" system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. Under a "first-to-file" system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. A third party that files a patent application in the USPTO after that date but before us could therefore be awarded a patent covering an invention of ours even if we made the invention before it was made by the third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application and be diligent in filing patent applications, but circumstances could prevent us from promptly filing patent applications on our inventions.

Among some of the other changes introduced by the AIA are changes that limit where a patentee may file a patent infringement suit and providing opportunities for third parties to challenge any issued patent in the USPTO. This applies to all of our U.S. patents, even those issued before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action.

Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. It is not clear what, if any, impact the AIA will have on the operation of our business. However, the AIA and its implementation could increase the uncertainties and costs surrounding the prosecution of our or our licensors' patent applications and the enforcement or defense of our or our licensors' issued patents.

We may become involved in opposition, interference, derivation, inter partes review or other proceedings challenging our or our licensors' patent rights, and the outcome of any proceedings are highly uncertain. An adverse determination in any such proceeding could reduce the scope of, or invalidate, our owned or in-licensed patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

In addition, the United States federal government retains certain rights in inventions produced with its financial assistance under the Bayh-Dole Act. The federal government retains a "nonexclusive, nontransferable, irrevocable, paid-up license" for its own benefit. The Bayh-Dole Act also provides federal agencies with "march-in rights". March-in rights allow the government, in specified circumstances, to require the contractor or successors in title to the patent to grant a "nonexclusive, partially exclusive, or exclusive license" to a "responsible applicant or applicants." If the patent owner refuses to do so, the government may grant the license itself. Some of our licensed patents are subject to the provisions of the Bayh-Dole Act. If our licensors fail to comply with the regulations of the Bayh-Dole Act, they could lose title to any patents subject to such regulations, which could affect our license rights under the patents and our ability to stop others from using or commercializing similar or identical technology and products, or limit patent protection for our technology and products.

Additionally, the U.S. Supreme Court has ruled on several patent cases in recent years either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations, and there are other open questions under patent law that courts have yet to decisively address. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways and could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. In addition, the European patent system is relatively stringent in the type of amendments that are allowed during prosecution, but, the complexity and uncertainty of European patent laws has also increased in recent years. Complying with these laws and regulations could limit our ability to obtain new patents in the future that may be important for our business.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

The USPTO, European and other patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In addition, periodic maintenance and annuity fees on any issued patent are due to be paid to the USPTO, European and other patent agencies over the lifetime of the patent. While an inadvertent failure to make payment of such fees or to comply with such provisions can in many cases be cured by additional payment of a late fee or by other means in accordance with the applicable rules, there are situations in which non-compliance with such provisions will result in the abandonment or lapse of the patent or patent application, and the partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents within prescribed time limits. If we or our licensors fail to maintain the patents and patent applications covering our product candidates or if we or our licensors otherwise allow our patents or patent applications to be abandoned or lapse, it can create opportunities for competitors to enter the market, which would hurt our competitive position and could impair our ability to successfully progress clinical development of or commercialize our product candidates in any indication for which they may be approved.

We enjoy only limited geographical protection with respect to certain patents and we may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents covering our product candidates in all countries throughout the world would be prohibitively expensive, and even in countries where we have sought protection for our intellectual property, such protection can be less extensive than those in the United States. The requirements for patentability may differ in certain countries, particularly developing countries, and the breadth of patent claims allowed can be inconsistent. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. In-licensing patents covering our product

candidates in all countries throughout the world may similarly be prohibitively expensive, if such opportunities are available at all. And in-licensing or filing, prosecuting and defending patents even in only those jurisdictions in which we develop or commercialize our product candidates may be prohibitively expensive or impractical. Competitors may use our and our licensors' technologies in jurisdictions where we have not obtained patent protection or licensed patents to develop their own products and, further, may export otherwise infringing products to territories where we and our licensors have patent protection, but where enforcement is not as strong as that in the United States or the European Union. These products may compete with our product candidates, and our or our licensors' patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

In addition, we may decide to abandon national and regional patent applications while they are still pending. The grant proceeding of each national or regional patent is an independent proceeding which may lead to situations in which applications may be rejected by the relevant patent office, while substantively similar applications are granted by others. For example, relative to other countries, China has a heightened requirement for patentability and specifically requires a detailed description of medical uses of a claimed drug. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our or our licensors' patents, requiring us or our licensors to engage in complex, lengthy and costly litigation or other proceedings. Generic drug manufacturers may develop, seek approval for and launch generic versions of our products. It is also quite common that depending on the country, the scope of patent protection may vary for the same product candidate or technology.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws or regulations in the United States and the European Union, and many companies have encountered significant difficulties in protecting and defending proprietary rights in such jurisdictions. Moreover, the legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets or other forms of intellectual property, particularly those relating to biotechnology products, which could make it difficult for us to prevent competitors in some jurisdictions from marketing competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, are likely to result in substantial costs and divert our efforts and attention from other aspects of our business, and additionally could put at risk our or our licensors' patents of being invalidated or interpreted narrowly, could increase the risk of our or our licensors' patent applications not issuing, or could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, while damages or other remedies may be awarded to the adverse party, which may be commercially significant. If we prevail, damages or other remedies awarded to us, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license. Furthermore, while we intend to protect our intellectual property rights in our expected significant markets, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our product candidates. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate, which may have an adverse effect on our ability to successfully commercialize our product candidates in all of our expected significant foreign markets. If we or our licensors encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished and we may face additional competition in those jurisdictions.

In some jurisdictions including European Union countries, compulsory licensing laws compel patent owners to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors are forced to grant a license to third parties under patents relevant to our business, or if we or our licensors are prevented from enforcing patent rights against third parties, our competitive position may be substantially impaired in such jurisdictions.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

The term of any individual patent depends on applicable law in the country where the patent is granted. In the United States, provided all maintenance fees are timely paid, a patent generally has a term of 20 years from its application filing date or earliest claimed non-provisional filing date. Extensions may be available under certain circumstances, but the life of a patent and, correspondingly, the protection it affords is limited. Even if we or our licensors obtain patents covering our product candidates, when the terms of all patents covering a product expire, our business may become subject to competition from competitive medications, including generic medications. Given the amount of time required for the development, testing and regulatory review and approval of new product candidates, patents protecting such candidates may expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain patent term extension in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of marketing exclusivity for our product candidates, our business may be harmed.

In the United States, a patent that covers an FDA-approved drug or biologic may be eligible for a term extension designed to restore the period of the patent term that is lost during the premarket regulatory review process conducted by the FDA. Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, which permits a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, and only claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. In the European Union, our product candidates may be eligible for term extensions based on similar legislation. In either jurisdiction, however, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Even if we are granted such extension, the duration of such extension may be less than our request. If we are unable to obtain a patent term extension, or if the term of any such extension is less than our request, the period during which we can enforce our patent rights for that product will be in effect shortened and our competitors may obtain approval to market competing products sooner. The resulting reduction of years of revenue from applicable products could be substantial.

Our proprietary rights may not adequately protect our technologies and product candidates, and do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make products that are the same as or similar to our product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed;
- others, including inventors or developers of our owned or in-licensed patented technologies who may become involved with competitors, may independently develop similar technologies that function as alternatives or replacements for any of our technologies without infringing our intellectual property rights;
- we or our licensors or our other collaboration partners might not have been the first to conceive and reduce to practice the inventions covered by the patents or patent applications that we own, license or will own or license;

we or our licensors or our other collaboration partners might not have been the first to file patent applications covering certain of the patents or patent applications that we or they own or have obtained a license, or will own or will have obtained a license;

we or our licensors may fail to meet obligations to the U.S. government with respect to in-licensed patents and patent applications funded by U.S. government grants, leading to the loss of patent rights;

it is possible that our pending patent applications will not result in issued patents;

it is possible that there are prior public disclosures that could invalidate our or our licensors' patents;

issued patents that we own or exclusively license may not provide us with any competitive advantage, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;

we may not exclusively license our patents and, therefore, may not have a competitive advantage if such patents are licensed to others, including for example, under our license agreements with REGENXBIO, pursuant to which REGENXBIO and its upstream licensors (SmithKline Beecham Corporation, or GSK, and UPenn) retain the exclusive right over certain antibodies expressed by AAV9 and GSK and UPenn retain a non-exclusive right over products that deliver RNA interference and antisense drugs using AAV9;

our competitors might conduct research and development activities in countries where we do not have patent rights, or in countries where research and development safe harbor laws exist, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;

ownership, validity or enforceability of our or our licensors' patents or patent applications may be challenged by third parties; and

the patents of third parties or pending or future applications of third parties, if issued, may have an adverse effect on our business.

Our reliance on third parties may require us to share our trade secrets, which increases the possibility that our trade secrets will be misappropriated or disclosed, and confidentiality agreements with employees and third parties may not adequately prevent disclosure of trade secrets and protect other proprietary information.

We consider proprietary trade secrets, confidential know-how and unpatented know-how to be important to our business. We may rely on trade secrets and confidential know-how to protect our technology, especially where patent protection is believed by us to be of limited value. However, trade secrets and confidential know-how are difficult to protect, and we have limited control over the protection of trade secrets and confidential know-how used by our licensors, collaborators and suppliers. Because we have relied in the past on third parties to manufacture our product candidates, because we may continue to do so in the future, and because we expect to collaborate with third parties on the development of our current product candidates and any future product candidates we develop, we may, at times, share trade secrets with them. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. Under such circumstances, trade secrets and confidential know-how can be difficult to maintain as confidential.

To protect this type of information against disclosure or appropriation by competitors, our policy is to require our employees, consultants, contractors and advisors to enter into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with us prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. However, current or former employees, consultants, contractors and advisers may unintentionally or willfully disclose our confidential information to competitors, and confidentiality agreements may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. The need to share trade secrets and other confidential

information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our competitive position is based, in part, on our know-how and trade secrets, a competitor' s discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have an adverse effect on our business and results of operations. Enforcing a claim that a third party obtained illegally and is using trade secrets and/or confidential know-how is expensive, time consuming and unpredictable, and the enforceability of confidentiality agreements may vary from jurisdiction to jurisdiction. Courts outside the United States are sometimes less willing to protect proprietary information, technology and know-how.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor' s discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our unregistered trademarks or trade names. Over the long term, if we are unable to successfully register our trademarks and trade names and establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

We may need to license additional intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

The growth of our business may depend in part on our ability to acquire or in-license additional proprietary rights. For example, our programs may involve product candidates that may require the use of additional proprietary rights held by third parties. Our product candidates may also require specific formulations to work effectively and efficiently. These formulations may be covered by intellectual property rights held by others. We may develop products containing our compositions and pre-existing pharmaceutical compositions. These pharmaceutical products may be covered by intellectual property rights held by others. We may be required by the FDA, EMA or other foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates. These diagnostic test or tests may be covered by intellectual property rights held by others. We may be unable to acquire or in-license any relevant third-party intellectual property rights that we identify as necessary or important to our business operations. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all, which would harm our business. We may need to cease use of the compositions or methods covered by such third-party intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on such intellectual property rights which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to obtain a license under such intellectual property rights, any such license may be non-exclusive, which may allow our competitors access to the same technologies licensed to us.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of their former employers or other third parties.

We do and may employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our licensors, competitors or potential competitors. Although we seek to protect our ownership of intellectual property rights by ensuring that our agreements with our employees, consultants, collaborators, independent contractors and other third parties with whom we do business include provisions requiring such parties to assign rights in inventions to us and to not use the know-how or confidential information of their former employer or other third parties, we may be subject to claims that we or our employees, consultants, collaborators or independent contractors have inadvertently or otherwise used or disclosed know-how or confidential information of their former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and if we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable personnel or intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property, which could result in customers seeking other sources for the technology, or in ceasing from doing business with us. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to progress our clinical development programs or commercialize our technology or product candidate. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful, litigation could result in substantial cost and reputational loss and be a distraction to our management and other employees. Moreover, any such litigation or the threat thereof may adversely affect our reputation, our ability to form strategic alliances or sublicense our rights to collaborators, engage with scientific advisors or hire employees or consultants, each of which would have an adverse effect on our business, results of operations and financial condition.

Risks Related to Employee Matters and Managing Growth

We will need to expand our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.

As of June 6, 2019, we had 43 full-time employees. We will need to significantly expand our organization, and we may have difficulty identifying, hiring and integrating new personnel. Future growth would impose significant additional responsibilities on our management, including the need to identify, recruit, maintain, motivate and integrate additional employees, consultants and contractors. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize our product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our expected growth, our expenses may increase more than expected, our potential ability to generate revenue could be reduced and we may not be able to implement our business strategy. Many of the biotechnology companies that we compete against for qualified personnel and consultants have greater financial and other resources, different risk profiles and a longer history in the industry than we do. If we are unable to continue to attract and retain high-quality personnel and consultants, the rate and success at which we can discover and develop product candidates and operate our business will be limited.

Our future success depends on our ability to retain our key personnel and to attract, retain and motivate qualified personnel.

Our industry has experienced a high rate of turnover of management personnel in recent years. We are highly dependent on the development, regulatory, commercialization and business development expertise of Asa Abeliovich, M.D., Ph.D., our founder and Chief Executive Officer, as well as the other principal members of our management, scientific and clinical teams. Although we have formal employment agreements with our executive officers, these agreements do not prevent them from terminating their employment with us at any time and, for certain of our executive officers, entitle them to receive severance payments in connection with their voluntary resignation of employment. Additional details regarding these arrangements can be found in the section "Executive Compensation−Executive Compensation Arrangements."

If we lose one or more of our executive officers or key employees, our ability to implement our business strategy successfully could be seriously harmed. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize product candidates successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be engaged by entities other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to advance the clinical development of and commercialize product candidates will be limited.

Our insurance policies are expensive and protect us only from some business risks, which leaves us exposed to significant uninsured liabilities.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include general liability, clinical trial liability, employment practices liability, property, auto, workers' compensation, umbrella, and directors' and officers' insurance.

Any additional product liability insurance coverage we acquire in the future, may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive and in the future we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If we obtain marketing approval for our product candidates, we intend to acquire insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. A successful product liability claim or series of claims brought against us could cause our share price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business, including preventing or limiting the commercialization of any product candidates we develop. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

We also expect that operating as a public company will make it more difficult and more expensive for us to obtain director and officer liability insurance, and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, it may be more

difficult for us to attract and retain qualified people to serve on our board of directors, our board committees or as executive officers. We do not know, however, if we will be able to maintain existing insurance with adequate levels of coverage. Any significant uninsured liability may require us to pay substantial amounts, which would adversely affect our cash position and results of operations.

Our employees and independent contractors, including consultants, vendors, and any third parties we may engage in connection with development and commercialization may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could harm our business.

Misconduct by our employees and independent contractors, including consultants, vendors, and any third parties we may engage in connection with development and commercialization, could include intentional, reckless or negligent conduct or unauthorized activities that violate: (1) the laws and regulations of the FDA, EMA and other similar regulatory authorities, including those laws that require the reporting of true, complete and accurate information to such authorities; (2) manufacturing standards; (3) data privacy, security, fraud and abuse and other healthcare laws and regulations; or (4) other laws that require the reporting of true, complete and accurate financial information and data. Specifically, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws could also involve the improper use or misrepresentation of information obtained in the course of clinical trials, creation of fraudulent data in preclinical studies or clinical trials or illegal misappropriation of drug product, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with such laws or regulations. Additionally, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgements, possible exclusion from participation in Medicare, Medicaid, other U.S. federal healthcare programs or healthcare programs in other jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, imprisonment, other sanctions, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations.

Our business and operations would suffer in the event of system failures.

Our computer systems, as well as those of our contractors and consultants, are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our product candidate development programs. For example, the loss of preclinical study or clinical trial data from completed, ongoing or planned trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications, or inappropriate disclosure of personal, confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, clinical trial data, proprietary business information, personal data and personally identifiable information of our clinical trial subjects and employees, in our data centers and on our networks. The secure processing,

maintenance and transmission of this information is critical to our operations. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or internal bad actors, or breached due to employee error, a technical vulnerability, malfeasance or other disruptions. Although, to our knowledge, we have not experienced any such material security breach to date, any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, significant regulatory penalties, and such an event could disrupt our operations, damage our reputation, and cause a loss of confidence in us and our ability to conduct clinical trials, which could adversely affect our reputation and delay clinical development of our product candidates.

We may engage in acquisitions that could disrupt our business, cause dilution to our stockholders or reduce our financial resources.

In the future, we may enter into transactions to acquire other businesses, products or technologies. If we do identify suitable candidates, we may not be able to make such acquisitions on favorable terms, or at all. Any acquisitions we make may not strengthen our competitive position, and these transactions may be viewed negatively by patients, collaborators, third party payors or investors. We may decide to incur debt in connection with an acquisition or issue our common stock or other equity securities to the stockholders of the acquired company, which would reduce the percentage ownership of our existing stockholders. We could incur losses resulting from undiscovered liabilities of the acquired business that are not covered by the indemnification we may obtain from the seller. In addition, we may not be able to successfully integrate the acquired personnel, technologies and operations into our existing business in an effective, timely and non-disruptive manner. Acquisitions may also divert management attention from day-to-day responsibilities, increase our expenses and reduce our cash available for operations and other uses. We cannot predict the number, timing or size of future acquisitions or the effect that any such transactions might have on our business, financial position or operating results.

Risks Related to Our Common Stock and this Offering

An active trading market for our common stock may not develop.

Prior to this offering, there has been no public market for our common stock, and an active trading market for our shares may never develop or be sustained following this offering. Any delay in the commencement of trading of our common stock on The Nasdaq Global Select Market, or Nasdaq, would impair the liquidity of the market for our common stock and make it more difficult for holders to sell their shares. The initial public offering price for our common stock will be determined through negotiations between the underwriters and us and may vary from the market price of our common stock following this offering. If an active market for our common stock does not develop, or it is not sustained, it may be difficult for you to sell your shares without depressing the market price for our common stock, or at all.

The market price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock in this offering.

Our share price is likely to be volatile. The stock market in general and the market for smaller biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the initial public offering price. The market price for our common stock may be influenced by many factors, including:

- timing and results of our preclinical studies and clinical trials or those of our competitors;
- the success of existing or new competitive therapies, products or technologies;

development of new product candidates that may address our markets and make our product candidates less attractive;

failure or discontinuation of any of our research or development programs;

changes in the level of expenses related to any of our research or development programs;

developments related to any existing or future collaborations;

the recruitment or departure of key personnel;

regulatory or legal developments in the United States and other countries;

announcements by us, our partners or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;

changes in the structure of healthcare payment systems;

the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;

developments or disputes concerning patent applications, issued patents or other proprietary rights;

changes in failure to meet or exceed financial estimates and projections of the investment community or that we provide to the public;

actual or expected changes in estimates as to financial results, development timelines or recommendations by securities analysts;

announcement or expectation of additional financing efforts;

sales of common stock by us, our executive officers, directors or principal stockholders, or others;

variations in our financial results or those of companies that are perceived to be similar to us;

market conditions in the pharmaceutical and biotechnology sectors;

general economic, industry and market conditions;

changes in accounting principles; and

the other factors described in this "Risk Factors" section and elsewhere in this prospectus.

In addition, the stock market in general, and Nasdaq and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. In the past, when the market price of a security has been volatile, holders of that security have sometimes instituted securities class action litigation against the issuer. If any of the holders of our common stock were to bring such a lawsuit against us, we could incur substantial costs defending the lawsuit and the attention of our senior management would be diverted from the operation of our business. Any adverse determination in litigation could also subject us to significant liabilities. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. Further, a decline in the financial markets and related factors beyond our control may cause the price of our common stock to decline rapidly and unexpectedly. If the market price of our common stock after the completion of this offering does not exceed the initial public offering price, you may not realize any return on, or you may lose some or all of your investment in us.

If securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our common stock, our share price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. We do not currently have, and may never obtain, research

coverage by securities and industry analysts. If no or few securities or industry analysts commence coverage of us, the trading price for our shares could be negatively impacted. In the event we obtain securities or industry analyst coverage, if any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our share performance, or if any of our preclinical studies or clinical trials and operating results fail to meet the expectations of analysts, our stock price would likely decline. If one or more of these analysts ceases coverage of us or fails to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

After this offering, our executive officers, directors and principal stockholders, if they choose to act together, will continue to have the ability to control or significantly influence all matters submitted to stockholders for approval.

Upon the closing of this offering, based on the number of common stock outstanding as of March 31, 2019, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock before this offering and their respective affiliates will, in the aggregate, hold common stock representing approximately 51.0% of our outstanding common stock. As a result, if these stockholders choose to act together, they would be able to control or significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control or significantly influence the election of directors, the composition of our management and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination that other stockholders may desire. Any of these actions could adversely affect the market price of our common stock.

We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. We expect that we will use the net proceeds of this offering as set forth in the section titled "Use of Proceeds." However, our use of these proceeds may differ substantially from our current plans. The failure by our management to apply these funds effectively could result in financial losses that could have a negative impact on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

If you purchase common stock in this offering, you will suffer immediate and substantial dilution of your investment.

The initial public offering price of our common stock will be substantially higher than the net tangible book value per share of our common stock. Therefore, if you purchase our common stock in this offering, you will pay a price per share that substantially exceeds our net tangible book value per share after this offering. To the extent shares subsequently are issued under outstanding options or warrants or to executive officers in connection with this offering, you will incur further dilution. Based on an assumed initial public offering price of $17.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, you will experience immediate dilution of $10.67 per share as of March 31, 2019, representing the difference between our pro forma as adjusted net tangible book value per share, after giving effect to this offering, and the assumed initial public offering price. See the section titled "Dilution" for additional information.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

Additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell

common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner, we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

Pursuant to the 2019 Plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. The number of shares of our common stock reserved for issuance under the 2019 Plan will automatically increase on January 1 of each calendar year, starting on January 1, 2020 through January 1, 2029, in an amount equal to 4% of the total number of shares of our common stock outstanding on the last day of the calendar month before the date of each automatic increase, or a lesser number of shares determined by our board of directors prior to the applicable January 1st. If our board of directors elects to increase the number of shares available for future grant by the maximum amount each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

A significant portion of our total outstanding shares are eligible to be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market, or the perception in the market that the holders of a large number of stockholders intend to sell shares of our common stock, could reduce the market price of our common stock. After this offering, we will have 33,997,131 shares of common stock outstanding, based on 26,644,131 shares of our common stock outstanding as of March 31, 2019. This includes the shares that we are selling in this offering, which may be resold in the public market immediately without restriction, unless purchased by our affiliates. Substantially all of the remaining 26,644,131 shares of common stock initially will be restricted as a result of securities laws, market standoff provisions or lock-up agreements, but will become eligible to be sold after this offering as described in the section titled "Shares Eligible for Future Sale."

Moreover, after this offering, holders of an aggregate of 24,295,131 shares of common stock will have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders, until such shares can otherwise be sold without restriction under Rule 144 under the Securities Act of 1933, as amended, or the Securities Act, or until the rights terminate pursuant to the terms of the stockholders agreement between us and such holders. We also intend to register all shares of common stock subject to equity awards issued or reserved for future issuance under our equity compensation plans on a registration statement on Form S-8. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates under Rule 144 under the Securities Act and the market standoff provisions and lock-up agreements described above. Any sales of securities by these stockholders could have a negative impact on the trading price of our common stock.

We are an emerging growth company and a smaller reporting company and the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act, and may remain an emerging growth company until the last day of the fiscal year following the fifth anniversary of the closing of this offering. However, if certain events occur prior to the end of such five-year period, including if we become a "large accelerated filer," our annual gross revenues exceed $1.07 billion or we issue more than $1.0 billion of non-convertible debt in any three-year period, we will cease to be an emerging growth company prior to the end of such five-year period. For so long as we remain an emerging growth company, we are permitted and intend to

rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management' s Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this prospectus;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor' s report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

As a result, the information we provide to stockholders will be different than the information that is available with respect to other public companies that are not emerging growth companies. For example, in this prospectus we have only included two years of audited financial statements and have not included all of the executive compensation-related information that would be required if we were not an emerging growth company. We cannot predict whether this will cause investors to find our common stock less attractive. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our share price may be reduced or more volatile.

Even following the termination of our status as an emerging growth company, we will be able to take advantage of the reduced disclosure requirements applicable to "smaller reporting companies," as that term is defined in Rule 12b-2 of the Exchange Act of 1934, as amended, or the Exchange Act, and, in particular, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. To the extent that we are no longer eligible to use exemptions from various reporting requirements, we may be unable to realize our anticipated cost savings from these exemptions, which could have a material adverse impact on our operating results.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the Nasdaq listing requirements and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, which in turn could make it more difficult for us to attract and retain qualified members of our board of directors.

We are evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we will be required to furnish a report by our management on our internal control over financial reporting beginning with our second filing of an Annual Report on Form 10-K with the SEC after we become a public company. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants, adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing whether such controls are functioning as documented, and implement a continuous reporting and improvement process for internal control over financial reporting. In addition, if we identify one or more material weaknesses as a result of this implementation and evaluation process, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Section 404(a) of the Sarbanes-Oxley Act, or Section 404(a), requires that beginning with our second annual report following our initial public offering, management assess and report annually on the effectiveness of our internal control over financial reporting and identify any material weaknesses in our internal control over financial reporting. Although Section 404(b) of the Sarbanes-Oxley Act, or Section 404(b), requires our independent registered public accounting firm to issue an annual report that addresses the effectiveness of our internal control over financial reporting, we have opted to rely on the exemptions provided in the JOBS Act, and consequently will not be required to comply with SEC rules that implement Section 404(b) until such time as we are no longer an emerging growth company.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inadequate internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

Upon the completion of this offering, we will become subject to the periodic reporting requirements of the Exchange Act. We designed our disclosure controls and procedures to reasonably assure that information we must disclose in reports we file or submit under the Exchange Act is accumulated and communicated to management, and recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well-conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. For example, our directors or executive officers could inadvertently fail to disclose a new relationship or arrangement causing us to fail to make any related party

transaction disclosures. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements due to error or fraud may occur and not be detected.

Future changes in financial accounting standards or practices may cause adverse and unexpected revenue fluctuations and adversely affect our reported results of operations.

Future changes in financial accounting standards may cause adverse, unexpected revenue fluctuations and affect our reported financial position or results of operations. Financial accounting standards in the United States are constantly under review and new pronouncements and varying interpretations of pronouncements have occurred with frequency in the past and are expected to occur again in the future. As a result, we may be required to make changes in our accounting policies. Those changes could affect our financial condition and results of operations or the way in which such financial condition and results of operations are reported. Compliance with new accounting standards may also result in additional expenses. As a result, we intend to invest all reasonably necessary resources to comply with evolving standards, and this investment may result in increased general and administrative expenses and a diversion of management time and attention from business activities to compliance activities. See the section titled "Management' s Discussion and Analysis of Financial Condition and Results of Operations−Recently Adopted Accounting Standards." As an emerging growth company, the JOBS Act allows us to delay adoption of new or revised accounting standards applicable to public companies until such pronouncements are made applicable to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, would be your sole source of gain.

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. As a result, capital appreciation, if any, of our common stock would be your sole source of gain on an investment in our common stock for the foreseeable future. See the section titled "Dividend Policy" for additional information.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management' s attention and resources, which could harm our business.

We have significant net operating losses which we may not be able to realize or which may be restricted following any future change of control. We also benefit from certain tax incentive regimes, such as research and development tax credits, in the jurisdictions in which we operate and any adverse change to these regimes, the application thereof or challenges to the tax position we have adopted under these regimes could adversely affect our results of operations and financial condition.

As of December 31, 2018, we had $12.8 million of U.S. federal and $25.5 million of state net operating loss, or NOL, carryforwards. Our U.S. federal NOL carryforwards generated prior to 2018 will expire if not utilized prior to 2038. Under the Tax Act, federal NOLs incurred in tax years ending after December 31, 2017, may be carried forward indefinitely, but the deductibility of federal NOLs generated in tax years beginning after December 31, 2017, is limited.

Our NOL carryforwards are subject to review and possible adjustment by the U.S. and state tax authorities. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of

state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation' s ability to use its pre-change NOL carryforwards and R&D credits to offset its post-change income may be limited. This could limit the amount of NOLs or R&D credit carryforwards that we can utilize annually to offset future taxable income or tax liabilities. Subsequent ownership changes and changes to the U.S. tax rules in respect of the utilization of NOLs and R&D credits carried forward may further affect the limitation in future years. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Additionally, we have not undertaken a study on our determination of our U.S. R&D credits. Consequently, our U.S. R&D credits may change, and in any event are subject to review and adjustment by the tax authorities.

U.S. federal income tax reform could adversely affect us.

On December 22, 2017, President Trump signed into law the Tax Act, which significantly revises the Internal Revenue Code. The Tax Act, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted taxable income (except for certain small businesses), limitation of the deduction for NOLs carried forward from taxable years beginning after December 31, 2017 to 80% of current year taxable income and elimination of most NOL carrybacks, one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. We do not expect changes under the Tax Act to have a material impact on our tax liabilities in the near future. However, we continue to examine the impact that the Tax Act may have on our business in the longer term. Accordingly, notwithstanding the reduction in the U.S. federal corporate income tax rate, the overall impact of the Tax Act is uncertain and our business and financial condition could be adversely affected by the Tax Act. The Tax Act may also have an impact on holders of our common stock. We urge prospective investors to consult with their legal and tax advisors with respect to the Tax Act and the potential tax consequences of investing in or holding our common stock.

Delaware law and provisions in our amended and restated certificate of incorporation and amended and restated bylaws that will be in effect at the completion of this offering could make a merger, tender offer or proxy contest difficult, thereby depressing the trading price of our common stock.

Following the completion of this offering, our status as a Delaware corporation and the anti-takeover provisions of the Delaware General Corporation Law, or DGCL may discourage, delay or prevent a change in control by prohibiting us from engaging in a business combination with an interested stockholder for a period of three years after the person becomes an interested stockholder, even if a change of control would be beneficial to our existing stockholders. In addition, our amended and restated certificate of incorporation and amended and restated bylaws that will be in effect at the completion of this offering will contain provisions that may make the acquisition of our company more difficult, including the following:

- a classified board of directors with three-year staggered terms, which could delay the ability of stockholders to change the membership of a majority of our board of directors;
- the ability of our board of directors to issue shares of preferred stock and to determine the price and other terms of those shares, including preferences and voting rights, without stockholder approval, which could be used to significantly dilute the ownership of a hostile acquirer;
- the exclusive right of our board of directors to elect a director to fill a vacancy created by the expansion of our board of directors or the resignation, death or removal of a director, which prevents stockholders from being able to fill vacancies on our board of directors;

a prohibition on stockholder action by written consent, which forces stockholder action to be taken at an annual or special meeting of our stockholders;

the requirement that a special meeting of stockholders may be called only by a majority vote of our entire board of directors, the chairman of our board of directors or our chief executive officer, which could delay the ability of our stockholders to force consideration of a proposal or to take action, including the removal of directors;

the requirement for the affirmative vote of holders of at least 66 2/3% of the voting power of all of the then-outstanding shares of the voting stock, voting together as a single class, to amend the provisions of our amended and restated certificate of incorporation relating to the management of our business or our amended and restated bylaws, which may inhibit the ability of an acquirer to affect such amendments to facilitate an unsolicited takeover attempt; and

advance notice procedures with which stockholders must comply to nominate candidates to our board of directors or to propose matters to be acted upon at a stockholders' meeting, which may discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer' s own slate of directors or otherwise attempting to obtain control of us.

In addition, as a Delaware corporation, we are subject to Section 203 of the DGCL. These provisions may prohibit large stockholders, in particular those owning 15% or more of our outstanding voting stock, from merging or combining with us for a certain period of time. A Delaware corporation may opt out of this provision by express provision in its original certificate of incorporation or by amendment to its certificate of incorporation or bylaws approved by its stockholders. However, we have not opted out of this provision.

These and other provisions in our amended and restated certificate of incorporation, amended and restated bylaws and Delaware law could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by our then-current board of directors, including delay or impede a merger, tender offer or proxy contest involving our company. The existence of these provisions could negatively affect the price of our common stock and limit opportunities for you to realize value in a corporate transaction.

For information regarding these and other provisions, see the section titled "Description of Capital Stock."

Our amended and restated certificate of incorporation that will become effective upon the closing of this offering designates the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against our company and our directors, officers and employees.

Our amended and restated certificate of incorporation that will become effective upon the closing of this offering provides that, to the fullest extent permitted by law, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery does not have jurisdiction, any state court located within the State of Delaware, or if all such state courts lack jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (1) any derivative action or proceeding brought on our behalf; (2) any action or proceeding asserting a claim of breach of fiduciary duty owed by any current or former director, officer or other employee, to us or our stockholders; (3) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the DGCL, our certificate of incorporation or our bylaws; (4) any action or proceeding to interpret, apply, enforce or determine the validity of our certificate of incorporation or our bylaws; (5) any action or proceeding as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware; or (6) any action asserting a claim against us, or any of our directors, officers or other employees, that is governed

by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court' s having personal jurisdiction over the indispensable parties named as defendants. For the avoidance of doubt, these choice of forum provisions will not apply to suits brought to enforce a duty or liability created by the Securities Act, the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction.

These choice of forum provisions may limit a stockholder' s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees and may discourage these types of lawsuits. Furthermore, if a court were to find the choice of forum provisions contained in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements. All statements other than statements of historical facts contained in this prospectus are forward-looking statements, including statements about:

- our expectations regarding the initiation, timing, scope and results of our development activities, including our planned clinical trials;
- the timing of and plans for regulatory filings;
- our plans to obtain and maintain regulatory approvals of our product candidates in any of the indications for which we plan to develop them;
- the potential benefits of our product candidates and technologies;
- our expectations regarding our ability to identify additional gene therapy product candidates;
- the market opportunities for our product candidates and our ability to maximize those opportunities;
- our business strategies and goals;
- estimates of our expenses, capital requirements and need for additional financing;
- our expectations regarding potentially establishing manufacturing capabilities;
- the performance of our third-party suppliers and manufacturers,
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates and our ability to operate our business without infringing on the intellectual property rights of others;
- our expectations regarding developments and projections relating to our competitors and any competing therapies that are or become available;
- our ability to identify, recruit and retain key personnel;
- regulatory development in the United States and foreign countries; and
- our expectations regarding the uses of the net proceeds from this offering and the sufficiency of such net proceeds together with our existing cash and cash equivalents to fund our operations.

In some cases, you can identify forward-looking statements by terms such as “anticipate,” “believe,” “continue” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target” or “will” or the negative of these terms or other similar expressions intended to identify statements about the future. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, you should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements.

In addition, statements that “we believe” and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this prospectus, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

You should read the section titled “Risk Factors” for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. Moreover,

we operate in an evolving environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. As a result of these factors, we cannot assure you that the forward-looking statements in this prospectus will prove to be accurate. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.

INDUSTRY AND OTHER DATA

We obtained the industry, statistical and market data in this prospectus from our own internal estimates and research as well as from industry and general publications and research, surveys and studies conducted by third parties. All of the market data used in this prospectus involves a number of assumptions and limitations. While we believe that each of these studies and publications is reliable, the industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of important factors, including those described in the section titled "Risk Factors." These and other factors could cause results to differ materially from those expressed in the estimates made by third parties and by us.

DIVIDEND POLICY

We have never declared or paid, and do not anticipate declaring or paying, in the foreseeable future, any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. Any future determination related to our dividend policy will be made at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant.

USE OF PROCEEDS

We estimate that the net proceeds from this offering will be approximately $113.4 million (or $130.8 million if the underwriters exercise their option to purchase additional shares in full), assuming an initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Each $1.00 increase or decrease in the assumed initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease the net proceeds to us from this offering by approximately $6.8 million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting estimated underwriting discounts and commissions. We may also increase or decrease the number of shares we are offering. A 1,000,000 share increase or decrease in the number of shares offered by us would increase or decrease the net proceeds to us from this offering by approximately $15.8 million, assuming that the assumed initial offering price to the public remains the same, and after deducting estimated underwriting discounts and commissions. We do not expect that a change in the initial price to the public or the number of shares by these amounts would have a material effect on the uses of the proceeds from this offering, although it may accelerate the time at which we will need to seek additional capital.

We intend to use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows:

- approximately $50.0 million to $60.0 million to advance the development of PR001, including to fund our planned Phase 1/2 trials for PR001 for the treatment of PD-GBA and neuronopathic Gaucher disease;
- approximately $18.0 million to $25.0 million to advance the development of PR006, including our ongoing preclinical studies for PR006 for the treatment of FTD-GRN; and
- approximately $20.0 million to $25.0 million to advance the development of PR004, including our ongoing preclinical studies for PR004 for the treatment of synucleinopathies.
- the remainder for working capital and other general corporate purposes.

Based on our planned use of the net proceeds from this offering and our existing cash and cash equivalents, we estimate that such funds will be sufficient to fund our operating expenses and capital expenditure requirements at least through the first half of 2021, and through initial expected data release from our Phase 1/2 clinical trials for PR001 for the treatment of PD-GBA and Type 2 Gaucher disease, the initiation of our anticipated Phase 1/2 clinical trials for PR001 for the treatment of Type 3 Gaucher disease and PR006 for the treatment of FTD-GRN and the completion of our IND-enabling studies for PR004 for the treatment of synucleinopathies. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect. The expected net proceeds from this offering, together with our existing cash and cash equivalents, will not be sufficient for us to fund any of our drug candidates through regulatory approval, and we will need to raise additional capital to complete the development and commercialization of our drug candidates.

This expected use of existing cash and cash equivalents and our net proceeds from this offering represent our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. Predicting the costs necessary to develop product candidates can be difficult and we will need additional funds to complete our clinical development of any of our product candidates. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development, the status of and results from clinical trials, as well as any collaborations that we may enter into with third parties for our product candidates, and any unforeseen cash needs.

Our management will have broad discretion in the application of the net proceeds from this offering, and investors will be relying on the judgment of our management regarding the application of those net proceeds. The timing and amount of our actual expenditures will be based on many factors, including cash flows from operations and the anticipated growth of our business. Pending these uses, we plan to invest these net proceeds in short-term, interest bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the United States.

CAPITALIZATION

The following table sets forth our cash and cash equivalents and capitalization as of March 31, 2019:

- on an actual basis;
- on a pro forma basis to reflect (1) the automatic conversion of all outstanding shares of convertible preferred stock into common stock as if such conversion had occurred on March 31, 2019, and (2) the filing and effectiveness of our amended and restated certificate of incorporation, each of which will occur immediately prior to the closing of this offering; and
- on a pro forma as adjusted basis to reflect (1) the pro forma adjustments set forth above and (2) our sale of 7,353,000 shares of common stock in this offering at an assumed initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

You should read the information in this table together with our financial statements and related notes included elsewhere in this prospectus and the sections titled "Selected Financial and Other Data" and "Management' s Discussion and Analysis of Financial Condition and Results of Operations."

	As of March 31, 2019		
	Actual	Pro Forma	Pro Forma As Adjusted[1]
	(in thousands, except share and per share data)		
Cash and cash equivalents	$100,268	$100,268	$213,619
Convertible preferred stock, par value $0.0001 per share, 20,000,000 shares authorized, 19,435,131 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	$129,544	$–	$–
Stockholders' (deficit) equity:			
Preferred stock, par value $0.0001 per share; no shares authorized, issued or outstanding, actual; 10,000,000 shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted	–	–	–
Common stock, par value $0.0001 per share; 35,640,000 shares authorized, 7,209,000 shares issued and outstanding, actual; 200,000,000 shares authorized, 26,644,131 shares issued and outstanding, pro forma; and 33,997,131 shares issued and outstanding, pro forma as adjusted	–	3	3
Additional paid-in capital	3,108	132,652	246,001
Accumulated deficit	(30,859)	(30,859)	(30,859)
Total stockholders' (deficit) equity	(27,751)	101,793	215,145
Total capitalization	$ 101,793	$ 101,793	$ 215,145

(1) The pro forma as adjusted information set forth above is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. Each $1.00 increase or decrease in the assumed initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease pro forma as adjusted cash and cash equivalents, additional paid-in capital, total stockholders' equity and total capitalization by approximately $6.8 million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting estimated underwriting discounts and commissions. We may also increase or decrease the number of shares we are offering. A 1,000,000 share increase or decrease in the number of shares offered by us would increase or decrease pro forma as adjusted cash and cash equivalents, additional paid-in capital, total stockholders' equity and total capitalization by approximately $15.8 million, assuming that the assumed initial offering price to the public remains the same, and after deducting estimated underwriting discounts and commissions.

The number of shares of our common stock to be outstanding after this offering is based on 26,644,131 shares of our common stock outstanding as of March 31, 2019, and excludes:

- 4,376,161 shares of common stock issuable upon the exercise of options outstanding as of March 31, 2019, with a weighted-average exercise price of $1.00 per share;
- 485,862 shares of common stock reserved for future issuance pursuant to the 2017 Plan;
- 2,773,562 shares of common stock reserved for future issuance pursuant to the 2019 Plan, as well as any automatic increases in the number of common shares reserved for future issuance under the 2019 Plan; and
- 330,000 shares of common stock reserved for future issuance pursuant to our 2019 ESPP, as well as any automatic increases in the number of common shares reserved for future issuance under the 2019 ESPP.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be immediately diluted to the extent of the difference between the initial public offering price per share and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

As of March 31, 2019, we had a historical net tangible book value (deficit) of $(27.8) million, or $(3.85) per share of common stock. Our historical net tangible book value (deficit) per share represents total tangible assets less total liabilities and convertible preferred stock, divided by the number of shares of our common stock outstanding as of March 31, 2019.

Our pro forma net tangible book value as of March 31, 2019 was $101.8 million, or $3.82 per share, after giving effect to (1) the automatic conversion of all outstanding shares of convertible preferred stock into common stock as if such conversion had occurred on March 31, 2019, and (2) the filing and effectiveness of our amended and restated certificate of incorporation, each of which will occur immediately prior to the closing of this offering

After giving further effect to the sale of 7,353,000 shares of common stock in this offering at an assumed initial public offering price of $17.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of March 31, 2019 would have been approximately $215.1 million, or approximately $6.33 per share. This amount represents an immediate increase in pro forma net tangible book value of $2.51 per share to our existing stockholders and immediate dilution of approximately $10.67 per share to new investors in this offering. We determine dilution by subtracting the as pro forma adjusted net tangible book value per share after this offering from the amount of cash that a new investor paid for a share of common stock in this offering.

The following table illustrates this dilution:

Assumed initial public offering price per share		$17.00
Historical net tangible book value (deficit) per share as of March 31, 2019	$(3.85)	
Increase per share attributable to the pro forma adjustments described above	7.67	
Pro forma net tangible book value per share as of March 31, 2019	3.82	
Increase per share attributable to this offering	2.51	
Pro forma as adjusted net tangible book value per share after this offering		6.33
Dilution per share to new investors in this offering		$10.67

The pro forma as adjusted dilution information discussed above is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. Each $1.00 increase or decrease in the assumed initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease the pro forma as adjusted net tangible book value per share by $0.20 per share and the dilution per share to investors participating in this offering by $0.80 per share, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions. We may also increase or decrease the number of shares we are offering. A 1,000,000 share increase in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase the pro forma as adjusted net tangible book value per share by $0.27 and decrease the dilution per share to investors participating in this offering by $0.27, assuming the assumed initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions. A 1,000,000 share decrease in the number of shares offered by us, as set forth on the cover page of this prospectus, would decrease the pro forma as adjusted net tangible

book value per share after this offering by $0.29 and increase the dilution per share to new investors participating in this offering by $0.29, assuming the assumed initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions.

If the underwriters exercise their over-allotment option to purchase an additional 1,102,950 shares of our common stock in full, the pro forma as adjusted net tangible book value of our common stock would increase to $6.63 per share, representing an immediate increase in the pro forma net tangible book value per share to existing stockholders of $2.81 per share and an immediate dilution of $10.37 per share to investors participating in this offering.

The following table summarizes as of March 31, 2019, on the pro forma as adjusted basis described above, the number of shares of our common stock, the total consideration and the average price per share (1) paid to us by our existing stockholders and (2) to be paid by investors purchasing our common stock in this offering at an assumed initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, before deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

	Shares Purchased		Total Consideration		Weighted-Average
	Number	Percent	Amount	Percent	Price Per Share
Existing stockholders	26,644,131	78.4 %	$130,279,670	51.0 %	$4.89
New investors	7,353,000	21.6	125,001,000	49.0	$ 17.00
Total	33,997,131	100.0%	$255,280,670	100.0%	

The number of shares of our common stock to be outstanding after this offering is based on 26,644,131 shares of our common stock outstanding as of March 31, 2019, and excludes:

- 4,376,161 shares of common stock issuable upon the exercise of options outstanding as of March 31, 2019, with a weighted-average exercise price of $1.00 per share;
- 485,862 shares of common stock reserved for future issuance pursuant to the 2017 Plan as of March 31, 2019;
- 2,773,562 shares of common stock reserved for future issuance pursuant to the 2019 Plan, as well as any automatic increases in the number of common shares reserved for future issuance under this plan; and
- 330,000 shares of common stock reserved for future issuance pursuant to our 2019 ESPP, as well as any automatic increases in the number of common shares reserved for future issuance under the 2019 ESPP.

To the extent that stock options are exercised, new stock options are issued under our equity incentive plan, or we issue additional shares of common stock in the future, there will be further dilution to investors participating in this offering. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

SELECTED FINANCIAL DATA

The following tables set forth selected financial data for the periods indicated. We have derived the selected statements of operations data for the period from July 6, 2017 (date of inception) through December 31, 2017 and the year ended December 31, 2018 from our audited financial statements included elsewhere in this prospectus. We have derived our balance sheet data as of December 31, 2017 and 2018 from our audited financial statements included elsewhere in this prospectus. The selected statements of operations data for the three months ended March 31, 2018 and 2019 and the selected balance sheet data as of March 31, 2019 have been derived from our unaudited interim financial statements included elsewhere in this prospectus. The unaudited financial statements have been prepared on the same basis as the audited financial statements, and in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly our financial position and results of operations.

Our interim and historical results are not necessarily indicative of the results that may be expected for the full year or any other future period. The following selected financial data should be read in conjunction with the section titled "Management' s Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes included elsewhere in this prospectus.

	Period from July 6, 2017 (Date of Inception) through December 31, 2017	Year Ended December 31, 2018	Three Months Ended March 31, 2018	Three Months Ended March 31, 2019
			(unaudited)	
	(in thousands, except share and per share data)			
Statement of Operations Data:				
Operating expenses:				
Research and development	$ 995	$ 14,127	$1,304	$8,411
General and administrative	804	4,682	641	1,885
Total operating expenses	1,799	18,809	1,945	10,296
Operating loss	(1,799)	(18,809)	(1,945)	(10,296)
Change in fair value of derivative liabilities	–	(781)	(781)	–
Other income	–	87	–	–
Interest (expense) income, net	(27)	416	(471)	351
Net loss	$ (1,826)	$ (19,088)	$(3,197)	$(9,945)
Net loss per share attributable to common stockholders, basic and diluted(1)	$ (0.45)	$ (3.71)	$(0.66)	$(1.73)
Weighted-average shares outstanding, basic and diluted(1)	4,050,000	5,145,469	4,860,000	5,740,874
Pro forma net loss per share attributable to common stockholders, basic and diluted(1)		$ (1.21)		$(0.51)
Pro forma weighted-average shares outstanding, basic and diluted(1)		15,748,039		19,597,958

(1) See note 14 to our financial statements included elsewhere in this prospectus for an explanation of the method used to calculate basic and diluted net loss per share.

	As of December 31, 2017	As of December 31, 2018	As of March 31, 2019
		(in thousands)	
Balance Sheet Data:			
Cash and cash equivalents	$ 12,744	$ 63,014	$ 100,268
Working capital(1)	2,134	59,941	98,894
Total assets	14,326	72,881	115,194
Convertible preferred stock	3,524	79,710	129,544
Accumulated deficit	(1,827)	(20,914)	(30,859)
Total stockholders' deficit	(940)	(18,417)	(27,751)

(1) We define working capital as current assets less current liabilities. See our financial statements and the related notes included elsewhere in this prospectus for further details regarding our current assets and current liabilities.

MANAGEMENT' S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with "Selected Financial Data" and our financial statements and related notes appearing elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. You should carefully read the "Risk Factors" section of this prospectus to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section entitled "Special Note Regarding Forward-Looking Statements."

Overview

We are a gene therapy company leveraging breakthroughs in human genetics with the goal of developing and commercializing disease-modifying AAV-based gene therapies for patients with devastating neurodegenerative diseases. We are applying a precision medicine approach to neurodegeneration by studying our gene therapies in genetically defined patient populations. We believe this will increase the probability of creating disease-modifying therapies that improve patients' lives. Our lead program is PR001 for the treatment of PD-GBA and neuronopathic Gaucher disease. We are focused on developing a broad pipeline of gene therapies for a range of neurodegenerative diseases, including PR006 for the treatment of FTD-GRN and PR004 for the treatment of synucleinopathies.

Since our inception in 2017, we have focused primarily on organizing and staffing our company, raising capital, establishing and protecting our intellectual property portfolio, in-licensing AAV9 in particular fields, developing and progressing our gene therapy product candidates through preclinical studies and preparing for clinical trials, and establishing our manufacturing capabilities. We do not have any product candidates approved for sale and have not generated any revenue from product sales. Since our inception, we have funded our operations primarily through equity and convertible debt financings, and have raised an aggregate of approximately $129.0 million of gross proceeds through these offerings which includes our Series B convertible preferred stock financing in March 2019. As of December 31, 2018 and March 31, 2019, we had cash and cash equivalents of $63.0 million and $100.3 million, respectively.

Since our inception, we have incurred significant operating losses. Our net losses were $1.8 million and $19.1 million for the period from July 6, 2017 (date of inception) through December 31, 2017 and the year ended December 31, 2018, respectively and were $3.2 million and $9.9 million for the three months ended March 31, 2018 and March, 31 2019, respectively. As of December 31, 2018 and March 31, 2019, we had an accumulated deficit of $20.9 million and $30.9 million, respectively. We expect our expenses and losses to increase substantially for the foreseeable future as we continue our development of, and seek regulatory approvals for, our product candidates, including PR001, PR006 and PR004, as well as hire additional personnel, pay for accounting, audit, legal, regulatory and consulting services, and pay costs associated with maintaining compliance with Nasdaq listing rules and SEC requirements, director and officer liability insurance, investor and public relations activities and other expenses associated with operating as a public company. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities.

We do not have any products approved for sale. We do not expect to generate any revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years. If we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Accordingly, until such time as we can generate substantial product revenues to

support our cost structure, if ever, we expect to finance our cash needs through equity offerings, debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies. If we are unable to raise additional capital when needed, we could be forced to delay, limit, reduce or terminate our product candidate development or future commercialization efforts or grant rights to third parties to develop and market our product candidates even if we would otherwise prefer to develop and market such product candidates ourselves.

License Agreements

License Agreement with REGENXBIO Inc. for GBA1

In August 2017, we entered into a license agreement, or the REGENXBIO GBA1 License, with REGENXBIO. Under the REGENXBIO GBA1 License, REGENXBIO granted us an exclusive, worldwide license under certain patents and patent applications to make, have made, use, import, sell and offer for sale products for the treatment of disease, including but not limited to Parkinson' s disease and Gaucher disease, whether or not caused by mutations in the gene that produces the GBA1 enzyme in humans by *in vivo* gene therapy using AAV9 delivering the gene (or any portion thereof) encoding for GBA1.

As consideration for the licensed rights under the REGENXBIO GBA1 License, we issued 2,430,000 shares of our common stock in a concurrent private placement to REGENXBIO. We are also obligated, pursuant to the REGENXBIO GBA1 License, to pay REGENXBIO: (1) an annual maintenance fee; (2) mid- to high-single digit royalty percentages on net sales of licensed products, subject to reduction in specified circumstances; and (3) mid-teen to low-twenties royalty percentages of any sublicense fees we receive from sublicensees for the licensed intellectual property rights. See the section titled "Business−License Agreements−License Agreement with REGENXBIO Inc. for GBA1" and note 3 to our financial statements appearing elsewhere in this prospectus for additional information regarding this license.

License Agreement with REGENXBIO Inc. for Option Genes

In May 2018, we entered into a license agreement, or the REGENXBIO Option Genes License, with REGENXBIO pursuant to which REGENXBIO granted us three distinct exclusive options for specified genes, or the Option Genes, exercisable at our sole discretion through May 10, 2019. Each option represents the right to obtain an exclusive, worldwide license under certain patents and patent applications to make, have made, use, import, sell and offer for sale products for the treatment or prevention of disease, including but not limited to Parkinson' s disease, whether or not caused by mutations in any Option Gene that is the subject of the applicable license, in humans by in vivo gene therapy using AAV9 delivering the applicable licensed Option Gene and/or RNA interference or antisense modalities that target the applicable licensed Option Gene.

Under the terms of the REGENXBIO Option Genes License, we paid REGENXBIO an initial fee of $0.6 million. In connection with the exercise of each option, we are required to pay REGENXBIO: (1) an additional up-front fee of $0.6 million; (2) an annual maintenance fee; (3) mid- to high-single digit royalty percentages on net sales of the licensed product, subject to reduction in specified circumstances; and (4) mid-teen to low-twenties royalty percentages of any sublicense fees we receive from sublicensees for the licensed intellectual property rights. If a licensed product includes the GBA1 gene and otherwise would be subject to royalties under the REGENXBIO GBA1 License, then royalties for that licensed product will only be due under the REGENXBIO Option Genes License. See the section titled "Business−License Agreements−License Agreement with REGENXBIO Inc. for Option Genes" and note 3 to our financial statements appearing elsewhere in this prospectus for additional information regarding this license.

Financial Operations Overview

Research and Development Expenses

Research and development expenses have related primarily to preclinical development of our gene therapy product candidates and discovery efforts, including conducting preclinical studies, manufacturing development efforts, preparing for clinical trials and activities related to regulatory filings for our product candidates. Research and development expenses are recognized as incurred and payments made prior to the receipt of goods or services to be used in research and development are capitalized until the goods or services are received. Our direct research and development expenses are not currently tracked on a program-by-program basis. Research and development expenses include or could include:

- employee related expenses, including salaries, bonuses, benefits, stock-based compensation, other related costs for those employees involved in research and development efforts;
- external research and development expenses incurred under agreements with CROs, investigative sites and consultants to conduct our preclinical studies;
- costs related to manufacturing material for our preclinical studies and clinical trials, including fees paid to CDMOs;
- laboratory supplies and research materials;
- costs related to compliance with regulatory requirements; and
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent, maintenance of facilities, insurance and equipment.

We plan to substantially increase our research and development expenses for the foreseeable future as we continue the development of our product candidates and manufacturing processes and conduct discovery and research activities for our preclinical programs. We cannot determine with certainty the timing of initiation, the duration or the completion costs of current or future preclinical studies and clinical trials of our product candidates due to the inherently unpredictable nature of preclinical and clinical development. Clinical and preclinical development timelines, the probability of success and development costs can differ materially from expectations. We anticipate that we will make determinations as to which product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to the results of ongoing and future preclinical studies and clinical trials, regulatory developments and our ongoing assessments as to each product candidate' s commercial potential. We will need to raise substantial additional capital in the future. Our clinical development costs are expected to increase significantly as we commence clinical trials. Our future expenses may vary significantly each period based on factors such as:

- per patient trial costs;
- the number of patients who enroll in each trial;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the drop-out or discontinuation rates of patients;
- potential additional safety monitoring requested by regulatory agencies;
- the duration of patient participation in the trials and follow-up;
- the phase of development of the product candidate; and

the efficacy and safety profile of the product candidate.

General and Administrative Expenses

General and administrative expenses consist primarily of costs related to third-party services, such as human resources, legal, patent, consulting, finance, accounting and audit services. It also includes employee-related expenses, including salaries, bonuses, benefits, stock-based compensation, other related costs, for those employees that support general and administrative functions.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our expanded infrastructure and begin to commercialize any approved products. We also anticipate that our general and administrative expenses will increase as a result of payments for accounting, audit, legal, consulting services, as well as costs associated with maintaining compliance with Nasdaq listing rules and SEC requirements, director and officer liability insurance, investor and public relations activities and other expenses associated with operating as a public company.

Change in Fair Value of Derivative Liability

To derive the fair value of the derivative liabilities embedded within a convertible note issued to one of our investors, we estimated the fair value of the convertible note with and without the derivative liabilities using a discounted cash flow approach. The difference between the "with" and "without" convertible note prices determined the fair value of the derivative liabilities at issuance and immediately prior to conversion. This convertible note converted into shares of our Series A convertible preferred stock in March 2018.

Other Income

In 2018, we received a New York City Biotechnology Tax Credit. Tax credits are recorded when funds are received and are included in other income on the statement of operations.

Interest (Expense) Income

We have institutional money market accounts that generate interest on a monthly basis.

In connection with the issuance of the convertible note, we incurred interest expense at 8% per annum. In addition, we incurred debt issuance costs, recorded as a discount on the debt and presented net of the principal balance on the balance sheet. These costs are amortized to interest expense over the life of the debt using the effective interest method. In addition, we recorded amortization of the debt issuance cost.

Results of Operations

Comparison of the Three Months Ended March 31, 2018 and the Three Months Ended March 31, 2019

The following table summarizes our results of operations for the three months ended March 31, 2018 and the three months ended March 31, 2019:

	Three Months Ended March 31, 2018	Three Months Ended March 31, 2019 (in thousands, unaudited)	Change
Operating expenses:			
Research and development	$ 1,304	$ 8,411	$7,107
General and administrative	641	1,885	1,244
Total operating expenses	1,945	10,296	8,351
Operating loss	(1,945)	(10,296)	(8,351)
Change in fair value of derivative liabilities	(781)	–	781
Interest (expense) income, net	(471)	351	822
Net loss	$ (3,197)	$ (9,945)	$ (6,748)

Research and Development Expenses

Research and development expenses increased by $7.1 million to $8.4 million for the three months ended March 31, 2019 as compared to $1.3 million for the same period in 2018. The increase was primarily due to increased headcount resulting in a $1.5 million increase in personnel costs and a $0.5 million increase for stock-based compensation, and a $5.1 million increase in external research and development expenses related to our research programs, as we conducted additional preclinical studies, incurred additional manufacturing costs, incurred costs for clinical start up activities, and incurred increased costs for study and lab materials, research consulting, rent and other professional fees. We expect our research and development expenses will continue to increase as we advance development of our programs and expect to commence clinical activities in 2019.

General and Administrative Expenses

General and administrative expenses increased by $1.2 million to $1.9 million for the three months ended March 31, 2019, as compared to $0.6 million for the same period in 2018. The increase was primarily due to a $0.8 million increase in rent expense, consulting and professional services fees, $0.3 million increase in personnel costs and a $0.1 million increase in stock-based compensation expense. We anticipate that our general and administrative expenses will increase in the future as we increase our general and administrative staff to support the increased research and development operations as well as increases in expense to establish and maintain public company compliant standards.

Change in Fair Value of Derivative Liability

We recorded a loss in the amount of $0.8 million attributable to changes in fair value of the derivative liability during the three months ended March 31, 2018. No such loss was incurred for the three months ended March 31, 2019.

Interest (Expense) Income

During the three months ended March 31, 2019, we generated $0.4 million in interest income from our institutional money market accounts. These accounts were opened in April 2018, and therefore no interest income was generated for the three months ended March 31, 2018. During the three months ended March 31, 2018, interest expense incurred of $0.4 million was related to a convertible note issued to one of our investors, which converted into shares of our Series A convertible preferred stock in March 2018. No such interest expense was incurred during the three months ended March 31, 2019.

Comparison of the Period from July 6, 2017 (date of inception) through December 31, 2017 and the Year Ended December 31, 2018:

The following table summarizes our results of operations for the period from July 6, 2017 (date of inception) through December 31, 2017 and the year ended December 31, 2018:

	Period from July 6, 2017 (Date of Inception) through December 31, 2017	Year Ended December 31, 2018	Change
		(in thousands)	
Operating expenses:			
Research and development	$995	$ 14,127	$ 13,132
General and administrative	804	4,682	3,878
Total operating expenses	1,799	18,809	17,010
Operating loss	(1,799)	(18,809)	(17,010)
Change in fair value of derivative liabilities	–	(781)	(781)
Other income	–	87	87
Interest (expense) income, net	(27)	416	389
Net loss	$ (1,826)	$ (19,088)	$ (17,261)

Research and Development Expenses

Research and development expenses increased by $13.1 million to $14.1 million for the year ended December 31, 2018. Expenses in 2017 were for the period from July 6, 2017 (date of inception) through December 31, 2017 whereas expenses in 2018 were for a full year. The increase was primarily due to increased headcount causing a $3.6 million increase in personnel costs and a $1.4 million increase for stock-based compensation, a $3.3 million increase in external research and development expenses related to our research programs, as we conducted additional preclinical studies, a $ 3.0 million increase in manufacturing costs for study materials and a $1.8 million increase for lab materials, research consulting and other professional fees. We anticipate our research and development expenses will continue to increase as we advance development of all our programs and commence clinical activities in 2019.

General and Administrative Expenses

General and administrative expenses increased by $3.9 million to $4.7 million for the year ended December 31, 2018. Expenses in 2017 were for the period from July 6, 2017 (date of inception) through December 31, 2017 whereas expenses in 2018 were for a full year. The increase was primarily due to a $3.1 million increase in rent, consulting and professional services costs, a $0.6 million increase in employee and related costs and a $0.2 million increase in stock-based compensation expense. We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our expanded operations as well as increases in costs related to our public company compliance efforts.

Change in Fair Value of Derivative Liability

We recorded a loss in the amount of $0.8 million attributable to changes in fair value of the derivative liability during the year ended December 31, 2018. No such loss was incurred for the period from July 6, 2017 (date of inception) through December 31, 2017.

Other Income

In 2018, we received a New York City Biotechnology Tax Credit. This program allows investors and owners of emerging technology companies focused on biotechnology to claim a refundable tax credit for

amounts paid or incurred for certain facilities, operations and employee training in New York City. We recognized $0.1 million in 2018 when these funds were received.

Interest (Expense) Income

In 2018, we generated $0.9 million in interest income from our institutional money market accounts partially offset by the interest expenses incurred related to a convertible note issued to one of our investors, which converted into shares of our Series A convertible preferred stock in March 2018. These accounts were opened in 2018, and therefore no interest income was generated in the period from July 6, 2017 (date of inception) through December 31, 2017.

Liquidity and Capital Resources

Since our inception, we have incurred significant operating losses. Our net losses were $1.8 million and $19.1 million for the period from July 6, 2017 (date of inception) through December 31, 2017 and the year ended December 31, 2018, respectively and were $3.2 million and $9.9 million for the three months ended March 31, 2018 and March 31, 2019, respectively. As of December 31, 2018 and March, 31, 2019, we had an accumulated deficit of $20.9 million and $30.9 million, respectively. To date, we have focused primarily on organizing and staffing our company, raising capital, establishing and protecting our intellectual property portfolio, in-licensing AAV9, developing and progressing our gene therapy product candidates through preclinical studies and preparing for clinical trials, and establishing our manufacturing platform. Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures, and to a lesser extent, general and administrative expenditures.

We do not have any products approved for sale. We do not expect to generate any revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years. Since our inception, we have funded our operations primarily through equity and convertible debt financings, and have raised an aggregate of approximately $129.0 million of gross proceeds through these offerings which includes our Series B convertible preferred stock financing in March 2019. As of December 31, 2018 and March 31, 2019, we had cash and cash equivalents of $63.0 million and $100.3 million, respectively. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to capital preservation and liquidity.

Cash Flows

Historical Cash Flows

The following table shows a summary of our cash flows for the periods presented:

	Period from July 6, 2017 (Date of Inception) through December 31, 2017	Year Ended December 31, 2018	Three Months Ended March 31, 2018	Three Months Ended March 31, 2019
Net cash used in operating activities	$(987)	$(14,011)	$(1,227)	$(12,324)
Net cash used in investing activities	(108)	(627)	(21)	(256)
Net cash provided by financing activities	13,931	64,907	56,910	49,834
Net increase in cash and cash equivalents	$ 12,836	$ 50,269	$ 55,662	$ 37,254

Operating Activities

Cash used in operating activities for the period from July 6, 2017 (date of inception) through December 31, 2017 was $1.0 million. Our net loss was $1.8 million which included noncash charges of $0.6 million, consisting primarily of the stock issuance to REGENXBIO in exchange for the licensing of AAV9, with an aggregate fair value of $0.4 million. The change in our net operating assets was primarily the result of a less than $0.1 million increase in prepaid expenses and other current assets, a $0.4 million increase in accounts payable and accrued liabilities primarily associated with research and development expenses and a decrease in operating lease liabilities of $0.1 million.

Cash used in operating activities for the year ended December 31, 2018 was $14.0 million. Our net loss was $19.1 million, which included noncash charges of $3.4 million, consisting primarily of $0.8 million attributable to changes in fair value of the derivative liabilities associated with the convertible note, $0.6 million related to the amortization of the convertible note issuance costs, discount, and other non-cash interest, $1.6 million of stock-based compensation expense, $0.5 million related to the right-of-use-asset derived from our lease for office and lab space and $0.1 million of depreciation and amortization expense. The change in our net operating assets was primarily the result of a $0.5 million increase in prepaid expenses and other current assets and a $2.3 million increase in accounts payable and accrued expenses primarily associated with research and development expenses and a decrease in operating lease liabilities of $0.2 million.

Cash used in operating activities for the three months ended March 31, 2019 was $12.3 million as compared to $1.2 million for the three months ended March 31, 2018. The increase in cash used was primarily the result of increased operating expenses for research and development type activities, rent and headcount.

Investing Activities

Cash used for investing activities was $0.1 million for the period from July 6, 2017 (date of inception) through December 31, 2017, primarily due to purchases of property and equipment.

Cash used for investing activities was $0.6 million for the year ended December 31, 2018, primarily due to purchases of property and equipment.

Cash used for investing activities was $0.3 million for the three months ended March 31, 2019 as compared to less than $0.1 million for the three months ended March 31, 2018, primarily due to the increase in purchases of property and equipment.

Financing Activities

Cash provided by financing activities during the period from July 6, 2017 (date of inception) through December 31, 2017 was $13.9 million. The inflow was related to net proceeds from the issuance of convertible preferred stock of $3.9 million, net of issuance costs, and convertible debt of $10.0 million, net of issuance costs.

Cash provided by financing activities during the year ended December 31, 2018 was $64.9 million. The inflow was related to net proceeds from the issuance of convertible preferred stock of $64.9 million, net of issuance costs.

Cash provided by financing activities for the three months ended March 31, 2019 was $49.8 million as compared to $56.9 million for the three months ended March 31, 2018, primarily due to the decrease in funds received from the Series B financing in March 2019, which generated $49.8 million of net proceeds, as compared to the Series A financing in March 2018, which generated $56.9 million of net proceeds.

Funding Requirements

We believe our existing cash and cash equivalents, together with the estimated net proceeds from this offering, will be sufficient to fund our operating expenses and capital expenditure requirements at least through the first half of 2021. However, our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially. We have based this estimate on assumptions that may prove to be wrong, and we could expend our capital resources sooner than we expect. Additionally, the process of testing product candidates in clinical trials is costly, and the timing of progress and expenses in these trials is uncertain.

Our future capital requirements will depend on many factors, including:

- the scope and rate of progress of our preclinical and toxicology studies, and clinical trials for PR001, PR006 and PR004 and any future product candidates;
- the scope and costs of manufacturing study materials and manufacturing development, both internally and externally, and clinical and commercial manufacturing activities;
- the cost, timing and outcome of regulatory review of our product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the terms and timing of establishing and maintaining collaborations, licenses and other similar arrangements;
- our efforts to enhance operational systems and our ability to attract, hire and retain qualified personnel, including personnel to support the development of our product candidates;
- the costs associated with being a public company;
- the timing of any milestone and royalty payments to current and future licensors, if any;
- the extent to which we acquire or in-license other best-in-class AAV-based viral vectors, product candidates or technologies; and
- the cost associated with commercializing any product candidates, if they receive marketing approval.

Until such time, if ever, as we can generate substantial product revenues to support our cost structure, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations and other similar arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing and equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise funds through collaborations, or other similar arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us and/or may reduce the value of our common stock. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market our product candidates even if we would otherwise prefer to develop and market such product candidates ourselves.

Contractual Obligations and Commitments

The following table summarizes our commitments to settle contractual obligations at December 31, 2018:

	Payments Due By Period(1)				
	Total	Less than 1 Year	1-3 Years	3-5 Years	More than 5 Years
			(in thousands)		
Operating Lease commitments	$8,874	$1,647	$3,699	$3,963	$2,265
Total	$8,874	$1,647	$3,699	$3,963	$2,265

(1) Total payments due by period is subject to a present value adjustment of $2.7 million. See note 10 to our financial statements appearing elsewhere in this prospectus.

We enter into contracts in the normal course of business with CROs, CDMOs and other third parties for preclinical studies, clinical trials and testing and manufacturing services. Most contracts do not contain minimum purchase commitments and are cancelable by us upon prior written notice. Payments due upon cancellation consist of payments for services provided or expenses incurred, including non-cancelable obligations of our service providers up to one year after the date of cancellation. These payments are not included in the table above as the amount and timing and such payments are not known. One contract with a CDMO does include cancellations fees for batches within 180 days of a statement of work.

We have also entered into license agreements under which we are obligated to make royalty payments and incur annual maintenance fees. We have not included future royalty payments under these agreements in the table above since the payment obligations are contingent upon future events, such as generating product sales. As of December 31, 2018 or March 31, 2019, we were unable to estimate the timing or likelihood of generating future product sales. See the section titled "−License Agreements" above as well as note 3 and note 17 to our financial statements appearing elsewhere in this prospectus for a description of our license agreements.

Critical Accounting Policies and Significant Judgments and Estimates

The preparation of our financial statements in conformity with accounting principles generally accepted in the U.S. requires us to make estimates and judgments that affect the amounts reported in those financial statements and accompanying notes. Although we believe that the estimates we use are reasonable, due to the inherent uncertainty involved in making those estimates, actual results reported in future periods could differ from those estimates.

We believe that the accounting policies described below involve a high degree of judgment and complexity. Accordingly, these are the policies we believe are the most critical to aid in fully understanding and evaluating our financial condition and results of our operations.

Research and Development Costs, Accrued Research and Development Costs and Related Prepaid Expenses

Research and development costs are expensed as incurred. Research and development expenses consist principally of personnel costs, including salaries, stock-based compensation, and benefits for employees, third-party license fees and other operational costs related to our research and development activities, including allocated facility-related expenses and external costs of outside vendors, and other direct and indirect costs. Non-refundable research and development advance payments are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or services are performed.

Leases

We determine if an arrangement is a lease at inception. Operating leases are included in operating lease right-of-use, or ROU, assets, operating lease liabilities, and long-term operating lease liabilities in our balance sheets.

ROU assets represent our right to use an underlying asset for the lease term and lease liabilities represent our obligation to make lease payments arising from the lease. Operating lease ROU assets and liabilities are recognized at commencement date based on the present value of lease payments over the lease term. As our leases do not provide a readily determinable implicit rate, we use our incremental borrowing rate based on the information available at commencement date in determining the present value of lease payments. The operating lease ROU asset also includes any lease prepayments, offset by lease incentives.

Our facilities operating leases have lease and non-lease components for which we have elected to apply the practical expedient and account for each lease component and related non-lease component as one single component. Operating lease cost is recognized on a straight-line basis over the lease term.

Stock-Based Compensation

We measure all stock options and other stock-based awards granted to our employees, directors, consultants and other non-employee service providers based on the fair value on the date of the grant, and we recognize compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. We recognize forfeitures at the time forfeitures occur.

We classify stock-based compensation expense in our statement of operations in the same way the award recipient' s payroll costs are classified or in which the award recipients' service payments are classified.

We use the Black-Scholes option pricing model to estimate the fair value of stock options on the date of grant. Using the Black-Scholes pricing model requires management to make significant assumptions and judgments. We determined these assumptions for the Black-Scholes option-pricing valuation model as discussed below.

Expected Term–The expected term represents the period that the stock-based awards are expected to be outstanding. As we do not have sufficient historical experience for determining the expected term of the stock option awards granted, we based our expected term for awards issued to employees and non-employees using the simplified method which is presumed to be the midpoint between the vesting date and the end of the contracted term.

Risk-Free Interest Rate–The risk-free interest rate is based on the U.S. Treasury yield curve in effect at the date of grant for zero-coupon U.S. Treasury constant maturity notes with terms approximately equal to the stock-based awards' expected term.

Expected Volatility–Since we do not have a trading history of common stock, the expected volatility was derived from the average historical stock volatilities of the common stock of several public companies within the industry that we consider to be comparable to our business over a period equivalent to the expected term of the stock-based awards.

Dividend Rate–The expected dividend rate is zero as we have not paid and do not anticipate paying any dividends in the foreseeable future.

Fair Value of Common Stock–Prior to this offering, the fair value of the shares of common stock underlying the stock-based awards has been determined by our board of directors with input from management. Because there has been no public market for our common stock, our board of directors has determined the fair value of our common stock at the time of grant of the stock-based award by considering a number of objective and subjective factors, including having valuations of the common stock performed by a third-party valuation specialist, as further described below.

The following table summarizes the assumptions for the Black-Scholes option-pricing valuation model used to estimate the fair value of stock options granted during the periods presented:

	Period from July 6, 2017 (Date of Inception) through December 31, 2017	Year Ended December 31, 2018	Three Months Ended March 31, 2018	Three Months Ended March 31, 2019
Expected term	6.1	6.1	6.1	6.0
Risk-free interest rate	2.15 %	2.75 %	2.72 %	2.52 %
Expected volatility	74.67 %	74.57 %	73.72 %	78.50 %
Dividend rate	–	–	–	–
Weighted-average grant date fair value of stock options	$ 0.13	$ 2.39	$ 2.54	$ 1.94

As of December 31, 2018, the total unrecognized compensation expense related to unvested employee and non-employee options was $7.1 million, which we expect to recognize over an estimated weighted-average period of 3.3 years. As of March 31, 2019, the total unrecognized compensation expense related to unvested employee and non-employee options was $7.2 million which we expect to recognize over an estimated weighted-average period of 3.1 years. Based upon the assumed initial public offering price of $17.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, the aggregate intrinsic value of options outstanding as of March 31, 2019 was $41.6 million, of which $10.8 million related to vested options and $30.8 million related to unvested options.

Common Stock Valuations

The fair value of the shares of common stock underlying our stock-based awards has historically been determined by our board of directors with input from management and contemporaneous third-party valuations. We believe that our board of directors has the relevant experience and expertise to determine the fair value of our common stock. Given the absence of a public trading market of our common stock, and in accordance with the *American Institute of Certified Public Accountants Practice Aid, Valuation of Privately-Held Company Equity Securities Issued as Compensation*, our board of directors exercised reasonable judgment and considered numerous and subjective factors to determine the best estimate of the fair value of our common stock at each grant date. These factors include:

- contemporaneous valuations of our common stock performed by independent third-party specialists;
- the prices, rights, preferences, and privileges of our convertible preferred stock relative to those of our common stock;
- the prices of common or convertible preferred stock sold to third-party investors by us and in secondary transactions or repurchased by us in arms-length transactions;
- lack of marketability of our common stock;
- our actual operating and financial performance;
- current business conditions and projections;
- hiring of key personnel and the experience of our management;
- the history of the company and the in-license of our viral vector technology;
- our stage of development;
- likelihood of achieving a liquidity event, such as an initial public offering or a merger or acquisition of our company given prevailing market conditions;
- the market performance of comparable publicly traded companies; and
- the U.S. and global capital market conditions.

In valuing our common stock, our board of directors determined the equity value of our business using the option pricing method, or OPM, with input from management. The OPM is based upon the concept that the securities of a firm' s capital structure can be thought of as call options on the value of a firm. The OPM is appropriate to use when the range of possible future outcomes is difficult to predict and thus creates highly speculative forecasts.

In addition, we also considered any secondary transactions involving our capital stock. In our evaluation of those transactions, we considered the facts and circumstances of each transaction to determine the extent to which they represented a fair value exchange. Factors considered include transaction volume, timing, whether the transactions occurred among willing and unrelated parties, and whether the transactions involved investors with access to our financial information.

Application of these approaches involves the use of estimates, judgment and assumptions that are highly complex and subjective, such as those regarding the time to the liquidation event and volatility. Changes in these estimates and assumptions or the relationships between these assumptions impact our valuations as of each valuation date and may have a material impact on the valuation of common stock.

For valuations after the completion of this offering, our board of directors will determine the fair value of each share of underlying common stock based on the closing price of our common stock as reported by Nasdaq on the date of grant. Future expense amounts for any particular period could be affected by changes in our assumptions or market conditions.

Income Taxes

We account for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the financial statements or our tax returns. Under this method, deferred tax assets and liabilities are determined on the basis of the differences between the financial statements and tax basis of the assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. The effect of a change in tax rates on deferred tax assets and liabilities is recognized in income in the period that includes the enactment date. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes.

We recognize deferred tax assets to the extent that we believe that these assets are more likely than not to be realized. In making such a determination, management considers all available positive and negative evidence, including future reversals of existing taxable temporary differences, projected future taxable income, tax-planning strategies, and results of recent operations. Valuation allowances are provided, if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. If management determines that we would be able to realize our deferred tax assets in the future in excess of their net recorded amount, management would make an adjustment to the deferred tax asset valuation allowance, which would reduce the provision for income taxes.

We record uncertain tax positions in accordance with ASC 740 on the basis of a two-step process in which (1) management determines whether it is more likely than not that the tax positions will be sustained on the basis of the technical merits of the position and (2) for those tax positions that meet the more-likely-than-not recognition threshold, management recognizes the largest amount of tax benefit that is more than 50% likely to be realized upon ultimate settlement with the related tax authority.

We provide reserves for potential payments of tax to various tax authorities related to uncertain tax positions. These reserves are based on a determination of whether and how much of a tax benefit taken by us in our tax filings or positions is more likely than not to be realized following resolution of any potential contingencies related to the tax benefit. Potential interest related to the underpayment of income taxes will be classified as a component of interest expense and any related penalties will be classified in operating expenses in the statement of operations.

Emerging Growth Company Status

In April 2012, the JOBS Act was enacted. Section 107 of the JOBS Act provides that an emerging growth company may take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. Therefore, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

Recently Adopted Accounting Pronouncements

Descriptions of recently issued accounting pronouncements that may potentially impact our financial position, result of operations or cash flows are disclosed in note 2 to our financial statements included elsewhere in this prospectus.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined by applicable SEC rules and regulations.

Quantitative and Qualitative Disclosures about Market Risk

We are exposed to market risks in the ordinary course of our business. Market risk represents the risk of loss that may impact our financial position due to adverse changes in financial market prices and rates. Our market risk exposure is primarily the result of fluctuations in interest rates.

Interest Rate Risk

As of December 31, 2018 and March 31, 2019, we had cash and cash equivalents of $63.0 million and $100.3 million, respectively. Interest-earning instruments carry a degree of interest rate risk. However, due to the nature of these investments, the primary aim of which is capital preservation and liquidity, a hypothetical 10% change in interest rates during any of the periods presented would not have had a material impact on our financial statements included elsewhere in this prospectus. We do not enter into investments for trading or speculative purposes and have not used any derivative financial instruments to manage our interest rate risk exposure, although we may choose to do so in the future.

BUSINESS

Overview

We are a gene therapy company leveraging breakthroughs in human genetics with the goal of developing and commercializing disease-modifying AAV-based gene therapies for patients with devastating neurodegenerative diseases. We are applying a precision medicine approach to neurodegeneration by studying our gene therapies in genetically defined patient populations. We believe this will increase the probability of creating disease-modifying therapies that improve patients' lives. Our lead program is PR001 for the treatment of Parkinson's disease with *GBA1* mutation, or PD-GBA, and neuronopathic Gaucher disease. We are focused on developing a broad pipeline of gene therapies for a range of neurodegenerative diseases, including PR006 for the treatment of frontotemporal dementia with *GRN* mutation and PR004 for the treatment of synucleinopathies.

Our differentiated approach to developing gene therapies for neurodegenerative diseases is designed to mitigate challenges faced by others in the development of therapeutics for the central nervous system, or CNS. We select targets for diseases that correspond to patient populations with particular genetic mutations whom we believe can be treated by increasing or decreasing the expression of a particular gene, which makes them well-suited for gene therapy. We apply our deep understanding of human genetics to design our gene therapy product candidates, each of which is intended to be a one-time treatment to address the key underlying genetic mutation that we believe drives disease progression.

We are developing our lead program, PR001, to treat patients with PD-GBA and neuronopathic Gaucher disease. PD-GBA affects 7% to 10% of the total Parkinson's disease population worldwide and an estimated 90,000 individuals in the United States alone. Gaucher disease is among the most common lysosomal storage disorders, with an estimated global prevalence of one per 30,000 to one per 100,000. Neuronopathic Gaucher disease patients exhibit neurological manifestations in addition to the non-CNS manifestations of Gaucher disease, and represent approximately 6% of all Gaucher disease cases in the United States.

PD-GBA and Gaucher disease share the same underlying genetic mechanism, and we believe they represent a continuum of pathology. The symptoms and severity of the CNS disease in PD-GBA and Gaucher disease depend on the level of enzyme deficiency, which is driven by both the severity and number of *GBA1* mutations. *GBA1* encodes the lysosomal enzyme, beta-glucocerebrosidase, or GCase. PD-GBA patients have a mutation in one chromosomal copy of *GBA1* and Gaucher disease patients have mutations in both chromosomal copies of *GBA1*. These mutations lead to a deficiency of GCase, resulting in the non-CNS manifestations of Gaucher disease as well as lysosomal dysfunction in CNS cells, which we believe leads to the inflammation and neurodegeneration present in PD-GBA and neuronopathic Gaucher disease patients. Approved enzyme replacement therapies, or ERTs, which restore GCase, are effective for the treatment of the non-CNS manifestations of Gaucher disease, but ERTs cannot cross the blood-brain barrier to treat neurodegeneration. Based on the common genetically driven mechanism of PD-GBA and Gaucher disease, we have designed PR001 to express *GBA1* in patients' CNS cells. We believe that restoring *GBA1* in the CNS will slow or stop disease progression in PD-GBA and neuronopathic Gaucher disease patients.

In our comprehensive preclinical program in both mouse models and non-human primates, PR001 was observed to be well tolerated and demonstrated robust and widespread biodistribution. Additionally, in mouse models, we observed significant increases in enzyme activity, reductions in lipid accumulation and improvements in motor function. We submitted an IND to the FDA for PR001 for the treatment of PD-GBA in May 2019, and the FDA has notified us that our trial may proceed. We intend to initiate our Phase 1/2 clinical trial for PR001 in PD-GBA in 2019. We plan to submit an IND to the FDA for PR001 for the treatment of neuronopathic Gaucher disease in mid-2019 and, subject to feedback from the FDA, we intend to initiate a Phase 1/2 clinical trial for PR001 in neuronopathic Gaucher disease in 2019. Our Phase 1/2 clinical trials in these patients will investigate the safety and tolerability of PR001, and will also measure key biomarkers and exploratory efficacy endpoints.

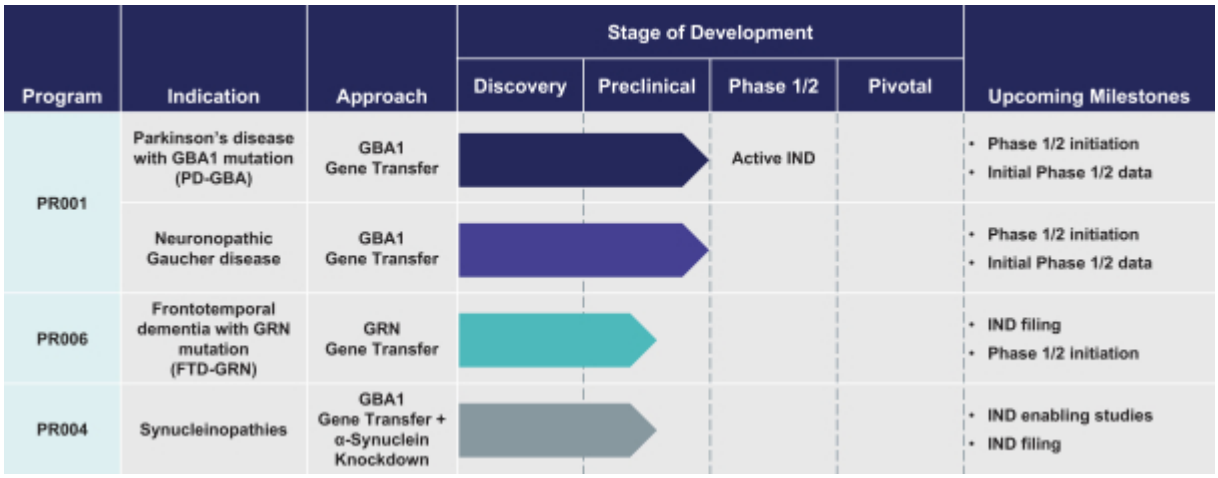
In addition to our lead program, we are developing PR006 for the treatment of frontotemporal dementia with *GRN* mutation, or FTD-GRN. We intend to submit an IND to the FDA for PR006 for the treatment of FTD-GRN in 2019. We are also currently conducting preclinical studies of PR004 for the treatment of synucleinopathies.

All of our current programs utilize adeno-associated virus, or AAV, gene therapy technology, which we believe is particularly well-suited for the treatment of CNS diseases. AAV-based viral vectors have been observed in third-party clinical trials to be well-tolerated and to have promise in delivering stable, long-lasting transgene expression in a range of tissues, including the CNS. We have initially chosen to use AAV9 based on its transformational biological properties and track record, which we believe will translate into a positive clinical effect in our initial indications. In a third-party Phase 1 clinical trial in Type 1 spinal muscular atrophy, AAV9 was observed to enable gene delivery in the CNS and broad brain-wide biodistribution with a single administration. We have entered into license agreements with REGENXBIO Inc., or REGENXBIO, pursuant to which they granted us an exclusive, worldwide license to use AAV9 delivering the gene encoding for GBA1 for the treatment of disease, as well as three distinct exclusive options for specified genes for the treatment of disease. In April 2019, we exercised all three options, including for AAV9 delivering the genes encoding for progranulin and α-Synuclein. For our initial programs, we plan to deliver directly to the cerebrospinal fluid via a minimally invasive non-surgical procedure.

Our company was founded through a collaborative effort by Asa Abeliovich, M.D., Ph.D., our Chief Executive Officer, OrbiMed and The Silverstein Foundation for Parkinson' s with GBA, who shared a common vision: to cure Parkinson' s disease and other neurodegenerative disorders. Dr. Abeliovich and our other scientific leaders bring a deep understanding of the human genetics of neurodegenerative diseases, the underlying molecular mechanisms by which genetic mutations cause these diseases, and how to optimally design potential therapies to restore the function impaired by these genetic mutations. We intend to apply our expertise to developing therapies that have potential to slow or stop disease progression for patients.

Our Pipeline

Our initial AAV9 gene therapy programs are summarized in the table below. We hold worldwide commercial rights to all of our programs.



We are focused on developing a broad pipeline of disease-modifying AAV gene therapies for the treatment of a range of neurodegenerative diseases with high unmet medical need, including Parkinson' s disease, frontotemporal dementia, or FTD, Alzheimer' s disease, amyotrophic lateral sclerosis, or ALS, dementia with Lewy bodies, or DLB, and related lysosomal disorders. Our goal is to use the capabilities we have established for our other product candidates to rapidly advance these programs towards clinical testing.

Our Strategy

We are leveraging breakthroughs in human genetics with the goal of developing and commercializing disease-modifying gene therapies for patients with neurodegenerative diseases with high unmet medical need. Key elements of our strategy to achieve this goal include:

Build a patient-focused gene therapy company. Our company was founded through a collaborative effort by Asa Abeliovich, M.D., Ph.D., OrbiMed and The Silverstein Foundation for Parkinson' s with GBA, who shared a common vision: to cure Parkinson' s disease and other neurodegenerative disorders. We believe we are uniquely positioned to discover and rapidly develop potential gene therapies for patients. We continue to work closely with the neurodegenerative disease community, including scientists and patients and their caretakers, to accomplish this goal.

Apply precision medicine to developing gene therapies for the treatment of neurodegenerative diseases. We select our targets based on our deep understanding of human genetics. By studying our gene therapies in genetically defined patient populations, we are applying a precision medicine approach to neurodegeneration, which we believe will increase the probability of creating disease-modifying therapies that improve patients' lives.

Leverage the transformational potential of AAV gene therapy technology. AAV-based viral vectors have been observed in third-party clinical trials to be well-tolerated and to have promise in delivering stable, long-lasting transgene expression in a range of tissues, including the CNS. We have chosen to use AAV9 for our initial programs and will continue to evaluate the latest scientific understanding of capsid technology for each of our future programs.

Rapidly advance PR001 through clinical trials. In 2019, we intend to initiate a Phase 1/2 clinical trial for PR001 in PD-GBA and a Phase 1/2 clinical trial for PR001 in neuronopathic Gaucher disease. We believe we have established the capabilities to efficiently advance PR001 and our future product candidates through clinical testing.

Continue to develop our innovative pipeline of gene therapies. We are focused on developing a broad pipeline of disease-modifying AAV gene therapies for the treatment of a range of neurodegenerative diseases with high unmet medical need, including Parkinson' s disease, FTD, Alzheimer' s disease, ALS, DLB, and related lysosomal disorders. We are rapidly advancing two of our additional gene therapy product candidates, PR006 and PR004, toward clinical development.

Continue to develop our manufacturing processes to meet clinical and commercial needs. We believe our manufacturing expertise is critical for successfully treating patients with gene therapies, and we have established high-yield, high-potency manufacturing capabilities. In addition, we are actively developing our processes for commercial-scale manufacturing of our gene therapy product candidates.

Our Approach

Our Precision Medicine Approach to Neurodegeneration

Major degenerative disorders of the CNS, including Parkinson' s disease, FTD, Alzheimer' s disease, ALS and DLB, are characterized by their relentless and devastating courses. Patients who suffer from these diseases currently have no therapies available to them that slow or reverse disease progression.

In the past, attempts to identify potential drug targets for neurodegenerative diseases have largely focused on addressing pathological manifestations instead of the underlying genetic cause of disease. Clinical trials have often enrolled patients diagnosed by symptoms only, leading to heterogeneity of the patient populations studied, which can confound trial results. Further, many clinical studies have lacked meaningful early clinical or biomarker endpoints to provide timely proof-of-concept data. As a result of these challenges, the vast majority of potential therapeutics developed to modify or cure neurodegenerative diseases have failed.

Our differentiated approach to developing therapies for neurodegenerative diseases is designed to mitigate these challenges. We select our targets based on our deep understanding of human genetics. By studying our gene therapies in genetically defined patient populations, we are applying a precision medicine approach to neurodegeneration, which we believe will increase the probability of creating disease-modifying therapies that improve patients' lives.

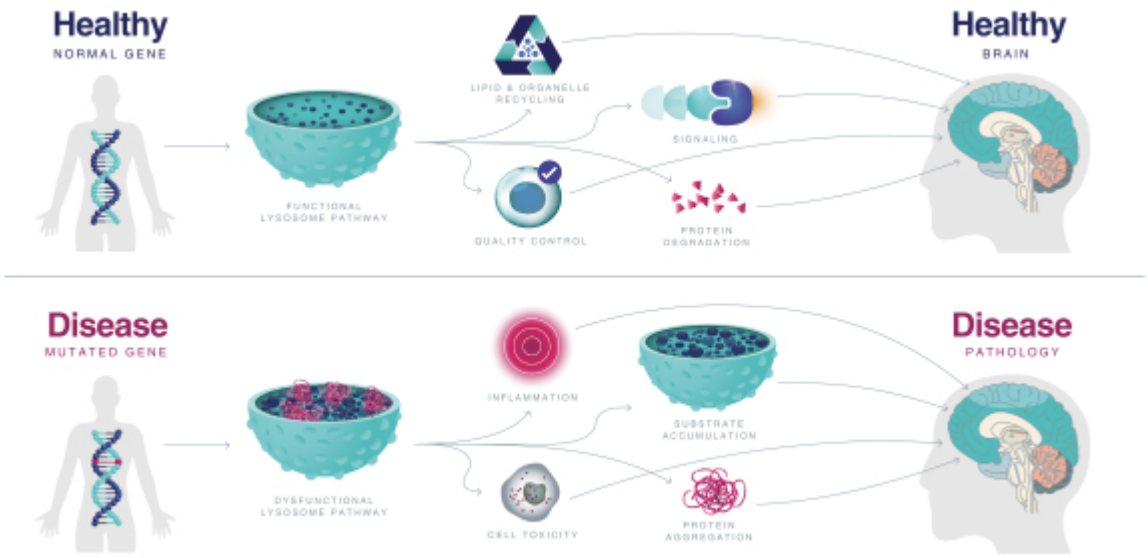
Our founder and Chief Executive Officer, Dr. Abeliovich, and our other scientific leaders bring a deep understanding of the human genetics of neurodegenerative diseases, the underlying molecular mechanisms by which genetic mutations cause these diseases, and how to optimally design potential therapies to restore the function impaired by these genetic mutations. Dr. Abeliovich has spent more than 25 years researching the genetics of neurodegenerative diseases and the molecular mechanisms that translate risk-associated gene variants into disease-causing pathology. In recent years, human genetic studies, including genome-wide association and deep sequencing studies that compare patients with a particular neurodegenerative disease to healthy controls, have identified a number of genes that are directly associated with neurodegenerative diseases, including Parkinson' s disease, FTD, Alzheimer' s disease, ALS and DLB. Many of these identified risk genes are known to play a role in lysosomal function and lysosomal trafficking, and Dr. Abeliovich has been a pioneer in investigating and explaining how these identified genetic mutations, and the resulting lysosomal dysfunction, can cause neurodegeneration. Our team is at the forefront of scientific discovery in the areas of human genetics of neurodegenerative disease, and we intend to apply our expertise to developing therapies that have potential to slow or stop disease progression for patients.

Human Genetics and the Role of Lysosomal Dysfunction in Neurodegeneration

Lysosomes are membrane-bound organelles found in all cells and serve as the cell' s "recycling center." Enzymes within the lysosome act to degrade proteins, lipids and sugars, as well as entire organelles such as mitochondria, that come in from the cell' s cytoplasm (through autophagic trafficking) or its exterior (through endosomal trafficking). Lysosomes play an especially critical role in long-lived cells, such as neurons, and in the aging process.

Genetic mutations in lysosomal genes alter the function of key lysosomal components, such as enzymes, which leads to the accumulation of macromolecules, such as glycolipids and toxic proteins. This in turn leads to insufficient lysosomal function, resulting in toxicity and inflammation, which we believe causes neurodegenerative disease, as shown in the figure below.

Mutations in Lysosomal Genes Cause Lysosomal Dysfunction
Leading to Toxicity, Inflammation and Neurodegenerative Disease



Severe lysosomal deficiencies lead to rare childhood illnesses, called lysosomal storage disorders. More modest lysosomal deficiencies can lead to the gradual accumulation of toxic substrates in long-lived cells, particularly in the CNS, and present as neurodegenerative diseases of aging. Enzyme replacement therapy, or ERT, has been shown to be efficacious in treating certain lysosomal storage disorders, but ERTs cannot cross the blood-brain barrier to treat neurodegenerative diseases or the neurological manifestations of lysosomal storage disorders, such as neuronopathic Gaucher disease.

Numerous studies have elucidated the molecular mechanisms by which lysosomal dysfunction causes the pathology observed in neurodegenerative diseases. For example, PD-GBA patients have reduced levels of the lysosomal acid beta-glucocerebrosidase, or GCase, the lysosomal enzyme encoded by the *GBA1* gene that catalyzes the conversion of the glycosphingolipid substrate glucosylceramide, or GluCer, into glucose and ceramide. In these patients the accumulation of toxic glycolipids induces further lysosomal dysfunction as well as aggregation of α-Synuclein, a pathological hallmark of Parkinson' s disease. Lysosomal dysfunction is also an important feature of FTD in patients with reduced levels of progranulin, caused by *GRN* mutations. Progranulin is required for the maintenance of lysosomes. Other genes associated with Parkinson' s disease, FTD, Alzheimer' s disease, ALS and DLB are also known to encode components of lysosomes or components of the machinery that traffic protein cargo to lysosomes.

We believe that restoring or enhancing lysosomal function will improve the health of CNS cells and slow or stop the progression of neurodegenerative diseases.

Indication and Target Selection

We are applying a precision medicine approach to developing gene therapies for the treatment of neurodegenerative diseases by leveraging our deep understanding of human genetics. We select the indications and targets for our pipeline programs based on the following criteria:

Patient populations with high unmet need. We aim to help patient populations with devastating neurodegenerative diseases where gene therapy could have a transformational impact.

Clearly defined and genetically driven disease mechanism. We believe our deep understanding of human genetics and neurodegenerative disease biology allows us to effectively select targets.

Well-suited for gene therapy. We select targets for diseases that we believe can be treated by increasing or decreasing the expression of a particular gene, which makes them well-suited for gene therapy.

Compelling preclinical data. When possible, we leverage existing preclinical or clinical evidence suggesting that modulating the target will be safe and will modify or cure the underlying pathology.

Genetically defined patient populations. We select targets that correspond to patient populations with a particular genetic mutation, increasing the homogeneity of our studied patient populations and potentially increasing the likelihood of demonstrating efficacy.

Biomarkers that may provide early clinical proof-of-mechanism. We plan to measure biomarkers of target engagement at an early time point in each of our clinical trials, potentially enabling us to establish proof-of-mechanism.

Targets with large potential market opportunity. We plan to pursue targets that have application in at least one sizeable patient population with high unmet need. For example, our lead indication for PR001 is PD-GBA, which affects more than 90,000 individuals in the United States alone.

Our Technological Approach

We are leveraging breakthroughs in human genetics and the transformational potential of AAV gene therapy with the goal of developing and commercializing disease-modifying gene therapies for patients with neurodegenerative diseases with high unmet medical need. AAV-based viral vectors have been observed in third-party clinical trials to be well-tolerated and to have promise in delivering stable, long-lasting transgene expression in a range of tissues, including in the CNS. Many clinical trials of AAV-based gene therapies are currently ongoing, and one AAV-based gene therapy has been approved in the United States.

We believe AAV gene therapy is a technology particularly well-suited for treating CNS diseases. Long-term gene expression and durable results may be achievable in the CNS following one-time dosing with an AAV vector, because most CNS cells typically do not divide or turn over. The CNS is an immune privileged site, which increases the likelihood of efficacy and reduces the risk of harmful immune response when delivery is localized. Targeted delivery to the CNS potentially requires a lower dose than systemic delivery, thereby lowering manufacturing requirements. Further, AAV is not known to cause any disease in humans and AAV gene therapies do not readily integrate into the genome of the target cell, reducing the potential for developing cancer as a result of treatment.

We have chosen to use AAV9 as the capsid, or the outer viral protein shell that encloses the DNA cargo, for our initial programs based on its transformational biological properties and track record, which we believe will translate into a positive clinical effect in our initial target indications. In a third-party Phase 1 clinical trial in Type 1 spinal muscular atrophy, AAV9 was observed to enable gene delivery in the CNS and broad brain-wide biodistribution with a single administration. Once the therapeutic gene is transduced to cells of the CNS, we believe the cells will be able to continue to produce the therapeutic protein for years and, potentially, the rest of the patient' s life.

By developing our AAV9-based gene therapies for genetically defined patient populations, we are applying a precision medicine approach to neurodegeneration, which we believe will increase the probability of creating disease-modifying therapies that improve patient outcomes. We are seeking to replace deficient proteins in genetically defined patient populations where CNS disease is driven by a loss-of-function mutation, and inhibit expression of toxic proteins in the context of a gain-of-function mutation. We have entered into a license agreement with REGENXBIO pursuant to which REGENXBIO granted us an exclusive, worldwide license to use AAV9 delivering the gene encoding for GBA1 for the treatment of disease. We have also entered into a license agreement with REGENXBIO pursuant to which REGENXBIO granted us three distinct exclusive options for specified genes for the treatment of disease. In April 2019, we exercised all three options, including for AAV9 delivering the genes encoding for progranulin and α-Synuclein.

We are engineering and optimizing AAV gene therapies that we believe are best suited for each of our target indications. The key components of an AAV gene therapy include: (1) the capsid, or the outer viral protein shell that encloses the DNA cargo; (2) the therapeutic gene, or transgene; and (3) the regulatory elements that drive the ultimate level of expression of the transgene.

To design the optimal gene therapy for a target patient population, we focus on optimizing each element in order to improve therapeutic outcomes:

Selection of capsid. We believe AAV viral vectors are uniquely well-suited to deliver genetic material to the CNS. We have initially selected AAV9 because it has demonstrated efficacy, an acceptable safety profile, efficient gene delivery in the CNS, broad brain-wide biodistribution and manufacturability in third-party clinical trials in other indications. We will continue to evaluate the latest scientific understanding of capsid technology to select the optimal capsid for each of our future programs.

Selection of transgenes. We design gene therapies with transgenes that express and/or knock down one or more genes that have been identified as potentially disease-modifying based on human genetic studies of neurodegenerative diseases. In the case of diseases caused by loss-of-function mutations, we design codon-optimized DNA encoding wild-type protein, in order to minimize safety risk. In the case of diseases caused by gain-of-function mutations, we design DNA encoding RNA molecules that inhibit expression of the target gene based on RNA interference, or RNAi, technology. We also have a unique approach that combines overexpression and RNAi.

Optimization of regulatory elements. We select the other elements of our vectors, including promotors and enhancers, to optimize expression level, localization, manufacturability and safety. For our initial programs, we have chosen elements that have been validated via inclusion in other gene therapy programs that have been tested in humans.

Route of administration. Our goal is to utilize vectors that can achieve the desired therapeutic effect efficiently following a one-time treatment via a minimally invasive route of administration. For our initial programs, we have chosen to deliver our gene therapies directly to the cerebrospinal fluid, or CSF, via a non-surgical procedure. In preclinical studies, CSF administration of AAV9 has demonstrated effective biodistribution broadly across the CNS and limited toxicity. Intra-CSF delivery typically requires less drug product than systemic delivery.

Feasibility of manufacturing. We seek to utilize vectors that can be produced in a cost-effective, reliable and scalable manner. We selected AAV9 for our initial programs in part due to its well-characterized manufacturing process.

We will continue to evaluate the optimal vector technology for the biology we are addressing and expect that our current and future product candidates will make use of technological advances.

Our Manufacturing Approach

Our strategy is to advance potential therapies into clinical trials as efficiently and safely as possible. To accomplish this goal, we have established our own internal process development capabilities, and we are working with one of our experienced contract development and manufacturing organizations, or CDMOs, to produce material compliant with current good manufacturing practices, or cGMPs, in a robust, state-of-the-art process based on adherent HEK293 cells. This approach is designed to increase our speed of development, ensure consistent product quality and regulatory compliance, ensure predictable production costs, and produce sufficient quantities of virus for our planned Phase 1/2 clinical trials in PD-GBA and neuronopathic Gaucher disease.

Since our goal is to develop therapies that may benefit patient populations of significant size, we are investing in developing a late stage and commercial manufacturing process that can scale to supply a large market. We are working with another CDMO to develop a scalable baculovirus production system for our pipeline in close collaboration with our internal process development team. In this baculovirus production system, AAV vectors are produced by infection of insect cells with recombinant baculoviruses. This scalable suspension production system, using single-use bioreactors, is designed to produce higher yields of vectors more cost-effectively and efficiently than current mammalian cell-based approaches. Other gene therapy companies have used baculovirus production systems to produce AAV material that has been used in human clinical trials and have demonstrated consistent process yields and product qualities. We believe the baculovirus production system will maximize our ability to ensure cost-efficient, safe and scalable supply at the higher quantities required for late-stage clinical development and commercialization.

PR001 for the Treatment of PD-GBA and Neuronopathic Gaucher Disease

We are currently developing PR001, our gene therapy candidate which utilizes an AAV9 vector to deliver codon-optimized DNA encoding wild-type GCase, for PD-GBA and neuronopathic Gaucher disease. We submitted an IND to the FDA for PR001 for the treatment of PD-GBA in May 2019, and the FDA has notified us that our trial may proceed. We intend to initiate our Phase 1/2 clinical trial for PR001 in PD-GBA in 2019. We plan to submit an IND to the FDA for PR001 for the treatment of neuronopathic Gaucher disease in mid-2019 and, subject to feedback from the FDA, we intend to initiate a Phase 1/2 clinical trial for PR001 in neuronopathic Gaucher disease in 2019.

The close causal link between PD-GBA and Gaucher disease has been established by both human genetic and clinical studies, supporting our strategy of developing PR001 for both indications. We believe Parkinson' s and Gaucher diseases represent a continuum of pathology with the same underlying mechanism. The symptoms and severity of the CNS disease depend on the level of enzyme deficiency, which is driven by both the type and number of *GBA1* mutations.

Overview of Parkinson's Disease and Gaucher Disease

Parkinson's Disease

Parkinson' s disease is a progressive neurodegenerative disorder most commonly characterized by resting tremor, bradykinesia (slow movement), rigidity and gait difficulty. While Parkinson' s disease is generally thought of as a disease of motor function, it is now well-recognized that it broadly affects the peripheral and central nervous system. Patients suffer from a range of non-motor symptoms, including loss of sense of smell, difficulty swallowing, urinary symptoms, constipation, sleep behavior disorders, hypotension, depression, psychosis, dementia and cognitive impairment. We estimate that Parkinson' s disease affects up to one million people in the United States and seven million people worldwide.

Parkinson' s disease pathology involves loss of certain neuronal populations (including dopamine neurons), the presence of intraneuronal protein aggregates and other lysosomal abnormalities. These are a consequence of ineffective lysosome-mediated degradation and recycling of defective proteins and other cellular components.

This lysosomal dysfunction leads to toxicity and inflammation as well as the accumulation of α-Synuclein. Alpha-Synuclein is a protein that, in Parkinson' s disease patients, forms toxic aggregates in the brain. Lewy bodies, which characterize Parkinson' s disease, are intraneuronal structures composed in part of these α-Synuclein aggregates.

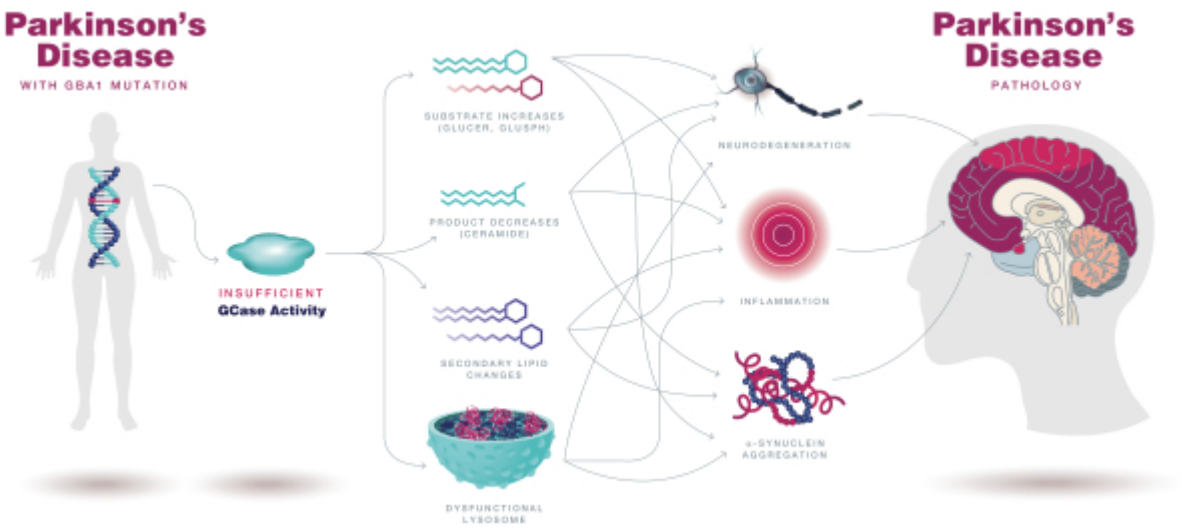
The scientific and medical communities now acknowledge that Parkinson' s disease is a brain-wide disorder. The progression of Lewy body pathology occurs in a predictable, anatomical sequence called Braak stages, with the clinical manifestations generally reflecting the areas of the nervous system involved. At the onset of motor symptoms, patients typically display abundant Lewy body pathology in the ventral midbrain, within the brain stem. However, as the disease progresses, Lewy bodies accumulate more broadly throughout the brain. Such progression can manifest as severe motor symptoms with gait impairment, neuropsychiatric symptoms, and dementia. We believe that disease-modifying therapeutic approaches for Parkinson' s disease should address this brain-wide pathology.

Human genetic studies have identified over 40 potentially causative and risk genes for Parkinson' s disease. Many of these genes are implicated in lysosomal function or lysosomal trafficking, indicating that lysosome dysfunction is the common denominator that underlies Parkinson' s disease pathology. We believe that these genetic mutations cause lysosomal defects, which lead to the neuropathological hallmarks of Parkinson' s disease including α-Synuclein aggregate accumulation, inflammation and further lysosomal abnormalities.

Among these Parkinson' s-associated genes, the *GBA1* gene is considered to be highly clinically relevant. Individuals with a single mutated copy of the *GBA1* gene have a three to ten times higher risk of developing Parkinson' s disease than individuals with no mutations in the *GBA1* gene. Seven to ten percent of Parkinson' s disease patients worldwide and 9% to 10% of Parkinson' s disease patients in the United States have a *GBA1* mutation, yielding an estimated prevalence of an estimated 90,000 to 100,000 PD-GBA patients in the United States alone. *GBA1* mutations impact the risk of developing Parkinson' s disease as well as many other aspects of the disease course, including the severity, age of onset and rate of progression of disease and the likelihood of dementia.

The *GBA1* gene encodes the lysosomal enzyme GCase, which catalyzes the conversion of GluCer into glucose and ceramide. Reduced levels of GCase activity in PD-GBA patients may lead to accumulation of glycolipid substrates including GluCer and glucosylsphingosine, or GluSph, as well as altered production of ceramide and secondary changes in other lipids. The glycolipid substrate accumulation is toxic and pro-inflammatory, leading to lysosomal dysfunction and accumulation and aggregation of α-Synuclein in cells. The reduction in ceramide has also been linked to neurodegeneration and α-Synuclein pathology in model systems, and has been observed in Parkinson' s disease brains. The mechanism by which *GBA1* mutation causes Parkinson' s disease is depicted in the figure below.

Parkinson' s Disease with *GBA1* Mutation



Alpha-Synuclein aggregation and GCase deficiency are thought to act in a "vicious cycle." GCase deficiency causes the accumulation of GluSph substrate, which has been reported to directly affect the accumulation and aggregation of α-Synuclein. In addition, increased α-Synuclein levels lead to less GCase activity, which in turn leads to more α-Synuclein accumulation.

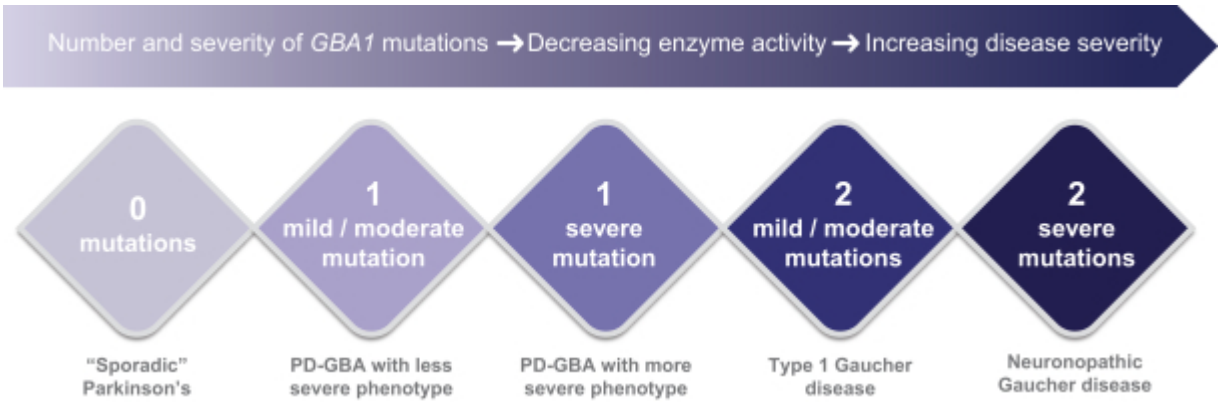
The genetics of *GBA1* mutations and the function of GCase are well-studied and characterized as a result of the study of Gaucher disease, a lysosomal storage disorder caused by deficient levels of active GCase enzyme due to mutations in both copies of the *GBA1* gene.

Gaucher Disease

Gaucher disease is a heterogeneous lysosomal storage disorder characterized by multi-organ pathology. It impacts the liver, spleen, hematopoietic system, bones, lungs and CNS. Gaucher disease pathology in these organs is characterized by accumulation of lipid substrate and degenerative and inflammatory changes. Gaucher disease is among the most common lysosomal storage disorders, with an estimated global prevalence of one per 30,000 to one per 100,000, with a substantially higher incidence in certain populations, such as in individuals of Ashkenazi Jewish descent, who have a prevalence of 118 per 100,000.

Gaucher disease has three subtypes, which are distinguished by the presence or absence of neurological symptoms, severity of symptoms, age at onset and age at death. Type 1, or non-neuronopathic, Gaucher disease is the most common form of the disease. It can occur at any age and involves symptoms including enlarged liver, enlarged spleen, anemia, bone pathology and lung disease. Historically, Type 1 Gaucher disease was distinguished by the absence of neurological symptoms, although it is now recognized that these patients are at elevated risk of developing Parkinson' s disease. Type 2 Gaucher disease represents approximately 1% of Gaucher disease cases in the United States, presents in infancy, affects the CNS and involves rapidly progressing neurodegeneration leading to death in infancy or early childhood. Type 3 Gaucher represents approximately 5% of Gaucher disease cases in the United States, presents in childhood or adulthood, affects the CNS, and involves neurological symptoms such as gaze and motor abnormalities, ataxia, spasticity, myoclonus (involuntary muscle jerks) and seizures. Both Type 2 and Type 3 Gaucher disease, together referred to as neuronopathic Gaucher disease, also result in severe systemic manifestations of the type seen in Type 1 Gaucher disease patients. Although relatively rare in the United States, neuronopathic Gaucher makes up a higher percentage of Gaucher patients in certain non-Western countries, including Japan, Korea, Egypt, India and China. We estimate that there are at least 1,000 neuronopathic Gaucher patients across North America, Europe and Japan.

The relationship between Gaucher disease and PD-GBA has been established by both human genetic and clinical studies, supporting our strategy of developing PR001 for both indications. We believe Gaucher disease and PD-GBA represent a continuum of pathology with the same underlying genetic mechanism. The symptoms and severity of the CNS disease depend on the level of enzyme deficiency, which is driven by both the type and number of *GBA1* mutations, as depicted in the figure below.



Gaucher disease patients have mutations in both chromosomal copies of *GBA1*. Patients with the most severe *GBA1* mutations, which cause severe enzyme deficiency, present with severe neuronopathic (Type 2) Gaucher disease. Patients with less severe mutations generally present with less severe neuronopathic (Type 3) Gaucher, although patients with more severe mutations can also present with Type 3 Gaucher disease. Patients with moderate *GBA1* mutations present with Type 1 non-neuronopathic Gaucher disease, and are at elevated risk of developing Parkinson' s disease. Individuals with a mutation in a single chromosomal copy of *GBA1* do not present with Gaucher disease, but have an elevated risk of developing Parkinson' s disease. In these PD-GBA patients, the severity of the mutation correlates with the severity of the symptoms and the rate of progression of the disease. In addition, clinical studies of Parkinson' s disease patients who do not carry *GBA1* or other known Parkinson' s disease mutations, referred to as "sporadic" Parkinson' s disease patients, also display reduced GCase activity.

Limitations of Current Therapies for Parkinson's Disease and Gaucher Disease

Parkinson's Disease

There are no treatments that modify the progressive underlying disease process of Parkinson' s disease. Current approved therapies for Parkinson' s disease are limited to symptomatic treatments and include levodopa, dopaminergic receptor agonists and inhibitors of enzymes related to dopamine metabolism such as monoamine oxidase inhibitors and catechol-O-methyltransferase inhibitors. These therapies aim to improve overall dopaminergic function. Deep brain stimulation, a procedure in which electrodes are surgically placed in the basal ganglia, either in the subthalamic nucleus or internal globus pallidus, is another option for the treatment of advanced Parkinson' s disease. The benefits of each of these treatments diminishes over time as the disease progresses, and they do not impact the non-motor symptoms or the progression of the disease. As the disease progresses, the non-motor symptoms, such as dementia and cognitive impairment, can lead to severe morbidity and mortality.

Gaucher Disease

There are no therapies approved by the FDA for the treatment of neuronopathic (Type 2 or Type 3) Gaucher disease.

In the United States, several ERTs that consist of recombinant GCase have been approved for the treatment of Type 1 Gaucher disease, including Cerezyme (marketed by Genzyme Corporation, a Sanofi company, or Sanofi Genzyme), Vpriv (marketed by Shire plc, an operating subsidiary of Takeda Pharmaceutical Company Limited) and Elelyso (marketed by Pfizer Inc.). ERTs are sometimes used off-label for the non-neurological manifestations of neuronopathic Gaucher patients; however, ERTs cannot cross the blood-brain barrier to treat the neurological manifestations. ERTs have further limitations, as some patients continue to experience bone pain, lung pathology, thrombocytopenia and enlargement of spleen following long-term ERT treatment. In addition, ERTs are dosed by intravenous infusion approximately once every two weeks, impacting patients' and caregivers' quality of life and productivity.

The FDA has approved several oral substrate reduction therapies, or SRTs, for the treatment of adults with Type 1 Gaucher disease, including Zavesca (marketed by Actelion) and Cerdelga (marketed by Sanofi Genzyme). These therapies are not approved for neuronopathic Gaucher disease.

As a result, we believe there is still significant unmet need among patients with Gaucher disease.

Our Solution

We are developing PR001 as a potentially disease-modifying, single-dose treatment for PD-GBA and neuronopathic Gaucher disease. PR001 is a gene therapy that utilizes an AAV9 viral vector to deliver codon-optimized DNA encoding wild-type GCase to a patient' s cells. Our growing understanding that the pathological process in Parkinson' s disease is brain-wide led us to select AAV9 for gene delivery because of its potential to broadly transduce the CNS. These properties are also important for neuronopathic Gaucher disease, which affects many regions of the CNS. Our intended route of administration for PR001 is via injection into the intra cisterna magna, or ICM, a ventricular space above the spinal canal containing CSF. We believe that PR001, when administered via injection into the CSF, will broadly transduce the cells of a patient' s CNS and produce sufficient GCase to restore healthy lysosomal function and neuronal survival, thereby slowing the progression of disease.

Preclinical Studies

Our comprehensive preclinical program for PR001 was designed to inform efficacy, biodistribution, dosing and safety.

Mouse Models of PD-GBA and Neuronopathic Gaucher Disease

GBA1 mutations are associated with a spectrum of disorders that include PD-GBA and Gaucher disease. Because PD-GBA and Gaucher disease result from the same core biochemical mechanism, many preclinical models are relevant for both diseases. We utilized three mouse models to study the amelioration of GCase deficiency and the associated phenotypes with PR001:

Pharmacological model: Conduritol-ß-epoxide, or CBE, is a pharmacological inhibitor of GCase, and mice treated with CBE display phenotypes consistent with GCase loss-of-function, including lipid accumulation, motor behavior abnormalities and widespread neuropathology, which is a signal of CNS injury. By varying CBE dosage and thus the degree of GCase inhibition *in vivo*, it is possible to recapitulate the degrees of enzyme deficiency seen in PD-GBA and different Gaucher disease types. In our experiments using this model, mice were dosed daily with CBE treatment to create and maintain GCase deficiency and associated phenotypes.

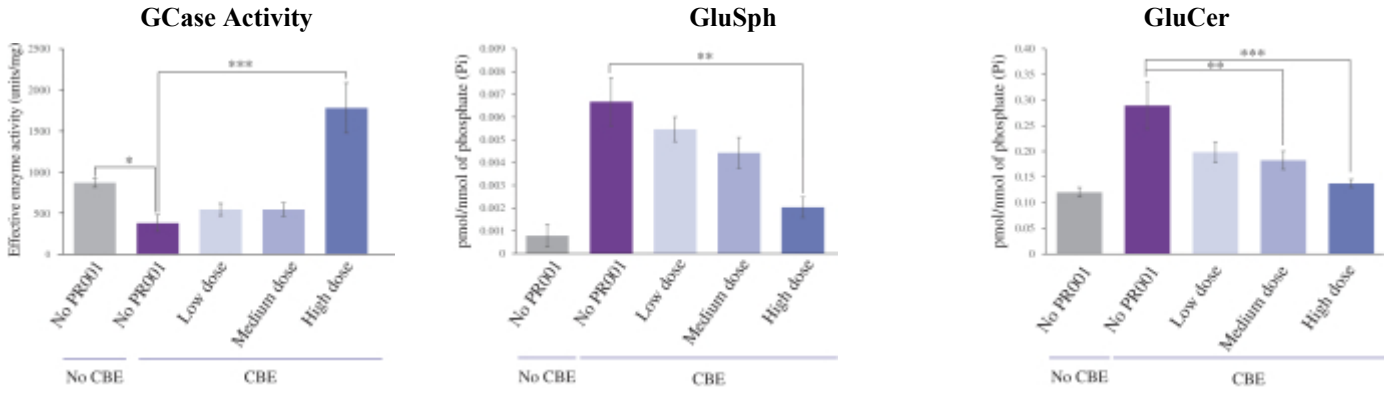
Genetic model: The 4L/PS-NA model of PD-GBA and Gaucher disease combines *Gba1* mutations with mutations in the gene encoding saposin C, an essential activator of GCase. This combination is designed to lead to a more severe reduction in GCase enzyme activity and phenotypes relevant to both PD-GBA and Gaucher disease, including motor behavior abnormalities, α-Synuclein accumulation, neuropathology and visceral involvement.

α-Synuclein model: A53T-SNCA mice, or A53T mice, express a human α-Synuclein A53T mutant transgene and are deficient in murine α-Synuclein. The A53T mutation is associated with familial PD in humans. However, A53T mice display relatively subtle phenotypes, including reduced gastrointestinal motility and variable motor abnormalities between six and 12 months of age. This mouse model does not exhibit widespread α-Synuclein pathology in the brain. Reduction of GCase activity by CBE can lead to changes in the levels of aggregated and toxic forms of α-Synuclein.

Review of Preclinical Data in CBE-Treated Mice

In a dose-finding study, three separate doses of PR001 were administered to CBE mice by intracerebroventricular, or ICV, injection, as ICM injection is not possible in mice. Viral vector doses are denoted by the number of vector genomes, or vg, delivered. For example, our low dose in this study was 3,200,000,000 vector genomes, or 3.2 x 10^9 vg; our medium dose in this study was 10,000,000,000 vector genomes, or 1.0 x 10^{10} vg; and our high dose in this study was 32,000,000,000 vector genomes, or 3.2 x 10^{10} vg. At all three doses, vector genomes were detected in the brain and spinal cord five weeks after administration. GCase activity was reduced in CBE-treated mice and was increased by the highest dose of PR001 in the brain and spinal cord at five weeks. CBE-treated mice also exhibited accumulation of the toxic glycolipids, GluCer and GluSph, in the brain, which was reduced by PR001 administration in a dose-dependent manner.

PR001 Observed to Increase GCase Activity and Reduce Lipid Accumulation in the Cerebral Cortex of CBE-Treated Mice



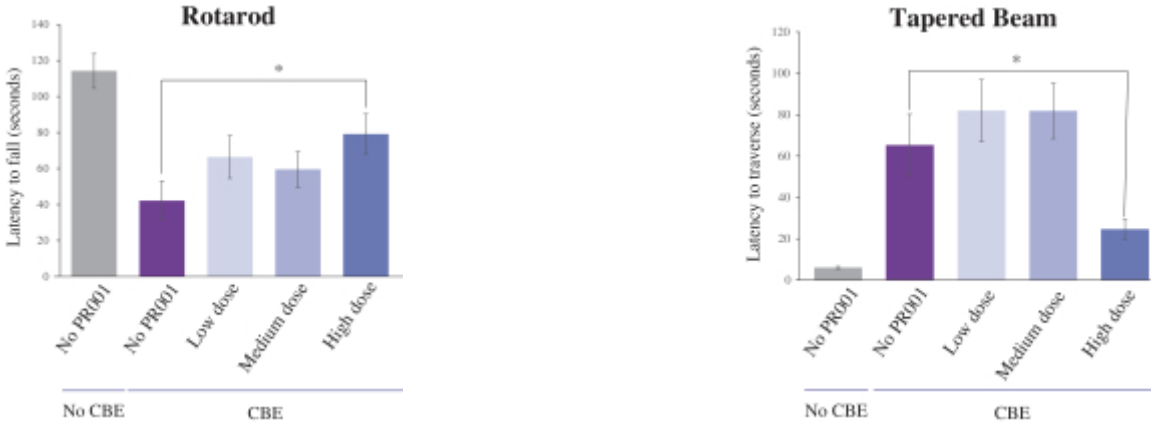
No PR001, no CBE: vehicle only, n = 10; **No PR001 + CBE**: vehicle only + CBE, n = 9; **Low dose**: 3.2 x 10^9 vg PR001 + CBE, n = 6; **Medium dose**: 1.0 x 10^{10} vg PR001 + CBE, n = 10; **High dose**: 3.2 x 10^{10} vg PR001 + CBE, n = 7.

Each bar represents the mean ± standard error of the mean, or SEM.

P-value: *p<0.05; **p<0.01; ***p<0.001 by analysis of variance followed by Tukey HSD, a standard post-hoc analysis. P-value is a conventional statistical method for measuring the statistical significance of results. A p-value of 0.05 or less represents statistical significance, meaning there is a less than 1-in-20 likelihood that the observed results occurred by chance.

In this study, PR001 demonstrated impact on behavior. CBE-treated mice exhibited reduced performance on tests of motor function (rotarod and tapered beam). The highest dose of PR001 resulted in a statistically significant improvement in motor function.

PR001 Observed to Improve Motor Function in CBE-Treated Mice



No PR001, no CBE: vehicle only, n = 10; **No PR001 + CBE:** vehicle only + CBE, n = 9; **Low dose**: 3.2 x 10^9 vg PR001 + CBE, n = 6; **Medium dose:** 1.0 x 10^{10} vg PR001 + CBE, n = 10; **High dose:** 3.2 x 10^{10} vg PR001 + CBE, n = 7.

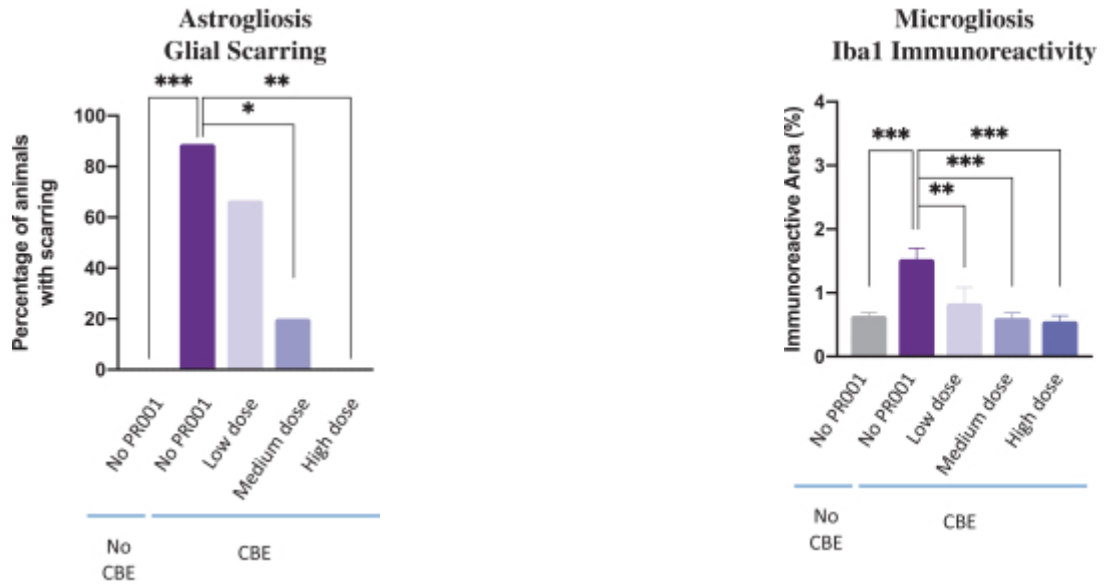
Each bar represents the mean ± SEM.

P-value: *p<0.05 by analysis of variance followed by Tukey HSD.

Additionally, GCase activity in the brain was positively correlated with performance on the rotarod test and negatively correlated with glycolipid levels in the brain in a statistically significant manner.

Lysosomal dysfunction in PD-GBA is thought to lead to neuroinflammation, a process which includes an abnormal increase in astrocytes, or reactive astrogliosis, and microglia, or microgliosis. In CBE-treated mice, CBE induced reactive astrogliosis (evidenced by glial scarring) and microgliosis (evidenced by Iba1 immunoreactivity). In this study, PR001 was observed to reduce reactive astrogliosis and microgliosis in a dose-dependent manner, as shown in the graphs below.

PR001 Observed to Reduce Reactive Astrogliosis and Microgliosis in the Cerebral Cortex of CBE-Treated Mice



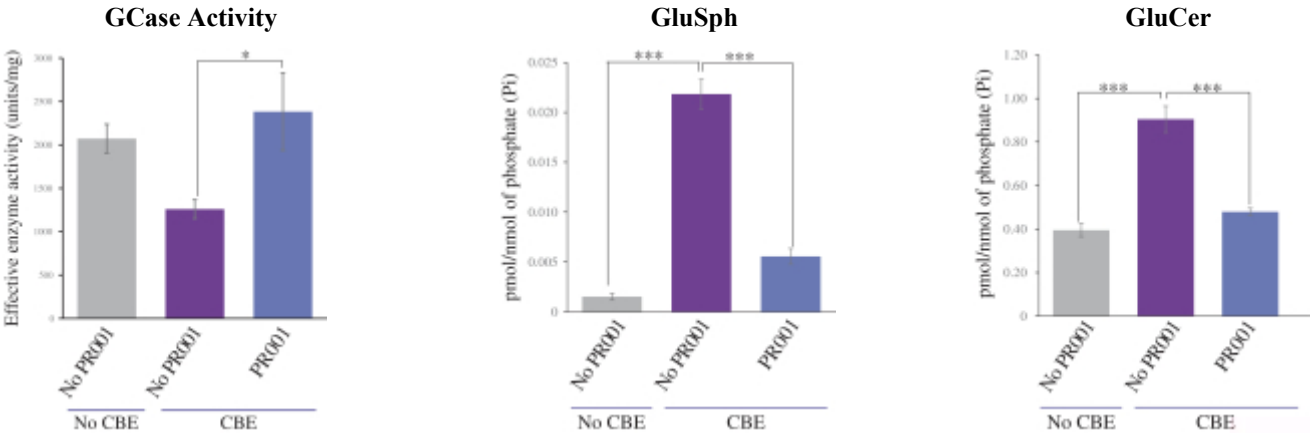
No PR001, no CBE: vehicle only, n = 10; **No PR001 + CBE:** vehicle only + CBE, n = 9; **Low dose**: 3.2 x 10^9 vg PR001 + CBE, n = 6; **Medium dose:** 1.0 x 10^{10} vg PR001 + CBE, n = 10; **High dose:** 3.2 x 10^{10} vg PR001 + CBE, n = 7.

Each bar represents the mean ± SEM.

P-value: *p<0.05; **p<0.01; ***p<0.001 by one-way ANOVA test, or ANalysis Of VAriance, a statistical test used to compare two or more means, followed by the Sidak correction, a method used to control error in making multiple comparisons (for Iba1 immunoreactivity), or by Fischer' s exact test, a test used to compare two categorical variables (for glial scarring).

In a second, longer-term study of PR001 in CBE-treated mice, we observed persistent and durable effects. Six months following ICV administration of a single PR001 dose, GCase activity levels remained significantly increased in the cerebral cortex and a corresponding reduction of lipid accumulation was observed, as shown in the graphs below.

PR001 Observed to Increase GCase Activity and Reduce Lipid Accumulation in the Cerebral Cortex of CBE-Treated Mice at Six Months



No PR001, no CBE: vehicle only, n = 10; **No PR001 + CBE**: vehicle only + CBE, n = 11; **High dose**: 3.2 x 10^{10} vg PR001 + CBE, n = 10.

Each bar represents the mean ± SEM.

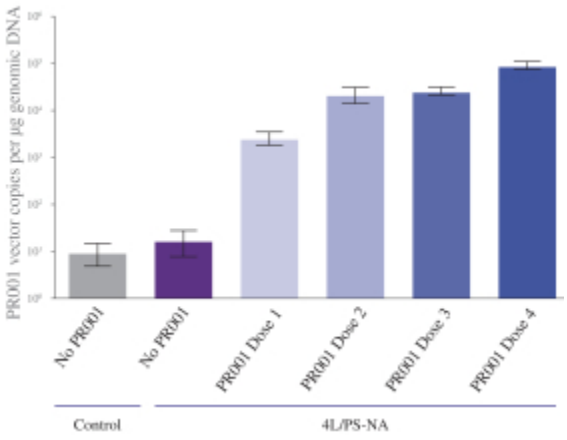
P-value: *p<0.05; ***p<0.001 by analysis of variance followed by Tukey HSD.

Review of Preclinical Data in Genetic Mouse Model

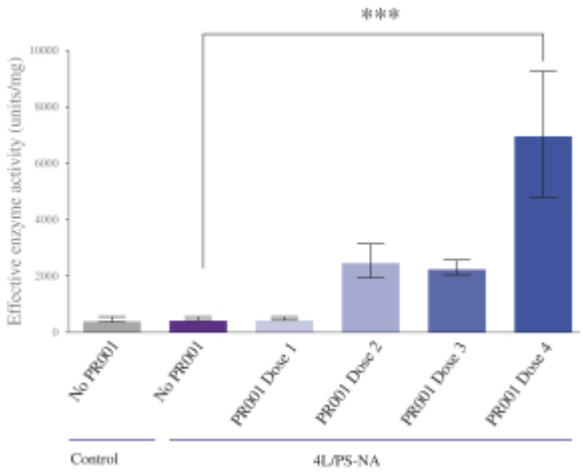
In a study using the 4L/PS-NA genetic mouse model, we observed vector genome presence and statistically significant increases in GCase activity in the cerebral cortex 14 weeks following PR001 administered via ICV injection, as shown in the graphs below.

PR001 Resulted in Biodistribution and Increased GCase Activity in the Cerebral Cortex in Genetic Mouse Model

Biodistribution



GCase Activity



No PR001, Control: vehicle only, n = 10; **No PR001, 4L/PS-NA**: vehicle only, n = 10; **Dose 1**: 4.3 x 10^9 vg PR001, n = 10; **Dose 2**: 4.3 x 10^{10} vg PR001, n = 10; **Dose 3**: 1.3 x 10^{11} vg PR001, n = 7; **Dose 4**: 4.3 x 10^{11} vg PR001, n = 8.

Each bar represents the mean ± SEM.

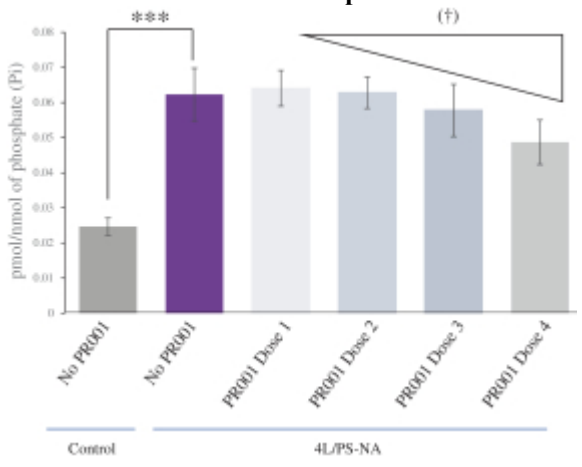
Values obtained in vehicle only injections without PR001 were indistinguishable from background.

P-value: ***p<0.001 by one-way analysis of variance followed by Tukey HSD.

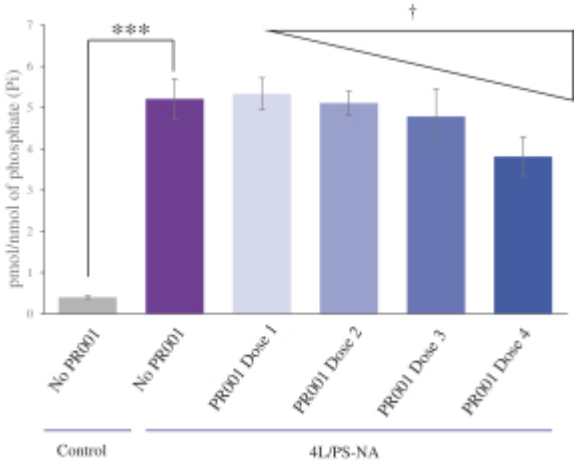
We also observed a dose-dependent trend toward reduced GluSph and GluCer accumulation in the cerebellum 14 weeks following PR001 administered via ICV injection, as shown in the graphs below.

PR001 Observed to Reduce Lipid Accumulation in the Cerebellum in Genetic Mouse Model

GluSph



GluCer



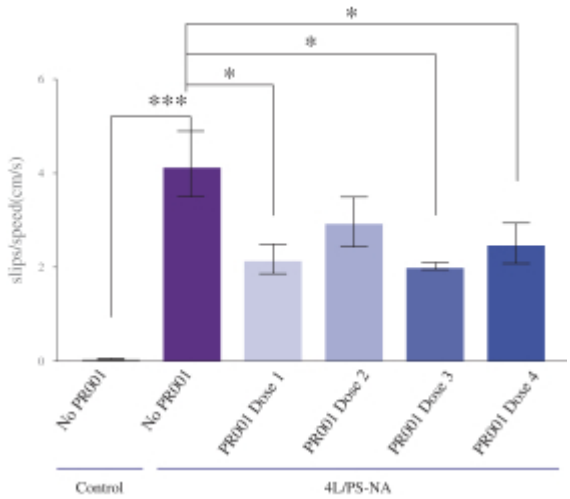
No PR001, Control: vehicle only, n = 10; **No PR001, 4L/PS-NA**: vehicle only, n = 10; **Dose 1**: 4.3 x 10^9 vg PR001, n = 10; **Dose 2**: 4.3 x 10^{10} vg PR001, n = 10; **Dose 3**: 1.3 x 10^{11} vg PR001, n = 7; **Dose 4**: 4.3 x 10^{11} vg PR001, n = 8.

Each bar represents the mean ± SEM.

P-value: ***p<0.001 by one-way analysis of variance followed by Tukey HSD; †p<0.05 for effect of PR001 injected dose by multiple linear regression for genotype and dose across all animals; (†)p<0.1 for effect of PR001 injected dose by multiple linear regression for genotype and dose across all animals.

In this study, administration of PR001 resulted in statistically significant improvement in performance on a beam walk test, a test of motor function, at all doses tested except the second lowest, as shown in the graph below.

PR001 Observed to Improve Motor Function in Genetic Mouse Model



No PR001, Control: vehicle only, n = 10; **No PR001, 4L/PS-NA**: vehicle only, n = 10; **Dose 1**: 4.3 x 10^9 vg PR001, n = 10; **Dose 2**: 4.3 x 10^{10} vg PR001, n = 10; **Dose 3**: 1.3 x 10^{11} vg PR001, n = 7; **Dose 4**: 4.3 x 10^{11} vg PR001, n = 8.

Each bar represents the mean ± SEM.

P-value: *p<0.05; ***p<0.001 by analysis of variance followed by Tukey HSD.

These models display phenotypes representative of PD-GBA and neuronopathic Gaucher disease. Because of known limitations of these preclinical mouse models, we believe that data from both mouse models may not fully reflect the potential efficacy of PR001. In the pharmacological model, CBE administered to mice is known to inhibit GCase. In the genetic model, mutations in an activator of GCase, saposin C, reduce GCase activity. Despite the limitations of these animal models, we observed strong evidence of increased GCase activity, reduced glycolipid accumulation and attenuation of behavioral phenotypes, which support our ongoing development of PR001.

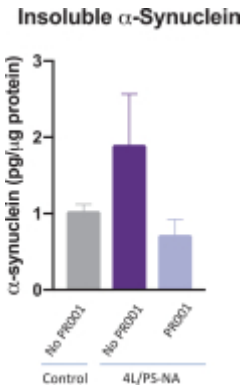
There were no negative histopathologic findings and no evidence of toxicity in either mouse model due to treatment with PR001.

Preclinical α-Synuclein Data

Defects in lysosomal recycling resulting from GCase deficiency are known to result in the accumulation of α-Synuclein protein, a hallmark of Parkinson' s disease. It has been reported that the glycolipid substrates that accumulate in the context of GCase insufficiency may interact directly with α-Synuclein, leading to increased aggregation and toxicity.

Published third-party studies using the 4L/PS-NA genetic mouse model have described the accumulation of insoluble, high molecular weight, or HMW, aggregates of α-Synuclein protein in brain samples. In a study we conducted, we observed a trend toward increased levels of insoluble α-Synuclein protein in the cerebral cortex of 4L/PS-NA mice. In this study, PR001 administration via ICV injection was observed to suppress the accumulation of insoluble α-Synuclein at 14 weeks after PR001 administration, as depicted in the graph below.

PR001 Observed to Reduce Insoluble α-Synuclein in Genetic Mouse Model



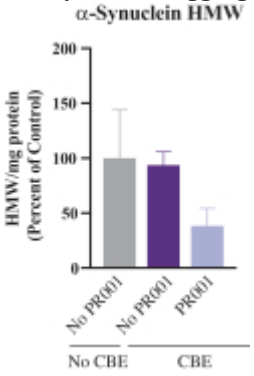
No PR001, Control: vehicle only, n = 5; **No PR001, 4L/PS-NA mice**: vehicle only, n = 5; **PR001, 4L/PS-NA**: 2.4 x 10^{10} vg PR001, n = 4.

Each bar represents the mean ± SEM.

Levels of insoluble α-Synuclein in the 4L/PS-NA genetic mouse model were quantified by enzyme-linked immunosorbent assays, or ELISAs, an analytical biochemistry assay.

We also studied the levels of HMW α-Synuclein in another mouse model, A53T mice treated with CBE. We observed a reduction in HMW α-Synuclein aggregates with PR001 treatment of CBE-dosed A53T mice, as depicted in the graph below.

PR001 Observed to Reduce HMW α-Synuclein Aggregates in α-Synuclein Mouse Model



No PR001, no CBE: vehicle only, n = 4; **No PR001 + CBE:** vehicle only + 100 mg/kg CBE, n = 4; **PR001 + CBE:** 100 mg/kg CBE + 4.3 x 10^{11} vg PR001, n = 5. All experiments were performed in A53T mice.

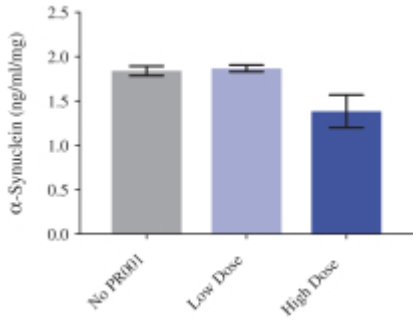
Each bar represents the mean ± SEM.

P value: *p<0.05 by ANOVA followed by Tukey' s HSD multiple tests correction.

Published third-party studies have reported that, in cell lines, increased levels of GCase activity lead to a reduction in the accumulation of α-Synuclein protein. We conducted *in vitro* studies of PR001 in HeLa cells, a

widely studied human cell line, and in mouse hippocampal neuron cultures. In these studies, we observed that administration of PR001 *in vitro* resulted in increased GCase activity and reduced α-Synuclein accumulation, as depicted in the graphs below.

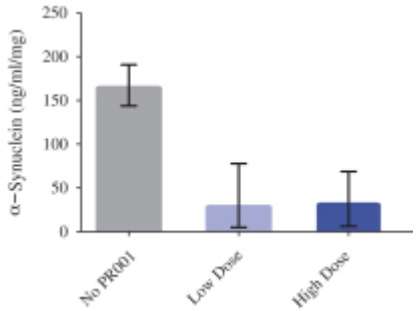
PR001 *In Vitro* Resulted in Reduced α-Synuclein in HeLa Cells



No PR001: vehicle only; **Low dose**: 2 × 10^5 vg/cell PR001; **High dose**: 2 × 10^6 vg/cell PR001.

Each bar represents the mean ± SEM.

PR001 *In Vitro* Resulted in Reduced α-Synuclein in Hippocampal Neurons



No PR001: vehicle only; **Low dose**: 1.3 × 10^5 vg/cell PR001; **High dose**: 1.3 × 10^6 vg/cell PR001.

Each bar represents the mean ± SEM.

Safety and Biodistribution in Non-Human Primates

We conducted PR001 safety and biodistribution testing in healthy non-human primates, or NHPs. NHPs were selected for this Good Laboratory Practice, or GLP, study because of their similarity to humans and because it is possible to deliver PR001 via ICM injection, which is the intended route of administration for our planned clinical trials. There are no NHP models for either PD-GBA or Gaucher disease that are suitable for efficacy measurements. This study included a total of 19 NHPs receiving one of three treatments: vehicle only, PR001 low dose (2.1 x 10^{10} vg/g brain, or vector genomes per gram of brain mass), or PR001 high dose (8.0 x 10^{10} vg/g brain). Pursuant to our pre-determined study design, the NHPs were sacrificed at three timepoints as follows: one NHP receiving PR001 high dose was sacrificed at Day 7 and nine NHPs, three receiving vehicle only, three receiving PR001 low dose and three receiving PR001 high dose, were sacrificed at each of Day 30 and Day 183. Biodistribution and safety were measured in all animals at each timepoint. We plan to use the safety and biodistribution results from these studies to inform dose selection in our clinical trials.

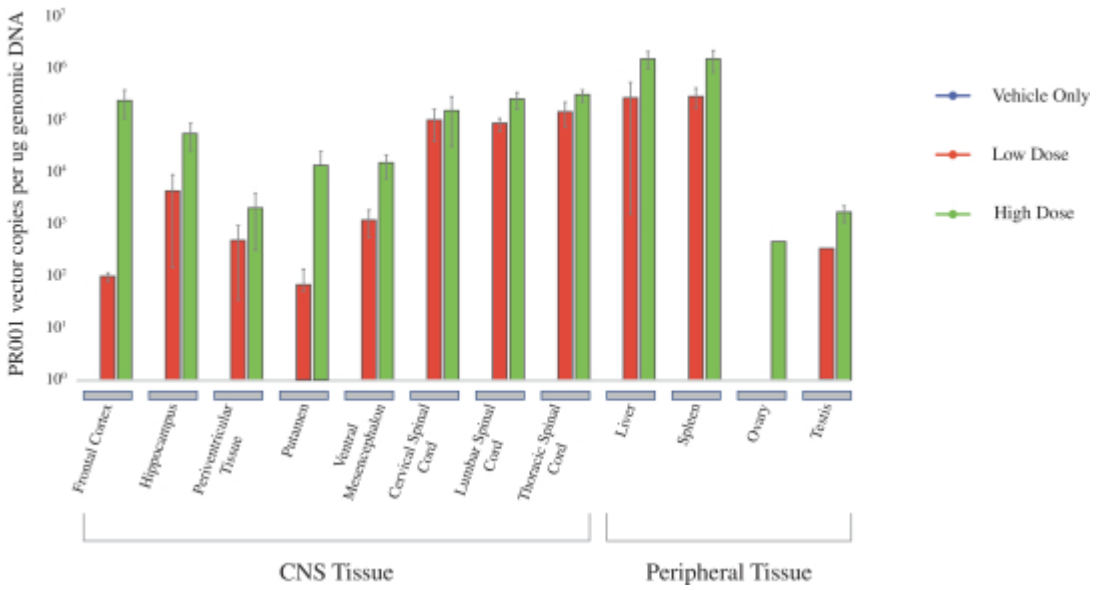
Safety

To characterize the safety profile of PR001, we analyzed multiple time points from the mouse efficacy studies described above and from the GLP toxicology study in NHPs described above. We did not observe any adverse histopathology or evidence of toxicity attributable to PR001 treatment in any of these studies. In the GLP toxicology study in NHPs, PR001 administered via ICM injection was well tolerated, and no toxicity was observed up to six months post-injection at any of the PR001 doses tested.

Biodistribution

To characterize the biodistribution of PR001, we measured the levels of PR001 vector genome copies present in different NHP brain regions at 30 days and six months after ICM injection of vehicle only, a low dose of PR001 or a high dose of PR001. In the NHPs that received PR001, we observed robust and widespread levels of PR001 vector genome copies throughout the CNS as well as peripheral organs at both time points measured, consistent with published studies of the biodistribution of other AAV9-based gene therapy programs with similar routes of administration. The levels of PR001 vector genome copies present in NHPs observed 30 days after dosing are depicted in the graph below.

PR001 Biodistribution Observed at 30 Days After Dosing

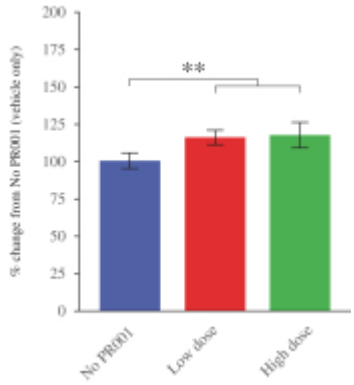


No PR001: vehicle only; **Low dose:** 2.1×10^{10} vg/g brain PR001; **High dose:** 8.0×10^{10} vg/g brain PR001

Each bar represents the mean ± SEM of three NHPs per group. Values for the No PR001 (vehicle only) group are below the detection limit of the vector genome copy assay.

The vector genome copy levels, or "brain exposure levels," of PR001 observed in the NHP CNS at 30 days were comparable to the levels that achieved efficacy in our mouse models. Consistent with widespread vector genome copy distribution, we observed widespread expression of the GCase enzyme encoded by PR001. GCase levels were quantified using an antibody-based automated assay, SimpleWestern. Results from cortex, hippocampus and midbrain samples were obtained from NHPs dosed with ICM injection of vehicle only, a low dose of PR001 or a high dose of PR001. NHPs that received either the low dose or high dose of PR001 exhibited consistently elevated levels of GCase six months after ICM injection, compared to samples from NHPs that received vehicle only. The graph below depicts the data across the cortex, hippocampus and midbrain regions, analyzed in aggregate.

PR001 GCase Expression Observed at Six Months After Dosing



No PR001: vehicle only; **Low dose:** 2.1×10^{10} vg/g brain PR001; **High dose:** 8.0×10^{10} vg/g brain PR001

Each bar represents the mean ± SEM of three NHPs per group.

P-value: ** $p < 0.01$ by analysis of variance followed by Tukey HSD.

We also observed the presence of anti-human GCase antibodies in the NHPs following PR001 administration, consistent with previous third-party studies that evaluated human proteins in NHPs. The presence of these antibodies provided further evidence of the expression of human GCase protein in PR001-treated NHPs.

Overall, this study demonstrated enduring and broad GCase protein expression in NHPs treated with PR001 via ICM injection.

Based on the biodistribution and efficacy data observed in our NHP and mouse studies, we believe that PR001 administered via ICM injection has the potential to increase GCase to non-pathological levels in the CNS of our target patient populations.

Dose Selection

The above studies have informed our proposed dose selection for our first-in-human clinical studies. The efficacy studies in mice helped us identify the level of biodistribution throughout the brain that we predict would translate to efficacy in humans. Our NHP toxicology studies helped us identify the dose that, when delivered by the intended clinical route of administration (ICM injection) in an NHP, corresponds to the same brain exposure level as the optimal dose in mice. We then scaled this NHP ICM dose to humans based on brain weight. This proposed human dose is lower than the highest dose that was observed to be well tolerated in both mouse and NHP studies.

PR001 Planned Clinical Development

Planned Clinical Development of PR001 for the Treatment of PD-GBA

We plan to initiate a randomized, double-blind, sham procedure-controlled, ascending dose Phase 1/2 clinical trial of PR001 in moderate-to-severe PD-GBA patients in 2019. This trial will include up to 16 patients with at least one *GBA1* mutation who are receiving a stable regimen of standard-of-care treatment. Two escalating dose cohorts are planned: 1×10^{14} vg and 2×10^{14} vg of PR001. In each cohort, six patients will receive PR001 and two patients will receive a sham procedure as control. We plan to investigate the effect of PR001 administered as a single injection into the ICM.

The primary outcomes of the trial will include safety and tolerability. Secondary outcomes will include biomarkers including GCase, GluCer and GluSph in the blood and CSF. Exploratory outcomes will include clinical efficacy endpoints used in Parkinson' s disease and additional biomarkers. Change from baseline in

GluCer, GluSph, and GCase enzyme activity will be measured in CSF and blood at three and 12 months, and in blood at additional timepoints. An unblinded interim analysis will be performed once all patients in the first cohort complete 12 months of treatment, and a second unblinded interim analysis will be performed once all patients in the second cohort complete 12 months of treatment. Provided that the safety and tolerability profile of PR001 is acceptable at the time of each of these interim analyses, we will consider rolling over patients who received the sham procedure to a separate protocol under which they would be administered PR001. Additional interim analyses may be performed. All patients will be followed for a total of five years to monitor safety and selected biomarker and efficacy measures.

Based on the results from this initial trial, we plan to obtain input from regulatory agencies and plan additional trials to obtain regulatory approvals for commercialization in geographies worldwide.

Planned Clinical Development of PR001 for the Treatment of Neuronopathic Gaucher Disease

We plan to initiate an open-label Phase 1/2 clinical trial of PR001 in patients with Type 2 Gaucher disease in 2019. This trial will include up to 15 patients who are infants with two *GBA1* mutations and a diagnosis of Type 2 Gaucher disease. We plan to investigate the effect of PR001 administered as a single injection into the ICM. The primary outcomes of the trial will include safety and tolerability. Secondary outcomes will include biomarkers, including GCase, GluCer and GluSph in the blood and CSF, as well as clinical efficacy endpoints appropriate for neuronopathic Gaucher disease. Exploratory outcomes will include additional biomarkers. Change from baseline in GluCer, GluSph, and GCase enzyme activity will be measured in CSF at six (initial interim analysis) and 12 months, and in blood at additional timepoints. An additional interim analysis including clinical endpoints will be performed at 12 months. All patients will be followed for a total of five years to monitor safety and selected biomarker and efficacy measures.

We plan to initiate a Phase 1/2 clinical trial of PR001 in patients with Type 3 Gaucher disease in 2020. This trial will include up to 20 patients with two *GBA1* mutations and a diagnosis of Type 3 Gaucher disease. We plan to investigate the effect of PR001 administered as a single injection into the ICM. The primary outcomes of the trial will include safety and tolerability. Secondary outcomes will include biomarkers, including GCase, GluCer and GluSph in the blood and CSF, as well as clinical efficacy endpoints appropriate for neuronopathic Gaucher disease. Exploratory outcomes will include additional biomarkers. Change from baseline in GluCer, GluSph, and GCase enzyme activity will be measured in CSF at three and 12 months, and in blood at additional timepoints. An analysis of clinical endpoints will be performed at 12 months. All patients will be followed for a total of five years to monitor safety and selected biomarker and efficacy measures.

Based on the results from this initial trial, we plan to obtain input from regulatory agencies on the requirements to file for regulatory approval for commercialization in geographies worldwide.

PR001 Life Cycle Planning

We are continuing to evaluate the opportunity to further develop PR001 in other diseases caused by a deficiency in GCase activity.

PR006, Our Gene Therapy Product Candidate for the Treatment of FTD-GRN

We are developing PR006, our gene therapy candidate which utilizes an AAV9 vector to deliver codon-optimized DNA encoding wild-type progranulin for patients with FTD-GRN. We intend to submit an IND to the FDA for PR006 for the treatment of FTD-GRN in 2019.

Overview of FTD and FTD-GRN

FTD is the most common cause of dementia in people under age 60 and results from the progressive degeneration of the frontal and temporal lobes of the brain. These areas of the brain control decision-making,

behavior, emotion and language. FTD patients present with a spectrum of symptoms, which have been broadly subdivided into behavioral, language and motor manifestations. Patients can experience personality changes, disinhibition, apathy, slow speech production, misuse of grammar, impaired word comprehension, memory loss and, in some cases, motor alterations. Although the clinical presentation of FTD is heterogeneous, FTD is rapidly progressive and invariably devastating.

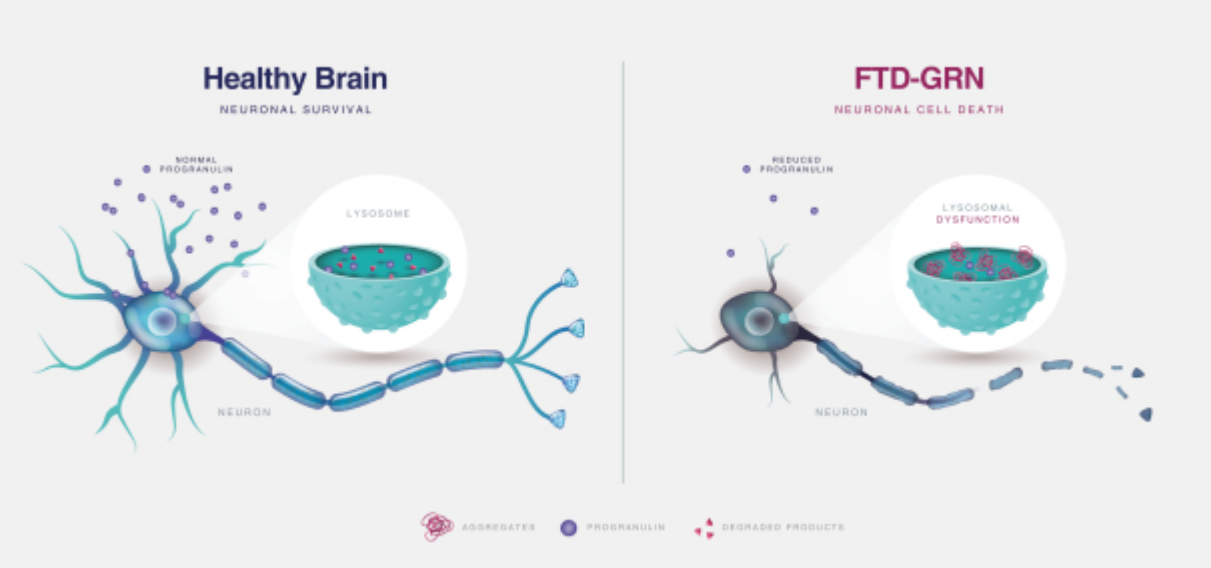
The age of onset of FTD is typically between 45 and 64 years and death typically occurs three to 10 years after symptom onset. Prevalence of FTD is estimated to be 50,000 to 60,000 individuals in the United States and 80,000 to 110,000 individuals in the European Union.

There are currently no approved therapies for FTD.

There are multiple forms of FTD that are caused by genetic mutations, including FTD-GRN. FTD-GRN represents 5% to 10% of all patients with FTD, and approximately 22% of heritable FTD cases. Healthy individuals carry two normal copies of the *GRN* gene that function together to produce sufficient levels of progranulin protein throughout the body. Loss-of-function mutations in a single copy of *GRN* lead to a 50% or greater decrease in the level of progranulin and a greater than 90% probability of developing FTD-GRN. Neuronal ceroid lipofucinosis, a very rare and severe lysosomal storage disorder, results from mutations in both copies of *GRN* and is characterized by childhood dementia, vision loss and epilepsy.

Progranulin is a glycoprotein encoded in humans by the *GRN* gene. Progranulin is found both extracellularly and in lysosomes. It is highly expressed by astroglia and microglia, which are immune cells that reside in the brain. Healthy levels of progranulin are necessary for cellular processes such as lysosomal function, neuronal survival and normal microglial activities. In FTD-GRN patients, reduced levels of progranulin lead to lysosomal dysfunction and ineffective protein degradation and recycling. Consistent with this, FTD-GRN pathology is characterized by aggregates of ubiquitin and TDP-43 proteins, which are believed to be toxic and inflammatory.

Role of Progranulin in FTD-GRN



Our Solution

We are developing PR006 as a potentially disease-modifying, single-dose treatment for FTD-GRN. PR006 is a gene therapy that utilizes an AAV9 viral vector to deliver codon-optimized DNA encoding wild-type

prognulin to a patient' s cells. We believe that PR006, when administered via injection into the CSF, will broadly transduce the cells of a patient' s CNS. Our goal is to produce sufficient progranulin to restore healthy lysosomal function and neuronal survival, thereby slowing or stopping the progression of disease.

Preclinical Studies

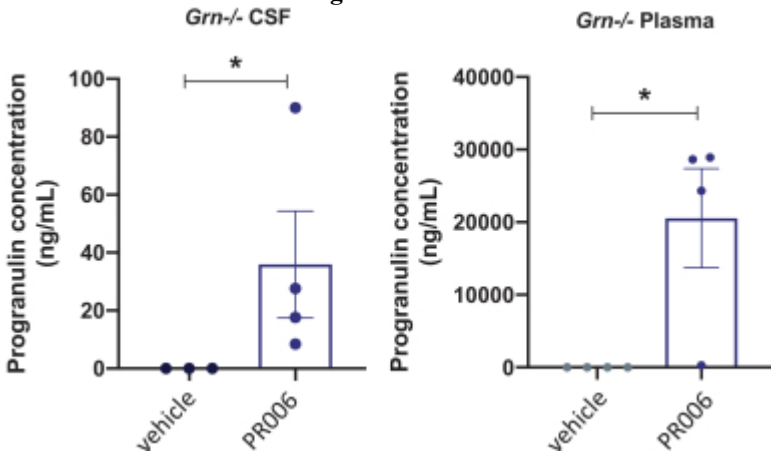
Our preclinical program for PR006 is designed to inform efficacy, biodistribution, dosing and safety. Our program includes several studies conducted in the mouse model of disease, induced pluripotent stem cells, or iPSCs, derived from FTD-GRN patients, and healthy NHPs. Preclinical studies of PR006 are ongoing.

Review of Preclinical Data in GRN Knockout Mice

GRN homozygous knockout, or *Grn-/-*, mice, which completely lack progranulin, are used to study progranulin function. *Grn-/-* mice develop age-dependent pathologies that overlap with FTD-GRN and are consistent with loss of progranulin function, including lysosomal alterations, microgliosis and neuroinflammation. However, these mice do not fully recapitulate FTD-GRN as they do not exhibit FTD-GRN-like neurodegeneration, TDP-43 pathology or cortical atrophy. We are using these mice to study the ability of PR006 to restore progranulin function. FTD-GRN patients have mutations in only one of the two chromosomal copies of *GRN*, in contrast to *Grn-/-* mice, but mice with mutations in only one copy of *Grn* do not display robust phenotypes that resemble FTD-GRN and thus are not useful as disease models.

In a study using 14-month old *Grn-/-* mice, we observed a statistically significant increase in secreted and intracellular progranulin protein in CSF, and plasma two months following ICV administration of 1.46 x 10^{11} vg PR006, as shown in the graphs below.

PR006 Observed to Increase Progranulin Levels in Knockout Mouse Model



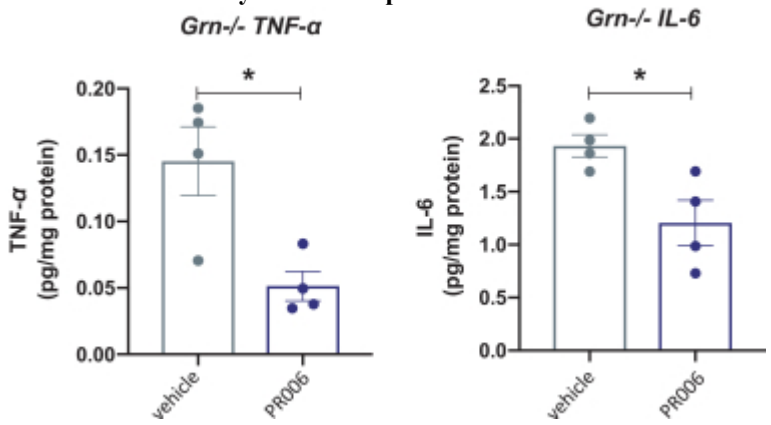
PR006 dose: 1.46 x 10^{11} vg. N = 4 per group.

Each bar represents the mean ± SEM.

P-value: *p < 0.05 by two-tailed Kruskal-Wallis test, a nonparametric statistical test used instead of an ANOVA to compare two or more means.

Chronic CNS inflammation is a hallmark phenotype of FTD-GRN, and *Grn-/-* mice also exhibit age-dependent neuroinflammation. We observed a significant reduction in the cerebrocortical expression of the proinflammatory markers TNF-α and IL-6 two months following ICV administration of PR006, as shown in the graphs below.

PR006 Observed to Suppress Proinflammatory Marker Expression in the Cerebral Cortex of Knockout Mouse Model



PR006 dose: 1.46 x 10^{11} vg. N = 4 per group.

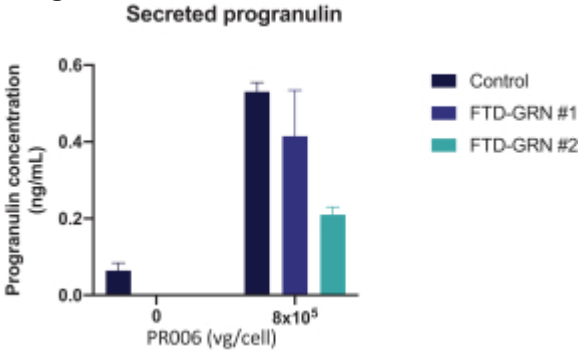
Each bar represents the mean ± SEM.

P-value: *p < 0.05 by two-tailed ANOVA test.

Review of Preclinical Data in Neurons from FTD-GRN Patients

To examine the activity of PR006 in a human model of FTD-GRN, iPSCs from two heterozygous *GRN* mutation carriers, FTD-GRN #1 and FTD-GRN #2, and a wild-type age-matched control were differentiated into neurons. We observed robust levels of secreted progranulin after transducing either control or FTD-GRN neurons with PR006.

PR006 Observed to Increase Levels of Progranulin Secreted in the Cell Media in Both Control and FTD-GRN Patient Neurons



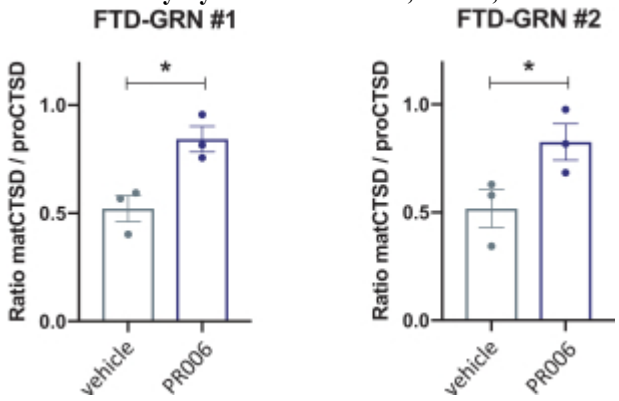
n = 3 or 4 per group.

Each bar represents the mean ± SEM. Values for the untreated group (0 vg/cell PR006) are below the detection limit of the progranulin assay.

Progranulin has been reported to regulate the lysosomal protease Cathepsin D, or CTSD. Loss of CTSD function has also been implicated in lysosomal storage disorders and neurodegeneration. CTSD is expressed as an inactive full-length protein, or proCTSD, that is processed into an enzymatically active mature protease, or matCTSD. Progranulin has been reported to act as a molecular chaperone that binds to proCTSD to enhance its maturation into the matCTSD protease, and absence of progranulin has been reported to decrease CTSD activity by reducing proCTSD processing.

In this study, FTD-GRN neurons displayed reduced maturation of CTSD, quantified as the ratio of matCTSD to proCTSD, compared to controls. We observed a significant increase in this ratio in FTD-GRN neurons after transduction with PR006, indicating a potential role for PR006 in promoting lysosomal function in FTD-GRN neurons, as shown in the graphs below.

PR006 Observed to Promote Maturation of a Key Lysosomal Protease, CTSD, in FTD-GRN Patient iPSC-Derived Neurons



PR006 dose: 1.46 x 10^5 vg/cell.

Each bar represents the mean ± SEM; n = 3 per group.

P-value: *p < 0.05 by a paired t-test, a statistical test comparing two population means.

Safety

We are conducting an ongoing GLP toxicology study in healthy NHPs. NHPs were selected for this GLP study because of their similarity to humans and because it is possible to deliver PR006 via ICM injection, which is the intended route of administration for our planned clinical trials. There are no NHP models for FTD that are suitable for efficacy measurements. When completed, this study will include a total of 19 NHPs receiving one of three treatments: vehicle only, PR006 low dose (1.8 x 10^{10} vg/g brain), or PR006 high dose (1.8 x 10^{11} vg/g brain). Pursuant to our pre-determined study design, one NHP receiving PR006 high dose was sacrificed at Day 7 and nine NHPs, three receiving vehicle only, three receiving PR006 low dose and three receiving PR006 high dose, were sacrificed at Day 30. A further nine NHPs, three receiving vehicle only, three receiving PR006 low dose and three receiving PR006 high dose, will be sacrificed at Day 183. Biodistribution and safety are being measured in all animals at each time point. At the current time, no in-life toxicity has been observed up to 90 days post-injection and no pathological findings have been made up to 30 days post-injection.

PR004, Our Gene Therapy Product Candidate for the Treatment of Synucleinopathies

We are developing PR004 as a potentially disease-modifying, single-dose treatment for certain synucleinopathies. PR004 utilizes an AAV9 vector to deliver codon-optimized DNA encoding wild-type GCase and a molecule that suppresses expression of, or knocks down, α-Synuclein. Our goal is to create an effective therapeutic for patients with neurodegenerative diseases where disease is driven by both synucleinopathy and GCase deficiency.

Overview of Synucleinopathies

Lewy bodies are the main component of brain pathology in synucleinopathies, including Parkinson' s disease and DLB. Genetic mutations in the α-Synuclein gene, including gene multiplications, cause rare familial types of synucleinopathy, demonstrating the relationship between the level of α-Synuclein and disease. The precise

pathological form of α-Synuclein remains to be determined. Various presentations of α-Synuclein have been implicated in disease pathology, including large insoluble neuronal aggregates, small soluble neuronal forms and extracellular forms.

Published third-party studies have highlighted the relationship between synucleinopathy pathology and GCase deficiency. GCase has been shown to directly suppress the accumulation of α-Synuclein and protect from synucleinopathy-related neuronal degeneration in animal models. Both α-Synuclein and GCase are directly implicated in lysosomal function, and abnormal levels of these proteins are thought to synergistically contribute to synucleinopathy pathology.

Our Solution

PR004 is designed to both reduce expression of α-Synuclein and increase expression of GCase. We believe that PR004 is distinct from other potential α-Synuclein-targeted therapies in that it is designed to reduce the expression of all pathological forms of α-Synuclein. In published third-party studies of mouse and cell models of synucleinopathy, it has been observed that higher than normal expression of GCase is protective against disease. We believe our dual-acting approach could have broad applicability across synucleinopathies.

Our preclinical program for PR004 is designed to inform efficacy, biodistribution, dosing and safety. Preclinical studies of PR004 in animal models are ongoing.

Additional Program Opportunities

We are focused on developing a broad pipeline of disease-modifying AAV gene therapies for the treatment of a range of neurodegenerative diseases with high unmet medical need. Beyond PR001, PR006 and PR004, we are studying a number of additional targets. Each of our programs uses AAV vector technology to deliver nucleic acids designed to express and/or knock down one or more genes that have been identified as causal based on human genetic studies of neurodegenerative diseases, including Parkinson' s disease, FTD, Alzheimer' s disease, ALS, DLB and related lysosomal disorders. We believe we have established the capabilities to rapidly advance these programs towards clinical testing.

License Agreements

License Agreement with REGENXBIO Inc. for GBA1

In August 2017, we entered into a license agreement, or the REGENXBIO GBA1 License, with REGENXBIO. Under the REGENXBIO GBA1 License, REGENXBIO granted us an exclusive, worldwide license under certain patents and patent applications to make, have made, use, import, sell and offer for sale products for the treatment of disease, including but not limited to Parkinson' s disease and Gaucher disease, whether or not caused by mutations in the gene that produces the GBA1 enzyme in humans by in vivo gene therapy using AAV9 delivering the gene (or any portion thereof) encoding for GBA1.

We have the right to sublicense the licensed technology to third parties subject to certain conditions as specified in the REGENXBIO GBA1 License. Under the REGENXBIO GBA1 License we granted a non-exclusive, worldwide, royalty-free, transferable, sublicenseable, irrevocable, perpetual license back to REGENXBIO to (1) use any patentable modifications or improvements to the licensed technology that we or our affiliates or sublicensees develop, or licensed back improvements, consummate in scope to REGENXBIO' s retained rights, and (2) to practice the licensed back improvements in connection with AAV9 outside of our field of use.

REGENXBIO and its upstream licensors (SmithKline Beecham Corporation, or GSK, and the Trustees of the University of Pennsylvania, or UPenn) retain the exclusive right over certain antibodies expressed by AAV9

and a nonexclusive right over products delivering RNA interference and antisense drugs using AAV9. GSK and UPenn also retain a non-exclusive right to use the licensed technology for non-commercial research purposes and discovery research efforts with non-profit organizations and collaborators. We do not expect the retention of these rights to affect our intellectual property rights licensed under the REGENXBIO GBA1 License or our ability to develop and commercialize PR001, our product candidate designed to express *GBA1*.

As consideration for the licensed rights under the REGENXBIO GBA1 License, we issued 2,430,000 shares of our common stock in a concurrent private placement to REGENXBIO. In addition, REGENXBIO has the option, at its sole discretion, to participate in any future financing in accordance with the terms of that certain Amended and Restated Investors Rights Agreement, dated as of March 19, 2019, by and among us, REGENXBIO and the other investors party thereto. We are also obligated, pursuant to the REGENXBIO GBA1 License, to pay REGENXBIO: (1) an annual maintenance fee; (2) mid- to high-single digit royalty percentages on net sales of licensed products, subject to reduction in specified circumstances; and (3) mid-teen to low-twenties royalty percentages of any sublicense fees we receive from sublicensees for the licensed intellectual property rights.

The REGENXBIO GBA1 License requires us to use commercially reasonable efforts to develop, commercialize, market, promote and sell a licensed product in our field of use. We are also obligated to achieve a certain development milestone with respect to a licensed product in our field of use within a specified time period. We do not have the right to control prosecution or undertake prosecution of any infringement of the in-licensed patent applications.

In May 2018, we entered into an amendment to the REGENXBIO GBA1 License pursuant to which we are permitted, under certain circumstances, to qualify royalty and diligence obligations that are fulfilled under the REGENGBIO Option Genes License (described below) toward fulfillment of our obligations under the REGENXBIO GBA1 License.

The REGENXBIO GBA1 License will expire on a country-by-country, licensed product-by-licensed product basis upon the later of (1) the expiration, lapse, abandonment or invalidation of the last valid claim of the licensed intellectual property and (2) seven years from the first commercial sale of each licensed product. The licensed patents under the REGENXBIO GBA1 License have expiration dates ranging from 2024 to 2026 in the United States and in 2024 outside of the United States in the absence of any regulatory extension. In addition, patents issuing in the future from any licensed patent applications are expected to have expiration dates in 2024 both inside and outside of the United States in the absence of any regulatory extension. We have the right to terminate the REGENXBIO GBA1 License upon a specified period of prior written notice. REGENXBIO may terminate the REGENXBIO GBA1 License immediately if we become insolvent, if we are late by a specified number of days in paying money due under the REGENXBIO GBA1 License, or if we or our affiliates commence any action against REGENXBIO or its licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate the REGENXBIO GBA1 License for material breach if such breach is not cured within a specified number of days.

License Agreement with REGENXBIO Inc. for Option Genes

In May 2018, we entered into a license agreement, or the REGENXBIO Option Genes License, with REGENXBIO pursuant to which REGENXBIO granted us three distinct exclusive options for specified genes, or the Option Genes, exercisable at our sole discretion through May 10, 2019. Each option represents the right to obtain an exclusive, worldwide license under certain patents and patent applications to make, have made, use, import, sell and offer for sale products for the treatment or prevention of disease, including but not limited to Parkinson' s disease, whether or not caused by mutations in any Option Gene that is the subject of the applicable license, in humans by in vivo gene therapy using AAV9 delivering the applicable licensed Option Gene and/or RNA interference or antisense modalities that target the applicable licensed Option Gene. In addition, we have the right to combine any licensed Option Gene with *GBA1* or any other genes with respect to which we have

acquired a license from REGENXBIO. We also received a non-exclusive, royalty-free, worldwide research license to perform research and development activities for each Option Gene solely for purposes of evaluating whether to exercise the applicable option.

We have the right to sublicense the licensed technology to third parties subject to certain conditions as specified in the REGENXBIO Option Genes License. Under the REGENXBIO Option Genes License we granted a non-exclusive, worldwide, royalty-free, transferable, sublicenseable, irrevocable, perpetual license back to REGENXBIO to (1) use any patentable modifications or improvements to the licensed technology that we or our affiliates or sublicensees develop, or licensed back improvements, consummate in scope to REGENXBIO' s retained rights, and (2) to practice the licensed back improvements in connection with AAV9 outside of our field of use.

REGENXBIO and its upstream licensors (GSK and UPenn) retain the exclusive right over certain antibodies expressed by AAV9. GSK and UPenn retain a non-exclusive right over products that deliver RNA interference and antisense drugs using AAV9, and a non-exclusive right to use the licensed technology for non-commercial research purposes and discovery research efforts with non-profit organizations and collaborators. We do not expect the retention of these rights to affect our intellectual property rights licensed under the REGENXBIO Option Genes License or our ability to develop and commercialize any product candidate that we may choose to pursue, however, the license of these technologies by GSK and UPenn to others could potentially lead to increased competition.

Under the terms of the REGENXBIO Option Genes License, we paid REGENXBIO an initial fee of $0.6 million. In connection with the exercise of each option, we are required to pay REGENXBIO: (1) an additional up-front fee of $0.6 million; (2) an annual maintenance fee; (3) mid- to high-single digit royalty percentages on net sales of the licensed product, subject to reduction in specified circumstances; and (4) mid-teen to low-twenties royalty percentages of any sublicense fees we receive from sublicensees for the licensed intellectual property rights. If a licensed product includes the GBA1 gene and otherwise would be subject to royalties under the REGENXBIO GBA1 License, then royalties for that licensed product will only be due under the REGENXBIO Option Genes License.

In April 2019, we exercised all three options under the REGENXBIO Option Genes License, including for AAV9 delivering the genes encoding for progranulin and α-Synuclein, and paid the additional up-front fee of $0.6 million per option, or an aggregate of $1.8 million, to REGENXBIO.

The REGENXBIO Option Genes License requires us to use commercially reasonable efforts to develop, commercialize, market, promote and sell a licensed product for each licensed Option Gene in our field of use. We are also obligated to achieve a certain development milestone with respect to a licensed product for each licensed Option Gene in our field of use within a specified time period. We do not have the right to control prosecution or undertake prosecution of any infringement of the in- licensed patent applications.

The REGENXBIO Option Genes License will expire on a country-by-country, licensed product-by-licensed product basis upon the later of (1) the expiration, lapse, abandonment or invalidation of the last valid claim of the licensed intellectual property and (2) seven years from the first commercial sale of each licensed product. The licensed patents under the REGENXBIO Option Genes License have expiration dates ranging from 2024 to 2026 in the United States and in 2024 outside of the United States in the absence of regulatory extension. In addition, patents issuing in the future from any licensed patent applications are expected to have expiration dates in 2024 both inside and outside of the United States in the absence of regulatory extension. We have the right to terminate the REGENXBIO Option Genes License upon a specified period of prior written notice. REGENXBIO may terminate the REGENXBIO Option Genes License immediately if we become insolvent, if we are late by a specified number of days in paying money due under the REGENXBIO Option Genes License, or if we or our affiliates commence any action against REGENXBIO or its licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate the REGENXBIO Option Genes License for material breach if such breach is not cured within a specified number of days.

Manufacturing

The ability to consistently produce cGMP-quality AAV vectors at a sufficient scale is a critical success factor for a gene therapy company. We have established our own internal process development capabilities, and we are working with CDMOs to supply our clinical trials with drug product. We intend to supply our first-in-human PR001 clinical trials with cGMP-compliant drug product produced at one of our experienced CDMOs, using a robust, state-of-the-art process based on adherent HEK293 cells. Our team has developed the analytical testing methods needed to support consistency and strict standards of quality and potency. This approach is designed to increase our speed of development, ensure consistent quality and regulatory compliance, ensure predictable production costs, and produce quantities of virus appropriate for our planned Phase 1/2 clinical trials in PD-GBA and neuronopathic Gaucher disease.

In parallel, we are developing the capability to produce PR001 and our other product candidates at a commercial scale. We are working with another CDMO to develop a scalable baculovirus production system for our pipeline in close collaboration with our internal process development team. In this baculovirus production system, AAV vectors are produced by infection of insect cells with recombinant baculoviruses. This scalable suspension production system, using single-use bioreactors, is designed to produce higher yields of vectors more cost-effectively and efficiently than mammalian cell-based approaches. We plan to have the baculovirus-based process developed and producing cGMP-compliant batches in time to support our pivotal clinical trials for each of our programs. We believe the baculovirus production system will maximize our ability to ensure cost-efficient, safe and scalable supply at the higher quantities required for late-stage clinical development and commercialization.

At each stage, we will continually work together with our CDMOs to improve our manufacturing processes and to optimize productivity, efficiency, yield, purity and scalability, and to meet the standards of global regulatory authorities. We will own or license the intellectual property created by our process development activities and will maintain the ability to transfer the process to other third-party CDMOs and/or to our own potential facility to ensure ongoing redundancy and reliability.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. This is also true for the development and commercialization of treatments for Parkinson’ s disease and other neurodegenerative diseases, Gaucher disease, and broadly across gene therapies. While we believe that our focus, strength of team, expertise in gene therapy, scientific knowledge and intellectual property provide us with competitive advantages, we face competition from several different sources, including large and small biopharmaceutical companies, academic research institutions, government agencies and public and private research institutions. Not only must we compete with other companies that are focused on gene transfer technology, but any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, clinical trials, regulatory approvals and product marketing than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We consider our most direct competitors with respect to PR001 to be companies developing GCase pathway-targeting therapies, including Sanofi Genzyme and Lysosomal Therapeutics, Inc. Sanofi Genzyme is

developing SAR402671, a small molecule GluCer synthase inhibitor for the treatment of Parkinson' s disease with a *GBA* mutation and for the treatment of Type 3 Gaucher disease in adult patients. Lysosomal Therapeutics, Inc. is developing LTI-291, a small molecule activator of the GCase enzyme, for the treatment of Parkinson' s patients with a heterozygous mutation in the *GBA* gene. In addition to these investigational programs, there are several products targeting the GCase pathway that are approved or in development for Type 1 Gaucher disease, including approved ERTs and SRTs, but these ERTs and SRTs are not approved for neuronopathic Gaucher disease in the United States. There are other gene therapy companies that are attempting to use both AAV and lentiviral gene therapy approaches to treat Gaucher disease, but to our knowledge, none of those companies has noted plans to pursue PD-GBA.

Several companies are also developing therapies designed to prevent the progression of Parkinson' s disease and FTD. Examples include therapies in development by Alector, Inc., Biogen Inc., Denali Therapeutics Inc., Prothena Corporation plc and Roche Holding AG.

Intellectual Property

Overview

We actively seek to protect our proprietary technology, inventions, and other intellectual property that is commercially important to the development of our business by a variety of means, for example seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. We also may rely on trade secrets and know-how relating to our proprietary technology platform, on continuing technological innovation and on in-licensing opportunities to develop, strengthen and maintain the strength of our position in the field of gene therapy that may be important for the development of our business. Additional regulatory protection may also be afforded through data exclusivity, market exclusivity and patent term extensions where available.

We have fourteen patent applications pending in the United States and foreign jurisdictions, of which six are international patent applications and eight are United States provisional patent applications. Of these, three international patent applications and three United States provisional patent applications relate to PR001, PR006 and PR004, and three international patent applications and five United States provisional patent applications relate to other technologies, in each case as described in more detail below. Each of our pending international patent applications has been filed under the Patent Cooperation Treaty and has not yet entered any national jurisdictions. Our policy is to file patent applications to protect technology, inventions and improvements to inventions that may be commercially important to the development of our business.

We seek United States and international patent protection for a variety of technologies, and own patent applications with claims directed to compositions of matter that relate to our gene therapy products, PR001, PR006 and PR004, and methods for treating diseases of interest using our gene therapy products. We also intend to seek patent protection or rely upon trade secret rights to protect other technologies that may be used to discover and validate targets, and that may be used to manufacture and develop novel gene therapy products. We are a party to license agreements that give us rights to use specific technologies in our gene therapy products and in manufacturing our products.

Patent applications directed to our most advanced programs are summarized below.

PR001

PR001 is an AAV-based gene product configured to express a codon-optimized, human beta-glucocerebrosidase (*GBA1*) gene, which encodes a wild-type GCase therapeutic protein. We own one pending international patent application and one pending United States provisional patent application that contain claims or supporting disclosure directed to the PR001 composition of matter and to methods of treating diseases of interest using PR001. Patents issuing from this application, if any, will have standard expiration dates in 2038.

PR006

PR006 is an AAV-based gene product configured to express a codon-optimized, human progranulin gene, which encodes a wild-type progranulin therapeutic protein. We own one pending international patent application and one pending United States provisional patent application that contain claims or supporting disclosure directed to the PR006 composition of matter and to methods of treating diseases of interest using PR006. Patents issuing from this application, if any, will have standard expiration dates in 2038.

PR004

PR004 is an AAV-based gene product configured to express a codon-optimized, human beta-glucocerebrosidase (*GBA1*) gene, which encodes a wild-type GCase therapeutic protein and a molecule that suppresses expression of α-Synuclein. We own one pending international patent application and one pending United States provisional patent application that contain claims or supporting disclosure directed to the PR004 composition of matter and to methods of treating diseases of interest using PR004. Patents issuing from these applications, if any, will have standard expiration dates in 2038.

Other

We plan to seek United States and international patent protection for a variety of additional technologies.

We own two pending international patent applications and four pending United States provisional patent applications that include claims directed to other AAV-based therapeutic transgenes and methods of treating diseases of interest. Patents issuing from these applications, if any, will have standard expiration dates between 2038 and 2039.

We own one pending international patent application that includes claims directed to compositions and methods for medical imaging. Patents issuing from this application, if any, will have standard expiration dates in 2039.

We own one pending United States provisional patent application that include claims directed to methods of delivering therapeutic molecules. Patents issuing from this application, if any, will have standard expiration dates in 2039.

Trade Secrets

We also rely on trade secrets, know-how, continuing technological innovation and confidential information to develop and maintain our proprietary position and protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. We seek to protect our proprietary technology and processes, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and others who may have access to proprietary information, under which they are bound to assign to us inventions made during the term of their employment or term of service. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems.

Government Regulation

The FDA and other regulatory authorities at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring and post-approval reporting of biologics such as those we are developing.

Biological products are subject to regulation under the Food, Drug, and Cosmetic Act, or FDCA, and the Public Health Service Act, or PHSA, and other federal, state, local and foreign statutes and regulations. We, along with third-party contractors, will be required to navigate the various preclinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates.

U.S. Biologics Regulation

The process required by the FDA before biologic product candidates may be marketed in the United States generally involves the following:

completion of extensive preclinical laboratory tests and animal studies performed in accordance with applicable regulations, including the FDA' s Good Laboratory Practice, or GLP, regulations;

submission to the FDA of an investigational new drug application, IND, which must become effective before clinical trials may begin;

approval by an independent institutional review board, or IRB, or ethics committee at each clinical site before the trial is commenced;

performance of adequate and well-controlled human clinical trials to establish the safety, purity and potency of the proposed biologic product candidate for its intended purpose;

preparation of and submission to the FDA of a biologics license application, or BLA, after completion of all pivotal clinical trials;

satisfactory completion of an FDA Advisory Committee review, if applicable;

a determination by the FDA within 60 days of its receipt of a BLA to file the application for review;

satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the proposed product is produced to assess compliance with current Good Manufacturing Practice requirements, or cGMPs, and to assure that the facilities, methods and controls are adequate to preserve the biological product' s continued safety, purity and potency, and of selected clinical investigation sites to assess compliance with the FDA' s good clinical practices, or GCPs; and

FDA review and approval, or licensure, of a BLA to permit commercial marketing of the product for particular indications for use in the United States.

Preclinical and Clinical Development

Prior to beginning the first clinical trial with a product candidate, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol or protocols for preclinical studies and clinical trials. The IND also includes results of animal and *in vitro* studies assessing the toxicology, pharmacokinetics, pharmacology and pharmacodynamic characteristics of the product, chemistry, manufacturing and controls information, and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day period, raises safety concerns or questions about the proposed clinical trial. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND therefore may or may not result in FDA authorization to begin a clinical trial.

In addition to the submission of an IND to the FDA before initiation of a clinical trial in the United States, certain human clinical trials involving recombinant or synthetic nucleic acid molecules are subject to oversight at

the local level as set forth in the NIH Guidelines. Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an Institutional Biosafety Committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site, and must monitor the study until completed. Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing preclinical studies and clinical trials and clinical study results to public registries.

For purposes of BLA approval of a product candidate, human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

Phase 1. The investigational gene therapy product is initially introduced into patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.

Phase 2. The investigational product is administered to a limited patient population to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks.

Phase 3. The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

When these phases overlap or are combined, the trials may be referred to as Phase 1/2 or Phase 2/3. A Phase 1/2 clinical trial is a first-in-human trial that investigates both safety and preliminary efficacy of an investigational therapy. A Phase 2/3 clinical trial is a human trial that investigates both preliminary and confirmatory efficacy and safety to potentially support submission of a marketing application with the applicable regulatory authorities.

In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may be made a

condition to approval of the BLA. Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the biological characteristics of the product candidate, and must finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product, or for biologics, the safety, purity and potency. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical study investigators. The FDA or the sponsor or its data safety monitoring board may suspend a clinical study at any time on various grounds, including a finding that the research patients or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB' s requirements or if the biological product candidate has been associated with unexpected serious harm to patients. There are also requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries. Sponsors of clinical trials of FDA-regulated products, including biologics, are required to register and disclose certain clinical trial information, which is publicly available at www.clinicaltrials.gov.

BLA Submission and Review

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, nonclinical studies and clinical trials are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. The BLA must include all relevant data available from pertinent preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product' s chemistry, manufacturing, controls, and proposed labeling, among other things. The submission of a BLA requires payment of a substantial application user fee to the FDA, unless a waiver or exemption applies. The FDA has sixty days from the applicant' s submission of a BLA to either issue a refusal to file letter or accept the BLA for filing, indicating that it is sufficiently complete to permit substantive review.

Once a BLA has been accepted for filing, the FDA' s goal is to review standard applications within ten months after it accepts the application for filing, or, if the application qualifies for priority review, six months after the FDA accepts the application for filing. In both standard and priority reviews, the review process is often significantly extended by FDA requests for additional information or clarification. The FDA reviews a BLA to determine, among other things, whether a product is safe, pure and potent for its intended use, and whether the facility in which it is manufactured, processed, packed or held meets standards designed to assure and preserve the product' s identity, safety, strength, quality, potency and purity. The FDA may convene an advisory committee to provide clinical insight on application review questions. Before approving a BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCPs. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates a BLA and conducts inspections of manufacturing facilities where the investigational product and/or its drug substance will be manufactured, the FDA may issue an approval letter or a Complete Response letter. An approval letter authorizes commercial marketing of the product with specific

prescribing information for specific indications. A Complete Response letter will describe all of the deficiencies that the FDA has identified in the BLA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the Complete Response letter without first conducting required inspections, testing submitted product lots and/or reviewing proposed labeling. In issuing the Complete Response letter, the FDA may recommend actions that the applicant might take to place the BLA in condition for approval, including requests for additional information or clarification, which may include the potential requirement for additional clinical studies. The FDA may delay or refuse approval of a BLA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with a Risk Evaluation and Mitigation Strategy, or REMS, to ensure the benefits of the product outweigh its risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a product and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. The FDA may require one or more Phase 4 post-market studies and surveillance to further assess and monitor the product' s safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies.

Expedited Development and Review Programs

The FDA offers a number of expedited development and review programs for qualifying product candidates. The fast track program is intended to expedite or facilitate the process for reviewing new products that meet certain criteria. Specifically, new products are eligible for fast track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a fast track product has opportunities for frequent interactions with the review team during product development and, once a BLA is submitted, the product may be eligible for priority review. A fast track product may also be eligible for rolling review, where the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA, the FDA agrees to accept sections of the BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA.

A product intended to treat a serious or life-threatening disease or condition may also be eligible for breakthrough therapy designation to expedite its development and review. A product can receive breakthrough therapy designation if preliminary clinical evidence indicates that the product, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the fast track program features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product, including involvement of senior managers.

Any marketing application for a biologic submitted to the FDA for approval, including a product with a fast track designation and/or breakthrough therapy designation, may be eligible for other types of FDA programs intended to expedite the FDA review and approval process, such as priority review and accelerated approval. A product is eligible for priority review if it has the potential to provide a significant improvement in the treatment,

diagnosis or prevention of a serious disease or condition. For original BLAs, priority review designation means the FDA' s goal is to take action on the marketing application within six months of the 60-day filing date.

Additionally, products studied for their safety and effectiveness in treating serious or life-threatening diseases or conditions may receive accelerated approval upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA will generally require the sponsor to perform adequate and well-controlled post-marketing clinical studies to verify and describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

Fast track designation, breakthrough therapy designation and priority review do not change the standards for approval but may expedite the development or approval process. Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for this type of disease or condition will be recovered from sales in the United States for that drug or biologic. Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. The orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusive approval (or exclusivity), which means that the FDA may not approve any other applications, including a full BLA, to market the same biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or if the FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the BLA application fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

Post-Approval Requirements

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and

promotion of the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing user fee requirements, under which the FDA assesses an annual program fee for each product identified in an approved BLA. Biologic manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMPs, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMPs and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMPs and other aspects of regulatory compliance.

The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of a product, complete withdrawal of the product from the market or product recalls;
- fines, warning or untitled letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of existing product approvals;
- product seizure or detention, or refusal of the FDA to permit the import or export of products;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of biologics. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. However, companies may share truthful and not misleading information that is otherwise consistent with a product' s FDA approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product' s labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer' s communications on the subject of off-label use of their products.

Biosimilars and Reference Product Exclusivity

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-approved reference biological product. To date, a number of biosimilars have been licensed under the BPCIA, and numerous biosimilars have been approved in Europe. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars.

Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. Complexities associated with the larger, and often more complex, structures of biological products, as well as the processes by which such products are manufactured, pose significant hurdles to implementation of the abbreviated approval pathway that are still being worked out by the FDA.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing that applicant' s own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

The BPCIA is complex and continues to be interpreted and implemented by the FDA. In addition, government proposals have sought to reduce the 12-year reference product exclusivity period. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. As a result, the ultimate impact, implementation, and impact of the BPCIA is subject to significant uncertainty.

Foreign Regulation

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety, and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we would need to obtain the necessary approvals by the comparable foreign regulatory authorities before we can commence clinical trials or marketing of the product in foreign countries and jurisdictions. Although many of the issues discussed above with respect to the United States apply similarly in the context of the European Union, the approval process varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

Other Healthcare Laws and Compliance Requirements

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business. Such laws

include, without limitation: the U.S. federal Anti-Kickback Statute, the civil False Claims Act, HIPAA and similar foreign, federal and state fraud and abuse, transparency and privacy laws.

The U.S. federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, to induce, or in return for, either the referral of an individual, or the purchase or recommendation of an item or service for which payment may be made under any federal healthcare program. The term remuneration has been interpreted broadly to include anything of value, including stock options. The U.S. federal Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, and others on the other hand. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, but they are drawn narrowly, and practices that involve remuneration, such as consulting agreements, that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the U.S. federal Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, a claim including items or services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act.

Civil and criminal false claims laws, and civil monetary penalty laws, including the civil False Claims Act, which can be enforced through civil whistleblower or qui tam actions, prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment to the federal government, including federal healthcare programs, that are false or fraudulent. For example, the civil False Claims Act prohibits any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. A claim includes “any request or demand” for money or property presented to the U.S. government. Several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product.

HIPAA created additional federal civil and criminal liability for, among other things, executing a scheme to defraud any healthcare benefit program, including private third-party payors, and making false statements relating to healthcare matters. In addition, HIPAA, as amended by HITECH, and their implementing regulations, impose certain requirements on HIPAA covered entities, which include certain healthcare providers, healthcare clearing houses and health plans, and individuals and entities that provide services on their behalf that involve individually identifiable health information, known as business associates, relating to the privacy, security and transmission of individually identifiable health information.

The U.S. federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children’ s Health Insurance Program, with specific exceptions, to annually report to CMS information related to payments or other transfers of value made to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members.

We are also subject to additional similar U.S. state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third party payors, including private insurers, or that apply regardless of payor, state laws which require pharmaceutical companies to comply with the pharmaceutical industry’ s voluntary compliance guidelines and the relevant compliance guidance

promulgated by the federal government, state and local laws which require pharmaceutical companies to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures, state laws which require the reporting of information related to drug pricing, state and local laws requiring the registration of pharmaceutical sales and medical representatives, and state and foreign laws governing the privacy and security of health information which, in some cases, differ from each other in significant ways, and may not have the same effect, thus complicating compliance efforts. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply, we may be subject to penalties, including, without limitation, significant civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical or biological product for which we obtain regulatory approval. Sales of any product, if approved, depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state, and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement, if any, for such product by third-party payors. Decisions regarding whether to cover any of our product candidates, if approved, the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. Further, no uniform policy for coverage and reimbursement exists in the United States, and coverage and reimbursement can differ significantly from payor to payor. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization. In addition, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics.

In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost effectiveness of pharmaceutical or biological products, medical devices and medical services, in addition to questioning safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit sales of any product that receives approval. Decreases in third-party reimbursement for any product or a decision by a third-party not to cover a product could reduce physician usage and patient demand for the product. No regulatory authority has granted approval for a personalized cancer immunotherapy based on a vaccine approach, and there is no model for reimbursement of this type of product.

Healthcare Reform

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare

systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by federal and state legislative initiatives, including those designed to limit the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded healthcare programs, and increased governmental control of drug pricing.

The ACA, which was enacted in March 2010, substantially changed the way healthcare is financed by both governmental and private insurers in the United States, and significantly affected the pharmaceutical industry. The ACA contains a number of provisions of particular import to the pharmaceutical and biotechnology industries, including, but not limited to, those governing enrollment in federal healthcare programs, a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, and annual fees based on pharmaceutical companies' share of sales to federal healthcare programs. Since its enactment, there have been judicial, Congressional and executive branch challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. For example, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, it has enacted laws that modify certain provisions of the ACA such as removing penalties, which started on January 1, 2019, for not complying with ACA' s individual mandate to carry health insurance, delaying the implementation of certain ACA-mandated fees, and increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D. On December 14, 2018, a U.S. District Court Judge in Texas ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act. While such U.S. District Court Judge, as well as the Trump administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA.

Other legislative changes have been proposed and adopted since the ACA was enacted, including aggregate reductions of Medicare payments to providers of 2% per fiscal year and reduced payments to several types of Medicare providers. These reductions went into effect in April 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2027 unless additional action is taken by Congress.

Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. At the federal level, the Trump administration' s budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Further, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. HHS has already started the process of soliciting feedback on certain of these measures and, additionally, is immediately implementing others under its existing authority. For example, in September 2018, CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019, and in October 2018, CMS proposed a new rule that would require direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product. On January 31, 2019, the HHS Office of Inspector General proposed modifications to U.S. federal Anti-Kickback Statute safe harbors which, among other things, will affect rebates paid by manufacturers to Medicare Part D plan sponsors, Medicaid

managed care organizations and those entities' pharmacy benefit managers, the purpose of which is to further reduce the cost of drug products to consumers. Although a number of these and other proposed measures may require additional authorization to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Additionally, the Right to Try Act, which was enacted on May 30, 2018, provides a federal framework for certain patients with life-threatening diseases or conditions to access certain investigational new drug products that have completed a Phase I clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a drug manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act.

Facilities

Our principal office is located at 430 East 29th Street, Suite 940, New York, New York 10016, where we lease 17,526 square feet of office and laboratory space. We lease this space under a lease that terminates in March 2025. We believe that these facilities will be adequate for our near-term needs. If required, we believe that suitable additional or alternative space would be available in the future on commercially reasonable terms.

Employees

As of June 6, 2019, we had 43 full-time employees, 24 of whom held an M.D. or Ph.D. degree and 35 of whom are engaged in research and development activities. None of our employees are represented by a labor union or covered by a collective bargaining agreement. We consider our relationship with our employees to be good.

Legal Proceedings

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not currently subject to any material legal proceedings.

MANAGEMENT

Executive Officers and Directors

The following table provides information regarding our current executive officers and directors, including their ages as of June 10, 2019:

Name	Age	Position(s)
Executive Officers		
Asa Abeliovich, M.D., Ph.D.	55	President, Chief Executive Officer and Director
Yong Dai, Ph.D.	48	Chief Technology Officer
Franz Hefti, Ph.D.	71	Chief Development Officer
Brett Kaplan, M.D.	45	Chief Financial Officer
Emily Minkow	37	Chief Business Officer
Jeffrey Sevigny, M.D.	50	Chief Medical Officer
Non-Employee Directors		
Timothy Adams(1)(2)	59	Director
Carl Gordon, Ph.D., C.F.A.(2)	54	Director
Francois Nader, M.D.(1)(3)	63	Director
Ran Nussbaum(1)(2)	46	Director
Peter Thompson, M.D.(3)	59	Director

(1) Member of the audit committee.
(2) Member of the compensation committee.
(3) Member of the nominating and corporate governance committee.

Executive Officers

Asa Abeliovich, M.D., Ph.D., our founder, has served as our Chief Executive Officer and as a member of our board of directors since July 2017. Dr. Abeliovich has extensive industry expertise and more than 25 years of scientific, business and executive management experience in the biotechnology industry. Previously, he was a co-founder of Alector, Inc. and served as a consultant there from October 2013 to December 2017 and he also served as a tenured Associate Professor of Pathology, Cell Biology and Neurology at Columbia University and member of the Taub Institute for Alzheimer' s Disease and the Aging Brain from July 2000 to October 2017. He also served as an attending physician at Neurology at the New York-Presbyterian Hospital and the New York Psychiatric Institute. He is currently on the Scientific Advisory Board of the Silverstein Foundation for Parkinson' s with GBA. Dr. Abeliovich received an M.D. from Harvard Medical School, a Ph.D. from Massachusetts Institute of Technology, and bachelor degrees in Life Sciences and Humanities from the Massachusetts Institute of Technology. We believe Dr. Abeliovich' s deep understanding of human genetics and his extensive scientific, business and executive management experience in the biotechnology industry make him particularly well-qualified to serve on our board of directors.

Yong Dai, Ph.D. has served as our Chief Technology Officer since February 2019. He joined us in March 2018 as the Senior Vice President of Gene Therapy, bringing more than 17 years of biotechnology industry experience focused on bioprocess development, chemistry, manufacturing and controls, or CMC, good manufacturing process, process validation and regulatory submissions, including extensive work with gene therapy drug development. Prior to joining us, Dr. Dai worked at uniQure NV, a gene therapy company, from November 2015 to March 2018, where he most recently served as Senior Director of Process and Analytical Development. Prior to his time at uniQure, he served as the Head of Process Development Commercial Support at Shire Pharmaceuticals from 2009 to 2015 and held senior CMC roles at ImmunoGen, Inc., a biotechnology company,

prior to that. Dr. Dai received an M.S. in biochemistry and molecular biology and a B.S. in Biochemistry from Peking University and a Ph.D. in biochemistry and molecular biology from Chinese Academy of Sciences. He completed his postdoctoral research at Harvard University.

Franz Hefti, Ph.D. has served as our Chief Development Officer since March 2018. Prior to joining us, he was Chief Operations Officer at Proclara Biosciences, Inc., a biotechnology company, from June 2014 to December 2017. Prior to this, he served as President and Chief Executive Officer of Acumen Pharmaceuticals, Inc. from November 2011 to January 2015. Previously, Dr. Hefti served as Chief Scientific Officer at Avid Radiopharmaceuticals, Inc., a pharmaceutical company acquired by Eli Lilly and Company, Executive Vice President of Drug Development at Rinat Neuroscience Corporation, a pharmaceutical company acquired by Pfizer Inc., Senior Vice President of Neuroscience Research at Merck & Co., Inc., or Merck, and Head of Neuroscience Research at Genentech, Inc., a biotechnology corporation acquired by F. Hoffmann-La Roche AG, or Roche. Dr. Hefti currently serves on the boards of directors of several private biotechnology companies, including Spinogenix, Inc., Sophren Inc., Cadent Therapeutics, Inc. and Proclara Biosciences Inc. He has also held positions as a Professor at the University of Southern California and as Associate Professor at the University of Miami, where he carried out discovery research on therapeutic approaches to neurodegenerative diseases. He has been a member of the Scientific Advisory Board of the Alzheimer' s Disease Imaging Initiative since 2013. Dr. Hefti received a Ph.D. in biology from the University of Zurich and completed his postdoctoral research at the Massachusetts Institute of Technology.

Brett Kaplan, M.D. has served as our Chief Financial Officer since November 2018. From August 2010 to November 2018, he worked at Evercore, an investment bank, where he most recently served as Managing Director with a focus on mergers and acquisitions and equity financings in the biopharmaceutical industry. Prior to Evercore, Dr. Kaplan was an Equity Research Analyst at Cowen and Company, an investment bank, from 2007 to 2010. Previously, Dr. Kaplan served as Director of Corporate Development at Cubist Pharmaceuticals, a pharmaceutical company acquired by Merck & Co., Inc., or Merck, in 2014, Manager of Strategic Medical Marketing at Biopure Corporation, and Manager of Corporate Development and Strategy at Eli Lilly and Company. Dr. Kaplan received an M.B.B.Ch. and an M.B.A. from the University of Witwatersrand.

Emily Minkow has served as our Chief Business Officer since November 2018 and previously served as our Executive Vice President of Business Development and Strategy since November 2017. Prior to joining us, Ms. Minkow worked at Celgene Corporation, a global biopharmaceutical company, from August 2010 to October 2017, where she most recently served as Executive Director of Business Development. At Celgene, Ms. Minkow also served as Principal to the Chairman and CEO and as Director of Global Marketing. Prior to Celgene, she was a consultant for Frankel Group, where she advised biotech and pharmaceutical companies on clinical and commercial strategy. Ms. Minkow received an M.B.A. from Harvard Business School and a B.A. in public and international policy from Princeton University.

Jeffrey Sevigny, M.D. has served as our Chief Medical Officer since March 2018. Prior to joining us, he served as Vice President and Global Head of Translational Medicine Neuroscience at Roche from January 2016 to March 2018. Prior to Roche, he was Senior Director of Clinical Development at Biogen Inc., a multinational biotechnology company, from September 2010 to January 2016. Previously, he served as Principal Medical Scientific Expert of Neuroscience at Novartis AG, a multinational pharmaceutical company, and as Associate Director of Neuroscience at Merck. Dr. Sevigny has also held academic appointments as Assistant Professor of Neurology at Albert Einstein School of Medicine and Assistant Professor of Clinical Neurology at Columbia University College of Physicians and Surgeons. Dr. Sevigny received an M.D. from Tufts University School of Medicine and an A.B. in biochemistry from Bowdoin College. He completed a neurology residency at the Neurological Institute of New York at Columbia University Medical Center and a fellowship in Aging & Dementia and Neuro-Epidemiology at Sergievsky Center at Columbia University and Columbia University Mailman School of Public Health.

Non-Employee Directors

Timothy Adams has served as a member of our board since April 2019. Mr. Adams has served as Chief Financial Officer of ObsEva SA since January 2017. From June 2014 to September 2016, Mr. Adams served as the Chief Financial Officer of Demandware, Inc., and prior to that, he served as Senior Vice President and Chief Financial Officer of athenahealth, Inc. from January 2010 to June 2014. Previously, Mr. Adams served as Chief Investment Officer of Constitution Medical Investors, Inc., a private investment firm focused on health-care-sector-related acquisitions and investments, as well as Senior Vice President of Corporate Strategy for Keystone Dental, Inc., a provider of dental health products and solutions. Earlier in his career, Mr. Adams was Chief Financial Officer at a number of other publicly traded companies. Mr. Adams began his career in public accounting at PricewaterhouseCoopers LLP, and is a Certified Public Accountant. Mr. Adams served as a member of the board of directors of ABILITY Network, a private healthcare technology company, from November 2014 to March 2018. Mr. Adams has served as a member of the board of directors of Model N, a public revenue management solutions company, since December 2016. Mr. Adams obtained a B.S. from Murray State University and an M.B.A. from Boston University. We believe Mr. Adams is qualified to serve on our board of directors because of his experience serving as chief financial officer of multiple public companies in the biotechnology and healthcare industries, as well as his investment, accounting and educational background.

Carl L. Gordon, Ph.D., C.F.A. has served as a member of our board since August 2017. Dr. Gordon is a founding Partner and Co-Head of Global Private Equity at OrbiMed Advisors, LLC, an investment firm focused on the healthcare sector, a position he has held since January 1998. Dr. Gordon currently serves on the board of directors of Turning Point Therapeutics, Inc. (Nasdaq: TPTX) and a number of private companies. He previously served on the boards of Alector Inc. (Nasdaq: ALEC), ARMO BioSciences Inc., X4 Pharmaceuticals Inc. (formerly Arsanis Inc.) (Nasdaq: XFOR), Intellia Therapeutics, Inc. (Nasdaq: NTLA) and Selecta BioSciences Inc. (Nasdaq: SELB). Prior to OrbiMed, he was a senior biotechnology analyst at Mehta and Isaly from 1995 to 1997. He was a Fellow at The Rockefeller University from 1993 to 1995. For the last five years (2014 to 2018), Forbes Magazine has named Dr. Gordon one of the top 100 venture capitalists in the world when it placed him on the "Forbes Midas List," Dr. Gordon received a Ph.D. in Molecular Biology from the Massachusetts Institute of Technology and a B.A. from Harvard College. We believe Dr. Gordon is qualified to serve on our board of directors because of his expertise and experience in the biotechnology industry through his role as founding Partner and Co-Head of Global Private Equity at OrbiMed over a 20-year period, in which he has been involved in the evaluation, investment and oversight of several biotechnology companies, his experience as a director of other life sciences companies, as well as his scientific educational background.

Francois Nader, M.D. has served as our chairman since April 2019 and as a member of our board of directors since May 2018. Since February 2015, Dr. Nader has served as founder and managing director of Jesra Advisors, LLC, a consulting services company. He served as the President and Chief Executive Officer of NPS Pharmaceuticals, Inc., or NPS, from 2008 through February 2015 when NPS was acquired by Shire plc. Dr. Nader joined NPS in 2006 and served as Executive Vice President and Chief Operating Officer until 2008. Since December 2014, Dr. Nader has served as a director of Acceleron Pharma and since March 2015 he has served as the Chair of their board of directors and is currently a member of their nominating and governance committee. Since November 2017, he has served as a director of Alexion Pharmaceuticals, Inc., and is currently a member of their audit and finance, and science and innovation committees. Dr. Nader has served as chairman of Talaris Therapeutics (formerly Regenerex) since December 2018 and serves as the chair of the nominating and governance committee and as a member of the audit and compensation committees. Dr. Nader previously served as a director of Clementia Pharmaceuticals, Inc. from 2014 to 2019, Advanced Accelerator Applications SA from 2016 to 2018, Baxalta, Inc. from 2015 to 2016, Trevena, Inc. from 2014 to 2015 and Noven Pharmaceuticals in 2009. Before joining NPS, Dr. Nader was a venture partner at Care Capital, LLC, where he served as Chief Medical Officer of its Clinical Development Capital unit from 2005 to 2006. From 2000 to 2004, he served as Senior Vice President, Integrated Healthcare Markets and Senior Vice President, North America Medical and Regulatory Affairs with Aventis Pharmaceuticals. He also held similar positions at Hoechst Marion Roussel and served as Head of Global Commercial Operations at the Pasteur Vaccines division of Rhone-Poulenc. Dr. Nader

is a past Chair of the board of BioNJ, a trade association representing the biotechnology industry in New Jersey, and a former board member of the New Jersey Chamber of Commerce. Dr. Nader received a French State Doctorate in Medicine from St. Joseph University (Lebanon) and a Physician Executive M.B.A. from the University of Tennessee. We believe Dr. Nader provides significant executive and business skills and scientific and industry knowledge to our board of directors, as well as valuable experience gained from prior and current board service.

Ran Nussbaum has served as a member of our board of directors since March 2018. Mr. Nussbaum is a managing partner and a co-founder of The Pontifax Group, or Pontifax, a group of Israel-based life sciences venture funds focusing on investments in development stage bio-pharmaceutical and med-tech technologies. Prior to founding Pontifax in 2004, he was Chief Executive Officer of Biomedix, Inc. and Spearhead Ltd, and a partner at Israel' s largest business intelligence and strategic consulting firm. He currently serves as a member of the boards of directors of Arqule Inc., Eloxx Pharmaceuticals, Inc., Keros Therapeutics, Inc. (Chairman), UroGen Pharma Ltd. and VBI Vaccines Inc. Previously, Mr. Nussbaum served as a member of the boards of directors of Kite Pharma, Inc., until its acquisition by Gilead Sciences, Inc. in 2017, and cCAM Biotherapeutics Ltd. as well as chairman of the boards of directors of OCON Medical Ltd., NasVax Ltd., Biomedix, Inc. and Spearhead Ltd. We believe that Mr. Nussbaum' s extensive investment experience in the life sciences industry qualifies him to serve on our board of directors.

Peter Thompson, M.D. has served as a member of our board of directors since August 2017. Dr. Thompson currently serves as a Partner for OrbiMed Advisors LLC, an investment firm focused on the healthcare sector, where he has also served as Venture Partner since joining in September 2010. Dr. Thompson is a co-founder of and serves as a member of the board of directors of Corvus Pharmaceuticals, Inc. (Nasdaq: CRVS). He also serves on the board of Alpine Immune Sciences Inc. (Nasdaq: ALPN), Synthorx Inc. (Nasdaq: THOR) and a number of private companies. Dr. Thompson is a board-certified internist and oncologist and has served as Affiliate Professor of Neurosurgery at the University of Washington since 2010. Dr. Thompson co-founded and served as the Chief Executive Officer of Trubion Pharmaceuticals, Inc., a biopharmaceutical company, from 2002 to 2009. Dr. Thompson previously held executive positions at Chiron Corporation and Becton Dickinson, and served on the faculty of the National Cancer Institute following his medical staff fellowship there. Dr. Thompson holds a Sc.B. in Molecular Biology and Mathematics from Brown University and an M.D. from Brown University Medical School. We believe Dr. Thompson' s experience as a director and executive officer of biopharmaceutical companies, his investment firm experience in the healthcare sector, his background in the medical field and his educational background provide him with the qualifications and skills to serve on our board of directors.

Board Composition

Our board of directors currently consists of six members. All of our directors currently serve on the board of directors pursuant to the provisions of a voting agreement between us and several of our stockholders. This agreement will terminate upon the closing of this offering, after which there will be no further contractual obligations regarding the election of our directors.

In accordance with the terms of our amended and restated certificate of incorporation and amended and restated bylaws, which will be effective immediately prior to the closing of this offering, our board of directors will be divided into three classes, Class I, Class II and Class III, with members of each class serving staggered three-year terms. Effective upon the closing of this offering, our board of directors will be divided into the following classes:

Class I, which will consist of Drs. Abeliovich and Gordon, whose terms will expire at our first annual meeting of stockholders to be held after the closing of this offering;

Class II, which will consist of Mr. Nussbaum and Dr. Nader, whose terms will expire at our second annual meeting of stockholders to be held after the closing of this offering; and

Class III, which will consist of Mr. Adams and Dr. Thompson, whose terms will expire at our third annual meeting of stockholders to be held after the closing of this offering.

At each annual meeting of stockholders to be held after the initial classification, the successors to directors whose terms then expire will be elected to serve from the time of election and qualification until the third annual meeting following their election and until their successors are duly elected and qualified. The authorized size of our board of directors is currently nine members, and may be changed only by resolution by a majority of the board of directors. We expect that additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors. This classification of the board of directors may have the effect of delaying or preventing changes in our control or management. Our directors may be removed for cause by the affirmative vote of the holders of at least 66 2/3% of our voting stock.

Director Independence

Applicable Nasdaq rules require a majority of a listed company' s board of directors to be comprised of independent directors within one year of listing. In addition, Nasdaq rules require that, subject to specified exceptions, each member of a listed company' s audit, compensation and nominating and corporate governance committees be independent and that audit committee members also satisfy independence criteria set forth in Rule 10A-3 under the Exchange Act of 1934, as amended, or the Exchange Act. The Nasdaq independence definition includes a series of objective tests, such as that the director is not, and has not been for at least three years, one of our employees, that neither the director nor any of his family members has engaged in various types of business dealings with us and that the director is not associated with the holders of more than 5% of our common stock. In addition, under applicable Nasdaq rules, a director will only qualify as an "independent director" if, in the opinion of the listed company' s board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

Our board of directors has determined that all of our directors, except for Dr. Abeliovich, by virtue of his position as our Chief Executive Officer, and Dr. Gordon, by virtue of his position at OrbiMed, are independent directors, as defined under applicable Nasdaq rules. In making such determination, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his independence, including the beneficial ownership of our capital stock by each non-employee director.

There are no family relationships among any of our directors or executive officers.

Role of the Board in Risk Oversight

One of the key functions of our board of directors is informed oversight of our risk management process. Our board of directors does not have a standing risk management committee, but rather administers this oversight function directly through the board of directors as a whole, as well as through various standing committees of our board of directors that address risks inherent in their respective areas of oversight. In particular, our board of directors is responsible for monitoring and assessing strategic risk exposure and our audit committee has the responsibility to consider and discuss our major financial risk exposures and the steps our management has taken to monitor and control these exposures, including guidelines and policies to govern the process by which risk assessment and management is undertaken. The audit committee also monitors compliance with legal and regulatory requirements.

Board Committees

Our board of directors has established an audit committee, compensation committee and a nominating and corporate governance committee, each of which operate pursuant to a committee charter. Our board of directors

may establish other committees to facilitate the management of our business. The composition and functions of each committee are described below.

Audit Committee

Upon completion of this offering, our audit committee will consist of Mr. Adams, Dr. Nader and Mr. Nussbaum, with Mr. Adams serving as chair of the audit committee. Our board of directors has determined that each of these individuals meets the independence requirements of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, Rule 10A-3 under the Securities Exchange Act of 1934, or the Exchange Act, and the applicable listing standards of Nasdaq. Each member of our audit committee can read and understand fundamental financial statements in accordance with Nasdaq audit committee requirements. In arriving at this determination, the board has examined each audit committee member' s scope of experience and the nature of their prior and/or current employment.

Our board of directors has determined that Mr. Adams qualifies as an audit committee financial expert within the meaning of SEC regulations and meets the financial sophistication requirements of the Nasdaq Listing Rules. In making this determination, our board has considered Mr. Adams' s formal education and previous and current experience in financial and accounting roles. Both our independent registered public accounting firm and management periodically meet privately with our audit committee.

The functions of this committee include, among other things:

- evaluating the performance, independence and qualifications of our independent auditors and determining whether to retain our existing independent auditors or engage new independent auditors;
- reviewing and approving the engagement of our independent auditors to perform audit services and any permissible non-audit services;
- monitoring the rotation of partners of our independent auditors on our engagement team as required by law;
- prior to engagement of any independent auditor, and at least annually thereafter, reviewing relationships that may reasonably be thought to bear on their independence, and assessing and otherwise taking the appropriate action to oversee the independence of our independent auditor;
- reviewing our annual and quarterly financial statements and reports, including the disclosures contained under the caption "Management' s Discussion and Analysis of Financial Condition and Results of Operations," and discussing the statements and reports with our independent auditors and management;
- reviewing with our independent auditors and management significant issues that arise regarding accounting principles and financial statement presentation and matters concerning the scope, adequacy and effectiveness of our financial controls;
- reviewing with management and our auditors any earnings announcements and other public announcements regarding material developments;
- establishing procedures for the receipt, retention and treatment of complaints received by us regarding financial controls, accounting or auditing matters and other matters;
- preparing the report that the U.S. Securities and Exchange Commission, or the SEC, requires in our annual proxy statement;
- reviewing and providing oversight of any related-person transactions in accordance with our related person transaction policy and reviewing and monitoring compliance with legal and regulatory responsibilities, including our code of business conduct and ethics;

reviewing our major financial risk exposures, including the guidelines and policies to govern the process by which risk assessment and risk management is implemented;

reviewing on a periodic basis our investment policy; and

reviewing and evaluating on an annual basis the performance of the audit committee and the audit committee charter.

We believe that the composition and functioning of our audit committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Compensation Committee

Upon completion of this offering, our compensation committee will consist of Dr. Gordon and Messrs. Adams and Nussbaum, with Dr. Gordon serving as chair of the compensation committee. Each of these individuals is a non-employee director, as defined in Rule 16b-3 promulgated under the Exchange Act. Our board of directors has determined that each of these individuals, other than Dr. Gordon by virtue of his position at OrbiMed, is ‘ ‘independent’ ’ as defined under the applicable listing standards of Nasdaq, including the standards specific to members of a compensation committee. The functions of this committee include, among other things:

reviewing, modifying and approving (or if it deems appropriate, making recommendations to the full board of directors regarding) our overall compensation strategy and policies;

making recommendations to the full board of directors regarding the compensation and other terms of employment of our executive officers;

reviewing and making recommendations to the full board of directors regarding performance goals and objectives relevant to the compensation of our executive officers and assessing their performance against these goals and objectives;

reviewing and approving (or if it deems it appropriate, making recommendations to the full board of directors regarding) the equity incentive plans, compensation plans and similar programs advisable for us, as well as modifying, amending or terminating existing plans and programs;

evaluating risks associated with our compensation policies and practices and assessing whether risks arising from our compensation policies and practices for our employees are reasonably likely to have a material adverse effect on us;

reviewing and making recommendations to the full board of directors regarding the type and amount of compensation to be paid or awarded to our non-employee board members;

establishing policies with respect to votes by our stockholders to approve executive compensation to the extent required by Section 14A of the Exchange Act and, if applicable, determining our recommendations regarding the frequency of advisory votes on executive compensation;

reviewing and assessing the independence of compensation consultants, legal counsel and other advisors as required by Section 10C of the Exchange Act;

administering our equity incentive plans;

establishing policies with respect to equity compensation arrangements;

reviewing the competitiveness of our executive compensation programs and evaluating the effectiveness of our compensation policy and strategy in achieving expected benefits to us;

reviewing and making recommendations to the full board of directors regarding the terms of any employment agreements, severance arrangements, change in control protections and any other compensatory arrangements for our executive officers;

reviewing with management and approving our disclosures under the caption "Compensation Discussion and Analysis" in our periodic reports or proxy statements to be filed with the SEC, to the extent such caption is included in any such report or proxy statement;

preparing the report that the SEC requires in our annual proxy statement; and

reviewing and evaluating on an annual basis the performance of the compensation committee and the compensation committee charter.

We believe that the composition and functioning of our compensation committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Nominating and Corporate Governance Committee

Upon completion of this offering, our nominating and corporate governance committee will consist of Drs. Nader and Thompson, with Dr. Nader serving as chair of the nominating and corporate governance committee. Our board of directors has determined that each of these individuals is "independent" as defined under the applicable listing standards of Nasdaq and SEC rules and regulations. The functions of this committee include, among other things:

identifying, reviewing and evaluating candidates to serve on our board of directors;

determining the minimum qualifications for service on our board of directors;

evaluating director performance on the board and applicable committees of the board and determining whether continued service on our board is appropriate;

evaluating, nominating and recommending individuals for membership on our board of directors;

evaluating nominations by stockholders of candidates for election to our board of directors;

considering and assessing the independence of members of our board of directors;

developing a set of corporate governance policies and principles and recommending to our board of directors any changes to such policies and principles;

reviewing and making recommendations to the board of directors with respect to management succession planning;

considering questions of possible conflicts of interest of directors as such questions arise; and

reviewing and evaluating on an annual basis the performance of the nominating and corporate governance committee and the nominating and corporate governance committee charter.

We believe that the composition and functioning of our nominating and corporate governance committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Our board of directors may from time to time establish other committees.

Compensation Committee Interlocks and Insider Participation

None of our directors who serve as a member of our compensation committee is, or has at any time during the past year been, one of our officers or employees. None of our executive officers currently serves, or in the past year has served, as a member of the board of directors or compensation committee of any other entity that has one or more executive officers serving on our board of directors or compensation committee.

Code of Business Conduct and Ethics

Effective upon the closing of this offering, we will adopt a Code of Business Conduct and Ethics, or the Code of Conduct, applicable to all of our employees, executive officers and directors. Following the closing of this offering, the Code of Conduct will be available on our website at *www.prevailtherapeutics.com*. We intend to post on our website all disclosures that are required by law or the listing standards of the Nasdaq Stock Market concerning any amendments to, or waivers from, any provision of the Code of Conduct.

Non-Employee Director Compensation

In the year ended December 31, 2018, we only provided compensation to Francois Nader, M.D., our only non-employee director who was not designated by holders of our convertible preferred stock. Dr. Nader' s compensation for 2018 is set forth in the table below. We did not provide any other compensation to our non-employee directors in 2018; however, we do have a policy of reimbursing all of our non-employee directors for their reasonable out-of-pocket expenses in connection with attending board of directors and committee meetings.

Name	Fees Earned or Paid in Cash	Option Awards(1)	Total
Carl L. Gordon, Ph.D., C.F.A.	$–	$–	$–
Francois Nader, M.D.	19,800	267,771	287,571
Ran Nussbaum	–	–	–
Peter Thompson, M.D.	–	–	–

(1) Amount reported represents the aggregate grant date fair value of stock options granted to Dr. Nader during 2018 under our 2017 Equity Incentive Plan, computed in accordance with ASC Topic 718. The assumptions used in calculating the grant date fair value of this stock option are set forth in the notes to our audited financial statements included elsewhere in this prospectus. This amount does not reflect the actual economic value that may be realized by Dr. Nader.

In May 2018, we granted an option to purchase 133,447 shares of our common stock to Dr. Nader, with an exercise price of $2.81 per share. The shares underlying this option vest over a period of four years, with 25% of the shares vesting on May 4, 2019, and the remainder vesting in 36 equal monthly installments on the first day of each calendar month thereafter, subject to Dr. Nader' s continuous service as of each such vesting date. As of December 31, 2018, all of the shares underlying this outstanding option remained unvested.

Our board of directors adopted a director compensation policy for non-employee directors, to be effective upon the closing of this offering. This compensation policy provides that each of our non-employee directors will receive the following compensation for service on our board of directors:

- an annual cash retainer of $38,750 for all non-employee directors other than the chair of our board of directors;
- an annual cash retainer of $75,000 for the chair of our board of directors (in lieu of the annual cash retainer above);
- an additional annual cash retainer of $7,500, $5,000 and $4,000 for service as a member of the audit committee, compensation committee and the nominating and corporate governance committee, respectively;
- an additional annual cash retainer of $15,000, $10,000 and $8,000 for service as chair of the audit committee, compensation committee and the nominating and corporate governance committee, respectively (in lieu of the committee member retainer above);
- an initial option grant, for new non-employee directors, to purchase 20,000 shares of our common stock, vesting in 36 equal monthly installments; and

an annual option grant to purchase 10,000 shares of our common stock, vesting on the earlier of (1) the one-year anniversary of the date of grant and (2) the date immediately prior to the next following annual stockholder meeting, which annual option grant shall be made at the close of business on the date of each of our annual stockholder meetings.

All vesting of the equity awards granted under this policy is subject to the director' s continuous service as of each applicable vesting date. Notwithstanding the foregoing, in the event of a "change in control" (as defined in the 2019 Plan), all shares subject to any then-outstanding and unvested equity awards granted pursuant to this policy will become fully vested immediately prior to the closing of such change in control.

EXECUTIVE COMPENSATION

Summary Compensation Table for Fiscal Year Ended December 31, 2018

The following table sets forth information regarding compensation earned with respect to the fiscal year ended December 31, 2018 by our principal executive officer and the next two most highly compensated executive officers for the fiscal year ended December 31, 2018, whom we refer to as our named executive officers.

Name and Principal Position	Salary	Bonus	Option Awards(1)	Non-Equity Incentive Plan Compensation(2)	All Other Compensation(3)	Total
Asa Abeliovich, M.D., Ph.D. President, Chief Executive Officer and Director	$390,000	$–	$3,581,687	$ 112,125	$ 1,950	$4,085,762
Jeffrey Sevigny, M.D.(4) Chief Medical Officer	312,500	25,000(5)	1,074,506	89,844	15,180	1,517,030
Franz Hefti, Ph.D.(4) Chief Development Officer	275,000	–	716,338	63,423	9,045	1,063,806

(1) Amounts reported represent the aggregate grant date fair value of stock options granted to our named executive officers during 2018 under our 2017 Equity Incentive Plan, computed in accordance with ASC Topic 718. The assumptions used in calculating the grant date fair value of the stock options are set forth in the notes to our audited financial statements included elsewhere in this prospectus. This amount does not reflect the actual economic value that may be realized by the named executive officer.

(2) Amounts reported represent compensation earned with respect to the year ended December 31, 2018 in accordance with our bonus plan, which is designed to motivate and reward executives for the attainment of company performance goals set by our board of directors. In 2018, these included certain development, regulatory, manufacturing and licensing goals for our product candidates, as well as financing and executive recruiting goals.

(3) Includes (a) for Dr. Abeliovich, $1,950 of 401(k) contributions, (b) for Dr. Sevigny, $750 of medical waiver allowance, $7,680 of 401(k) contributions and $6,750 of travel allowance and (c) for Dr. Hefti, $750 of medical waiver allowance and $8,295 of 401(k) contributions.

(4) Drs. Sevigny and Hefti joined us on March 1, 2018. The salary and non-equity incentive plan compensation amounts set forth in the table represent the pro rata portion of their respective 2018 annual base salary and bonus target as set forth in the section titled "–Employment Arrangements and Severance Agreements with our Named Executive Officers."

(5) Represents the upfront portion of Dr. Sevigny' s one-time sign-on bonus.

Outstanding Equity Awards as of December 31, 2018

The following table sets forth certain information about outstanding equity awards granted to our named executive officers that remain outstanding as of December 31, 2018.

	Option Awards(1)			
Name	Number of Securities Underlying Unexercised Options Exercisable	Number of Securities Underlying Unexercised Options Unexercisable	Option Exercise Price	Option Expiration Date
Asa Abeliovich, M.D., Ph.D.	–	1,334,475 (2)	$ 0.18	3/5/2028
Jeffrey Sevigny, M.D.	–	400,342 (2)	0.18	3/5/2028
Franz Hefti, Ph.D.	–	266,895 (2)	0.18	3/5/2028

(1) All awards were granted under our 2017 Equity Incentive Plan.

(2) 25% of the shares underlying this option vest on March 5, 2019, with the remaining vesting in 36 equal monthly installments thereafter, subject to the named executive officer' s continuous service as of each such vesting date.

Employment Arrangements and Severance Agreements with our Named Executive Officers

Effective upon the closing of this offering, we will enter into an amended and restated employment agreement with each of our named executive officers. The agreements generally provide for at-will employment without any specific term and set forth the named executive officer' s initial base salary and eligibility for employee benefits. The key terms of the employment agreements with our named executive officers, including potential payments upon termination or change in control, are described below. In addition, all of our current named executive officers are eligible to participate in our employee benefit plans, including our medical, dental, vision, disability and life insurance plans, in each case on the same basis as all of our other employees. Each of our named executive officers has executed a form of our standard confidential information, inventions, non-solicitation and non-competition agreement.

Asa Abeliovich, M.D., Ph.D.

Pursuant to his amended and restated employment agreement, Dr. Abeliovich, our Chief Executive Officer, will be entitled to an annual base salary of $488,000 and an annual target bonus equal to 55% of his annual base salary, based on the attainment of certain threshold performance goals, as determined by our board of directors. Additionally, Dr. Abeliovich will be entitled to certain severance benefits pursuant to his agreement, the terms of which are described under "−Potential Payments and Benefits upon Termination or Change in Control" below.

Jeffrey Sevigny, M.D.

Pursuant to his amended and restated employment agreement, Dr. Sevigny, our Chief Medical Officer, will be entitled to an annual base salary of $405,000 and an annual target bonus equal to 35% of his annual base salary, based on the attainment of certain threshold performance goals, as determined by our board of directors. Additionally, Dr. Sevigny will be entitled to certain severance benefits pursuant to his agreement, the terms of which are described under "−Potential Payments and Benefits upon Termination or Change in Control" below.

Franz Hefti, Ph.D.

Pursuant to his amended and restated employment agreement, Dr. Hefti, our Chief Development Officer, will be entitled to an annual base salary of $370,000 and an annual target bonus equal to 35% of his annual base salary, based on the attainment of certain threshold performance goals, as determined by our board of directors. Additionally, Dr. Hefti will be entitled to certain severance benefits pursuant to his agreement, the terms of which are described under "−Potential Payments and Benefits upon Termination or Change in Control" below.

Potential Payments and Benefits upon Termination or Change in Control

Regardless of the manner in which a named executive officer' s employment with us terminates, the named executive officer is entitled to receive amounts earned during his term of service, including salary and accrued unused vacation pay if required by company policy at the time of termination. In addition, each of the named executive officers is eligible for the following payments and benefits upon a qualifying termination of employment or a change in control:

Asa Abeliovich, M.D., Ph.D.

Pursuant to his amended and restated employment agreement, in the event of a qualifying termination, which includes an involuntary termination without "cause" and a "resignation for good reason" (each as defined in his agreement), Dr. Abeliovich will be eligible to receive: (1) 12 months of his monthly base salary, payable in installments on our regular payroll dates; (2) up to 12 months of COBRA reimbursements equal to the company' s portion of the monthly cost of his health insurance premiums at the time of termination; and (3) if termination occurs between January 1 and the target bonus payment date, an amount equal to his target bonus that would have otherwise been earned, in all cases, subject to his execution of a separation agreement and general release of claims in our favor.

In addition, in the event of a qualifying termination within 12 months of a "change in control" (as defined in the 2019 Plan), Dr. Abeliovich will be eligible to receive: (1) 18 months of his monthly base salary plus 1.5 times his annual target bonus; and (2) up to 18 months of COBRA reimbursements equal to the company' s portion of the monthly cost of his health insurance premiums at the time of termination; and (3) accelerated vesting of all outstanding equity, in all cases, subject to his execution of a separation agreement and general release of claims in our favor.

Jeff Sevigny, M.D.

Pursuant to his amended and restated employment agreement, in the event of a qualifying termination, which includes an involuntary termination without "cause" and a "resignation for good reason" (each as defined in his agreement) Dr. Sevigny will be eligible to receive: (1) 12 months of his monthly base salary, payable in installments on our regular payroll dates; and (2) up to 12 months of COBRA reimbursements equal to the company' s portion of the monthly cost of his health insurance premiums at the time of termination; and (3) if termination occurs between January 1 and the target bonus payment date, an amount equal to his target bonus that would have otherwise been earned, in all cases, subject to his execution of a separation agreement and general release of claims in our favor.

In addition, in the event of a qualifying termination within 12 months of a "change in control" (as defined in the 2019 Plan), Dr. Sevigny will be eligible to receive: (1) 12 months of his monthly base salary plus 1.0 times his annual target bonus; (2) up to 12 months of COBRA reimbursements equal to the company' s portion of the monthly cost of his health insurance premiums at the time of termination; and (3) accelerated vesting of all outstanding equity, in all cases, subject to his execution of a separation agreement and general release of claims in our favor.

Franz Hefti

Pursuant to his amended and restated employment agreement, in the event of a qualifying termination, which includes an involuntary termination without "cause" and a "resignation for good reason" (each as defined in his agreement), Dr. Hefti will be eligible to receive: (1) 12 months of his monthly base salary, payable in installments on our regular payroll dates; (2) up to 12 months of COBRA reimbursements equal to the company' s portion of the monthly cost of his health insurance premiums at the time of termination; and (3) if termination occurs between January 1 and the target bonus payment date, an amount equal to his target bonus that would have otherwise been earned, in all cases, subject to his execution of a separation agreement and general release of claims in our favor.

In addition, in the event of a qualifying termination within 12 months of a "change in control" (as defined in the 2019 Plan), Dr. Hefti will be eligible to receive: (1) 12 months of his monthly base salary plus 1.0 times his annual target bonus; (2) up to 12 months of COBRA reimbursements equal to the company' s portion of the monthly cost of his health insurance premiums at the time of termination; and (3) accelerated vesting of all outstanding equity, in all cases, subject to his execution of a separation agreement and general release of claims in our favor.

Equity Incentive Plans

The principal features of our equity incentive plans are summarized below. These summaries are qualified in their entirety by reference to the actual text of the plan, which is filed as exhibits to the registration statement of which this prospectus is a part.

2019 Equity Incentive Plan

Our board of directors adopted and our stockholders approved our 2019 Plan in June 2019. We did not utilize our 2019 Plan until immediately after the date of execution of the underwriting agreement for this offering, at which point no further grants will be made under our 2017 Equity Incentive Plan, or the 2017 Plan, as described under "–2017 Equity Incentive Plan" below. No awards have been granted and no shares of our common stock have been issued under our 2019 Plan.

Stock Awards

Our 2019 Plan provides for the grant of incentive stock options (within the meaning of Section 422 of the Code), nonstatutory stock options, stock appreciation rights, or SARs, restricted stock awards, restricted stock unit awards, performance-based stock awards and other forms of equity compensation, which are collectively referred to as stock awards. Our 2019 Plan also provides for the grant of performance cash awards. Incentive stock options may be granted only to employees. All other awards may be granted to employees, including officers, and to non-employee directors and consultants.

Share Reserve

Initially, the aggregate number of shares of our common stock that may be issued pursuant to stock awards under our 2019 Plan after it becomes effective is 2,773,562, which is the sum of (1) 2,550,000 shares plus (2) the number of shares reserved, and remaining available for issuance, under our 2017 Plan at the time our 2019 Plan becomes effective and (3) the number of shares subject to stock options or other stock awards granted under our 2017 Plan that would have otherwise returned to our 2017 Plan (such as upon the expiration or termination of a stock award prior to vesting). The number of shares of our common stock reserved for issuance under our 2019 Plan will automatically increase on January 1 of each year, beginning on January 1, 2020 (assuming our 2019 Plan becomes effective before such date) and continuing through and including January 1, 2029, by 4% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. The maximum number of shares that may be issued upon the exercise of incentive stock options under our 2019 Plan is 26,000,000 shares.

If a stock award granted under our 2019 Plan expires or otherwise terminates without being exercised in full, or is settled in cash, the shares of our common stock not acquired pursuant to the stock award again will become available for subsequent issuance under our 2019 Plan. In addition, the following types of shares under our 2019 Plan may become available for the grant of new stock awards under our 2019 Plan: (1) shares that are forfeited to or repurchased by us prior to becoming fully vested; (2) shares withheld to satisfy income or employment withholding taxes; or (3) shares used to pay the exercise or purchase price of a stock award. Shares issued under our 2019 Plan may be previously unissued shares or reacquired shares bought by us on the open market.

Administration

Our board of directors, or a duly authorized committee thereof, has the authority to administer our 2019 Plan. Our board of directors may also delegate to one or more of our officers the authority to (1) designate employees (other than other officers) to be recipients of certain stock awards, (2) determine the number of shares of common stock to be subject to such stock awards and (3) specify the other terms and conditions, including the strike price or purchase price and vesting schedule, applicable to such awards. Subject to the terms of our 2019 Plan, our board of directors or the authorized committee, referred to as the plan administrator, determines recipients, dates of grant, the numbers and types of stock awards to be granted and the terms and conditions of the stock awards, including the period of their exercisability and vesting schedule applicable to a stock award. Subject to the limitations set forth below, the plan administrator will also determine the exercise price, strike price or purchase price of awards granted and the types of consideration to be paid for the award.

The plan administrator has the authority to modify outstanding awards under our 2019 Plan. Subject to the terms of our 2019 Plan, the plan administrator has the authority, without stockholder approval, to reduce the exercise, purchase or strike price of any outstanding stock award, cancel any outstanding stock award in exchange for new stock awards, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

Stock Options

Incentive and nonstatutory stock options are evidenced by stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, within the terms and

conditions of our 2019 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under our 2019 Plan vest at the rate specified by the plan administrator.

The plan administrator determines the term of stock options granted under our 2019 Plan, up to a maximum of ten years. Unless the terms of an option holder' s stock option agreement provide otherwise, if an option holder' s service relationship with us, or any of our affiliates, ceases for any reason other than disability, death or cause, the option holder may generally exercise any vested options for a period of three months following the cessation of service. The option term will automatically be extended in the event that exercise of the option following such a termination of service is prohibited by applicable securities laws or our insider trading policy. If an option holder' s service relationship with us or any of our affiliates ceases due to disability or death, or an option holder dies within a certain period following cessation of service, the option holder or a beneficiary may generally exercise any vested options for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, options generally terminate immediately. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and may include (1) cash, check, bank draft or money order, (2) a broker-assisted cashless exercise, (3) the tender of shares of our common stock previously owned by the option holder, (4) a net exercise of the option if it is an nonqualified stock option and (5) other legal consideration approved by the plan administrator.

Unless the plan administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or pursuant to a domestic relations order. An option holder may designate a beneficiary, however, who may exercise the option following the option holder' s death.

Tax Limitations on Incentive Stock Options

The aggregate fair market value, determined at the time of grant, of our common stock with respect to incentive stock options that are exercisable for the first time by an option holder during any calendar year under all of our stock plans may not exceed $100,000. Options or portions thereof that exceed such limit will be treated as nonqualified stock options. No incentive stock option may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant and (2) the term of the incentive stock option does not exceed five years from the date of grant.

Restricted Stock Awards

Restricted stock awards are evidenced by restricted stock award agreements adopted by the plan administrator. Restricted stock awards may be granted in consideration for (1) cash, check, bank draft or money order, (2) services rendered to us or our affiliates or (3) any other form of legal consideration. Common stock acquired under a restricted stock award may, but need not, be subject to a share repurchase option in our favor in accordance with a vesting schedule as determined by the plan administrator. Rights to acquire shares under a restricted stock award may be transferred only upon such terms and conditions as set by the plan administrator. Except as otherwise provided in the applicable award agreement, restricted stock unit awards that have not vested will be forfeited upon the participant' s cessation of continuous service for any reason.

Restricted Stock Unit Awards

Restricted stock unit awards evidenced by restricted stock unit award agreements adopted by the plan administrator. Restricted stock unit awards may be granted in consideration for any form of legal consideration or for no consideration. A restricted stock unit award may be settled by cash, delivery of stock, a combination of cash and stock as deemed appropriate by the plan administrator, or in any other form of consideration set forth in the restricted stock unit award agreement. Additionally, dividend equivalents may be credited in respect of shares

covered by a restricted stock unit award. Rights under a restricted stock units award may be transferred only upon such terms and conditions as set by the plan administrator. Restricted stock unit awards may be subject to vesting as determined by the plan administrator. Except as otherwise provided in the applicable award agreement, restricted stock units that have not vested will be forfeited upon the participant' s cessation of continuous service for any reason.

Stock Appreciation Rights

SARs are evidenced by SAR grant agreements adopted by the plan administrator. The plan administrator determines the strike price for a SAR, which generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Upon the exercise of a SAR, we will pay the participant an amount in cash or stock equal to (1) the excess of the per share fair market value of our common stock on the date of exercise over the strike price, multiplied by (2) the number of shares of common stock with respect to which the SAR is exercised. A SAR granted under our 2019 Plan vests at the rate specified in the SAR agreement as determined by the plan administrator.

The plan administrator determines the term of SARs granted under our 2019 Plan, up to a maximum of ten years. Unless the terms of a participant' s SAR agreement provides otherwise, if a participant' s service relationship with us or any of our affiliates ceases for any reason other than cause, disability or death, the participant may generally exercise any vested SAR for a period of three months following the cessation of service. The SAR term will be further extended in the event that exercise of the SAR following such a termination of service is prohibited by applicable securities laws. If a participant' s service relationship with us, or any of our affiliates, ceases due to disability or death, or a participant dies within a certain period following cessation of service, the participant or a beneficiary may generally exercise any vested SAR for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, SARs generally terminate immediately upon the occurrence of the event giving rise to the termination of the individual for cause. In no event may a SAR be exercised beyond the expiration of its term.

Unless the plan administrator provides otherwise, SARs generally are not transferable except by will, the laws of descent and distribution, or pursuant to a domestic relations order. A SAR holder may designate a beneficiary, however, who may exercise the SAR following the holder' s death.

Performance Awards.

Our 2019 Plan permits the grant of performance-based stock and cash awards. Our compensation committee can structure such awards so that stock or cash will be issued or paid pursuant to such award only after the achievement of certain pre-established performance goals during a designated performance period.

The performance goals that may be selected include one or more of the following: (1) earnings (including earnings per share and net earnings); (2) earnings before interest, taxes and depreciation; (3) earnings before interest, taxes, depreciation and amortization; (4) total stockholder return; (5) return on equity or average stockholder' s equity; (6) return on assets, investment, or capital employed; (7) stock price; (8) margin (including gross margin); (9) income (before or after taxes); (10) operating income; (11) operating income after taxes; (12) pre-tax profit; (13) operating cash flow; (14) sales or revenue targets; (15) increases in revenue or product revenue; (16) expenses and cost reduction goals; (17) improvement in or attainment of working capital levels; (18) economic value added (or an equivalent metric); (19) market share; (20) cash flow; (21) cash flow per share; (22) share price performance; (23) debt reduction; (24) customer satisfaction; (25) stockholders' equity; (26) capital expenditures; (27) debt levels; (28) operating profit or net operating profit; (29) workforce diversity; (30) growth of net income or operating income; (31) billings; (32) pre-clinical development related compound goals; (33) financing; (34) regulatory milestones, including approval of a compound; (35) stockholder liquidity; (36) corporate governance and compliance; (37) product commercialization; (38) intellectual property; (39) personnel matters; (40) progress of internal research or clinical programs; (41) progress of partnered programs; (42) partner satisfaction; (43) budget management; (44) clinical achievements; (45) completing phases of a clinical study (including the treatment phase); (46) announcing or presenting preliminary or final data from clinical studies; in each case, whether on particular timelines or generally; (47) timely completion of clinical

trials; (48) submission of INDs and New Drug Applications and other regulatory achievements; (49) partner or collaborator achievements; (50) internal controls, including those related to the Sarbanes-Oxley Act of 2002; (51) research progress, including the development of programs; (52) investor relations, analysts and communication; (53) manufacturing achievements (including obtaining particular yields from manufacturing runs and other measurable objectives related to process development activities); (54) strategic partnerships or transactions (including in-licensing and out-licensing of intellectual property; (55) establishing relationships with commercial entities with respect to the marketing, distribution and sale of our products (including with group purchasing organizations, distributors and other vendors); (56) supply chain achievements (including establishing relationships with manufacturers or suppliers of active pharmaceutical ingredients and other component materials and manufacturers of our products); (57) co-development, co-marketing, profit sharing, joint venture or other similar arrangements; (58) individual performance goals; (59) corporate development and planning goals; and (60) other measures of performance selected by our board of directors or committee thereof.

The performance goals may be based on company-wide performance or performance of one or more business units, divisions, affiliates, or business segments, and may be either absolute or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects; (3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; (5) to exclude the effects of any items that are unusual in nature or occur infrequently as determined under generally accepted accounting principles; (6) to exclude the dilutive effects of acquisitions or joint ventures; (7) to assume that any business divested by our achieved performance objectives at targeted levels during the balance of a performance period following such divestiture; (8) to exclude the effect of any change in the outstanding shares of our common stock by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger, consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to common stockholders other than regular cash dividends; (9) to exclude the effects of stock based compensation and the award of bonuses under our bonus plans; (10) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to be expensed under generally accepted accounting principles; (11) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles; and (12) to exclude the effect of any other unusual, non-recurring gain or loss or other extraordinary item. In addition, we retain the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of the goals. The performance goals may differ from participant to participant and from award to award.

Other Stock Awards

The plan administrator may grant other awards based in whole or in part by reference to our common stock. The plan administrator will set the number of shares under the stock award and all other terms and conditions of such awards.

Changes to Capital Structure

In the event that there is a specified type of change in our capital structure, such as a stock split or recapitalization, appropriate adjustments will be made to (1) the class and maximum number of shares reserved for issuance under our 2019 Plan, (2) the class and maximum number of shares by which the share reserve may increase automatically each year, (3) the class and maximum number of shares that may be issued upon the exercise of incentive stock options and (4) the class and number of shares and exercise price, strike price or purchase price, if applicable, of all outstanding stock awards.

Corporate Transactions

In the event of certain specified significant corporate transactions, the plan administrator has the discretion to take any of the following actions with respect to stock awards:

- arrange for the assumption, continuation or substitution of a stock award by a surviving or acquiring entity or parent company;
- arrange for the assignment of any reacquisition or repurchase rights held by us to the surviving or acquiring entity or parent company;
- accelerate the vesting of the stock award and provide for its termination prior to the effective time of the corporate transaction;
- arrange for the lapse of any reacquisition or repurchase right held by us;
- cancel or arrange for the cancellation of the stock award in exchange for such cash consideration, if any, as our board of directors may deem appropriate or for no consideration; or
- make a payment equal to the excess of (1) the value of the property the participant would have received upon exercise of the stock award over (2) the exercise price or strike price otherwise payable in connection with the stock award.

Our plan administrator is not obligated to treat all stock awards, even those that are of the same type, in the same manner.

Under our 2019 Plan, a significant corporate transaction is generally the consummation of (1) a sale or other disposition of all or substantially all of our consolidated assets, (2) a sale or other disposition of at least 50% of our outstanding securities, (3) a merger, consolidation or similar transaction following which we are not the surviving corporation or (4) a merger, consolidation or similar transaction following which we are the surviving corporation but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

Change in Control

The plan administrator may provide, in an individual award agreement or in any other written agreement between a participant and us that the stock award will be subject to additional acceleration of vesting and exercisability or settlement in the event of a change in control. Under our 2019 Plan, a change in control is generally (1) the acquisition by a person or entity of more than 50% of our combined voting power other than by merger, consolidation or similar transaction, (2) a consummated merger, consolidation or similar transaction immediately after which our stockholders cease to own more than 50% of the combined voting power of the surviving entity or (3) a consummated sale, lease or exclusive license or other disposition of all or substantially all of our consolidated assets.

Amendment and Termination

Our board of directors has the authority to amend, suspend or terminate our 2019 Plan, provided that such action does not materially impair the existing rights of any participant without such participant' s written consent and provided further that certain types of amendments will require the approval of our stockholders. No incentive stock options may be granted after the tenth anniversary of the date that our board of directors adopts our 2019 Plan.

2019 Employee Stock Purchase Plan

Our board has adopted and our stockholders have approved our 2019 Employee Stock Purchase Plan, or 2019 ESPP.

Share Reserve

The maximum number of shares of our common stock that may be issued under our 2019 ESPP is 330,000 shares. Additionally, the number of shares of our common stock reserved for issuance under our 2019 ESPP will automatically increase on January 1 of each year, beginning on January 1, 2020 and continuing through and including January 1, 2027, by the lesser of (1)1% of the total number of shares of our common stock outstanding on December 31 of the preceding calendar year, (2) 1,500,000 shares of our common stock or (3) such lesser number of shares of common stock as determined by our board of directors. Shares subject to purchase rights granted under our 2019 ESPP that terminate without having been exercised in full will not reduce the number of shares available for issuance under our 2019 ESPP.

Administration

Our board of directors, or a duly authorized committee thereof, will administer our 2019 ESPP. Our board of directors has delegated its authority to administer our 2019 ESPP to our compensation committee under the terms of the compensation committee' s charter.

Limitations

Our employees, including executive officers, and the employees of any of our designated affiliates will be eligible to participate in our 2019 ESPP, provided they may have to satisfy one or more of the following service requirements before participating in our 2019 ESPP, as determined by the administrator: (1) customary employment with us or one of our affiliates for more than 20 hours per week and five or more months per calendar year or (2) continuous employment with us or one of our affiliates for a minimum period of time, not to exceed two years, prior to the first date of an offering. An employee may not be granted rights to purchase stock under our 2019 ESPP (a) if such employee immediately after the grant would own stock possessing 5% or more of the total combined voting power or value of all classes of our common stock or (b) to the extent that such rights would accrue at a rate that exceeds $25,000 worth of our stock for each calendar year that the rights remain outstanding.

Our 2019 ESPP is intended to qualify as an employee stock purchase plan under Section 423 of the Code. The administrator may specify offerings with a duration of not more than 27 months, and may specify one or more shorter purchase periods within each offering. Each offering will have one or more purchase dates on which shares of our common stock will be purchased for the employees who are participating in the offering. The administrator, in its discretion, will determine the terms of offerings under our 2019 ESPP.

A participant may not transfer purchase rights under our 2019 ESPP other than by will, the laws of descent and distribution or as otherwise provided under our 2019 ESPP.

Payroll Deductions

Our 2019 ESPP permits participants to purchase shares of our common stock through payroll deductions up to 15% of their earnings. Unless otherwise determined by the administrator, the purchase price of the shares will be 85% of the lower of the fair market value of our common stock on the first day of an offering or on the date of purchase. Participants may end their participation at any time during an offering and will be paid their accrued contributions that have not yet been used to purchase shares. Participation ends automatically upon termination of employment with us.

Corporate Transactions

In the event of certain specified significant corporate transactions, such as a merger or change in control, a successor corporation may assume, continue or substitute each outstanding purchase right. If the successor

corporation does not assume, continue or substitute for the outstanding purchase rights, the offering in progress will be shortened and a new exercise date will be set. The participants' purchase rights will be exercised on the new exercise date and such purchase rights will terminate immediately thereafter.

2017 Equity Incentive Plan

Our board of directors and our stockholders approved our 2017 Plan in October 2017. As of March 31, 2019, there were 485,862 shares remaining available for the future grant of stock awards under our 2017 Plan. As of March 31, 2019, there were outstanding stock options covering a total of 4,376,161 shares of our common stock that were granted under our 2017 Plan.

Stock Awards. Our 2017 Plan provides for the grant of ISOs within the meaning of Section 422 of the Internal Revenue Code of 1986, as amended, or the Code, to employees, including employees of any parent or subsidiary, and for the grant of NSOs, stock appreciation rights, restricted and unrestricted stock awards, restricted stock unit awards, performance awards and other forms of stock awards to employees, directors and consultants, including employees and consultants of our affiliates. We have only granted stock options under the 2017 Plan.

Authorized Shares. Subject to certain capitalization adjustments, the aggregate number of shares of common stock that may be issued pursuant to stock awards under the 2017 Plan will not exceed 4,826,023 shares.

Shares subject to stock awards granted under our 2017 Plan that expire, become unexercisable or terminate without being exercised in full or that are settled in cash rather than in shares do not reduce the number of shares available for issuance under our 2017 Plan. Additionally, if any shares issued pursuant to a stock award are forfeited back to or repurchased because of the failure to meet a contingency or condition required to vest, then the shares that are forfeited or repurchased will revert to and become available for issuance under the 2019 Plan. This includes shares used to pay the exercise price of a stock award or to satisfy the tax withholding obligations related to a stock award.

Plan Administration. Our board of directors, or a duly authorized committee of our board of directors, will administer our 2017 Plan and is referred to as the "plan administrator" herein. The plan administrator may also delegate to one or more of our officers the authority to (1) designate employees (other than officers) to receive specified stock awards and (2) determine the number of shares subject to such stock awards. Under our 2017 Plan, the plan administrator has the authority to determine award recipients, dates of grant, the numbers and types of stock awards to be granted, the applicable fair market value and the provisions of each stock award, including the period of their exercisability and the vesting schedule applicable to a stock award.

Under the 2017 Plan, the plan administrator also generally has the authority to effect, with the consent of any adversely affected participant, (A) the reduction of the exercise, purchase, or strike price of any outstanding award; (B) the cancellation of any outstanding award and the grant in substitution therefore of other awards, cash, or other consideration; or (C) any other action that is treated as a repricing under generally accepted accounting principles.

Stock Options. ISOs and NSOs are granted under stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for stock options, within the terms and conditions of the 2017 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the 2017 Plan vest at the rate specified in the stock option agreement as determined by the plan administrator.

The plan administrator determines the term of stock options granted under the 2017 Plan, up to a maximum of 10 years. If an optionholder' s service relationship with us or any of our affiliates ceases for any reason other than death or cause, the optionholder may generally exercise any vested options for a period of 90 days following

the cessation of service. This period may be extended in the event that exercise of the option is prohibited by applicable securities laws. If an optionholder' s service relationship with us or any of our affiliates ceases due to death, or an optionholder dies within a certain period following cessation of service, the optionholder or a beneficiary may generally exercise any vested options for a period of 12 months following the date of death. In the event of a termination for cause, options generally terminate upon the termination date. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option include (1) surrender of previously acquired unrestricted shares (2) cash or check, (3) a broker-assisted cashless exercise, or (4) any combination of the foregoing.

Unless the plan administrator provides otherwise, options generally are not transferable except by will or the laws of descent and distribution. Subject to approval of the plan administrator or a duly authorized officer in each case, (i) an option may be transferred pursuant to a domestic relations order, official marital settlement agreement, or other divorce or separation instrument, (ii) an optionholder may designate a beneficiary who may exercise the option following the optionholder' s death, or (iii) an option may be transferred pursuant to a gratuitous transfer of awards (but not an ISO), subject to the terms of the Right of First Refusal and Co-Sale Agreement (as defined in the 2017 Plan) and such other limitations as the plan administrator may impose.

Tax Limitations on ISOs. The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed $100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant, and (2) the term of the ISO does not exceed five years from the date of grant.

Restricted Stock Unit Awards. Restricted stock unit awards are granted under restricted stock unit award agreements adopted by the plan administrator. Restricted stock unit awards may be granted in consideration for any form of legal consideration that may be acceptable to our board of directors and permissible under applicable law. A restricted stock unit awards may be settled by cash, delivery of stock, a combination of cash and stock as deemed appropriate by the plan administrator, or in any other form of consideration set forth in the restricted stock unit award agreement. Additionally, dividend equivalents may be credited in respect of shares covered by a restricted stock unit award. Except as otherwise provided in the applicable award agreement, restricted stock unit awards that have not vested will be forfeited once the participant' s continuous service ends for any reason.

Restricted Stock Awards. Restricted stock awards are granted under restricted stock award agreements adopted by the plan administrator. A restricted stock award may be awarded in consideration for cash, check, bank draft or money order, past or future services to us, or any other form of legal consideration that may be acceptable to our board of directors and permissible under applicable law. The plan administrator determines the terms and conditions of restricted stock awards, including vesting and forfeiture terms. If a participant' s service relationship with us ends for any reason, we may receive any or all of the shares of common stock held by the participant that have not vested as of the date the participant terminates service with us through a forfeiture condition or a repurchase right.

Changes to Capital Structure. In the event there is a specified type of change in our capital structure, such as a stock split, reverse stock split, or recapitalization, appropriate adjustments will be made to (1) the class and maximum number of shares reserved for issuance under the 2017 Plan, (2) the class and maximum number of shares that may be issued on the exercise of ISOs, and (3) the class and number of shares and exercise price, strike price, or purchase price, if applicable, of all outstanding stock awards.

Covered Transactions. Our 2017 Plan provides that in the event of certain specified significant corporate transactions, unless otherwise provided in an award agreement or other written agreement between us and the

award holder, the plan administrator may take one or more of the following actions with respect to such stock awards:

arrange for the assumption, continuation, or substitution of a stock award by a surviving or acquiring corporation;

make a payment equal to the excess, if any, of (A) the fair market value of the stock times the number of shares subject to the award, over (B) any exercise or purchase price payable by the participant in connection with the exercise or purchase;

accelerate the vesting, in whole or in part, of the stock award;

terminate, immediately upon consummation of the covered transaction, each award, other than those assumed or substituted for.

The plan administrator is not obligated to treat all stock awards or portions of stock awards in the same manner and is not obligated to treat all participants in the same manner.

Under the 2017 Plan, a covered transaction is generally the consummation of: (1) a consolidation, merger, or similar transaction or series of related transaction, (2) the sale or transfer of all or substantially all of our assets, or (3) our dissolution or liquidation.

Plan Amendment or Termination. In connection with this offering, our 2017 Plan will be terminated and no further stock awards will be granted thereunder. All outstanding stock awards under the 2017 Plan will continue to be governed by their existing terms.

401(k) Plan

We maintain a 401(k) plan that provides eligible U.S. employees with an opportunity to save for retirement on a tax advantaged basis. Eligible employees are able to defer eligible compensation up to certain Code limits, which are updated annually. We have the ability to make matching and discretionary contributions to the 401(k) plan. In 2018, we contributed 100% of the first 6% contributions. The 401(k) plan is intended to be qualified under Section 401(a) of the Code, with the related trust intended to be tax exempt under Section 501(a) of the Code. As a tax-qualified retirement plan, contributions to the 401(k) plan are deductible by us when made, and contributions and earnings on those amounts are not generally taxable to the employees until withdrawn or distributed from the 401(k) plan.

Limitations on Liability and Indemnification Matters

Upon the closing of this offering, our amended and restated certificate of incorporation will contain provisions that limit the liability of our current and former directors for monetary damages to the fullest extent permitted by Delaware law. Delaware law provides that directors of a corporation will not be personally liable for monetary damages for any breach of fiduciary duties as directors, except liability for:

any breach of the director' s duty of loyalty to the corporation or its stockholders;

any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;

unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the Delaware General Corporation Law, or the DGCL; or

any transaction from which the director derived an improper personal benefit.

This limitation of liability does not apply to liabilities arising under federal securities laws and does not affect the availability of equitable remedies such as injunctive relief or rescission.

Our amended and restated certificate of incorporation to be in effect upon the closing of this offering will provide that we are authorized to indemnify our directors and officers to the fullest extent permitted by Delaware law. Our amended and restated bylaws to be in effect upon the closing of this offering will provide that we are required to indemnify our directors and executive officers to the fullest extent permitted by Delaware law. Our amended and restated bylaws will also provide that, upon satisfaction of certain conditions, we are required to advance expenses incurred by a director or executive officer in advance of the final disposition of any action or proceeding, and permit us to secure insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions in that capacity regardless of whether we would otherwise be permitted to indemnify him or her under the provisions of Delaware law. Our amended and restated bylaws will also provide our board of directors with discretion to indemnify our other officers and employees when determined appropriate by our board of directors. We have entered and expect to continue to enter into agreements to indemnify our directors, executive officers and other employees as determined by the board of directors. With certain exceptions, these agreements provide for indemnification for related expenses, including, among other things, attorneys' fees, judgments, fines and settlement amounts incurred by any of these individuals in any action or proceeding. We believe that these provisions and agreements are necessary to attract and retain qualified persons as directors and officers. We also maintain customary directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and amended and restated bylaws to be in effect upon the closing of this offering may discourage stockholders from bringing a lawsuit against our directors for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and other stockholders. Further, a stockholder' s investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and officers as required by these indemnification provisions. At present, there is no pending litigation or proceeding involving any of our directors, officers or employees for which indemnification is sought, and we are not aware of any threatened litigation that may result in claims for indemnification.

Rule 10b5-1 Sales Plans

Our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or executive officer when entering into the plan, without further direction from them. The director or executive officer may amend a Rule 10b5-1 plan in some circumstances and may terminate a plan at any time. Our directors and executive officers also may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material nonpublic information subject to compliance with the terms of our insider trading policy. Prior to 180 days after the date of this offering, subject to early termination, the sale of any shares under such plan would be subject to the lock-up agreement that the director or executive officer has entered into with the underwriters.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

The following is a summary of transactions since July 6, 2017 (date of inception) to which we have been a participant in which the amount involved exceeded or will exceed $120,000, and in which any of our then directors, executive officers or holders of more than 5% of any class of our capital stock at the time of such transaction, or any members of their immediate family, had or will have a direct or indirect material interest.

Sales of Convertible Preferred Stock

Series B Convertible Preferred Stock Financing. In March 2019, we issued an aggregate of 3,958,046 shares of our Series B convertible preferred stock at $12.63 per share, for aggregate gross proceeds of approximately $50.0 million.

Series A Convertible Preferred Stock Financing. In March and April 2018, we issued an aggregate of 8,997,085 shares of our Series A convertible preferred stock at $8.48 per share, of which 1,330,369 shares were issued at a 10% discount upon conversion of a convertible note, for aggregate gross proceeds of approximately $75.0 million.

Series Seed Convertible Preferred Stock Financing. In August 2017, we issued an aggregate of 6,480,000 shares of our Series Seed convertible preferred stock at $0.62 per share, for aggregate gross proceeds of $4.0 million.

The following table sets forth the aggregate number of shares of our capital stock acquired by our directors, executive officers or holders of more than 5% of any class of our capital stock in the financing transactions described above.

Participant	Shares of Series Seed Convertible Preferred Stock	Shares of Series A Convertible Preferred Stock	Shares of Series B Convertible Preferred Stock	Aggregate Cash Purchase Price
Greater than 5% Stockholders:				
OrbiMed Private Investments VI, LP(1)(2)	6,399,000	3,099,612	1,011,499	$ 31,727,737
Entities affiliated with RA Capital(2)	–	1,356,420	791,613	21,500,036
Entities affiliated with Pontifax(2)	–	943,597	633,286	15,999,970
Entities affiliated with EcoR1 Capital(2)	–	1,356,420	197,902	14,000,006

(1) Includes 1,330,369 shares of Series A convertible preferred stock issued as part of Series A convertible preferred stock financing upon conversion of a note having a principal balance of $10.0 million issued to OrbiMed Private Investments VI, LP in December 2017.
(2) Additional details regarding these entities and their holdings are provided in the section titled "Principal Stockholders."

Sales of Common Stock

In connection with the founding of our company, in August 2017, we issued 2,430,000 shares of our common stock to OrbiMed Private Investments VI, LP and 2,349,000 shares of our common stock to Asa Abeliovich, M.D., Ph.D., our founder, President and Chief Executive Officer, in each case, at a purchase price of $0.0001 per share.

In August 2017, we issued 2,430,000 shares of our common stock to REGENXBIO as consideration for entry into the REGENXBIO GBA1 License. See the section titled "Business–License Agreements–License Agreement with REGENXBIO Inc. for *GBA1*" for additional information.

Employment Arrangements

We have entered into employment agreements with certain of our executive officers. For more information regarding these agreements with our named executive officers, see "Executive Compensation".

Stock Option Grants to Directors and Executive Officers

We have granted stock options to certain of our directors and executive officers. For more information regarding the stock options and stock awards granted to our directors and named executive officers see "Management–Director Compensation" and "Executive Compensation."

Indemnification Agreements

We plan to enter into indemnification agreements with each of our directors and executive officers in connection with this offering. The indemnification agreements and our amended and restated bylaws, each to be in effect upon the closing of this offering, require us to indemnify our directors and executive officers to the fullest extent permitted by Delaware law. For more information regarding these agreements, see "Executive Compensation–Limitations on Liability and Indemnification Matters."

Related Person Transaction Policy

Prior to this offering, we have not had a formal policy regarding approval of transactions with related parties. We have adopted a written related person transaction policy that sets forth our procedures for the identification, review, consideration and approval or ratification of related person transactions. The policy will become effective immediately upon the execution of the underwriting agreement for this offering. For purposes of our policy only, a related person transaction is a transaction, arrangement or relationship, or any series of similar transactions, arrangements or relationships, in which we and any related person are, were or will be participants and in which the amount involved exceeds $120,000. Transactions involving compensation for services provided to us as an employee or director are not covered by this policy. A related person is any executive officer, director or beneficial owner of more than 5% of any class of our voting securities, including any of their immediate family members and any entity owned or controlled by such persons.

Under the policy, if a transaction has been identified as a related person transaction, including any transaction that was not a related person transaction when originally consummated or any transaction that was not initially identified as a related person transaction prior to consummation, our management must present information regarding the related person transaction to our audit committee, or, if audit committee approval would be inappropriate, to another independent body of our board of directors, for review, consideration and approval or ratification. The presentation must include a description of, among other things, the material facts, the interests, direct and indirect, of the related persons, the benefits to us of the transaction and whether the transaction is on terms that are comparable to the terms available to or from, as the case may be, an unrelated third party or to or from employees generally. Under the policy, we will collect information that we deem reasonably necessary from each director, executive officer and, to the extent feasible, significant stockholder to enable us to identify any existing or potential related person transactions and to effectuate the terms of the policy.

In addition, under our Code of Conduct, our employees and directors have an affirmative responsibility to disclose any transaction or relationship that reasonably could be expected to give rise to a conflict of interest.

In considering related person transactions, our audit committee, or other independent body of our board of directors, will take into account the relevant available facts and circumstances including, but not limited to:

- the risks, costs and benefits to us;
- the impact on a director' s independence in the event that the related person is a director, immediate family member of a director or an entity with which a director is affiliated;
- the availability of other sources for comparable services or products; and
- the terms available to or from, as the case may be, unrelated third parties or to or from employees generally.

The policy requires that, in determining whether to approve, ratify or reject a related person transaction, our audit committee, or other independent body of our board of directors, must consider, in light of known circumstances, whether the transaction is in, or is not inconsistent with, our best interests and those of our stockholders, as our audit committee, or other independent body of our board of directors, determines in the good faith exercise of its discretion.

All of the transactions described above were entered into prior to the adoption of the written policy, but all were approved by our board of directors considering similar factors to those described above.

PRINCIPAL STOCKHOLDERS

The following table sets forth information regarding beneficial ownership of our common stock as of March 31, 2019 by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;
- each of our directors;
- each of our named executive officers; and
- all of our current executive officers and directors as a group.

We have determined beneficial ownership in accordance with the rules of the SEC. Under these rules, beneficial ownership includes any shares of common stock as to which the individual or entity has sole or shared voting power or investment power. Applicable percentage ownership is based on 26,644,131 shares of common stock outstanding as of March 31, 2019, after giving effect to the conversion of shares of our convertible preferred stock outstanding as of March 31, 2019 into an aggregate of 19,435,131 shares of our common stock immediately prior to the closing of this offering. In computing the number of shares beneficially owned by an individual or entity and the percentage ownership of that person, shares of common stock subject to options held by such person that are currently exercisable or will become exercisable within 60 days of March 31, 2019 are considered outstanding, although these shares are not considered outstanding for purposes of computing the percentage ownership of any other person.

Unless noted otherwise, the address of all listed stockholders is c/o Prevail Therapeutics Inc., 430 East 29th Street, Suite 940, New York, New York 10016.

Each of the stockholders listed has sole voting and investment power with respect to the shares beneficially owned by the stockholder unless noted otherwise, subject to community property laws where applicable.

NAME AND ADDRESS OF BENEFICIAL OWNER	NUMBER OF SHARES BENEFICIALLY OWNED	PERCENTAGE OF SHARES BENEFICIALLY OWNED	
		BEFORE OFFERING	AFTER OFFERING
Greater than 5% Stockholders:			
OrbiMed Private Investments VI, LP(1)	12,940,111	48.6 %	38.1 %
Asa Abeliovich, M.D., Ph.D(2)	2,738,219	10.1	8.0
REGENXBIO Inc(3)	2,430,000	9.1	7.1
Entities Affiliated with RA Capital(4)	2,148,032	8.1	6.3
Entities Affiliated with Pontifax(5)	1,576,881	5.9	4.6
Entities Affiliated with EcoR1 Capital(6)	1,554,321	5.8	4.6
Directors and Named Executive Officers:			
Jeffrey Sevigny, M.D.(7)	116,764	*	*
Franz Hefti, Ph.D.(7)	77,842	*	*
Timothy Adams	–	–	–
Carl Gordon, Ph.D., C.F.A.(1)	12,940,111	48.6	38.1
Francois Nader, M.D.(8)	57,107	*	*
Ran Nussbaum(5)	1,576,881	5.9	4.6
Peter Thompson, M.D.(1)	–	–	–
All current executive officers and directors as a group (11 persons)(9)	17,853,448	64.6	51.0

* Represents beneficial ownership of less than 1%.

(1) OrbiMed Capital GP VI LLC, or GP VI, is the general partner of OrbiMed Private Investments VI, LP, or OrbiMed VI. OrbiMed Advisors LLC, or OrbiMed Advisors, is the managing member of GP VI. OrbiMed Advisors exercises investment and voting power through a management committee comprised of Carl Gordon, Ph.D., C.F.A., Sven H. Borho and Jonathan T. Silverstein. By virtue of such relationships, GP VI, OrbiMed Advisors and Dr. Gordon may be deemed to have voting and investment power with respect to the shares held by OrbiMed VI and as a result may be deemed to have beneficial ownership of these shares. Dr. Gordon is a member and Peter Thompson, M.D. is an employee at OrbiMed Advisors and both are members of our board of directors. Each of GP VI, OrbiMed Advisors, Messrs. Borho and Silverstein and Drs. Gordon and Thompson disclaims beneficial ownership of the shares held by OrbiMed VI, except to the extent of its or his pecuniary interest therein, if any. OrbiMed Advisors' mailing address is 601 Lexington Avenue, 54th Floor, New York, New York 10022.

(2) Consists of (a) 2,349,000 shares of restricted stock, which vest over four years, with any unvested portion subject to a repurchase right in our favor and (b) 389,219 shares underlying stock options exercisable within 60 days of March 31, 2019. 1,027,697 of the shares of restricted stock were vested within 60 days of March 31, 2019, with the remainder vesting in 27 equal monthly installments on the 4th calendar day of each month thereafter.

(3) REGENXBIO Inc. is a public company. Its address is 9600 Blackwell Road, Suite 210, Rockville, Maryland 20850.

(4) Consists of (a) 1,772,858 shares held by RA Capital Healthcare Fund, L.P., or RA Capital, and (b) 375,174 shares held by Blackwell Partners LLC – Series A, or Blackwell. Dr. Peter Kolchinsky is the managing member of RA Capital Management, LLC, the general partner of RA Capital and the investment advisor of Blackwell. Dr. Kolchinsky and RA Capital Management, LLC may be deemed to beneficially own the shares held by RA Capital and Blackwell. Dr. Kolchinsky and RA Capital Management, LLC disclaim beneficial ownership of all applicable shares except to the extent of their actual pecuniary interest therein. The address of RA Capital is c/o RA Capital Management, LLC, 20 Park Plaza, Suite 1200, Boston, Massachusetts 02116, and the address of Blackwell is 280 S. Magnum Street, Suite 210, Durham, North Carolina 27701.

(5) Consists of (a) 242,141 shares held by Pontifax (Caymen) V L.P., (b) 352,209 shares held by Pontifax (China) V L.P., (c) 906,537 shares held by Pontifax (Israel) V Limited Partnership, and (d) 75,994 shares held by Pontifax Late Stage Fund, L.P., together, the Pontifax Entities. Pontifax 5 G.P. L.P., or Pontifax 5 G.P., is the general partner of each of the Pontifax Entities, and Pontifax Management 4 G.P. (2015) Ltd., or Pontifax Management, is the general partner of Pontifax 5 G.P. Tomer Kariv and Ran Nussbaum, a member of our board of directors, are the Managing Partners of Pontifax Management and, as a result, may be deemed to share voting and investment power with respect to the shares held by each of the Pontifax Entities. The address of each of the Pontifax Entities is c/o The Pontifax Group, 14 Shenkar Street, Herzelia, Israel.

(6) Consists of (a) 313,552 shares held by EcoR1 Capital Fund, L.P., or EcoR1 Capital, and (b) 1,240,769 shares held by EcoR1 Capital Fund Qualified, L.P., or EcoR1 Qualified. Oleg Nodelman owns and controls EcoR1 Capital LLC, the general partner of EcoR1 Capital and EcoR1 Qualified, may be deemed to have voting and investment power with respect to the shares held by EcoR1 Capital and EcoR1 Qualified and, as a result, may be deemed to have beneficial ownership of these shares. The address of EcoR1 Capital and EcoR1 Qualified is 409 Illinois Street, San Francisco, California 94158.

(7) Consists of options held by the executive officer and exercisable within 60 days of March 31, 2019.

(8) Consists of (a) 33,360 options held by Francois Nader and exercisable within 60 days of March 31, 2019 and (b) 23,747 shares held by Jesra Ventures LLC, or Jesra. Dr. Nader, a member of our board of directors, is a member and manager of Jesra. The address of Jesra is 6538 Collins Avenue, Suite 223, Miami Beach, Florida 33141.

(9) In addition to the shares described in footnotes (1), (2), (5), (7) and (8), includes 346,514 shares underlying stock options exercisable within 60 days of March 31, 2019 held by our executive officers.

DESCRIPTION OF CAPITAL STOCK

The following description of our capital stock and provisions of our amended and restated certificate of incorporation and amended and restated bylaws to be effective following the completion of this offering are summaries. You should also refer to the amended and restated certificate of incorporation and the amended and restated bylaws, which are filed as exhibits to the registration statement of which this prospectus is part.

General

Upon the completion of this offering and the filing of our amended and restated certificate of incorporation, our authorized capital stock will consist of 200,000,000 shares of common stock, par value $0.0001 per share, and shares of convertible preferred stock, par value $0.0001 per share.

Common Stock

Outstanding Shares

As of March 31, 2019, we had 26,644,131 shares of common stock outstanding, held of record by 34 stockholders, assuming the automatic conversion of all outstanding shares of our convertible preferred stock into 19,435,131 shares of common stock upon the completion of this offering.

Voting Rights

Each holder of common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders. The affirmative vote of holders of at least 66 2/3% of the voting power of all of the then-outstanding shares of capital stock, voting as a single class, will be required to amend certain provisions of our amended and restated certificate of incorporation, including provisions relating to amending our amended and restated bylaws, the classified board, the size of our board, removal of directors, director liability, vacancies on our board, special meetings, stockholder notices, actions by written consent and exclusive jurisdiction.

Dividends

Subject to preferences that may apply to any outstanding convertible preferred stock, holders of our common stock are entitled to receive ratably any dividends that our board of directors may declare out of funds legally available for that purpose on a non-cumulative basis.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities, subject to the satisfaction of any liquidation preference granted to the holders of any outstanding shares of convertible preferred stock.

Rights and Preferences

Holders of our common stock have no preemptive, conversion or subscription rights, and there are no redemption or sinking fund provisions applicable to our common stock. The rights, preferences and privileges of the holders of our common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of our convertible preferred stock that we may designate and issue in the future.

Preferred Stock

Immediately prior to the closing of this offering, all outstanding shares of convertible preferred stock will convert into shares of our common stock on a one-to-one basis. As of March 31, 2019, we had 19,435,131 shares

of convertible preferred stock outstanding, held of record by 30 stockholders. Immediately after the completion of this offering, our certificate of incorporation will be amended and restated to delete all references to such shares of convertible preferred stock. Under the amended and restated certificate of incorporation, our board of directors will have the authority, without further action by the stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the rights, preferences and privileges of the shares of each wholly unissued series and any qualifications, limitations or restrictions thereon and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding.

Our board of directors may authorize the issuance preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of the common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in our control that may otherwise benefit holders of our common stock and may adversely affect the market price of the common stock and the voting and other rights of the holders of common stock. We have no current plans to issue any shares of preferred stock.

Stock Options

As of March 31, 2019, 4,376,161 shares of common stock were issuable upon the exercise of outstanding stock options, at a weighted-average exercise price of $1.00 per share. For additional information regarding terms of our equity incentive plans, see the section titled "Executive Compensation."

Registration Rights

Upon the completion of this offering, certain holders of shares of our common stock, including certain of those shares of our common stock that will be issued in connection with this offering upon the conversion of our convertible preferred stock will initially be entitled to certain rights with respect to registration of such shares under the Securities Act. These shares are referred to as registrable securities. The holders of these registrable securities possess registration rights pursuant to the terms of our amended and restated investors' rights agreement and are described in additional detail below. The registration of shares of our common stock pursuant to the exercise of the registration rights described below would enable the holders to trade these shares without restriction under the Securities Act when the applicable registration statement is declared effective. As of the completion of this offering, after giving effect to the conversion of all outstanding shares of our convertible preferred stock into shares of our common stock in connection with the completion of the offering, there would have been an aggregate of 24,295,131 registrable securities that were entitled to these demand, piggyback and S-3 registration rights. We will pay the registration expenses, other than underwriting discounts and selling commissions, of the shares registered pursuant to the demand, piggyback and Form S-3 registrations described below.

Generally, in an underwritten offering, the managing underwriter, if any, has the right, subject to specified conditions, to limit the number of shares the holders may include. The demand, piggyback and Form S-3 registration rights described below will expire no later than five years after the completion of this offering, or with respect to any particular holder, at such time that such holder can sell its shares under Rule 144 of the Securities Act during any three-month period.

Demand Registration Rights

The holders of registrable securities will be entitled to certain demand registration rights. At any time beginning 180 days after the completion of this offering, the holders of a majority of the registrable securities then outstanding may, on not more than two occasions, request that we register all or a portion of their shares. Such request for registration must cover shares with an anticipated aggregate offering price of at least $15 million.

Piggyback Registration Rights

The holders of registrable securities will be entitled to certain piggyback registration rights. After this offering, in the event that we propose to register any of our securities under the Securities Act, either for our own account or for the account of other security holders, the holders of registrable securities will be entitled to certain piggyback registration rights allowing the holder to include their shares in such registration, subject to certain marketing and other limitations. As a result, whenever we propose to file a registration statement under the Securities Act, other than with respect to a (1) a registration relating to the sale of securities to our employees pursuant to a stock option, stock purchase, or similar plan; (2) a registration relating to an SEC Rule 145 transaction; (3) a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the registrable securities; (4) a registration in which the only common stock being registered is common stock issuable upon conversion of debt securities that are also being registered, or (5) this offering, the holders of these shares are entitled to notice of the registration and have the right to include their shares in the registration, subject to limitations that the underwriters may impose on the number of shares included in the offering.

S-3 Registration Rights

The holders of registrable securities will be entitled to certain Form S-3 registration rights. A holder of these shares can make a request that we register their shares on Form S-3 if we are qualified to file a registration statement on Form S-3. We will not be required to effect a registration on Form S-3 within 90 days of a registration initiated by us, to effect more than one registration on Form S-3 within any 12-month period.

Anti-Takeover Provisions of Delaware Law and Our Charter Documents

Section 203 of the DGCL

We are subject to Section 203 of the DGCL, which prohibits a Delaware corporation from engaging in any business combination with any interested stockholder for a period of three years after the date that such stockholder became an interested stockholder, with the following exceptions:

before such date, the board of directors of the corporation approved either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder;

upon completion of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction began, excluding for purposes of determining the voting stock outstanding (but not the outstanding voting stock owned by the interested stockholder) those shares owned (1) by persons who are directors and also officers and (2) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or

on or after such date, the business combination is approved by the board of directors and authorized at an annual or special meeting of the stockholders, and not by written consent, by the affirmative vote of at least 66 2/3% of the outstanding voting stock that is not owned by the interested stockholder.

In general, Section 203 defines a “business combination” to include the following:

any merger or consolidation involving the corporation and the interested stockholder;

any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder;

subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;

any transaction involving the corporation that has the effect of increasing the proportionate share of the stock or any class or series of the corporation beneficially owned by the interested stockholder; and

the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits by or through the corporation.

In general, Section 203 defines an "interested stockholder" as an entity or person who, together with the person' s affiliates and associates, beneficially owns, or within three years prior to the time of determination of interested stockholder status did own, 15% or more of the outstanding voting stock of the corporation.

Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws

Among other things, our amended and restated certificate of incorporation and amended and restated bylaws will:

permit our board of directors to issue up to 10,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate, including the right to approve an acquisition or other change in control;

provide that the authorized number of directors may be changed only by resolution of our board of directors;

provide that our board of directors will be classified into three classes of directors;

provide that, subject to the rights of any series of preferred stock to elect directors, directors may only be removed for cause, which removal may be effected, subject to any limitation imposed by law, by the holders of at least a majority of the voting power of all of our then-outstanding shares of the capital stock entitled to vote generally at an election of directors;

provide that all vacancies, including newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;

require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent or electronic transmission;

provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide advance notice in writing, and also specify requirements as to the form and content of a stockholder' s notice;

provide that special meetings of our stockholders may be called only by the chairman of our board of directors, our chief executive officer or president or by our board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors; and

not provide for cumulative voting rights, therefore allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election, if they should so choose.

The amendment of any of these provisions would require approval by the holders of at least 66 2/3% of the voting power of all of our then-outstanding common stock entitled to vote generally in the election of directors, voting together as a single class.

The combination of these provisions will make it more difficult for our existing stockholders to replace our board of directors as well as for another party to obtain control of us by replacing our board of directors. Because our board of directors has the power to retain and discharge our officers, these provisions could also make it more difficult for existing stockholders or another party to effect a change in management. In addition, the authorization of undesignated preferred stock makes it possible for our board of directors to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to change our control.

These provisions are intended to enhance the likelihood of continued stability in the composition of our board of directors and its policies and to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to reduce our vulnerability to hostile takeovers and to discourage certain tactics that may be used in proxy fights. However, such provisions could have the effect of discouraging others from making tender offers for our shares and may have the effect of delaying changes in our control or management. As a consequence, these provisions may also inhibit fluctuations in the market price of our stock that could result from actual or rumored takeover attempts. We believe that the benefits of these provisions, including increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure our company, outweigh the disadvantages of discouraging takeover proposals, because negotiation of takeover proposals could result in an improvement of their terms.

Choice of Forum

Our amended and restated certificate of incorporation will provide that the Court of Chancery of the State of Delaware will be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (1) any derivative action or proceeding brought on our behalf; (2) any action or proceeding asserting a claim of breach of fiduciary duty owed by any current or former director, officer or other employee, to us or our stockholders; (3) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the DGCL, our certificate of incorporation or our bylaws; (4) any action or proceeding to interpret, apply, enforce or determine the validity of our certificate of incorporation or our bylaws; (5) any action or proceeding as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware; (6) or any action asserting a claim against us, or any of our directors, officers or other employees, that is governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court' s having personal jurisdiction over the indispensable parties named as defendants. These provisions of our amended and restated certificate of incorporation will not apply to suits brought to enforce a duty or liability created by the Securities Act, the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction.

Limitation on Liability and Indemnification Matters

See the section titled "Executive Compensation–Limitation on Liability and Indemnification of Directors and Officers."

Listing

We have applied to list our common stock on the Nasdaq Global Market under the trading symbol "PRVL".

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, no public market existed for our common stock. Future sales of our common stock in the public market, or the availability of such shares for sale in the public market, could adversely affect market prices prevailing from time to time. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Nevertheless, sales of our common stock in the public market after such restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price at such time and our ability to raise equity capital in the future.

Based on the number of shares outstanding as of March 31, 2019, upon the closing of this offering and assuming no exercise of the underwriters' option to purchase additional shares, 33,997,131 shares of common stock will be outstanding, assuming no outstanding options are exercised. All of the shares of common stock sold in this offering will be freely tradable without restrictions or further registration under the Securities Act, except for any shares sold to our "affiliates," as that term is defined under Rule 144 under the Securities Act. Substantially all of the remaining 26,644,131 shares of common stock held by existing stockholders are "restricted securities," as that term is defined in Rule 144 under the Securities Act. Restricted securities may be sold in the public market only if registered or if their resale qualifies for exemption from registration described below under Rule 144 promulgated under the Securities Act or another available exemption.

As a result of the lock-up agreements described below and the provisions of Rules 144 and 701 under the Securities Act, the shares of common stock that will be deemed restricted securities after this offering will be available for sale in the public market as follows:

- all of the 7,353,000 shares sold in this offering will be eligible for immediate sale upon the completion of this offering; and
- the remaining outstanding shares that are restricted shares will be eligible for sale in the public market upon expiration of lock-up agreements 180 days after the date of this prospectus, subject in certain circumstances to the volume, manner of sale and other limitations under Rule 144 and Rule 701 under the Securities Act, which are summarized below.

Rule 144

In general, non-affiliate persons who have beneficially owned restricted shares of our common stock for at least six months, and any affiliate of the company who owns either restricted or unrestricted shares of our common stock, are entitled to sell their securities without registration with the SEC under an exemption from registration provided by Rule 144 under the Securities Act.

Non-Affiliates

Any person who is not deemed to have been one of our affiliates at the time of, or at any time during the three months preceding, a sale may sell an unlimited number of restricted securities under Rule 144 if:

- the restricted securities have been held for at least six months, including the holding period of any prior owner other than one of our affiliates (subject to certain exceptions);
- we have been subject to the Exchange Act periodic reporting requirements for at least 90 days before the sale; and
- we are current in our Exchange Act reporting at the time of sale.

Any person who is not deemed to have been an affiliate of ours at the time of, or at any time during the three months preceding, a sale and has held the restricted securities for at least one year, including the holding period of any prior owner other than one of our affiliates, will be entitled to sell an unlimited number of restricted

securities without regard to the length of time we have been subject to Exchange Act periodic reporting or whether we are current in our Exchange Act reporting. Non-affiliate resales are not subject to the manner of sale, volume limitation or notice filing provisions of Rule 144.

Affiliates

Persons seeking to sell restricted securities who are our affiliates at the time of, or any time during the three months preceding, a sale, would be subject to the restrictions described above. They are also subject to additional restrictions, by which such person would be required to comply with the manner of sale and notice provisions of Rule 144 and would be entitled to sell within any three-month period only that number of securities that does not exceed the greater of either of the following:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately 339,971 shares immediately after the completion of this offering based on the number of shares outstanding as of March 31, 2019; or
- the average weekly trading volume of our common stock on the stock exchange on which our shares are listed during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale.

Additionally, persons who are our affiliates at the time of, or any time during the three months preceding, a sale may sell unrestricted securities under the requirements of Rule 144 described above, without regard to the six month holding period of Rule 144, which does not apply to sales of unrestricted securities.

Rule 701

Rule 701 under the Securities Act, as in effect on the date of this prospectus, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions of Rule 144, including the holding period requirement. Most of our employees, executive officers or directors who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares. However, substantially all Rule 701 shares are subject to lock-up agreements as described below and in the section titled “Underwriting” and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

Form S-8 Registration Statements

We intend to file one or more registration statements on Form S-8 under the Securities Act to register all shares of common stock subject to outstanding stock options and common stock issued or issuable under our plan. We expect to file the registration statement covering shares offered pursuant to our stock plans as soon as practicable after the closing of this offering, permitting the resale of such shares by non-affiliates in the public market without restriction under the Securities Act and the sale by affiliates in the public market, subject to compliance with the resale provisions of Rule 144 and expiration or release from the terms of the lock-up agreements described above.

Lock-up Agreements

We, our executive officers and directors and substantially all of the holders of our common stock outstanding on the date of this prospectus have entered into lock-up agreements with the underwriters or otherwise agreed, subject to certain exceptions, that we and they will not, directly or indirectly, offer, sell, contract to sell, pledge, grant any option to purchase, make any short sale, or otherwise dispose of or hedge any of our shares of common stock, any options or warrants to purchase shares of our common stock, or any securities convertible into, or exchangeable for or that represent the right to receive shares of our common stock, without the prior written consent of Morgan Stanley & Co. LLC, BofA Securities, Inc. and Cowen and Company, LLC for a period of 180 days from the date of this prospectus.

Registration Rights

Upon the closing of this offering, the holders of 24,295,131 shares of our common stock, including certain holders of common stock issuable upon the conversion of our preferred stock, or their transferees, will be entitled to specified rights with respect to the registration of their registrable shares under the Securities Act, subject to certain limitations and the expiration, waiver or termination of the lock-up agreements. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act immediately upon effectiveness of the registration. See "Description of Capital Stock–Registration Rights" for additional information.

MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS

The following is a summary of the material U.S. federal income tax consequences to non-U.S. holders (as defined below) of the purchase, ownership and disposition of our common stock issued pursuant to this offering. This discussion is not a complete analysis of all potential U.S. federal income tax consequences relating thereto, does not address the potential application of the alternative minimum tax or the Medicare contribution tax on net investment income, and does not address any estate or gift tax consequences or any tax consequences arising under any state, local or foreign tax laws, or any other U.S. federal tax laws. This discussion is based on the Internal Revenue Code of 1986, as amended, or the Code, U.S. Treasury Regulations promulgated thereunder, judicial decisions, and published rulings and administrative pronouncements of the Internal Revenue Service, or the IRS, all as in effect on the date of this prospectus. These authorities are subject to differing interpretations and may change, possibly retroactively, resulting in U.S. federal income tax consequences different from those discussed below. We have not requested a ruling from the IRS with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS or a court will agree with such statements and conclusions.

This discussion is limited to non-U.S. holders who purchase our common stock pursuant to this offering and who hold our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment). This discussion does not address all of the U.S. federal income tax consequences that may be relevant to an individual holder in light of such holder' s particular circumstances. This discussion also does not consider any specific facts or circumstances that may be relevant to holders subject to special rules under the U.S. federal income tax laws, including:

certain former citizens or long-term residents of the United States;

partnerships or other pass-through entities or arrangements treated as partnerships for U.S. federal income tax purposes (and investors therein);

"controlled foreign corporations";

"passive foreign investment companies";

corporations that accumulate earnings to avoid U.S. federal income tax;

banks, financial institutions, investment funds, insurance companies, brokers, dealers or traders in securities; tax-exempt organizations and governmental organizations;

tax-qualified retirement plans;

"qualified foreign pension funds" as defined in Section 897(l)(2) of the Code and entities all of the interests of which are held by qualified foreign pension funds;

persons subject to special tax accounting rules under Section 451(b) of the Code as a result of any item of gross income with respect to our common stock being taken into account in an applicable financial statement;

persons who have elected to mark securities to market; and

persons holding our common stock as part of a hedging or conversion transaction or straddle, or a constructive sale, or other risk reduction strategy or integrated investment.

If an entity or arrangement that is classified as a partnership for U.S. federal income tax purposes holds our common stock, the U.S. federal income tax treatment of a partner in the partnership will generally depend on the status of the partner and the activities of the partnership. Partnerships holding our common stock and the partners in such partnerships are urged to consult their tax advisors about the particular U.S. federal income tax consequences to them of holding and disposing of our common stock.

THIS DISCUSSION IS FOR INFORMATIONAL PURPOSES ONLY AND IS NOT TAX ADVICE. PROSPECTIVE INVESTORS SHOULD CONSULT THEIR TAX ADVISORS REGARDING THE PARTICULAR U.S. FEDERAL INCOME TAX CONSEQUENCES TO THEM OF ACQUIRING, OWNING AND DISPOSING OF OUR COMMON STOCK, AS WELL AS ANY TAX CONSEQUENCES ARISING UNDER ANY STATE, LOCAL OR FOREIGN TAX LAWS AND ANY OTHER U.S. FEDERAL TAX LAWS.

Definition of Non-U.S. Holder

For purposes of this discussion, a non-U.S. holder is any beneficial owner of our common stock that is not a "U.S. person" or a partnership (including any entity or arrangement treated as a partnership) for U.S. federal income tax purposes. A U.S. person is any person that, for U.S. federal income tax purposes, is or is treated as any of the following:

an individual who is a citizen or resident of the United States;

a corporation (including any entity treated as a corporation for U.S. federal income tax purposes) created or organized under the laws of the United States, any state thereof or the District of Columbia;

an estate, the income of which is subject to U.S. federal income tax regardless of its source; or

a trust (1) whose administration is subject to the primary supervision of a U.S. court and which has one or more U.S. persons who have the authority to control all substantial decisions of the trust or (2) that has a valid election in effect under applicable U.S. Treasury Regulations to be treated as a U.S. person for U.S. federal income tax purposes.

Distributions on Our Common Stock

As described in the section entitled "Dividend Policy," we do not anticipate declaring or paying dividends to holders of our common stock in the foreseeable future. However, if we distribute cash or other property on our common stock, such distributions will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. Amounts not treated as dividends for U.S. federal income tax purposes will constitute a return of capital and will first be applied against and reduce a holder' s tax basis in our common stock, but not below zero. Any excess will be treated as gain realized on the sale or other taxable disposition of our common stock and will be treated as described under "−Gain On Disposition of Our Common Stock" below.

Subject to the discussion below regarding effectively connected income, backup withholding and FATCA (as defined below), dividends paid to a non-U.S. holder of our common stock generally will be subject to U.S. federal withholding tax at a rate of 30% of the gross amount of the dividends or such lower rate specified by an applicable income tax treaty. To receive the benefit of a reduced treaty rate, a non-U.S. holder must furnish us or our withholding agent with a valid IRS Form W-8BEN or IRS Form W-8BEN-E (or applicable successor form) certifying such holder' s qualification for the reduced rate. This certification must be provided to us or our withholding agent before the payment of dividends and must be updated periodically. If the non-U.S. holder holds our common stock through a financial institution or other agent acting on the non-U.S. holder' s behalf, the non-U.S. holder will be required to provide appropriate documentation to the agent, which then will be required to provide certification to us or our withholding agent, either directly or through other intermediaries.

If a non-U.S. holder holds our common stock in connection with the conduct of a trade or business in the United States, and dividends paid on our common stock are effectively connected with such holder' s U.S. trade or business (and are attributable to such holder' s permanent establishment or fixed base in the United States if required by an applicable tax treaty), the non-U.S. holder will be exempt from U.S. federal withholding tax. To claim the exemption, the non-U.S. holder must generally furnish a valid IRS Form W-8ECI (or applicable successor form) to the applicable withholding agent.

However, any such effectively connected dividends paid on our common stock generally will be subject to U.S. federal income tax on a net income basis at the regular U.S. federal income tax rates in the same manner as if such holder were a resident of the United States. A non-U.S. holder that is a foreign corporation also may be subject to an additional branch profits tax equal to 30% (or such lower rate specified by an applicable income tax treaty) of its effectively connected earnings and profits for the taxable year, as adjusted for certain items.

Non-U.S. holders that do not provide the required certification on a timely basis, but that qualify for a reduced treaty rate, may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS. Non-U.S. holders should consult their tax advisors regarding any applicable income tax treaties that may provide for different rules.

Gain on Disposition of Our Common Stock

Subject to the discussion below regarding backup withholding and FATCA, a non-U.S. holder generally will not be subject to U.S. federal income tax on any gain realized on the sale or other taxable disposition of our common stock, unless:

- the gain is effectively connected with the non-U.S. holder' s conduct of a trade or business in the United States and, if required by an applicable income tax treaty, is attributable to a permanent establishment or fixed base maintained by the non-U.S. holder in the United States;
- the non-U.S. holder is a nonresident alien individual present in the United States for 183 days or more during the taxable year of the disposition, and certain other requirements are met; or
- our common stock constitutes a "United States real property interest" by reason of our status as a United States real property holding corporation, or a USRPHC, for U.S. federal income tax purposes.

Whether we are a USRPHC depends on the fair market value of our U.S. real property interests relative to the fair market value of our worldwide real property interests plus our trade and business assets. We believe we are not currently and we do not anticipate becoming a USRPHC for U.S. federal income tax purposes, although there can be no assurance we will not in the future become a USRPHC. Even if we are treated as a USRPHC, gain realized by a non-U.S. holder on a sale or other taxable disposition of our common stock will not be subject to U.S. federal income tax so long as (1) the non-U.S. holder owned, directly, indirectly and constructively, no more than 5% of our common stock at all times within the shorter of (a) the five-year period preceding the disposition and (b) the non-U.S. holder' s holding period and (2) our common stock is regularly traded on an established securities market within the meaning of applicable U.S. Treasury Regulations. There can be no assurance that our common stock will qualify as regularly traded on an established securities market. If any gain on a non-U.S. holder' s disposition of our common stock is subject to U.S. federal income tax because we are a USRPHC and either the ownership of our common stock exceeds 5% or our common stock is not regularly traded on an established securities market, a non-U.S. holder will be taxed on such disposition as described below; in addition a purchaser of our common stock may be required to withhold tax with respect to that obligation.

Gain described in the first bullet point above generally will be subject to U.S. federal income tax on a net income basis at the regular U.S. federal income tax rates in the same manner as if such holder were a resident of the United States. A non-U.S. holder that is a foreign corporation also may be subject to an additional branch profits tax equal to 30% (or such lower rate specified by an applicable income tax treaty) of its effectively connected earnings and profits for the taxable year, as adjusted for certain items. Gain described in the second bullet point above will be subject to U.S. federal income tax at a flat 30% rate (or such lower rate specified by an applicable income tax treaty), but may be offset by certain U.S.-source capital losses (even though the individual is not considered a resident of the United States), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses. Gain described in the third bullet point above will generally be subject to U.S. federal income tax in the same manner as gain that is effectively connected with the conduct of a U.S. trade or business (subject to any provisions under an applicable income tax treaty), except that the branch profits tax generally will not apply.

Non-U.S. holders should consult their tax advisors regarding any applicable income tax treaties that may provide for different rules.

Information Reporting and Backup Withholding

Annual reports are required to be filed with the IRS and may also be provided to each non-U.S. holder indicating the amount of dividends on our common stock paid to such holder and the amount of any tax withheld with respect to those dividends. These information reporting requirements apply even if no withholding was required because the dividends were effectively connected with the holder' s conduct of a U.S. trade or business, or withholding was reduced or eliminated by an applicable income tax treaty. This information also may be made available under a specific treaty or agreement with the tax authorities in the country in which the non-U.S. holder resides or is established. Backup withholding, currently at a 24% rate, generally will not apply to payments to a non-U.S. holder of dividends on or the gross proceeds of a disposition of our common stock provided the non-U.S. holder furnishes the required certification for its non-U.S. status, such as by providing a valid IRS Form W-8BEN, IRS Form W-8BEN-E or IRS Form W-8ECI, or certain other requirements are met. Backup withholding may apply if the payor or the applicable withholding agent has actual knowledge, or reason to know, that the holder is a U.S. person or is not otherwise an exempt recipient. Proceeds of a disposition of our common stock conducted through a non-U.S. office of a non-U.S. broker generally will not be subject to backup withholding or information reporting.

Backup withholding is not an additional tax. Any amount withheld under the backup withholding rules may be allowed as a refund or a credit against the non-U.S. holder' s U.S. federal income tax liability, if any, provided the required information is timely furnished to the IRS.

Withholding on Payments Made to Foreign Accounts

Withholding taxes may be imposed under Sections 1471 to 1474 of the Code (such sections commonly referred to as the Foreign Account Tax Compliance Act, or FATCA) on certain types of payments made to non-U.S. financial institutions and other non-U.S. entities. Specifically, a U.S. federal withholding tax of 30% may apply to dividends and, subject to the discussion of certain proposed U.S. Treasury Regulations below, the gross proceeds of a sale or other taxable disposition of our common stock paid to a foreign financial institution (as specifically defined by applicable rules), including when the foreign financial institution holds our common stock on behalf of a non-U.S. holder, unless such institution enters into an agreement with the U.S. government to withhold on certain payments and to collect and provide to the U.S. tax authorities certain information regarding U.S. account holders of such institution (which may include certain equity holders of such institution, as well as certain account holders that are foreign entities with U.S. owners). Foreign financial institutions located in jurisdictions that have an intergovernmental agreement with the United States governing these withholding and reporting requirements may be subject to different rules. This U.S. federal withholding tax of 30% will also apply to dividends on and, subject to the discussion of certain proposed U.S. Treasury Regulations below, the gross proceeds of a sale or other taxable disposition of our common stock paid to a non-financial foreign entity unless such entity provides the withholding agent with either a certification that it does not have any substantial direct or indirect U.S. owners or provides information regarding direct and indirect U.S. owners of the entity. The withholding tax described above will not apply if the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from these rules. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits of such taxes.

The U.S. Treasury recently released proposed regulations which, if finalized in their present form, would eliminate the federal withholding tax of 30% applicable to the gross proceeds of a sale or other taxable disposition of our common stock. In its preamble to such proposed regulations, the U.S. Treasury stated that taxpayers may generally rely on the proposed regulations until final regulations are issued.

Prospective investors should consult their tax advisors regarding the possible impact of these rules on their investment in our common stock, and the possible impact of these rules on the entities through which they hold our common stock.

UNDERWRITING

Under the terms and subject to the conditions in an underwriting agreement dated the date of this prospectus, the underwriters named below, for whom Morgan Stanley & Co. LLC, BofA Securities, Inc. and Cowen and Company, LLC are acting as representatives, have severally agreed to purchase, and we have agreed to sell to them, severally, the number of shares indicated below:

Underwriter	Number of Shares
Morgan Stanley & Co. LLC	
BofA Securities, Inc.	
Cowen and Company, LLC	
Wedbush Securities Inc.	
Total:	7,353,000

The underwriters and the representatives are collectively referred to as the "underwriters" and the "representatives," respectively. The underwriters are offering the shares of common stock subject to their acceptance of the shares from us and subject to prior sale. The underwriting agreement provides that the obligations of the several underwriters to pay for and accept delivery of the shares of common stock offered by this prospectus are subject to the approval of certain legal matters by their counsel and to certain other conditions. The underwriters are obligated to take and pay for all of the shares of common stock offered by this prospectus if any such shares are taken. However, the underwriters are not required to take or pay for the shares covered by the underwriters' over-allotment option described below.

The underwriters initially propose to offer part of the shares of common stock directly to the public at the offering price listed on the cover page of this prospectus and part to certain dealers at a price that represents a concession not in excess of $ per share under the public offering price. After the initial offering of the shares of common stock, the offering price and other selling terms may from time to time be varied by the representatives.

We have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase up to 1,102,950 additional shares of common stock at the public offering price listed on the cover page of this prospectus, less underwriting discounts and commissions. The underwriters may exercise this option solely for the purpose of covering over-allotments, if any, made in connection with the offering of the shares of common stock offered by this prospectus. To the extent the option is exercised, each underwriter will become obligated, subject to certain conditions, to purchase about the same percentage of the additional shares of common stock as the number listed next to the underwriter' s name in the preceding table bears to the total number of shares of common stock listed next to the names of all underwriters in the preceding table.

The following table shows the per share and total public offering price, underwriting discounts and commissions, and proceeds before expenses to us. These amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase up to an additional 1,102,950 shares of common stock.

		Total	
	Per Share	No Exercise	Full Exercise
Public offering price	$	$	$
Underwriting discounts and commissions to be paid by us	$	$	$
Proceeds, before expenses, to us	$	$	$

The estimated offering expenses payable by us, exclusive of the underwriting discounts and commissions, are approximately $2.9 million. We have agreed to reimburse the underwriters for expenses relating to clearance of this offering with the Financial Industry Regulatory Authority up to $35,000.

The underwriters have informed us that they do not intend sales to discretionary accounts to exceed 5% of the total number of shares of common stock offered by them.

We have applied to have our common stock listed on the Nasdaq Global Market under the trading symbol "PRVL".

We and all directors and officers and the holders of all of our outstanding stock, stock options, and other securities convertible into or exchangeable or exercisable for our common stock have agreed that, without the prior written consent of Morgan Stanley & Co. LLC, BofA Securities, Inc. and Cowen and Company, LLC on behalf of the underwriters, we and they will not, during the period ending on and including the 180th day after the date of this prospectus (the "restricted period"):

- offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of common stock or any securities convertible into or exercisable or exchangeable for shares of common stock;
- file any registration statement with the SEC relating to the offering of any shares of common stock or any securities convertible into or exercisable or exchangeable for common stock; or
- enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the common stock.

whether any such transaction described above is to be settled by delivery of common stock or such other securities, in cash or otherwise subject to certain exceptions, including, but not limited to, pursuant to certain transactions for a maximum of 7% of our total outstanding share capital immediately following this offering. In addition, we and each such person agrees that, without the prior written consent of Morgan Stanley & Co. LLC, BofA Securities, Inc. and Cowen and Company, LLC on behalf of the underwriters, we or such other person will not, during the restricted period, make any demand for, or exercise any right with respect to, the registration of any shares of common stock or any security convertible into or exercisable or exchangeable for common stock.

Morgan Stanley & Co. LLC, BofA Securities, Inc. and Cowen and Company, LLC, in their sole discretion, may release the common stock and other securities subject to the lock-up agreements described above in whole or in part at any time with or without notice.

In order to facilitate the offering of the common stock, the underwriters may engage in transactions that stabilize, maintain or otherwise affect the price of the common stock. Specifically, the underwriters may sell more shares than they are obligated to purchase under the underwriting agreement, creating a short position. A short sale is covered if the short position is no greater than the number of shares available for purchase by the underwriters under the over-allotment option described above. The underwriters can close out a covered short sale by exercising the over-allotment option or purchasing shares in the open market. In determining the source of shares to close out a covered short sale, the underwriters will consider, among other things, the open market price of shares compared to the price available under the over-allotment option. The underwriters may also sell shares in excess of the over-allotment option, creating a naked short position. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in this offering. As an additional means of facilitating this offering, the underwriters may bid for, and purchase, shares of common stock in the open market to stabilize the price of the common stock. These activities may raise or maintain the market price of the common stock above independent market levels or prevent or retard a decline in the market price of the common stock. The underwriters are not required to engage in these activities and may end any of these activities at any time.

We and the underwriters have agreed to indemnify each other against certain liabilities, including liabilities under the Securities Act.

A prospectus in electronic format may be made available on websites maintained by one or more underwriters, or selling group members, if any, participating in this offering. The representatives may agree to allocate a number of shares of common stock to underwriters for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters that may make Internet distributions on the same basis as other allocations.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, investment research, principal investment, hedging. financing and brokerage activities. Certain of the underwriters and their respective affiliates have, from time to time, performed, and may in the future perform, various financial advisory and investment banking services for us, for which they received or will receive customary fees and expenses.

In addition, in the ordinary course of their various business activities, the underwriters and their respective affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers and may at any time hold long and short positions in such securities and instruments. Such investment and securities activities may involve our securities and instruments. The underwriters and their respective affiliates may also make investment recommendations or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long or short positions in such securities and instruments.

Pricing of the Offering

Prior to this offering, there has been no public market for our common stock. The initial public offering price was determined by negotiations between us and the representatives. Among the factors considered in determining the initial public offering price were our future prospects and those of our industry in general, our sales, earnings and certain other financial and operating information in recent periods, and the price-earnings ratios, price-sales ratios, market prices of securities, and certain financial and operating information of companies engaged in activities similar to ours.

Selling Restrictions

Canada

The shares of our common stock may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares of our common stock must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser' s province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser' s province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 (or, in the case of securities issued or guaranteed by the government of a non-Canadian jurisdiction, section 3A.4) of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a Relevant Member State) an offer to the public of any shares of our common stock may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of any shares of our common stock may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

(a) to any legal entity which is a qualified investor as defined in the Prospectus Directive;

(b) to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives for any such offer; or

(c) in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of shares of our common stock shall result in a requirement for the publication by us or any underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an "offer to the public" in relation to any shares of our common stock in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares of our common stock to be offered so as to enable an investor to decide to purchase any shares of our common stock, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression "Prospectus Directive" means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State, and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

United Kingdom

Each underwriter has represented and agreed that:

(a) it has only communicated or caused to be communicated and will only communicate or cause to be communicated an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the Financial Services and Markets Act 2000 (FSMA)) received by it in connection with the issue or sale of the shares of our common stock in circumstances in which Section 21(1) of the FSMA does not apply to us; and

(b) it has complied and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to the shares of our common stock in, from or otherwise involving the United Kingdom.

Hong Kong

The shares of common stock have not been offered or sold and will not be offered or sold in Hong Kong, by means of any document, other than (a) to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong and any rules made under that Ordinance; or (b) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32) of Hong Kong or which do not constitute an offer to the public within the meaning of that Ordinance. No advertisement, invitation, or document relating to the shares of common stock has been or may be issued or has been or may be in the possession of any person for the purposes of issuance, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares of common stock which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" as defined in the Securities and Futures Ordinance and any rules made under that Ordinance.

Japan

No registration pursuant to Article 4, paragraph 1 of the Financial Instruments and Exchange Law of Japan (Law No. 25 of 1948, as amended) (FIEL) has been made or will be made with respect to the solicitation of the application for the acquisition of the shares of common stock.

Accordingly, the shares of common stock have not been, directly or indirectly, offered or sold and will not be, directly or indirectly, offered or sold in Japan or to, or for the benefit of, any resident of Japan (which term as used herein means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or re-sale, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan except pursuant to an exemption from the registration requirements, and otherwise in compliance with, the FIEL and the other applicable laws and regulations of Japan.

For Qualified Institutional Investors (QII)

Please note that the solicitation for newly-issued or secondary securities (each as described in Paragraph 2, Article 4 of the FIEL) in relation to the shares of common stock constitutes either a "QII only private placement" or a "QII only secondary distribution" (each as described in Paragraph 1, Article 23-13 of the FIEL). Disclosure regarding any such solicitation, as is otherwise prescribed in Paragraph 1, Article 4 of the FIEL, has not been made in relation to the shares of common stock. The shares of common stock may only be transferred to QIIs.

For Non-QII Investors

Please note that the solicitation for newly-issued or secondary securities (each as described in Paragraph 2, Article 4 of the FIEL) in relation to the shares of common stock constitutes either a "small number private placement" or a "small number private secondary distribution" (each as is described in Paragraph 4, Article 23-13 of the FIEL). Disclosure regarding any such solicitation, as is otherwise prescribed in Paragraph 1, Article 4 of the FIEL, has not been made in relation to the shares of common stock. The shares of common stock may only be transferred en bloc without subdivision to a single investor.

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares of our common stock may not be circulated or distributed, nor may the shares of our common stock be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore (SFA), (ii) to a relevant person pursuant to Section 275(1), or any person pursuant to Section 275(1A), and in accordance with the conditions specified in Section 275 of the SFA, or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares of our common stock are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

(a) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

(b) a trust (where the trustee is not an accredited investor) the sole purpose of which is to hold investments and each beneficiary of the trust is an individual who is an accredited investor, securities (as defined in Section 239(1) of the SFA) of that corporation or the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has

acquired the shares of our common stock pursuant to an offer made under Section 275 of the SFA except:

(a) to an institutional investor or to a relevant person defined in Section 275(2) of the SFA, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i) (B) of the SFA;

(b) where no consideration is or will be given for the transfer;

(c) where the transfer is by operation of law;

(d) as specified in Section 276(7) of the SFA; or

(e) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore.

LEGAL MATTERS

The validity of the shares of common stock offered hereby will be passed upon for us by Cooley LLP, New York, New York. Certain legal matters will be passed upon for the underwriters by Latham & Watkins LLP, New York, New York. As of the date of this prospectus, partners of Cooley LLP beneficially own an aggregate of 2,444 shares of our convertible preferred stock, which shares of convertible preferred stock will convert into 3,958 shares of our common stock upon the closing of this offering.

EXPERTS

Ernst & Young LLP, independent registered public accounting firm, has audited our financial statements at December 31, 2017 and 2018, and for the period from July 6, 2017 (date of inception) to December 31, 2017 and the year ended December 31, 2018, as set forth in their report. We have included our financial statements in the prospectus and elsewhere in the registration statement in reliance on Ernst & Young LLP' s report, given on their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act, with respect to the shares of common stock being offered by this prospectus. This prospectus, which constitutes part of the registration statement, does not contain all of the information in the registration statement and its exhibits. For further information with respect to our company and the common stock offered by this prospectus, we refer you to the registration statement and its exhibits. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You can read our SEC filings, including the registration statement, over the internet at the SEC' s website at www.sec.gov.

Upon completion of this offering, we will be subject to the information reporting requirements of the Exchange Act, and we will file reports, proxy statements and other information with the SEC. These reports, proxy statements and other information will be available at the website of the SEC referred to above. We also maintain a website at www.prevailtherapeutics.com, at which you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. The information contained in, or that can be accessed through, our website is not part of, and is not incorporated into, this prospectus.

PREVAIL THERAPEUTICS INC.

INDEX TO FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Prevail Therapeutics Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheets of Prevail Therapeutics Inc. (the Company) as of December 31, 2017 and 2018, the related statements of operations, changes in redeemable convertible preferred stock and stockholders' deficit and cash flows for the period from July 6, 2017 to December 31, 2017, and for the year ended December 31, 2018, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2017 and 2018, and the results of its operations and its cash flows for the period from July 6, 2017 to December 31, 2017, and for the year ended December 31, 2018, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company' s management. Our responsibility is to express an opinion on the Company' s financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting, but not for the purpose of expressing an opinion on the effectiveness of the Company' s internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company' s auditor since 2019.

New York, New York

March 26, 2019,
except for the section entitled "Forward Stock Split" in Note 17, as to which the date is June 10, 2019

PREVAIL THERAPEUTICS INC.

BALANCE SHEETS

	December 31,		March 31, 2019 (unaudited)	Pro Forma Stockholder's Deficit March 31, 2019 (unaudited)
	2017	2018		
ASSETS				
CURRENT ASSETS				
Cash and cash equivalents	$12,744,474	$63,014,130	$100,268,188	$213,619,118
Prepaid expenses and other current assets	104,719	562,728	4,382,840	4,382,840
Total current assets	12,849,193	63,576,858	104,651,028	218,201,958
Property and equipment, net	106,067	677,867	897,480	897,480
Operating lease right-of-use assets	1,279,273	8,534,415	8,217,727	8,217,727
Deferred offering costs	–	–	1,336,507	1,336,507
Restricted cash	91,414	91,414	91,414	91,414
TOTAL ASSETS	$14,325,947	$72,880,554	$115,194,156	$228,545,086
LIABILITIES, REDEEMABLE CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' DEFICIT				
CURRENT LIABILITIES:				
Accounts payable	265,408	1,241,417	3,249,984	3,249,984
Accrued expenses	149,226	1,477,213	1,451,771	1,451,771
Operating lease liabilities	285,456	917,490	1,055,457	1,055,457
Derivative liabilities	2,241,770	–	–	–
Convertible note payable, net - related party	7,773,300	–	–	–
Total current liabilities	10,715,160	3,636,120	5,757,212	5,757,212
Long-term operating lease liabilities	1,027,662	7,952,046	7,643,746	7,643,746
TOTAL LIABILITIES	11,742,822	11,588,166	13,400,958	13,400,958
COMMITMENTS AND CONTINGENCIES (Note 15)				
REDEEMABLE CONVERTIBLE PREFERRED STOCK				
Series Seed preferred stock - $0.0001 par value, 6,480,000 shares authorized, issued, and outstanding as of December 31, 2017, December 31, 2018 and March 31, 2019 (unaudited); liquidation preference of $4,000,000 as of December 31, 2017, December 31, 2018 and March 31, 2019 (unaudited)	3,523,541	3,523,541	3,523,541	–
Series A preferred stock - $0.0001 par value, 9,072,000 shares authorized, 0, 8,997,085 and 8,997,085 shares issued and outstanding as of December 31, 2017, December 31, 2018 and March 31, 2019 (unaudited), respectively; liquidation preference of $0, $76,279,227 and $76,279,227 as of December 31, 2017, December 31, 2018 and March 31, 2019 (unaudited), respectively	–	76,186,176	76,186,176	–
Series B preferred stock - $0.0001 par value, 0, 0 and 3,958,046 shares authorized, issued and outstanding as of December 31, 2017, December 31, 2018 and March 31, 2019 (unaudited), respectively; liquidation preference of $0 as of December 31, 2017, and December 31, 2018 and $50,000,011 as of March 31, 2019 (unaudited)	–	–	49,834,124	–
STOCKHOLDERS' DEFICIT				
Common stock - $0.0001 par value, 16,200,000, 28,398,600 and 35,640,000 shares authorized as of December 31, 2017, December 31, 2018 and March 31, 2019 (unaudited), respectively, 7,209,000 shares issued and outstanding as of December 31, 2017, December 31, 2018 and March 31, 2019 (unaudited)	721	721	721	2,664
Additional paid-in capital	885,406	2,496,032	3,107,887	246,000,715
Accumulated deficit	(1,826,543)	(20,914,082)	(30,859,251)	(30,859,251)
Total stockholders' deficit	(940,416)	(18,417,329)	(27,750,643)	(215,144,128)
TOTAL LIABILITIES, REDEEMABLE CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' DEFICIT	$14,325,947	$72,880,554	$115,194,156	$228,545,086

PREVAIL THERAPEUTICS INC.

STATEMENTS OF OPERATIONS

	For the period from July 6, 2017 to December 31, 2017	For the Year Ended December 31, 2018	Three Months Ended March 31, 2018 (unaudited)	Three Months Ended March 31, 2019 (unaudited)
Operating Expenses:				
Research and development	$ 995,350	$ 14,127,013	$ 1,304,421	$ 8,411,317
General and administrative	803,736	4,681,534	641,223	1,885,189
Operating loss	(1,799,086)	(18,808,547)	(1,945,644)	(10,296,506)
Change in fair value of derivative liabilities	–	781,463	781,463	–
Other income	–	(86,868)	–	–
Interest income	(12)	(886,681)	(11)	(351,337)
Interest expense	27,469	471,078	471,078	–
Total other expense (income), net	27,457	278,992	1,252,530	(351,337)
Net loss	$ (1,826,543)	$ (19,087,539)	$ (3,198,174)	$ (9,945,169)
Net loss per share:				
Basic and Diluted	$ (0.45)	$ (3.71)	$ (0.66)	$ (1.73)
Weighted average shares outstanding:				
Basic and Diluted	4,050,000	5,145,469	4,860,000	5,740,874

PREVAIL THERAPEUTICS INC.

STATEMENTS OF CHANGES IN REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT

	Series Seed Preferred Stock		Series A Preferred Stock		Series B Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount			
Balance at July 6, 2017 (inception)	–	$–	–	$–	–	$–	–	$ –	$ –	$–	$–
Issuance of common stock to founder	–	–	–	–	–	–	2,349,000	235	(90)	–	145
Issuance of common stock	–	–	–	–	–	–	2,430,000	243	419,757	–	420,000
Issuance of common stock in exchange for license	–	–	–	–	–	–	2,430,000	243	419,757	–	420,000
Issuance of Series Seed Preferred Stock, net of issuance costs of $56,609	6,480,000	3,523,541	–	–	–	–	–	–	–	–	–
Stock-based compensation	–	–	–	–	–	–	–	–	45,982	–	45,982
Net loss	–	–	–	–	–	–	–	–	–	(1,826,543)	(1,826,543)
Balance at December 31, 2017	6,480,000	$3,523,541	–	$–	–	$–	7,209,000	$ 721	$ 885,406	$(1,826,543)	(940,416)
Issuance of Series A Preferred Stock, net of issuance costs of $93,051	–	–	7,666,716	64,907,028		–	–	–	–	–	–
Series A Preferred Stock shares issued as a result of conversion of convertible note - related party	–	–	1,330,369	11,279,148		–	–	–	–	–	–
Stock-based compensation	–	–	–	–		–	–	–	1,610,626	–	1,610,626
Net loss	–	–	–	–		–	–	–	–	(19,087,539)	(19,087,539)
Balance at December 31, 2018	6,480,000	$3,523,541	8,997,085	$76,186,176		$–	7,209,000	$ 721	$ 2,496,032	$(20,914,082)	$(18,417,329)
Issuance of Series B Preferred Stock, net of issuance costs of $165,887 (unaudited)	–	–	–	–	3,958,046	49,834,124	–	–	–	–	–
Stock-based compensation (unaudited)	–	–	–	–	–	–	–	–	611,855	–	611,855
Net loss (unaudited)	–	–	–	–	–	–	–	–	–	(9,945,169)	(9,945,169)
Balance at March 31, 2019 (unaudited)	6,480,000	$3,523,541	8,997,085	$76,186,176	3,958,046	$49,834,124	7,209,000	$ 721	$ 3,107,887	$(30,859,251)	$(27,750,643)

PREVAIL THERAPEUTICS INC.

STATEMENTS OF CASH FLOWS

	For the period from July 6, 2017 (Date of Inception) to December 31, 2017	For the year ended December 31, 2018	Three months ended March 31, 2018 (unaudited)	Three months ended March 31, 2019 (unaudited)
Cash flows from operating activities				
Net loss	$(1,826,543)	$(19,087,539)	$(3,198,174)	$(9,945,169)
Adjustments to reconcile net loss to net cash used in operating activities				
Depreciation	2,018	54,860	5,084	36,825
Stock-based compensation	45,982	1,610,626	152,368	611,855
Noncash research and development license expense	420,000	–	–	–
Amortization of convertible note discount, issuance costs and other non-cash interest	27,469	471,078	471,078	–
Change in fair value of derivative liabilities	–	781,463	781,463	–
Other	–	11,537	11,537	–
Changes in operating assets and liabilities				
Prepaid expenses and other current assets	(104,719)	(458,009)	(19,291)	(3,820,111)
Operating lease right-of-use asset	70,451	484,605	73,243	316,688
Accounts payable	265,408	976,009	459,699	713,230
Accrued expenses	149,226	1,327,987	105,774	(66,613)
Operating lease liabilities	(36,606)	(183,329)	(69,869)	(170,333)
Net cash used in operating activities	(987,314)	(14,010,712)	(1,227,088)	(12,323,628)
Cash flows from investing activities				
Purchases of property and equipment	(108,085)	(626,660)	(20,957)	(256,438)
Net cash used in investing activities	(108,085)	(626,660)	(20,957)	(256,438)
Cash flows from financing activities				
Proceeds from issuance of common stock	420,145	–	–	–
Payment of issuance costs for convertible note	(12,399)	–	–	–
Proceeds from issuance of convertible note	10,000,000	–	–	–
Payment of issuance costs for preferred stock	(56,609)	(93,051)	(93,051)	(165,887)
Proceeds from issuance of Series Seed preferred stock	3,580,150	–	–	–
Proceeds from issuance of Series A preferred stock	–	65,000,079	57,003,345	–
Proceeds from issuance of Series B preferred stock	–	–	–	50,000,011
Net cash provided by financing activities	13,931,287	64,907,028	56,910,294	49,834,124
Net increase in cash, cash equivalents, and restricted cash	12,835,888	50,269,656	55,662,249	37,254,058
Cash, cash equivalents, and restricted cash at beginning of period	–	12,835,888	12,835,888	63,105,544
Cash, cash equivalents, and restricted cash at end of period(1)	$12,835,888	$63,105,544	$68,498,137	$100,359,602
Supplemental disclosure of non-cash investing and financing activities				
Conversion of convertible note plus accrued interest into 1,330,369 shares of Series A preferred stock	–	11,279,148	11,279,148	–
Right-of-use asset obtained in exchange for operating lease obligation	1,349,724	7,739,747	–	–
Deferred offering costs	–	–	–	1,336,507

(1) Reconciliation of cash, cash equivalents and restricted cash reported within the Balance Sheets:

	December 31, 2017	December 31, 2018	March 31, 2018	March 31, 2019
Cash and cash equivalents	$ 12,744,474	$ 63,014,130	$ 68,406,723	$ 100,268,188
Restricted cash	91,414	91,414	91,414	91,414
Total cash, cash equivalents and restricted cash	$ 12,835,888	$ 63,105,544	$ 68,498,137	$ 100,359,602

PREVAIL THERAPEUTICS INC.

NOTES TO FINANCIAL STATEMENTS

FOR THE PERIOD FROM JULY 6, 2017 (DATE OF INCEPTION) TO DECEMBER 31, 2017 AND FOR THE YEAR ENDED DECEMBER 31, 2018 (INFORMATION AS OF MARCH 31, 2019 AND FOR THE THREE MONTHS ENDED MARCH 31, 2019 AND MARCH 31, 2018 IS UNAUDITED)

1. NATURE OF THE BUSINESS

Prevail Therapeutics Inc. (the "Company") was incorporated in the State of Delaware on July 6, 2017. The Company is a biotechnology company engaged in the research and development of novel biologic gene therapies in an effort to treat Parkinson' s disease and other neurodegenerative diseases. Since beginning operations, the Company has devoted substantially all its efforts to research and development, recruiting management and technical staff, administration, and raising capital.

The Company is subject to a number of risks common to early-stage companies in the biotechnology industry. Principal among these risks are the uncertainties in the development process, development of the same or similar technological innovations by competitors, protection of proprietary technology, dependence on key personnel, compliance with government regulations and approval requirements, and the need to obtain additional financing to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive pre-clinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities.

There can be no assurance that the Company' s research and development will be successfully completed, that adequate protection for the Company' s technology will be obtained, that any products developed will obtain necessary government regulatory approval, or that any approved products will be commercially viable. The Company operates in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services of its employees, consultants and contractors.

Since its inception, the Company has incurred operating losses and has consistently used cash in operations. The Company has no revenue to date, devoting its efforts to research and development. The Company' s activities have been primarily funded by the sale of preferred stock (see Note 7). The Company manages its capital to ensure the company will continue as a going concern while maximizing the return to stakeholders through the optimization of the debt and equity balances.

The Company' s cash and cash equivalents as of December 31, 2018 and March 31, 2019 (unaudited), respectively, were $63,014,130 and $100,268,188. The increase in the cash balance of approximately $50,000,000 was raised through a Series B financing in March 2019. This will enable the Company to fund its operating expenses and capital expenditure requirements through at least one year from the issuance of these financial statements. The Company will need to secure additional funding, from one or more equity or debt financings, collaborations, or other sources, in order to carry out all of its planned research and development and commercialization activities. However, there is no assurance that the Company will be able to obtain additional equity under acceptable terms, if at all. If the Company is unable to obtain additional financing, the lack of liquidity could have a material adverse effect on the Company' s future prospects.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation−The accompanying financial statements have been prepared in conformity with U.S. generally accepted accounting principles ("GAAP"). Any reference in these notes to applicable guidance is

meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

In April 2012, the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act") was enacted. Section 107(b) of the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. The Company has irrevocably elected not to avail itself of this extended transition period, and, as a result, the Company will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

Use of Estimates–The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of expenses during the reporting period. Significant estimates in the financial statements include, but are not limited to stock-based compensation, fair value of common and preferred stock derivative liabilities, operating lease right-of-use assets and liabilities, the recoverability of the Company' s net deferred tax assets and related valuation allowance, and accrued liabilities related to expenses incurred for research and development from external vendors. The Company bases its estimates on historical experience, known trends and other market-specific or other relevant factors that it believes to be reasonable under the circumstances. The Company tracks the progress of its various research and development studies and manufacturing projects to ensure related prepaid expenses and accrued expenses are in line with progress of each. On an ongoing basis, management evaluates its estimates, as there are changes in circumstances, facts and experience. Actual results may differ materially from those estimates or assumptions.

Unaudited Interim Financial Information–The accompanying balance sheet as of March 31, 2019, the related statements of operations and cash flows for the three months ended March 31, 2018 and March 31, 2019 and the statements of redeemable preferred stock and stockholders' deficit for the three months ended March 31, 2018 and March 31, 2019 and related footnote disclosures are unaudited. The accompanying unaudited interim financial information has been prepared from the books and records of the Company in accordance with U.S. GAAP. All adjustments, consisting only of normal recurring adjustments, necessary for a fair presentation of the accompanying balance sheets, statements of operations, changes in redeemable preferred stock and stockholders' deficit and cash flows have been made. The results for the three months ended March 31, 2018 and March 31, 2019 and related footnote disclosures are unaudited and are not necessarily indicative of results to be expected for the year ended December 31, 2019, any other interim periods or any future year or period.

Unaudited Pro Forma Balance Sheet Information–The unaudited pro forma balance sheet information as of March 31, 2019 assumes the conversion of all outstanding shares of redeemable preferred stock into 19,435,131 shares of the Company' s common stock and the resulting reclassification of the carrying value of the convertible preferred stock to stockholders' deficit upon the completion of the Company' s proposed initial public offering (the "IPO). The unaudited pro forma balance sheet information as of March 31, 2019 also assumes that the completion of the IPO had occurred as of March 31, 2019 and excludes shares of common stock issued in the IPO.

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Cash, Cash Equivalents and Restricted Cash–The Company' s cash and cash equivalents include short-term highly liquid investments which are readily convertible into cash. These investments include money market securities and commercial paper with maturities of three months or less when acquired. The Company' s institutional money market accounts permit daily redemption and the fair values of these investments are based upon the quoted prices in active markets provided by the holding financial institutions, which are considered Level 1 inputs in the fair value hierarchy. The Company had no cash equivalents as of December 31, 2017. Restricted cash represents cash on deposit with a financial institution as collateral for in support of a letter of credit outstanding in favor of the Company' s landlord for office space. The restricted cash balance has been excluded from the cash balance and is classified as non-current restricted cash on the balance sheets as the lease expires after March 31, 2020.

Concentration of Credit Risk–The Company maintains cash deposits in excess of government-provided insurance limits. The Company maintains its cash balances with one high quality, accredited financial institution, and accordingly, such funds are not exposed to significant credit risk.

Leases–The Company determines if an arrangement is a lease at inception. Operating leases are included in operating lease right-of-use ("ROU") assets, operating lease liabilities, and long-term operating lease liabilities in the Company' s balance sheets.

ROU assets represent the Company' s right to use an underlying asset for the lease term and lease liabilities represent the Company' s obligation to make lease payments arising from the lease. Operating lease ROU assets and liabilities are recognized at commencement date based on the present value of lease payments over the lease term. As the Company' s leases do not provide a readily determinable implicit rate, the Company' s uses its incremental borrowing rate based on the information available at commencement date in determining the present value of lease payments. The operating lease ROU asset also includes any lease prepayments, offset by lease incentives.

The Company' s facilities operating leases have lease and non-lease components for which the Company has elected to apply the practical expedient and account for each lease component and related non-lease component as one single component. Operating lease cost is recognized on a straight-line basis over the lease term.

Property and Equipment–Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over the estimated useful life of the asset, ranging from 3-7 years as follows:

Fixed Asset Type	Estimated useful life
Laboratory Equipment	7 Years
Computer Equipment	3 Years
Furniture and Fixtures	7 Years

Expenditures for repairs and maintenance of assets are charged to expense as incurred, while major betterments are capitalized. Upon retirement or sale, the cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in the Statements of Operations.

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Impairment of Long-Lived Assets–Long-lived assets, comprised of property and equipment, to be held and used and the right-of-use asset associated with the company' s leased office space are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset over its current fair value. To date, the Company has not recorded any impairment losses on long-lived assets.

Comprehensive Loss–The Company does not have items of other comprehensive loss for the period from July 6, 2017 to December 31, 2017 the year ended December 31, 2018, nor for the three months ended March 31, 2018 (unaudited) and March 31, 2019 (unaudited), respectively, and therefore does not present a statement of comprehensive loss. The Company' s comprehensive loss equals its net loss.

Fair Value Measurements–Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. A framework is used for measuring fair value utilizing a three-tier hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

Level 1–Unadjusted quoted prices in active markets for identical assets or liabilities.

Level 2–Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.

Level 3–Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

Derivative Liabilities–From time to time, the Company may issue certain financial instruments with embedded features which, dependent on their specific contractual terms or other conditions, may be required to be accounted for as separate derivative assets or liabilities. These instruments are required to be measured at fair value. In determining the appropriate fair value, the Company' s uses a discounted cash flow analysis because these instruments are not quoted on an active market. These instruments are then adjusted to reflect fair value at each period end. Any increase or decrease in the fair value is recorded in the statement of operations as change in fair value of derivative liabilities.

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Research and Development Costs, Accrued Research and Development Costs and related Prepaid Expenses–Research and development costs are expensed as incurred. Research and development expenses consist principally of personnel costs, including salaries, stock-based compensation, and benefits of employees, third-party license fees and other operational costs related to the Company' s research and development activities, including allocated facility-related expenses and external costs of outside vendors, and other direct and indirect costs. Non-refundable research and development advance payments are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or services are performed. As of December 31, 2017, December 31, 2018, and March 31, 2019 the Company recorded $13,215, $197,164 and $3,703,572 (unaudited), as prepaid expenses and other current assets, respectively and recorded $12,525, $273,496 and $483,769 (unaudited) of accrued expenses, respectively.

Stock-Based Compensation–The Company measures all stock options and other stock-based awards granted to employees, directors, consultants and other nonemployees based on the fair value on the date of the grant and recognizes compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. The Company recognizes forfeitures at the time forfeitures occur.

The Company classifies stock-based compensation expense in its statement of operations in the same way the payroll costs or service payments are classified for the related stock-based award recipient.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option pricing model. The Company lacks company specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until it has adequate historical data regarding the volatility of its own traded stock price.

Debt Issuance Costs–The Company incurred third-party costs in connection with the Company' s convertible note as described in Note 7. These costs are classified on the balance sheet as a direct deduction from the convertible note. The Company amortizes these costs over the term of its agreement as interest expense in the statement of operations.

Deferred Offering Costs–Deferred public offering costs primarily consist of legal, accounting and filing fees related to the IPO. The deferred offering costs will be offset against the IPO proceeds upon the consummation of the offering. In the event the offering is delayed or aborted, incurred offering costs will be expensed.

Income Taxes–The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the financial statements or the Company' s tax returns. Under this method, deferred tax assets and liabilities are determined on the basis of the differences between the financial statements and tax basis of the assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. The effect of a change in tax rates on deferred tax assets and liabilities is recognized in income in the period that includes the enactment date. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes.

The Company recognizes deferred tax assets to the extent that the Company believes that these assets are more likely than not to be realized. In making such a determination, management considers all available positive

and negative evidence, including future reversals of existing taxable temporary differences, projected future taxable income, tax-planning strategies, and results of recent operations. Valuation allowances are provided, if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. If management determines that the Company would be able to realize its deferred tax assets in the future in excess of their net recorded amount, management would make an adjustment to the deferred tax asset valuation allowance, which would reduce the provision for income taxes.

The Company records uncertain tax positions in accordance with ASC 740 on the basis of a two-step process in which (1) management determines whether it is more likely than not that the tax positions will be sustained on the basis of the technical merits of the position and (2) for those tax positions that meet the more-likely-than-not recognition threshold, management recognizes the largest amount of tax benefit that is more than 50 percent likely to be realized upon ultimate settlement with the related tax authority.

The Company provides reserves for potential payments of tax to various tax authorities related to uncertain tax positions. These reserves are based on a determination of whether and how much of a tax benefit taken by the Company in its tax filings or positions is more likely than not to be realized following resolution of any potential contingencies related to the tax benefit. Potential interest related to the underpayment of income taxes will be classified as a component of interest expense and any related penalties will be classified in operating expenses in the statement of operations. The Company has no significant uncertain tax positions as of December 31, 2017, December 31, 2018, and March 31, 2019 (unaudited).

Tax Credit–In 2018**,** the Company received a New York City Biotechnology Tax Credit. This program allows investors and owners of emerging technology companies focused on biotechnology to claim a refundable tax credit for amounts paid or incurred for certain facilities, operations, and employee training in New York City. Tax credits are recorded when funds are received and are included in other income on the statement of operations.

Net Loss per Common Share–Basic net loss per Share is computed using the "two-class" method which includes the weighted average number of shares of common stock outstanding during the period and other securities that participate in dividends (a participating security). The Company' s convertible preferred stock are participating securities as defined by ASC 260-10, Earnings per Share. During the periods where the Company incurs net losses, the Company allocates no loss to participating securities because these securities have no contractual obligation to share in the losses of the Company. Under the two-class method, basic net loss per share applicable to common stockholders is computed by dividing the net loss applicable to common stockholders by the weighted average number of common shares outstanding for the period. Diluted net loss per share is computed similar to basic net loss per share except that the denominator is increased to include the number of additional shares for the potential dilutive effects of convertible debt, convertible preferred stock and stock options outstanding during the period calculated in accordance with the treasury stock method, or the two-class method, whichever is more dilutive. The Company allocates net earnings on a pari passu (equal) basis to both common and preferred stockholders. Net losses are not allocated to preferred stockholders as they do not have an obligation to share in the Company' s net losses. For all periods presented, basic and diluted net loss per share are the same, as any additional share equivalents would be anti-dilutive (Note 14).

Segment Reporting–Operating segments are identified as components of an enterprise for which separate discrete financial information is available for evaluation by the Company' s Chief Operating Decision Maker in making decisions regarding resource allocation and assessing performance. To date, the Company has viewed its operations and manages its business as one operating segment.

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Recently Issued Accounting Pronouncements (Adopted)–In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update No. 2014-09 ("ASU 2014-09"), which replaces existing revenue recognition guidance. For nonpublic entities, the new guidance is effective for annual reporting periods beginning after December 15, 2018, and interim periods within annual periods beginning after December 15, 2019. Among other things, the updated guidance requires companies to recognize revenue in a way that depicts the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. Although the Company does not currently have revenue generating activities, the Company has elected to early adopt this guidance on July 6, 2017.

In November 2015, the FASB issued ASU 2015-17, which removes the requirement to split deferred income taxes between current and non-current. Instead, the new accounting guidance requires all deferred income taxes to be reported as non-current. This standard is effective for financial statements issued for annual periods beginning after December 15, 2017, and interim periods within annual periods beginning after December 15, 2018, with early adoption permitted. The Company has elected to adopt ASU 2015-17 as of its inception date of July 6, 2017. The adoption of this guidance did not have a material impact on the Company' s financial statements.

In February 2016, the FASB issued ASU 2016-02, *Leases*. This guidance requires an entity to recognize lease liabilities and a right-of-use asset for most leases on the balance sheet and to disclose key information about the entity' s leasing arrangements. ASU 2016-02 is effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020, with earlier adoption permitted. ASU 2016-02 states that the guidance must be adopted using a modified retrospective approach for all leases existing at, or entered into after the date of initial adoption, with an option to elect to use certain transition relief. The Company has elected to adopt ASU 2016-02 as of its inception date of July 6, 2017. Therefore, all leases are presented under the new guidance. Refer to Note 10.

In March 2016, the FASB issued ASU 2016-09, *Compensation-Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting,* which is intended to simplify several aspects of the accounting for share-based payment transactions, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. ASU 2016-09 is effective for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years beginning after December 15, 2018, with early adoption permitted. The Company has elected to adopt ASU 2016-09 as of its inception date of July 6, 2017. Therefore, all employee stock-compensation were accounted for under the new guidance. Refer to Note 11.

In November 2016, FASB issued ASU 2016-18, *Statements of Cash Flows (Topic 230): Restricted Cash*. The Amendments in this ASU require that a statement of cash flows explain the change during the period in the total of cash, cash equivalents, and amounts generally described as restricted cash or restricted cash equivalents. Therefore, amounts generally described as restricted cash and restricted cash equivalents should be included with cash and cash equivalents when reconciling the beginning-of-period and end-of period total amounts shown on the statement of cash flows. For private companies, the amendments are effective for fiscal years beginning after December 15, 2018, and interim periods within fiscal years beginning after December 15, 2019. The Company has elected to early adopt ASU 2016-18 as of its inception date of July 6, 2017. Refer to the Statement of Cash Flows.

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In January 2017, the FASB issued ASU No. 2017-01, *Business Combinations (Topic 805): Clarifying the Definition of a Business.* The amendments to the guidance are intended to help companies evaluate whether transactions should be accounted for as acquisitions (or disposals) of assets or businesses. When substantially all of the fair value of gross assets acquired is concentrated in a single asset (or a group of similar assets), the assets acquired would not represent a business. This amendment introduces an initial required screening that, if met, eliminates the need for further assessment. To be considered a business, an acquisition would have to include an input and a substantive process that together significantly contribute to the ability to create outputs. To be a business without outputs, there will need to be an organized workforce. These amendments are effective for annual periods beginning after December 15, 2017, including interim periods within those periods. The changes to the definition of a business may result in more acquisitions being accounted for as asset acquisitions. The Company has elected to early adopt ASU 2017-01 as of its inception date of July 6, 2017. The adoption of this guidance did not have an impact on the Company' s financial statements.

In June 2018, the FASB issued ASU 2018-07, *Compensation-Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting*, as part of an initiative to reduce the cost and complexity in financial reporting and improve the usefulness of the information provided related to share-based payment transaction for acquiring goods and services from nonemployees. Under ASU 2018-07, the guidance under ASC 505-50 is superseded as ASC 718 is expanded to include awards to nonemployees. In general, companies will no longer be required to remeasure (i.e., mark-to-market) the fair value of awards granted to nonemployees at each reporting date until the awards vest (the date the vesting condition is achieved). Instead, grants to nonemployees will be valued and accounted for much in the same way as awards to employees, including the ability to use the simplified method/practical expedient when determining the expected term assumption. For non-public entities, ASU 2018-07 will be effective for annual periods beginning after December 15, 2019 and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted, but no earlier than the Company' s adoption date of ASC 606. The amendments should be applied on a modified retrospective basis upon adoption with remeasurement of equity awards to nonemployees that have not yet vested through a cumulative-effect adjustment (i.e., transition adjustment) to retained earnings as of the beginning of the fiscal year of adoption. As the Company has early adopted ASC 606 as of its inception date, the Company has also elected to adopt ASU 2018-07 as of its inception date of July 6, 2017. Therefore, all nonemployee stock-compensation will be accounted for under the new guidance. Refer to Note 11.

In November 2018, the U.S. Securities and Exchange Commission (SEC)' s release, Disclosure Update and Simplification, became effective. The included amendments are intended to simplify and update the SEC' s disclosure requirements and eliminate duplicative disclosures between the SEC rules and GAAP. The amendments included new interim financial statement disclosures to reconcile the beginning balance to the ending balance in stockholders' equity for each period for which an income statement is required to filed.

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Accordingly, the reconciliation of the beginning balance to the ending balance in redeemable convertible preferred stock and stockholders' deficit for the three months ended March 31, 2018 is as follows (unaudited):

	Series Seed Preferred Stock		Series A Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount			
Balance at December 31, 2017	6,480,000	$3,523,541	–	$–	7,209,000	$ 721	$ 885,406	$(1,826,543)	$(940,416)
Issuance of Series A Preferred Stock, net of issuance costs of $93,051	–	–	6,723,120	56,910,294	–	–	–	–	–
Series A Preferred Stock shares issued as a result of conversation of convertible note-related party	–	–	1,330,369	11,279,148	–	–	–	–	–
Stock-based compensation	–	–	–	–	–	–	152,368	–	152,368
Net loss	–	–	–	–	–	–	–	(3,198,174)	(3,198,174)
Balance at March 31, 2018	6,480,000	$3,523,541	8,053,489	$68,189,442	7,209,000	$ 721	$ 1,037,774	$(5,024,717)	$(3,986,222)

In July 2018, the FASB issued ASU 2018-09, *Codification Improvements* ("ASU 2018-09"). The Company adopted this amendment on January 1, 2019. This amendment makes changes to a variety of topics to clarify, correct errors in, or make minor improvements to the Accounting Standards Codification. The adoption of ASU 2018-09 did not have a material impact on its financial statements.

Recently Issued Accounting Pronouncements (Not Yet Adopted)–In August 2018, the FASB issued ASC 2018-13 *Fair Value Measurement - Disclosure Framework-Changes to the Disclosure Requirement for Fair Value Measurement* ("ASU 2018-13"). The amendments in ASU 2018-13 modify the disclosure requirements on fair value measurements in ASC 820, Fair Value Measurement, based on the concepts in the FASB Concepts Statement, including the consideration of costs and benefits. The amendments under ASU 2018-13 are effective for interim and annual fiscal periods beginning after December 15, 2019, with early adoption permitted. The Company is currently evaluating the effects the adoption of ASU 2018-13 will have on its financial statements and related disclosures.

In August 2018, the FASB issued ASU No. 2018-15, *Intangible-Goodwill and Other Internal-Use Software (Subtopic 350-40)* ("ASU 2018-15"). ASU 2018-15 updates guidance regarding accounting for implementation costs associated with a cloud computing arrangement that is a service contract. The amendments under ASU 2018-15 are effective for interim and annual fiscal periods beginning after December 15, 2019, with early adoption permitted. The Company does not expect the adoption of ASU 2018-15 to have a material impact on its financial statements.

3. LICENSE AGREEMENTS

REGENXBIO Inc.

In August 2017, the Company entered into a License Agreement (the "REGENXBIO GBA1 License") with REGENXBIO Inc. ("REGENXBIO"). Under the terms of the REGENXBIO GBA1 License, REGENXBIO granted the Company an exclusive, worldwide license under certain patents and patent applications to make, have

made, use, import, sell and offer for sale products for the treatment of disease, including but not limited to Parkinson' s disease and Gaucher disease, whether or not caused by mutations in the gene that produces the GBA1 enzyme in humans by in vivo gene therapy using AAV9 delivering the gene (or any portion thereof) encoding for GBA1.

As consideration for the licensed rights under the REGENXBIO GBA1 License, the Company issued 2,430,000 shares of its common stock with an aggregate fair value of $420,000 in a concurrent private placement to REGENXBIO. The Company is obligated, pursuant to the REGENXBIO GBA1 License, to pay REGENXBIO: (1) an annual maintenance fee; (2) mid-to high-single digit royalty percentages on net sales of licensed products, subject to reduction in specified circumstances; and (3) mid-teen to low-twenties royalty percentages of any sublicense fees the Company receives from sublicensees for the licensed intellectual property rights. The initial license fee paid in connection with the REGENXBIO GBA1 License, including the fair value of the common stock issued to REGENXBIO, was recognized as Research and development expense in the period ended December 31, 2017. The initial annual maintenance fee was recognized as research and development expense for the year ended December 31, 2018.

The REGENXBIO GBA1 License will expire on a country-by-country, licensed product-by-licensed product basis upon the later of (1) the expiration, lapse, abandonment or invalidation of the last valid claim of the licensed intellectual property and (2) seven years from the first commercial sale of each licensed product. The Company has the right to terminate the REGENXBIO GBA1 License upon a specified period of prior written notice. REGENXBIO may terminate the REGENXBIO GBA1 License immediately if the Company becomes insolvent, if the Company is late by a specified number of days in paying money due under the REGENXBIO GBA1 License, or if the Company or its affiliates commence any action against REGENXBIO or its licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate the REGENXBIO GBA1 License for material breach if such breach is not cured within a specified number of days.

In May 2018, the Company entered into a license agreement (the "REGENXBIO Option Genes License") with REGENXBIO pursuant to which REGENXBIO granted the Company three distinct exclusive options for specified genes (the "Option Genes") exercisable at the Company' s sole discretion through May 10, 2019. Each option represents the right to obtain an exclusive, worldwide license under certain patents and patent applications to make, have made, use, import, sell and offer for sale products for the treatment or prevention of disease, including but not limited to Parkinson' s disease, whether or not caused by mutations in any Option Gene that is the subject of the applicable license, in humans by in vivo gene therapy using AAV9 delivering the applicable licensed Option Gene and/or RNA interference or antisense modalities that target the applicable licensed Option Gene. The Company also received a non-exclusive, royalty-free, worldwide research license to perform research and development activities for each Option Gene solely for purposes of evaluating whether to exercise the applicable option.

Under the terms of the REGENXBIO Option Genes License, the Company paid REGENXBIO an initial fee of $600,000. In connection with the exercise of each option, the Company is required to pay REGENXBIO: (1) an additional up-front fee of $600,000; (2) an annual maintenance fee; (3) mid- to high-single digit royalty percentages on net sales of the licensed product, subject to reduction in specified circumstances; and (4) mid-teen to low-twenties royalty percentages of any sublicense fees the Company receives from sublicensees for the licensed intellectual property rights. If a licensed product includes the GBA1 gene and otherwise would be

subject to royalties under the REGENXBIO GBA1 License, then royalties for that licensed product will only be due under the REGENXBIO Option Genes License. The initial fee paid for the REGENXBIO Option Genes License, was recognized as Research and development expense for the year ended December 31, 2018.

As of December 31, 2018 and March 31, 2019, the Company had not exercised any option under the REGENXBIO Option Genes License.

The REGENXBIO Option Genes License will expire on a country-by-country, licensed product-by-licensed product basis upon the later of (1) the expiration, lapse, abandonment or invalidation of the last valid claim of the licensed intellectual property and (2) seven years from the first commercial sale of each licensed product. The Company has the right to terminate the REGENXBIO Option Genes License upon a specified period of prior written notice. REGENXBIO may terminate the REGENXBIO Option Genes License immediately if the Company becomes insolvent, if the Company is late by a specified number of days in paying money due under the REGENXBIO Option Genes License, or if the Company or its affiliates commence any action against REGENXBIO or its licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate the REGENXBIO Option Genes License for material breach if such breach is not cured within a specified number of days.

In April 2019, the company exercised all of the options under the REGENEXBIO Option Genes License. Refer to note 17.

4. FAIR VALUE OF FINANCIAL INSTRUMENTS

The Company' s financial instruments consist of cash equivalents, the derivative liabilities related to certain redemption rights pursuant to the issuance of the convertible note to OrbiMed Private Investments VI, LP and corresponding convertible note (Note 7), accounts payable and accrued expenses. Fair value estimates of these instruments are made at a specific point in time, based on relevant market information. The fair value of cash equivalents, which consisted of money-market funds, were determined using Level 1 inputs reflecting quoted prices in active markets. Both the automatic conversion upon a qualified financing and the automatic redemption based upon the Series Seed price are treated as embedded redemption features and were determined to be freestanding instruments that impose an obligation on the Company to issue shares, which results in liability classification under ASC 480, *Distinguishing Liabilities from Equity*. Fair value was determined using Level 3 inputs. The fair value of the convertible note was $10,000,000 as of December 31, 2017. The carrying amount of accounts payable and accrued expenses as reported on the balance sheets as of December 31, 2017, December 31, 2018 and March 31, 2019 (unaudited), approximates fair value, due to the short-term duration of these instruments.

In December 2017, the Company issued and sold $10,000,000 in the principal amount of a convertible note. At issuance, the Company bifurcated the automatic conversion and automatic redemption features from the respective host debt instruments and determined the fair value of the derivative liabilities of $2,241,770 which was classified in Level 3 as of December 31, 2017.

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The following table sets forth a summary of the changes in the fair value of the Company' s Level 3 financial liabilities:

Balance–July 6, 2017	$–
Issuance of convertible note derivative liabilities	2,241,770
Change in fair value of Level 3 liabilities	–
Balance–December 31, 2017	$2,241,770
Change in fair value of Level 3 derivative liabilities	781,463
Settlement of convertible note	(3,023,233)
Balance–December 31, 2018	$–

To derive the fair value of the convertible note derivative liabilities, the Company estimated the fair value of the convertible notes with and without the derivative liabilities using a discounted cash flow approach. The difference between the "with" and "without" convertible note prices represents the fair value of the derivative liabilities at issuance and immediately prior to conversion. Key inputs for this valuation were the stated interest rate of the convertible notes, the assumed cost of debt, an assessment of the likelihood and timing of conversion, and the discount upon conversion of the note into equity. For the years ended December 31, 2017 and 2018, the Company recorded a total loss of $0 and $781,463, respectively in changes in fair value of derivative liabilities, due to the change in the fair value of the derivative liabilities during the respective periods. The Company recorded a total loss of $781,463 (unaudited) and $0 (unaudited), respectively, in changes in fair value of derivative liabilities, for the three months ended March 31, 2018 and March 31, 2019, due to the change in fair value of the derivative liabilities during the respective periods.

As of December 31, 2018, and March 31, 2019 (unaudited) there were no financial instruments classified as Level 3 investments. Further, during the period July 6, 2017 (date of inception) to December 31, 2017, the year ended December 31, 2018, and for the three months ended March 31, 2018 (unaudited) and March 31, 2019 (unaudited) there were no transfers between Level 1, Level 2, and Level 3.

5. PROPERTY AND EQUIPMENT, NET

Property and equipment, net, as of December 31, 2017, December 31, 2018 and March 31, 2019, respectively, consisted of the following:

	December 31,		March 31, 2019
	2017	2018	(unaudited)
Laboratory Equipment	$94,500	$677,215	$911,289
Computer Equipment	13,585	56,550	78,914
Furniture & Fixtures	–	980	980
Gross property and equipment	108,085	734,745	991,183
Less: Accumulated depreciation	(2,018)	(56,878)	(93,703)
Property and equipment, net	$106,067	$677,867	$897,480

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Depreciation expense was approximately $2,018 and $54,860 for the period from July 6, 2017 to December 31, 2017 and for the year ended December 31, 2018, respectively. Depreciation expense was approximately $5,084 (unaudited) and $36,825 (unaudited) for the three months ended March 31, 2018 and March 31, 2019, respectively.

6. ACCRUED EXPENSES

Accrued expenses consisted of the following:

	December 31,		March 31, 2019
	2017	2018	(unaudited)
Accrued compensation	$76,701	$840,144	$679,133
Accrued research and development expense	12,525	273,496	483,769
Other	60,000	363,573	288,869
Total accrued expenses	$149,226	$1,477,213	$1,451,771

7. CONVERTIBLE NOTE - RELATED PARTY

In December 2017, the Company entered into a Convertible Note Purchase Agreement with OrbiMed Private Investments VI, LP (the "Holder"), an existing investor of the Company (Note 12). Under the terms of the agreement, the note had a principal amount of $10,000,000 and accrued interest at 8.0% per annum, with a maturity date of December 31, 2018. Under the terms of the agreement, the note would be convertible in two scenarios: i) if the Company closes a sale of preferred stock in an equity financing with aggregate proceeds of at least $25,000,000 ("Qualified Financing"), and ii) upon a Corporate Transaction, which is defined as a deemed liquidation event such as a merger or consolidation, or sale of the Company.

In the first scenario, the note automatically converts into preferred stock of the Company at a conversion price equal to 90% of the price per share paid in the financing ("Automatic Conversion Upon a Qualified Financing"). In the second scenario, the Holder could elect to receive either i) the outstanding balance of the note immediately prior to the corporate transaction, or ii) the amount that the Holder would have received if the Holder converted the outstanding balance immediately prior to such Corporate Transaction into the number of shares of Series Seed Preferred Stock determined by dividing the outstanding balance by the Original Issue Price of Series Seed Preferred Stock, which becomes due and payable in cash as and when such amounts are paid to holders of the Series Seed Preferred Stock ("Automatic Redemption Based Upon Series Seed Price").

In connection with the issuance of the convertible note, the Company incurred issuance costs of $12,399. The debt issuance costs are recorded as a discount on the debt and presented net of the principal balance on the balance sheet. The costs are amortized to interest expense over the life of the debt using the effective interest method.

Derivative Liabilities

The convertible note was considered a hybrid financial instrument consisting of a fixed interest rate host with certain embedded features requiring evaluation for bifurcation and separate accounting. The Company

determined that the Automatic Conversion Upon a Qualified Financing and Automatic Redemption Based Upon Series Seed Price features were considered freestanding financial instruments which required bifurcation from the host debt instrument. At the issuance date of the convertible note, the Company bifurcated from the respective host debt instrument the automatic conversion and automatic redemption features and recorded derivative liabilities of $2,241,770 as of December 31, 2017, and $0 as of March 31, 2018, December 31, 2018 and March 31, 2019, at the estimated fair value of each feature. The derivative liabilities were revalued at each reporting date and immediately prior to conversion with changes in fair value recorded to Change in fair value of derivative liabilities in the statement of operations. The company recorded losses of $0 and $781,463 in the periods ended December 31, 2017 and 2018, respectively, attributable to changes in fair value of the derivative liabilities. Further, the company recorded losses of $781,463 (unaudited) and $0 (unaudited) in the three months ended March 31, 2018 and March 31, 2019, respectively, attributable to changes in the fair value of the derivative liabilities.

The resulting debt discount from the derivative liabilities was presented as a direct deduction from the carrying amount of the convertible note payable and was amortized to interest expense using the effective interest rate method.

Interest expense on the convertible notes, including amortization of debt issuance costs, consisted of the following:

	Period from July 6, 2017 (date of inception) to December 31, 2017	Year ended December 31, 2018	Three months ended March 31, 2018 (unaudited)	Three months ended March 31, 2019 (unaudited)
Coupon Interest	$ 8,767	$ 142,466	$142,466	$ –
Discount Amortization	18,702	328,612	328,612	–
Total Interest Expense	$27,469	$471,078	$ 471,078	$ –

In March and April 2018, the Company issued 7,666,716 shares of Series A Preferred Stock (Note 8) for total net proceeds of $64,907,028. In connection with the issuance, the derivative liabilities of $3,023,233 were extinguished and the principal amount of the note of $10,000,000 together with $151,233 of accrued interest thereon, was automatically converted into 1,330,369 shares of Series A preferred stock (Note 8).

8. REDEEMABLE CONVERTIBLE PREFERRED STOCK

In August 2017, the Company authorized the sale and issuance of up to 6,480,000 shares of Series Seed preferred stock (“Series Seed”), with a par value per share of $0.0001. On the same date, the Company issued 6,480,000 shares of Series Seed preferred stock to OrbiMed Private Investments VI, LP and the Silverstein Foundation (the “Initial Investors”), at a price of $0.62 per share for aggregate proceeds of $4,000,000. Issuance costs were $56,609. The Company may offer or sell new securities in subsequent closings, however, must first offer such new securities to the Initial Investors.

In March 2018, the Company authorized the sale and issuance of up to 9,072,000 shares of Series A preferred stock (“Series A”), with a par value per share of $0.0001. During March and April 2018 the Company issued 7,666,716 shares of Series A preferred stock at a price of $8.48 per share for aggregate proceeds of

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$65,000,079. Issuance costs were $93,051. In connection with the Series A issuance, 1,330,369 shares of Series A preferred stock were issued upon the conversion of the convertible note with OrbiMed, resulting in a total Series A stock issuance of 8,997,085 shares.

In March 2019, the Company authorized the sale and issuance of 3,958,046 shares of Series B preferred stock ("Series B") with a par value per share of $0.0001 at a price of $12.63 per share for aggregate proceeds of $50,000,011. Issuance costs were $165,887 (unaudited).

Series Seed preferred stock, Series A preferred stock and Series B preferred stock are collectively referred to as "Preferred Stock".

The rights, privileges, and preferences of the shares of redeemable convertible Preferred Stock are summarized as follows:

Voting

At every meeting of the shareholders of the corporation, each holder of the Preferred Stock shall be entitled to the same number of votes as the number of whole shares of common stock into which such holder' s shares of preferred stock could be converted on the record date for the determination of shareholders entitled to vote at any such meeting. Except as otherwise expressly provided in the stock purchase agreement or as required by law, the common stock and the preferred stock will vote together as a single class.

Dividends

The holders of the Preferred Stock are entitled to receive dividends, out of any assets legally available, prior to and in preference to any other dividend, at a rate of 6% of the original issue price. The right to receive preferred dividends shall not be cumulative, and therefore, if not declared in any year, the right to receive such dividends shall not carry forward into the next year. The holders of the Preferred Stock are also entitled to receive dividends payable when, as and if declared by the Board of Directors of the Corporation, with the holders of common stock, paid out of any assets or on the common stock of the Company, on an as-converted to common stock basis.

Conversion

Each share of Preferred Stock will be convertible into common stock at a one-to-one conversion ratio, subject to adjustments based on certain events specified in the Certificate of Incorporation ("COI") including anti-dilution adjustments, at the option of the holder, at any time and from time to time, and without the payment of additional consideration by the holder, into such number of fully paid and nonassessable shares of common stock as is determined by dividing the Series Seed Original Issue Price by the Series Seed Conversion Price, the Series A Original Issue Price by the Series A Conversion Price, or the Series B Original Issue Price by the Series B Conversion Price in effect at the time of conversion. At December 31, 2018 and March 31, 2019 (unaudited), the conversion price of the Series Seed and Series A preferred shares were $0.62 and $8.48 per share, respectively. At March 31, 2019, the conversion price of the Series B preferred shares was $12.63 per share.

Upon the occurrence of a Trigger Event as defined in the COI, each share of Preferred Stock will be automatically converted into such number of shares of common stock at the then effective conversion rate.

Redemption

The Preferred Stock may be redeemed upon a Deemed Liquidation Event. The Series B Preferred Stock may be redeemed at $12.63 per share, or the holders of the Series B Preferred Stock may receive an amount equal to the amount entitled if the Series B Preferred Stock converted into shares of common stock on the redemption date. The Series A Preferred Stock may be redeemed at $8.48 per share, or the holders of Series A Preferred Stock may receive an amount equal to the amount entitled if the Series A Preferred Stock converted into shares of common stock on the redemption date. The Series Seed Preferred Stock may be redeemed at $0.62 per share, or the Series Seed Preferred Stock may receive an amount equal to the amount entitled if the Series Seed Preferred Stock converted into shares of common stock on the redemption date. At March 31, 2019, the shares of Preferred Stock were not redeemable and the likelihood of an occurrence of a Deemed Liquidation Event was not deemed to be probable.

Right of First Offer

Upon the proposal or sale of any New Securities, the Company shall first offer such New Securities to each investor of the Preferred Stock, and each investor shall be entitled to apportion the right of first offer in such proportions as it deems appropriate. Upon the occurrence of a Trigger Event as defined in the COI, each share of Series A preferred stock will be automatically converted into such number of shares of common stock at the then effective conversion rate.

Liquidation

In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the holders of shares of Preferred Stock then outstanding shall be entitled to be paid out of the assets of the Corporation available for distribution to its stockholders before any payment shall be made to the holders of Common Stock, according to their priority, based on the greater of the Preferred Stock original issue price or amounts that would be payable had the shares been converted into common stock immediately prior to such liquidation event.

If upon any such liquidation event, the assets of the Corporation available for distribution to its stockholders shall be insufficient to pay the holders of shares of Series A and Series B Preferred Stock the full amount to which they shall be entitled, distributions would first be made to holders of the Series B Preferred Stock equal to their original issuance price plus any declared but unpaid dividends. After distribution to the holders Series B Preferred Stock, the holders of Series A Preferred Stock as a class would receive a distribution equal to their original issue price plus any declared but unpaid dividends.

After payment of all preferential amounts required to be paid to the holders of preferred stock, the remaining funds and assets available for distribution to the shareholders of the Company will be distributed among the holders of Series B Preferred Stock, Series A Preferred Stock, Series Seed Preferred Stock, and common stock, pro rata based on the number of shares held by each such holder on an as-converted basis.

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9. COMMON STOCK

General

As of July 6, 2017, the date of incorporation, the Company was authorized to issue 6,480,000 shares of common stock at a per share par value of $0.0001, which was subsequently amended in August 2017 to a total amount of 16,200,000 authorized shares of common stock issued at a par value of $0.0001. Total authorized shares were subsequently amended to 27,540,000 authorized shares of common stock in March 2018, 28,398,600 authorized shares of common stock in December 2018 and 35,640,000 authorized shares of common stock in March 2019 (unaudited).

In August 2017, OrbiMed Private Investments VI, LP purchased 2,430,000 shares for $150. Proceeds of $420,000 were allocated to these shares based on relative fair value.

In August 2017, the Company' s founder and CEO purchased 2,349,000 shares for $145 pursuant to a restricted stock purchase agreement. Of the stock purchased, 25% of the restricted shares shall vest on the one-year anniversary of the effective date of the purchase agreement; and an additional 1/48th of the restricted shares shall vest at the end of each successive one-month period for 36 months thereafter in equal installments, provided that the founder and CEO remains in continuing service to the Company.

In August 2017, 2,430,000 shares were sold to REGENXBIO in exchange for license rights (Note 3).

There were 7,209,000 issued and outstanding shares of common stock as of December 31, 2017, December 31, 2018 and March 31, 2019. The voting, dividend, and liquidation rights of the holders of common stock are subject to and qualified by the rights, powers, and preferences of the holders of the shares of preferred stock.

Reserve for future issuance

As of December 31, 2017, December 31, 2018, and March 31, 2019 the Company has reserved the following number of shares of common stock for future issuance upon the conversion of preferred stock, exercise of options or grant of equity awards:

	December 31,		March 31, 2019
	2017	2018	(unaudited)
Redeemable convertible preferred stock outstanding, as converted	6,480,000	15,477,085	19,435,131
Options and restricted stock issued and outstanding	3,092,580	6,366,739	6,725,161
Shares available for future stock option grants	1,767,420	844,287	485,862
Total	11,340,000	22,688,111	26,646,154

10. LEASES

Lease Agreements–The Company leases office and laboratory space in its New York City location under an operating lease agreement entered in September 2017, with an original term of 49 months. In connection with the lease, the Company paid a security deposit of $91,414 in the form of an unconditional and irrevocable letter

of credit, which is secured with cash on deposit classified as restricted cash. The original lease was modified in October 2018, which extended the original term of the lease and included two additional floors, each floor representing a separate lease component. The Company accounted for the new agreement as a modification of the original agreement and recorded an additional right-of-use asset and corresponding lease liability based on the incremental borrowing rate determined as of the effective date of the modified lease. The additional floors were recognized as additional lease components. One of these lease components provides an incentive allowance to reimburse the Company for the cost of qualified leasehold improvement. During the three months ended March 31, 2019, the Company incurred qualified costs of $32,395 which were payable to the Company as of March 31, 2019 and will be recognized over the remaining lease term. The agreement does not include any options to extend or terminate the lease, and no restrictions or covenants are imposed by the lease agreement.

The Company identified and assessed the following significant assumption in recognizing the right-of-use assets and corresponding liabilities:

Incremental borrowing rate–The Company' s lease agreement does not provide a readily determinable implicit rate. As the Company does not have any external borrowings for comparable terms of the lease, the Company estimated the incremental borrowing rate based on the credit quality of the Company and by comparing interest rates available in the market for similar borrowings adjusted for the impact of collateral over the term of the lease.

The Company is required to pay for operating costs, including insurance, maintenance, and taxes, which are billed annually based on the Company' s share of the total rentable square footage. These additional charges are considered variable lease cost and are recognized in the period in which the costs are incurred.

The components of the lease expense were as follows:

	For the period from July 6, 2017 to December 31, 2017	For the Year Ended December 31, 2018	For the three months ended March 31, 2018 (unaudited)	For the three months ended March 31, 2019 (unaudited)
Operating lease cost	$ 97,530	$751,971	$97,530	$459,379
Variable lease cost	28,101	225,840	27,460	138,576
Total lease cost	$ 125,631	$977,811	124,990	597,955
Weighted-average remaining lease term	3.83 years	6.17 years	3.58 years	5.92 years
Weighted-average discount rate	8.50 %	9.00 %	8.50 %	9.00 %

Cash paid for amounts included in the measurement of the lease liabilities were $60,924 for the period from July 6, 2017 to December 31, 2017 and $568,642 for the year ended December 31, 2018. Cash paid for amounts included in the measurement of the lease liabilities were $27,661 for the three months ended March 31, 2018. Cash paid for amounts included in the measurement of the lease liabilities were $321,441 for the three months ended March 31, 2019, which included $140,544 that was prepaid in 2018.

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As of December 31, 2018 and March 31, 2019 (unaudited), the maturities of the Company' s remaining operating lease liabilities were as follows:

	December 31, 2018	March 31, 2019 (unaudited)
2019	$1,647,147	$1,381,236
2020	1,817,964	1,817,964
2021	1,881,593	1,881,593
2022	1,947,449	1,947,449
2023	2,015,610	2,015,610
Thereafter	2,264,526	2,264,526
Present value adjustment	(2,704,753)	(2,609,585)
Present value of lease payments	$8,869,536	$8,698,793

11. STOCK-BASED COMPENSATION

In August 2017, the Company adopted the Prevail Therapeutics Inc. 2017 Equity Incentive Plan (the "Plan") under which the Company may grant incentive stock options, nonqualified stock options, stock appreciate rights (SARs), restricted stock, unrestricted stock, restricted stock units, performance awards, or other awards that are convertible into or based on Company stock. The maximum number of shares that may be issued under the Plan was 2,511,000 shares as of December 31, 2017. In March 2018, the Company amended the Plan and increased the number of shares available to be issued under the Plan to 4,003,427. Shares underlying any award that are forfeited, expired, or repurchased shall be excluded from the maximum number that may be issued. In December 2018 and April 2019, the Company amended the Plan and increased the number of shares available to be issued under the Plan to 4,862,027 and 6,029,733, respectively.

The Company' s Board of Directors determines the exercise price for all stock options and SARs and the vesting schedule for all equity awards. The exercise price for a stock option awarded under the Plan shall not be less than 100% of the fair market value of the Company' s common stock on the date of grant. Options granted under the Plan vest 25% after the first year and monthly thereafter over the following three years and expire ten years from the date of grant.

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Stock Options

The following tables summarize stock option activity under the Plan:

	Number of Awards	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (Years)	Aggregate Intrinsic Value
Outstanding, December 31, 2017	743,580	$ 0.18		
Granted	3,617,840	$ 0.91		
Cancelled/Forfeited	(343,684)	$ 0.18		
Outstanding, December 31, 2018	4,017,736	$ 0.83	9.3	7,938,557
Granted	364,500	$ 2.81		
Cancelled/Forfeited	(6,075)	$ 0.18		
Outstanding, March 31, 2019 (unaudited)	4,376,161	$ 1.00	9.1	7,922,582
Exercisable, December 31, 2018	137,223	$ 0.18	8.9	376,823
Vested and expected to vest, December 31, 2018	4,017,736	$ 0.83	9.3	7,938,557
Exercisable, March 31, 2019 (unaudited)	799,043	$ 0.18	8.9	2,101,190
Vested and expected to vest, March 31, 2019 (unaudited)	4,376,161	$ 1.00	9.1	7,922,582

No options were exercised during the periods ended December 31, 2017, December 31, 2018 or March 31, 2019 (unaudited).

As of December 31, 2018, the total unrecognized compensation expense related to unvested employee and non-employee options was $7,149,808, which the Company expects to recognize over an estimated weighted-average period of 3.3 years. As of March, 31, 2019, the total unrecognized compensation expense related to unvested employee and non-employee options was $7,208,003 (unaudited), which the Company expects to recognize over an estimated weighted-average period of 3.1 years (unaudited).

The Company uses the Black-Scholes option pricing model to estimate the fair value of stock options on the date of grant. The Company determined the assumptions for the Black-Scholes option-pricing valuation model as discussed below. Each of these inputs is subjective and generally requires significant judgment to determine. The weighted-average fair value of stock option awards and assumptions used to determine the fair value of stock options granted during the years ended December 31, 2017 and 2018, and for the three months ended March 31, 2018 and March 31, 2019 was as follows:

	For the period from July 6, 2017 to December 31, 2017	For the Year Ended December 31, 2018	For the Three Months Ended March 31, 2018 (unaudited)	For the Three Months Ended March 31, 2019 (unaudited)
Weighted-average grant date fair value of stock option awards	$0.13	$2.39	$2.54	$1.94
Expected term	6.1	6.1	6.1	6.0
Risk-free interest rate	2.15 %	2.75 %	2.72 %	2.52 %
Expected volatility	74.67 %	74.57 %	73.72 %	78.50 %
Dividend rate	–	–	–	–

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Expected Term − The expected term represents the period that the stock-based awards are expected to be outstanding. As the Company does not have sufficient historical experience for determining the expected term of the stock option awards granted, the Company based its expected term for awards issued to employees and non-employees using the simplified method, which is presumed to be the midpoint between the vesting date and the end of the contractual term.

Risk-Free Interest Rate − The risk-free interest rate is based on the U.S. Treasury yield curve in effect at the date of grant for zero-coupon U.S. Treasury constant maturity notes with terms approximately equal to the stock-based awards' expected term.

Expected Volatility − Since the Company does not have a trading history of common stock, the expected volatility was derived from the average historical stock volatilities of the common stock of several public companies within the industry that the Company considers to be comparable to its business over a period equivalent to the expected term of the stock-based awards.

Dividend Rate − The expected dividend rate is zero as the Company has not paid and does not anticipate paying any dividends in the foreseeable future.

Fair Value of Common Stock − The fair value of the shares of common stock underlying the stock-based awards has historically been determined by the Board of Directors with input from management. Because there has been no public market for the common stock, the Board of Directors has determined the fair value of the common stock at the time of grant of the stock-based award by considering a number of objective and subjective factors, including having valuations of the common stock performed by a third-party valuation specialist. The fair value of the underlying common stock will be determined by the Board of Directors until such time as the common stock is listed on an established stock exchange or national market system.

Restricted Stock

As of December 31, 2018, and March 31, 2019 (unaudited), 2,349,000 shares of common stock are subject to a repurchase right by the company.

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Non-vested Restricted Stock Unit (RSU) Awards Outstanding

The following table presents a summary of the Company' s non-vested restricted stock unit award activity under all plans and related information for the year ended December 31, 2018 and for the three months ended March 31, 2019:

	Number of Restricted Stock Awards Outstanding	Weighted Average Grant Date Fair Value Per Share
Non-vested restricted stock unit awards outstanding as of December 31, 2017	2,349,000	$0.18
Restricted stock unit awards vested, December 31, 2018	(782,999)	$0.18
Non-vested restricted stock unit awards outstanding as of December 31, 2018	1,566,001	$0.18
Restricted stock unit awards vested, March 31, 2019 (unaudited)	(146,809)	$0.18
Non-vested restricted stock unit awards outstanding as of March 31, 2019 (unaudited)	1,419,192	$0.18

	Years Ended December 31,		For the Three Months Ended March 31, 2019 (unaudited)
	2017	2018	
Aggregate grant date fair value of restricted stock unit awards vested	–	$135,290	$ 25,367

Restricted stock unit awards are generally granted at the fair market value of the Company' s common stock on the date of grant and vest 25% after the first year and monthly thereafter over the following three years and expire ten years from the date of grant. Forfeitures are based on actual forfeitures in the given period.

There was $270,308 of total unrecognized compensation cost related to non-vested restricted stock unit awards granted under the Company' s equity incentive plans as of December 31, 2018. This cost is expected to be recognized over a weighted-average period of 2.66 years. There was $244,933 (unaudited) of total unrecognized compensation cost related to non-vested restricted stock unit awards granted under the Company' s equity incentive plans as of March 31, 2019. The cost is expected to be recognized over a weighted-average period of 2.42 years.

Total stock-based compensation expense is recognized for restricted stock and stock options granted to employees and non-employees and has been reported in the Company' s statements of operations as follows:

	For the period from July 6, 2017 to December 31, 2017	For the Year Ended December 31, 2018	For the Three Months Ended March 31, 2018 (unaudited)	For the Three Months Ended March 31, 2019 (unaudited)
Research and development	$ 45,088	$ 1,454,243	$ 142,371	$ 512,337
General and administrative	894	156,383	9,997	99,518
Total stock-based compensation expense	$45,982	$1,610,626	$152,368	$611,855

PREVAIL THERAPEUTICS INC.

NOTES TO FINANCIAL STATEMENTS

FOR THE PERIOD FROM JULY 6, 2017 (DATE OF INCEPTION) TO DECEMBER 31, 2017
AND FOR THE YEAR ENDED DECEMBER 31, 2018
(INFORMATION AS OF MARCH 31, 2019 AND FOR THE THREE MONTHS ENDED MARCH 31, 2019 AND MARCH 31, 2018 IS UNAUDITED)

12. INCOME TAXES

The effective tax rate for the Company for the years ended December 31, 2017 and December 31, 2018, and for the three month periods ended March 31, 2018, and March 31, 2019, was zero percent. A reconciliation of income tax computed at the statutory federal income tax rate to the provision (benefit) for income taxes included in the accompanying statements of operations for the Company is as follows:

	For the period from July 6, 2017 to December 31, 2017		For the Year Ended December 31, 2018	
Expected provision at statutory federal rate	34.0	%	21.0	%
State tax - net of federal benefit	11.1	%	10.1	%
Nondeductible interest expense	(0.5	)%	(1.4	)%
Tax credits	3.2	%	1.2	%
Changes in valuation allowance	(34.3	)%	(29.3	)%
Tax reform	(12.3	)%	0.0	%
Other	(1.2	)%	(1.6	)%
Effective Income Tax Rate	0.0	%	0.0	%

Deferred income taxes reflect the net tax effects of (a) temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes, and (b) operating losses and tax credit carryforwards. Significant components of the Company' s deferred tax assets and liability are as follows:

	December 31,	
	2017	2018
Net operating loss carryforwards	$518,409	$4,177,272
Accruals and reserves	25,099	274,925
Deferred Rent	11,075	129,275
Stock Compensation	13,947	76,203
Tax credits	59,157	289,113
Section 195 start up cost	–	1,287,620
Other	–	56
Gross Deferred Tax Assets	627,687	6,234,464
Valuation Allowance	(626,931)	(6,211,756)
Net Deferred Tax Assets	$756	$22,708
Deferred Tax Liabilities		
Fixed Assets	(756)	(22,708)
Net Deferred Tax Assets	$–	$–

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based upon the Company' s history of operating losses, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for its deferred tax assets as of December 31, 2017, December 31, 2018, and as of March 31, 2018 and March 31, 2019.

PREVAIL THERAPEUTICS INC.

NOTES TO FINANCIAL STATEMENTS

FOR THE PERIOD FROM JULY 6, 2017 (DATE OF INCEPTION) TO DECEMBER 31, 2017 AND FOR THE YEAR ENDED DECEMBER 31, 2018 (INFORMATION AS OF MARCH 31, 2019 AND FOR THE THREE MONTHS ENDED MARCH 31, 2019 AND MARCH 31, 2018 IS UNAUDITED)

As of December 31, 2017, and December 31, 2018, the Company' s federal net operating loss carryforwards were approximately $1,584,207 and $12,765,318, respectively. The Company had federal research and development credit carryforwards as of December 31, 2017 and December 31, 2018 of approximately $59,157 and $289,113, respectively. The federal net operating loss incurred prior to January 1, 2018 and tax credit carryforwards will begin to expire in 2037 if not utilized. Federal net operating losses incurred after December 31, 2017 will not expire. The Company has not completed a formal research credit and development analysis, therefore when this analysis is finalized, the Company plans to update its research and development credit carryforward. As of December 31, 2017, and December 31, 2018, the Company had state net operating loss carryforwards of approximately $3,168,414 and $25,530,636, respectively. The state net operating loss carryforwards will begin to expire in 2037, if not utilized.

Future utilization of the Company' s net operating loss and research and development credit carryforwards to offset future taxable income may be subject to an annual limitation, pursuant to IRC Sections 382 and 383, as a result of ownership changes that may have occurred or that could occur in the future. An ownership change occurs when a cumulative change in ownership of more than 50% occurs within a three-year period. The Company has not completed an IRC Section 382/383 analysis regarding the limitation of net operating loss and research and development credit carryforwards.

In accordance with ASC 740, *Income Taxes* ("ASC 740"), specifically related to uncertain tax positions, a Company is required to use a recognition threshold and a measurement attribute for the financial statement recognition and measurement of tax positions taken or expected to be taken in a tax return. For those benefits to be recognized, a tax position must be more likely than not to be sustained upon examination by taxing authorities. The Company believes its income tax filing positions and deductions will be sustained upon examination, and accordingly, no reserves or related accruals for interest and penalties have been recorded as of December 31, 2017, December 31, 2018 or March 31, 2019 (unaudited).

In accordance with this guidance, the Company has adopted a policy under which, if required to be recognized in the future, interest related to the underpayment of income taxes will be classified as a component of interest expense and any related penalties will be classified in operating expenses in the statements of operations.

The Tax Cuts and Jobs Act ("the Act") was enacted in December 2017. The Act includes a number of changes to then-existing U.S. tax laws that impact the Company, most notably a reduction of the U.S. federal corporate tax rate from a maximum of 35% to a flat 21%, effective January 1, 2018. As a result of the new law, as of December 31, 2017, the Company remeasured its deferred tax assets based on the rates at which they are expected to reverse in the future, resulting in a reduction in the deferred tax asset balance of $225,558, which was offset by a reduction in the valuation allowance by a corresponding amount.

The Company is subject to taxation in the United States federal and state jurisdictions. The Company' s federal income tax and state income tax returns are subject to examination by tax authorities. The Company is not currently under examination by any tax authority.

13. RELATED PARTY TRANSACTIONS

Since the Company' s inception in July 2017, the Company has engaged in transactions with related parties, which included OrbiMed Private Investments VI, LP principal owners of the Company and REGENXBIO.

PREVAIL THERAPEUTICS INC.

NOTES TO FINANCIAL STATEMENTS

FOR THE PERIOD FROM JULY 6, 2017 (DATE OF INCEPTION) TO DECEMBER 31, 2017 AND FOR THE YEAR ENDED DECEMBER 31, 2018 (INFORMATION AS OF MARCH 31, 2019 AND FOR THE THREE MONTHS ENDED MARCH 31, 2019 AND MARCH 31, 2018 IS UNAUDITED)

In August 2017, the OrbiMed Private Investments VI, LP purchased the initial Common Stock of the entity. See Note 9. In August 2017, the Company entered into a Series Seed Preferred Stock Purchase Agreement with OrbiMed Private Investments VI, LP. See Note 8.

In August 2017, the Company entered into a Patent License Agreement and Stock Purchase Agreement with REGENXBIO Inc. See Note 3.

No payments were made to related parties during the period from July 6, 2017 (inception) to December 31, 2017. Aggregate payments in connection with the above related party relationships totaled $683,256 for the year ended December 31, 2018, including $675,000 paid to REGENXBIO under licensing agreements and $8,256 to OrbiMed for reimbursement of travel costs related to the attendance at Company board meetings. The fees paid to related parties are included within research and development in the statement of operations. No payments were made to related parties for the three months ended March 31, 2018 (unaudited) and March 31, 2019 (unaudited).

In April 2019, the Company exercised all of the options under the REGENXBIO Option Gene License and paid the additional up-front fee of $600,000 per option, or an aggregate of $1,800,000 (unaudited), to REGENXBIO. In addition, the company purchased materials pursuant to a material transfer agreement with REGENXBIO in the amount of $90,584 (unaudited).

14. NET LOSS PER SHARE

The following outstanding potentially dilutive common stock equivalents have been excluded from the calculation of diluted net loss per share for the periods presented due to their anti-dilutive effect:

	For the period from July 6, 2017 to December 31, 2017	For the Year Ended December 31, 2018	For the three months ended March 31, 2018 (unaudited)	For the three months ended March 31, 2019 (unaudited)
Redeemable convertible preferred stock (as converted)	6,480,000	15,477,085	14,533,489	19,435,131
Common stock options issued and outstanding	743,580	4,017,739	3,119,572	4,376,161
Restricted stock subject to future vesting	2,349,000	1,566,002	2,349,000	1,419,191
Total	9,572,580	21,060,826	20,002,061	25,230,483

Neither the Company' s redeemable convertible preferred stock nor restricted stock subject to future vesting participates in losses.

	For the period from July 6, 2017 to December 31, 2017	For the Year Ended December 31, 2018	For the Three Months Ended March 31, 2018 (unaudited)	For the Three Months Ended March 31, 2019 (unaudited)
Net loss	$ (1,826,543)	$ (19,087,539)	$ (3,198,174)	$ (9,946,155)
Weighted-average number of shares-basic and diluted	4,050,000	5,145,469	4,860,000	5,740,874
Net loss per share–basic and diluted	$ (0.45)	$ (3.71)	$ (0.66)	$ (1.73)

PREVAIL THERAPEUTICS INC.

NOTES TO FINANCIAL STATEMENTS

FOR THE PERIOD FROM JULY 6, 2017 (DATE OF INCEPTION) TO DECEMBER 31, 2017 AND FOR THE YEAR ENDED DECEMBER 31, 2018 (INFORMATION AS OF MARCH 31, 2019 AND FOR THE THREE MONTHS ENDED MARCH 31, 2019 AND MARCH 31, 2018 IS UNAUDITED)

15. COMMITMENTS AND CONTINGENCIES

Contingencies−From time to time, the Company may be involved in disputes or regulatory inquiries that arise in the ordinary course of business. When the Company determines that a loss is both probable and reasonably estimable, a liability is recorded and disclosed if the amount is material to the financial statements taken as a whole. When a material loss contingency is only reasonably possible, the Company does not record a liability, but instead discloses the nature and the amount of the claim, and an estimate of the loss or range of loss, if such an estimate can reasonably be made.

The Company is not a party to any litigation and does not have accruals established for any litigation liabilities as of December 31, 2017 and 2018 and as of March 31, 2019 (unaudited).

The Company is also party to various agreements, principally relating to licensed technology, that require future payments relating to milestones, or royalties on future sales of specified products. No material milestone or royalty payments under these agreements are expected to be payable in the immediate future. See Note 3 for further details of these agreements.

16. EMPLOYEE BENEFIT PLAN

The Company maintains a 401(k) plan that provides eligible U.S. employees with an opportunity to save for retirement on a tax advantaged basis. Eligible employees are able to defer eligible compensation up to certain Code limits, which are updated annually. We have the ability to make matching and discretionary contributions to the 401(k) plan. In 2018, we contributed 100% of the first 6% contributions.

Total employer matching contributions to the Company' s 401(k) Plan for the period from July 6, 2017 (date of inception) to December 31, 2017 and for the year ended December 31, 2018 were $0 and $60,726, respectively, and were $0 (unaudited) and $96,949 (unaudited) for the three months ended March 31, 2018 and March 31, 2019, respectively.

17. SUBSEQUENT EVENTS

The Company has evaluated subsequent events through June 10, 2019, the date on which the financial statements were available to be issued.

In March 2019, the Company completed a Series B financing in the amount of approximately $50,000,000. In connection with the financing, the Company issued an aggregate of 3,958,046 shares of Series B Preferred Stock.

In April 2019, the Company exercised all of the options under the REGENXBIO Option Gene License and paid the additional up-front fee of $600,000 per option, or an aggregate of $1,800,000 (unaudited), to REGENXBIO.

In April and May 2019, we issued an aggregate of 1,429,992 shares of our common stock to our employees, consultants and directors having exercise prices ranging from $10.26 to $12.13 per share.

PREVAIL THERAPEUTICS INC.

NOTES TO FINANCIAL STATEMENTS

FOR THE PERIOD FROM JULY 6, 2017 (DATE OF INCEPTION) TO DECEMBER 31, 2017 AND FOR THE YEAR ENDED DECEMBER 31, 2018 (INFORMATION AS OF MARCH 31, 2019 AND FOR THE THREE MONTHS ENDED MARCH 31, 2019 AND MARCH 31, 2018 IS UNAUDITED)

Forward Stock Split

In June 2019, the Board of Directors of the Company approved a 1.62-for-one forward stock split of the Company' s outstanding shares of common stock and convertible preferred stock. The stock split became effective on June 7, 2019. Stockholders entitled to fractional shares as a result of the forward stock split will receive cash payment in lieu of receiving fractional shares. Accordingly, all share and per share amounts for all periods presented in the accompanying financial statements and notes thereto have been retroactively adjusted, where applicable, to reflect this forward stock split. Shares of common stock underlying outstanding stock options and other equity instruments were proportionately increased and the respective per share value and exercise prices, if applicable, were proportionately decreased in accordance with the terms of the agreements governing such securities.

7,353,000 Shares

Common Stock

Prospectus

MORGAN STANLEY

BofA MERRILL LYNCH

COWEN

WEDBUSH PACGROW

, 2019

Part II

Information Not Required in Prospectus

Item 13. Other Expenses of Issuance and Distribution.

The following table indicates the expenses to be incurred in connection with the offering described in this registration statement, other than underwriting discounts and commissions, all of which will be paid by us. All amounts are estimated except the U.S. Securities and Exchange Commission, or the SEC, registration fee, the Financial Industry Regulatory Authority, Inc., or FINRA, filing fee and the Nasdaq Global Market fee.

	Amount
SEC Registration fee	$18,448
FINRA filing fee	23,332
Nasdaq Global Market initial listing fee	150,000
Accountants' fees and expenses	1,000,000
Legal fees and expenses	1,300,000
Blue Sky fees and expenses	5,000
Transfer Agent' s fees and expenses	15,000
Printing and engraving expenses	300,000
Miscellaneous	88,220
Total expenses	$2,900,000

Item 14. Indemnification of Directors and Officers.

We are incorporated under the laws of the State of Delaware. Section 102 of the Delaware General Corporation Law, or the DGCL, permits a corporation to eliminate the personal liability of directors of a corporation to the corporation or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit.

Section 145 of the DGCL provides that a corporation has the power to indemnify a director, officer, employee or agent of the corporation and certain other persons serving at the request of the corporation in related capacities against expenses (including attorneys' fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he is or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or such other court shall deem proper.

As permitted by the DGCL, our amended and restated certificate of incorporation and bylaws to be in effect upon the closing of this offering will provide that: (i) we are required to indemnify our directors to the fullest extent permitted by the DGCL; (ii) we may, in our discretion, indemnify our officers, employees and agents as set forth in the DGCL; (iii) we are required, upon satisfaction of certain conditions, to advance all expenses incurred by our directors in connection with certain legal proceedings; (iv) the rights conferred in the bylaws are not exclusive; and (v) we are authorized to enter into indemnification agreements with our directors, officers, employees and agents.

In connection with this offering, we expect to enter into indemnification agreements with each of our directors and executive officers that require us to indemnify them against expenses, judgments, fines, settlements and other amounts that any such person becomes legally obligated to pay (including with respect to a derivative action) in connection with any proceeding, whether actual or threatened, to which such person may be made a party by reason of the fact that such person is or was a director or officer of us or any of our affiliates, provided such person acted in good faith and in a manner such person reasonably believed to be in, or not opposed to, our best interests. The indemnification agreements will also set forth certain procedures that will apply in the event of a claim for indemnification thereunder. We intend to enter into similar indemnification agreements with our executive officers prior to the closing of this offering. At present, no litigation or proceeding is pending that involves any of our directors or officers regarding which indemnification is sought, nor are we aware of any threatened litigation that may result in claims for indemnification.

We maintain a directors' and officers' liability insurance policy. The policy insures directors and officers against unindemnified losses arising from certain wrongful acts in their capacities as directors and officers and reimburses us for those losses for which we have lawfully indemnified the directors and officers. The policy contains various exclusions.

In addition, the underwriting agreement filed as Exhibit 1.1 to this Registration Statement provides for indemnification by the underwriters of us and our officers and directors for certain liabilities arising under the Securities Act, or otherwise. Our investor rights agreement with certain investors also provides for cross-indemnification in connection with the registration of our common stock on behalf of such investors.

Item 15. Recent Sales of Unregistered Securities.

The following list sets forth information regarding all unregistered securities issued by us since July 6, 2017 (date of inception):

In March 2019, we issued an aggregate of 3,958,046 shares of our Series B convertible preferred stock at $12.63 per share, for aggregate consideration of approximately $50.0 million.

In March and April 2018, we issued an aggregate of 8,997,085 shares of our Series A convertible preferred stock at $8.48 per share, of which 1,330,369 were issued at a 10% discount upon conversion of a convertible note for aggregate consideration of approximately $75.0 million.

In December 2017, we issued a convertible note with an aggregate principal value of $10.0 million.

In August 2017, we issued an aggregate of 6,480,000 shares of our Series Seed convertible preferred stock at $0.62 per share, for aggregate consideration of approximately $4.0 million.

In August 2017, we issued (a) an aggregate of 4,779,000 shares of our common stock to our founder and Chief Executive Officer and OrbiMed Private Investments VI, LP, for aggregate net proceeds of $295, and (b) 2,430,000 shares of our common stock as consideration for the rights licensed under our agreement with REGENXBIO Inc.

We have granted, under our 2017 Equity Incentive Plan, options to purchase an aggregate of 5,806,153 shares of our common stock to our employees, consultants and directors, having exercise prices ranging from $0.18 to $12.13 per share.

No underwriters were involved in the foregoing sales of securities. The securities described in this Item 15 were issued to investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(2) under the Securities Act and Regulation D promulgated thereunder relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All purchasers of shares of convertible preferred stock described above represented to us in connection with their purchase that they were accredited investors and were acquiring the shares for their own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof and that they could bear the risks of the investment and could hold the securities for an indefinite period of time. The purchasers received written disclosures that the securities had not been registered under the Securities Act and

that any resale must be made pursuant to a registration statement or an available exemption from such registration.

The issuances of stock options described above were issued pursuant to written compensatory plans or arrangements with our employees, directors and consultants, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act, or pursuant to Section 4(a)(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required.

All of the foregoing securities are deemed restricted securities for purposes of the Securities Act. All certificates representing the issued shares of capital stock described in this Item 15 included appropriate legends setting forth that the securities have not been registered and the applicable restrictions on transfer.

Item 16. Exhibits and Financial Statement Schedules.

The exhibits to the registration statement are listed below. Financial statement schedules have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

Exhibit Number	Description of Exhibit
1.1†	Form of Underwriting Agreement.
3.1	Third Amended and Restated Certificate of Incorporation of the Registrant, as amended.
3.2†	Bylaws of the Registrant (currently in effect).
3.3†	Form of Amended and Restated Certificate of Incorporation of the Registrant (to be effective upon the closing of this offering).
3.4†	Form of Amended and Restated Bylaws of the Registrant (to be effective upon the closing of this offering).
4.1†	Specimen Stock Certificate evidencing the shares of common stock.
4.2†	Amended and Restated Investors' Rights Agreement, dated March 19, 2019, by and among the Registrant and the investors party thereto.
5.1	Opinion of Cooley LLP.
10.1#†	License Agreement, dated August 7, 2017, between the Registrant and REGENXBIO Inc., as amended.
10.2#†	License Agreement, dated May 10, 2018, between the Registrant and REGENXBIO Inc.
10.3+†	2017 Equity Incentive Plan and forms of agreements thereunder.
10.4+	2019 Equity Incentive Plan and forms of agreements thereunder.
10.5+†	Form of Indemnity Agreement between Registrant and each of its directors and executive officers.
10.6+	Form of Amended and Restated Employment Agreement between Registrant and Asa Abeliovich, M.D., Ph.D.
10.7+	Form of Amended and Restated Employment Agreement between Registrant and Jeffrey Sevigny, M.D.
10.8+	Form of Amended and Restated Employment Agreement between Registrant and Franz Hefti, Ph.D.
10.9~†	Lease Agreement, dated September 29, 2017, between ARE-East River Science Park, LLC and the Registrant, as amended.

Exhibit Number	Description of Exhibit
10.10+	Non-Employee Director Compensation Policy.
10.11+	2019 Employee Stock Purchase Plan.
23.1	Consent of Ernst & Young LLP, independent registered public accounting firm.
23.2	Consent of Cooley LLP (included in Exhibit 5.1).
24.1†	Power of Attorney.

+ Indicates management contract or compensatory plan.
† Previously filed.
# Portions of this exhibit (indicated by asterisks) have been omitted because the Registrant has determined they are not material and would likely cause competitive harm to the Registrant if publicly disclosed.
~ Certain exhibits and schedules have been omitted pursuant to Item 601(a)(5) of Regulation S-K. The Company hereby undertakes to furnish supplementally a copy of any omitted exhibit or schedule upon request by the SEC.

Item 17. Undertakings.

The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement, certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser. Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this Registration Statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this Registration Statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

SIGNATURES

Pursuant to the requirements of the Securities Act, the registrant has duly caused this Amendment No. 1 to Registration Statement to be signed on its behalf by the undersigned, thereunto duly authorized, in New York, New York on this 10th day of June 2019.

PREVAIL THERAPEUTICS INC.

By: /S/ ASA ABELIOVICH, M.D., PH.D.
Asa Abeliovich, M.D., Ph.D.
President and Chief Executive Officer

Pursuant to the requirements of the Securities Act of 1933, this Amendment No. 1 to Registration Statement on Form S-1 has been signed by the following persons in the capacities held on the dates indicated.

Signature	Title	Date
/S/ ASA ABELIOVICH, M.D., PH.D. Asa Abeliovich, M.D., Ph.D.	President, Chief Executive Officer and Director (*Principal Executive Officer*)	June 10, 2019
/S/ BRETT KAPLAN, M.D. Brett Kaplan, M.D.	Chief Financial Officer (*Principal Financial and Accounting Officer*)	June 10, 2019
* Timothy Adams	Director	June 10, 2019
* Carl Gordon, Ph.D., C.F.A.	Director	June 10, 2019
* Francois Nader, M.D.	Director	June 10, 2019
* Ran Nussbaum	Director	June 10, 2019
* Peter Thompson, M.D.	Director	June 10, 2019

*By: /S/ ASA ABELIOVICH, M.D., PH.D.
Asa Abeliovich, M.D., Ph.D.
President and Chief Executive Officer
Attorney-in-Fact

Exhibit 3.1

THIRD AMENDED AND RESTATED
CERTIFICATE OF INCORPORATION
OF
PREVAIL THERAPEUTICS INC.

(Pursuant to Sections 242 and 245 of the
General Corporation Law of the State of Delaware)

Prevail Therapeutics Inc., a corporation organized and existing under and by virtue of the provisions of the General Corporation Law of the State of Delaware (the "**General Corporation Law**"),

DOES HEREBY CERTIFY:

1. That the name of this corporation is Prevail Therapeutics Inc., and that this corporation was originally incorporated pursuant to the General Corporation Law on July 6, 2017, under the name Prevail Therapeutics Inc.

2. Pursuant to Sections 242 and 245 of the General Corporate Law, and having been adopted in accordance therewith, this Third Amended and Restated Certificate of Incorporation (this "**Certificate**") restates, integrates and further amends the provisions of the Restated Certificate. The Board of Directors duly adopted resolutions proposing to amend and restate the Restated Certificate, declaring said amendment and restatement to be advisable and in the best interests of this corporation and its stockholders, and authorizing the appropriate officers of this corporation to solicit the consent of the stockholders therefor, which resolution setting forth the proposed amendment and restatement is as follows:

RESOLVED, that the Certificate of Incorporation of this corporation be amended and restated in its entirety to read as follows:

FIRST: The name of this corporation is Prevail Therapeutics Inc. (the "**Corporation**").

SECOND: The address of the registered office of the Corporation in the State of Delaware is 1209 Orange Street, in the City of Wilmington, County of New Castle, 19801. The name of its registered agent at such address is The Corporation Trust Company.

THIRD: The nature of the business or purposes to be conducted or promoted is to engage in any lawful act or activity for which corporations may be organized under the General Corporation Law.

FOURTH: The total number of shares of all classes of stock which the Corporation shall have authority to issue is (i) 22,000,000 shares of Common Stock, $0.0001 par value per share ("**Common Stock**") and (ii) 11,997,003 shares of Preferred Stock, $0.0001 par value per share.

The following is a statement of the designations and the powers, privileges and rights, and the qualifications, limitations or restrictions thereof in respect of each class of capital stock of the Corporation.

A. COMMON STOCK

1. General. The voting, dividend and liquidation rights of the holders of the Common Stock are subject to and qualified by the rights, powers and preferences of the holders of the Preferred Stock set forth herein.

2. Voting. The holders of the Common Stock are entitled to one vote for each share of Common Stock held at all meetings of stockholders (and written actions in lieu of meetings); provided, however, that, except as otherwise required by law, holders of Common Stock, as such, shall not be entitled to vote on any amendment to the Certificate of Incorporation that relates solely to the terms of one or more outstanding series of Preferred Stock if the holders of such affected series are entitled, either separately or together with the holders of one or more other such series, to vote thereon pursuant to the Certificate of Incorporation or pursuant to the General Corporation Law. There shall be no cumulative voting. The number of authorized shares of Common Stock may be increased or decreased (but not below the number of shares thereof then outstanding) by (in addition to any vote of the holders of one or more series of Preferred Stock that may be required by the terms of the Certificate of Incorporation) the affirmative vote of the holders of shares of Common Stock and Preferred Stock, voting together on an as-converted basis, of the Corporation representing a majority of the votes represented by all outstanding shares of Common Stock and Preferred Stock of the Corporation entitled to vote, irrespective of the provisions of Section 242(b)(2) of the General Corporation Law.

B. PREFERRED STOCK

Of the authorized Preferred Stock (as defined below), 4,000,000 shares have been designated Series Seed Preferred Stock, $0.0001 par value per share (the "**Series Seed Preferred Stock**"), 5,553,759 shares have been designated Series A Preferred Stock, $0.0001 par value per share (the "**Series A Preferred Stock**"), and 2,443,244 shares have been designated Series B Preferred Stock, $0.0001 par value per share (the "**Series B Preferred Stock**") each of such series with the following rights, preferences, powers, privileges and restrictions, qualifications and limitations. The term "**Preferred Stock**," as used in this Certificate, shall mean the Series Seed Preferred Stock, the Series A Preferred Stock and the Series B Preferred Stock. Unless otherwise indicated, references to "sections" or "subsections" in this Part B of this Article Fourth refer to sections and subsections of Part B of this Article Fourth.

1. Dividends.

The holders of Preferred Stock shall be entitled to receive dividends, out of any assets legally available therefor, prior and in preference to any declaration or payment of any other dividend (payable other than in Common Stock or other securities and rights convertible into or entitling the holder thereof to receive, directly or indirectly, additional shares of Common Stock of the Corporation) at the rate of six percent (6%) of the applicable Original Issue Price (as defined below) per share of Preferred Stock per annum (the "**Preferred Dividends**"), only when, as and if declared by the Board of Directors of the Corporation. The right to receive Preferred Dividends shall not be cumulative, and therefore, if not declared in any year, the right to receive such dividends shall terminate and not carry forward into the next year. In addition, the Corporation shall not declare, pay or set aside any dividends on shares of any other class or series of capital stock of the Corporation (other than dividends on shares of Common Stock payable in shares of Common Stock) unless (in addition to the obtaining of any consents required elsewhere in the

Certificate of Incorporation) the holders of Preferred Stock then outstanding shall first receive, or simultaneously receive, a dividend on each outstanding share of Preferred Stock in an amount equal to (i) in the case of a dividend on Common Stock or any class or series that is convertible into Common Stock, that dividend per share of Preferred Stock as would equal the product of (x) the dividend payable on each share of such class or series determined, if applicable, as if all shares of such class or series had been converted into Common Stock and (y) the number of shares of Common Stock issuable upon conversion of a share of Preferred Stock, in each case calculated on the record date for determination of holders entitled to receive such dividend, or (ii) in the case of a dividend on any class or series that is not convertible into Common Stock, at a rate per share of Preferred Stock determined by (x) dividing the amount of the dividend payable on each share of such class or series of capital stock by the original issuance price of such class or series of capital stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to such class or series) and (y) multiplying such fraction by an amount equal to the applicable Original Issue Price (as defined below); *provided that*, if the Corporation declares, pays or sets aside, on the same date, a dividend on shares of more than one class or series of capital stock of the Corporation, the dividend payable to the holders of Preferred Stock pursuant to this Section 1 shall be calculated based upon the dividend on the class or series of capital stock that would result in the highest Preferred Dividend.

The "**Series Seed Original Issue Price**" shall mean $1.00 per share, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series Seed Preferred Stock. The term "**Series A Original Issue Price**" shall mean $13.7347 per share of Series A Preferred Stock, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A Preferred Stock. The term "**Series B Original Issue Price**" shall mean $20.4646 per share of Series B Preferred Stock, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series B Preferred Stock. The applicable "**Original Issue Price**" shall mean (i) with respect to the Series Seed Preferred Stock, the Series Seed Original Issue Price, (ii) with respect to the Series A Preferred Stock, the Series A Original Issue Price, and (iii) with respect to the Series B Preferred Stock, the Series B Original Issue Price.

2. Liquidation, Dissolution or Winding Up; Certain Mergers, Consolidations and Asset Sales.

2.1 Preferential Payments to Holders of Preferred Stock.

2.1.1 Series B Preferred Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the holders of shares of Series B Preferred Stock then outstanding shall be entitled to be paid out of the assets of the Corporation available for distribution to its stockholders before any payment shall be made to the holders of Series A Preferred Stock, Series Seed Preferred Stock or Common Stock by reason of their ownership thereof, an amount per share equal to the greater of (A) the sum of the Series B Original Issue Price plus an amount equal to all declared but unpaid dividends on the Series B Preferred Stock, or (B) such amount per share as would have been payable had all shares of Series B Preferred Stock been converted into Common Stock pursuant to Section 4 immediately prior to such liquidation, dissolution, winding up or Deemed Liquidation Event (the amount payable pursuant to this sentence is hereinafter referred to as the "**Series B Liquidation Amount**"). If upon any such liquidation, dissolution or winding up of the

Corporation or Deemed Liquidation Event, the assets of the Corporation available for distribution to its stockholders shall be insufficient to pay the holders of shares of Series B Preferred Stock the full amount to which they shall be entitled under this Subsection 2.1.1, the holders of shares of Series B Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

2.1.2 Series A Preferred Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the holders of shares of Series A Preferred Stock then outstanding shall be entitled to be paid out of the assets of the Corporation available for distribution to its stockholders after payment of the Series B Liquidation Amount is made to holders of the Series B Preferred Stock pursuant to Subsection 2.1.1, but before any payment shall be made to the holders of Series Seed Preferred Stock or Common Stock by reason of their ownership thereof, an amount per share equal to the greater of (A) the sum of the Series A Original Issue Price plus an amount equal to all declared but unpaid dividends on the Series A Preferred Stock, or (B) such amount per share as would have been payable had all shares of Series A Preferred Stock been converted into Common Stock pursuant to Section 4 immediately prior to such liquidation, dissolution, winding up or Deemed Liquidation Event (the amount payable pursuant to this sentence is hereinafter referred to as the "**Series A Liquidation Amount**"). If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the assets of the Corporation available for distribution to its stockholders shall be insufficient to pay the holders of shares of Series A Preferred Stock the full amount to which they shall be entitled under this Subsection 2.1.2, the holders of shares of Series A Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

2.1.3 Series Seed Preferred Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the holders of shares of Series Seed Preferred Stock then outstanding shall be entitled to be paid out of the assets of the Corporation available for distribution to its stockholders after payment of the Series B Liquidation Amount is made to holders of the Series B Preferred Stock pursuant to Subsection 2.1.1 and after payment of the Series A Liquidation Amount is made to holders of the Series A Preferred Stock pursuant to Subsection 2.1.2, but before any payment shall be made to the holders of Common Stock by reason of their ownership thereof, an amount per share equal to the greater of (A) the sum of the Series Seed Original Issue Price plus an amount equal to all declared but unpaid dividends on the Series Seed Preferred Stock, or (B) such amount per share as would have been payable had all shares of Series Seed Preferred Stock been converted into Common Stock pursuant to Section 4 immediately prior to such liquidation, dissolution, winding up or Deemed Liquidation Event (the amount payable pursuant to this sentence is hereinafter referred to as the "**Series Seed Liquidation Amount**"). If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the remaining assets of the Corporation available for distribution to its stockholders shall be insufficient to pay the holders of shares of Series Seed Preferred Stock the full amount to which they shall be entitled under this Subsection 2.1.3, the holders of shares of Series Seed Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

2.2 Payments to Holders of Common Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, after the payment of all preferential amounts required to be paid to the holders of shares of Preferred Stock of the amounts specified or contemplated under Sections 2.1.1, 2.1.2, and 2.1.3, the remaining assets of the Corporation available for distribution to its stockholders shall be distributed among the holders of shares of Common Stock, pro rata based on the number of shares held by each such holder.

2.3 Deemed Liquidation Events.

2.3.1 Definition. Each of the following events shall be considered a "**Deemed Liquidation Event**" unless the holders of at least a majority of the outstanding shares of Preferred Stock (voting together as a single class and on an as-converted basis) elect otherwise by written notice sent to the Corporation at least 10 days prior to the effective date of any such event:

(a) a merger or consolidation in which

(i) the Corporation is a constituent party or

(ii) a subsidiary of the Corporation is a constituent party and the Corporation issues shares of its capital stock pursuant to such merger or consolidation,

except any such merger or consolidation involving the Corporation or a subsidiary in which the shares of capital stock of the Corporation outstanding immediately prior to such merger or consolidation continue to represent, or are converted into or exchanged for shares of capital stock that represent, immediately following such merger or consolidation, at least a majority, by voting power, of the capital stock of (1) the surviving or resulting corporation; or (2) if the surviving or resulting corporation is a wholly owned subsidiary of another corporation immediately following such merger or consolidation, the parent corporation of such surviving or resulting corporation; or

(b) the sale, lease, transfer, exclusive license or other disposition, in a single transaction or series of related transactions, by the Corporation or any subsidiary of the Corporation of all or substantially all the assets of the Corporation and its subsidiaries taken as a whole, or the sale or disposition (whether by merger, consolidation or otherwise) of one or more subsidiaries of the Corporation if substantially all of the assets of the Corporation and its subsidiaries taken as a whole are held by such subsidiary or subsidiaries, except where such sale, lease, transfer, exclusive license or other disposition is to a wholly owned subsidiary of the Corporation.

2.3.2 Effecting a Deemed Liquidation Event.

(a) The Corporation shall not have the power to effect a Deemed Liquidation Event referred to in Subsection 2.3.1(a)(i) unless the agreement or plan of merger or consolidation for such transaction (the "**Merger Agreement**") provides that the consideration payable to the stockholders of the Corporation shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2.

(b) In the event of a Deemed Liquidation Event referred to in Subsection 2.3.1(a)(ii) or 2.3.1(b), if the Corporation does not effect a dissolution of the Corporation under the General Corporation Law within ninety (90) days after such Deemed Liquidation Event, then (i) the Corporation shall send a written notice to each holder of Preferred Stock no later than the ninetieth (90th) day after the Deemed Liquidation Event advising such holders of their right (and the requirements to be met to secure such right) pursuant to the terms of the following clause; (ii) to require the redemption of such shares of Preferred Stock, and (iii) if the holders of at least a majority of the then outstanding shares of Preferred Stock so request in a written instrument delivered to the Corporation not later than one hundred twenty (120) days after such Deemed Liquidation Event, the Corporation shall use the consideration received by the Corporation for such Deemed Liquidation Event (net of any retained liabilities associated with the assets sold or technology licensed, as determined in good faith by the Board of Directors of the Corporation)**,** together with any other assets of the Corporation available for distribution to its stockholders, all to the extent permitted by Delaware law governing distributions to stockholders (the "**Available Proceeds**"), on the one hundred fiftieth (150th) day after such Deemed Liquidation Event, to redeem all outstanding shares of Series Seed Preferred Stock at a price per share equal to the Series Seed Liquidation Amount, all outstanding shares of Series A Preferred Stock at a price per share equal to the Series A Liquidation Amount, and all outstanding shares of Series B Preferred Stock at a price per share equal to the Series B Liquidation Amount. Notwithstanding the foregoing, in the event of a redemption pursuant to the preceding sentence, if the Available Proceeds are not sufficient to redeem all outstanding shares of Preferred Stock, the Corporation shall first ratably redeem each holder' s shares of Series B Preferred Stock to the fullest extent of such Available Proceeds and shall redeem the remaining shares of Series B Preferred Stock as soon as it may lawfully do so under Delaware law governing distributions to stockholders, and second, after all shares of Series B Preferred Stock have been redeemed, ratably redeem each holder' s shares of Series A Preferred Stock to the fullest extent of such remaining Available Proceeds and shall redeem the remaining shares of Series A Preferred Stock as soon as it may lawfully do so under Delaware law governing distributions to stockholders, and third, after all shares of Series A Preferred Stock have been redeemed, ratably redeem each holder' s shares of Series Seed Preferred Stock to the fullest extent of such remaining Available Proceeds and shall redeem the remaining shares of Series Seed Preferred Stock as soon as it may lawfully do so under Delaware law governing distributions to stockholders. Prior to the distribution or redemption provided for in this Subsection 2.3.2(b), the Corporation shall not expend or dissipate the consideration received for such Deemed Liquidation Event, except to discharge expenses incurred in connection with such Deemed Liquidation Event or in the ordinary course of business. If the Corporation is required by the provisions this Section 2.3.2(b) to redeem shares, the redemption shall occur in accordance with the provisions of Sections 2.3.2(b), (c), (d) and (e). The date upon which any such redemption is required to be effected pursuant to this Section 2.3.2(b) shall be the "**Redemption Date**."

(c) The Corporation shall send written notice of any redemption pursuant to this Section 2.3.2 (the "**Redemption Notice**") to each holder of record of Preferred Stock as required by Section 2.3.2(b). Each Redemption Notice shall state:

(i) the number of shares held by the holder that the Corporation shall redeem on the Redemption Date specified in the Redemption Notice (which number shall not be less than the number of shares the Corporation is then required to redeem);

(ii) the Redemption Date and the redemption price; and

(iii) that the holder is to surrender to the Corporation, in the manner and at the place designated, his, her or its certificate or certificates representing the shares of Preferred Stock to be redeemed.

If the Corporation receives, on or prior to the 10th day after the date of delivery of the Redemption Notice to a holder of Preferred Stock, written notice from such holder that such holder elects to be excluded from the redemption provided in this Section 2.3.2, then the shares of Preferred Stock registered on the books of the Corporation in the name of such holder at the time of the Corporation' s receipt of such notice shall thereafter be "**Excluded Shares**." Excluded Shares shall not be redeemed or redeemable pursuant to this Section 2.3.2, whether on such Redemption Date or thereafter.

(d) On or before the applicable Redemption Date, each holder of shares to be redeemed on such Redemption Date, shall surrender the certificate or certificates representing such shares (or, if such registered holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation, in the manner and at the place designated in the Redemption Notice, and thereupon the redemption price for such shares shall be payable to the order of the person whose name appears on such certificate or certificates as the owner thereof. In the event less than all of the shares represented by a certificate are redeemed, a new certificate representing the unredeemed shares shall promptly be issued to such holder.

(e) If the Redemption Notice shall have been duly given, and if on the applicable Redemption Date the redemption price payable upon redemption of the shares to be redeemed on such Redemption Date is paid or tendered for payment or deposited with an independent payment agent so as to be available therefor in a timely manner, then notwithstanding that the certificates evidencing any of the shares so called for redemption shall not have been surrendered, dividends with respect to such shares shall cease to accrue after such Redemption Date and all rights with respect to such shares shall forthwith after the Redemption Date terminate, except only the right of the holders to receive the redemption price without interest upon surrender of their certificate or certificates therefor. Any shares of Preferred Stock that are redeemed or otherwise acquired by the Corporation or any of its subsidiaries shall be automatically and immediately cancelled and retired and shall not be reissued, sold or transferred.

2.3.3 Amount Deemed Paid or Distributed. The amount deemed paid or distributed to the holders of capital stock of the Corporation upon any such merger, consolidation, sale, transfer, exclusive license, other disposition or redemption shall be the cash or the value of the property, rights or securities paid or distributed to such holders by the Corporation or the acquiring person, firm or other entity. The value of such property, rights or securities shall be determined in good faith by the Board of Directors of the Corporation.

2.3.4 Allocation of Escrow and Contingent Consideration. In the event of a Deemed Liquidation Event pursuant to Subsection 2.3.1(a)(i), if any portion of the consideration payable to the stockholders of the Corporation is payable only upon satisfaction of contingencies (the "**Additional Consideration**"), the Merger Agreement shall provide that (a) the portion of such consideration that is not Additional Consideration (such portion, the "**Initial Consideration**") shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2 as if the Initial Consideration were the only consideration payable in connection with such Deemed Liquidation Event; and (b) any Additional Consideration which becomes payable to the stockholders of the Corporation upon satisfaction of such contingencies shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2 after taking into account the previous payment of the Initial Consideration as part of the same transaction. For the purposes of this Subsection 2.3.4, consideration placed into escrow or retained as holdback to be available for satisfaction of indemnification or similar obligations in connection with such Deemed Liquidation Event shall be deemed to be Additional Consideration.

3. Voting.

3.1 General. On any matter presented to the stockholders of the Corporation for their action or consideration at any meeting of stockholders of the Corporation (or by written consent of stockholders in lieu of meeting), each holder of outstanding shares of Preferred Stock shall be entitled to cast the number of votes equal to the number of whole shares of Common Stock into which the shares of Preferred Stock held by such holder are convertible as of the record date for determining stockholders entitled to vote on such matter. Except as provided by law or by the other provisions of the Certificate of Incorporation, holders of Preferred Stock shall vote together with the holders of Common Stock as a single class.

3.2 Election of Directors. The holders of record of the shares of Series Seed Preferred Stock, exclusively and as a separate class, shall be entitled to elect three (3) directors of the Corporation (the "**Series Seed Directors**"); the holders of record of the shares of Series A Preferred Stock, exclusively and as a separate class, shall be entitled to elect one (1) director of the Corporation (the "**Series A Director**," and together with the Series Seed Directors, the "**Preferred Directors**"); and the holders of record of the shares of Common Stock, exclusively and as a separate class, shall be entitled to elect one (1) director of the Corporation. Any director elected as provided in the preceding sentence may be removed without cause by, and only by, the affirmative vote of the holders of the shares of the class or series of capital stock entitled to elect such director or directors, given either at a special meeting of such stockholders duly called for that purpose or pursuant to a written consent of stockholders. If the holders of shares of Series Seed Preferred Stock, Series A Preferred Stock or Common Stock, as the case may be, fail to elect a sufficient number of directors to fill all directorships for which they are entitled to elect directors, voting exclusively and as a separate class, pursuant to the first sentence of this Subsection 3.2, then any directorship not so filled shall remain vacant until such time as the holders of the Series Seed

Preferred Stock, Series A Preferred Stock or Common Stock, as the case may be, elect a person to fill such directorship by vote or written consent in lieu of a meeting; and no such directorship may be filled by stockholders of the Corporation other than by the stockholders of the Corporation that are entitled to elect a person to fill such directorship, voting exclusively and as a separate class. The holders of record of the shares of Common Stock and of any other class or series of voting stock (including the Preferred Stock), exclusively and voting together as a single class, shall be entitled to elect the balance of the total number of directors of the Corporation. At any meeting held for the purpose of electing a director, the presence in person or by proxy of the holders of a majority of the outstanding shares of the class or series entitled to elect such director shall constitute a quorum for the purpose of electing such director. Except as otherwise provided in this Subsection 3.2, a vacancy in any directorship filled by the holders of any class or series shall be filled only by vote or written consent in lieu of a meeting of the holders of such class or series or by any remaining director or directors elected by the holders of such class or series pursuant to this Subsection 3.2.

3.3 Preferred Stock Protective Provisions. At any time when 1,000,000 shares of Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Preferred Stock) are outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote of the holders of at least a majority of the then outstanding shares of Preferred Stock given in writing or by vote at a meeting, consenting or voting (as the case may be) separately as a class, and any such act or transaction entered into without such consent or vote shall be null and void ab initio, and of no force or effect.

3.3.1 liquidate, dissolve or wind-up the business and affairs of the Corporation, effect any merger or consolidation or any other Deemed Liquidation Event, or consent to any of the foregoing;

3.3.2 amend, alter or repeal any provision of the Certificate of Incorporation or Bylaws of the Corporation in a manner that adversely affects the powers, preferences or rights of the Series Seed Preferred Stock, Series A Preferred Stock or Series B Preferred Stock;

3.3.3 create, or authorize the creation of, or issue or obligate itself to issue shares of, any additional class or series of capital stock, or increase the authorized number of shares of Series Seed Preferred Stock, Series A Preferred Stock, or Series B Preferred Stock or increase the authorized number of shares of any additional class or series of capital stock (in each case excluding up to 3,001,251 shares of Common Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination of other similar recapitalization), issued or issuable to officers, directors, consultants, advisors or employees of the Corporation (it being understood that any such shares issued that expire or terminate unexercised or are repurchased by the Corporation shall not be counted towards the maximum number unless and until regranted or reissued) issued pursuant to an equity incentive plan of the Corporation);

3.3.4 purchase or redeem (or permit any subsidiary to purchase or redeem) or pay or declare any dividend or make any distribution on, any shares of capital stock of the Corporation other than (i) dividends or distributions on the Preferred Stock as expressly authorized herein, (ii) dividends or other distributions payable on the Common Stock solely in the form of additional shares of Common Stock and (iii) repurchases of stock from former employees or consultants who performed services for the Corporation or any subsidiary in connection with the cessation of such employment or service at the lower of the original purchase price or the then-current fair market value thereof, or (iv) as approved by the Board of Directors, including the approval of at least a majority of the Preferred Directors;

3.3.5 create, or authorize the creation of, or issue, or authorize the issuance of any debt security, or permit any subsidiary to take any such action with respect to any debt security, if the aggregate indebtedness of the Corporation and its subsidiaries for borrowed money following such action would exceed $500,000 (in each case other than equipment leases or bank lines of credit) unless such debt security has received the prior approval of the Board of Directors, including the approval of at least a majority of the Preferred Directors;

3.3.6 create, or hold capital stock in, any subsidiary that is not wholly owned (either directly or through one or more other subsidiaries) by the Corporation, or sell, transfer or otherwise dispose of any capital stock of any direct or indirect subsidiary of the Corporation, or permit any direct or indirect subsidiary to sell, lease, transfer, exclusively license or otherwise dispose (in a single transaction or series of related transactions) of all or substantially all of the assets of such subsidiary;

3.3.7 increase or decrease the authorized number of directors constituting the Board of Directors; or

3.3.8 enter into a (or amend any existing) transaction or agreement with affiliates of the Corporation or senior management, except for (i) arms-length employment agreements on customary terms and (ii) transactions approved by the Corporation' s Board of Directors, including the affirmative vote or consent of at least a majority of the Preferred Directors.

3.4 Series B Preferred Stock Protective Provisions. At any time when 488,648 shares of Series B Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Preferred Stock) are outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote of the holders of at least a majority of the then outstanding shares of Series B Preferred Stock, which majority must include at least one holder of Series B Preferred Stock that, together with its affiliates, does not own any shares of Common Stock, Series Seed Preferred Stock or Series A Preferred Stock as of the date of the Series B Original Issue Date and owns at least 244,324 shares of Series B Preferred Stock as of the date of the Series B Original Issue Date (the "**Series B Requisite Holders**"), given in writing or by vote at a meeting, consenting or voting (as the case may be) separately as a class, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect.

3.4.1 amend, alter, waive, repeal or modify any provision of the Certificate of Incorporation or Bylaws of the Corporation in a manner that adversely affects the rights, preferences or privileges of the Series B Preferred Stock;

3.4.2 increase the authorized number of shares of Series B Preferred Stock;

3.4.3 reclassify, amend or modify existing securities of the Corporation (including the Series A Preferred Stock, Series Seed Preferred Stock and Common Stock) in a manner that adversely affects the rights, preferences or privileges of the Series B Preferred Stock; or

3.4.4 amend, alter, waive, repeal or modify this Section 3.4, the Series B Original Issue Price, the Series B Conversion Price (including any anti-dilution rights with respect thereto) or the Series B Liquidation Amount.

4. Optional Conversion.

The holders of the Preferred Stock shall have conversion rights as follows (the "**Conversion Rights**"):

4.1 Right to Convert. Each share of Preferred Stock shall be convertible, at the option of the holder thereof, at any time and from time to time, and without the payment of additional consideration by the holder thereof, into such number of fully paid and non-assessable shares of Common Stock as is determined by dividing the applicable Original Issue Price by the applicable Conversion Price (as defined below) in effect at the time of conversion for the series of Preferred Stock then being converted. The "**Series Seed Conversion Price**" shall initially be equal to the Series Seed Original Issue Price. The "**Series A Conversion Price**" shall initially be the Series A Original Issue Price. The "**Series B Conversion Price**" shall initially be the Series B Original Issue Price. The applicable "**Conversion Price**" shall mean (i) with respect to the Series Seed Preferred Stock, the Series Seed Conversion Price, (ii) with respect to the Series A Preferred Stock, the Series A Conversion Price, and (iii) with respect to the Series B Preferred Stock, the Series B Conversion Price. Such initial Conversion Price, and the rate at which shares of Preferred Stock may be converted into shares of Common Stock, shall be subject to adjustment as provided below.

4.2 Fractional Shares. No fractional shares of Common Stock shall be issued upon conversion of the Preferred Stock. In lieu of any fractional shares to which the holder would otherwise be entitled, the Corporation shall pay cash equal to such fraction multiplied by the fair market value of a share of Common Stock as determined in good faith by the Board of Directors of the Corporation. Whether or not fractional shares would be issuable upon such conversion shall be determined on the basis of the total number of shares of Preferred Stock the holder is at the time converting into Common Stock and the aggregate number of shares of Common Stock issuable upon such conversion.

4.3 Mechanics of Conversion.

4.3.1 Notice of Conversion. In order for a holder of Preferred Stock to voluntarily convert shares of Preferred Stock into shares of Common Stock, such holder shall (a) provide written notice to the Corporation' s transfer agent at the office of the transfer agent for the Preferred Stock (or at the principal office of the Corporation if the Corporation serves as its own transfer agent) that such holder elects to convert all or any number of such holder' s shares of

Preferred Stock and, if applicable, any event on which such conversion is contingent and (b), if such holder' s shares are certificated, surrender the certificate or certificates for such shares of Preferred Stock (or, if such registered holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate), at the office of the transfer agent for the Preferred Stock (or at the principal office of the Corporation if the Corporation serves as its own transfer agent). Such notice shall state such holder' s name or the names of the nominees in which such holder wishes the shares of Common Stock to be issued. If required by the Corporation, any certificates surrendered for conversion shall be endorsed or accompanied by a written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or his, her or its attorney duly authorized in writing. The close of business on the date of receipt by the transfer agent (or by the Corporation if the Corporation serves as its own transfer agent) of such notice and, if applicable, certificates (or lost certificate affidavit and agreement) shall be the time of conversion (the "**Conversion Time**"), and the shares of Common Stock issuable upon conversion of the specified shares shall be deemed to be outstanding of record as of such date. The Corporation shall, as soon as practicable after the Conversion Time (i) issue and deliver to such holder of Preferred Stock, or to his, her or its nominees, a certificate or certificates for the number of full shares of Common Stock issuable upon such conversion in accordance with the provisions hereof and a certificate for the number (if any) of the shares of Preferred Stock represented by the surrendered certificate that were not converted into Common Stock, (ii) pay in cash such amount as provided in Subsection 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and (iii) pay all declared but unpaid dividends on the shares of Preferred Stock converted.

4.3.2 Reservation of Shares. The Corporation shall at all times when the Preferred Stock shall be outstanding, reserve and keep available out of its authorized but unissued capital stock, for the purpose of effecting the conversion of the Preferred Stock, such number of its duly authorized shares of Common Stock as shall from time to time be sufficient to effect the conversion of all outstanding Preferred Stock; and if at any time the number of authorized but unissued shares of Common Stock shall not be sufficient to effect the conversion of all then outstanding shares of the Preferred Stock, the Corporation shall take such corporate action as may be necessary to increase its authorized but unissued shares of Common Stock to such number of shares as shall be sufficient for such purposes, including, without limitation, engaging in best efforts to obtain the requisite stockholder approval of any necessary amendment to the Certificate of Incorporation. Before taking any action which would cause an adjustment reducing the Conversion Price below the then par value of the shares of Common Stock issuable upon conversion of the Preferred Stock, the Corporation will take any corporate action which may, in the opinion of its counsel, be necessary in order that the Corporation may validly and legally issue fully paid and non-assessable shares of Common Stock at such adjusted Conversion Price.

4.3.3 Effect of Conversion. All shares of Preferred Stock which shall have been surrendered for conversion as herein provided shall no longer be deemed to be outstanding and all rights with respect to such shares shall immediately cease and terminate at the Conversion Time, except only the right of the holders thereof to receive shares of Common Stock in exchange therefor, to receive payment in lieu of any fraction of a share otherwise issuable upon such conversion as provided in Subsection 4.2 and to receive payment of any dividends declared but unpaid thereon. Any shares of Preferred Stock so converted shall be retired and cancelled and may not be reissued as shares of such series, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of Preferred Stock accordingly.

4.3.4 No Further Adjustment. Upon any such conversion, no adjustment to the Conversion Price shall be made for any declared but unpaid dividends on the Preferred Stock surrendered for conversion or on the Common Stock delivered upon conversion.

4.3.5 Taxes. The Corporation shall pay any and all issue and other similar taxes that may be payable in respect of any issuance or delivery of shares of Common Stock upon conversion of shares of Preferred Stock pursuant to this Section 4. The Corporation shall not, however, be required to pay any tax which may be payable in respect of any transfer involved in the issuance and delivery of shares of Common Stock in a name other than that in which the shares of Preferred Stock so converted were registered, and no such issuance or delivery shall be made unless and until the person or entity requesting such issuance has paid to the Corporation the amount of any such tax or has established, to the satisfaction of the Corporation, that such tax has been paid.

4.4 Adjustments to Conversion Price for Diluting Issues.

4.4.1 Special Definitions. For purposes of this Article Fourth, the following definitions shall apply:

(a) "**Option**" shall mean rights, options or warrants to subscribe for, purchase or otherwise acquire Common Stock or Convertible Securities.

(b) "**Series B Original Issue Date**" shall mean the date on which the first share of Series B Preferred Stock was issued.

(c) "**Convertible Securities**" shall mean any evidences of indebtedness, shares or other securities directly or indirectly convertible into or exchangeable for Common Stock, but excluding Options.

(d) "**Additional Shares of Common Stock**" shall mean all shares of Common Stock issued (or, pursuant to Subsection 4.4.3 below, deemed to be issued) by the Corporation after the Series B Original Issue Date, other than (1) the following shares of Common Stock and (2) shares of Common Stock deemed issued pursuant to the following Options and Convertible Securities (clauses (1) and (2), collectively, "**Exempted Securities**"):

(i) shares of Common Stock, Options or Convertible Securities issued as a dividend or distribution on Preferred Stock;

(ii) shares of Common Stock, Options or Convertible Securities issued by reason of a dividend, stock split, split-up or other distribution on shares of Common Stock that is covered by Subsection 4.5, 4.6, 4.7 or 4.8**;**

(iii) shares of Common Stock or Options issued to employees or directors of, or consultants or advisors to, the Corporation or any of its subsidiaries pursuant to any equity incentive plan, agreement or arrangement approved by the Board of Directors of the Corporation, including at least a majority of the Preferred Directors;

(iv) shares of Common Stock or Convertible Securities actually issued upon the exercise of Options or shares of Common Stock actually issued upon the conversion or exchange of Convertible Securities, in each case provided such issuance is pursuant to the terms of such Option or Convertible Security;

(v) shares of Common Stock, Options or Convertible Securities issued to banks, equipment lessors or other financial institutions, or to real property lessors, pursuant to a debt financing, equipment leasing or real property leasing transaction approved by the Board of Directors of the Corporation, including at least a majority of the Preferred Directors;

(vi) shares of Common Stock, Options or Convertible Securities issued to suppliers or third party service providers in connection with the provision of goods or services pursuant to transactions approved by the Board of Directors of the Corporation, including at least a majority of the Preferred Directors;

(vii) shares of Common Stock, Options or Convertible Securities issued pursuant to the acquisition of another corporation by the Corporation by merger, purchase of substantially all of the assets or other reorganization or to a joint venture agreement, provided that such issuances are approved by the Board of Directors of the Corporation, including at least a majority of the Preferred Directors; or

(viii) shares of Common Stock, Options or Convertible Securities issued in connection with sponsored research, collaboration, technology license, development, OEM, marketing or other similar agreements or strategic partnerships approved by the Board of Directors of the Corporation, including at least a majority of the Preferred Directors.

4.4.2 No Adjustment of Conversion Price. No adjustment in the Conversion Price shall be made as the result of the issuance or deemed issuance of Additional Shares of Common Stock if the Corporation receives written notice from the holders of at least a majority of the then outstanding shares of Preferred Stock (voting together on an as-converted basis) agreeing that no such adjustment shall be made as the result of the issuance or deemed issuance of such Additional Shares of Common Stock; provided, however, that notwithstanding the foregoing, no waiver of adjustment in the Series B Conversion Price shall be made without the written consent of the Series B Requisite Holders.

4.4.3 Deemed Issue of Additional Shares of Common Stock.

(a) If the Corporation at any time or from time to time after the Series B Original Issue Date shall issue any Options or Convertible Securities (excluding Options or Convertible Securities which are themselves Exempted Securities) or shall fix a record date for the determination of holders of any class of securities entitled to receive any such Options or Convertible Securities, then the maximum number of shares of Common Stock (as set forth in the instrument relating thereto, assuming the satisfaction of any conditions to exercisability, convertibility or exchangeability but without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or, in the case of Convertible Securities and Options therefor, the conversion or exchange of such Convertible Securities, shall be deemed to be Additional Shares of Common Stock issued as of the time of such issue or, in case such a record date shall have been fixed, as of the close of business on such record date.

(b) If the terms of any Option or Convertible Security, the issuance of which resulted in an adjustment to the Conversion Price pursuant to the terms of Subsection 4.4.4, are revised as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (1) any increase or decrease in the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any such Option or Convertible Security or (2) any increase or decrease in the consideration payable to the Corporation upon such exercise, conversion and/or exchange, then, effective upon such increase or decrease becoming effective, such Conversion Price computed upon the original issue of such Option or Convertible Security (or upon the occurrence of a record date with respect thereto) shall be readjusted to such Conversion Price as would have obtained had such revised terms been in effect upon the original date of issuance of such Option or Convertible Security. Notwithstanding the foregoing, no readjustment pursuant to this clause (b) shall have the effect of increasing the Conversion Price of such series of Preferred Stock to an amount which exceeds the lower of (i) the Conversion Price of such series of Preferred Stock in effect immediately prior to the original

adjustment made as a result of the issuance of such Option or Convertible Security, or (ii) the Conversion Price of such series of Preferred Stock that would have resulted from any issuances of Additional Shares of Common Stock (other than deemed issuances of Additional Shares of Common Stock as a result of the issuance of such Option or Convertible Security) between the original adjustment date and such readjustment date.

(c) If the terms of any Option or Convertible Security (excluding Options or Convertible Securities which are themselves Exempted Securities), the issuance of which did not result in an adjustment to the Conversion Price of such series of Preferred Stock pursuant to the terms of Subsection 4.4.4 (either because the consideration per share (determined pursuant to Subsection 4.4.5) of the Additional Shares of Common Stock subject thereto was equal to or greater than the such Conversion Price then in effect, or because such Option or Convertible Security was issued before the Series B Original Issue Date), are revised after the Series B Original Issue Date as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (1) any increase in the number of shares of Common Stock issuable upon the exercise, conversion or exchange of any such Option or Convertible Security or (2) any decrease in the consideration payable to the Corporation upon such exercise, conversion or exchange, then such Option or Convertible Security, as so amended or adjusted, and the Additional Shares of Common Stock subject thereto (determined in the manner provided in Subsection 4.4.3(a) shall be deemed to have been issued effective upon such increase or decrease becoming effective.

(d) Upon the expiration or termination of any unexercised Option or unconverted or unexchanged Convertible Security (or portion thereof) which resulted (either upon its original issuance or upon a revision of its terms) in an adjustment to the Conversion Price of such series of Preferred Stock pursuant to the terms of Subsection 4.4.4, such Conversion Price shall be readjusted to such Conversion Price as would have obtained had such Option or Convertible Security (or portion thereof) never been issued.

(e) If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, is calculable at the time such Option or Convertible Security is issued or amended but is subject to adjustment based upon subsequent events, any adjustment to the Conversion Price of such series of Preferred Stock provided for in this Subsection 4.4.3 shall be effected at the time of such issuance or amendment based on such number of shares or amount of consideration without regard to any provisions for subsequent adjustments (and any subsequent adjustments shall be treated as provided in clauses (b) and (c) of this Subsection 4.4.3). If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, cannot be calculated at all at the time such Option or Convertible Security is issued or amended, any adjustment to the Conversion Price of such series of Preferred Stock that would result under the terms of this Subsection 4.4.3 at the time of such issuance or amendment shall instead be effected at the time such number of shares and/or amount of consideration is first calculable (even if subject to subsequent adjustments), assuming for purposes of calculating such adjustment to such Conversion Price that such issuance or amendment took place at the time such calculation can first be made.

4.4.4 Adjustment of Conversion Price Upon Issuance of Additional Shares of Common Stock. In the event the Corporation shall at any time after the Series B Original Issue Date issue Additional Shares of Common Stock (including Additional Shares of Common Stock deemed to be issued pursuant to Subsection 4.4.3), without consideration or for a consideration per share less than the Conversion Price in effect immediately prior to such issue, then such Conversion Price shall be reduced, concurrently with such issue, to a price (calculated to the nearest one-hundredth of a cent) determined in accordance with the following formula:

$$CP_2 = CP_1 * (A + B) \div (A + C).$$

For purposes of the foregoing formula, the following definitions shall apply:

(a) "CP_2" shall mean the Conversion Price in effect immediately after such issue of Additional Shares of Common Stock

(b) "CP_1" shall mean the Conversion Price in effect immediately prior to such issue of Additional Shares of Common Stock;

(c) "A" shall mean the number of shares of Common Stock outstanding immediately prior to such issue of Additional Shares of Common Stock (treating for this purpose as outstanding all shares of Common Stock issuable upon exercise of Options outstanding immediately prior to such issue or upon conversion or exchange of Convertible Securities (including the Preferred Stock) outstanding (assuming exercise of any outstanding Options therefor) immediately prior to such issue);

(d) "B" shall mean the number of shares of Common Stock that would have been issued if such Additional Shares of Common Stock had been issued at a price per share equal to CP_1 (determined by dividing the aggregate consideration received by the Corporation in respect of such issue by CP_1); and

(e) "C" shall mean the number of such Additional Shares of Common Stock issued in such transaction.

4.4.5 Determination of Consideration. For purposes of this Subsection 4.4, the consideration received by the Corporation for the issue of any Additional Shares of Common Stock shall be computed as follows:

(a) Cash and Property: Such consideration shall:

(i) insofar as it consists of cash, be computed at the aggregate amount of cash received by the Corporation, excluding amounts paid or payable for accrued interest;

(ii) insofar as it consists of property other than cash, be computed at the fair market value thereof at the time of such issue, as determined in good faith by the Board of Directors of the Corporation; and

(iii) in the event Additional Shares of Common Stock are issued together with other shares or securities or other assets of the Corporation for consideration which covers both, be the proportion of such consideration so received, computed as provided in clauses (i) and (ii) above, as determined in good faith by the Board of Directors of the Corporation.

(b) Options and Convertible Securities. The consideration per share received by the Corporation for Additional Shares of Common Stock deemed to have been issued pursuant to Subsection 4.4.3, relating to Options and Convertible Securities, shall be determined by dividing:

(i) The total amount, if any, received or receivable by the Corporation as consideration for the issue of such Options or Convertible Securities, plus the minimum aggregate amount of additional consideration (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such consideration) payable to the Corporation upon the exercise of such Options or the conversion or exchange of such Convertible Securities, or in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities, by

(ii) the maximum number of shares of Common Stock (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or the conversion or exchange of such Convertible Securities, or in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities.

4.4.6 Multiple Closing Dates. In the event the Corporation shall issue on more than one date Additional Shares of Common Stock that are a part of one transaction or a series of related transactions and that would result in an adjustment to the Conversion Price pursuant to the terms of Subsection 4.4.4, and such issuance dates occur within a period of no more than ninety (90) days from the first such issuance to the final such issuance, then, upon the final such issuance, such Conversion Price shall be readjusted to give effect to all such issuances as if they occurred on the date of the first such issuance (and without giving effect to any additional adjustments as a result of any such subsequent issuances within such period).

4.5 Adjustment for Stock Splits and Combinations. If the Corporation shall at any time or from time to time after the Series B Original Issue Date effect a subdivision of the outstanding Common Stock, the Conversion Price in effect immediately before that subdivision shall be proportionately decreased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be increased in proportion to such increase in the aggregate number of shares of Common Stock outstanding. If the Corporation shall at any time or from time to time after the Series B Original Issue Date combine the outstanding shares of Common Stock, the Conversion Price of any series of Preferred Stock in effect immediately before the combination shall be proportionately increased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be decreased in proportion to such decrease in the aggregate number of shares of Common Stock outstanding. Any adjustment under this subsection shall become effective at the close of business on the date the subdivision or combination becomes effective.

4.6 Adjustment for Certain Dividends and Distributions. In the event the Corporation at any time or from time to time after the Series B Original Issue Date shall make or issue, or fix a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable on the Common Stock in additional shares of Common Stock, then and in each such event the Conversion Price in effect immediately before such event shall be decreased as of the time of such issuance or, in the event such a record date shall have been fixed, as of the close of business on such record date, by multiplying such Conversion Price then in effect by a fraction:

(1) the numerator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date, and

(2) the denominator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date plus the number of shares of Common Stock issuable in payment of such dividend or distribution.

Notwithstanding the foregoing (a) if such record date shall have been fixed and such dividend is not fully paid or if such distribution is not fully made on the date fixed therefor, the Conversion Price shall be recomputed accordingly as of the close of business on such record date and thereafter such Conversion Price shall be adjusted pursuant to this subsection as of the time of actual payment of such dividends or distributions; and (b) that no such adjustment shall be made if the holders of Preferred Stock simultaneously receive a dividend or other distribution of shares of Common Stock in a number equal to the number of shares of Common Stock as they would have received if all outstanding shares of Preferred Stock had been converted into Common Stock on the date of such event.

4.7 Adjustments for Other Dividends and Distributions. In the event the Corporation at any time or from time to time after the Series B Original Issue Date shall make or issue, or fix a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable in securities of the Corporation (other than a distribution of shares of Common Stock in respect of outstanding shares of Common Stock) or in other property and the provisions of Section 1 do not apply to such dividend or distribution, then and in each such event the holders of Preferred Stock shall receive, simultaneously with the distribution to the holders of Common Stock, a dividend or other distribution of such securities or other property in an amount equal to the amount of such securities or other property as they would have received if all outstanding shares of Preferred Stock had been converted into Common Stock on the date of such event.

4.8 Adjustment for Merger or Reorganization, etc. Subject to the provisions of Subsection 2.3, if there shall occur any reorganization, recapitalization, reclassification, consolidation or merger involving the Corporation in which the Common Stock (but not all outstanding Preferred Stock) is converted into or exchanged for securities, cash or other property (other than a transaction covered by Subsections 4.4, 4.6 or 4.7), then, following any such reorganization, recapitalization, reclassification, consolidation or merger, each share of any series of Preferred Stock shall thereafter be convertible in lieu of the Common Stock into which it was convertible prior to such event into the kind and amount of securities, cash or other property which a holder of the number of shares of Common Stock of the Corporation issuable upon conversion of one share of Preferred Stock immediately prior to such reorganization, recapitalization, reclassification, consolidation or merger would have been entitled to receive pursuant to such transaction; and, in such case, appropriate adjustment (as determined in good faith by the Board of Directors of the Corporation) shall be made in the application of the provisions in this Section 4 with respect to the rights and interests thereafter of the holders of the Preferred Stock, to the end that the provisions set forth in this Section 4 (including provisions with respect to changes in and other adjustments of the Conversion Price of such series of Preferred Stock) shall thereafter be applicable, as nearly as reasonably may be, in relation to any securities or other property thereafter deliverable upon the conversion of the Preferred Stock. For the avoidance of doubt, nothing in this Subsection 4.8 shall be construed as preventing the holders of any series of Preferred Stock from seeking any appraisal rights to which they are otherwise entitled under the DGCL in connection with a merger triggering an adjustment hereunder, nor shall this Subsection 4.8 be deemed conclusive evidence of the fair value of the shares of Preferred Stock in any such appraisal proceeding.

4.9 Certificate as to Adjustments. Upon the occurrence of each adjustment or readjustment of the Conversion Price of any series of Preferred Stock pursuant to this Section 4, the Corporation at its expense shall, as promptly as reasonably practicable but in any event not later than ten (10) days thereafter, compute such adjustment or readjustment in accordance with the terms hereof and furnish to each holder of such series of Preferred Stock a certificate setting forth such adjustment or readjustment (including the kind and amount of securities, cash or other property into which the such series of Preferred Stock is convertible) and showing in detail the facts upon which such adjustment or readjustment is based. The Corporation shall, as promptly as reasonably practicable, furnish or cause to be furnished to such holder a certificate setting forth (i) the Conversion Price of each series of Preferred Stock then in effect, and (ii) the number of shares of Common Stock and the amount, if any, of other securities, cash or property which then would be received upon the conversion of each series of Preferred Stock.

4.10 Notice of Record Date. In the event:

(a) the Corporation shall take a record of the holders of its Common Stock (or other capital stock or securities at the time issuable upon conversion of any series of Preferred Stock) for the purpose of entitling or enabling them to receive any dividend or other distribution, or to receive any right to subscribe for or purchase any shares of capital stock of any class or any other securities, or to receive any other security; or

(b) of any capital reorganization of the Corporation, any reclassification of the Common Stock of the Corporation, or any Deemed Liquidation Event; or

(c) of the voluntary or involuntary dissolution, liquidation or winding-up of the Corporation,

then, and in each such case, the Corporation will send or cause to be sent to each holder of Preferred Stock a notice specifying, as the case may be, (i) the record date for such dividend, distribution or right, and the amount and character of such dividend, distribution or right, or (ii) the effective date on which such reorganization, reclassification, consolidation, merger, transfer, dissolution, liquidation or winding-up is proposed to take place, and the time, if any is to be fixed, as of which the holders of record of Common Stock (or such other capital stock or securities at the time issuable upon the conversion of any series of Preferred Stock) shall be entitled to exchange their shares of Common Stock (or such other capital stock or securities) for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, transfer, dissolution, liquidation or winding-up, and the amount per share and character of such exchange applicable to the Preferred Stock and the Common Stock. Such notice shall be sent at least ten (10) days prior to the record date or effective date for the event specified in such notice.

5. Mandatory Conversion.

5.1 Trigger Events. Upon the earlier of (a) immediately prior to the closing of a firm commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, resulting in at least $50,000,000 of gross proceeds ("**Qualified Public Offering**") to the Corporation, or (b) the date and time, or the occurrence of an event, specified by vote or written consent of the holders of at least a majority of the then outstanding shares of Preferred Stock (voting together as a single class on an as-converted basis) (the time of such closing or the date and time specified or the time of the event specified in such vote or written consent is referred to herein as the "**Mandatory Conversion Time**"), then (i) all outstanding shares of Preferred Stock shall automatically be converted into shares of Common Stock, at the then effective conversion rate as calculated pursuant to Subsection 4.1.1. and (ii) such shares may not be reissued by the Corporation.

5.2 Procedural Requirements. All holders of record of shares of Preferred Stock shall be sent written notice of the Mandatory Conversion Time and the place designated for mandatory conversion of all such shares of Preferred Stock pursuant to this Section 5. Such notice need not be sent in advance of the occurrence of the Mandatory Conversion Time.

Upon receipt of such notice, each holder of shares of Preferred Stock in certificated form shall surrender his, her or its certificate or certificates for all such shares (or, if such holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation at the place designated in such notice. If so required by the Corporation, any certificates surrendered for conversion shall be endorsed or accompanied by written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or by his, her or its attorney duly authorized in writing. All rights with respect to the Preferred Stock converted pursuant to Subsection 5.1, including the rights, if any, to receive notices and vote (other than as a holder of Common Stock), will terminate at the Mandatory Conversion Time (notwithstanding the failure of the holder or holders thereof to surrender any certificates at or prior to such time), except only the rights of the holders thereof, upon surrender of any certificate or certificates of such holders (or lost certificate affidavit and agreement) therefor, to receive the items provided for in the next sentence of this Subsection 5.2. As soon as practicable after the Mandatory Conversion Time and, if applicable, the surrender of any certificate or certificates (or lost certificate affidavit and agreement) for Preferred Stock, the Corporation shall (a) issue and deliver to such holder, or to his, her or its nominees, a certificate or certificates for the number of full shares of Common Stock issuable on such conversion in accordance with the provisions hereof and (b)pay cash as provided in Subsection 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and the payment of any declared but unpaid dividends on the shares of Preferred Stock converted. Such converted Preferred Stock shall be retired and cancelled and may not be reissued as shares of such series, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of Preferred Stock accordingly.

6. Redemption. The Preferred Stock shall not be redeemable absent the mutual agreement of the Corporation and the holders thereof.

7. Redeemed or Otherwise Acquired Shares. Any shares of Preferred Stock that are redeemed or otherwise acquired by the Corporation or any of its subsidiaries shall be automatically and immediately cancelled and retired and shall not be reissued, sold or transferred. Neither the Corporation nor any of its subsidiaries may exercise any voting or other rights granted to the holders of Preferred Stock following redemption.

8. Waiver. Except as otherwise set forth herein, any of the rights, powers, preferences and other terms of the Preferred Stock set forth herein may be waived on behalf of all holders of Preferred Stock by the affirmative written consent or vote of the holders of at least a majority of the then-outstanding shares of Preferred Stock.

9. Notices. Any notice required or permitted by the provisions of this Article Fourth to be given to a holder of shares of Preferred Stock shall be mailed, postage prepaid, to the post office address last shown on the records of the Corporation, or given by electronic communication in compliance with the provisions of the General Corporation Law, and shall be deemed sent upon such mailing or electronic transmission.

FIFTH: Subject to any additional vote required by the Certificate of Incorporation or Bylaws, in furtherance and not in limitation of the powers conferred by statute, the Board of Directors is expressly authorized to make, repeal, alter, amend and rescind any or all of the Bylaws of the Corporation.

SIXTH: Subject to any additional vote required by the Certificate of Incorporation, the number of directors of the Corporation shall be determined in the manner set forth in the Bylaws of the Corporation.

SEVENTH: Elections of directors need not be by written ballot unless the Bylaws of the Corporation shall so provide.

EIGHTH: Meetings of stockholders may be held within or without the State of Delaware, as the Bylaws of the Corporation may provide. The books of the Corporation may be kept outside the State of Delaware at such place or places as may be designated from time to time by the Board of Directors or in the Bylaws of the Corporation.

NINTH: To the fullest extent permitted by law, a director of the Corporation shall not be personally liable to the Corporation or its stockholders for monetary damages for breach of fiduciary duty as a director. If the General Corporation Law or any other law of the State of Delaware is amended after approval by the stockholders of this Article Ninth to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of a director of the Corporation shall be eliminated or limited to the fullest extent permitted by the General Corporation Law as so amended.

Any repeal or modification of the foregoing provisions of this Article Ninth by the stockholders of the Corporation shall not adversely affect any right or protection of a director of the Corporation existing at the time of, or increase the liability of any director of the Corporation with respect to any acts or omissions of such director occurring prior to, such repeal or modification.

TENTH: To the fullest extent permitted by applicable law, the Corporation is authorized to provide indemnification of (and advancement of expenses to) directors, officers and agents of the Corporation (and any other persons to which General Corporation Law permits the Corporation to provide indemnification) through Bylaw provisions, agreements with such agents or other persons, vote of stockholders or disinterested directors or otherwise, in excess of the indemnification and advancement otherwise permitted by Section 145 of the General Corporation Law.

Any amendment, repeal or modification of the foregoing provisions of this Article Tenth shall not adversely affect any right or protection of any director, officer or other agent of the Corporation existing at the time of such amendment, repeal or modification.

ELEVENTH: The Corporation renounces, to the fullest extent permitted by law, any interest or expectancy of the Corporation in, or in being offered an opportunity to participate in, any Excluded Opportunity. An "**Excluded Opportunity**" is any matter, transaction or interest that is presented to, or acquired, created or developed by, or which otherwise comes into the possession of (i) any director of the Corporation who is not an employee of the Corporation or any of its subsidiaries, or (ii) any holder of Preferred Stock or any partner, member, director, stockholder, employee or agent of any such holder, other than someone who is an employee of the Corporation or any of its subsidiaries (collectively, "**Covered Persons**"), unless such matter, transaction or interest is presented to, or acquired, created or developed by, or otherwise comes into the possession of, a Covered Person expressly and solely in such Covered Person' s capacity as a director of the Corporation.

TWELFTH: For purposes of Section 500 of the California Corporations Code (to the extent applicable), in connection with any repurchase of shares of Common Stock permitted under this Certificate of Incorporation from employees, officers, directors or consultants of the Corporation in connection with a termination of employment or services pursuant to agreements or arrangements approved by the Board of Directors (in addition to any other consent required under this Certificate of Incorporation), such repurchase may be made without regard to any "preferential dividends arrears amount" or "preferential rights amount" (as those terms are defined in Section 500 of the California Corporations Code). Accordingly, for purposes of making any calculation under California Corporations Code Section 500 in connection with such repurchase, the amount of any "preferential dividends arrears amount" or "preferential rights amount" (as those terms are defined therein) shall be deemed to be zero (0).

* * *

3. That the foregoing amendment and restatement was approved by the holders of the requisite number of shares of this corporation in accordance with Section 228 of the General Corporation Law.

4. That this Certificate, which restates and integrates and further amends the provisions of this Corporation' s Certificate of Incorporation, has been duly adopted in accordance with Sections 242 and 245 of the General Corporation Law.

IN WITNESS WHEREOF, this Certificate has been executed by a duly authorized officer of this corporation on this 18th day of March, 2019.

By: /s/ Asa Abeliovich
Asa Abeliovich, President

[Signature Page to Third Amended and Restated Certificate of Incorporation]

CERTIFICATE OF AMENDMENT TO
THIRD AMENDED AND RESTATED CERTIFICATE OF INCORPORATION OF PREVAIL THERAPEUTICS INC.

Prevail Therapeutics Inc. (the "***Corporation***"), a corporation organized and existing under and by virtue of the General Corporation Law of the State of Delaware (the "***DGCL***"), does hereby certify that:

ONE: The name of the Corporation is Prevail Therapeutics Inc.

TWO: The date of filing the original Certificate of Incorporation of this corporation with the Secretary of State of the State of Delaware was July 6, 2017.

THREE: The first paragraph of Article FOURTH of the Third Amended and Restated Certificate of Incorporation of the Corporation is hereby amended and restated in its entirety as follows:

"The total number of shares of all classes of stock which the Corporation shall have authority to issue is (i) 36,000,000 shares of Common Stock, $0.0001 par value per share ("**Common Stock**") and (ii) 20,000,000 shares of Preferred Stock, $0.0001 par value per share ("**Preferred Stock**").

Effective at the time of filing of this Certificate of Amendment to Third Amended and Restated Certificate of Incorporation with the Secretary of State of the State of Delaware, every one share of Common Stock issued and outstanding shall, automatically and without any action on the part of the respective holders thereof, be converted into 1.62 shares of Common Stock without increasing or decreasing the par value of each share of Common Stock (the "**Forward Split**"); *provided, however,* that the Corporation shall issue no fractional shares of Common Stock as a result of the Forward Split, but shall instead pay to any stockholder who would be entitled to receive a fractional share as a result of the actions set forth herein a sum in cash equal to the fair market value of the shares constituting such fractional share as determined by the Board of Directors of the Corporation. The Forward Split shall occur whether or not the certificates representing such shares of Common Stock are surrendered to the Corporation or its transfer agent. The Forward Split shall be effected on a record holder-by-record holder basis, such that any fractional shares of Common Stock resulting from the Forward Split and held by a single record holder shall be aggregated.

All of the outstanding share amounts, amounts per share and per share numbers for the Common Stock and each series of Preferred Stock set forth in the Certificate shall be appropriately adjusted to give effect to the Forward Stock Split, as applicable."

FOUR: This Certificate of Amendment to Third Amended and Restated Certificate of Incorporation has been duly approved by the Board of Directors of the Corporation, acting in accordance with the provisions of Sections 141 and 242 of the DGCL.

FIVE: This Certificate of Amendment to Third Amended and Restated Certificate of Incorporation was approved by the holders of the requisite number of shares of the Corporation in accordance with Sections 228 and 242 of the DGCL.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, Prevail Therapeutics Inc. has caused this Certificate of Amendment to Third Amended and Restated Certificate of Incorporation to be signed by its President and Chief Executive Officer this 7th day of June, 2019.

PREVAIL THERAPEUTICS INC.

/s/ Asa Abeliovich, M.D., Ph.D.
Asa Abeliovich, M.D., Ph.D.
President and Chief Executive Officer

Exhibit 5.1

Cooley

Divakar Gupta
+1 212 479 6474
dgupta@cooley.com

June 10, 2019

Prevail Therapeutics Inc.
430 East 29th Street, Suite 940
New York, NY 10016

Ladies and Gentlemen:

We have represented Prevail Therapeutics Inc., a Delaware corporation (the "***Company***"), in connection with the filing by the Company of a Registration Statement (No. 333-231754) on Form S-1 (the "***Registration Statement***") with the Securities and Exchange Commission, including a related prospectus filed with the Registration Statement (the "***Prospectus***"), covering an underwritten public offering of up to 8,455,950 shares of the Company' s common stock, par value $0.0001 per share (the "***Shares***"), which includes up to 1,102,950 shares that may be sold pursuant to the exercise of an option to purchase additional shares.

In connection with this opinion, we have (i) examined and relied upon (a) the Registration Statement and Prospectus, (b) the Company' s Third Amended and Restated Certificate of Incorporation, as amended, and Amended and Restated Bylaws, each as currently in effect, (c) the forms of the Company' s Fourth Amended and Restated Certificate of Incorporation and the Company' s Amended and Restated Bylaws, filed as Exhibits 3.3 and 3.4 to the Registration Statement, respectively, each of which is to be in effect prior to the closing of the offering contemplated by the Registration Statement and (d) originals or copies certified to our satisfaction of such records, documents, certificates, memoranda and other instruments as in our judgment are necessary or appropriate to enable us to render the opinion expressed below, and (ii) assumed that the Shares will be sold at a price established by the Board of Directors of the Company, or a duly authorized committee thereof, and that the Fourth Amended and Restated Certificate of Incorporation referred to in clause (i)(c) is filed with the Secretary of State of the State of Delaware before issuance of the Shares. We have undertaken no independent verification with respect to such matters. We have assumed the genuineness and authenticity of all documents submitted to us as originals, and the conformity to originals of all documents, other than by the Company, submitted to us as copies and the due execution and delivery of all documents where due execution and delivery are a prerequisite to the effectiveness thereof. As to certain factual matters, we have relied upon a certificate of an officer of the Company and have not sought independently to verify such matters.

Our opinion is expressed only with respect to the General Corporation Law of the State of Delaware. We express no opinion to the extent that any other laws are applicable to the subject matter hereof and express no opinion and provide no assurance as to compliance with any federal or state securities law, rule or regulation.

On the basis of the foregoing, and in reliance thereon, we are of the opinion that the Shares, when sold and issued against payment therefor as described in the Registration Statement and the Prospectus, will be validly issued, fully paid and non-assessable.

We consent to the reference to our firm under the caption "Legal Matters" in the Prospectus included in the Registration Statement and to the filing of this opinion as an exhibit to the Registration Statement.

Sincerely,

Cooley LLP

By: /s/ Divakar Gupta
Divakar Gupta

Cooley LLP 55 Hudson Yards New York, NY 10001
t: (212) 479-6000 cooley.com

Exhibit 10.4

PREVAIL THERAPEUTICS, INC.

2019 EQUITY INCENTIVE PLAN

ADOPTED BY THE BOARD OF DIRECTORS: JUNE 6, 2019
APPROVED BY THE STOCKHOLDERS: JUNE 6, 2019
IPO DATE: JUNE , 2019

1. GENERAL.

(a) Successor to and Continuation of Prior Plan. The Plan is intended as the successor to and continuation of the Company' s 2017 Equity Incentive Plan (the "***Prior Plan***"). From and after 12:01 a.m. Eastern time on the IPO Date, no additional awards will be granted under the Prior Plan. All Awards granted on or after 12:01 a.m. Eastern Time on the IPO Date will be granted under this Plan. All awards granted under the Prior Plan will remain subject to the terms of the Prior Plan.

(i) Any shares that would otherwise remain available for future grants under the Prior Plan as of 12:01 a.m. Eastern Time on the IPO Date (the "***Prior Plan's Available Reserve***") will cease to be available under the Prior Plan at such time. Instead, that number of shares of Common Stock equal to the Prior Plan' s Available Reserve will be added to the Share Reserve (as further described in Section 3(a) below) and will be immediately available for grants and issuance pursuant to Stock Awards hereunder, up to the maximum number set forth in Section 3(a) below.

(ii) In addition, from and after 12:01 a.m. Eastern time on the IPO Date, any shares subject, at such time, to outstanding stock awards granted under the Prior Plan that (i) expire or terminate for any reason prior to exercise or settlement; (ii) are forfeited because of the failure to meet a contingency or condition required to vest such shares or otherwise return to the Company; or (iii) are reacquired, withheld (or not issued) to satisfy a tax withholding obligation in connection with an award or to satisfy the purchase price or exercise price of a stock award (such shares the "***Returning Shares***") will immediately be added to the Share Reserve (as further described in Section 3(a) below) as and when such shares become Returning Shares, up to the maximum number set forth in Section 3(a) below.

(b) Eligible Award Recipients. Employees, Directors and Consultants are eligible to receive Awards.

(c) Available Awards. The Plan provides for the grant of the following Awards: (i) Incentive Stock Options, (ii) Nonstatutory Stock Options, (iii) Stock Appreciation Rights, (iv) Restricted Stock Awards, (v) Restricted Stock Unit Awards, (vi) Performance Stock Awards, (vii) Performance Cash Awards, and (viii) Other Stock Awards.

(d) Purpose. The Plan, through the grant of Awards, is intended to help the Company secure and retain the services of eligible award recipients, provide incentives for such persons to exert maximum efforts for the success of the Company and any Affiliate, and provide a means by which the eligible recipients may benefit from increases in value of the Common Stock.

2. ADMINISTRATION.

(a) Administration by Board. The Board will administer the Plan. The Board may delegate administration of the Plan to a Committee or Committees, as provided in Section 2(c).

(b) Powers of Board. The Board will have the power, subject to, and within the limitations of, the express provisions of the Plan:

(i) To determine: (A) who will be granted Awards; (B) when and how each Award will be granted; (C) what type of Award will be granted; (D) the provisions of each Award (which need not be identical), including when a person will be permitted to exercise or otherwise receive cash or Common Stock under the Award; (E) the number of shares of Common Stock subject to, or the cash value of, an Award; and (F) the Fair Market Value applicable to a Stock Award.

(ii) To construe and interpret the Plan and Awards granted under it, and to establish, amend and revoke rules and regulations for administration of the Plan and Awards. The Board, in the exercise of these powers, may correct any defect, omission or inconsistency in the Plan or in any Award Agreement or in the written terms of a Performance Cash Award, in a manner and to the extent it will deem necessary or expedient to make the Plan or Award fully effective.

(iii) To settle all controversies regarding the Plan and Awards granted under it.

(iv) To accelerate, in whole or in part, the time at which an Award may be exercised or vest (or the time at which cash or shares of Common Stock may be issued in settlement thereof).

(v) To suspend or terminate the Plan at any time. Except as otherwise provided in the Plan or an Award Agreement, suspension or termination of the Plan will not materially impair a Participant' s rights under the Participant' s then-outstanding Award without the Participant' s written consent, except as provided in subsection (viii) below.

(vi) To amend the Plan in any respect the Board deems necessary or advisable, including, without limitation, by adopting amendments relating to Incentive Stock Options and certain nonqualified deferred compensation under Section 409A of the Code and/or bringing the Plan or Awards granted under the Plan into compliance with the requirements for Incentive Stock Options or ensuring that they are exempt from, or compliant with, the requirements for nonqualified deferred compensation under Section 409A of the Code, subject to the limitations, if any, of applicable law. If required by applicable law or listing requirements, and except as provided in Section 9(a) relating to Capitalization Adjustments, the Company will seek stockholder approval of any amendment of the Plan that (A) materially increases the number of shares of Common Stock available for issuance under the Plan, (B) materially expands the class of individuals eligible to receive Awards under the Plan, (C) materially increases the benefits accruing to Participants under the Plan, (D) materially reduces the price at which shares of

Common Stock may be issued or purchased under the Plan, (E) materially extends the term of the Plan, or (F) materially expands the types of Awards available for issuance under the Plan. Except as otherwise provided in the Plan or an Award Agreement, no amendment of the Plan will materially impair a Participant' s rights under an outstanding Award without the Participant' s written consent.

(vii) To submit any amendment to the Plan for stockholder approval, including, but not limited to, amendments to the Plan intended to satisfy the requirements of (A) Section 422 of the Code regarding "incentive stock options" or (B) Rule 16b-3.

(viii) To approve forms of Award Agreements for use under the Plan and to amend the terms of any one or more Awards, including, but not limited to, amendments to provide terms more favorable to the Participant than previously provided in the Award Agreement, subject to any specified limits in the Plan that are not subject to Board discretion; *provided, however,* that a Participant' s rights under any Award will not be impaired by any such amendment unless (A) the Company requests the consent of the affected Participant, and (B) such Participant consents in writing. Notwithstanding the foregoing, (1) a Participant' s rights will not be deemed to have been impaired by any such amendment if the Board, in its sole discretion, determines that the amendment, taken as a whole, does not materially impair the Participant' s rights, and (2) subject to the limitations of applicable law, if any, the Board may amend the terms of any one or more Awards without the affected Participant' s consent (A) to maintain the qualified status of the Award as an Incentive Stock Option under Section 422 of the Code; (B) to change the terms of an Incentive Stock Option, if such change results in impairment of the Award solely because it impairs the qualified status of the Award as an Incentive Stock Option under Section 422 of the Code; (C) to clarify the manner of exemption from, or to bring the Award into compliance with, Section 409A of the Code; or (D) to comply with other applicable laws or listing requirements.

(ix) Generally, to exercise such powers and to perform such acts as the Board deems necessary or expedient to promote the best interests of the Company and that are not in conflict with the provisions of the Plan or Awards.

(x) To adopt such procedures and sub-plans as are necessary or appropriate to permit participation in the Plan by Employees, Directors or Consultants who are foreign nationals or employed outside the United States (provided that Board approval will not be necessary for immaterial modifications to the Plan or any Award Agreement that are required for compliance with the laws of the relevant foreign jurisdiction).

(xi) To effect, with the consent of any adversely affected Participant, (A) the reduction of the exercise, purchase or strike price of any outstanding Stock Award; (B) the cancellation of any outstanding Stock Award and the grant in substitution therefor of a new (1) Option or SAR, (2) Restricted Stock Award, (3) Restricted Stock Unit Award, (4) Other Stock Award, (5) cash and/or (6) other valuable consideration determined by the Board, in its sole discretion, with any such substituted award (x) covering the same or a different number of shares of Common Stock as the cancelled Stock Award and (y) granted under the Plan or another equity or compensatory plan of the Company; or (C) any other action that is treated as a repricing under generally accepted accounting principles.

(c) Delegation to Committee.

(i) General. The Board may delegate some or all of the administration of the Plan to a Committee or Committees. If administration of the Plan is delegated to a Committee, the Committee will have, in connection with the administration of the Plan, the powers theretofore possessed by the Board that have been delegated to the Committee, including the power to delegate to a subcommittee of the Committee any of the administrative powers the Committee is authorized to exercise (and references in this Plan to the Board will thereafter be construed as being to the Committee or subcommittee, as applicable). Any delegation of administrative powers will be reflected in resolutions, not inconsistent with the provisions of the Plan, adopted from time to time by the Board or Committee (as applicable). The Board may retain the authority to concurrently administer the Plan with the Committee and may, at any time, revest in the Board some or all of the powers previously delegated.

(ii) Rule 16b-3 Compliance. The Committee may consist solely of two or more Non-Employee Directors in accordance with Rule 16b-3.

(d) Delegation to an Officer. The Board may delegate to one (1) or more Officers the authority to do one or both of the following (i) designate Employees who are not Officers to be recipients of Options and SARs (and, to the extent permitted by applicable law, other Stock Awards) and, to the extent permitted by applicable law, the terms of such Awards, and (ii) determine the number of shares of Common Stock to be subject to such Stock Awards granted to such Employees; *provided, however*, that the Board resolutions regarding such delegation will specify the total number of shares of Common Stock that may be subject to the Stock Awards granted by such Officer and that such Officer may not grant a Stock Award to himself or herself. Any such Stock Awards will be granted on the form of Stock Award Agreement most recently approved for use by the Committee or the Board, unless otherwise provided in the resolutions approving the delegation authority. The Board may not delegate authority to an Officer who is acting solely in the capacity of an Officer (and not also as a Director) to determine the Fair Market Value pursuant to Section 13(w)(iii) below.

(e) Effect of Board' s Decision. All determinations, interpretations and constructions made by the Board in good faith will not be subject to review by any person and will be final, binding and conclusive on all persons.

3. SHARES SUBJECT TO THE PLAN.

(a) Share Reserve. Subject to Section 9(a) relating to Capitalization Adjustments, and the following sentence regarding the annual increase, the aggregate number of shares of Common Stock that may be issued pursuant to Stock Awards will not exceed 8,579,715 shares (the "***Share Reserve***"), which number is the sum of (i) 2,550,000 new shares, *plus* (ii) the number of shares subject to the Prior Plan' s Available Reserve, *plus* (iii) the number of shares that are Returning Shares, as such shares become available from time to time.

In addition, the Share Reserve will automatically increase on January 1st of each year, for a period of not more than ten years, commencing on January 1st of the year following the year in which the IPO Date occurs and ending on (and including) January 1, 2029, in an amount equal to

4% of the total number of shares of Capital Stock outstanding on December 31st of the preceding calendar year. Notwithstanding the foregoing, the Board may act prior to January 1st of a given year to provide that there will be no January 1st increase in the Share Reserve for such year or that the increase in the Share Reserve for such year will be a lesser number of shares of Common Stock than would otherwise occur pursuant to the preceding sentence.

For clarity, the Share Reserve in this Section 3(a) is a limitation on the number of shares of Common Stock that may be issued pursuant to the Plan. Accordingly, this Section 3(a) does not limit the granting of Stock Awards except as provided in Section 7(a). Shares may be issued in connection with a merger or acquisition as permitted by NASDAQ Listing Rule 5635(c) or, if applicable, NYSE Listed Company Manual Section 303A.08, AMEX Company Guide Section 711 or other applicable rule, and such issuance will not reduce the number of shares available for issuance under the Plan.

(b) Reversion of Shares to the Share Reserve. If a Stock Award or any portion thereof (i) expires or otherwise terminates without all of the shares covered by such Stock Award having been issued or (ii) is settled in cash (*i.e.*, the Participant receives cash rather than stock), such expiration, termination or settlement will not reduce (or otherwise offset) the number of shares of Common Stock that may be available for issuance under the Plan. If any shares of Common Stock issued pursuant to a Stock Award are forfeited back to or repurchased by the Company because of the failure to meet a contingency or condition required to vest such shares in the Participant, then the shares that are forfeited or repurchased will revert to and again become available for issuance under the Plan. Any shares reacquired by the Company in satisfaction of tax withholding obligations on a Stock Award or as consideration for the exercise or purchase price of a Stock Award will again become available for issuance under the Plan.

(c) Incentive Stock Option Limit. Subject to the provisions of Section 9(a) relating to Capitalization Adjustments, the aggregate maximum number of shares of Common Stock that may be issued pursuant to the exercise of Incentive Stock Options will be 26,000,000 shares of Common Stock.

(d) Limitation on Grants to Non-Employee Directors. The maximum number of shares of Common Stock subject to Stock Awards granted under the Plan or otherwise during any one calendar year to any Non-Employee Director, taken together with any cash fees paid by the Company to such Non-Employee Director during such calendar year for service on the Board, will not exceed $600,000 in total value (calculating the value of any such Stock Awards based on the grant date fair value of such Stock Awards for financial reporting purposes), or, with respect to the calendar year in which a Non-Employee Director is first appointed or elected to the Board, $900,000.

(e) Source of Shares. The stock issuable under the Plan will be shares of authorized but unissued or reacquired Common Stock, including shares repurchased by the Company on the open market or otherwise.

4. ELIGIBILITY.

(a) Eligibility for Specific Stock Awards. Incentive Stock Options may be granted only to employees of the Company or a "parent corporation" or "subsidiary corporation" thereof (as such terms are defined in Sections 424(e) and 424(f) of the Code). Stock Awards other than Incentive Stock Options may be granted to Employees, Directors and Consultants; *provided, however*, that Stock Awards may not be granted to Employees, Directors and Consultants who are providing Continuous Service only to any "parent" of the Company, as such term is defined in Rule 405 of the Securities Act, unless (i) the stock underlying such Stock Awards is treated as "service recipient stock" under Section 409A of the Code (for example, because the Stock Awards are granted pursuant to a corporate transaction such as a spin off transaction), (ii) the Company, in consultation with its legal counsel, has determined that such Stock Awards are otherwise exempt from Section 409A of the Code, or (iii) the Company, in consultation with its legal counsel, has determined that such Stock Awards comply with the distribution requirements of Section 409A of the Code.

(b) Ten Percent Stockholders. A Ten Percent Stockholder will not be granted an Incentive Stock Option unless the exercise price of such Option is at least 110% of the Fair Market Value on the date of grant and the Option is not exercisable after the expiration of five years from the date of grant.

5. PROVISIONS RELATING TO OPTIONS AND STOCK APPRECIATION RIGHTS.

Each Option or SAR will be in such form and will contain such terms and conditions as the Board deems appropriate. All Options will be separately designated Incentive Stock Options or Nonstatutory Stock Options at the time of grant, and, if certificates are issued, a separate certificate or certificates will be issued for shares of Common Stock purchased on exercise of each type of Option. If an Option is not specifically designated as an Incentive Stock Option, or if an Option is designated as an Incentive Stock Option but some portion or all of the Option fails to qualify as an Incentive Stock Option under the applicable rules, then the Option (or portion thereof) will be a Nonstatutory Stock Option. The provisions of separate Options or SARs need not be identical; *provided, however*, that each Award Agreement will conform to (through incorporation of provisions hereof by reference in the applicable Award Agreement or otherwise) the substance of each of the following provisions:

(a) Term. Subject to the provisions of Section 4(b) regarding Ten Percent Stockholders, no Option or SAR will be exercisable after the expiration of ten years from the date of its grant or such shorter period specified in the Award Agreement.

(b) Exercise Price. Subject to the provisions of Section 4(b) regarding Ten Percent Stockholders, the exercise or strike price of each Option or SAR will be not less than 100% of the Fair Market Value of the Common Stock subject to the Option or SAR on the date the Award is granted. Notwithstanding the foregoing, an Option or SAR may be granted with an exercise or strike price lower than 100% of the Fair Market Value of the Common Stock subject to the Award if such Award is granted pursuant to an assumption of or substitution for another option or stock appreciation right pursuant to a Corporate Transaction and in a manner consistent with the provisions of Section 409A of the Code and, if applicable, Section 424(a) of the Code. Each SAR will be denominated in shares of Common Stock equivalents.

(c) Purchase Price for Options. The purchase price of Common Stock acquired pursuant to the exercise of an Option may be paid, to the extent permitted by applicable law and as determined by the Board in its sole discretion, by any combination of the methods of payment set forth below. The Board will have the authority to grant Options that do not permit all of the following methods of payment (or otherwise restrict the ability to use certain methods) and to grant Options that require the consent of the Company to use a particular method of payment. The permitted methods of payment are as follows:

(i) by cash, check, bank draft or money order payable to the Company;

(ii) pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of the stock subject to the Option, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds;

(iii) by delivery to the Company (either by actual delivery or attestation) of shares of Common Stock;

(iv) if an Option is a Nonstatutory Stock Option, by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issuable upon exercise by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price; *provided, however*, that the Company will accept a cash or other payment from the Participant to the extent of any remaining balance of the aggregate exercise price not satisfied by such reduction in the number of whole shares to be issued. Shares of Common Stock will no longer be subject to an Option and will not be exercisable thereafter to the extent that (A) shares issuable upon exercise are used to pay the exercise price pursuant to the "net exercise," (B) shares are delivered to the Participant as a result of such exercise, and (C) shares are withheld to satisfy tax withholding obligations; or

(v) in any other form of legal consideration that may be acceptable to the Board and specified in the applicable Award Agreement.

(d) Exercise and Payment of a SAR. To exercise any outstanding SAR, the Participant must provide written notice of exercise to the Company in compliance with the provisions of the Stock Appreciation Right Agreement evidencing such SAR. The appreciation distribution payable on the exercise of a SAR will be not greater than an amount equal to the excess of (A) the aggregate Fair Market Value (on the date of the exercise of the SAR) of a number of shares of Common Stock equal to the number of Common Stock equivalents in which the Participant is vested under such SAR, and with respect to which the Participant is exercising the SAR on such date, over (B) the aggregate strike price of the number of Common Stock equivalents with respect to which the Participant is exercising the SAR on such date. The appreciation distribution may be paid in Common Stock, in cash, in any combination of the two or in any other form of consideration, as determined by the Board and contained in the Award Agreement evidencing such SAR.

(e) Transferability of Options and SARs. The Board may, in its sole discretion, impose such limitations on the transferability of Options and SARs as the Board will determine. In the absence of such a determination by the Board to the contrary, the following restrictions on the transferability of Options and SARs will apply:

(i) Restrictions on Transfer. An Option or SAR will not be transferable except by will or by the laws of descent and distribution (or pursuant to subsections (ii) and (iii) below), and will be exercisable during the lifetime of the Participant only by the Participant. The Board may permit transfer of the Option or SAR in a manner that is not prohibited by applicable tax and securities laws. Except as explicitly provided in the Plan, neither an Option nor a SAR may be transferred for consideration.

(ii) Domestic Relations Orders. Subject to the approval of the Board or a duly authorized Officer, an Option or SAR may be transferred pursuant to the terms of a domestic relations order, official marital settlement agreement or other divorce or separation instrument as permitted by Treasury Regulations Section 1.421-1(b)(2). If an Option is an Incentive Stock Option, such Option may be deemed to be a Nonstatutory Stock Option as a result of such transfer.

(iii) Beneficiary Designation. Subject to the approval of the Board or a duly authorized Officer, a Participant may, by delivering written notice to the Company, in a form approved by the Company (or the designated broker), designate a third party who, on the death of the Participant, will thereafter be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, upon the death of the Participant, the executor or administrator of the Participant' s estate will be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. However, the Company may prohibit designation of a beneficiary at any time, including due to any conclusion by the Company that such designation would be inconsistent with the provisions of applicable laws.

(f) Vesting Generally. The total number of shares of Common Stock subject to an Option or SAR may vest and become exercisable in periodic installments that may or may not be equal. The Option or SAR may be subject to such other terms and conditions on the time or times when it may or may not be exercised (which may be based on the satisfaction of Performance Goals or other criteria) as the Board may deem appropriate. The vesting provisions of individual Options or SARs may vary. The provisions of this Section 5(f) are subject to any Option or SAR provisions governing the minimum number of shares of Common Stock as to which an Option or SAR may be exercised.

(g) Termination of Continuous Service. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if a Participant' s Continuous Service terminates (other than for Cause and other than upon the Participant' s death or Disability), the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Award as of the date of termination of Continuous Service) within the period of time ending on the earlier of (i) the date that is 90 days following the termination of the Participant' s Continuous Service (or such longer or shorter period specified in the applicable Award Agreement), and (ii) the expiration of the term of the

Option or SAR as set forth in the Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR (as applicable) within the applicable time frame, the Option or SAR will terminate.

(h) Extension of Termination Date. If the exercise of an Option or SAR following the termination of the Participant' s Continuous Service (other than for Cause and other than upon the Participant' s death or Disability) would be prohibited at any time solely because the issuance of shares of Common Stock would violate the registration requirements under the Securities Act, then the Option or SAR will terminate on the earlier of (i) the expiration of a total period of time (that need not be consecutive) equal to the applicable post termination exercise period after the termination of the Participant' s Continuous Service during which the exercise of the Option or SAR would not be in violation of such registration requirements, and (ii) the expiration of the term of the Option or SAR as set forth in the applicable Award Agreement. In addition, unless otherwise provided in a Participant' s Award Agreement, if the sale of any Common Stock received on exercise of an Option or SAR following the termination of the Participant' s Continuous Service (other than for Cause) would violate the Company' s insider trading policy, then the Option or SAR will terminate on the earlier of (i) the expiration of a period of months (that need not be consecutive) equal to the applicable post-termination exercise period after the termination of the Participant' s Continuous Service during which the sale of the Common Stock received upon exercise of the Option or SAR would not be in violation of the Company' s insider trading policy, or (ii) the expiration of the term of the Option or SAR as set forth in the applicable Award Agreement.

(i) Disability of Participant. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if a Participant' s Continuous Service terminates as a result of the Participant' s Disability, the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Option or SAR as of the date of termination of Continuous Service), but only within such period of time ending on the earlier of (i) the date 12 months following such termination of Continuous Service (or such longer or shorter period specified in the Award Agreement), and (ii) the expiration of the term of the Option or SAR as set forth in the Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR within the applicable time frame, the Option or SAR (as applicable) will terminate.

(j) Death of Participant. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if (i) a Participant' s Continuous Service terminates as a result of the Participant' s death, or (ii) the Participant dies within the period (if any) specified in the Award Agreement for exercisability after the termination of the Participant' s Continuous Service for a reason other than death, then the Option or SAR may be exercised (to the extent the Participant was entitled to exercise such Option or SAR as of the date of death) by the Participant' s estate, by a person who acquired the right to exercise the Option or SAR by bequest or inheritance or by a person designated to exercise the Option or SAR upon the Participant' s death, but only within the period ending on the earlier of (i) the date 18 months following the date of death (or such longer or shorter period specified in the Award Agreement), and (ii) the expiration of the term of such Option or SAR as set forth in the Award Agreement. If, after the Participant' s death, the Option or SAR is not exercised within the applicable time frame, the Option or SAR (as applicable) will terminate.

(k) Termination for Cause. Except as explicitly provided otherwise in a Participant' s Award Agreement or other individual written agreement between the Company and the Participant, if a Participant' s Continuous Service is terminated for Cause, the Option or SAR will terminate immediately upon such Participant' s termination of Continuous Service, and the Participant will be prohibited from exercising his or her Option or SAR from and after the time of such termination of Continuous Service.

(l) Non-Exempt Employees. If an Option or SAR is granted to an Employee who is a non-exempt employee for purposes of the Fair Labor Standards Act of 1938, as amended, the Option or SAR will not be first exercisable for any shares of Common Stock until at least six months following the date of grant of the Option or SAR (although the Award may vest prior to such date). Consistent with the provisions of the Worker Economic Opportunity Act, (i) if such non-exempt Employee dies or suffers a Disability, (ii) upon a Corporate Transaction in which such Option or SAR is not assumed, continued, or substituted, (iii) upon a Change in Control, or (iv) upon the Participant' s retirement (as such term may be defined in the Participant' s Award Agreement in another agreement between the Participant and the Company, or, if no such definition, in accordance with the Company' s then current employment policies and guidelines), the vested portion of any Options and SARs may be exercised earlier than six months following the date of grant. The foregoing provision is intended to operate so that any income derived by a non-exempt employee in connection with the exercise or vesting of an Option or SAR will be exempt from his or her regular rate of pay. To the extent permitted and/or required for compliance with the Worker Economic Opportunity Act to ensure that any income derived by a non-exempt employee in connection with the exercise, vesting or issuance of any shares under any other Stock Award will be exempt from the employee' s regular rate of pay, the provisions of this Section 5(l) will apply to all Stock Awards and are hereby incorporated by reference into such Stock Award Agreements.

6. PROVISIONS OF STOCK AWARDS OTHER THAN OPTIONS AND SARS.

(a) Restricted Stock Awards. Each Restricted Stock Award Agreement will be in such form and will contain such terms and conditions as the Board will deem appropriate. To the extent consistent with the Company' s bylaws, at the Board' s election, shares of Common Stock may be (x) held in book entry form subject to the Company' s instructions until any restrictions relating to the Restricted Stock Award lapse; or (y) evidenced by a certificate, which certificate will be held in such form and manner as determined by the Board. The terms and conditions of Restricted Stock Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Award Agreements need not be identical. Each Restricted Stock Award Agreement will conform to (through incorporation of the provisions hereof by reference in the agreement or otherwise) the substance of each of the following provisions:

(i) Consideration. A Restricted Stock Award may be awarded in consideration for (A) cash, check, bank draft or money order payable to the Company, (B) past or future services to the Company or an Affiliate, or (C) any other form of legal consideration that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) Vesting. Shares of Common Stock awarded under the Restricted Stock Award Agreement may be subject to forfeiture to the Company in accordance with a vesting schedule to be determined by the Board.

(iii) Termination of Participant' s Continuous Service. If a Participant' s Continuous Service terminates, the Company may receive through a forfeiture condition or a repurchase right any or all of the shares of Common Stock held by the Participant that have not vested as of the date of termination of Continuous Service under the terms of the Restricted Stock Award Agreement.

(iv) Transferability. Rights to acquire shares of Common Stock under the Restricted Stock Award Agreement will be transferable by the Participant only upon such terms and conditions as are set forth in the Restricted Stock Award Agreement, as the Board will determine in its sole discretion, so long as Common Stock awarded under the Restricted Stock Award Agreement remains subject to the terms of the Restricted Stock Award Agreement.

(v) Dividends. A Restricted Stock Award Agreement may provide that any dividends paid on Restricted Stock will be subject to the same vesting and forfeiture restrictions as apply to the shares subject to the Restricted Stock Award to which they relate.

(b) Restricted Stock Unit Awards. Each Restricted Stock Unit Award Agreement will be in such form and will contain such terms and conditions as the Board will deem appropriate. The terms and conditions of Restricted Stock Unit Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Unit Award Agreements need not be identical. Each Restricted Stock Unit Award Agreement will conform to (through incorporation of the provisions hereof by reference in the Agreement or otherwise) the substance of each of the following provisions:

(i) Consideration. At the time of grant of a Restricted Stock Unit Award, the Board will determine the consideration, if any, to be paid by the Participant upon delivery of each share of Common Stock subject to the Restricted Stock Unit Award. The consideration to be paid (if any) by the Participant for each share of Common Stock subject to a Restricted Stock Unit Award may be paid in any form of legal consideration that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) Vesting. At the time of the grant of a Restricted Stock Unit Award, the Board may impose such restrictions on or conditions to the vesting of the Restricted Stock Unit Award as it, in its sole discretion, deems appropriate.

(iii) Payment. A Restricted Stock Unit Award may be settled by the delivery of shares of Common Stock, their cash equivalent, any combination thereof or in any other form of consideration, as determined by the Board and contained in the Restricted Stock Unit Award Agreement.

(iv) Additional Restrictions. At the time of the grant of a Restricted Stock Unit Award, the Board, as it deems appropriate, may impose such restrictions or conditions that delay the delivery of the shares of Common Stock (or their cash equivalent) subject to a Restricted Stock Unit Award to a time after the vesting of such Restricted Stock Unit Award.

(v) Dividend Equivalents. Dividend equivalents may be credited in respect of shares of Common Stock covered by a Restricted Stock Unit Award, as determined by the Board and contained in the Restricted Stock Unit Award Agreement. At the sole discretion of the Board, such dividend equivalents may be converted into additional shares of Common Stock covered by the Restricted Stock Unit Award in such manner as determined by the Board. Any additional shares covered by the Restricted Stock Unit Award credited by reason of such dividend equivalents will be subject to all of the same terms and conditions of the underlying Restricted Stock Unit Award Agreement to which they relate.

(vi) Termination of Participant' s Continuous Service. Except as otherwise provided in the applicable Restricted Stock Unit Award Agreement, such portion of the Restricted Stock Unit Award that has not vested will be forfeited upon the Participant' s termination of Continuous Service.

(c) Performance Awards.

(i) Performance Stock Awards. A Performance Stock Award is a Stock Award that is payable (including that may be granted, may vest or may be exercised) contingent upon the attainment during a Performance Period of certain Performance Goals. A Performance Stock Award may, but need not, require the Participant' s completion of a specified period of Continuous Service. The length of any Performance Period, the Performance Goals to be achieved during the Performance Period, and the measure of whether and to what degree such Performance Goals have been attained will be conclusively determined by the Board or Committee, in its sole discretion. In addition, to the extent permitted by applicable law and the applicable Award Agreement, the Board (or Committee, as the case may be) may determine that cash may be used in payment of Performance Stock Awards.

(ii) Performance Cash Awards. A Performance Cash Award is a cash award that is payable contingent upon the attainment during a Performance Period of certain Performance Goals. A Performance Cash Award may also require the completion of a specified period of Continuous Service. At the time of grant of a Performance Cash Award, the length of any Performance Period, the Performance Goals to be achieved during the Performance Period, and the measure of whether and to what degree such Performance Goals have been attained will be conclusively determined by the Board or Committee, in its sole discretion. The Board (or Committee, as the case may be) may specify the form of payment of Performance Cash Awards, which may be cash or other property, or may provide for a Participant to have the option for his or her Performance Cash Award, or such portion thereof as the Board (or Committee, as the case may be) may specify, to be paid in whole or in part in cash or other property.

(iii) Board Discretion. The Board (or Committee, as the case may be) retains the discretion to adjust or eliminate the compensation or economic benefit due upon attainment of Performance Goals and to define the manner of calculating the Performance Criteria it selects to use for a Performance Period. Partial achievement of the specified criteria may result in the payment or vesting corresponding to the degree of achievement as specified in the Stock Award Agreement or the written terms of a Performance Cash Award.

(d) Other Stock Awards. Other forms of Stock Awards valued in whole or in part by reference to, or otherwise based on, Common Stock, including the appreciation in value thereof (e.g., options or stock rights with an exercise price or strike price less than 100% of the Fair Market Value of the Common Stock at the time of grant) may be granted either alone or in addition to Stock Awards provided for under Section 5 and the preceding provisions of this Section 6. Subject to the provisions of the Plan, the Board will have sole and complete authority to determine the persons to whom and the time or times at which such Other Stock Awards will be granted, the number of shares of Common Stock (or the cash equivalent thereof) to be granted pursuant to such Other Stock Awards and all other terms and conditions of such Other Stock Awards.

7. COVENANTS OF THE COMPANY.

(a) Availability of Shares. The Company will keep available at all times the number of shares of Common Stock reasonably required to satisfy then-outstanding Awards.

(b) Securities Law Compliance. The Company will seek to obtain from each regulatory commission or agency, as necessary, such authority as may be required to grant Stock Awards and to issue and sell shares of Common Stock upon exercise or vesting of the Stock Awards; *provided, however*, that this undertaking will not require the Company to register under the Securities Act or other securities or applicable laws, the Plan, any Stock Award or any Common Stock issued or issuable pursuant to any such Stock Award. If, after reasonable efforts and at a reasonable cost, the Company is unable to obtain from any such regulatory commission or agency the authority that counsel for the Company deems necessary or advisable for the lawful issuance and sale of Common Stock under the Plan, the Company will be relieved from any liability for failure to issue and sell Common Stock upon exercise or vesting of such Stock Awards unless and until such authority is obtained. A Participant will not be eligible for the grant of an Award or the subsequent issuance of cash or Common Stock pursuant to the Award if such grant or issuance would be in violation of any applicable law.

(c) No Obligation to Notify or Minimize Taxes. The Company will have no duty or obligation to any Participant to advise such holder as to the tax treatment or time or manner of exercising such Stock Award. Furthermore, the Company will have no duty or obligation to warn or otherwise advise such holder of a pending termination or expiration of an Award or a possible period in which the Award may not be exercised. The Company has no duty or obligation to minimize the tax consequences of an Award to the holder of such Award.

8. MISCELLANEOUS.

(a) Use of Proceeds from Sales of Common Stock. Proceeds from the sale of shares of Common Stock pursuant to Awards will constitute general funds of the Company.

(b) Corporate Action Constituting Grant of Awards. Corporate action constituting a grant by the Company of an Award to any Participant will be deemed completed as of the date of such corporate action, unless otherwise determined by the Board, regardless of when the instrument, certificate, or letter evidencing the Award is communicated to, or actually received or accepted by, the Participant. In the event that the corporate records (e.g., Board

consents, resolutions or minutes) documenting the corporate action constituting the grant contain terms (e.g., exercise price, vesting schedule or number of shares) that are inconsistent with those in the Award Agreement or related grant documents as a result of a clerical error in the papering of the Award Agreement or related grant documents, the corporate records will control and the Participant will have no legally binding right to the incorrect term in the Award Agreement or related grant documents.

(c) Stockholder Rights. No Participant will be deemed to be the holder of, or to have any of the rights of a holder with respect to, any shares of Common Stock subject to an Award unless and until (i) such Participant has satisfied all requirements for exercise of, or the issuance of shares of Common Stock under, the Award pursuant to its terms, and (ii) the issuance of the Common Stock subject to such Award has been entered into the books and records of the Company.

(d) No Employment or Other Service Rights. Nothing in the Plan, any Award Agreement or any other instrument executed thereunder or in connection with any Award granted pursuant thereto will confer upon any Participant any right to continue to serve the Company or an Affiliate in the capacity in effect at the time the Award was granted or will affect the right of the Company or an Affiliate to terminate (i) the employment of an Employee with or without notice and with or without cause, (ii) the service of a Consultant pursuant to the terms of such Consultant' s agreement with the Company or an Affiliate, or (iii) the service of a Director pursuant to the bylaws of the Company or an Affiliate, and any applicable provisions of the corporate law of the state or foreign jurisdiction in which the Company or the Affiliate is domiciled or incorporated, as the case may be.

(e) Change in Time Commitment. In the event a Participant' s regular level of time commitment in the performance of his or her services for the Company and any Affiliates is reduced (for example, and without limitation, if the Participant is an Employee of the Company and the Employee has a change in status from a full-time Employee to a part-time Employee or takes an extended leave of absence) after the date of grant of any Award to the Participant, the Board has the right in its sole discretion to (x) make a corresponding reduction in the number of shares or cash amount subject to any portion of such Award that is scheduled to vest or become payable after the date of such change in time commitment, and (y) in lieu of or in combination with such a reduction, extend the vesting or payment schedule applicable to such Award. In the event of any such reduction, the Participant will have no right with respect to any portion of the Award that is so reduced or extended.

(f) Incentive Stock Option Limitations. To the extent that the aggregate Fair Market Value (determined at the time of grant) of Common Stock with respect to which Incentive Stock Options are exercisable for the first time by any Optionholder during any calendar year (under all plans of the Company and any Affiliates) exceeds $100,000 (or such other limit established in the Code) or otherwise does not comply with the rules governing Incentive Stock Options, the Options or portions thereof that exceed such limit (according to the order in which they were granted) or otherwise do not comply with such rules will be treated as Nonstatutory Stock Options, notwithstanding any contrary provision of the applicable Option Agreement(s).

(g) Investment Assurances. The Company may require a Participant, as a condition of exercising or acquiring Common Stock under any Award, (i) to give written assurances satisfactory to the Company as to the Participant' s knowledge and experience in financial and business matters and/or to employ a purchaser representative reasonably satisfactory to the Company who is knowledgeable and experienced in financial and business matters and that such Participant is capable of evaluating, alone or together with the purchaser representative, the merits and risks of exercising the Award; and (ii) to give written assurances satisfactory to the Company stating that the Participant is acquiring Common Stock subject to the Award for the Participant' s own account and not with any present intention of selling or otherwise distributing the Common Stock. The foregoing requirements, and any assurances given pursuant to such requirements, will be inoperative if (A) the issuance of the shares upon the exercise or acquisition of Common Stock under the Award has been registered under a then currently effective registration statement under the Securities Act, or (B) as to any particular requirement, a determination is made by counsel for the Company that such requirement need not be met in the circumstances under the then applicable securities laws. The Company may, upon advice of counsel to the Company, place legends on stock certificates issued under the Plan as such counsel deems necessary or appropriate in order to comply with applicable securities laws, including, but not limited to, legends restricting the transfer of the Common Stock.

(h) Withholding Obligations. Unless prohibited by the terms of an Award Agreement, the Company may, in its sole discretion, satisfy any federal, state or local tax withholding obligation relating to an Award by any of the following means or by a combination of such means: (i) causing the Participant to tender a cash payment; (ii) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to the Participant in connection with the Award; *provided, however,* that no shares of Common Stock are withheld with a value exceeding the maximum amount of tax required to be withheld by law (or such lesser amount as may be necessary to avoid classification of the Stock Award as a liability for financial accounting purposes); (iii) withholding cash from an Award settled in cash; (iv) withholding payment from any amounts otherwise payable to the Participant; or (v) by such other method as may be set forth in the Award Agreement.

(i) Electronic Delivery. Any reference herein to a "written" agreement or document will include any agreement or document delivered electronically, filed publicly at www.sec.gov (or any successor website thereto) or posted on the Company' s intranet (or other shared electronic medium controlled by the Company to which the Participant has access).

(j) Deferrals. To the extent permitted by applicable law, the Board, in its sole discretion, may determine that the delivery of Common Stock or the payment of cash, upon the exercise, vesting or settlement of all or a portion of any Award may be deferred and may establish programs and procedures for deferral elections to be made by Participants. Deferrals by Participants will be made in accordance with Section 409A of the Code. Consistent with Section 409A of the Code, the Board may provide for distributions while a Participant is still an employee or otherwise providing services to the Company. The Board is authorized to make deferrals of Awards and determine when, and in what annual percentages, Participants may receive payments, including lump sum payments, following the Participant' s termination of Continuous Service, and implement such other terms and conditions consistent with the provisions of the Plan and in accordance with applicable law.

(k) Compliance with Section 409A of the Code. Unless otherwise expressly provided for in an Award Agreement, the Plan and Award Agreements will be interpreted to the greatest extent possible in a manner that makes the Plan and the Awards granted hereunder exempt from Section 409A of the Code, and, to the extent not so exempt, in compliance with Section 409A of the Code. If the Board determines that any Award granted hereunder is not exempt from and is therefore subject to Section 409A of the Code, the Award Agreement evidencing such Award will incorporate the terms and conditions necessary to avoid the consequences specified in Section 409A(a)(1) of the Code, and to the extent an Award Agreement is silent on terms necessary for compliance, such terms are hereby incorporated by reference into the Award Agreement. Notwithstanding anything to the contrary in this Plan (and unless the Award Agreement specifically provides otherwise), if the shares of Common Stock are publicly traded, and if a Participant holding an Award that constitutes "deferred compensation" under Section 409A of the Code is a "specified employee" for purposes of Section 409A of the Code, no distribution or payment of any amount that is due because of a "separation from service" (as defined in Section 409A of the Code without regard to alternative definitions thereunder) will be issued or paid before the date that is six months following the date of such Participant' s "separation from service" (as defined in Section 409A of the Code without regard to alternative definitions thereunder) or, if earlier, the date of the Participant' s death, unless such distribution or payment can be made in a manner that complies with Section 409A of the Code, and any amounts so deferred will be paid in a lump sum on the day after such six month period elapses, with the balance paid thereafter on the original schedule.

(l) Clawback/Recovery. All Awards granted under the Plan will be subject to recoupment in accordance with any clawback policy that the Company is required to adopt pursuant to the listing standards of any national securities exchange or association on which the Company' s securities are listed or as is otherwise required by the Dodd-Frank Wall Street Reform and Consumer Protection Act or other applicable law. In addition, the Board may impose such other clawback, recovery or recoupment provisions in an Award Agreement as the Board determines necessary or appropriate, including but not limited to a reacquisition right in respect of previously acquired shares of Common Stock or other cash or property upon the occurrence of an event constituting Cause. No recovery of compensation under such a clawback policy will be an event giving rise to a right to resign for "good reason" or "constructive termination" (or similar term) under any agreement with the Company.

9. ADJUSTMENTS UPON CHANGES IN COMMON STOCK; OTHER CORPORATE EVENTS.

(a) Capitalization Adjustments. In the event of a Capitalization Adjustment, the Board will appropriately and proportionately adjust: (i) the class(es) and maximum number of securities subject to the Plan pursuant to Section 3(a), (ii) the class(es) of securities by which the share reserve is to increase automatically each year pursuant to Section 3(a), (iii) the class(es) and maximum number of securities that may be issued pursuant to the exercise of Incentive Stock Options pursuant to Section 3(c) and (iv) the class(es) and number of securities and price per share of stock subject to outstanding Stock Awards. The Board will make such adjustments, and its determination will be final, binding and conclusive.

(b) Dissolution. Except as otherwise provided in the Stock Award Agreement, in the event of a Dissolution of the Company, all outstanding Stock Awards (other than Stock Awards

consisting of vested and outstanding shares of Common Stock not subject to a forfeiture condition or the Company' s right of repurchase) will terminate immediately prior to the completion of such Dissolution, and the shares of Common Stock subject to the Company' s repurchase rights or subject to a forfeiture condition may be repurchased or reacquired by the Company notwithstanding the fact that the holder of such Stock Award is providing Continuous Service; *provided, however*, that the Board may, in its sole discretion, cause some or all Stock Awards to become fully vested, exercisable and/or no longer subject to repurchase or forfeiture (to the extent such Stock Awards have not previously expired or terminated) before the Dissolution is completed but contingent on its completion.

(c) Transaction. The following provisions shall apply to Stock Awards in the event of a Transaction unless otherwise provided in the instrument evidencing the Stock Award or any other written agreement between the Company or any Affiliate and the Participant or unless otherwise expressly provided by the Board at the time of grant of a Stock Award. In the event of a Transaction, then, notwithstanding any other provision of the Plan, the Board shall take one or more of the following actions with respect to Stock Awards, contingent upon the closing or completion of the Transaction:

(i) arrange for the surviving corporation or acquiring corporation (or the surviving or acquiring corporation' s parent company) to assume or continue the Stock Award or to substitute a similar stock award for the Stock Award (including, but not limited to, an award to acquire the same consideration paid to the stockholders of the Company pursuant to the Transaction);

(ii) arrange for the assignment of any reacquisition or repurchase rights held by the Company in respect of Common Stock issued pursuant to the Stock Award to the surviving corporation or acquiring corporation (or the surviving or acquiring corporation' s parent company);

(iii) accelerate the vesting, in whole or in part, of the Stock Award (and, if applicable, the time at which the Stock Award may be exercised) to a date prior to the effective time of such Transaction as the Board shall determine (or, if the Board shall not determine such a date, to the date that is five days prior to the effective date of the Transaction), with such Stock Award terminating if not exercised (if applicable) at or prior to the effective time of the Transaction;

(iv) arrange for the lapse, in whole or in part, of any reacquisition or repurchase rights held by the Company with respect to the Stock Award;

(v) cancel or arrange for the cancellation of the Stock Award, to the extent not vested or not exercised prior to the effective time of the Transaction, in exchange for such cash consideration, if any, as the Board, in its sole discretion, may consider appropriate; and

(vi) make a payment, in such form as may be determined by the Board equal to the excess, if any, of (A) the value of the property the Participant would have received upon the exercise of the Stock Award immediately prior to the effective time of the Transaction, over (B) any exercise price payable by such holder in connection with such exercise. For clarity, this

payment may be zero ($0) if the value of the property is equal to or less than the exercise price. Payments under this provision may be delayed to the same extent that payment of consideration to the holders of Common Stock in connection with the Transaction is delayed as a result of escrows, earn outs, holdbacks or other contingencies.

The Board need not take the same action or actions with respect to all Stock Awards or portions thereof or with respect to all Participants. The Board may take different actions with respect to the vested and unvested portions of a Stock Award.

(d) Change in Control. A Stock Award may be subject to additional acceleration of vesting and exercisability upon or after a Change in Control as may be provided in the Stock Award Agreement for such Stock Award or as may be provided in any other written agreement between the Company or any Affiliate and the Participant.

10. PLAN TERM; EARLIER TERMINATION OR SUSPENSION OF THE PLAN.

The Board may suspend or terminate the Plan at any time. No Incentive Stock Options may be granted after the tenth anniversary of the earlier of (i) the date the Plan is adopted by the Board (the "***Adoption Date***"), or (ii) the date the Plan is approved by the stockholders of the Company. No Awards may be granted under the Plan while the Plan is suspended or after it is terminated.

11. EXISTENCE OF THE PLAN; TIMING OF FIRST GRANT OR EXERCISE.

The Plan will come into existence on the Adoption Date; *provided, however*, that no Stock Award may be granted prior to the IPO Date. In addition, no Stock Award will be exercised (or, in the case of a Restricted Stock Award, Restricted Stock Unit Award, Performance Stock Award, or Other Stock Award, no Stock Award will be granted) and no Performance Cash Award will be settled unless and until the Plan has been approved by the stockholders of the Company, which approval will be within 12 months after the date the Plan is adopted by the Board.

12. CHOICE OF LAW.

The law of the State of Delaware will govern all questions concerning the construction, validity and interpretation of this Plan, without regard to that state' s conflict of laws rules.

13. DEFINITIONS. As used in the Plan, the following definitions will apply to the capitalized terms indicated below:

(a) "***Affiliate***" means, at the time of determination, any "parent" or "subsidiary" of the Company as such terms are defined in Rule 405 of the Securities Act. The Board will have the authority to determine the time or times at which "parent" or "subsidiary" status is determined within the foregoing definition.

(b) "***Award***" means a Stock Award or a Performance Cash Award.

(c) "***Award Agreement***" means a written agreement between the Company and a Participant evidencing the terms and conditions of an Award.

(d) "***Board***" means the Board of Directors of the Company.

(e) "***Capital Stock***" means each and every class of common stock of the Company, regardless of the number of votes per share.

(f) "***Capitalization Adjustment***" means any change that is made in, or other events that occur with respect to, the Common Stock subject to the Plan or subject to any Stock Award after the Adoption Date without the receipt of consideration by the Company through merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, stock split, reverse stock split, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or any similar equity restructuring transaction, as that term is used in Statement of Financial Accounting Standards Board Accounting Standards Codification Topic 718 (or any successor thereto). Notwithstanding the foregoing, the conversion of any convertible securities of the Company will not be treated as a Capitalization Adjustment.

(g) "***Cause***" shall have the meaning ascribed to such term in any written agreement between the Participant and the Company defining such term and, in the absence of such agreement, such term means, with respect to a Participant, the occurrence of any of the following events: (i) such Participant' s commission of any felony or any crime involving fraud, dishonesty or moral turpitude under the laws of the United States or any state thereof; (ii) such Participant' s attempted commission of, or participation in, a fraud or act of dishonesty against the Company; (iii) such Participant' s intentional, material violation of any contract or agreement between the Participant and the Company or of any statutory duty owed to the Company; (iv) such Participant' s unauthorized use or disclosure of the Company' s confidential information or trade secrets; or (v) such Participant' s gross misconduct.[1] The determination that a termination of the Participant' s Continuous Service is either for Cause or without Cause shall be made by the Company, in its sole discretion. Any determination by the Company that the Continuous Service of a Participant was terminated with or without Cause for the purposes of outstanding Awards held by such Participant shall have no effect upon any determination of the rights or obligations of the Company or such Participant for any other purpose.

(h) "***Change in Control***" means the occurrence, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) any Exchange Act Person becomes the Owner, directly or indirectly, of securities of the Company representing more than 50% of the combined voting power of the Company' s then outstanding securities other than by virtue of a merger, consolidation or similar transaction. Notwithstanding the foregoing, a Change in Control will not be deemed to occur (A) on account of the acquisition of securities of the Company directly from the Company, (B) on account of the acquisition of securities of the Company by an investor, any affiliate thereof or any other Exchange Act Person that acquires the Company' s securities in a transaction

[1] Company to advise as to whether it has a different definition it would prefer to use.

or series of related transactions the primary purpose of which is to obtain financing for the Company through the issuance of equity securities, (C) on account of the acquisition of securities of the Company by any individual who is, on the IPO Date, either an executive officer or a Director (either, an "***IPO Investor***") and/or any entity in which an IPO Investor has a direct or indirect interest (whether in the form of voting rights or participation in profits or capital contributions) of more than 50% (collectively, the "***IPO Entities***") or on account of the IPO Entities continuing to hold shares that come to represent more than 50% of the combined voting power of the Company' s then outstanding securities as a result of the conversion of any class of the Company' s securities into another class of the Company' s securities having a different number of votes per share pursuant to the conversion provisions set forth in the Company' s Amended and Restated Certificate of Incorporation; or (D) solely because the level of Ownership held by any Exchange Act Person (the "***Subject Person***") exceeds the designated percentage threshold of the outstanding voting securities as a result of a repurchase or other acquisition of voting securities by the Company reducing the number of shares outstanding, provided that if a Change in Control would occur (but for the operation of this sentence) as a result of the acquisition of voting securities by the Company, and after such share acquisition, the Subject Person becomes the Owner of any additional voting securities that, assuming the repurchase or other acquisition had not occurred, increases the percentage of the then outstanding voting securities Owned by the Subject Person over the designated percentage threshold, then a Change in Control will be deemed to occur;

(ii) there is consummated a merger, consolidation or similar transaction involving (directly or indirectly) the Company and, immediately after the consummation of such merger, consolidation or similar transaction, the stockholders of the Company immediately prior thereto do not Own, directly or indirectly, either (A) outstanding voting securities representing more than 50% of the combined outstanding voting power of the surviving Entity in such merger, consolidation or similar transaction or (B) more than 50% of the combined outstanding voting power of the parent of the surviving Entity in such merger, consolidation or similar transaction, in each case in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such transaction; *provided, however*, that a merger, consolidation or similar transaction will not constitute a Change in Control under this prong of the definition if the outstanding voting securities representing more than 50% of the combined voting power of the surviving Entity or its parent are owned by the IPO Entities;

(iii) there is consummated a sale, lease, exclusive license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries, other than a sale, lease, license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries to an Entity, more than 50% of the combined voting power of the voting securities of which are Owned by stockholders of the Company in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such sale, lease, license or other disposition; *provided, however*, that a sale, lease, exclusive license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries will not constitute a Change in Control under this prong of the definition if the outstanding voting securities representing more than 50% of the combined voting power of the acquiring Entity or its parent are owned by the IPO Entities;

(iv) the stockholders of the Company approve or the Board approves a plan of complete dissolution or liquidation of the Company, or a complete dissolution or liquidation of the Company will otherwise occur, except for a liquidation into a parent corporation; or

(v) individuals who, on the date the Plan is adopted by the Board, are members of the Board (the "***Incumbent Board***") cease for any reason to constitute at least a majority of the members of the Board; *provided, however,* that if the appointment or election (or nomination for election) of any new Board member was approved or recommended by a majority vote of the members of the Incumbent Board then still in office, such new member will, for purposes of this Plan, be considered as a member of the Incumbent Board.

Notwithstanding the foregoing definition or any other provision of the Plan, the term Change in Control will not include a sale of assets, merger or other transaction effected exclusively for the purpose of changing the domicile of the Company and the definition of Change in Control (or any analogous term) in an individual written agreement between the Company or any Affiliate and the Participant will supersede the foregoing definition with respect to Awards subject to such agreement; *provided, however*, that if no definition of Change in Control or any analogous term is set forth in such an individual written agreement, the foregoing definition will apply.

(i) "***Code***" means the Internal Revenue Code of 1986, as amended, including any applicable regulations and guidance thereunder.

(j) "***Committee***" means a committee of one or more Directors to whom authority has been delegated by the Board in accordance with Section 2(c).

(k) "***Common Stock***" means, as of the IPO Date, the common stock of the Company, having one vote per share.

(l) "***Company***" means Prevail Therapeutics Inc., a Delaware corporation.

(m) "***Consultant***" means any person, including an advisor, who is (i) engaged by the Company or an Affiliate to render consulting or advisory services and is compensated for such services, or (ii) serving as a member of the board of directors of an Affiliate and is compensated for such services. However, service solely as a Director, or payment of a fee for such service, will not cause a Director to be considered a "Consultant" for purposes of the Plan. Notwithstanding the foregoing, a person is treated as a Consultant under this Plan only if a Form S-8 Registration Statement under the Securities Act is available to register either the offer or the sale of the Company' s securities to such person.

(n) "***Continuous Service***" means that the Participant' s service with the Company or an Affiliate, whether as an Employee, Director or Consultant, is not interrupted or terminated. A change in the capacity in which the Participant renders service to the Company or an Affiliate as an Employee, Consultant or Director or a change in the entity for which the Participant renders such service, provided that there is no interruption or termination of the Participant' s service with the Company or an Affiliate, will not terminate a Participant' s Continuous Service; *provided, however*, that if the Entity for which a Participant is rendering services ceases to qualify as an Affiliate, as determined by the Board, in its sole discretion, such Participant' s

Continuous Service will be considered to have terminated on the date such Entity ceases to qualify as an Affiliate. To the extent permitted by law, the Board or the chief executive officer of the Company, in that party' s sole discretion, may determine whether Continuous Service will be considered interrupted in the case of (i) any leave of absence approved by the Board or chief executive officer, including sick leave, military leave or any other personal leave, or (ii) transfers between the Company, an Affiliate, or their successors. Notwithstanding the foregoing, a leave of absence will be treated as Continuous Service for purposes of vesting in an Award only to such extent as may be provided in the Company' s leave of absence policy, in the written terms of any leave of absence agreement or policy applicable to the Participant, or as otherwise required by law.

(o) "***Corporate Transaction***" means the consummation, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) a sale or other disposition of all or substantially all, as determined by the Board, in its sole discretion, of the consolidated assets of the Company and its Subsidiaries;

(ii) a sale or other disposition of more than 50% of the outstanding securities of the Company;

(iii) a merger, consolidation or similar transaction following which the Company is not the surviving corporation; or

(iv) a merger, consolidation or similar transaction following which the Company is the surviving corporation but the shares of Common Stock outstanding immediately preceding the merger, consolidation or similar transaction are converted or exchanged by virtue of the merger, consolidation or similar transaction into other property, whether in the form of securities, cash or otherwise.

(p) "***Director***" means a member of the Board.

(q) "***Disability***" means, with respect to a Participant, the inability of such Participant to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment that can be expected to result in death or that has lasted or can be expected to last for a continuous period of not less than 12 months, as provided in Sections 22(e)(3) and 409A(a)(2)(c)(i) of the Code, and will be determined by the Board on the basis of such medical evidence as the Board deems warranted under the circumstances.

(r) "***Dissolution***" means when the Company, after having executed a certificate of dissolution with the State of Delaware (or other applicable state), has completely wound up its affairs. Conversion of the Company into a Limited Liability Company (or any other pass-through entity) will not be considered a "Dissolution" for purposes of the Plan.

(s) "***Employee***" means any person employed by the Company or an Affiliate. However, service solely as a Director, or payment of a fee for such services, will not cause a Director to be considered an "Employee" for purposes of the Plan.

(t) "***Entity***" means a corporation, partnership, limited liability company or other entity.

(u) "***Exchange Act***" means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

(v) "***Exchange Act Person***" means any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act), except that "Exchange Act Person" will not include (i) the Company or any Subsidiary of the Company, (ii) any employee benefit plan of the Company or any Subsidiary of the Company or any trustee or other fiduciary holding securities under an employee benefit plan of the Company or any Subsidiary of the Company, (iii) an underwriter temporarily holding securities pursuant to a registered public offering of such securities, (iv) an Entity Owned, directly or indirectly, by the stockholders of the Company in substantially the same proportions as their Ownership of stock of the Company; or (v) any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act) that, as of the IPO Date, is the Owner, directly or indirectly, of securities of the Company representing more than 50% of the combined voting power of the Company' s then outstanding securities.

(w) "***Fair Market Value***" means, as of any date, the value of the Common Stock determined as follows:

(i) If the Common Stock is listed on any established stock exchange or traded on any established market, the Fair Market Value of a share of Common Stock will be, unless otherwise determined by the Board, the closing sales price for such stock as quoted on such exchange or market (or the exchange or market with the greatest volume of trading in the Common Stock) on the date of determination, as reported in a source the Board deems reliable.

(ii) Unless otherwise provided by the Board, if there is no closing sales price for the Common Stock on the date of determination, then the Fair Market Value will be the closing selling price on the last preceding date for which such quotation exists.

(iii) In the absence of such markets for the Common Stock, the Fair Market Value will be determined by the Board in good faith and in a manner that complies with Sections 409A and 422 of the Code.

(x) "***Incentive Stock Option***" means an option granted pursuant to Section 5 of the Plan that is intended to be, and qualifies as, an "incentive stock option" within the meaning of Section 422 of the Code.

(y) "***IPO Date***" means the date of the underwriting agreement between the Company and the underwriter(s) managing the initial public offering of the Common Stock, pursuant to which the Common Stock is priced for the initial public offering.

(z) "***Non-Employee Director***" means a Director who either (i) is not a current employee or officer of the Company or an Affiliate, does not receive compensation, either directly or indirectly, from the Company or an Affiliate for services rendered as a consultant or in any capacity other than as a Director (except for an amount as to which disclosure would not

be required under Item 404(a) of Regulation S-K promulgated pursuant to the Securities Act ("***Regulation S-K***")), does not possess an interest in any other transaction for which disclosure would be required under Item 404(a) of Regulation S-K, and is not engaged in a business relationship for which disclosure would be required pursuant to Item 404(b) of Regulation S-K; or (ii) is otherwise considered a "non-employee director" for purposes of Rule 16b-3.

(aa) "***Nonstatutory Stock Option***" means any Option granted pursuant to Section 5 of the Plan that does not qualify as an Incentive Stock Option.

(bb) "***Officer***" means a person who is an officer of the Company within the meaning of Section 16 of the Exchange Act.

(cc) "***Option***" means an Incentive Stock Option or a Nonstatutory Stock Option to purchase shares of Common Stock granted pursuant to the Plan.

(dd) "***Option Agreement***" means a written agreement between the Company and an Optionholder evidencing the terms and conditions of an Option grant. Each Option Agreement will be subject to the terms and conditions of the Plan.

(ee) "***Optionholder***" means a person to whom an Option is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Option.

(ff) "***Other Stock Award***" means an award based in whole or in part by reference to the Common Stock which is granted pursuant to the terms and conditions of Section 6(d).

(gg) "***Other Stock Award Agreement***" means a written agreement between the Company and a holder of an Other Stock Award evidencing the terms and conditions of an Other Stock Award grant. Each Other Stock Award Agreement will be subject to the terms and conditions of the Plan.

(hh) "***Own,***" "***Owned,***" "***Owner,***" "***Ownership***" means a person or Entity will be deemed to "Own," to have "Owned," to be the "Owner" of, or to have acquired "Ownership" of securities if such person or Entity, directly or indirectly, through any contract, arrangement, understanding, relationship or otherwise, has or shares voting power, which includes the power to vote or to direct the voting, with respect to such securities.

(ii) "***Participant***" means a person to whom an Award is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Stock Award.

(jj) "***Performance Cash Award***" means an award of cash granted pursuant to the terms and conditions of Section 6(c)(ii).

(kk) "***Performance Criteria***" means the one or more criteria that the Board or Committee (as applicable) will select for purposes of establishing the Performance Goals for a Performance Period. The Performance Criteria that will be used to establish such Performance Goals may be based on any one of, or combination of, the following as determined by the Board: (i) earnings (including earnings per share and net earnings); (ii) earnings before interest, taxes and depreciation; (iii) earnings before interest, taxes, depreciation and amortization; (iv) total

stockholder return; (v) return on equity or average stockholder' s equity; (vi) return on assets, investment, or capital employed; (vii) stock price; (viii) margin (including gross margin); (ix) income (before or after taxes); (x) operating income; (xi) operating income after taxes; (xii) pre-tax profit; (xiii) operating cash flow; (xiv) sales or revenue targets; (xv) increases in revenue or product revenue; (xvi) expenses and cost reduction goals; (xvii) improvement in or attainment of working capital levels; (xviii) economic value added (or an equivalent metric); (xix) market share; (xx) cash flow; (xxi) cash flow per share; (xxii) share price performance; (xxiii) debt reduction; (xxiv) customer satisfaction; (xxv) stockholders' equity; (xxvi) capital expenditures; (xxvii) debt levels; (xxviii) operating profit or net operating profit; (xxix) workforce diversity; (xxx) growth of net income or operating income; (xxxi) billings; (xxxii) pre-clinical development related compound goals; (xxxiii) financing; (xxxiv) regulatory milestones, including approval of a compound; (xxxv) stockholder liquidity; (xxvi) corporate governance and compliance; (xxxvii) product commercialization; (xxxviii) intellectual property; (xxxix) personnel matters; (xl) progress of internal research or clinical programs; (xli) progress of partnered programs; (xlii) partner satisfaction; (xliii) budget management; (xliv) clinical achievements; (xlv) completing phases of a clinical study (including the treatment phase); (xlvi) announcing or presenting preliminary or final data from clinical studies; in each case, whether on particular timelines or generally; (xlvii) timely completion of clinical trials; (xlviii) submission of INDs and NDAs and other regulatory achievements; (xlix) partner or collaborator achievements; (l) internal controls, including those related to the Sarbanes-Oxley Act of 2002; (li) research progress, including the development of programs; (lii) investor relations, analysts and communication; (liii) manufacturing achievements (including obtaining particular yields from manufacturing runs and other measurable objectives related to process development activities); (liv) strategic partnerships or transactions (including in-licensing and out-licensing of intellectual property; (lv) establishing relationships with commercial entities with respect to the marketing, distribution and sale of the Company' s products (including with group purchasing organizations, distributors and other vendors); (lvi) supply chain achievements (including establishing relationships with manufacturers or suppliers of active pharmaceutical ingredients and other component materials and manufacturers of the Company' s products); (lvii) co-development, co-marketing, profit sharing, joint venture or other similar arrangements; (lviii) individual performance goals; (lix) corporate development and planning goals; and (lx) other measures of performance selected by the Board or Committee.

(ll) "***Performance Goals***" means, for a Performance Period, the one or more goals established by the Board or Committee (as applicable) for the Performance Period based upon the Performance Criteria. Performance Goals may be based on a Company-wide basis, with respect to one or more business units, divisions, Affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise by the Board (i) in the Award Agreement at the time the Award is granted or (ii) in such other document setting forth the Performance Goals at the time the Performance Goals are established, the Board will appropriately make adjustments in the method of calculating the attainment of Performance Goals for a Performance Period as follows: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects; (3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; (5) to exclude the effects of any items that are unusual in nature or occur infrequently as determined under generally accepted accounting principles; (6) to exclude the

dilutive effects of acquisitions or joint ventures; (7) to assume that any business divested by the Company achieved performance objectives at targeted levels during the balance of a Performance Period following such divestiture; (8) to exclude the effect of any change in the outstanding shares of common stock of the Company by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger, consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to common stockholders other than regular cash dividends; (9) to exclude the effects of stock based compensation and the award of bonuses under the Company' s bonus plans; (10) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to be expensed under generally accepted accounting principles; (11) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles; and (12) to exclude the effect of any other unusual, non-recurring gain or loss or other extraordinary item. In addition, the Board or Committee (as applicable) retains the discretion to adjust or eliminate the compensation or economic benefit due upon attainment of Performance Goals and to define the manner of calculating the Performance Criteria it selects to use for such Performance Period. Partial achievement of the specified criteria may result in the payment or vesting corresponding to the degree of achievement as specified in the Stock Award Agreement or the written terms of a Performance Cash Award.

(mm) "***Performance Period***" means the period of time selected by the Board or Committee (as applicable) over which the attainment of one or more Performance Goals will be measured for the purpose of determining a Participant' s right to and the payment of a Stock Award or a Performance Cash Award. Performance Periods may be of varying and overlapping duration, at the sole discretion of the Board or Committee.

(nn) "***Performance Stock Award***" means a Stock Award granted under the terms and conditions of Section 6(c)(i).

(oo) "***Plan***" means this Prevail Therapeutics Inc. 2019 Equity Incentive Plan.

(pp) "***Restricted Stock Award***" means an award of shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(a).

(qq) "***Restricted Stock Award Agreement***" means a written agreement between the Company and a holder of a Restricted Stock Award evidencing the terms and conditions of a Restricted Stock Award grant. Each Restricted Stock Award Agreement will be subject to the terms and conditions of the Plan.

(rr) "***Restricted Stock Unit Award***" means a right to receive shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(b).

(ss) "***Restricted Stock Unit Award Agreement***" means a written agreement between the Company and a holder of a Restricted Stock Unit Award evidencing the terms and conditions of a Restricted Stock Unit Award grant. Each Restricted Stock Unit Award Agreement will be subject to the terms and conditions of the Plan.

(tt) "***Rule 16b-3***" means Rule 16b-3 promulgated under the Exchange Act or any successor to Rule 16b-3, as in effect from time to time.

(uu) "***Securities Act***" means the Securities Act of 1933, as amended.

(vv) "***Stock Appreciation Right***" or "***SAR***" means a right to receive the appreciation on Common Stock that is granted pursuant to the terms and conditions of Section 5.

(ww) "***Stock Appreciation Right Agreement***" means a written agreement between the Company and a holder of a Stock Appreciation Right evidencing the terms and conditions of a Stock Appreciation Right grant. Each Stock Appreciation Right Agreement will be subject to the terms and conditions of the Plan.

(xx) "***Stock Award***" means any right to receive Common Stock granted under the Plan, including an Incentive Stock Option, a Nonstatutory Stock Option, a Restricted Stock Award, a Restricted Stock Unit Award, a Stock Appreciation Right, a Performance Stock Award or any Other Stock Award.

(yy) "***Stock Award Agreement***" means a written agreement between the Company and a Participant evidencing the terms and conditions of a Stock Award grant. Each Stock Award Agreement will be subject to the terms and conditions of the Plan.

(zz) "***Subsidiary***" means, with respect to the Company, (i) any corporation of which more than 50% of the outstanding capital stock having ordinary voting power to elect a majority of the board of directors of such corporation (irrespective of whether, at the time, stock of any other class or classes of such corporation will have or might have voting power by reason of the happening of any contingency) is at the time, directly or indirectly, Owned by the Company, and (ii) any partnership, limited liability company or other entity in which the Company has a direct or indirect interest (whether in the form of voting or participation in profits or capital contribution) of more than 50%.

(aaa) "***Ten Percent Stockholder***" means a person who Owns (or is deemed to Own pursuant to Section 424(d) of the Code) stock possessing more than 10% of the total combined voting power of all classes of stock of the Company or any Affiliate.

(bbb) "***Transaction***" means a Corporate Transaction or a Change in Control.

PREVAIL THERAPEUTICS INC.

STOCK OPTION GRANT NOTICE
(2019 EQUITY INCENTIVE PLAN)

Prevail Therapeutics Inc. (the "***Company***"), pursuant to its 2019 Equity Incentive Plan (the "***Plan***"), hereby grants to Optionholder an option to purchase the number of shares of the Company' s Common Stock set forth below. This option is subject to all of the terms and conditions as set forth in this Stock Option Grant Notice, in the Option Agreement, the Plan and the Notice of Exercise, all of which are attached hereto and incorporated herein in their entirety. Capitalized terms not explicitly defined herein but defined in the Plan or the Option Agreement will have the same definitions as in the Plan or the Option Agreement. If there is any conflict between the terms in this Stock Option Grant Notice and the Plan, the terms of the Plan will control.

Optionholder:	_____
Date of Grant:	_____
Vesting Commencement Date:	_____
Number of Shares Subject to Option:	_____
Exercise Price (Per Share):	_____
Total Exercise Price:	_____
Expiration Date:	_____

Type of Grant: ☐ Incentive Stock Option[1] ☐ Nonstatutory Stock Option

Exercise Schedule: Same as Vesting Schedule

Vesting Schedule: **[**_____, subject to Optionholder' s Continuous Service as of each such date]

Payment: By one or a combination of the following items (described in the Option Agreement):

☐ By cash, check, bank draft or money order payable to the Company
☐ Pursuant to a Regulation T Program if the shares are publicly traded
☐ By delivery of already-owned shares if the shares are publicly traded
☐ If and only to the extent this option is a Nonstatutory Stock Option, and subject to the Company' s consent at the time of exercise, by a "net exercise" arrangement

1 If this is an Incentive Stock Option, it (plus other outstanding Incentive Stock Options) cannot be first *exercisable* for more than $100,000 in value (measured by exercise price) in any calendar year. Any excess over $100,000 is a Nonstatutory Stock Option.

Additional Terms/Acknowledgements: Optionholder acknowledges receipt of, and understands and agrees to, this Stock Option Grant Notice, the Option Agreement and the Plan. Optionholder acknowledges and agrees that this Stock Option Grant Notice and the Option Agreement may not be modified, amended or revised except as provided in the Plan. Optionholder further acknowledges that as of the Date of Grant, this Stock Option Grant Notice, the Option Agreement, and the Plan set forth the entire understanding between Optionholder and the Company regarding this option award and supersede all prior oral and written agreements, promises and/or representations on that subject with the exception of, if applicable, (i) equity awards previously granted and delivered to Optionholder, (ii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law and (iii) any written employment agreement, severance agreement, offer letter or other written agreement entered into between the Company and Participant specifying the terms that should govern this specific option. By accepting this option, Optionholder consents to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

PREVAIL THERAPEUTICS INC.	**OPTIONHOLDER:**
By: _____	_____
Signature	Signature
Title: _____	Date: _____
Date: _____	

ATTACHMENTS: Option Agreement, 2019 Equity Incentive Plan and Notice of Exercise

ATTACHMENT I

PREVAIL THERAPEUTICS INC.

OPTION AGREEMENT
(2019 EQUITY INCENTIVE PLAN)
(INCENTIVE STOCK OPTION OR NONSTATUTORY STOCK OPTION)

Pursuant to your Stock Option Grant Notice ("***Grant Notice***") and this Option Agreement, Prevail Therapeutics Inc. (the "***Company***") has granted you an option under its 2019 Equity Incentive Plan (the "***Plan***") to purchase the number of shares of the Company' s Common Stock indicated in your Grant Notice at the exercise price indicated in your Grant Notice. The option is granted to you effective as of the date of grant set forth in the Grant Notice (the "***Date of Grant***"). If there is any conflict between the terms in this Option Agreement and the Plan, the terms of the Plan will control. Capitalized terms not explicitly defined in this Option Agreement or in the Grant Notice but defined in the Plan will have the same definitions as in the Plan.

The details of your option, in addition to those set forth in the Grant Notice and the Plan, are as follows:

1. VESTING. Subject to the provisions contained herein, your option will vest as provided in your Grant Notice. Vesting will cease upon the termination of your Continuous Service.

2. NUMBER OF SHARES AND EXERCISE PRICE. The number of shares of Common Stock subject to your option and your exercise price per share in your Grant Notice will be adjusted for Capitalization Adjustments.

3. EXERCISE RESTRICTION FOR NON-EXEMPT EMPLOYEES. If you are an Employee eligible for overtime compensation under the Fair Labor Standards Act of 1938, as amended (that is, a "***Non-Exempt Employee***"), and except as otherwise provided in the Plan, you may not exercise your option until you have completed at least six (6) months of Continuous Service measured from the Date of Grant, even if you have already been an employee for more than six (6) months. Consistent with the provisions of the Worker Economic Opportunity Act, you may exercise your option as to any vested portion prior to such six (6) month anniversary in the case of (i) your death or disability, (ii) a Corporate Transaction in which your option is not assumed, continued or substituted, (iii) a Change in Control or (iv) your termination of Continuous Service on your "retirement" (as defined in the Company' s benefit plans).

4. METHOD OF PAYMENT. You must pay the full amount of the exercise price for the shares you wish to exercise. You may pay the exercise price in cash or by check, bank draft or money order payable to the Company or in any other manner ***permitted by your Grant Notice,*** which may include one or more of the following:

(a) Provided that at the time of exercise the Common Stock is publicly traded, pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of Common Stock, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds. This manner of payment is also known as a "broker-assisted exercise", "same day sale", or "sell to cover".

(b) Provided that at the time of exercise the Common Stock is publicly traded, by delivery to the Company (either by actual delivery or attestation) of already-owned shares of Common Stock that are owned free and clear of any liens, claims, encumbrances or security interests, and that are valued at Fair Market Value on the date of exercise. "Delivery" for these purposes, in the sole discretion of the Company at the time you exercise your option, will include delivery to the Company of your attestation of ownership of such shares of Common Stock in a form approved by the Company. You may not exercise your option by delivery to the Company of Common Stock if doing so would violate the provisions of any law, regulation or agreement restricting the redemption of the Company' s stock.

(c) If this option is a Nonstatutory Stock Option, subject to the consent of the Company at the time of exercise, by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issued upon exercise of your option by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price. You must pay any remaining balance of the aggregate exercise price not satisfied by the "net exercise" in cash or other permitted form of payment. Shares of Common Stock will no longer be outstanding under your option and will not be exercisable thereafter if those shares (i) are used to pay the exercise price pursuant to the "net exercise," (ii) are delivered to you as a result of such exercise, and (iii) are withheld to satisfy your tax withholding obligations.

5. WHOLE SHARES. You may exercise your option only for whole shares of Common Stock.

6. SECURITIES LAW COMPLIANCE. In no event may you exercise your option unless the shares of Common Stock issuable upon exercise are then registered under the Securities Act or, if not registered, the Company has determined that your exercise and the issuance of the shares would be exempt from the registration requirements of the Securities Act. The exercise of your option also must comply with all other applicable laws and regulations governing your option, and you may not exercise your option if the Company determines that such exercise would not be in material compliance with such laws and regulations (including any restrictions on exercise required for compliance with Treas. Reg. 1.401(k)-1(d)(3), if applicable).

7. TERM. You may not exercise your option before the Date of Grant or after the expiration of the option' s term. The term of your option expires, subject to the provisions of Section 5(h) of the Plan, upon the earliest of the following:

(a) immediately upon the termination of your Continuous Service for Cause;

(b) three (3) months after the termination of your Continuous Service for any reason other than Cause, your Disability or your death (except as otherwise provided in Section 8(d) below); *provided, however,* that if during any part of such three (3) month period your option is not exercisable solely because of the condition set forth in the section above regarding "Securities Law Compliance," your option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service; *provided further,* if during any part of such three (3) month period, the sale of any Common Stock received upon exercise of your option would violate the Company' s insider trading policy, then your option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service during which the sale of the Common Stock received upon exercise of your option would not be in violation of the Company' s insider trading policy. Notwithstanding the foregoing, if (i) you are a Non-Exempt Employee, (ii) your Continuous Service terminates within six (6) months after the Date of Grant, and (iii) you have vested in a portion of your option at the time of your termination of Continuous Service, your option will not expire until the earlier of (x) the later of (A) the date that is seven (7) months after the Date of Grant, and (B) the date that is three (3) months after the termination of your Continuous Service, and (y) the Expiration Date;

(c) twelve (12) months after the termination of your Continuous Service due to your Disability (except as otherwise provided in Section 7(d)) below;

(d) eighteen (18) months after your death if you die either during your Continuous Service or within three (3) months after your Continuous Service terminates for any reason other than Cause;

(e) the Expiration Date indicated in your Grant Notice; or

(f) the day before the tenth (10th) anniversary of the Date of Grant.

If your option is an Incentive Stock Option, note that to obtain the federal income tax advantages associated with an Incentive Stock Option, the Code requires that at all times beginning on the Date of Grant and ending on the day three (3) months before the date of your option' s exercise, you must be an employee of the Company or an Affiliate, except in the event of your death or Disability. The Company has provided for extended exercisability of your option under certain circumstances for your benefit but cannot guarantee that your option will necessarily be treated as an Incentive Stock Option if you continue to provide services to the Company or an Affiliate as a Consultant or Director after your employment terminates or if you otherwise exercise your option more than three (3) months after the date your employment with the Company or an Affiliate terminates.

8. EXERCISE.

(a) You may exercise the vested portion of your option (and the unvested portion of your option if your Grant Notice so permits) during its term by (i) delivering a Notice of Exercise (in a form designated by the Company) or completing such other documents and/or procedures designated by the Company for exercise and (ii) paying the exercise price and any applicable withholding taxes to the Company' s Secretary, stock plan administrator, or such other person as the Company may designate, together with such additional documents as the Company may then require.

(b) By exercising your option you agree that, as a condition to any exercise of your option, the Company may require you to enter into an arrangement providing for the payment by you to the Company of any tax withholding obligation of the Company arising by reason of (i) the exercise of your option, (ii) the lapse of any substantial risk of forfeiture to which the shares of Common Stock are subject at the time of exercise, or (iii) the disposition of shares of Common Stock acquired upon such exercise.

(c) If your option is an Incentive Stock Option, by exercising your option you agree that you will notify the Company in writing within fifteen (15) days after the date of any disposition of any of the shares of the Common Stock issued upon exercise of your option that occurs within two (2) years after the Date of Grant or within one (1) year after such shares of Common Stock are transferred upon exercise of your option.

9. TRANSFERABILITY. Except as otherwise provided in this Section 9, your option is not transferable, except by will or by the laws of descent and distribution, and is exercisable during your life only by you.

(a) Certain Trusts. Upon receiving written permission from the Board or its duly authorized designee, you may transfer your option to a trust if you are considered to be the sole beneficial owner (determined under Section 671 of the Code and applicable state law) while the option is held in the trust. You and the trustee must enter into transfer and other agreements required by the Company.

(b) Domestic Relations Orders. Upon receiving written permission from the Board or its duly authorized designee, and provided that you and the designated transferee enter into transfer and other agreements required by the Company, you may transfer your option pursuant to the terms of a domestic relations order, official marital settlement agreement or other divorce or separation instrument as permitted by Treasury Regulation 1.421-1(b)(2) that contains the information required by the Company to effectuate the transfer. You are encouraged to discuss the proposed terms of any division of this option with the Company prior to finalizing the domestic relations order or marital settlement agreement to help ensure the required information is contained within the domestic relations order or marital settlement agreement. If this option is an Incentive Stock Option, this option may be deemed to be a Nonstatutory Stock Option as a result of such transfer.

(c) Beneficiary Designation. Upon receiving written permission from the Board or its duly authorized designee, you may, by delivering written notice to the Company, in a form approved by the Company and any broker designated by the Company to handle option exercises, designate a third party who, on your death, will thereafter be entitled to exercise this option and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, your executor or administrator of your estate will be entitled to exercise this option and receive, on behalf of your estate, the Common Stock or other consideration resulting from such exercise.

10. OPTION NOT A SERVICE CONTRACT. Your option is not an employment or service contract, and nothing in your option will be deemed to create in any way whatsoever any obligation on your part to continue in the employ of the Company or an Affiliate, or of the Company or an Affiliate to continue your employment. In addition, nothing in your option will obligate the Company or an Affiliate, their respective stockholders, boards of directors, officers or employees to continue any relationship that you might have as a Director or Consultant for the Company or an Affiliate.

11. WITHHOLDING OBLIGATIONS.

(a) At the time you exercise your option, in whole or in part, and at any time thereafter as requested by the Company, you hereby authorize withholding from payroll and any other amounts payable to you, and otherwise agree to make adequate provision for (including by means of a "same day sale" pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board to the extent permitted by the Company), any sums required to satisfy the federal, state, local and foreign tax withholding obligations of the Company or an Affiliate, if any, which arise in connection with the exercise of your option.

(b) If this option is a Nonstatutory Stock Option, then upon your request and subject to approval by the Company, and compliance with any applicable legal conditions or restrictions, the Company may withhold from fully vested shares of Common Stock otherwise issuable to you upon the exercise of your option a number of whole shares of Common Stock having a Fair Market Value, determined by the Company as of the date of exercise, not in excess of the maximum amount of tax required to be withheld by law (or such lower amount as may be necessary to avoid classification of your option as a liability for financial accounting purposes). Notwithstanding the filing of such election, shares of Common Stock shall be withheld solely from fully vested shares of Common Stock determined as of the date of exercise of your option that are otherwise issuable to you upon such exercise. Any adverse consequences to you arising in connection with such share withholding procedure shall be your sole responsibility.

(c) You may not exercise your option unless the tax withholding obligations of the Company and/or any Affiliate are satisfied. Accordingly, you may not be able to exercise your option when desired even though your option is vested, and the Company will have no obligation to issue a certificate for such shares of Common Stock or release such shares of Common Stock from any escrow provided for herein, if applicable, unless such obligations are satisfied.

12. TAX CONSEQUENCES. You hereby agree that the Company does not have a duty to design or administer the Plan or its other compensation programs in a manner that minimizes your tax liabilities. You will not make any claim against the Company, or any of its Officers, Directors, Employees or Affiliates related to tax liabilities arising from your option or your other compensation. In particular, you acknowledge that this option is exempt from Section 409A of the Code only if the exercise price per share specified in the Grant Notice is at least equal to the "fair market value" per share of the Common Stock on the Date of Grant and there is no other impermissible deferral of compensation associated with the option.

13. NOTICES. Any notices provided for in your option or the Plan will be given in writing (including electronically) and will be deemed effectively given upon receipt or, in the case of notices delivered by mail by the Company to you, five (5) days after deposit in the United States mail, postage prepaid, addressed to you at the last address you provided to the Company. The Company may, in its sole discretion, decide to deliver any documents related to participation in the Plan and this option by electronic means or to request your consent to participate in the Plan by electronic means. By accepting this option, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

14. GOVERNING PLAN DOCUMENT. Your option is subject to all the provisions of the Plan, the provisions of which are hereby made a part of your option, and is further subject to all interpretations, amendments, rules and regulations, which may from time to time be promulgated and adopted pursuant to the Plan. If there is any conflict between the provisions of your option and those of the Plan, the provisions of the Plan will control. In addition, your option (and any compensation paid or shares issued under your option) is subject to recoupment in accordance with The Dodd‑Frank Wall Street Reform and Consumer Protection Act and any implementing regulations thereunder, any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law.

15. OTHER DOCUMENTS. You hereby acknowledge receipt of and the right to receive a document providing the information required by Rule 428(b)(1) promulgated under the Securities Act, which includes the Plan prospectus. In addition, you acknowledge receipt of the Company' s policy permitting certain individuals to sell shares only during certain "window" periods and the Company' s insider trading policy, in effect from time to time.

16. EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of this option will not be included as compensation, earnings, salaries, or other similar terms used when calculating your benefits under any employee benefit plan sponsored by the Company or any Affiliate, except as such plan otherwise expressly provides. The Company expressly reserves its rights to amend, modify, or terminate any of the Company' s or any Affiliate' s employee benefit plans.

17. VOTING RIGHTS. You will not have voting or any other rights as a stockholder of the Company with respect to the shares to be issued pursuant to this option until such shares are issued to you. Upon such issuance, you will obtain full voting and other rights as a stockholder of the Company. Nothing contained in this option, and no action taken pursuant to its provisions, will create or be construed to create a trust of any kind or a fiduciary relationship between you and the Company or any other person.

18. SEVERABILITY. If all or any part of this Option Agreement or the Plan is declared by any court or governmental authority to be unlawful or invalid, such unlawfulness or invalidity will not invalidate any portion of this Option Agreement or the Plan not declared to be unlawful or invalid. Any Section of this Option Agreement (or part of such a Section) so declared to be unlawful or invalid shall, if possible, be construed in a manner which will give effect to the terms of such Section or part of a Section to the fullest extent possible while remaining lawful and valid.

19. MISCELLANEOUS.

(a) The rights and obligations of the Company under your option will be transferable to any one or more persons or entities, and all covenants and agreements hereunder will inure to the benefit of, and be enforceable by the Company' s successors and assigns.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of your option.

(c) You acknowledge and agree that you have reviewed your option in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting your option, and fully understand all provisions of your option.

(d) This Option Agreement will be subject to all applicable laws, rules, and regulations, and to such approvals by any governmental agencies or national securities exchanges as may be required.

(e) All obligations of the Company under the Plan and this Option Agreement will be binding on any successor to the Company, whether the existence of such successor is the result of a direct or indirect purchase, merger, consolidation, or otherwise, of all or substantially all of the business and/or assets of the Company.

* * *

This Option Agreement will be deemed to be signed by you upon the signing by you of the Stock Option Grant Notice to which it is attached.

ATTACHMENT II

2019 EQUITY INCENTIVE PLAN

ATTACHMENT III

NOTICE OF EXERCISE

PREVAIL THERAPEUTICS INC.

Date of Exercise:

This constitutes notice to Prevail Therapeutics Inc. (the "***Company***") under my stock option that I elect to purchase the below number of shares of Common Stock of the Company (the "***Shares***") for the price set forth below.

Type of option (check one):		Incentive ☐	Nonstatutory ☐
Stock option dated:			
Number of Shares as to which option is exercised:			
Certificates to be issued in name of:			
Total exercise price:		$	$
Cash payment delivered herewith:		$	$
[Value of	Shares delivered herewith[1]:	$	$]
[Value of	Shares pursuant to net exercise[2]:	$	$]
[Regulation T Program (cashless exercise[3]):		$	$]

1 Shares must meet the public trading requirements set forth in the option. Shares must be valued in accordance with the terms of the option being exercised, and must be owned free and clear of any liens, claims, encumbrances or security interests. Certificates must be endorsed or accompanied by an executed assignment separate from certificate.

2 The option must be a Nonstatutory Stock Option, and the Company must have established net exercise procedures at the time of exercise, in order to utilize this payment method.

3 Shares must meet the public trading requirements set forth in the option.

By this exercise, I agree (i) to provide such additional documents as you may require pursuant to the terms of the Prevail Therapeutics Inc. 2019 Equity Incentive Plan, (ii) to provide for the payment by me to you (in the manner designated by you) of your withholding obligation, if any, relating to the exercise of this option, and (iii) if this exercise relates to an incentive stock option, to notify you in writing within fifteen (15) days after the date of any disposition of any of the Shares issued upon exercise of this option that occurs within two (2) years after the date of grant of this option or within one (1) year after such Shares are issued upon exercise of this option.

Very truly yours,

_____

PREVAIL THERAPEUTICS INC.

RESTRICTED STOCK UNIT GRANT NOTICE
(2019 EQUITY INCENTIVE PLAN)

Prevail Therapeutics Inc. (the "***Company***"), pursuant to its 2019 Equity Incentive Plan (the "***Plan***"), hereby awards to Participant a Restricted Stock Unit Award for the number of shares of the Company' s Common Stock ("***Restricted Stock Units***") set forth below (the "***Award***"). The Award is subject to all of the terms and conditions as set forth in this notice of grant (this "***Restricted Stock Unit Grant Notice***"), and in the Plan and the Restricted Stock Unit Award Agreement (the "***Award Agreement***"), both of which are attached hereto and incorporated herein in their entirety. Capitalized terms not explicitly defined herein shall have the meanings set forth in the Plan or the Award Agreement. In the event of any conflict between the terms in this Restricted Stock Unit Grant Notice or the Award Agreement and the Plan, the terms of the Plan shall control.

Participant:	_____
Date of Grant:	_____
Vesting Commencement Date:	_____
Number of Restricted Stock Units:	_____

Vesting Schedule: [, subject to Participant' s Continuous Service through each such vesting date.]

Issuance Schedule: Subject to any Capitalization Adjustment, one share of Common Stock (or its cash equivalent, at the discretion of the Company) will be issued for each Restricted Stock Unit that vests at the time set forth in Section 6 of the Award Agreement.

Additional Terms/Acknowledgements: Participant acknowledges receipt of, and understands and agrees to, this Restricted Stock Unit Grant Notice, the Award Agreement and the Plan. Participant further acknowledges that as of the Date of Grant, this Restricted Stock Unit Grant Notice, the Award Agreement and the Plan set forth the entire understanding between Participant and the Company regarding the acquisition of the Common Stock pursuant to the Award specified above and supersede all prior oral and written agreements on the terms of this Award, with the exception, if applicable, of (i) restricted stock unit awards or options previously granted and delivered to Participant, (ii) the written employment agreement, offer letter or other written agreement entered into between the Company and Participant specifying the terms that should govern this specific Award, and (iii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law.

By accepting this Award, Participant acknowledges having received and read the Restricted Stock Unit Grant Notice, the Award Agreement and the Plan and agrees to all of the terms and conditions set forth in these documents. Participant consents to receive Plan documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

PREVAIL THERAPEUTICS INC.	**PARTICIPANT**
By: _____	_____
Signature	Signature
Title: _____	Date: _____
Date: _____	

ATTACHMENTS: Award Agreement and 2019 Equity Incentive Plan

ATTACHMENT I

PREVAIL THERAPEUTICS INC.

2019 EQUITY INCENTIVE PLAN
RESTRICTED STOCK UNIT AWARD AGREEMENT

Pursuant to the Restricted Stock Unit Grant Notice (the "***Grant Notice***") and this Restricted Stock Unit Award Agreement (the "***Agreement***"), Prevail Therapeutics Inc. (the "***Company***") has awarded you ("***Participant***") a Restricted Stock Unit Award (the "***Award***") pursuant to the Company' s 2019 Equity Incentive Plan (the "***Plan***") for the number of Restricted Stock Units/shares indicated in the Grant Notice. Capitalized terms not explicitly defined in this Agreement or the Grant Notice shall have the same meanings given to them in the Plan. The terms of your Award, in addition to those set forth in the Grant Notice, are as follows.

1. GRANT OF THE AWARD. This Award represents the right to be issued on a future date one (1) share of Common Stock for each Restricted Stock Unit that vests on the applicable vesting date(s) (subject to any adjustment under Section 3 below) as indicated in the Grant Notice. As of the Date of Grant, the Company will credit to a bookkeeping account maintained by the Company for your benefit (the "***Account***") the number of Restricted Stock Units/shares of Common Stock subject to the Award. Notwithstanding the foregoing, the Company reserves the right to issue you the cash equivalent of Common Stock, in part or in full satisfaction of the delivery of Common Stock in connection with the vesting of the Restricted Stock Units, and, to the extent applicable, references in this Agreement and the Grant Notice to Common Stock issuable in connection with your Restricted Stock Units will include the potential issuance of its cash equivalent pursuant to such right. This Award was granted in consideration of your services to the Company.

2. VESTING. Subject to the limitations contained herein, your Award will vest, if at all, in accordance with the vesting schedule provided in the Grant Notice. Vesting will cease upon the termination of your Continuous Service and the Restricted Stock Units credited to the Account that were not vested on the date of such termination will be forfeited at no cost to the Company and you will have no further right, title or interest in or to such Award or the shares of Common Stock to be issued in respect of such portion of the Award.

3. NUMBER OF SHARES. The number of Restricted Stock Units subject to your Award may be adjusted from time to time for Capitalization Adjustments, as provided in the Plan. Any additional Restricted Stock Units, shares, cash or other property that becomes subject to the Award pursuant to this Section 3, if any, shall be subject, in a manner determined by the Board, to the same forfeiture restrictions, restrictions on transferability, and time and manner of delivery as applicable to the other Restricted Stock Units and shares covered by your Award. Notwithstanding the provisions of this Section 3, no fractional shares or rights for fractional shares of Common Stock shall be created pursuant to this Section 3. Any fraction of a share will be rounded down to the nearest whole share.

4. SECURITIES LAW COMPLIANCE. You may not be issued any Common Stock under your Award unless the shares of Common Stock underlying the Restricted Stock Units are either (i) then registered under the Securities Act, or (ii) the Company has determined that such issuance would be exempt from the registration requirements of the Securities Act. Your Award must also comply with other applicable laws and regulations governing the Award, and you shall not receive such Common Stock if the Company determines that such receipt would not be in material compliance with such laws and regulations.

5. TRANSFER RESTRICTIONS. Prior to the time that shares of Common Stock have been delivered to you, you may not transfer, pledge, sell or otherwise dispose of this Award or the shares issuable in respect of your Award, except as expressly provided in this Section 5. For example, you may not use shares that may be issued in respect of your Restricted Stock Units as security for a loan. The restrictions on transfer set forth herein will lapse upon delivery to you of shares in respect of your vested Restricted Stock Units.

(a) Death. Your Award is transferable by will and by the laws of descent and distribution. At your death, vesting of your Award will cease and your executor or administrator of your estate shall be entitled to receive, on behalf of your estate, any Common Stock or other consideration that vested but was not issued before your death.

(b) Domestic Relations Orders. Upon receiving written permission from the Board or its duly authorized designee, and provided that you and the designated transferee enter into transfer and other agreements required by the Company, you may transfer your right to receive the distribution of Common Stock or other consideration hereunder, pursuant to a domestic relations order, marital settlement agreement or other divorce or separation instrument as permitted by applicable law that contains the information required by the Company to effectuate the transfer. You are encouraged to discuss the proposed terms of any division of this Award with the Company General Counsel prior to finalizing the domestic relations order or marital settlement agreement to verify that you may make such transfer, and if so, to help ensure the required information is contained within the domestic relations order or marital settlement agreement.

6. DATE OF ISSUANCE.

(a) The issuance of shares in respect of the Restricted Stock Units is intended to comply with Treasury Regulations Section 1.409A-1(b)(4) and will be construed and administered in such a manner. Subject to the satisfaction of the Withholding Obligation set forth in Section 11 of this Agreement, in the event one or more Restricted Stock Units vests, the Company shall issue to you one (1) share of Common Stock for each Restricted Stock Unit that vests on the applicable vesting date(s) (subject to any adjustment under Section 3 above, and subject to any different provisions in the Grant Notice). Each issuance date determined by this paragraph is referred to as an "***Original Issuance Date***".

(b) If the Original Issuance Date falls on a date that is not a business day, delivery shall instead occur on the next following business day. In addition, if:

(i) the Original Issuance Date does not occur (1) during an "open window period" applicable to you, as determined by the Company in accordance with the Company' s then-effective policy on trading in Company securities, or (2) on a date when you are otherwise permitted to sell shares of Common Stock on an established stock exchange or stock market (including but not limited to under a previously established written trading plan that meets the requirements of Rule 10b5-1 under the Exchange Act and was entered into in compliance with the Company' s policies (a "***10b5-1 Arrangement***")), *and*

(ii) either (1) a Withholding Obligation does not apply, or (2) the Company decides, prior to the Original Issuance Date, (A) not to satisfy the Withholding Obligation by withholding shares of Common Stock from the shares otherwise due, on the Original Issuance Date, to you under this Award, and (B) not to permit you to enter into a "same day sale" commitment with a broker-dealer pursuant to Section 11 of this Agreement (including but not limited to a commitment under a 10b5-1 Arrangement) and (C) not to permit you to pay your Withholding Obligation in cash,

then the shares that would otherwise be issued to you on the Original Issuance Date will not be delivered on such Original Issuance Date and will instead be delivered on the first business day when you are not prohibited from selling shares of the Company' s Common Stock in the open public market, but in no event later than December 31 of the calendar year in which the Original Issuance Date occurs (that is, the last day of your taxable year in which the Original Issuance Date occurs), or, if and only if permitted in a manner that complies with Treasury Regulations Section 1.409A-1(b)(4), no later than the date that is the 15th day of the third calendar month of the applicable year following the year in which the shares of Common Stock under this Award are no longer subject to a "substantial risk of forfeiture" within the meaning of Treasury Regulations Section 1.409A-1(d).

(c) The form of delivery (*e.g.*, a stock certificate or electronic entry evidencing such shares) shall be determined by the Company.

7. DIVIDENDS. You shall receive no benefit or adjustment to your Award with respect to any cash dividend, stock dividend or other distribution that does not result from a Capitalization Adjustment; provided, however, that this sentence will not apply with respect to any shares of Common Stock that are delivered to you in connection with your Award after such shares have been delivered to you.

8. RESTRICTIVE LEGENDS. The shares of Common Stock issued in respect of your Award shall be endorsed with appropriate legends as determined by the Company.

9. EXECUTION OF DOCUMENTS. You hereby acknowledge and agree that the manner selected by the Company by which you indicate your consent to your Grant Notice is also deemed to be your execution of your Grant Notice and of this Agreement. You further agree that such manner of indicating consent may be relied upon as your signature for establishing your execution of any documents to be executed in the future in connection with your Award.

10. AWARD NOT A SERVICE CONTRACT.

(a) Nothing in this Agreement (including, but not limited to, the vesting of your Award or the issuance of the shares in respect of your Award), the Plan or any covenant of good faith and fair dealing that may be found implicit in this Agreement or the Plan shall: (i) confer upon you any right to continue in the employ or service of, or affiliation with, the Company or an Affiliate; (ii) constitute any promise or commitment by the Company or an Affiliate regarding the fact or nature of future positions, future work assignments, future compensation or any other term or condition of employment or affiliation; (iii) confer any right or benefit under this Agreement or the Plan unless such right or benefit has specifically accrued under the terms of this Agreement or Plan; or (iv) deprive the Company of the right to terminate you at will and without regard to any future vesting opportunity that you may have.

(b) By accepting this Award, you acknowledge and agree that the right to continue vesting in the Award pursuant to the vesting schedule provided in the Grant Notice may not be earned unless (in addition to any other conditions described in the Grant Notice and this Agreement) you continue as an employee, director or consultant at the will of the Company and affiliate, as applicable (not through the act of being hired, being granted this Award or any other award or benefit) and that the Company has the right to reorganize, sell, spin-out or otherwise restructure one or more of its businesses or Affiliates at any time or from time to time, as it deems appropriate (a "***reorganization***"). You acknowledge and agree that such a reorganization could result in the termination of your Continuous Service, or the termination of Affiliate status of your employer and the loss of benefits available to you under this Agreement, including but not limited to, the termination of the right to continue vesting in the Award. You further acknowledge and agree that this Agreement, the Plan, the transactions contemplated

hereunder and the vesting schedule set forth herein or any covenant of good faith and fair dealing that may be found implicit in any of them do not constitute an express or implied promise of continued engagement as an employee or consultant for the term of this Agreement, for any period, or at all, and shall not interfere in any way with the Company' s right to terminate your Continuous Service at any time, with or without your cause or notice, or to conduct a reorganization.

11. WITHHOLDING OBLIGATION.

(a) On each vesting date, and on or before the time you receive a distribution of the shares of Common Stock in respect of your Restricted Stock Units, and at any other time as reasonably requested by the Company in accordance with applicable tax laws, you hereby authorize any required withholding from the Common Stock issuable to you and/or otherwise agree to make adequate provision, including in cash, for any sums required to satisfy the federal, state, local and foreign tax withholding obligations of the Company or any Affiliate that arise in connection with your Award (the "***Withholding Obligation***").

(b) By accepting this Award, you acknowledge and agree that the Company or any Affiliate may, in its sole discretion, satisfy all or any portion of the Withholding Obligation relating to your Restricted Stock Units by any of the following means or by a combination of such means: (i) causing you to pay any portion of the Withholding Obligation in cash; (ii) withholding from any compensation otherwise payable to you by the Company; (iii) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to you in connection with the Award with a Fair Market Value (measured as of the date shares of Common Stock are issued pursuant to Section 6) equal to the amount of such Withholding Obligation; provided, however, that the number of such shares of Common Stock so withheld will not exceed the amount necessary to satisfy the Withholding Obligation using the maximum statutory withholding rates for federal, state, local and foreign tax purposes, including payroll taxes, that are applicable to supplemental taxable income; and *provided*, further, that to the extent necessary to qualify for an exemption from application of Section 16(b) of the Exchange Act, if applicable, such share withholding procedure will be subject to the express prior approval of the Board or the Company' s Compensation Committee; and/or (iv) permitting or requiring you to enter into a "same day sale" commitment, if applicable, with a broker-dealer that is a member of the Financial Industry Regulatory Authority (a "***FINRA Dealer***"), pursuant to this authorization and without further consent, whereby you irrevocably elect to sell a portion of the shares to be delivered in connection with your Restricted Stock Units to satisfy the Withholding Obligation and whereby the FINRA Dealer irrevocably commits to forward the proceeds necessary to satisfy the Withholding Obligation directly to the Company and/or its Affiliates. Unless the Withholding Obligation is satisfied, the Company shall have no obligation to deliver to you any Common Stock or any other consideration pursuant to this Award.

(c) In the event the Withholding Obligation arises prior to the delivery to you of Common Stock or it is determined after the delivery of Common Stock to you that the amount of the Withholding Obligation was greater than the amount withheld by the Company, you agree to indemnify and hold the Company harmless from any failure by the Company to withhold the proper amount.

12. TAX CONSEQUENCES. The Company has no duty or obligation to minimize the tax consequences to you of this Award and shall not be liable to you for any adverse tax consequences to you arising in connection with this Award. You are hereby advised to consult with your own personal tax, financial and/or legal advisors regarding the tax consequences of this Award and by signing the Grant Notice, you have agreed that you have done so or knowingly and voluntarily declined to do so. You understand that you (and not the Company) shall be responsible for your own tax liability that may arise as a result of this investment or the transactions contemplated by this Agreement.

13. UNSECURED OBLIGATION. Your Award is unfunded, and as a holder of a vested Award, you shall be considered an unsecured creditor of the Company with respect to the Company' s obligation, if any, to issue shares or other property pursuant to this Agreement. You shall not have voting or any other rights as a stockholder of the Company with respect to the shares to be issued pursuant to this Agreement until such shares are issued to you pursuant to Section 6 of this Agreement. Upon such issuance, you will obtain full voting and other rights as a stockholder of the Company. Nothing contained in this Agreement, and no action taken pursuant to its provisions, shall create or be construed to create a trust of any kind or a fiduciary relationship between you and the Company or any other person.

14. NOTICES. Any notice or request required or permitted hereunder shall be given in writing (including electronically) and will be deemed effectively given upon receipt or, in the case of notices delivered by mail by the Company to you, five (5) days after deposit in the United States mail, postage prepaid, addressed to you at the last address you provided to the Company. The Company may, in its sole discretion, decide to deliver any documents related to participation in the Plan and this Award by electronic means or to request your consent to participate in the Plan by electronic means. By accepting this Award, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

15. HEADINGS. The headings of the Sections in this Agreement are inserted for convenience only and shall not be deemed to constitute a part of this Agreement or to affect the meaning of this Agreement.

16. MISCELLANEOUS.

(a) The rights and obligations of the Company under your Award shall be transferable by the Company to any one or more persons or entities, and all covenants and agreements hereunder shall inure to the benefit of, and be enforceable by, the Company' s successors and assigns.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of your Award.

(c) You acknowledge and agree that you have reviewed your Award in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting your Award and fully understand all provisions of your Award.

(d) This Agreement shall be subject to all applicable laws, rules, and regulations, and to such approvals by any governmental agencies or national securities exchanges as may be required.

(e) All obligations of the Company under the Plan and this Agreement shall be binding on any successor to the Company, whether the existence of such successor is the result of a direct or indirect purchase, merger, consolidation, or otherwise, of all or substantially all of the business and/or assets of the Company.

17. GOVERNING PLAN DOCUMENT. Your Award is subject to all the provisions of the Plan, the provisions of which are hereby made a part of your Award, and is further subject to all interpretations, amendments, rules and regulations which may from time to time be promulgated and adopted pursuant to the Plan. Your Award (and any compensation paid or shares issued under your Award) is subject to recoupment in accordance with The Dodd‑ Frank Wall Street Reform and Consumer Protection Act and

any implementing regulations thereunder, any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law. No recovery of compensation under such a clawback policy will be an event giving rise to a right to voluntarily terminate employment upon a resignation for "good reason," or for a "constructive termination" or any similar term under any plan of or agreement with the Company.

18. EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of the Award subject to this Agreement shall not be included as compensation, earnings, salaries, or other similar terms used when calculating benefits under any employee benefit plan (other than the Plan) sponsored by the Company or any Affiliate except as such plan otherwise expressly provides. The Company expressly reserves its rights to amend, modify, or terminate any or all of the employee benefit plans of the Company or any Affiliate.

19. SEVERABILITY. If all or any part of this Agreement or the Plan is declared by any court or governmental authority to be unlawful or invalid, such unlawfulness or invalidity shall not invalidate any portion of this Agreement or the Plan not declared to be unlawful or invalid. Any Section of this Agreement (or part of such a Section) so declared to be unlawful or invalid shall, if possible, be construed in a manner which will give effect to the terms of such Section or part of a Section to the fullest extent possible while remaining lawful and valid.

20. OTHER DOCUMENTS. You hereby acknowledge receipt or the right to receive a document providing the information required by Rule 428(b)(1) promulgated under the Securities Act. In addition, you acknowledge receipt of the Company' s policy permitting certain individuals to sell shares only during certain "window" periods and the Company' s insider trading policy, in effect from time to time.

21. AMENDMENT. This Agreement may not be modified, amended or terminated except by an instrument in writing, signed by you and by a duly authorized representative of the Company. Notwithstanding the foregoing, this Agreement may be amended solely by the Board by a writing which specifically states that it is amending this Agreement, so long as a copy of such amendment is delivered to you, and provided that, except as otherwise expressly provided in the Plan, no such amendment materially adversely affecting your rights hereunder may be made without your written consent. Without limiting the foregoing, the Board reserves the right to change, by written notice to you, the provisions of this Agreement in any way it may deem necessary or advisable to carry out the purpose of the Award as a result of any change in applicable laws or regulations or any future law, regulation, ruling, or judicial decision, provided that any such change shall be applicable only to rights relating to that portion of the Award which is then subject to restrictions as provided herein.

22. COMPLIANCE WITH SECTION 409A OF THE CODE. This Award is intended to be exempt from the application of Section 409A of the Code, including but not limited to by reason of complying with the "short-term deferral" rule set forth in Treasury Regulation Section 1.409A-1(b)(4) and any ambiguities herein shall be interpreted accordingly. Notwithstanding the foregoing, if it is determined that the Award fails to satisfy the requirements of the short-term deferral rule and is otherwise not exempt from, and determined to be deferred compensation subject to Section 409A of the Code, this Award shall comply with Section 409A to the extent necessary to avoid adverse personal tax consequences and any ambiguities herein shall be interpreted accordingly. If it is determined that the Award is deferred compensation subject to Section 409A and you are a "Specified Employee" (within the meaning set forth in Section 409A(a)(2)(B)(i) of the Code) as of the date of your "Separation from Service" (as defined in Section 409A), then the issuance of any shares that would otherwise be made upon the date of your Separation from Service or within the first six (6) months thereafter will not be made on the originally scheduled date(s) and will instead be issued in a lump sum on the date that is six (6) months

and one day after the date of the Separation from Service, with the balance of the shares issued thereafter in accordance with the original vesting and issuance schedule set forth above, but if and only if such delay in the issuance of the shares is necessary to avoid the imposition of adverse taxation on you in respect of the shares under Section 409A of the Code. Each installment of shares that vests is intended to constitute a "separate payment" for purposes of Treasury Regulation Section 1.409A-2(b)(2).

* * * * *

This Restricted Stock Unit Award Agreement shall be deemed to be signed by the Company and the Participant upon the signing by the Participant of the Restricted Stock Unit Grant Notice to which it is attached.

ATTACHMENT II

2019 EQUITY INCENTIVE PLAN

Exhibit 10.6

AMENDED AND RESTATED EMPLOYMENT AGREEMENT

This **AMENDED AND RESTATED EMPLOYMENT AGREEMENT** (the "***Agreement***") is entered into effective as of (the "***Effective Date***"), by and between Asa Abeliovich ("***Executive***") and Prevail Therapeutics Inc. (the "***Company***").

Executive has been employed by the Company as its Chief Executive Officer pursuant to an offer letter with the Company dated September 15, 2017 (the "***Prior Agreement***").

The Company desires to continue to employ Executive and, in connection therewith, to compensate Executive for Executive' s personal services to the Company; and

Executive wishes to continue to be employed by the Company and provide personal services to the Company in return for certain compensation.

Accordingly, in consideration of the mutual promises and covenants contained herein, the parties agree to the following:

1. EMPLOYMENT BY THE COMPANY.

1.1 At-Will Employment. Executive shall continue to be employed by the Company on an "at-will" basis, meaning either the Company or Executive may terminate Executive' s employment at any time, with or without Cause (as defined in Section 6.2(f) below), Good Reason (as defined in Section 6.2(e) below), or advance notice. Any contrary representations that may have been made to Executive shall be superseded by this Agreement. This Agreement shall constitute the full and complete agreement between Executive and the Company on the "at-will" nature of Executive' s employment with the Company, which may be changed only in an express written agreement signed by Executive and a duly authorized officer of the Company. Executive' s rights to any salary or cash bonus following a termination shall be only as set forth in Section 6 or under any applicable benefit or equity plan.

1.2 Position; Board Role. Subject to the terms set forth herein, the Company agrees to continue to employ Executive and Executive hereby accepts such continued employment. In addition, Executive shall continue to serve as Chief Executive Officer. During the term of Executive' s employment with the Company, and excluding periods of vacation and sick leave to which Executive is entitled, Executive shall devote all business time and attention to the affairs of the Company necessary to discharge the responsibilities assigned hereunder, and shall use commercially reasonable efforts to perform faithfully and efficiently such responsibilities. Executive shall continue to serve as a Director of the Board of Directors of the Company (the "***Board***") at the pleasure of the Board in accordance with the governing documents and applicable law.

1.3 Duties. Executive will report to the Board and will render such business and professional services in the performance of Executive' s duties, consistent with Executive' s position as Chief Executive Officer, as shall reasonably be assigned to him by the Board, subject to the oversight and direction of the Board. Executive shall perform Executive' s duties under this Agreement principally out of the Company' s corporate headquarters in New York, New York, or such other location as assigned. In addition, Executive shall make such business trips to such places as may be reasonably necessary or advisable for the efficient operations of the Company.

1.4 Company Policies and Benefits. The employment relationship between the parties shall continue to be subject to the Company' s written personnel policies and procedures as they may be adopted, revised, or deleted from time to time in the Company' s sole discretion. Executive shall be expected to continue to comply with all applicable laws, regulations, rules, directives and other legal requirements of federal, state and other governmental and regulatory bodies having jurisdiction over the Company and of the professional bodies of which the Company is a member. During Executive' s employment with the Company, Executive continues to be required to maintain in good standing any licenses and certifications necessary for the performance of Executive' s duties for the Company. Executive will continue to be eligible to participate on the same basis as similarly-situated employees in the Company' s benefit plans in effect from time to time during Executive' s employment. Subject to the preceding sentence, the Company reserves the right to change, alter, or terminate any benefit plan in its sole discretion. All matters of eligibility for coverage or benefits under any benefit plan shall be determined in accordance with the provisions of such plan. Notwithstanding the foregoing, in the event that the terms of this Agreement differ from or are in conflict with the Company' s general employment policies or practices, this Agreement shall control.

2. COMPENSATION.

2.1 Salary. Executive shall receive an annualized base salary of $488,000, subject to review and adjustment from time to time by the Company in its sole discretion, payable subject to standard federal and state payroll withholding requirements in accordance with the Company' s standard payroll practices (the "***Base Salary***").

2.2 Bonus.

(a) During Employment. Executive shall be eligible to earn an annual performance bonus (the "***Annual Bonus***") with an annual target of 55% (the "***Target Percentage***") of Executive' s then-current Base Salary (the "***Target Bonus***"). The Annual Bonus will be based upon the assessment of the Board or a committee thereof of Executive' s performance and the Company' s attainment of targeted goals (as set by the Company and confirmed by the Board in its reasonable good faith discretion) over the applicable calendar year. The Annual Bonus, if any, will be subject to applicable payroll deductions and withholdings. No amount of any Annual Bonus is guaranteed at any time, and, except as otherwise stated in Sections 6.2(a)(iii) or 6.3(a)(iii), Executive must be an employee in good standing through the date the Annual Bonus is paid to be eligible to receive an Annual Bonus. No partial or prorated bonuses will be provided. Subject to Section 6.3(b) related to payments upon certain terminations of employment, any Annual Bonus, if earned, will be paid at the same time annual bonuses are generally paid to other similarly-situated employees of the Company. Executive' s eligibility for an Annual Bonus is subject to change in the discretion of the Board (or any authorized committee thereof).

(b) Upon Termination. Subject to the provisions of Section 6, in the event Executive leaves the employ of the Company for any reason prior to the date the Annual Bonus is paid, Executive is not eligible to earn such Annual Bonus, prorated or otherwise.

2.3 Future Equity Awards. Executive remains eligible to be considered for future equity awards as may be determined by the Board or a committee of the Board in its discretion in accordance with the terms of any applicable equity plan or arrangement that may be in effect from time to time.

2.4 Expense Reimbursement. The Company will reimburse Executive for reasonable business expenses in accordance with the Company' s standard expense reimbursement policy. For the avoidance of doubt, to the extent that any reimbursements payable to Executive are subject to the provisions of Section 409A of the Internal Revenue Code of 1986, as amended (the "***Code***"): (a) any such reimbursements will be paid no later than December 31 of the year following the year in which the expense was incurred, (b) the amount of expenses reimbursed in one year will not affect the amount eligible for reimbursement in any subsequent year, and (c) the right to reimbursement under this Agreement will not be subject to liquidation or exchange for another benefit.

3. CONFIDENTIAL INFORMATION, INVENTIONS, NON-SOLICITATION AND NON-COMPETITION OBLIGATIONS. In connection with Executive' s continued employment with the Company, Executive will continue to receive and continue to have access to the Company' s confidential information and trade secrets. Accordingly, and in consideration of the benefits that Executive is eligible to receive under this Agreement, Executive agrees to sign the Company' s Employee Confidential Information, Inventions, Non-Solicitation and Non-Competition Agreement (the "***Confidential Information Agreement***"), attached as **Exhibit A**, which contains restrictive covenants and prohibits unauthorized use or disclosure of the Company' s confidential information and trade secrets, among other obligations. The Confidential Information Agreement contains provisions that are intended by the parties to survive and do survive termination or expiration of this Agreement and will supersede, prospectively only, the agreement that Executive previously signed relating to the same subject matter.

4. OUTSIDE ACTIVITIES. Except with the prior written consent of the Board, Executive will not, while employed by the Company, undertake or engage in any other employment, occupation, or business enterprise that would interfere with Executive' s responsibilities and the performance of Executive' s duties hereunder except for (i) reasonable time devoted to volunteer services for or on behalf of such religious, educational, non-profit, and/or other charitable organization as Executive may wish to serve, (ii) reasonable time devoted to activities in the non-profit and business communities consistent with Executive' s position with the Company, (iii) reasonable time serving as trustee, director, or advisor to any family companies or trusts, or (iv) with prior written notice to the Board, reasonable time devoted to service as a member of the board of directors (or its equivalent in the case of a non-corporate entity) of a non-competing business; so long as the activities set forth in clauses (i), (ii), (iii), and (iv) do not interfere, individually or in the aggregate, with the performance of Executive' s duties for the Company, are not competitive with the business of the Company, will not otherwise result in Executive' s breach of the Confidential Information Agreement, or create a business or fiduciary conflict. This restriction shall not, however, preclude Executive from (x) owning less than one percent (1%) of the total outstanding shares of a publicly traded company, (y) managing Executive' s passive personal investments, or (z) employment or service in any capacity with Affiliates of the Company. As used in this Agreement, "***Affiliates***" means, at the time of determination, any "parent" or "subsidiary" of the Company as such terms are defined in Rule 405 of the Securities Act of 1933, as amended. The Board will have the authority to determine the time or times at which "parent" or "subsidiary" status is determined within the foregoing definition.

5. NO CONFLICT WITH EXISTING OBLIGATIONS. Executive represents that Executive' s performance of all the terms of this Agreement and continued service as an employee of the Company do not and will not breach any agreement or obligation of any kind made prior to Executive' s employment by the Company, including agreements or obligations Executive may have with prior employers or entities for which Executive has provided services. Executive has not entered into, and Executive agrees that Executive will not enter into, any agreement or obligation, either written or oral, in conflict herewith or with Executive' s duties to the Company.

6. TERMINATION OF EMPLOYMENT. The parties acknowledge that Executive' s employment relationship with the Company continues to be at-will. Either Executive or the Company may terminate the employment relationship at any time, with or without Cause (as defined below) or advance notice. The provisions in this Section govern the amount of compensation, if any, to be provided to Executive upon termination of employment and do not alter this at-will status.

6.1 Termination by Virtue of Death or Disability of Executive.

(a) In the event of Executive' s death while employed pursuant to this Agreement, all obligations of the parties hereunder and Executive' s employment shall terminate immediately, and the Company shall, pursuant to the Company' s standard payroll policies and applicable law, pay to Executive' s legal representatives the Accrued Obligations (as defined in Section 6.2(d) below) due to Executive.

(b) Subject to applicable state and federal law, the Company shall at all times have the right, upon written notice to Executive, to terminate this Agreement based on Executive' s Disability (as defined below). Termination by the Company of Executive' s employment based on "***Disability***" shall mean termination because Executive is unable due to a physical or mental condition to perform the essential functions of Executive' s position with or without reasonable accommodation for six (6) months in the aggregate during any twelve (12) month period or based on the written certification by two licensed physicians of the likely continuation of such condition for such period. This definition shall be interpreted and applied consistent with the Americans with Disabilities Act, the Family and Medical Leave Act, and other applicable law. In the event Executive' s employment is terminated based on Executive' s Disability, Executive will be entitled to the Accrued Obligations due to Executive.

6.2 Termination by the Company or Resignation by Executive.

(a) The Company shall have the right to terminate Executive' s employment pursuant to this Section 6.2 at any time (subject to any applicable cure period stated in Section 6.2(f)) with or without Cause or advance notice, by giving notice as described in Section 7.1 of this Agreement. Likewise, Executive can resign from employment with or without Good Reason, by giving notice as described in Section 7.1 of this Agreement. Executive hereby agrees to comply with the additional notice requirements set forth in Section 6.2(e) below for any resignation for Good Reason. If Executive is terminated by the Company (with or without Cause) or resigns from employment with the Company (with or without Good Reason), then Executive shall be entitled to the Accrued Obligations (as defined below). In addition, if Executive is terminated without Cause or resigns for Good Reason, and provided that such termination constitutes a "separation from service"

(as defined under Treasury Regulation Section 1.409A-1(h), without regard to any alternative definition thereunder, a "***Separation from Service***"), and further provided that Executive executes and allows to become effective a separation agreement that includes, among other terms, a general release of claims in favor of the Company and its Affiliates and representatives, substantially in the form attached hereto as **Exhibit B** (the "***Separation Agreement***"), as may be modified only to reflect changes in the law, and subject to Section 6.2(b) (the date that the general release of claims in the Separation Agreement becomes effective and may no longer be revoked by Executive is referred to as the "***Release Date***"), then Executive shall be eligible to receive the following severance benefits (collectively the "***Non-CIC Severance Benefits***"):

(i) An amount equal to twelve (12) months of Executive' s then current Base Salary, less standard payroll deductions and withholdings, paid in installments on the Company' s regular payroll dates;

(ii) Provided Executive or Executive' s covered dependents, as the case may be, timely elects continued coverage under COBRA under the Company' s group health plans following such termination, the portion of the COBRA premiums which is equal to the cost of the coverage that the Company was paying as of the date of termination, to continue Executive' s (and Executive' s covered dependents, as applicable) health insurance coverage in effect on the termination date until the earliest of: (1) twelve (12) months following the termination date; or (2) the date Executive ceases to be eligible for COBRA continuation coverage for any reason, including plan termination (such period from the termination date through the earlier of (1) and (2), (the "***COBRA Payment Period***")). Notwithstanding the foregoing, if at any time the Company determines that its payment of COBRA premiums on Executive' s behalf would result in a violation of applicable law (including, but not limited to, the 2010 Patient Protection and Affordable Care Act, as amended by the 2010 Health Care and Education Reconciliation Act), then in lieu of paying COBRA premiums pursuant to this Section, the Company shall pay Executive on the last day of each remaining month of the COBRA Payment Period, a fully taxable cash payment equal to 100% of the COBRA premium for such month, subject to applicable tax withholding, for the remainder of the COBRA Payment Period. Nothing in this Agreement shall deprive Executive of Executive' s rights under COBRA or ERISA for benefits under plans and policies arising under Executive' s employment by the Company; and

(iii) If Executive is terminated pursuant to this Section 6.2 between January 1 and the payment date of the Target Bonus, the Company will pay a lump sum cash payment in an amount equal to the amount of the Target Bonus that Executive would have otherwise earned for performance in the calendar year preceding Executive' s termination (the "***Bonus Severance***"). The Bonus Severance will be subject to standard payroll deductions and withholdings and will be paid on the first payroll date after the 60th day following Executive' s date of termination, provided that Executive has delivered an effective Separation Agreement as of such date.

(b) Executive shall not receive the Non-CIC Severance Benefits pursuant to Section 6.2(a) unless Executive executes the Separation Agreement within the consideration period specified therein, which shall in no event be more than forty-five (45) days, and until the Separation Agreement becomes effective and can no longer be revoked by Executive under its terms.

Executive' s ability to receive benefits pursuant to Section 6.2(a) is further conditioned upon Executive: (i) returning all Company property; (ii) complying with Executive' s post-termination obligations under this Agreement and the Confidential Information Agreement; (iii) complying with the Separation Agreement, including without limitation any non-disparagement and confidentiality provisions contained therein; and (iv) resignation from any other positions Executive holds with the Company, effective no later than Executive' s date of termination (or such other date as requested by the Board).

(c) The Company will not make any payments to Executive with respect to any of the benefits pursuant to Section 6.2(a) prior to the 60th day following Executive' s date of termination. On the first payroll date after the 60th day following Executive' s date of termination, and provided that Executive has delivered an effective Separation Agreement, the Company will make the first payment to Executive under Section 6.2(a)(i) and, in a lump sum, an amount equal to the aggregate amount of payments that the Company would have paid Executive through such date had the payments commenced on Executive' s date of termination through such 60th day, with the balance of the payments paid thereafter on the schedule described above, subject to any delay in payment required by Section 6.6.

(d) For purposes of this Agreement, "***Accrued Obligations***" are (i) Executive' s accrued but unpaid salary through the date of termination and, if required by applicable law and the Company' s applicable policy as of the time of termination, any accrued but unused vacation through the date of termination (both of which, for purpose of clarity, shall be paid in cash), (ii) any unreimbursed business expenses incurred by Executive payable in accordance with the Company' s standard expense reimbursement policies, and (iii) benefits owed to Executive under any qualified retirement plan or health and welfare benefit plan in which Executive was a participant in accordance with applicable law and the provisions of such plan.

(e) For purposes of this Agreement, "***Good Reason***" means any of the following actions taken by the Company without Executive' s express prior written consent: (i) a material reduction by the Company of Executive' s Base Salary (other than in a broad based reduction similarly affecting all other members of the Company' s executive management); (ii) a material breach by the Company of this Agreement or any other material written agreement between Executive and the Company concerning the terms and conditions of Executive' s employment; (iii) the relocation of Executive' s principal place of employment, without Executive' s consent, to a place that increases Executive' s one-way commute by more than twenty-five (25) miles as compared to Executive' s then-current principal place of employment immediately prior to such relocation; or (iv) a material reduction in Executive' s duties, authority, or responsibilities for the Company relative to Executive' s duties, authority, or responsibilities in effect immediately prior to such reduction; provided, however, that, any such termination by Executive shall only be deemed for Good Reason pursuant to this definition if: (1) Executive gives the Company written notice of Executive' s intent to terminate for Good Reason within thirty (30) days following Executive' s learning of the occurrence of the condition(s) that Executive believes constitute(s) Good Reason, which notice shall describe such condition(s); (2) the Company fails to remedy such condition(s) within thirty (30) days following receipt of the written notice (the "***Cure Period***"); and (3) Executive voluntarily terminates Executive' s employment within thirty (30) days following the end of the Cure Period. For the avoidance of doubt, any change in Executive' s title or the entity structure of the Company, in each case, without a corresponding material reduction in Executive' s duties, authority, or responsibilities, in accordance with clause (iv) above, shall not constitute Good Reason.

(f) For purposes of this Agreement, "***Cause***" for termination shall mean that Executive has engaged in any of the following: (i) a material breach of any covenant or condition under this Agreement or any other material agreement between the parties; (ii) any act constituting dishonesty, fraud, immoral or disreputable conduct which is reasonably likely to cause harm (including reputational harm) to the Company; (iii) any conduct which constitutes a felony under applicable law; (iv) material violation of any Company policy, after the expiration of ten (10) days without cure after written notice of such violation to the extent such violation is curable; (v) refusal to follow or implement a clear, lawful and reasonable directive of Company after the expiration of ten (10) days without cure after written notice of such failure to the extent such failure is curable; (vi) gross negligence or incompetence in the performance of Executive' s duties after the expiration of ten (10) days without cure after written notice of such failure; or (vii) breach of fiduciary duty to the Company.

(g) The benefits provided to Executive pursuant to this Section 6.2 are in lieu of, and not in addition to, any benefits to which Executive may otherwise be entitled under any Company severance plan, policy, or program.

(h) Any damages caused by the termination of Executive' s employment without Cause or for Good Reason would be difficult to ascertain; therefore, the Non-CIC Severance Benefits for which Executive is eligible pursuant to Section 6.2(a) above in exchange for the Separation Agreement is agreed to by the parties as liquidated damages, to serve as full compensation, and not a penalty.

(i) If the Company terminates Executive' s employment for Cause, or Executive resigns from employment with the Company without Good Reason, regardless of whether or not such termination is in connection with a Change in Control (as defined in the Company' s 2019 Equity Incentive Plan), then Executive shall be entitled to the Accrued Obligations, but Executive will not receive the Non-CIC Severance Benefits, the CIC Severance Benefits, or any other severance compensation or benefit.

6.3 Resignation by Executive for Good Reason or Termination by the Company without Cause (in connection with a Change in Control).

(a) In the event that the Company terminates Executive' s employment without Cause or Executive resigns for Good Reason within twelve (12) months following the effective date of a Change in Control ("***Change in Control Termination Date***"), then Executive shall be entitled to the Accrued Obligations and, subject to Executive' s compliance with Section 6.2(b) above, Executive shall be eligible to receive the following severance benefits (collectively the "***CIC Severance Benefits***"), subject to the terms and conditions set forth in Section 6.3(b):

(i) An amount equal to eighteen (18) months of Executive' s then current Base Salary, less standard payroll deductions and withholdings, paid in installments on the Company' s regular payroll dates;

(ii) Provided Executive or Executive' s covered dependents, as the case may be, timely elects continued coverage under COBRA under the Company' s group health plans following such termination, the portion of the COBRA premiums which is equal to the cost of the coverage that the Company was paying as of the date of termination, to continue Executive' s (and Executive' s covered dependents, as applicable) health insurance coverage in effect on the termination date until the earliest of: (1) eighteen (18) months following the termination date; or (2) the date Executive ceases to be eligible for COBRA continuation coverage for any reason, including plan termination (such period from the termination date through the earlier of (1) and (2), (the "***CIC COBRA Payment Period***"). Notwithstanding the foregoing, if at any time the Company determines that its payment of COBRA premiums on Executive' s behalf would result in a violation of applicable law (including, but not limited to, the 2010 Patient Protection and Affordable Care Act, as amended by the 2010 Health Care and Education Reconciliation Act), then in lieu of paying COBRA premiums pursuant to this Section, the Company shall pay Executive on the last day of each remaining month of the CIC COBRA Payment Period, a fully taxable cash payment equal to 100% of the COBRA premium for such month, subject to applicable tax withholding, for the remainder of the CIC COBRA Payment Period. Nothing in this Agreement shall deprive Executive of Executive' s rights under COBRA or ERISA for benefits under plans and policies arising under Executive' s employment by the Company;

(iii) A lump sum cash payment in an amount equal to one and a half (1.5) times the Target Bonus for the year in which the termination occurs, subject to standard payroll deductions and withholdings, which will be paid on the first payroll date after the 60th day following Executive' s date of termination, provided that Executive has delivered an effective Separation Agreement as of such date; and

(iv) Effective as of Executive' s Change in Control Termination Date, the vesting and exercisability of all outstanding equity awards held by Executive immediately prior to the Change in Control Termination Date shall be accelerated in full.

(b) The Company will not make any payments to Executive with respect to any of the benefits pursuant to Section 6.3(a) prior to the 60th day following Executive' s date of termination. On the first payroll date after the 60th day following Executive' s date of termination, and provided that Executive has delivered an effective Separation Agreement, the Company will (i) make the first payment to Executive under Section 6.2(a)(i) and, in a lump sum, an amount equal to the aggregate amount of payments that the Company would have paid Executive through such date had the payments commenced on Executive' s date of termination through such 60th day, with the balance of the payments paid thereafter on the schedule described above; and (ii) make the lump sum payment specified in Section 6.3(a)(iii) that has not yet been made due to this Section 6.3(b), in the cases of (i) and (ii) subject to any delay in payment required by Section 6.6.

(c) The benefits provided to Executive pursuant to this Section 6.3 are in lieu of, and not in addition to, any benefits to which Executive may otherwise be entitled under any Company severance plan, policy, or program.

(d) Any damages caused by the termination of Executive' s employment without Cause or for Good Reason in connection with a Change in Control would be difficult to ascertain; therefore, the CIC Severance Benefits for which Executive is eligible pursuant to Section 6.3(a) above in exchange for the Separation Agreement is agreed to by the parties as liquidated damages, to serve as full compensation, and not a penalty.

6.4 Cooperation With the Company After Termination of Employment. Following termination of Executive' s employment for any reason, Executive shall reasonably cooperate with the Company in all matters relating to the winding up of Executive' s pending work including, but not limited to, any litigation in which the Company is involved, and the orderly transfer of any such pending work to such other executives as may be designated by the Company; provided, that the Company agrees that the Company (a) shall make reasonable efforts to minimize disruption of Executive' s other activities, and (b) shall reimburse Executive for all reasonable expenses incurred in connection with such cooperation.

6.5 Effect of Termination. Executive agrees that should Executive' s employment be terminated for any reason, Executive shall be deemed to have resigned from any and all positions with the Company, including, but not limited to, a position on the Board and all positions with any and all subsidiaries and Affiliates of the Company.

6.6 Application of Section 409A.

(a) It is intended that all of the compensation payable under this Agreement, to the greatest extent possible, either complies with the requirements of Section 409A of the Code and the regulations and other guidance thereunder and any state law of similar effect (collectively, "***Section 409A***") or satisfies one or more of the exemptions from the application of Section 409A, and this Agreement will be construed in a manner consistent with such intention, incorporating by reference all required definitions and payment terms.

(b) No severance payments will be made under this Agreement unless Executive' s termination of employment constitutes a Separation from Service. For purposes of Section 409A (including, without limitation, for purposes of Treasury Regulations Section 1.409A-2(b)(2)(iii)), Executive' s right to receive any installment payments under this Agreement (whether severance payments or otherwise) shall be treated as a right to receive a series of separate payments and, accordingly, each installment payment hereunder shall at all times be considered a separate and distinct payment.

(c) To the extent that any severance payments are deferred compensation under Section 409A, and are not otherwise exempt from the application of Section 409A, then, to the extent required to comply with Section 409A, if the period during which Executive may consider and sign the Separation Agreement spans two calendar years, the severance payments will not begin until the second calendar year. If the Company determines that the severance benefits provided under this Agreement constitutes "deferred compensation" under Section 409A and if Executive is a "specified employee" of the Company, as such term is defined in Section 409A(a)(2)(B)(i) of the Code at the time of Executive' s Separation from Service, then, solely to the extent necessary to avoid the incurrence of the adverse personal tax consequences under Section 409A, the timing of the severance will be delayed as follows: on the earlier to occur of (a) the date that is six months and one day after

Executive' s Separation from Service, and (b) the date of Executive' s death, the Company will: (i) pay to Executive a lump sum amount equal to the sum of the severance benefits that Executive would otherwise have received if the commencement of the payment of the severance benefits had not been delayed pursuant to this Section 6.6(c); and (ii) commence paying the balance of the severance benefits in accordance with the applicable payment schedule set forth in Sections 6.2 and 6.3. No interest shall be due on any amounts deferred pursuant to this Section 6.6(c).

(d) To the extent required to avoid accelerated taxation and/or tax penalties under Section 409A, amounts reimbursable to Executive under this Agreement shall be paid to Executive on or before the last day of the year following the year in which the expense was incurred and the amount of expenses eligible for reimbursement (and in-kind benefits provided to Executive) during any one year may not effect amounts reimbursable or provided in any subsequent year. The Company makes no representation that compensation paid pursuant to the terms of this Agreement will be exempt from or comply with Section 409A and makes no undertaking to preclude Section 409A from applying to any such payment.

6.7 Excise Tax Adjustment.

(a) If any payment or benefit Executive will or may receive from the Company or otherwise (a "***280G Payment***") would (i) constitute a "parachute payment" within the meaning of Section 280G of the Code, and (ii) but for this Section, be subject to the excise tax imposed by Section 4999 of the Code (the "***Excise Tax***"), then any such 280G Payment provided pursuant to this Agreement (a "***Payment***") shall be equal to the Reduced Amount. The "***Reduced Amount***" shall be either (x) the largest portion of the Payment that would result in no portion of the Payment (after reduction) being subject to the Excise Tax, or (y) the largest portion, up to and including the total, of the Payment, whichever amount (i.e., the amount determined by clause (x) or by clause (y)), after taking into account all applicable federal, state, and local employment taxes, income taxes, and the Excise Tax (all computed at the highest applicable marginal rate), results in Executive' s receipt, on an after-tax basis, of the greater economic benefit notwithstanding that all or some portion of the Payment may be subject to the Excise Tax. If a reduction in a Payment is required pursuant to the preceding sentence and the Reduced Amount is determined pursuant to clause (x) of the preceding sentence, the reduction shall occur in the manner (the "***Reduction Method***") that results in the greatest economic benefit for Executive. If more than one method of reduction will result in the same economic benefit, the items so reduced will be reduced pro rata (the "***Pro Rata Reduction Method***").

(b) Notwithstanding any provision of this Section 6.7 to the contrary, if the Reduction Method or the Pro Rata Reduction Method would result in any portion of the Payment being subject to taxes pursuant to Section 409A that would not otherwise be subject to taxes pursuant to Section 409A, then the Reduction Method and/or the Pro Rata Reduction Method, as the case may be, shall be modified so as to avoid the imposition of taxes pursuant to Section 409A as follows: (A) as a first priority, the modification shall preserve to the greatest extent possible, the greatest economic benefit for Executive as determined on an after-tax basis; (B) as a second priority, Payments that are contingent on future events (*e.g.*, being terminated without Cause), shall be reduced (or eliminated) before Payments that are not contingent on future events; and (C) as a third priority, Payments that are "deferred compensation" within the meaning of Section 409A shall be reduced (or eliminated) before Payments that are not deferred compensation within the meaning of Section 409A.

(c) Unless Executive and the Company agree on an alternative accounting firm or law firm, the accounting firm engaged by the Company for general tax compliance purposes as of the day prior to the effective date of the Change in Control transaction shall perform the foregoing calculations. If the accounting firm so engaged by the Company is serving as accountant or auditor for the individual, entity, or group effecting the Change in Control transaction, the Company shall appoint a nationally recognized accounting or law firm to make the determinations required by this Section 6.7. The Company shall bear all expenses with respect to the determinations by such accounting or law firm required to be made hereunder. The Company shall use commercially reasonable efforts to cause the accounting or law firm engaged to make the determinations hereunder to provide its calculations, together with detailed supporting documentation, to Executive and the Company within fifteen (15) calendar days after the date on which Executive' s right to a 280G Payment becomes reasonably likely to occur (if requested at that time by Executive or the Company) or such other time as requested by Executive or the Company.

(d) If Executive receives a Payment for which the Reduced Amount was determined pursuant to clause (x) of Section 6.7(a) and the Internal Revenue Service determines thereafter that some portion of the Payment is subject to the Excise Tax, Executive agrees to promptly return to the Company a sufficient amount of the Payment (after reduction pursuant to clause (x) of Section 6.7(a)) so that no portion of the remaining Payment is subject to the Excise Tax. For the avoidance of doubt, if the Reduced Amount was determined pursuant to clause (y) of Section 6.7(a), Executive shall have no obligation to return any portion of the Payment pursuant to the preceding sentence.

7. GENERAL PROVISIONS.

7.1 Notices. Any notices required hereunder shall be in writing and shall be deemed effectively given: (a) upon personal delivery to the party to be notified, (b) when sent by electronic mail or confirmed facsimile if sent during normal business hours of the recipient, and if not, then on the next business day, (c) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid, or (d) one (1) day after deposit with a nationally recognized overnight courier, specifying next day delivery, with written verification of receipt. All communications shall be sent to the Company at its primary office location and to Executive at Executive' s address as listed on the Company payroll or (if notice is given prior to Executive' s termination of employment) to Executive' s Company-issued email address, or at such other address as the Company or Executive may designate by ten (10) days' advance written notice to the other.

7.2 Severability. Whenever possible, each provision of this Agreement will be interpreted in such manner as to be effective and valid under applicable law, but if any provision of this Agreement is held to be invalid, illegal, or unenforceable in any respect under any applicable law or rule in any jurisdiction, such invalidity, illegality, or unenforceability will not affect any other provision or any other jurisdiction, but this Agreement will be reformed, construed, and enforced in such jurisdiction as if such invalid, illegal, or unenforceable provisions had never been contained herein.

7.3 Waiver. If either party should waive any breach of any provisions of this Agreement, Executive or the Company shall not thereby be deemed to have waived any preceding or succeeding breach of the same or any other provision of this Agreement.

7.4 Complete Agreement. This Agreement (including Exhibit A), and any other separate agreement relating to equity awards constitute the entire agreement between Executive and the Company with regard to the subject matter hereof and supersede any prior oral discussions or written communications and agreements, including the Prior Agreement. This Agreement is entered into without reliance on any promise or representation other than those expressly contained herein, and it cannot be modified or amended except in writing signed by Executive and an authorized officer of the Company.

7.5 Counterparts. This Agreement may be executed by electronic transmission and in separate counterparts, any one of which need not contain signatures of more than one party, but all of which taken together will constitute one and the same Agreement.

7.6 Headings. The headings of the sections hereof are inserted for convenience only and shall not be deemed to constitute a part hereof nor to affect the meaning thereof.

7.7 Successors and Assigns. The Company shall assign this Agreement and its rights and obligations hereunder in whole, but not in part, to any company or other entity with or into which the Company may hereafter merge or consolidate or to which the Company may transfer all or substantially all of its assets, if in any such case said company or other entity shall by operation of law or expressly in writing assume all obligations of the Company hereunder as fully as if it had been originally made a party hereto, but may not otherwise assign this Agreement or its rights and obligations hereunder. Executive may not assign or transfer this Agreement or any rights or obligations hereunder, other than to Executive' s estate upon Executive' s death.

7.8 Choice of Law. All questions concerning the construction, validity, and interpretation of this Agreement will be governed by the laws of the State of New York.

7.9 Resolution of Disputes. To ensure the timely and economical resolution of disputes that may arise in connection with Executive' s employment with the Company, Executive and the Company agree that any and all disputes, claims, or causes of action arising from or relating to the enforcement, breach, performance, negotiation, execution, or interpretation of this Agreement, or Executive' s employment, or the termination of Executive' s employment, including but not limited to all statutory claims, will be resolved pursuant to the Federal Arbitration Act, 9 U.S.C. §1-16, and to the fullest extent permitted by law, by final, binding and confidential arbitration by a single arbitrator conducted in New York, New York by Judicial Arbitration and Mediation Services Inc. ("JAMS") under the then applicable JAMS rules (at the following web address: https://www.jamsadr.com/rules-employment-arbitration/); provided, however, this arbitration provision shall not apply to sexual harassment claims to the extent prohibited by applicable law. A hard copy of the rules will be provided to Executive upon request. **By agreeing to this arbitration procedure, both Executive and the Company waive the right to resolve any such dispute through a trial by jury or judge or administrative proceeding.** In addition, all claims, disputes, or causes of action under this provision, whether by Executive or the Company, must be brought in an individual capacity, and shall not be brought as a plaintiff (or claimant) or class member in any purported class or representative proceeding, nor joined or consolidated with the claims of any other person or entity. The arbitrator may not consolidate the claims of more than one person or entity, and may not preside over any form of representative or class proceeding. To the extent that the preceding sentences regarding class claims or proceedings are found to violate applicable law or are otherwise

found unenforceable, any claim(s) alleged or brought on behalf of a class shall proceed in a court of law rather than by arbitration. The Company acknowledges that Executive will have the right to be represented by legal counsel at any arbitration proceeding. Questions of whether a claim is subject to arbitration under this Agreement shall be decided by the arbitrator. Likewise, procedural questions which grow out of the dispute and bear on the final disposition are also matters for the arbitrator. The arbitrator shall: (a) have the authority to compel adequate discovery for the resolution of the dispute and to award such relief as would otherwise be permitted by law; (b) issue a written arbitration decision, to include the arbitrator' s essential findings and conclusions and a statement of the award; (c) be authorized to award any or all remedies that Executive or the Company would be entitled to seek in a court of law; and (d) is authorized to award attorneys' fees to the prevailing party. Subject to the foregoing sentence, Executive and the Company shall equally share all JAMS' arbitration fees and each party is responsible for its own attorneys' fees. Nothing in this Agreement is intended to prevent either Executive or the Company from obtaining injunctive relief in court to prevent irreparable harm pending the conclusion of any such arbitration. Any awards or orders in such arbitrations may be entered and enforced as judgments in the federal and state courts of any competent jurisdiction. To the extent applicable law prohibits mandatory arbitration of sexual harassment claims, in the event Executive intends to bring multiple claims, including a sexual harassment claim, the sexual harassment claim may be publicly filed with a court, while any other claims will remain subject to mandatory arbitration.

[Remainder of page intentionally left blank.]

IN WITNESS WHEREOF, the parties have executed this Employment Agreement on the day and year first written above.

PREVAIL THERAPEUTICS INC.

By: _____
Name:
Title:

EXECUTIVE:

_____
Asa Abeliovich

Exhibit A

EMPLOYEE CONFIDENTIAL INFORMATION, INVENTIONS, NON-SOLICITATION AND NON-COMPETITION AGREEMENT

Exhibit B
Release Agreement

This Release Agreement ("***Release***" or "***Agreement'***) is made by and between Asa Abeliovich ("***you***") and Prevail Therapeutics Inc. (the "***Company***"). A copy of this Release is an attachment to the Employment Agreement between the Company and you dated (the "***Employment Agreement***"). Capitalized terms not defined in this Agreement carry the definition found in the Employment Agreement.

1. Severance Payments; Other Payments.

a. In consideration for your execution, return and non-revocation of this Release on or after your last day of employment (the "***Separation Date***"), the Company will provide you with the **[Non-CIC Severance Benefits/CIC Severance Benefits]** described in Section **[6.2(a)/6.3(a)]** of the Employment Agreement (the "***Severance Benefits***").

b. In addition, regardless of whether you sign this Agreement, the Company affirms that it will pay the following on the next regularly scheduled date on which payroll is run, or sooner if required by applicable law, as required under Section 6 of the Employment Agreement: to include payment of all salary, business expense reimbursements and other amounts due to employee that are not part of the severance.

2. Compliance with Section 409A. The Severance Benefits offered to you by the Company are payable in reliance on Treasury Regulation Section 1.409A-1(b)(9) and the short term deferral exemption in Treasury Regulation Section 1.409A-1(b)(4). For purposes of Code Section 409A, your right to receive any installment payments (whether pay in lieu of notice, Severance Benefits, reimbursements or otherwise) shall be treated as a right to receive a series of separate payments and, accordingly, each installment payment shall at all times be considered a separate and distinct payment. All payments and benefits are subject to applicable withholdings and deductions.

3. Release. In exchange for the Severance Benefits and other consideration, to which you would not otherwise be entitled, and except as otherwise set forth in this Agreement, you, on behalf of yourself and, to the extent permitted by law, on behalf of your spouse, heirs, executors, administrators, assigns, insurers, attorneys and other persons or entities, acting or purporting to act on your behalf (collectively, the "***Employee Parties***"), hereby generally and completely release, acquit and forever discharge the Company, its parents and subsidiaries, and its and their officers, directors, managers, partners, agents, representatives, employees, attorneys, shareholders, predecessors, successors, assigns, insurers and affiliates (the "***Company Parties***") of and from any and all claims, liabilities, demands, contentions, actions, causes of action, suits, costs, expenses, attorneys' fees, damages, indemnities, debts, judgments, levies, executions and obligations of every kind and nature, in law, equity, or otherwise, both known and unknown, suspected and unsuspected, disclosed and undisclosed, arising out of or in any way related to my employment with the Company and separation therefrom, arising at any time prior to and including the execution date of this Agreement, including but not limited to: all such claims and demands directly or indirectly arising out of or in any way connected with your employment with the Company or the termination of that employment; claims or demands related to salary, bonuses, commissions, vacation pay, the right to receive additional grants of stock, stock options or other ownership interests in the Company, fringe

benefits, expense reimbursements, severance pay, or any other form of compensation; claims pursuant to any federal, state or local law, statute, or cause of action; tort law; or contract law (individually a "***Claim***" and collectively "***Claims***"). The Claims you are releasing and waiving in this Agreement include, but are not limited to, any and all Claims that any of the Company Parties:

has violated its personnel policies, handbooks, contracts of employment, or covenants of good faith and fair dealing;

has discriminated against you on the basis of age, race, color, sex (including sexual harassment), national origin, ancestry, disability, religion, sexual orientation, marital status, parental status, source of income, entitlement to benefits, any union activities or other protected category in violation of any local, state or federal law, constitution, ordinance, or regulation, including but not limited to: the Age Discrimination in Employment Act, as amended ("***ADEA***"); Title VII of the Civil Rights Act of 1964, as amended; the Civil Rights Act of 1991; 42 U.S.C. § 1981, as amended; the Equal Pay Act; the Americans With Disabilities Act; the Genetic Information Nondiscrimination Act; the Family and Medical Leave Act; the New York State Human Rights Law, the New York Equal Opportunity for Disabled Persons Act; the New York City Human Rights Law; the Employee Retirement Income Security Act; the Employee Polygraph Protection Act; the Worker Adjustment and Retraining Notification Act; the Older Workers Benefit Protection Act; the anti-retaliation provisions of the Sarbanes-Oxley Act, or any other federal or state law regarding whistleblower retaliation; the Lilly Ledbetter Fair Pay Act; the Uniformed Services Employment and Reemployment Rights Act; the Fair Credit Reporting Act; and the National Labor Relations Act; and

has violated any statute, public policy or common law (including, but not limited to, Claims for retaliatory discharge; negligent hiring, retention or supervision; defamation; intentional or negligent infliction of emotional distress and/or mental anguish; intentional interference with contract; negligence; detrimental reliance; loss of consortium to you or any member of your family and/or promissory estoppel).

Notwithstanding the foregoing, other than events expressly contemplated by this Agreement you do not waive or release rights or Claims that may arise: (i) from events that occur after the date this Release is executed; (ii) that relate to a breach of this Agreement; (iii) that relate to any existing ownership interest in the Company as of the date this Release is executed; (iv) that relate to your existing rights under any Company benefit plan or any plan or agreement related to equity ownership in the Company that arise after this Release is executed; and (v) any Claims which cannot be waived by law, including, without limitation, any rights you may have under applicable workers' compensation laws. Nothing in this Agreement shall prevent you from filing, cooperating with, or participating in any proceeding or investigation before the Equal Employment Opportunity Commission, United States Department of Labor, the National Labor Relations Board, the Occupational Safety and Health Administration, the Securities and Exchange Commission or any other federal government agency, or similar state or local agency ("***Government Agencies***"), or exercising any rights pursuant to Section 7 of the National Labor Relations Act. You further understand this Agreement does not limit your ability to voluntarily communicate with any Government Agencies or otherwise participate in any investigation or proceeding that may be

conducted by any Government Agency, including providing documents or other information, without notice to the Company. While this Agreement does not limit your right to receive an award for information provided to the Securities and Exchange Commission, you understand and agree that, you are otherwise waiving, to the fullest extent permitted by law, any and all rights you may have to individual relief based on any Claims that you have released and any rights you have waived by signing this Agreement. If any Claim is not subject to release, to the extent permitted by law, you waive any right or ability to be a class or collective action representative or to otherwise participate in any putative or certified class, collective or multi-party action or proceeding based on such a Claim in which any of the Company Parties is a party.

4. Your Acknowledgments and Affirmations. You also acknowledge and agree that (i) the consideration given to you in exchange for the waiver and release in this Agreement is in addition to anything of value to which you were already entitled, and (ii) that you have been paid for all time worked, have received all the leave, leaves of absence and leave benefits and protections for which you are eligible, and have not suffered any on-the-job injury for which you have not already filed a Claim. You affirm that all of the decisions of the Company Parties regarding your pay and benefits through the date of your execution of this Agreement were not discriminatory based on age, disability, race, color, sex, religion, national origin or any other classification protected by law. You affirm that you have not filed or caused to be filed, and are not presently a party to, a Claim against any of the Company Parties. You further affirm that you have no known workplace injuries or occupational diseases. You acknowledge and affirm that you have not been retaliated against for reporting any allegation of corporate fraud or other wrongdoing by any of the Company Parties, or for exercising any rights protected by law, including any rights protected by the Fair Labor Standards Act, the Family Medical Leave Act or any related statute or local leave or disability accommodation laws, or any applicable state workers' compensation law. In addition, you acknowledge that you are knowingly and voluntarily waiving and releasing any rights you may have under the ADEA ("***ADEA Waiver***"). You also acknowledge that the consideration given for the ADEA Waiver is in addition to anything of value to which you were already entitled. You further acknowledge that you have been advised by this writing, as required by the ADEA, that: (a) your release and waiver herein does not apply to any rights or claims that arise after the date you sign this Agreement; (b) you should consult with an attorney prior to signing this Agreement; (c) you have **[twenty-one (21)/forty-five (45)]** days to consider this Agreement (although you may choose to voluntarily sign it sooner); (d) you have seven (7) days following the date you sign this Agreement to revoke it; and (e) the Agreement will not be effective until the date upon which the revocation period has expired unexercised, which will be the eighth (8th) day after you sign this Agreement.

5. Return of Company Property. Within five (5) days following the Separation Date, you agree to return to the Company all Company documents (and all copies thereof) and other Company property that you have had in your possession at any time, including, but not limited to, Company files, notes, drawings, records, business plans and forecasts, financial information, specifications, computer-recorded information, tangible property (including, but not limited to, computers), credit cards, entry cards, identification badges and keys; and, any materials of any kind that contain or embody any proprietary or confidential information of the Company (and all reproductions thereof). Please coordinate return of Company property with [ ]. Receipt of the Severance Benefits described in Section 1 of this Agreement is expressly conditioned upon return of all Company property.

6. Confidential Information, Non-Competition and Non-Solicitation Obligations. Both during and after your employment you acknowledge your continuing obligations under your Employee Confidential Information, Inventions, Non-Solicitation and Non-Competition Agreement not to use or disclose any confidential or proprietary information of the Company and to comply with your post-employment non-competition and non-solicitation restrictions. The Company acknowledges that you will not be held criminally or civilly liable under any federal or state trade secret law for the disclosure of a trade secret that: (A) is made (i) in confidence to a federal, state, or local government official, either directly or indirectly, or to an attorney and (ii) solely for the purpose of reporting or investigating a suspected violation of law; or (B) is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal. In addition, in the event that you file a lawsuit for retaliation by the Company for reporting a suspected violation of law, you may disclose the trade secret to your attorney and use the trade secret information in the court proceeding, if you: (A) file any document containing the trade secret under seal; and (B) do not disclose the trade secret, except pursuant to court order.

7. Confidentiality. The provisions of this Agreement will be held in strictest confidence by you and will not be publicized or disclosed in any manner whatsoever; *provided, however*, that (a) you may disclose this Agreement to your immediate family; (b) you may disclose this Agreement in confidence to your attorney, accountant, auditor, tax preparer, and financial advisor, and (c) you may disclose this Agreement insofar as such disclosure may be required by law. Notwithstanding the foregoing, nothing in this Agreement shall limit your right to voluntarily communicate with the Equal Employment Opportunity Commission, United States Department of Labor, the National Labor Relations Board, the Securities and Exchange Commission, other federal government agency or similar state or local agency or to discuss the terms and conditions of your employment with others to the extent expressly permitted by Section 7 of the National Labor Relations Act.

8. Non-Disparagement. You and the Company agree not to disparage each other, and the other' s attorneys, directors, managers, partners, employees, agents and affiliates, in any manner likely to be harmful to them or their business, business reputation or personal reputation; provided that you and the Company will respond accurately and folly to any question, inquiry or request for information when required by legal process. The Company' s obligations under this Section are limited to Company representatives with knowledge of this provision. Notwithstanding the foregoing, nothing in this Agreement shall limit your right to voluntarily communicate with the Equal Employment Opportunity Commission, United States Department of Labor, the National Labor Relations Board, the Securities and Exchange Commission, other federal government agency or similar state or local agency or to discuss the terms and conditions of your employment with others to the extent expressly permitted by Section 7 of the National Labor Relations Act.

9. Cooperation. You agree to reasonably cooperate with the Company in all matters relating to the winding up of your pending work including, but not limited to, any litigation in which the Company is involved, and the orderly transfer of any such pending work to such other executives as may be designated by the Company; provided, that the Company agrees that the Company (a) shall make reasonable efforts to minimize disruption of your other activities, and (b) shall reimburse you for all reasonable expenses incurred in connection with such cooperation.

10. No Admission. This Agreement does not constitute an admission by you or by the Company of any wrongful action or violation of any federal, state, or local statute, or common law rights, including those relating to the provisions of any law or statute concerning employment actions, or of any other possible or claimed violation of law or rights.

11. Breach. You agree that upon any material breach .of this Agreement you will forfeit all amounts paid or owing to you under this Agreement. Further, you acknowledge that it may be impossible to assess the damages caused by your violation of the terms of Sections 5, 6, 7 and 8 of this Agreement and further agree that any threatened or actual violation or breach of those Sections of this Agreement will constitute immediate and irreparable injury to the Company. You therefore agree that, in addition to any and all other damages and remedies available to the Company upon your breach of this Agreement, the Company shall be entitled to an injunction to prevent you from violating or breathing this Agreement.

12. Miscellaneous. This Agreement is entered into without reliance on any promise or representation, written or oral, other than those expressly contained herein, and it supersedes any other such promises, warranties or representations. This Agreement may not be modified or amended except in a writing signed by both you and a duly authorized officer of the Company. This Agreement will bind the heirs, personal representatives, successors and assigns of both you and the Company, and inure to the benefit of both you and the Company, their heirs, successors and assigns. If any provision of this Agreement is determined to be invalid or unenforceable, in whole or in part, this determination will not affect any other provision of this Agreement and the provision in question will be modified by the court so as to be rendered enforceable. This Agreement will be deemed to have been entered into and will be construed and enforced in accordance with the laws of the State of New York as applied to contracts made and to be performed entirely within the State of New York.

Prevail Therapeutics Inc.

By: _____
Name:
Title:

_____
Asa Abeliovich

Exhibit 10.7

AMENDED AND RESTATED EMPLOYMENT AGREEMENT

This **AMENDED AND RESTATED EMPLOYMENT AGREEMENT** (the "***Agreement***") is entered into effective as of (the "***Effective Date***"), by and between Jeff Sevigny ("***Executive***") and Prevail Therapeutics Inc. (the "***Company***").

Executive has been employed by the Company as its Chief Medical Officer pursuant to an offer letter with the Company dated March 1, 2018 (the "***Prior Agreement***").

The Company desires to continue to employ Executive and, in connection therewith, to compensate Executive for Executive' s personal services to the Company; and

Executive wishes to continue to be employed by the Company and provide personal services to the Company in return for certain compensation.

Accordingly, in consideration of the mutual promises and covenants contained herein, the parties agree to the following:

1. EMPLOYMENT BY THE COMPANY.

1.1 At-Will Employment. Executive shall continue to be employed by the Company on an "at-will" basis, meaning either the Company or Executive may terminate Executive' s employment at any time, with or without Cause (as defined in Section 6.2(f) below), Good Reason (as defined in Section 6.2(e) below), or advance notice. Any contrary representations that may have been made to Executive shall be superseded by this Agreement. This Agreement shall constitute the full and complete agreement between Executive and the Company on the "at-will" nature of Executive' s employment with the Company, which may be changed only in an express written agreement signed by Executive and a duly authorized officer of the Company. Executive' s rights to any salary or cash bonus following a termination shall be only as set forth in Section 6 or under any applicable benefit or equity plan.

1.2 Position. Subject to the terms set forth herein, the Company agrees to continue to employ Executive and Executive hereby accepts such continued employment. In addition, Executive shall continue to serve as Chief Medical Officer. During the term of Executive' s employment with the Company, and excluding periods of vacation and sick leave to which Executive is entitled, Executive shall devote all business time and attention to the affairs of the Company necessary to discharge the responsibilities assigned hereunder, and shall use commercially reasonable efforts to perform faithfully and efficiently such responsibilities.

1.3 Duties. Executive will report to the Chief Executive Officer and will render such business and professional services in the performance of Executive' s duties, consistent with Executive' s position as Chief Medical Officer, as shall reasonably be assigned to him by the Chief Executive Officer. Executive shall continue to be expected to perform Executive' s duties under this Agreement out of the Company' s corporate headquarters in New York, New York approximately one to two weeks each a calendar month, or such other location as assigned. In addition, Executive shall make such business trips to such places as may be reasonably necessary or advisable for the efficient operations of the Company.

1.4 Company Policies and Benefits. The employment relationship between the parties shall continue to be subject to the Company' s written personnel policies and procedures as they may be adopted, revised, or deleted from time to time in the Company' s sole discretion. Executive shall be expected to continue to comply with all applicable laws, regulations, rules, directives and other legal requirements of federal, state and other governmental and regulatory bodies having jurisdiction over the Company and of the professional bodies of which the Company is a member. During Executive' s employment with the Company, Executive continues to be required to maintain in good standing any licenses and certifications necessary for the performance of Executive' s duties for the Company. Executive will continue to be eligible to participate on the same basis as similarly-situated employees in the Company' s benefit plans in effect from time to time during Executive' s employment. Subject to the preceding sentence, the Company reserves the right to change, alter, or terminate any benefit plan in its sole discretion. All matters of eligibility for coverage or benefits under any benefit plan shall be determined in accordance with the provisions of such plan. Notwithstanding the foregoing, in the event that the terms of this Agreement differ from or are in conflict with the Company' s general employment policies or practices, this Agreement shall control.

2. COMPENSATION.

2.1 Salary. Executive shall receive an annualized base salary of $405,000, subject to review and adjustment from time to time by the Company in its sole discretion, payable subject to standard federal and state payroll withholding requirements in accordance with the Company' s standard payroll practices (the "***Base Salary***").

2.2 Bonus.

(a) During Employment. Executive shall be eligible to earn an annual performance bonus (the "***Annual Bonus***") with an annual target of 35% (the "***Target Percentage***") of Executive' s then-current Base Salary (the "***Target Bonus***"). The Annual Bonus will be based upon the assessment by the Company' s Board of Directors (the "***Board***") or a committee thereof of Executive' s performance and the Company' s attainment of targeted goals (as set by the Company and confirmed by the Board in its reasonable good faith discretion) over the applicable calendar year. The Annual Bonus, if any, will be subject to applicable payroll deductions and withholdings. No amount of any Annual Bonus is guaranteed at any time, and, except as otherwise stated in Sections 6.2(a)(iii) or 6.3(a)(iii), Executive must be an employee in good standing through the date the Annual Bonus is paid to be eligible to receive an Annual Bonus. No partial or prorated bonuses will be provided. Subject to Section 6.3(b) related to payments upon certain terminations of employment, any Annual Bonus, if earned, will be paid at the same time annual bonuses are generally paid to other similarly-situated employees of the Company. Executive' s eligibility for an Annual Bonus is subject to change in the discretion of the Board (or any authorized committee thereof).

(b) Upon Termination. Subject to the provisions of Section 6, in the event Executive leaves the employ of the Company for any reason prior to the date the Annual Bonus is paid, Executive is not eligible to earn such Annual Bonus, prorated or otherwise.

2.3 Future Equity Awards. Executive remains eligible to be considered for future equity awards as may be determined by the Board or a committee of the Board in its discretion in accordance with the terms of any applicable equity plan or arrangement that may be in effect from time to time.

2.4 Expense Reimbursement. The Company will reimburse Executive for reasonable business expenses in accordance with the Company' s standard expense reimbursement policy, subject to any applicable payroll withholdings and deductions (if any). For the avoidance of doubt, to the extent that any reimbursements payable to Executive are subject to the provisions of Section 409A of the Internal Revenue Code of 1986, as amended (the "***Code***"): (a) any such reimbursements will be paid no later than December 31 of the year following the year in which the expense was incurred, (b) the amount of expenses reimbursed in one year will not affect the amount eligible for reimbursement in any subsequent year, and (c) the right to reimbursement under this Agreement will not be subject to liquidation or exchange for another benefit.

3. CONFIDENTIAL INFORMATION, INVENTIONS, NON-SOLICITATION AND NON-COMPETITION OBLIGATIONS. In connection with Executive' s continued employment with the Company, Executive will continue to receive and continue to have access to the Company' s confidential information and trade secrets. Accordingly, and in consideration of the benefits that Executive is eligible to receive under this Agreement, Executive agrees to sign the Company' s Employee Confidential Information, Inventions, Non-Solicitation and Non-Competition Agreement (the "***Confidential Information Agreement***"), attached as **Exhibit A**, which contains restrictive covenants and prohibits unauthorized use or disclosure of the Company' s confidential information and trade secrets, among other obligations. The Confidential Information Agreement contains provisions that are intended by the parties to survive and do survive termination or expiration of this Agreement and will supersede, prospectively only, the agreement that Executive previously signed relating to the same subject matter.

4. OUTSIDE ACTIVITIES. Except with the prior written consent of the Board, Executive will not, while employed by the Company, undertake or engage in any other employment, occupation, or business enterprise that would interfere with Executive' s responsibilities and the performance of Executive' s duties hereunder except for (i) reasonable time devoted to volunteer services for or on behalf of such religious, educational, non-profit, and/or other charitable organization as Executive may wish to serve, (ii) reasonable time devoted to activities in the non-profit and business communities consistent with Executive' s position with the Company, (iii) reasonable time serving as trustee, director, or advisor to any family companies or trusts, or (iv) with prior written notice to the Board, reasonable time devoted to service as a member of the board of directors (or its equivalent in the case of a non-corporate entity) of a non-competing business; so long as the activities set forth in clauses (i), (ii), (iii), and (iv) do not interfere, individually or in the aggregate, with the performance of Executive' s duties for the Company, are not competitive with the business of the Company, will not otherwise result in Executive' s breach of the Confidential Information Agreement, or create a business or fiduciary conflict. This restriction shall not, however, preclude Executive from (x) owning less than one percent (1%) of the total outstanding shares of a publicly traded company, (y) managing Executive' s passive personal investments, or (z) employment or service in any capacity with Affiliates of the Company. As used in this Agreement, "***Affiliates***" means, at the time of determination, any "parent" or "subsidiary" of the Company as such terms are defined in Rule 405 of the Securities Act of 1933, as amended. The Board will have the authority to determine the time or times at which "parent" or "subsidiary" status is determined within the foregoing definition.

5. NO CONFLICT WITH EXISTING OBLIGATIONS. Executive represents that Executive' s performance of all the terms of this Agreement and continued service as an employee of the Company do not and will not breach any agreement or obligation of any kind made prior to Executive' s employment by the Company, including agreements or obligations Executive may have with prior employers or entities for which Executive has provided services. Executive has not entered into, and Executive agrees that Executive will not enter into, any agreement or obligation, either written or oral, in conflict herewith or with Executive' s duties to the Company.

6. TERMINATION OF EMPLOYMENT. The parties acknowledge that Executive' s employment relationship with the Company continues to be at-will. Either Executive or the Company may terminate the employment relationship at any time, with or without Cause (as defined below) or advance notice. The provisions in this Section govern the amount of compensation, if any, to be provided to Executive upon termination of employment and do not alter this at-will status.

6.1 Termination by Virtue of Death or Disability of Executive.

(a) In the event of Executive' s death while employed pursuant to this Agreement, all obligations of the parties hereunder and Executive' s employment shall terminate immediately, and the Company shall, pursuant to the Company' s standard payroll policies and applicable law, pay to Executive' s legal representatives the Accrued Obligations (as defined in Section 6.2(d) below) due to Executive.

(b) Subject to applicable state and federal law, the Company shall at all times have the right, upon written notice to Executive, to terminate this Agreement based on Executive' s Disability (as defined below). Termination by the Company of Executive' s employment based on "***Disability***" shall mean termination because Executive is unable due to a physical or mental condition to perform the essential functions of Executive' s position with or without reasonable accommodation for six (6) months in the aggregate during any twelve (12) month period or based on the written certification by two licensed physicians of the likely continuation of such condition for such period. This definition shall be interpreted and applied consistent with the Americans with Disabilities Act, the Family and Medical Leave Act, and other applicable law. In the event Executive' s employment is terminated based on Executive' s Disability, Executive will be entitled to the Accrued Obligations due to Executive.

6.2 Termination by the Company or Resignation by Executive.

(a) The Company shall have the right to terminate Executive' s employment pursuant to this Section 6.2 at any time (subject to any applicable cure period stated in Section 6.2(f)) with or without Cause or advance notice, by giving notice as described in Section 7.1 of this Agreement. Likewise, Executive can resign from employment with or without Good Reason, by giving notice as described in Section 7.1 of this Agreement. Executive hereby agrees to comply with the additional notice requirements set forth in Section 6.2(e) below for any resignation for Good Reason. If Executive is terminated by the Company (with or without Cause) or resigns from employment with the Company (with or without Good Reason), then Executive shall be entitled to the Accrued Obligations (as defined below). In addition, if Executive is terminated without Cause or resigns for Good Reason, and provided that such termination constitutes a "separation from service"

(as defined under Treasury Regulation Section 1.409A-1(h), without regard to any alternative definition thereunder, a "***Separation from Service***"), and further provided that Executive executes and allows to become effective a separation agreement that includes, among other terms, a general release of claims in favor of the Company and its Affiliates and representatives, substantially in the form attached hereto as **Exhibit B** (the "***Separation Agreement***"), as may be modified only to reflect changes in the law, and subject to Section 6.2(b) (the date that the general release of claims in the Separation Agreement becomes effective and may no longer be revoked by Executive is referred to as the "***Release Date***"), then Executive shall be eligible to receive the following severance benefits (collectively the "***Non-CIC Severance Benefits***"):

(i) An amount equal to twelve (12) months of Executive' s then current Base Salary, less standard payroll deductions and withholdings, paid in installments on the Company' s regular payroll dates;

(ii) Provided Executive or Executive' s covered dependents, as the case may be, timely elects continued coverage under COBRA under the Company' s group health plans following such termination, the portion of the COBRA premiums which is equal to the cost of the coverage that the Company was paying as of the date of termination, to continue Executive' s (and Executive' s covered dependents, as applicable) health insurance coverage in effect on the termination date until the earliest of: (1) twelve (12) months following the termination date; or (2) the date Executive ceases to be eligible for COBRA continuation coverage for any reason, including plan termination (such period from the termination date through the earlier of (1) and (2), (the "***COBRA Payment Period***")). Notwithstanding the foregoing, if at any time the Company determines that its payment of COBRA premiums on Executive' s behalf would result in a violation of applicable law (including, but not limited to, the 2010 Patient Protection and Affordable Care Act, as amended by the 2010 Health Care and Education Reconciliation Act), then in lieu of paying COBRA premiums pursuant to this Section, the Company shall pay Executive on the last day of each remaining month of the COBRA Payment Period, a fully taxable cash payment equal to 100% of the COBRA premium for such month, subject to applicable tax withholding, for the remainder of the COBRA Payment Period. Nothing in this Agreement shall deprive Executive of Executive' s rights under COBRA or ERISA for benefits under plans and policies arising under Executive' s employment by the Company; and

(iii) If Executive is terminated pursuant to this Section 6.2 between January 1 and the payment date of the Target Bonus, the Company will pay a lump sum cash payment in an amount equal to the amount of the Target Bonus that Executive would have otherwise earned for performance in the calendar year preceding Executive' s termination (the "***Bonus Severance***"). The Bonus Severance will be subject to standard payroll deductions and withholdings and will be paid on the first payroll date after the 60th day following Executive' s date of termination, provided that Executive has delivered an effective Separation Agreement as of such date.

(b) Executive shall not receive the Non-CIC Severance Benefits pursuant to Section 6.2(a) unless Executive executes the Separation Agreement within the consideration period specified therein, which shall in no event be more than forty-five (45) days, and until the Separation Agreement becomes effective and can no longer be revoked by Executive under its terms.

Executive' s ability to receive benefits pursuant to Section 6.2(a) is further conditioned upon Executive: (i) returning all Company property; (ii) complying with Executive' s post-termination obligations under this Agreement and the Confidential Information Agreement; (iii) complying with the Separation Agreement, including without limitation any non-disparagement and confidentiality provisions contained therein; and (iv) resignation from any other positions Executive holds with the Company, effective no later than Executive' s date of termination (or such other date as requested by the Board).

(c) The Company will not make any payments to Executive with respect to any of the benefits pursuant to Section 6.2(a) prior to the 60th day following Executive' s date of termination. On the first payroll date after the 60th day following Executive' s date of termination, and provided that Executive has delivered an effective Separation Agreement, the Company will make the first payment to Executive under Section 6.2(a)(i) and, in a lump sum, an amount equal to the aggregate amount of payments that the Company would have paid Executive through such date had the payments commenced on Executive' s date of termination through such 60th day, with the balance of the payments paid thereafter on the schedule described above, subject to any delay in payment required by Section 6.6.

(d) For purposes of this Agreement, "***Accrued Obligations***" are (i) Executive' s accrued but unpaid salary through the date of termination and, if required by applicable law and the Company' s applicable policy as of the time of termination, any accrued but unused vacation through the date of termination (both of which, for purpose of clarity, shall be paid in cash), (ii) any unreimbursed business expenses incurred by Executive payable in accordance with the Company' s standard expense reimbursement policies, and (iii) benefits owed to Executive under any qualified retirement plan or health and welfare benefit plan in which Executive was a participant in accordance with applicable law and the provisions of such plan.

(e) For purposes of this Agreement, "***Good Reason***" means any of the following actions taken by the Company without Executive' s express prior written consent: (i) a material reduction by the Company of Executive' s Base Salary (other than in a broad based reduction similarly affecting all other members of the Company' s executive management); (ii) a material breach by the Company of this Agreement or any other material written agreement between Executive and the Company concerning the terms and conditions of Executive' s employment; (iii) the relocation of Executive' s principal place of employment, without Executive' s consent, to a place that increases Executive' s one-way commute by more than twenty-five (25) miles as compared to Executive' s then-current principal place of employment immediately prior to such relocation; or (iv) a material reduction in Executive' s duties, authority, or responsibilities for the Company relative to Executive' s duties, authority, or responsibilities in effect immediately prior to such reduction; provided, however, that, any such termination by Executive shall only be deemed for Good Reason pursuant to this definition if: (1) Executive gives the Company written notice of Executive' s intent to terminate for Good Reason within thirty (30) days following Executive' s learning of the occurrence of the condition(s) that Executive believes constitute(s) Good Reason, which notice shall describe such condition(s); (2) the Company fails to remedy such condition(s) within thirty (30) days following receipt of the written notice (the "***Cure Period***"); and (3) Executive voluntarily terminates Executive' s employment within thirty (30) days following the end of the Cure Period. For the avoidance of doubt, any change in Executive' s title or the entity structure of the Company, in each case, without a corresponding material reduction in Executive' s duties, authority, or responsibilities, in accordance with clause (iv) above, shall not constitute Good Reason.

(f) For purposes of this Agreement, "***Cause***" for termination shall mean that Executive has engaged in any of the following: (i) a material breach of any covenant or condition under this Agreement or any other material agreement between the parties; (ii) any act constituting dishonesty, fraud, immoral or disreputable conduct which is reasonably likely to cause harm (including reputational harm) to the Company; (iii) any conduct which constitutes a felony under applicable law; (iv) material violation of any Company policy, after the expiration of ten (10) days without cure after written notice of such violation to the extent such violation is curable; (v) refusal to follow or implement a clear, lawful and reasonable directive of Company after the expiration of ten (10) days without cure after written notice of such failure to the extent such failure is curable; (vi) gross negligence or incompetence in the performance of Executive' s duties after the expiration of ten (10) days without cure after written notice of such failure; or (vii) breach of fiduciary duty to the Company.

(g) The benefits provided to Executive pursuant to this Section 6.2 are in lieu of, and not in addition to, any benefits to which Executive may otherwise be entitled under any Company severance plan, policy, or program.

(h) Any damages caused by the termination of Executive' s employment without Cause or for Good Reason would be difficult to ascertain; therefore, the Non-CIC Severance Benefits for which Executive is eligible pursuant to Section 6.2(a) above in exchange for the Separation Agreement is agreed to by the parties as liquidated damages, to serve as full compensation, and not a penalty.

(i) If the Company terminates Executive' s employment for Cause, or Executive resigns from employment with the Company without Good Reason, regardless of whether or not such termination is in connection with a Change in Control (as defined in the Company' s 2019 Equity Incentive Plan), then Executive shall be entitled to the Accrued Obligations, but Executive will not receive the Non-CIC Severance Benefits, the CIC Severance Benefits, or any other severance compensation or benefit.

6.3 Resignation by Executive for Good Reason or Termination by the Company without Cause (in connection with a Change in Control).

(a) In the event that the Company terminates Executive' s employment without Cause or Executive resigns for Good Reason within twelve (12) months following the effective date of a Change in Control ("***Change in Control Termination Date***"), then Executive shall be entitled to the Accrued Obligations and, subject to Executive' s compliance with Section 6.2(b) above, Executive shall be eligible to receive the following severance benefits (collectively the "***CIC Severance Benefits***"), subject to the terms and conditions set forth in Section 6.3(b):

(i) An amount equal to twelve (12) months of Executive' s then current Base Salary, less standard payroll deductions and withholdings, paid in installments on the Company' s regular payroll dates;

(ii) Provided Executive or Executive' s covered dependents, as the case may be, timely elects continued coverage under COBRA under the Company' s group health plans following such termination, the portion of the COBRA premiums which is equal to the cost of the coverage that the Company was paying as of the date of termination, to continue Executive' s (and Executive' s covered dependents, as applicable) health insurance coverage in effect on the termination date until the earliest of: (1) twelve (12) months following the termination date; or (2) the date Executive ceases to be eligible for COBRA continuation coverage for any reason, including plan termination (such period from the termination date through the earlier of (1) and (2), (the "***CIC COBRA Payment Period***"). Notwithstanding the foregoing, if at any time the Company determines that its payment of COBRA premiums on Executive' s behalf would result in a violation of applicable law (including, but not limited to, the 2010 Patient Protection and Affordable Care Act, as amended by the 2010 Health Care and Education Reconciliation Act), then in lieu of paying COBRA premiums pursuant to this Section, the Company shall pay Executive on the last day of each remaining month of the CIC COBRA Payment Period, a fully taxable cash payment equal to 100% of the COBRA premium for such month, subject to applicable tax withholding, for the remainder of the CIC COBRA Payment Period. Nothing in this Agreement shall deprive Executive of Executive' s rights under COBRA or ERISA for benefits under plans and policies arising under Executive' s employment by the Company;

(iii) A lump sum cash payment in an amount equal to the full Target Bonus for the year in which the termination occurs, subject to standard payroll deductions and withholdings, which will be paid on the first payroll date after the 60th day following Executive' s date of termination, provided that Executive has delivered an effective Separation Agreement as of such date; and

(iv) Effective as of Executive' s Change in Control Termination Date, the vesting and exercisability of all outstanding equity awards held by Executive immediately prior to the Change in Control Termination Date shall be accelerated in full.

(b) The Company will not make any payments to Executive with respect to any of the benefits pursuant to Section 6.3(a) prior to the 60th day following Executive' s date of termination. On the first payroll date after the 60th day following Executive' s date of termination, and provided that Executive has delivered an effective Separation Agreement, the Company will (i) make the first payment to Executive under Section 6.2(a)(i) and, in a lump sum, an amount equal to the aggregate amount of payments that the Company would have paid Executive through such date had the payments commenced on Executive' s date of termination through such 60th day, with the balance of the payments paid thereafter on the schedule described above; and (ii) make the lump sum payment specified in Section 6.3(a)(iii) that has not yet been made due to this Section 6.3(b), in the cases of (i) and (ii) subject to any delay in payment required by Section 6.6.

(c) The benefits provided to Executive pursuant to this Section 6.3 are in lieu of, and not in addition to, any benefits to which Executive may otherwise be entitled under any Company severance plan, policy, or program.

(d) Any damages caused by the termination of Executive' s employment without Cause or for Good Reason in connection with a Change in Control would be difficult to ascertain; therefore, the CIC Severance Benefits for which Executive is eligible pursuant to Section 6.3(a) above in exchange for the Separation Agreement is agreed to by the parties as liquidated damages, to serve as full compensation, and not a penalty.

6.4 Cooperation With the Company After Termination of Employment. Following termination of Executive' s employment for any reason, Executive shall reasonably cooperate with the Company in all matters relating to the winding up of Executive' s pending work including, but not limited to, any litigation in which the Company is involved, and the orderly transfer of any such pending work to such other executives as may be designated by the Company; provided, that the Company agrees that the Company (a) shall make reasonable efforts to minimize disruption of Executive' s other activities, and (b) shall reimburse Executive for all reasonable expenses incurred in connection with such cooperation.

6.5 Effect of Termination. Executive agrees that should Executive' s employment be terminated for any reason, Executive shall be deemed to have resigned from any and all positions with the Company, including, but not limited to, a position on the Board and all positions with any and all subsidiaries and Affiliates of the Company.

6.6 Application of Section 409A.

(a) It is intended that all of the compensation payable under this Agreement, to the greatest extent possible, either complies with the requirements of Section 409A of the Code and the regulations and other guidance thereunder and any state law of similar effect (collectively, "***Section 409A***") or satisfies one or more of the exemptions from the application of Section 409A, and this Agreement will be construed in a manner consistent with such intention, incorporating by reference all required definitions and payment terms.

(b) No severance payments will be made under this Agreement unless Executive' s termination of employment constitutes a Separation from Service. For purposes of Section 409A (including, without limitation, for purposes of Treasury Regulations Section 1.409A-2(b)(2)(iii)), Executive' s right to receive any installment payments under this Agreement (whether severance payments or otherwise) shall be treated as a right to receive a series of separate payments and, accordingly, each installment payment hereunder shall at all times be considered a separate and distinct payment.

(c) To the extent that any severance payments are deferred compensation under Section 409A, and are not otherwise exempt from the application of Section 409A, then, to the extent required to comply with Section 409A, if the period during which Executive may consider and sign the Separation Agreement spans two calendar years, the severance payments will not begin until the second calendar year. If the Company determines that the severance benefits provided under this Agreement constitutes "deferred compensation" under Section 409A and if Executive is a "specified employee" of the Company, as such term is defined in Section 409A(a)(2)(B)(i) of the Code at the time of Executive' s Separation from Service, then, solely to the extent necessary to avoid the incurrence of the adverse personal tax consequences under Section 409A, the timing of the severance will be delayed as follows: on the earlier to occur of (a) the date that is six months and one day after

Executive' s Separation from Service, and (b) the date of Executive' s death, the Company will: (i) pay to Executive a lump sum amount equal to the sum of the severance benefits that Executive would otherwise have received if the commencement of the payment of the severance benefits had not been delayed pursuant to this Section 6.6(c); and (ii) commence paying the balance of the severance benefits in accordance with the applicable payment schedule set forth in Sections 6.2 and 6.3. No interest shall be due on any amounts deferred pursuant to this Section 6.6(c).

(d) To the extent required to avoid accelerated taxation and/or tax penalties under Section 409A, amounts reimbursable to Executive under this Agreement shall be paid to Executive on or before the last day of the year following the year in which the expense was incurred and the amount of expenses eligible for reimbursement (and in-kind benefits provided to Executive) during any one year may not effect amounts reimbursable or provided in any subsequent year. The Company makes no representation that compensation paid pursuant to the terms of this Agreement will be exempt from or comply with Section 409A and makes no undertaking to preclude Section 409A from applying to any such payment.

6.7 Excise Tax Adjustment.

(a) If any payment or benefit Executive will or may receive from the Company or otherwise (a "***280G Payment***") would (i) constitute a "parachute payment" within the meaning of Section 280G of the Code, and (ii) but for this Section, be subject to the excise tax imposed by Section 4999 of the Code (the "***Excise Tax***"), then any such 280G Payment provided pursuant to this Agreement (a "***Payment***") shall be equal to the Reduced Amount. The "***Reduced Amount***" shall be either (x) the largest portion of the Payment that would result in no portion of the Payment (after reduction) being subject to the Excise Tax, or (y) the largest portion, up to and including the total, of the Payment, whichever amount (i.e., the amount determined by clause (x) or by clause (y)), after taking into account all applicable federal, state, and local employment taxes, income taxes, and the Excise Tax (all computed at the highest applicable marginal rate), results in Executive' s receipt, on an after-tax basis, of the greater economic benefit notwithstanding that all or some portion of the Payment may be subject to the Excise Tax. If a reduction in a Payment is required pursuant to the preceding sentence and the Reduced Amount is determined pursuant to clause (x) of the preceding sentence, the reduction shall occur in the manner (the "***Reduction Method***") that results in the greatest economic benefit for Executive. If more than one method of reduction will result in the same economic benefit, the items so reduced will be reduced pro rata (the "***Pro Rata Reduction Method***").

(b) Notwithstanding any provision of this Section 6.7 to the contrary, if the Reduction Method or the Pro Rata Reduction Method would result in any portion of the Payment being subject to taxes pursuant to Section 409A that would not otherwise be subject to taxes pursuant to Section 409A, then the Reduction Method and/or the Pro Rata Reduction Method, as the case may be, shall be modified so as to avoid the imposition of taxes pursuant to Section 409A as follows: (A) as a first priority, the modification shall preserve to the greatest extent possible, the greatest economic benefit for Executive as determined on an after-tax basis; (B) as a second priority, Payments that are contingent on future events (*e.g.*, being terminated without Cause), shall be reduced (or eliminated) before Payments that are not contingent on future events; and (C) as a third priority, Payments that are "deferred compensation" within the meaning of Section 409A shall be reduced (or eliminated) before Payments that are not deferred compensation within the meaning of Section 409A.

(c) Unless Executive and the Company agree on an alternative accounting firm or law firm, the accounting firm engaged by the Company for general tax compliance purposes as of the day prior to the effective date of the Change in Control transaction shall perform the foregoing calculations. If the accounting firm so engaged by the Company is serving as accountant or auditor for the individual, entity, or group effecting the Change in Control transaction, the Company shall appoint a nationally recognized accounting or law firm to make the determinations required by this Section 6.7. The Company shall bear all expenses with respect to the determinations by such accounting or law firm required to be made hereunder. The Company shall use commercially reasonable efforts to cause the accounting or law firm engaged to make the determinations hereunder to provide its calculations, together with detailed supporting documentation, to Executive and the Company within fifteen (15) calendar days after the date on which Executive' s right to a 280G Payment becomes reasonably likely to occur (if requested at that time by Executive or the Company) or such other time as requested by Executive or the Company.

(d) If Executive receives a Payment for which the Reduced Amount was determined pursuant to clause (x) of Section 6.7(a) and the Internal Revenue Service determines thereafter that some portion of the Payment is subject to the Excise Tax, Executive agrees to promptly return to the Company a sufficient amount of the Payment (after reduction pursuant to clause (x) of Section 6.7(a)) so that no portion of the remaining Payment is subject to the Excise Tax. For the avoidance of doubt, if the Reduced Amount was determined pursuant to clause (y) of Section 6.7(a), Executive shall have no obligation to return any portion of the Payment pursuant to the preceding sentence.

7. GENERAL PROVISIONS.

7.1 Notices. Any notices required hereunder shall be in writing and shall be deemed effectively given: (a) upon personal delivery to the party to be notified, (b) when sent by electronic mail or confirmed facsimile if sent during normal business hours of the recipient, and if not, then on the next business day, (c) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid, or (d) one (1) day after deposit with a nationally recognized overnight courier, specifying next day delivery, with written verification of receipt. All communications shall be sent to the Company at its primary office location and to Executive at Executive' s address as listed on the Company payroll or (if notice is given prior to Executive' s termination of employment) to Executive' s Company-issued email address, or at such other address as the Company or Executive may designate by ten (10) days' advance written notice to the other.

7.2 Severability. Whenever possible, each provision of this Agreement will be interpreted in such manner as to be effective and valid under applicable law, but if any provision of this Agreement is held to be invalid, illegal, or unenforceable in any respect under any applicable law or rule in any jurisdiction, such invalidity, illegality, or unenforceability will not affect any other provision or any other jurisdiction, but this Agreement will be reformed, construed, and enforced in such jurisdiction as if such invalid, illegal, or unenforceable provisions had never been contained herein.

7.3 Waiver. If either party should waive any breach of any provisions of this Agreement, Executive or the Company shall not thereby be deemed to have waived any preceding or succeeding breach of the same or any other provision of this Agreement.

7.4 Complete Agreement. This Agreement (including Exhibit A), and any other separate agreement relating to equity awards constitute the entire agreement between Executive and the Company with regard to the subject matter hereof and supersede any prior oral discussions or written communications and agreements, including the Prior Agreement. This Agreement is entered into without reliance on any promise or representation other than those expressly contained herein, and it cannot be modified or amended except in writing signed by Executive and an authorized officer of the Company.

7.5 Counterparts. This Agreement may be executed by electronic transmission and in separate counterparts, any one of which need not contain signatures of more than one party, but all of which taken together will constitute one and the same Agreement.

7.6 Headings. The headings of the sections hereof are inserted for convenience only and shall not be deemed to constitute a part hereof nor to affect the meaning thereof.

7.7 Successors and Assigns. The Company shall assign this Agreement and its rights and obligations hereunder in whole, but not in part, to any company or other entity with or into which the Company may hereafter merge or consolidate or to which the Company may transfer all or substantially all of its assets, if in any such case said company or other entity shall by operation of law or expressly in writing assume all obligations of the Company hereunder as fully as if it had been originally made a party hereto, but may not otherwise assign this Agreement or its rights and obligations hereunder. Executive may not assign or transfer this Agreement or any rights or obligations hereunder, other than to Executive' s estate upon Executive' s death.

7.8 Choice of Law. All questions concerning the construction, validity, and interpretation of this Agreement will be governed by the laws of the State of New York.

7.9 Resolution of Disputes. To ensure the timely and economical resolution of disputes that may arise in connection with Executive' s employment with the Company, Executive and the Company agree that any and all disputes, claims, or causes of action arising from or relating to the enforcement, breach, performance, negotiation, execution, or interpretation of this Agreement, or Executive' s employment, or the termination of Executive' s employment, including but not limited to all statutory claims, will be resolved pursuant to the Federal Arbitration Act, 9 U.S.C. §1-16, and to the fullest extent permitted by law, by final, binding and confidential arbitration by a single arbitrator conducted in New York, New York by Judicial Arbitration and Mediation Services Inc. ("JAMS") under the then applicable JAMS rules (at the following web address: https://www.jamsadr.com/rules-employment-arbitration/); provided, however, this arbitration provision shall not apply to sexual harassment claims to the extent prohibited by applicable law. A hard copy of the rules will be provided to Executive upon request. **By agreeing to this arbitration procedure, both Executive and the Company waive the right to resolve any such dispute through a trial by jury or judge or administrative proceeding.** In addition, all claims, disputes, or causes of action under this provision, whether by Executive or the Company, must be brought in an individual capacity, and shall not be brought as a plaintiff (or claimant) or class member in any purported class or representative proceeding, nor joined or consolidated with the claims of any other person or entity. The arbitrator may not consolidate the claims of more than one person or entity, and may not preside over any form of representative or class proceeding. To the extent that the preceding sentences regarding class claims or proceedings are found to violate applicable law or are otherwise

found unenforceable, any claim(s) alleged or brought on behalf of a class shall proceed in a court of law rather than by arbitration. The Company acknowledges that Executive will have the right to be represented by legal counsel at any arbitration proceeding. Questions of whether a claim is subject to arbitration under this Agreement shall be decided by the arbitrator. Likewise, procedural questions which grow out of the dispute and bear on the final disposition are also matters for the arbitrator. The arbitrator shall: (a) have the authority to compel adequate discovery for the resolution of the dispute and to award such relief as would otherwise be permitted by law; (b) issue a written arbitration decision, to include the arbitrator' s essential findings and conclusions and a statement of the award; (c) be authorized to award any or all remedies that Executive or the Company would be entitled to seek in a court of law; and (d) is authorized to award attorneys' fees to the prevailing party. Subject to the foregoing sentence, Executive and the Company shall equally share all JAMS' arbitration fees and each party is responsible for its own attorneys' fees. Nothing in this Agreement is intended to prevent either Executive or the Company from obtaining injunctive relief in court to prevent irreparable harm pending the conclusion of any such arbitration. Any awards or orders in such arbitrations may be entered and enforced as judgments in the federal and state courts of any competent jurisdiction. To the extent applicable law prohibits mandatory arbitration of sexual harassment claims, in the event Executive intends to bring multiple claims, including a sexual harassment claim, the sexual harassment claim may be publicly filed with a court, while any other claims will remain subject to mandatory arbitration.

[Remainder of page intentionally left blank.]

IN WITNESS WHEREOF, the parties have executed this Employment Agreement on the day and year first written above.

PREVAIL THERAPEUTICS INC.

By: _____
Name:
Title:

EXECUTIVE:

_____
Jeff Sevigny

Exhibit A

EMPLOYEE CONFIDENTIAL INFORMATION, INVENTIONS, NON-SOLICITATION AND NON-COMPETITION AGREEMENT

Exhibit B
Release Agreement

This Release Agreement ("***Release***" or "***Agreement'***) is made by and between Jeff Sevigny ("***you***") and Prevail Therapeutics Inc. (the "***Company***"). A copy of this Release is an attachment to the Employment Agreement between the Company and you dated (the "***Employment Agreement***"). Capitalized terms not defined in this Agreement carry the definition found in the Employment Agreement.

1. Severance Payments; Other Payments.

a. In consideration for your execution, return and non-revocation of this Release on or after your last day of employment (the "***Separation Date***"), the Company will provide you with the **[Non-CIC Severance Benefits/CIC Severance Benefits]** described in Section **[6.2(a)/6.3(a)]** of the Employment Agreement (the "***Severance Benefits***").

b. In addition, regardless of whether you sign this Agreement, the Company affirms that it will pay the following on the next regularly scheduled date on which payroll is run, or sooner if required by applicable law, as required under Section 6 of the Employment Agreement: to include payment of all salary, business expense reimbursements and other amounts due to employee that are not part of the severance.

2. Compliance with Section 409A. The Severance Benefits offered to you by the Company are payable in reliance on Treasury Regulation Section 1.409A-1(b)(9) and the short term deferral exemption in Treasury Regulation Section 1.409A-1(b)(4). For purposes of Code Section 409A, your right to receive any installment payments (whether pay in lieu of notice, Severance Benefits, reimbursements or otherwise) shall be treated as a right to receive a series of separate payments and, accordingly, each installment payment shall at all times be considered a separate and distinct payment. All payments and benefits are subject to applicable withholdings and deductions.

3. Release. In exchange for the Severance Benefits and other consideration, to which you would not otherwise be entitled, and except as otherwise set forth in this Agreement, you, on behalf of yourself and, to the extent permitted by law, on behalf of your spouse, heirs, executors, administrators, assigns, insurers, attorneys and other persons or entities, acting or purporting to act on your behalf (collectively, the "***Employee Parties***"), hereby generally and completely release, acquit and forever discharge the Company, its parents and subsidiaries, and its and their officers, directors, managers, partners, agents, representatives, employees, attorneys, shareholders, predecessors, successors, assigns, insurers and affiliates (the "***Company Parties***") of and from any and all claims, liabilities, demands, contentions, actions, causes of action, suits, costs, expenses, attorneys' fees, damages, indemnities, debts, judgments, levies, executions and obligations of every kind and nature, in law, equity, or otherwise, both known and unknown, suspected and unsuspected, disclosed and undisclosed, arising out of or in any way related to my employment with the Company and separation therefrom, arising at any time prior to and including the execution date of this Agreement, including but not limited to: all such claims and demands directly or indirectly arising out of or in any way connected with your employment with the Company or the termination of that employment; claims or demands related to salary, bonuses, commissions, vacation pay, the right to receive additional grants of stock, stock options or other ownership interests in the Company, fringe

benefits, expense reimbursements, severance pay, or any other form of compensation; claims pursuant to any federal, state or local law, statute, or cause of action; tort law; or contract law (individually a "***Claim***" and collectively "***Claims***"). The Claims you are releasing and waiving in this Agreement include, but are not limited to, any and all Claims that any of the Company Parties:

has violated its personnel policies, handbooks, contracts of employment, or covenants of good faith and fair dealing;

has discriminated against you on the basis of age, race, color, sex (including sexual harassment), national origin, ancestry, disability, religion, sexual orientation, marital status, parental status, source of income, entitlement to benefits, any union activities or other protected category in violation of any local, state or federal law, constitution, ordinance, or regulation, including but not limited to: the Age Discrimination in Employment Act, as amended ("***ADEA***"); Title VII of the Civil Rights Act of 1964, as amended; the Civil Rights Act of 1991; 42 U.S.C. § 1981, as amended; the Equal Pay Act; the Americans With Disabilities Act; the Genetic Information Nondiscrimination Act; the Family and Medical Leave Act; the New York State Human Rights Law, the New York Equal Opportunity for Disabled Persons Act; the New York City Human Rights Law; the Employee Retirement Income Security Act; the Employee Polygraph Protection Act; the Worker Adjustment and Retraining Notification Act; the Older Workers Benefit Protection Act; the anti-retaliation provisions of the Sarbanes-Oxley Act, or any other federal or state law regarding whistleblower retaliation; the Lilly Ledbetter Fair Pay Act; the Uniformed Services Employment and Reemployment Rights Act; the Fair Credit Reporting Act; and the National Labor Relations Act; and

has violated any statute, public policy or common law (including, but not limited to, Claims for retaliatory discharge; negligent hiring, retention or supervision; defamation; intentional or negligent infliction of emotional distress and/or mental anguish; intentional interference with contract; negligence; detrimental reliance; loss of consortium to you or any member of your family and/or promissory estoppel).

Notwithstanding the foregoing, other than events expressly contemplated by this Agreement you do not waive or release rights or Claims that may arise: (i) from events that occur after the date this Release is executed; (ii) that relate to a breach of this Agreement; (iii) that relate to any existing ownership interest in the Company as of the date this Release is executed; (iv) that relate to your existing rights under any Company benefit plan or any plan or agreement related to equity ownership in the Company that arise after this Release is executed; and (v) any Claims which cannot be waived by law, including, without limitation, any rights you may have under applicable workers' compensation laws. Nothing in this Agreement shall prevent you from filing, cooperating with, or participating in any proceeding or investigation before the Equal Employment Opportunity Commission, United States Department of Labor, the National Labor Relations Board, the Occupational Safety and Health Administration, the Securities and Exchange Commission or any other federal government agency, or similar state or local agency ("***Government Agencies***"), or exercising any rights pursuant to Section 7 of the National Labor Relations Act. You further understand this Agreement does not limit your ability to voluntarily communicate with any Government Agencies or otherwise participate in any investigation or proceeding that may be

conducted by any Government Agency, including providing documents or other information, without notice to the Company. While this Agreement does not limit your right to receive an award for information provided to the Securities and Exchange Commission, you understand and agree that, you are otherwise waiving, to the fullest extent permitted by law, any and all rights you may have to individual relief based on any Claims that you have released and any rights you have waived by signing this Agreement. If any Claim is not subject to release, to the extent permitted by law, you waive any right or ability to be a class or collective action representative or to otherwise participate in any putative or certified class, collective or multi-party action or proceeding based on such a Claim in which any of the Company Parties is a party.

4. Your Acknowledgments and Affirmations. You also acknowledge and agree that (i) the consideration given to you in exchange for the waiver and release in this Agreement is in addition to anything of value to which you were already entitled, and (ii) that you have been paid for all time worked, have received all the leave, leaves of absence and leave benefits and protections for which you are eligible, and have not suffered any on-the-job injury for which you have not already filed a Claim. You affirm that all of the decisions of the Company Parties regarding your pay and benefits through the date of your execution of this Agreement were not discriminatory based on age, disability, race, color, sex, religion, national origin or any other classification protected by law. You affirm that you have not filed or caused to be filed, and are not presently a party to, a Claim against any of the Company Parties. You further affirm that you have no known workplace injuries or occupational diseases. You acknowledge and affirm that you have not been retaliated against for reporting any allegation of corporate fraud or other wrongdoing by any of the Company Parties, or for exercising any rights protected by law, including any rights protected by the Fair Labor Standards Act, the Family Medical Leave Act or any related statute or local leave or disability accommodation laws, or any applicable state workers' compensation law. In addition, you acknowledge that you are knowingly and voluntarily waiving and releasing any rights you may have under the ADEA ("***ADEA Waiver***"). You also acknowledge that the consideration given for the ADEA Waiver is in addition to anything of value to which you were already entitled. You further acknowledge that you have been advised by this writing, as required by the ADEA, that: (a) your release and waiver herein does not apply to any rights or claims that arise after the date you sign this Agreement; (b) you should consult with an attorney prior to signing this Agreement; (c) you have **[twenty-one (21)/forty-five (45)]** days to consider this Agreement (although you may choose to voluntarily sign it sooner); (d) you have seven (7) days following the date you sign this Agreement to revoke it; and (e) the Agreement will not be effective until the date upon which the revocation period has expired unexercised, which will be the eighth (8th) day after you sign this Agreement.

5. Return of Company Property. Within five (5) days following the Separation Date, you agree to return to the Company all Company documents (and all copies thereof) and other Company property that you have had in your possession at any time, including, but not limited to, Company files, notes, drawings, records, business plans and forecasts, financial information, specifications, computer-recorded information, tangible property (including, but not limited to, computers), credit cards, entry cards, identification badges and keys; and, any materials of any kind that contain or embody any proprietary or confidential information of the Company (and all reproductions thereof). Please coordinate return of Company property with [ ]. Receipt of the Severance Benefits described in Section 1 of this Agreement is expressly conditioned upon return of all Company property.

6. Confidential Information, Non-Competition and Non-Solicitation Obligations. Both during and after your employment you acknowledge your continuing obligations under your Employee Confidential Information, Inventions, Non-Solicitation and Non-Competition Agreement not to use or disclose any confidential or proprietary information of the Company and to comply with your post-employment non-competition and non-solicitation restrictions. The Company acknowledges that you will not be held criminally or civilly liable under any federal or state trade secret law for the disclosure of a trade secret that: (A) is made (i) in confidence to a federal, state, or local government official, either directly or indirectly, or to an attorney and (ii) solely for the purpose of reporting or investigating a suspected violation of law; or (B) is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal. In addition, in the event that you file a lawsuit for retaliation by the Company for reporting a suspected violation of law, you may disclose the trade secret to your attorney and use the trade secret information in the court proceeding, if you: (A) file any document containing the trade secret under seal; and (B) do not disclose the trade secret, except pursuant to court order.

7. Confidentiality. The provisions of this Agreement will be held in strictest confidence by you and will not be publicized or disclosed in any manner whatsoever; *provided, however*, that (a) you may disclose this Agreement to your immediate family; (b) you may disclose this Agreement in confidence to your attorney, accountant, auditor, tax preparer, and financial advisor, and (c) you may disclose this Agreement insofar as such disclosure may be required by law. Notwithstanding the foregoing, nothing in this Agreement shall limit your right to voluntarily communicate with the Equal Employment Opportunity Commission, United States Department of Labor, the National Labor Relations Board, the Securities and Exchange Commission, other federal government agency or similar state or local agency or to discuss the terms and conditions of your employment with others to the extent expressly permitted by Section 7 of the National Labor Relations Act.

8. Non-Disparagement. You and the Company agree not to disparage each other, and the other' s attorneys, directors, managers, partners, employees, agents and affiliates, in any manner likely to be harmful to them or their business, business reputation or personal reputation; provided that you and the Company will respond accurately and folly to any question, inquiry or request for information when required by legal process. The Company' s obligations under this Section are limited to Company representatives with knowledge of this provision. Notwithstanding the foregoing, nothing in this Agreement shall limit your right to voluntarily communicate with the Equal Employment Opportunity Commission, United States Department of Labor, the National Labor Relations Board, the Securities and Exchange Commission, other federal government agency or similar state or local agency or to discuss the terms and conditions of your employment with others to the extent expressly permitted by Section 7 of the National Labor Relations Act.

9. Cooperation. You agree to reasonably cooperate with the Company in all matters relating to the winding up of your pending work including, but not limited to, any litigation in which the Company is involved, and the orderly transfer of any such pending work to such other executives as may be designated by the Company; provided, that the Company agrees that the Company (a) shall make reasonable efforts to minimize disruption of your other activities, and (b) shall reimburse you for all reasonable expenses incurred in connection with such cooperation.

10. No Admission. This Agreement does not constitute an admission by you or by the Company of any wrongful action or violation of any federal, state, or local statute, or common law rights, including those relating to the provisions of any law or statute concerning employment actions, or of any other possible or claimed violation of law or rights.

11. Breach. You agree that upon any material breach .of this Agreement you will forfeit all amounts paid or owing to you under this Agreement. Further, you acknowledge that it may be impossible to assess the damages caused by your violation of the terms of Sections 5, 6, 7 and 8 of this Agreement and further agree that any threatened or actual violation or breach of those Sections of this Agreement will constitute immediate and irreparable injury to the Company. You therefore agree that, in addition to any and all other damages and remedies available to the Company upon your breach of this Agreement, the Company shall be entitled to an injunction to prevent you from violating or breathing this Agreement.

12. Miscellaneous. This Agreement is entered into without reliance on any promise or representation, written or oral, other than those expressly contained herein, and it supersedes any other such promises, warranties or representations. This Agreement may not be modified or amended except in a writing signed by both you and a duly authorized officer of the Company. This Agreement will bind the heirs, personal representatives, successors and assigns of both you and the Company, and inure to the benefit of both you and the Company, their heirs, successors and assigns. If any provision of this Agreement is determined to be invalid or unenforceable, in whole or in part, this determination will not affect any other provision of this Agreement and the provision in question will be modified by the court so as to be rendered enforceable. This Agreement will be deemed to have been entered into and will be construed and enforced in accordance with the laws of the State of New York as applied to contracts made and to be performed entirely within the State of New York.

Prevail Therapeutics Inc.

By: _____
Name:
Title:

_____
Jeff Sevigny

Exhibit 10.8

AMENDED AND RESTATED EMPLOYMENT AGREEMENT

This **AMENDED AND RESTATED EMPLOYMENT AGREEMENT** (the "***Agreement***") is entered into effective as of (the "***Effective Date***"), by and between Franz Hefti ("***Executive***") and Prevail Therapeutics Inc. (the "***Company***").

Executive has been employed by the Company as its Chief Development Officer pursuant to an offer letter with the Company dated February 26, 2018 (the "***Prior Agreement***").

The Company desires to continue to employ Executive and, in connection therewith, to compensate Executive for Executive' s personal services to the Company; and

Executive wishes to continue to be employed by the Company and provide personal services to the Company in return for certain compensation.

Accordingly, in consideration of the mutual promises and covenants contained herein, the parties agree to the following:

1. EMPLOYMENT BY THE COMPANY.

1.1 At-Will Employment. Executive shall continue to be employed by the Company on an "at-will" basis, meaning either the Company or Executive may terminate Executive' s employment at any time, with or without Cause (as defined in Section 6.2(f) below), Good Reason (as defined in Section 6.2(e) below), or advance notice. Any contrary representations that may have been made to Executive shall be superseded by this Agreement. This Agreement shall constitute the full and complete agreement between Executive and the Company on the "at-will" nature of Executive' s employment with the Company, which may be changed only in an express written agreement signed by Executive and a duly authorized officer of the Company. Executive' s rights to any salary or cash bonus following a termination shall be only as set forth in Section 6 or under any applicable benefit or equity plan.

1.2 Position. Subject to the terms set forth herein, the Company agrees to continue to employ Executive and Executive hereby accepts such continued employment. In addition, Executive shall continue to serve as Chief Development Officer. During the term of Executive' s employment with the Company, and excluding periods of vacation and sick leave to which Executive is entitled, Executive shall devote all business time and attention to the affairs of the Company necessary to discharge the responsibilities assigned hereunder, and shall use commercially reasonable efforts to perform faithfully and efficiently such responsibilities.

1.3 Duties. Executive will report to the Chief Executive Officer and will render such business and professional services in the performance of Executive' s duties, consistent with Executive' s position as Chief Development Officer, as shall reasonably be assigned to him by the Chief Executive Officer. Executive shall perform Executive' s duties under this Agreement principally out of the Company' s corporate headquarters in New York, New York, or such other location as assigned. In addition, Executive shall make such business trips to such places as may be reasonably necessary or advisable for the efficient operations of the Company.

1.4 Company Policies and Benefits. The employment relationship between the parties shall continue to be subject to the Company' s written personnel policies and procedures as they may be adopted, revised, or deleted from time to time in the Company' s sole discretion. Executive shall be expected to continue to comply with all applicable laws, regulations, rules, directives and other legal requirements of federal, state and other governmental and regulatory bodies having jurisdiction over the Company and of the professional bodies of which the Company is a member. During Executive' s employment with the Company, Executive continues to be required to maintain in good standing any licenses and certifications necessary for the performance of Executive' s duties for the Company. Executive will continue to be eligible to participate on the same basis as similarly-situated employees in the Company' s benefit plans in effect from time to time during Executive' s employment. Subject to the preceding sentence, the Company reserves the right to change, alter, or terminate any benefit plan in its sole discretion. All matters of eligibility for coverage or benefits under any benefit plan shall be determined in accordance with the provisions of such plan. Notwithstanding the foregoing, in the event that the terms of this Agreement differ from or are in conflict with the Company' s general employment policies or practices, this Agreement shall control.

2. COMPENSATION.

2.1 Salary. Executive shall receive an annualized base salary of $370,000, subject to review and adjustment from time to time by the Company in its sole discretion, payable subject to standard federal and state payroll withholding requirements in accordance with the Company' s standard payroll practices (the "***Base Salary***").

2.2 Bonus.

(a) During Employment. Executive shall be eligible to earn an annual performance bonus (the "***Annual Bonus***") with an annual target of 35% (the "***Target Percentage***") of Executive' s then-current Base Salary (the "***Target Bonus***"). The Annual Bonus will be based upon the assessment by the Company' s Board of Directors (the "***Board***") or a committee thereof of Executive' s performance and the Company' s attainment of targeted goals (as set by the Company and confirmed by the Board in its reasonable good faith discretion) over the applicable calendar year. The Annual Bonus, if any, will be subject to applicable payroll deductions and withholdings. No amount of any Annual Bonus is guaranteed at any time, and, except as otherwise stated in Sections 6.2(a)(iii) or 6.3(a)(iii), Executive must be an employee in good standing through the date the Annual Bonus is paid to be eligible to receive an Annual Bonus. No partial or prorated bonuses will be provided. Subject to Section 6.3(b) related to payments upon certain terminations of employment, any Annual Bonus, if earned, will be paid at the same time annual bonuses are generally paid to other similarly-situated employees of the Company. Executive' s eligibility for an Annual Bonus is subject to change in the discretion of the Board (or any authorized committee thereof).

(b) Upon Termination. Subject to the provisions of Section 6, in the event Executive leaves the employ of the Company for any reason prior to the date the Annual Bonus is paid, Executive is not eligible to earn such Annual Bonus, prorated or otherwise.

2.3 Future Equity Awards. Executive remains eligible to be considered for future equity awards as may be determined by the Board or a committee of the Board in its discretion in accordance with the terms of any applicable equity plan or arrangement that may be in effect from time to time.

2.4 Expense Reimbursement. The Company will reimburse Executive for reasonable business expenses in accordance with the Company' s standard expense reimbursement policy. For the avoidance of doubt, to the extent that any reimbursements payable to Executive are subject to the provisions of Section 409A of the Internal Revenue Code of 1986, as amended (the "***Code***"): (a) any such reimbursements will be paid no later than December 31 of the year following the year in which the expense was incurred, (b) the amount of expenses reimbursed in one year will not affect the amount eligible for reimbursement in any subsequent year, and (c) the right to reimbursement under this Agreement will not be subject to liquidation or exchange for another benefit.

3. CONFIDENTIAL INFORMATION, INVENTIONS, NON-SOLICITATION AND NON-COMPETITION OBLIGATIONS. In connection with Executive' s continued employment with the Company, Executive will continue to receive and continue to have access to the Company' s confidential information and trade secrets. Accordingly, and in consideration of the benefits that Executive is eligible to receive under this Agreement, Executive agrees to sign the Company' s Employee Confidential Information, Inventions, Non-Solicitation and Non-Competition Agreement (the "***Confidential Information Agreement***"), attached as **Exhibit A**, which contains restrictive covenants and prohibits unauthorized use or disclosure of the Company' s confidential information and trade secrets, among other obligations. The Confidential Information Agreement contains provisions that are intended by the parties to survive and do survive termination or expiration of this Agreement and will supersede, prospectively only, the agreement that Executive previously signed relating to the same subject matter.

4. OUTSIDE ACTIVITIES. Except with the prior written consent of the Board, Executive will not, while employed by the Company, undertake or engage in any other employment, occupation, or business enterprise that would interfere with Executive' s responsibilities and the performance of Executive' s duties hereunder except for (i) reasonable time devoted to volunteer services for or on behalf of such religious, educational, non-profit, and/or other charitable organization as Executive may wish to serve, (ii) reasonable time devoted to activities in the non-profit and business communities consistent with Executive' s position with the Company, (iii) reasonable time serving as trustee, director, or advisor to any family companies or trusts, or (iv) with prior written notice to the Board, reasonable time devoted to service as a member of the board of directors (or its equivalent in the case of a non-corporate entity) of a non-competing business; so long as the activities set forth in clauses (i), (ii), (iii), and (iv) do not interfere, individually or in the aggregate, with the performance of Executive' s duties for the Company, are not competitive with the business of the Company, will not otherwise result in Executive' s breach of the Confidential Information Agreement, or create a business or fiduciary conflict. This restriction shall not, however, preclude Executive from (x) owning less than one percent (1%) of the total outstanding shares of a publicly traded company, (y) managing Executive' s passive personal investments, or (z) employment or service in any capacity with Affiliates of the Company. As used in this Agreement, "***Affiliates***" means, at the time of determination, any "parent" or "subsidiary" of the Company as such terms are defined in Rule 405 of the Securities Act of 1933, as amended. The Board will have the authority to determine the time or times at which "parent" or "subsidiary" status is determined within the foregoing definition.

5. NO CONFLICT WITH EXISTING OBLIGATIONS. Executive represents that Executive' s performance of all the terms of this Agreement and continued service as an employee of the Company do not and will not breach any agreement or obligation of any kind made prior to Executive' s employment by the Company, including agreements or obligations Executive may have with prior employers or entities for which Executive has provided services. Executive has not entered into, and Executive agrees that Executive will not enter into, any agreement or obligation, either written or oral, in conflict herewith or with Executive' s duties to the Company.

6. TERMINATION OF EMPLOYMENT. The parties acknowledge that Executive' s employment relationship with the Company continues to be at-will. Either Executive or the Company may terminate the employment relationship at any time, with or without Cause (as defined below) or advance notice. The provisions in this Section govern the amount of compensation, if any, to be provided to Executive upon termination of employment and do not alter this at-will status.

6.1 Termination by Virtue of Death or Disability of Executive.

(a) In the event of Executive' s death while employed pursuant to this Agreement, all obligations of the parties hereunder and Executive' s employment shall terminate immediately, and the Company shall, pursuant to the Company' s standard payroll policies and applicable law, pay to Executive' s legal representatives the Accrued Obligations (as defined in Section 6.2(d) below) due to Executive.

(b) Subject to applicable state and federal law, the Company shall at all times have the right, upon written notice to Executive, to terminate this Agreement based on Executive' s Disability (as defined below). Termination by the Company of Executive' s employment based on "***Disability***" shall mean termination because Executive is unable due to a physical or mental condition to perform the essential functions of Executive' s position with or without reasonable accommodation for six (6) months in the aggregate during any twelve (12) month period or based on the written certification by two licensed physicians of the likely continuation of such condition for such period. This definition shall be interpreted and applied consistent with the Americans with Disabilities Act, the Family and Medical Leave Act, and other applicable law. In the event Executive' s employment is terminated based on Executive' s Disability, Executive will be entitled to the Accrued Obligations due to Executive.

6.2 Termination by the Company or Resignation by Executive.

(a) The Company shall have the right to terminate Executive' s employment pursuant to this Section 6.2 at any time (subject to any applicable cure period stated in Section 6.2(f)) with or without Cause or advance notice, by giving notice as described in Section 7.1 of this Agreement. Likewise, Executive can resign from employment with or without Good Reason, by giving notice as described in Section 7.1 of this Agreement. Executive hereby agrees to comply with the additional notice requirements set forth in Section 6.2(e) below for any resignation for Good Reason. If Executive is terminated by the Company (with or without Cause) or resigns from employment with the Company (with or without Good Reason), then Executive shall be entitled to the Accrued Obligations (as defined below). In addition, if Executive is terminated without Cause or resigns for Good Reason, and provided that such termination constitutes a "separation from service"

(as defined under Treasury Regulation Section 1.409A-1(h), without regard to any alternative definition thereunder, a "***Separation from Service***"), and further provided that Executive executes and allows to become effective a separation agreement that includes, among other terms, a general release of claims in favor of the Company and its Affiliates and representatives, substantially in the form attached hereto as **Exhibit B** (the "***Separation Agreement***"), as may be modified only to reflect changes in the law, and subject to Section 6.2(b) (the date that the general release of claims in the Separation Agreement becomes effective and may no longer be revoked by Executive is referred to as the "***Release Date***"), then Executive shall be eligible to receive the following severance benefits (collectively the "***Non-CIC Severance Benefits***"):

(i) An amount equal to twelve (12) months of Executive' s then current Base Salary, less standard payroll deductions and withholdings, paid in installments on the Company' s regular payroll dates;

(ii) Provided Executive or Executive' s covered dependents, as the case may be, timely elects continued coverage under COBRA under the Company' s group health plans following such termination, the portion of the COBRA premiums which is equal to the cost of the coverage that the Company was paying as of the date of termination, to continue Executive' s (and Executive' s covered dependents, as applicable) health insurance coverage in effect on the termination date until the earliest of: (1) twelve (12) months following the termination date; or (2) the date Executive ceases to be eligible for COBRA continuation coverage for any reason, including plan termination (such period from the termination date through the earlier of (1) and (2), (the "***COBRA Payment Period***")). Notwithstanding the foregoing, if at any time the Company determines that its payment of COBRA premiums on Executive' s behalf would result in a violation of applicable law (including, but not limited to, the 2010 Patient Protection and Affordable Care Act, as amended by the 2010 Health Care and Education Reconciliation Act), then in lieu of paying COBRA premiums pursuant to this Section, the Company shall pay Executive on the last day of each remaining month of the COBRA Payment Period, a fully taxable cash payment equal to 100% of the COBRA premium for such month, subject to applicable tax withholding, for the remainder of the COBRA Payment Period. Nothing in this Agreement shall deprive Executive of Executive' s rights under COBRA or ERISA for benefits under plans and policies arising under Executive' s employment by the Company; and

(iii) If Executive is terminated pursuant to this Section 6.2 between January 1 and the payment date of the Target Bonus, the Company will pay a lump sum cash payment in an amount equal to the amount of the Target Bonus that Executive would have otherwise earned for performance in the calendar year preceding Executive' s termination (the "***Bonus Severance***"). The Bonus Severance will be subject to standard payroll deductions and withholdings and will be paid on the first payroll date after the 60th day following Executive' s date of termination, provided that Executive has delivered an effective Separation Agreement as of such date.

(b) Executive shall not receive the Non-CIC Severance Benefits pursuant to Section 6.2(a) unless Executive executes the Separation Agreement within the consideration period specified therein, which shall in no event be more than forty-five (45) days, and until the Separation Agreement becomes effective and can no longer be revoked by Executive under its terms.

Executive' s ability to receive benefits pursuant to Section 6.2(a) is further conditioned upon Executive: (i) returning all Company property; (ii) complying with Executive' s post-termination obligations under this Agreement and the Confidential Information Agreement; (iii) complying with the Separation Agreement, including without limitation any non-disparagement and confidentiality provisions contained therein; and (iv) resignation from any other positions Executive holds with the Company, effective no later than Executive' s date of termination (or such other date as requested by the Board).

(c) The Company will not make any payments to Executive with respect to any of the benefits pursuant to Section 6.2(a) prior to the 60th day following Executive' s date of termination. On the first payroll date after the 60th day following Executive' s date of termination, and provided that Executive has delivered an effective Separation Agreement, the Company will make the first payment to Executive under Section 6.2(a)(i) and, in a lump sum, an amount equal to the aggregate amount of payments that the Company would have paid Executive through such date had the payments commenced on Executive' s date of termination through such 60th day, with the balance of the payments paid thereafter on the schedule described above, subject to any delay in payment required by Section 6.6.

(d) For purposes of this Agreement, "***Accrued Obligations***" are (i) Executive' s accrued but unpaid salary through the date of termination and, if required by applicable law and the Company' s applicable policy as of the time of termination, any accrued but unused vacation through the date of termination (both of which, for purpose of clarity, shall be paid in cash), (ii) any unreimbursed business expenses incurred by Executive payable in accordance with the Company' s standard expense reimbursement policies, and (iii) benefits owed to Executive under any qualified retirement plan or health and welfare benefit plan in which Executive was a participant in accordance with applicable law and the provisions of such plan.

(e) For purposes of this Agreement, "***Good Reason***" means any of the following actions taken by the Company without Executive' s express prior written consent: (i) a material reduction by the Company of Executive' s Base Salary (other than in a broad based reduction similarly affecting all other members of the Company' s executive management); (ii) a material breach by the Company of this Agreement or any other material written agreement between Executive and the Company concerning the terms and conditions of Executive' s employment; (iii) the relocation of Executive' s principal place of employment, without Executive' s consent, to a place that increases Executive' s one-way commute by more than twenty-five (25) miles as compared to Executive' s then-current principal place of employment immediately prior to such relocation; or (iv) a material reduction in Executive' s duties, authority, or responsibilities for the Company relative to Executive' s duties, authority, or responsibilities in effect immediately prior to such reduction; provided, however, that, any such termination by Executive shall only be deemed for Good Reason pursuant to this definition if: (1) Executive gives the Company written notice of Executive' s intent to terminate for Good Reason within thirty (30) days following Executive' s learning of the occurrence of the condition(s) that Executive believes constitute(s) Good Reason, which notice shall describe such condition(s); (2) the Company fails to remedy such condition(s) within thirty (30) days following receipt of the written notice (the "***Cure Period***"); and (3) Executive voluntarily terminates Executive' s employment within thirty (30) days following the end of the Cure Period. For the avoidance of doubt, any change in Executive' s title or the entity structure of the Company, in each case, without a corresponding material reduction in Executive' s duties, authority, or responsibilities, in accordance with clause (iv) above, shall not constitute Good Reason.

(f) For purposes of this Agreement, "***Cause***" for termination shall mean that Executive has engaged in any of the following: (i) a material breach of any covenant or condition under this Agreement or any other material agreement between the parties; (ii) any act constituting dishonesty, fraud, immoral or disreputable conduct which is reasonably likely to cause harm (including reputational harm) to the Company; (iii) any conduct which constitutes a felony under applicable law; (iv) material violation of any Company policy, after the expiration of ten (10) days without cure after written notice of such violation to the extent such violation is curable; (v) refusal to follow or implement a clear, lawful and reasonable directive of Company after the expiration of ten (10) days without cure after written notice of such failure to the extent such failure is curable; (vi) gross negligence or incompetence in the performance of Executive' s duties after the expiration of ten (10) days without cure after written notice of such failure; or (vii) breach of fiduciary duty to the Company.

(g) The benefits provided to Executive pursuant to this Section 6.2 are in lieu of, and not in addition to, any benefits to which Executive may otherwise be entitled under any Company severance plan, policy, or program.

(h) Any damages caused by the termination of Executive' s employment without Cause or for Good Reason would be difficult to ascertain; therefore, the Non-CIC Severance Benefits for which Executive is eligible pursuant to Section 6.2(a) above in exchange for the Separation Agreement is agreed to by the parties as liquidated damages, to serve as full compensation, and not a penalty.

(i) If the Company terminates Executive' s employment for Cause, or Executive resigns from employment with the Company without Good Reason, regardless of whether or not such termination is in connection with a Change in Control (as defined in the Company' s 2019 Equity Incentive Plan), then Executive shall be entitled to the Accrued Obligations, but Executive will not receive the Non-CIC Severance Benefits, the CIC Severance Benefits, or any other severance compensation or benefit.

6.3 Resignation by Executive for Good Reason or Termination by the Company without Cause (in connection with a Change in Control).

(a) In the event that the Company terminates Executive' s employment without Cause or Executive resigns for Good Reason within twelve (12) months following the effective date of a Change in Control ("***Change in Control Termination Date***"), then Executive shall be entitled to the Accrued Obligations and, subject to Executive' s compliance with Section 6.2(b) above, Executive shall be eligible to receive the following severance benefits (collectively the "***CIC Severance Benefits***"), subject to the terms and conditions set forth in Section 6.3(b):

(i) An amount equal to twelve (12) months of Executive' s then current Base Salary, less standard payroll deductions and withholdings, paid in installments on the Company' s regular payroll dates;

(ii) Provided Executive or Executive' s covered dependents, as the case may be, timely elects continued coverage under COBRA under the Company' s group health plans following such termination, the portion of the COBRA premiums which is equal to the cost of the coverage that the Company was paying as of the date of termination, to continue Executive' s (and Executive' s covered dependents, as applicable) health insurance coverage in effect on the termination date until the earliest of: (1) twelve (12) months following the termination date; or (2) the date Executive ceases to be eligible for COBRA continuation coverage for any reason, including plan termination (such period from the termination date through the earlier of (1) and (2), (the "***CIC COBRA Payment Period***"). Notwithstanding the foregoing, if at any time the Company determines that its payment of COBRA premiums on Executive' s behalf would result in a violation of applicable law (including, but not limited to, the 2010 Patient Protection and Affordable Care Act, as amended by the 2010 Health Care and Education Reconciliation Act), then in lieu of paying COBRA premiums pursuant to this Section, the Company shall pay Executive on the last day of each remaining month of the CIC COBRA Payment Period, a fully taxable cash payment equal to 100% of the COBRA premium for such month, subject to applicable tax withholding, for the remainder of the CIC COBRA Payment Period. Nothing in this Agreement shall deprive Executive of Executive' s rights under COBRA or ERISA for benefits under plans and policies arising under Executive' s employment by the Company;

(iii) A lump sum cash payment in an amount equal to the full Target Bonus for the year in which the termination occurs, subject to standard payroll deductions and withholdings, which will be paid on the first payroll date after the 60th day following Executive' s date of termination, provided that Executive has delivered an effective Separation Agreement as of such date; and

(iv) Effective as of Executive' s Change in Control Termination Date, the vesting and exercisability of all outstanding equity awards held by Executive immediately prior to the Change in Control Termination Date shall be accelerated in full.

(b) The Company will not make any payments to Executive with respect to any of the benefits pursuant to Section 6.3(a) prior to the 60th day following Executive' s date of termination. On the first payroll date after the 60th day following Executive' s date of termination, and provided that Executive has delivered an effective Separation Agreement, the Company will (i) make the first payment to Executive under Section 6.2(a)(i) and, in a lump sum, an amount equal to the aggregate amount of payments that the Company would have paid Executive through such date had the payments commenced on Executive' s date of termination through such 60th day, with the balance of the payments paid thereafter on the schedule described above; and (ii) make the lump sum payment specified in Section 6.3(a)(iii) that has not yet been made due to this Section 6.3(b), in the cases of (i) and (ii) subject to any delay in payment required by Section 6.6.

(c) The benefits provided to Executive pursuant to this Section 6.3 are in lieu of, and not in addition to, any benefits to which Executive may otherwise be entitled under any Company severance plan, policy, or program.

(d) Any damages caused by the termination of Executive' s employment without Cause or for Good Reason in connection with a Change in Control would be difficult to ascertain; therefore, the CIC Severance Benefits for which Executive is eligible pursuant to Section 6.3(a) above in exchange for the Separation Agreement is agreed to by the parties as liquidated damages, to serve as full compensation, and not a penalty.

6.4 Cooperation With the Company After Termination of Employment. Following termination of Executive' s employment for any reason, Executive shall reasonably cooperate with the Company in all matters relating to the winding up of Executive' s pending work including, but not limited to, any litigation in which the Company is involved, and the orderly transfer of any such pending work to such other executives as may be designated by the Company; provided, that the Company agrees that the Company (a) shall make reasonable efforts to minimize disruption of Executive' s other activities, and (b) shall reimburse Executive for all reasonable expenses incurred in connection with such cooperation.

6.5 Effect of Termination. Executive agrees that should Executive' s employment be terminated for any reason, Executive shall be deemed to have resigned from any and all positions with the Company, including, but not limited to, a position on the Board and all positions with any and all subsidiaries and Affiliates of the Company.

6.6 Application of Section 409A.

(a) It is intended that all of the compensation payable under this Agreement, to the greatest extent possible, either complies with the requirements of Section 409A of the Code and the regulations and other guidance thereunder and any state law of similar effect (collectively, "***Section 409A***") or satisfies one or more of the exemptions from the application of Section 409A, and this Agreement will be construed in a manner consistent with such intention, incorporating by reference all required definitions and payment terms.

(b) No severance payments will be made under this Agreement unless Executive' s termination of employment constitutes a Separation from Service. For purposes of Section 409A (including, without limitation, for purposes of Treasury Regulations Section 1.409A-2(b)(2)(iii)), Executive' s right to receive any installment payments under this Agreement (whether severance payments or otherwise) shall be treated as a right to receive a series of separate payments and, accordingly, each installment payment hereunder shall at all times be considered a separate and distinct payment.

(c) To the extent that any severance payments are deferred compensation under Section 409A, and are not otherwise exempt from the application of Section 409A, then, to the extent required to comply with Section 409A, if the period during which Executive may consider and sign the Separation Agreement spans two calendar years, the severance payments will not begin until the second calendar year. If the Company determines that the severance benefits provided under this Agreement constitutes "deferred compensation" under Section 409A and if Executive is a "specified employee" of the Company, as such term is defined in Section 409A(a)(2)(B)(i) of the Code at the time of Executive' s Separation from Service, then, solely to the extent necessary to avoid the incurrence of the adverse personal tax consequences under Section 409A, the timing of the severance will be delayed as follows: on the earlier to occur of (a) the date that is six months and one day after

Executive' s Separation from Service, and (b) the date of Executive' s death, the Company will: (i) pay to Executive a lump sum amount equal to the sum of the severance benefits that Executive would otherwise have received if the commencement of the payment of the severance benefits had not been delayed pursuant to this Section 6.6(c); and (ii) commence paying the balance of the severance benefits in accordance with the applicable payment schedule set forth in Sections 6.2 and 6.3. No interest shall be due on any amounts deferred pursuant to this Section 6.6(c).

(d) To the extent required to avoid accelerated taxation and/or tax penalties under Section 409A, amounts reimbursable to Executive under this Agreement shall be paid to Executive on or before the last day of the year following the year in which the expense was incurred and the amount of expenses eligible for reimbursement (and in-kind benefits provided to Executive) during any one year may not effect amounts reimbursable or provided in any subsequent year. The Company makes no representation that compensation paid pursuant to the terms of this Agreement will be exempt from or comply with Section 409A and makes no undertaking to preclude Section 409A from applying to any such payment.

6.7 Excise Tax Adjustment.

(a) If any payment or benefit Executive will or may receive from the Company or otherwise (a "***280G Payment***") would (i) constitute a "parachute payment" within the meaning of Section 280G of the Code, and (ii) but for this Section, be subject to the excise tax imposed by Section 4999 of the Code (the "***Excise Tax***"), then any such 280G Payment provided pursuant to this Agreement (a "***Payment***") shall be equal to the Reduced Amount. The "***Reduced Amount***" shall be either (x) the largest portion of the Payment that would result in no portion of the Payment (after reduction) being subject to the Excise Tax, or (y) the largest portion, up to and including the total, of the Payment, whichever amount (i.e., the amount determined by clause (x) or by clause (y)), after taking into account all applicable federal, state, and local employment taxes, income taxes, and the Excise Tax (all computed at the highest applicable marginal rate), results in Executive' s receipt, on an after-tax basis, of the greater economic benefit notwithstanding that all or some portion of the Payment may be subject to the Excise Tax. If a reduction in a Payment is required pursuant to the preceding sentence and the Reduced Amount is determined pursuant to clause (x) of the preceding sentence, the reduction shall occur in the manner (the "***Reduction Method***") that results in the greatest economic benefit for Executive. If more than one method of reduction will result in the same economic benefit, the items so reduced will be reduced pro rata (the "***Pro Rata Reduction Method***").

(b) Notwithstanding any provision of this Section 6.7 to the contrary, if the Reduction Method or the Pro Rata Reduction Method would result in any portion of the Payment being subject to taxes pursuant to Section 409A that would not otherwise be subject to taxes pursuant to Section 409A, then the Reduction Method and/or the Pro Rata Reduction Method, as the case may be, shall be modified so as to avoid the imposition of taxes pursuant to Section 409A as follows: (A) as a first priority, the modification shall preserve to the greatest extent possible, the greatest economic benefit for Executive as determined on an after-tax basis; (B) as a second priority, Payments that are contingent on future events (*e.g.*, being terminated without Cause), shall be reduced (or eliminated) before Payments that are not contingent on future events; and (C) as a third priority, Payments that are "deferred compensation" within the meaning of Section 409A shall be reduced (or eliminated) before Payments that are not deferred compensation within the meaning of Section 409A.

(c) Unless Executive and the Company agree on an alternative accounting firm or law firm, the accounting firm engaged by the Company for general tax compliance purposes as of the day prior to the effective date of the Change in Control transaction shall perform the foregoing calculations. If the accounting firm so engaged by the Company is serving as accountant or auditor for the individual, entity, or group effecting the Change in Control transaction, the Company shall appoint a nationally recognized accounting or law firm to make the determinations required by this Section 6.7. The Company shall bear all expenses with respect to the determinations by such accounting or law firm required to be made hereunder. The Company shall use commercially reasonable efforts to cause the accounting or law firm engaged to make the determinations hereunder to provide its calculations, together with detailed supporting documentation, to Executive and the Company within fifteen (15) calendar days after the date on which Executive' s right to a 280G Payment becomes reasonably likely to occur (if requested at that time by Executive or the Company) or such other time as requested by Executive or the Company.

(d) If Executive receives a Payment for which the Reduced Amount was determined pursuant to clause (x) of Section 6.7(a) and the Internal Revenue Service determines thereafter that some portion of the Payment is subject to the Excise Tax, Executive agrees to promptly return to the Company a sufficient amount of the Payment (after reduction pursuant to clause (x) of Section 6.7(a)) so that no portion of the remaining Payment is subject to the Excise Tax. For the avoidance of doubt, if the Reduced Amount was determined pursuant to clause (y) of Section 6.7(a), Executive shall have no obligation to return any portion of the Payment pursuant to the preceding sentence.

7. GENERAL PROVISIONS.

7.1 Notices. Any notices required hereunder shall be in writing and shall be deemed effectively given: (a) upon personal delivery to the party to be notified, (b) when sent by electronic mail or confirmed facsimile if sent during normal business hours of the recipient, and if not, then on the next business day, (c) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid, or (d) one (1) day after deposit with a nationally recognized overnight courier, specifying next day delivery, with written verification of receipt. All communications shall be sent to the Company at its primary office location and to Executive at Executive' s address as listed on the Company payroll or (if notice is given prior to Executive' s termination of employment) to Executive' s Company-issued email address, or at such other address as the Company or Executive may designate by ten (10) days' advance written notice to the other.

7.2 Severability. Whenever possible, each provision of this Agreement will be interpreted in such manner as to be effective and valid under applicable law, but if any provision of this Agreement is held to be invalid, illegal, or unenforceable in any respect under any applicable law or rule in any jurisdiction, such invalidity, illegality, or unenforceability will not affect any other provision or any other jurisdiction, but this Agreement will be reformed, construed, and enforced in such jurisdiction as if such invalid, illegal, or unenforceable provisions had never been contained herein.

7.3 Waiver. If either party should waive any breach of any provisions of this Agreement, Executive or the Company shall not thereby be deemed to have waived any preceding or succeeding breach of the same or any other provision of this Agreement.

7.4 Complete Agreement. This Agreement (including Exhibit A), and any other separate agreement relating to equity awards constitute the entire agreement between Executive and the Company with regard to the subject matter hereof and supersede any prior oral discussions or written communications and agreements, including the Prior Agreement. This Agreement is entered into without reliance on any promise or representation other than those expressly contained herein, and it cannot be modified or amended except in writing signed by Executive and an authorized officer of the Company.

7.5 Counterparts. This Agreement may be executed by electronic transmission and in separate counterparts, any one of which need not contain signatures of more than one party, but all of which taken together will constitute one and the same Agreement.

7.6 Headings. The headings of the sections hereof are inserted for convenience only and shall not be deemed to constitute a part hereof nor to affect the meaning thereof.

7.7 Successors and Assigns. The Company shall assign this Agreement and its rights and obligations hereunder in whole, but not in part, to any company or other entity with or into which the Company may hereafter merge or consolidate or to which the Company may transfer all or substantially all of its assets, if in any such case said company or other entity shall by operation of law or expressly in writing assume all obligations of the Company hereunder as fully as if it had been originally made a party hereto, but may not otherwise assign this Agreement or its rights and obligations hereunder. Executive may not assign or transfer this Agreement or any rights or obligations hereunder, other than to Executive' s estate upon Executive' s death.

7.8 Choice of Law. All questions concerning the construction, validity, and interpretation of this Agreement will be governed by the laws of the State of New York.

7.9 Resolution of Disputes. To ensure the timely and economical resolution of disputes that may arise in connection with Executive' s employment with the Company, Executive and the Company agree that any and all disputes, claims, or causes of action arising from or relating to the enforcement, breach, performance, negotiation, execution, or interpretation of this Agreement, or Executive' s employment, or the termination of Executive' s employment, including but not limited to all statutory claims, will be resolved pursuant to the Federal Arbitration Act, 9 U.S.C. §1-16, and to the fullest extent permitted by law, by final, binding and confidential arbitration by a single arbitrator conducted in New York, New York by Judicial Arbitration and Mediation Services Inc. ("JAMS") under the then applicable JAMS rules (at the following web address: https://www.jamsadr.com/rules-employment-arbitration/); provided, however, this arbitration provision shall not apply to sexual harassment claims to the extent prohibited by applicable law. A hard copy of the rules will be provided to Executive upon request. **By agreeing to this arbitration procedure, both Executive and the Company waive the right to resolve any such dispute through a trial by jury or judge or administrative proceeding.** In addition, all claims, disputes, or causes of action under this provision, whether by Executive or the Company, must be brought in an individual capacity, and shall not be brought as a plaintiff (or claimant) or class member in any purported class or representative proceeding, nor joined or consolidated with the claims of any other person or entity. The arbitrator may not consolidate the claims of more than one person or entity, and may not preside over any form of representative or class proceeding. To the extent that the preceding sentences regarding class claims or proceedings are found to violate applicable law or are otherwise

found unenforceable, any claim(s) alleged or brought on behalf of a class shall proceed in a court of law rather than by arbitration. The Company acknowledges that Executive will have the right to be represented by legal counsel at any arbitration proceeding. Questions of whether a claim is subject to arbitration under this Agreement shall be decided by the arbitrator. Likewise, procedural questions which grow out of the dispute and bear on the final disposition are also matters for the arbitrator. The arbitrator shall: (a) have the authority to compel adequate discovery for the resolution of the dispute and to award such relief as would otherwise be permitted by law; (b) issue a written arbitration decision, to include the arbitrator' s essential findings and conclusions and a statement of the award; (c) be authorized to award any or all remedies that Executive or the Company would be entitled to seek in a court of law; and (d) is authorized to award attorneys' fees to the prevailing party. Subject to the foregoing sentence, Executive and the Company shall equally share all JAMS' arbitration fees and each party is responsible for its own attorneys' fees. Nothing in this Agreement is intended to prevent either Executive or the Company from obtaining injunctive relief in court to prevent irreparable harm pending the conclusion of any such arbitration. Any awards or orders in such arbitrations may be entered and enforced as judgments in the federal and state courts of any competent jurisdiction. To the extent applicable law prohibits mandatory arbitration of sexual harassment claims, in the event Executive intends to bring multiple claims, including a sexual harassment claim, the sexual harassment claim may be publicly filed with a court, while any other claims will remain subject to mandatory arbitration.

[Remainder of page intentionally left blank.]

IN WITNESS WHEREOF, the parties have executed this Employment Agreement on the day and year first written above.

PREVAIL THERAPEUTICS INC.

By: _____
Name:
Title:

EXECUTIVE:

_____
Franz Hefti

Exhibit A

EMPLOYEE CONFIDENTIAL INFORMATION, INVENTIONS, NON-SOLICITATION AND NON-COMPETITION AGREEMENT

Exhibit B
Release Agreement

This Release Agreement ("***Release***" or "***Agreement'***) is made by and between Franz Hefti (**"*you*"**) and Prevail Therapeutics Inc. (the "***Company***"). A copy of this Release is an attachment to the Employment Agreement between the Company and you dated (the "***Employment Agreement***"). Capitalized terms not defined in this Agreement carry the definition found in the Employment Agreement.

1. Severance Payments; Other Payments.

a. In consideration for your execution, return and non-revocation of this Release on or after your last day of employment (the "***Separation Date***"), the Company will provide you with the **[Non-CIC Severance Benefits/CIC Severance Benefits]** described in Section **[6.2(a)/6.3(a)]** of the Employment Agreement (the "***Severance Benefits***").

b. In addition, regardless of whether you sign this Agreement, the Company affirms that it will pay the following on the next regularly scheduled date on which payroll is run, or sooner if required by applicable law, as required under Section 6 of the Employment Agreement: to include payment of all salary, business expense reimbursements and other amounts due to employee that are not part of the severance.

2. Compliance with Section 409A. The Severance Benefits offered to you by the Company are payable in reliance on Treasury Regulation Section 1.409A-1(b)(9) and the short term deferral exemption in Treasury Regulation Section 1.409A-1(b)(4). For purposes of Code Section 409A, your right to receive any installment payments (whether pay in lieu of notice, Severance Benefits, reimbursements or otherwise) shall be treated as a right to receive a series of separate payments and, accordingly, each installment payment shall at all times be considered a separate and distinct payment. All payments and benefits are subject to applicable withholdings and deductions.

3. Release. In exchange for the Severance Benefits and other consideration, to which you would not otherwise be entitled, and except as otherwise set forth in this Agreement, you, on behalf of yourself and, to the extent permitted by law, on behalf of your spouse, heirs, executors, administrators, assigns, insurers, attorneys and other persons or entities, acting or purporting to act on your behalf (collectively, the "***Employee Parties***"), hereby generally and completely release, acquit and forever discharge the Company, its parents and subsidiaries, and its and their officers, directors, managers, partners, agents, representatives, employees, attorneys, shareholders, predecessors, successors, assigns, insurers and affiliates (the "***Company Parties***") of and from any and all claims, liabilities, demands, contentions, actions, causes of action, suits, costs, expenses, attorneys' fees, damages, indemnities, debts, judgments, levies, executions and obligations of every kind and nature, in law, equity, or otherwise, both known and unknown, suspected and unsuspected, disclosed and undisclosed, arising out of or in any way related to my employment with the Company and separation therefrom, arising at any time prior to and including the execution date of this Agreement, including but not limited to: all such claims and demands directly or indirectly arising out of or in any way connected with your employment with the Company or the termination of that employment; claims or demands related to salary, bonuses, commissions, vacation pay, the right to receive additional grants of stock, stock options or other ownership interests in the Company, fringe

benefits, expense reimbursements, severance pay, or any other form of compensation; claims pursuant to any federal, state or local law, statute, or cause of action; tort law; or contract law (individually a "***Claim***" and collectively "***Claims***"). The Claims you are releasing and waiving in this Agreement include, but are not limited to, any and all Claims that any of the Company Parties:

has violated its personnel policies, handbooks, contracts of employment, or covenants of good faith and fair dealing;

has discriminated against you on the basis of age, race, color, sex (including sexual harassment), national origin, ancestry, disability, religion, sexual orientation, marital status, parental status, source of income, entitlement to benefits, any union activities or other protected category in violation of any local, state or federal law, constitution, ordinance, or regulation, including but not limited to: the Age Discrimination in Employment Act, as amended ("***ADEA***"); Title VII of the Civil Rights Act of 1964, as amended; the Civil Rights Act of 1991; 42 U.S.C. § 1981, as amended; the Equal Pay Act; the Americans With Disabilities Act; the Genetic Information Nondiscrimination Act; the Family and Medical Leave Act; the New York State Human Rights Law, the New York Equal Opportunity for Disabled Persons Act; the New York City Human Rights Law; the Employee Retirement Income Security Act; the Employee Polygraph Protection Act; the Worker Adjustment and Retraining Notification Act; the Older Workers Benefit Protection Act; the anti-retaliation provisions of the Sarbanes-Oxley Act, or any other federal or state law regarding whistleblower retaliation; the Lilly Ledbetter Fair Pay Act; the Uniformed Services Employment and Reemployment Rights Act; the Fair Credit Reporting Act; and the National Labor Relations Act; and

has violated any statute, public policy or common law (including, but not limited to, Claims for retaliatory discharge; negligent hiring, retention or supervision; defamation; intentional or negligent infliction of emotional distress and/or mental anguish; intentional interference with contract; negligence; detrimental reliance; loss of consortium to you or any member of your family and/or promissory estoppel).

Notwithstanding the foregoing, other than events expressly contemplated by this Agreement you do not waive or release rights or Claims that may arise: (i) from events that occur after the date this Release is executed; (ii) that relate to a breach of this Agreement; (iii) that relate to any existing ownership interest in the Company as of the date this Release is executed; (iv) that relate to your existing rights under any Company benefit plan or any plan or agreement related to equity ownership in the Company that arise after this Release is executed; and (v) any Claims which cannot be waived by law, including, without limitation, any rights you may have under applicable workers' compensation laws. Nothing in this Agreement shall prevent you from filing, cooperating with, or participating in any proceeding or investigation before the Equal Employment Opportunity Commission, United States Department of Labor, the National Labor Relations Board, the Occupational Safety and Health Administration, the Securities and Exchange Commission or any other federal government agency, or similar state or local agency ("***Government Agencies***"), or exercising any rights pursuant to Section 7 of the National Labor Relations Act. You further understand this Agreement does not limit your ability to voluntarily communicate with any Government Agencies or otherwise participate in any investigation or proceeding that may be

conducted by any Government Agency, including providing documents or other information, without notice to the Company. While this Agreement does not limit your right to receive an award for information provided to the Securities and Exchange Commission, you understand and agree that, you are otherwise waiving, to the fullest extent permitted by law, any and all rights you may have to individual relief based on any Claims that you have released and any rights you have waived by signing this Agreement. If any Claim is not subject to release, to the extent permitted by law, you waive any right or ability to be a class or collective action representative or to otherwise participate in any putative or certified class, collective or multi-party action or proceeding based on such a Claim in which any of the Company Parties is a party.

4. Your Acknowledgments and Affirmations. You also acknowledge and agree that (i) the consideration given to you in exchange for the waiver and release in this Agreement is in addition to anything of value to which you were already entitled, and (ii) that you have been paid for all time worked, have received all the leave, leaves of absence and leave benefits and protections for which you are eligible, and have not suffered any on-the-job injury for which you have not already filed a Claim. You affirm that all of the decisions of the Company Parties regarding your pay and benefits through the date of your execution of this Agreement were not discriminatory based on age, disability, race, color, sex, religion, national origin or any other classification protected by law. You affirm that you have not filed or caused to be filed, and are not presently a party to, a Claim against any of the Company Parties. You further affirm that you have no known workplace injuries or occupational diseases. You acknowledge and affirm that you have not been retaliated against for reporting any allegation of corporate fraud or other wrongdoing by any of the Company Parties, or for exercising any rights protected by law, including any rights protected by the Fair Labor Standards Act, the Family Medical Leave Act or any related statute or local leave or disability accommodation laws, or any applicable state workers' compensation law. In addition, you acknowledge that you are knowingly and voluntarily waiving and releasing any rights you may have under the ADEA ("***ADEA Waiver***"). You also acknowledge that the consideration given for the ADEA Waiver is in addition to anything of value to which you were already entitled. You further acknowledge that you have been advised by this writing, as required by the ADEA, that: (a) your release and waiver herein does not apply to any rights or claims that arise after the date you sign this Agreement; (b) you should consult with an attorney prior to signing this Agreement; (c) you have **[twenty-one (21)/forty-five (45)]** days to consider this Agreement (although you may choose to voluntarily sign it sooner); (d) you have seven (7) days following the date you sign this Agreement to revoke it; and (e) the Agreement will not be effective until the date upon which the revocation period has expired unexercised, which will be the eighth (8th) day after you sign this Agreement.

5. Return of Company Property. Within five (5) days following the Separation Date, you agree to return to the Company all Company documents (and all copies thereof) and other Company property that you have had in your possession at any time, including, but not limited to, Company files, notes, drawings, records, business plans and forecasts, financial information, specifications, computer-recorded information, tangible property (including, but not limited to, computers), credit cards, entry cards, identification badges and keys; and, any materials of any kind that contain or embody any proprietary or confidential information of the Company (and all reproductions thereof). Please coordinate return of Company property with [ ]. Receipt of the Severance Benefits described in Section 1 of this Agreement is expressly conditioned upon return of all Company property.

6. Confidential Information, Non-Competition and Non-Solicitation Obligations. Both during and after your employment you acknowledge your continuing obligations under your Employee Confidential Information, Inventions, Non-Solicitation and Non-Competition Agreement not to use or disclose any confidential or proprietary information of the Company and to comply with your post-employment non-competition and non-solicitation restrictions. The Company acknowledges that you will not be held criminally or civilly liable under any federal or state trade secret law for the disclosure of a trade secret that: (A) is made (i) in confidence to a federal, state, or local government official, either directly or indirectly, or to an attorney and (ii) solely for the purpose of reporting or investigating a suspected violation of law; or (B) is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal. In addition, in the event that you file a lawsuit for retaliation by the Company for reporting a suspected violation of law, you may disclose the trade secret to your attorney and use the trade secret information in the court proceeding, if you: (A) file any document containing the trade secret under seal; and (B) do not disclose the trade secret, except pursuant to court order.

7. Confidentiality. The provisions of this Agreement will be held in strictest confidence by you and will not be publicized or disclosed in any manner whatsoever; *provided, however*, that (a) you may disclose this Agreement to your immediate family; (b) you may disclose this Agreement in confidence to your attorney, accountant, auditor, tax preparer, and financial advisor, and (c) you may disclose this Agreement insofar as such disclosure may be required by law. Notwithstanding the foregoing, nothing in this Agreement shall limit your right to voluntarily communicate with the Equal Employment Opportunity Commission, United States Department of Labor, the National Labor Relations Board, the Securities and Exchange Commission, other federal government agency or similar state or local agency or to discuss the terms and conditions of your employment with others to the extent expressly permitted by Section 7 of the National Labor Relations Act.

8. Non-Disparagement. You and the Company agree not to disparage each other, and the other' s attorneys, directors, managers, partners, employees, agents and affiliates, in any manner likely to be harmful to them or their business, business reputation or personal reputation; provided that you and the Company will respond accurately and folly to any question, inquiry or request for information when required by legal process. The Company' s obligations under this Section are limited to Company representatives with knowledge of this provision. Notwithstanding the foregoing, nothing in this Agreement shall limit your right to voluntarily communicate with the Equal Employment Opportunity Commission, United States Department of Labor, the National Labor Relations Board, the Securities and Exchange Commission, other federal government agency or similar state or local agency or to discuss the terms and conditions of your employment with others to the extent expressly permitted by Section 7 of the National Labor Relations Act.

9. Cooperation. You agree to reasonably cooperate with the Company in all matters relating to the winding up of your pending work including, but not limited to, any litigation in which the Company is involved, and the orderly transfer of any such pending work to such other executives as may be designated by the Company; provided, that the Company agrees that the Company (a) shall make reasonable efforts to minimize disruption of your other activities, and (b) shall reimburse you for all reasonable expenses incurred in connection with such cooperation.

10. No Admission. This Agreement does not constitute an admission by you or by the Company of any wrongful action or violation of any federal, state, or local statute, or common law rights, including those relating to the provisions of any law or statute concerning employment actions, or of any other possible or claimed violation of law or rights.

11. Breach. You agree that upon any material breach .of this Agreement you will forfeit all amounts paid or owing to you under this Agreement. Further, you acknowledge that it may be impossible to assess the damages caused by your violation of the terms of Sections 5, 6, 7 and 8 of this Agreement and further agree that any threatened or actual violation or breach of those Sections of this Agreement will constitute immediate and irreparable injury to the Company. You therefore agree that, in addition to any and all other damages and remedies available to the Company upon your breach of this Agreement, the Company shall be entitled to an injunction to prevent you from violating or breathing this Agreement.

12. Miscellaneous. This Agreement is entered into without reliance on any promise or representation, written or oral, other than those expressly contained herein, and it supersedes any other such promises, warranties or representations. This Agreement may not be modified or amended except in a writing signed by both you and a duly authorized officer of the Company. This Agreement will bind the heirs, personal representatives, successors and assigns of both you and the Company, and inure to the benefit of both you and the Company, their heirs, successors and assigns. If any provision of this Agreement is determined to be invalid or unenforceable, in whole or in part, this determination will not affect any other provision of this Agreement and the provision in question will be modified by the court so as to be rendered enforceable. This Agreement will be deemed to have been entered into and will be construed and enforced in accordance with the laws of the State of New York as applied to contracts made and to be performed entirely within the State of New York.

Prevail Therapeutics Inc.

By: _____
Name:
Title:

_____
Franz Hefti

Exhibit 10.10

PREVAIL THERAPEUTICS INC.

NON-EMPLOYEE DIRECTOR COMPENSATION POLICY

Each member of the Board of Directors (the "***Board***") of Prevail Therapeutics Inc. (the "***Company***") who is a non-employee director of the Company (each such member, a "***Non-Employee Director***") will receive the compensation described in this Non-Employee Director Compensation Policy (the "***Director Compensation Policy***") for his or her Board service following the closing of the initial public offering of the Company' s common stock (the "***IPO***").

The Director Compensation Policy will be effective upon the closing of the IPO (the "***IPO Date***"). The Director Compensation Policy may be amended at any time in the sole discretion of the Board or the Compensation Committee of the Board.

A Non-Employee Director may decline all or any portion of his or her compensation by giving notice to the Company prior to the date cash is to be paid or equity awards are to be granted, as the case may be.

Annual Cash Compensation

Commencing at the beginning of the first calendar quarter following the IPO Date, each Non-Employee Director will receive the cash compensation set forth below for service on the Board. The annual cash compensation amounts will be payable in equal quarterly installments, in arrears following the end of each quarter in which the service occurred, pro-rated for any partial months of service. All annual cash fees are vested upon payment.

1. Annual Board Service Retainer:
    a. All Eligible Directors: $38,750
2. Annual Board Chair Service Retainer (in lieu of Board Service Retainer):
    a. Chair of the Board: $75,000
3. Annual Committee Member Service Retainer:
    a. Member of the Audit Committee: $7,500
    b. Member of the Compensation Committee: $5,000
    c. Member of the Nominating and Corporate Governance Committee: $4,000
4. Annual Committee Chair Service Retainer (in lieu of Committee Member Service Retainer):
    a. Chair of the Audit Committee: $15,000
    b. Chair of the Compensation Committee: $10,000
    c. Chair of the Nominating and Corporate Governance Committee: $8,000

Equity Compensation

Equity awards will be granted under the Company' s 2019 Equity Incentive Plan (the "***Plan***"), adopted in connection with the IPO. All stock options granted under this policy will be Nonstatutory Stock Options (as defined in the Plan), with a term of ten years from the date of grant and an exercise price per share equal to 100% of the Fair Market Value (as defined in the Plan) of the underlying common stock of the Company on the date of grant.

(a) Automatic Equity Grants.

(i) Initial Grant for New Directors. Without any further action of the Board, each person who, after the IPO Date, is elected or appointed for the first time to be a Non-Employee Director will automatically, upon the date of his or her initial election or appointment to be a Non-Employee Director (or, if such date is not a market trading day, the first market trading day thereafter), be granted a Nonstatutory Stock Option to purchase 20,000 shares of common stock of the Company (the "***Initial Option Grant***"). Each Initial Option Grant will vest in a series of 36 successive equal monthly installments over the three-year period measured from the date of grant.

(ii) Annual Grant. Without any further action of the Board, at the close of business on the date of each Annual Meeting following the IPO, each person who is then a Non-Employee Director will automatically be granted a Nonstatutory Stock Option to purchase 10,000 shares of common stock (the "***Annual Option Grant***"). Each Annual Option Grant will vest on the earlier of (1) the one-year anniversary of the date of grant and (2) the date immediately prior to the next following annual stockholder meeting of the Company.

(b) Vesting; Change in Control. All vesting is subject to the Non-Employee Director' s "***Continuous Service***" (as defined in the Plan) on each applicable vesting date. Notwithstanding the foregoing vesting schedules, for each Non-Employee Director who remains in Continuous Service with the Company until immediately prior to the closing of a "***Change in Control***" (as defined in the Plan), the shares subject to his or her then-outstanding equity awards that were granted pursuant to this policy will become fully vested immediately prior to the closing of such Change in Control.

(c) Remaining Terms. The remaining terms and conditions of each award, including transferability, will be as set forth in the Company' s Director Option Grant Package in the form adopted from time to time by the Board.

Expenses

The Company will reimburse Non-Employee Director for ordinary, necessary and reasonable out-of-pocket travel expenses to cover in-person attendance at and participation in Board and committee meetings; *provided* that the Non-Employee Director timely submit to the Company appropriate documentation substantiating such expenses in accordance with the Company' s travel and expense policy, as in effect from time to time.

Exhibit 10.11

PREVAIL THERAPEUTICS INC.

2019 EMPLOYEE STOCK PURCHASE PLAN

ADOPTED BY THE BOARD OF DIRECTORS: JUNE 6, 2019
APPROVED BY THE STOCKHOLDERS: JUNE 6, 2019
IPO DATE: JUNE , 2019

1. GENERAL; PURPOSE.

(a) The Plan provides a means by which Eligible Employees of the Company and certain designated Related Corporations may be given an opportunity to purchase shares of Common Stock. The Plan permits the Company to grant a series of Purchase Rights to Eligible Employees under an Employee Stock Purchase Plan.

(b) The Company, by means of the Plan, seeks to retain the services of such Employees, to secure and retain the services of new Employees and to provide incentives for such persons to exert maximum efforts for the success of the Company and its Related Corporations.

2. ADMINISTRATION.

(a) The Board will administer the Plan unless and until the Board delegates administration of the Plan to a Committee or Committees, as provided in Section 2(c).

(b) The Board will have the power, subject to, and within the limitations of, the express provisions of the Plan:

(i) To determine how and when Purchase Rights will be granted and the provisions of each Offering (which need not be identical).

(ii) To designate from time to time which Related Corporations of the Company will be eligible to participate in the Plan.

(iii) To construe and interpret the Plan and Purchase Rights, and to establish, amend and revoke rules and regulations for its administration. The Board, in the exercise of this power, may correct any defect, omission or inconsistency in the Plan, in a manner and to the extent it deems necessary or expedient to make the Plan fully effective.

(iv) To settle all controversies regarding the Plan and Purchase Rights granted under the Plan.

(v) To suspend or terminate the Plan at any time as provided in Section 12.

(vi) To amend the Plan at any time as provided in Section 12.

(vii) Generally, to exercise such powers and to perform such acts as it deems necessary or expedient to promote the best interests of the Company and its Related Corporations and to carry out the intent that the Plan be treated as an Employee Stock Purchase Plan.

(viii) To adopt such rules, procedures and sub-plans relating to the operation and administration of the Plan as are necessary or appropriate under applicable local laws, regulations and procedures to permit or facilitate participation in the Plan by Employees who are foreign nationals or employed or located outside the United States.

(c) The Board may delegate some or all of the administration of the Plan to a Committee or Committees. If administration is delegated to a Committee, the Committee will have, in connection with the administration of the Plan, the powers theretofore possessed by the Board that have been delegated to the Committee, including the power to delegate to a subcommittee any of the administrative powers the Committee is authorized to exercise (and references in this Plan to the Board will thereafter be to the Committee or subcommittee), subject, however, to such resolutions, not inconsistent with the provisions of the Plan, as may be adopted from time to time by the Board. The Board may retain the authority to concurrently administer the Plan with the Committee and may, at any time, revest in the Board some or all of the powers previously delegated. Whether or not the Board has delegated administration of the Plan to a Committee, the Board will have the final power to determine all questions of policy and expediency that may arise in the administration of the Plan.

(d) All determinations, interpretations and constructions made by the Board in good faith will not be subject to review by any person and will be final, binding and conclusive on all persons.

3. SHARES OF COMMON STOCK SUBJECT TO THE PLAN.

(a) Subject to the provisions of Section 11(a) relating to Capitalization Adjustments, the maximum number of shares of Common Stock that may be issued under the Plan will not exceed 330,000 shares of Common Stock, plus the number of shares of Common Stock that are automatically added on January 1st of each year for a period of up to ten years, commencing on the first January 1 following the year in which the IPO Date occurs and ending on (and including) January 1, 2029, in an amount equal to the lesser of (i) 1% of the total number of shares of Capital Stock outstanding on December 31st of the preceding calendar year, and (ii) 1,500,000 shares of Common Stock. Notwithstanding the foregoing, the Board may act prior to the first day of any calendar year to provide that there will be no January 1st increase in the share reserve for such calendar year or that the increase in the share reserve for such calendar year will be a lesser number of shares of Common Stock than would otherwise occur pursuant to the preceding sentence.

(b) If any Purchase Right granted under the Plan terminates without having been exercised in full, the shares of Common Stock not purchased under such Purchase Right will again become available for issuance under the Plan.

(c) The stock purchasable under the Plan will be shares of authorized but unissued or reacquired Common Stock, including shares repurchased by the Company on the open market.

4. GRANT OF PURCHASE RIGHTS; OFFERING.

(a) The Board may from time to time grant or provide for the grant of Purchase Rights to Eligible Employees under an Offering (consisting of one or more Purchase Periods) on an Offering Date or Offering Dates selected by the Board. Each Offering will be in such form and will contain such terms and conditions as the Board will deem appropriate, and will comply with the requirement of Section 423(b)(5) of the Code that all Employees granted Purchase Rights will have the same rights and privileges. The terms and conditions of an Offering shall be incorporated by reference into the Plan and treated as part of the Plan. The provisions of separate Offerings need not be identical, but each Offering will include (through incorporation of the provisions of this Plan by reference in the document comprising the Offering or otherwise) the period during which the Offering will be effective, which period will not exceed 27 months beginning with the Offering Date, and the substance of the provisions contained in Sections 5 through 8, inclusive.

(b) If a Participant has more than one Purchase Right outstanding under the Plan, unless he or she otherwise indicates in forms delivered to the Company: (i) each form will apply to all of his or her Purchase Rights under the Plan, and (ii) a Purchase Right with a lower exercise price (or an earlier-granted Purchase Right, if different Purchase Rights have identical exercise prices) will be exercised to the fullest possible extent before a Purchase Right with a higher exercise price (or a later-granted Purchase Right if different Purchase Rights have identical exercise prices) will be exercised.

(c) The Board will have the discretion to structure an Offering so that if the Fair Market Value of a share of Common Stock on the first Trading Day of a new Purchase Period within that Offering is less than or equal to the Fair Market Value of a share of Common Stock on the Offering Date for that Offering, then (i) that Offering will terminate immediately as of that first Trading Day, and (ii) the Participants in such terminated Offering will be automatically enrolled in a new Offering beginning on the first Trading Day of such new Purchase Period.

5. ELIGIBILITY.

(a) Purchase Rights may be granted only to Employees of the Company or, as the Board may designate in accordance with Section 2(b), to Employees of a Related Corporation. Except as provided in Section 5(b), an Employee will not be eligible to be granted Purchase Rights unless, on the Offering Date, the Employee has been in the employ of the Company or the Related Corporation, as the case may be, for such continuous period preceding such Offering Date as the Board may require, but in no event will the required period of continuous employment be equal to or greater than two years. In addition, the Board may (unless prohibited by law) provide that no Employee will be eligible to be granted Purchase Rights under the Plan unless, on the Offering Date, such Employee' s customary employment with the Company or the Related Corporation is more than 20 hours per week and more than five months per calendar year or such other criteria as the Board may determine consistent with Section 423 of the Code. The Board may also exclude from participation in the Plan or any Offering Employees who are "highly compensated employees" (within the meaning of Section 414(q) of the Code) of the Company or a Related Corporation or a subset of such highly compensated employees.

(b) The Board may provide that each person who, during the course of an Offering, first becomes an Eligible Employee will, on a date or dates specified in the Offering which coincides with the day on which such person becomes an Eligible Employee or which occurs thereafter, receive a Purchase Right under that Offering, which Purchase Right will thereafter be deemed to be a part of that Offering. Such Purchase Right will have the same characteristics as any Purchase Rights originally granted under that Offering, as described herein, except that:

(i) the date on which such Purchase Right is granted will be the "Offering Date" of such Purchase Right for all purposes, including determination of the exercise price of such Purchase Right;

(ii) the period of the Offering with respect to such Purchase Right will begin on its Offering Date and end coincident with the end of such Offering; and

(iii) the Board may provide that if such person first becomes an Eligible Employee within a specified period of time before the end of the Offering, he or she will not receive any Purchase Right under that Offering.

(c) No Employee will be eligible for the grant of any Purchase Rights if, immediately after any such Purchase Rights are granted, such Employee owns stock possessing five percent or more of the total combined voting power or value of all classes of stock of the Company or of any Related Corporation. For purposes of this Section 5(c), the rules of Section 424(d) of the Code will apply in determining the stock ownership of any Employee, and stock which such Employee may purchase under all outstanding Purchase Rights and options will be treated as stock owned by such Employee.

(d) As specified by Section 423(b)(8) of the Code, an Eligible Employee may be granted Purchase Rights only if such Purchase Rights, together with any other rights granted under all Employee Stock Purchase Plans of the Company and any Related Corporations, do not permit such Eligible Employee' s rights to purchase stock of the Company or any Related Corporation to accrue at a rate which, when aggregated, exceeds US $25,000 of Fair Market Value of such stock (determined at the time such rights are granted, and which, with respect to the Plan, will be determined as of their respective Offering Dates) for each calendar year in which such rights are outstanding at any time.

(e) Officers of the Company and any designated Related Corporation, if they are otherwise Eligible Employees, will be eligible to participate in Offerings under the Plan. Notwithstanding the foregoing, the Board may (unless prohibited by law) provide in an Offering that Employees who are highly compensated Employees within the meaning of Section 423(b)(4)(D) of the Code will not be eligible to participate.

6. PURCHASE RIGHTS; PURCHASE PRICE.

(a) On each Offering Date, each Eligible Employee, pursuant to an Offering made under the Plan, will be granted a Purchase Right to purchase up to that number of shares of Common Stock purchasable either with a percentage or with a maximum dollar amount, as designated by the Board, but in either case not exceeding 15% of such Employee' s earnings (as defined by the Board in each Offering) during the period that begins on the Offering Date (or such later date as the Board determines for a particular Offering) and ends on the date stated in the Offering, which date will be no later than the end of the Offering.

(b) The Board will establish one or more Purchase Dates during an Offering on which Purchase Rights granted for that Offering will be exercised and shares of Common Stock will be purchased in accordance with such Offering.

(c) In connection with each Offering made under the Plan, the Board may specify (i) a maximum number of shares of Common Stock that may be purchased by any Participant on any Purchase Date during such Offering, (ii) a maximum aggregate number of shares of Common Stock that may be purchased by all Participants pursuant to such Offering and/or (iii) a maximum aggregate number of shares of Common Stock that may be purchased by all Participants on any Purchase Date under the Offering. If the aggregate purchase of shares of Common Stock issuable upon exercise of Purchase Rights granted under the Offering would exceed any such maximum aggregate number, then, in the absence of any Board action otherwise, a pro rata (based on each Participant' s accumulated Contributions) allocation of the shares of Common Stock (rounded down to the nearest whole share) available will be made in as nearly a uniform manner as will be practicable and equitable.

(d) The purchase price of shares of Common Stock acquired pursuant to Purchase Rights will be not less than the lesser of:

(i) an amount equal to 85% of the Fair Market Value of the shares of Common Stock on the Offering Date; or

(ii) an amount equal to 85% of the Fair Market Value of the shares of Common Stock on the applicable Purchase Date.

7. PARTICIPATION; WITHDRAWAL; TERMINATION.

(a) An Eligible Employee may elect to participate in an Offering and authorize payroll deductions as the means of making Contributions by completing and delivering to the Company, within the time specified in the Offering, an enrollment form provided by the Company. The enrollment form will specify the amount of Contributions not to exceed the maximum amount specified by the Board. Each Participant' s Contributions will be credited to a bookkeeping account for such Participant under the Plan and will be deposited with the general funds of the Company except where applicable law or regulations requires that Contributions be deposited with a third party. If permitted in the Offering, a Participant may begin such Contributions with the first payroll occurring on or after the Offering Date (or, in the case of a payroll date that occurs after the end of the prior Offering but before the Offering Date of the next new Offering, Contributions from such payroll will be included in the new Offering). If permitted in the Offering, a Participant may thereafter reduce (including to zero) or increase his or her Contributions. If required under applicable law or regulations or if specifically provided in the Offering, in addition to or instead of making Contributions by payroll deductions, a Participant may make Contributions through the payment by cash, check or wire transfer prior to a Purchase Date.

(b) During an Offering, a Participant may cease making Contributions and withdraw from the Offering by delivering to the Company a withdrawal form provided by the Company. The Company may impose a deadline before a Purchase Date for withdrawing. Upon such withdrawal, such Participant' s Purchase Right in that Offering will immediately terminate and the Company will distribute as soon as practicable to such Participant all of his or her accumulated but unused Contributions and such Participant' s Purchase Right in that Offering shall thereupon terminate. A Participant' s withdrawal from that Offering will have no effect upon his or her eligibility to participate in any other Offerings under the Plan, but such Participant will be required to deliver a new enrollment form to participate in subsequent Offerings.

(c) Unless otherwise required by applicable law or regulations, Purchase Rights granted pursuant to any Offering under the Plan will terminate immediately if the Participant either (i) is no longer an Employee for any reason or for no reason (subject to any post-employment participation period required by law) or (ii) is otherwise no longer eligible to participate. The Company will distribute as soon as practicable to such individual all of his or her accumulated but unused Contributions.

(d) During a Participant' s lifetime, Purchase Rights will be exercisable only by such Participant. Purchase Rights are not transferable by a Participant, except by will, by the laws of descent and distribution, or, if permitted by the Company, by a beneficiary designation as described in Section 10.

(e) Unless otherwise specified in the Offering or required by applicable law or regulations, the Company will have no obligation to pay interest on Contributions.

8. EXERCISE OF PURCHASE RIGHTS.

(a) On each Purchase Date, each Participant' s accumulated Contributions will be applied to the purchase of shares of Common Stock, up to the maximum number of shares of Common Stock permitted by the Plan and the applicable Offering, at the purchase price specified in the Offering. No fractional shares will be issued unless specifically provided for in the Offering.

(b) Unless otherwise provided in the Offering, if any amount of accumulated Contributions remains in a Participant' s account after the purchase of shares of Common Stock on the final Purchase Date of an Offering, then such remaining amount will not roll over to the next Offering and will instead be distributed in full to such Participant after the final Purchase Date of such Offering without interest (unless otherwise required by applicable law or regulations).

(c) No Purchase Rights may be exercised to any extent unless the shares of Common Stock to be issued upon such exercise under the Plan are covered by an effective registration statement pursuant to the Securities Act and the Plan is in material compliance with all applicable federal, state, foreign and other securities and other laws applicable to the Plan. If on a Purchase Date the shares of Common Stock are not so registered or the Plan is not in such compliance, no Purchase Rights will be exercised on such Purchase Date, and the Purchase Date

will be delayed until the shares of Common Stock are subject to such an effective registration statement and the Plan is in material compliance, except that the Purchase Date will in no event be more than 27 months from the Offering Date. If, on the Purchase Date, as delayed to the maximum extent permissible, the shares of Common Stock are not registered and the Plan is not in material compliance with all applicable laws and regulations, no Purchase Rights will be exercised and all accumulated but unused Contributions will be distributed to the Participants without interest.

9. COVENANTS OF THE COMPANY.

The Company will seek to obtain from each federal, state, foreign or other regulatory commission or agency having jurisdiction over the Plan such authority as may be required to grant Purchase Rights and issue and sell shares of Common Stock thereunder unless the Company determines, in its sole discretion, that doing so would cause the Company to incur costs that are unreasonable. If, after commercially reasonable efforts, the Company is unable to obtain the authority that counsel for the Company deems necessary for the grant of Purchase Rights or the lawful issuance and sale of Common Stock under the Plan, and at a commercially reasonable cost, the Company will be relieved from any liability for failure to grant Purchase Rights and/or to issue and sell Common Stock upon exercise of such Purchase Rights.

10. DESIGNATION OF BENEFICIARY.

(a) The Company may, but is not obligated to, permit a Participant to submit a form designating a beneficiary who will receive any shares of Common Stock and/or Contributions from the Participant' s account under the Plan if the Participant dies before such shares and/or Contributions are delivered to the Participant. The Company may, but is not obligated to, permit the Participant to change such designation of beneficiary. Any such designation and/or change must be on a form approved by the Company.

(b) If a Participant dies, and in the absence of a valid beneficiary designation, the Company will deliver any shares of Common Stock and/or Contributions to the executor or administrator of the estate of the Participant. If no executor or administrator has been appointed (to the knowledge of the Company), the Company, in its sole discretion, may deliver such shares of Common Stock and/or Contributions, without interest, to the Participant' s spouse, dependents or relatives, or if no spouse, dependent or relative is known to the Company, then to such other person as the Company may designate.

11. ADJUSTMENTS UPON CHANGES IN COMMON STOCK; CORPORATE TRANSACTIONS.

(a) In the event of a Capitalization Adjustment, the Board will appropriately and proportionately adjust: (i) the class(es) and maximum number of securities subject to the Plan pursuant to Section 3(a), (ii) the class(es) and maximum number of securities by which the share reserve is to increase automatically each year pursuant to Section 3(a), (iii) the class(es) and number of securities subject to, and the purchase price applicable to outstanding Offerings and Purchase Rights, and (iv) the class(es) and number of securities that are the subject of the purchase limits under each ongoing Offering. The Board will make these adjustments, and its determination will be final, binding and conclusive.

(b) In the event of a Corporate Transaction, then: (i) any surviving corporation or acquiring corporation (or the surviving or acquiring corporation' s parent company) may assume or continue outstanding Purchase Rights or may substitute similar rights (including a right to acquire the same consideration paid to the stockholders in the Corporate Transaction) for outstanding Purchase Rights, or (ii) if any surviving or acquiring corporation (or its parent company) does not assume or continue such Purchase Rights or does not substitute similar rights for such Purchase Rights, then the Participants' accumulated Contributions will be used to purchase shares of Common Stock (rounded down to the nearest whole share) within ten business days prior to the Corporate Transaction under the outstanding Purchase Rights, and the Purchase Rights will terminate immediately after such purchase.

12. AMENDMENT, TERMINATION OR SUSPENSION OF THE PLAN.

(a) The Board may amend the Plan at any time in any respect the Board deems necessary or advisable. However, except as provided in Section 11(a) relating to Capitalization Adjustments, stockholder approval will be required for any amendment of the Plan for which stockholder approval is required by applicable law, regulations or listing requirements.

(b) The Board may suspend or terminate the Plan at any time. No Purchase Rights may be granted under the Plan while the Plan is suspended or after it is terminated.

(c) Any benefits, privileges, entitlements and obligations under any outstanding Purchase Rights granted before an amendment, suspension or termination of the Plan will not be materially impaired by any such amendment, suspension or termination except (i) with the consent of the person to whom such Purchase Rights were granted, (ii) as necessary to comply with any laws, listing requirements, or governmental regulations (including, without limitation, the provisions of Section 423 of the Code and the regulations and other interpretive guidance issued thereunder relating to Employee Stock Purchase Plans) including without limitation any such regulations or other guidance that may be issued or amended after the date the Plan is adopted by the Board, or (iii) as necessary to obtain or maintain favorable tax, listing, or regulatory treatment. To be clear, the Board may amend outstanding Purchase Rights without a Participant' s consent if such amendment is necessary to ensure that the Purchase Right and/or the Plan complies with the requirements of Section 423 of the Code.

13. EFFECTIVE DATE OF PLAN.

The Plan will become effective immediately prior to and contingent upon the IPO Date. No Purchase Rights will be exercised unless and until the Plan has been approved by the stockholders of the Company, which approval must be within 12 months before or after the date the Plan is adopted (or if required under Section 12(a) above, materially amended) by the Board.

14. MISCELLANEOUS PROVISIONS.

(a) Proceeds from the sale of shares of Common Stock pursuant to Purchase Rights will constitute general funds of the Company.

(b) A Participant will not be deemed to be the holder of, or to have any of the rights of a holder with respect to, shares of Common Stock subject to Purchase Rights unless and until the Participant' s shares of Common Stock acquired upon exercise of Purchase Rights are recorded in the books of the Company (or its transfer agent).

(c) The Plan and Offering do not constitute an employment contract. Nothing in the Plan or in the Offering will in any way alter the at will nature of a Participant' s employment, if applicable, or be deemed to create in any way whatsoever any obligation on the part of any Participant to continue in the employ of the Company or a Related Corporation, or on the part of the Company or a Related Corporation to continue the employment of a Participant.

(d) The provisions of the Plan will be governed by the laws of the State of Delaware without resort to that state' s conflicts of laws rules.

(e) If any particular provision of the Plan is found to be invalid or otherwise unenforceable, such provision will not affect the other provisions of the Plan, but the Plan will be construed in all respects as if such invalid provision were omitted.

(f) If any provision of the Plan does not comply with applicable law or regulations, such provision shall be construed in such a manner as to comply with applicable law or regulations.

15. DEFINITIONS.

As used in the Plan, the following definitions will apply to the capitalized terms indicated below:

(a) "***Board***" means the Board of Directors of the Company.

(b) "***Capital Stock***" means each and every class of common stock of the Company, regardless of the number of votes per share.

(c) "***Capitalization Adjustment***" means any change that is made in, or other events that occur with respect to, the Common Stock subject to the Plan or subject to any Purchase Right after the date the Plan is adopted by the Board without the receipt of consideration by the Company through merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, stock split, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or other similar equity restructuring transaction, as that term is used in Financial Accounting Standards Board Accounting Standards Codification Topic 718 (or any successor thereto). Notwithstanding the foregoing, the conversion of any convertible securities of the Company will not be treated as a Capitalization Adjustment.

(d) "***Code***" means the Internal Revenue Code of 1986, as amended, including any applicable regulations and guidance thereunder.

(e) "***Committee***" means a committee of one or more members of the Board to whom authority has been delegated by the Board in accordance with Section 2(c).

(f) "***Common Stock***" means, as of the IPO Date, the common stock of the Company, having one vote per share.

(g) "***Company***" means Prevail Therapeutics Inc., a Delaware corporation.

(h) "***Contributions***" means the payroll deductions and other additional payments specifically provided for in the Offering that a Participant contributes to fund the exercise of a Purchase Right. A Participant may make additional payments into his or her account if specifically provided for in the Offering, and then only if the Participant has not already had the maximum permitted amount withheld during the Offering through payroll deductions.

(i) "***Corporate Transaction***" means the consummation, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) a sale or other disposition of all or substantially all, as determined by the Board in its sole discretion, of the consolidated assets of the Company and its Subsidiaries;

(ii) a sale or other disposition of more than 50% of the outstanding securities of the Company;

(iii) a merger, consolidation or similar transaction following which the Company is not the surviving corporation; or

(iv) a merger, consolidation or similar transaction following which the Company is the surviving corporation but the shares of Common Stock outstanding immediately preceding the merger, consolidation or similar transaction are converted or exchanged by virtue of the merger, consolidation or similar transaction into other property, whether in the form of securities, cash or otherwise.

(j) "***Director***" means a member of the Board.

(k) "***Eligible Employee***" means an Employee who meets the requirements set forth in the document(s) governing the Offering for eligibility to participate in the Offering, provided that such Employee also meets the requirements for eligibility to participate set forth in the Plan.

(l) "***Employee***" means any person, including an Officer or Director, who is "employed" for purposes of Section 423(b)(4) of the Code by the Company or a Related Corporation. However, service solely as a Director, or payment of a fee for such services, will not cause a Director to be considered an "Employee" for purposes of the Plan.

(m) "***Employee Stock Purchase Plan***" means a plan that grants Purchase Rights intended to be options issued under an "employee stock purchase plan," as that term is defined in Section 423(b) of the Code.

(n) "***Exchange Act***" means the Securities Exchange Act of 1934, as amended and the rules and regulations promulgated thereunder.

(o) "***Fair Market Value***" means, as of any date, the value of the Common Stock determined as follows:

(i) If the Common Stock is listed on any established stock exchange or traded on any established market, the Fair Market Value of a share of Common Stock will be the closing sales price for such stock as quoted on such exchange or market (or the exchange or market with the greatest volume of trading in the Common Stock) on the date of determination, as reported in such source as the Board deems reliable. Unless otherwise provided by the Board, if there is no closing sales price for the Common Stock on the date of determination, then the Fair Market Value will be the closing sales price on the last preceding date for which such quotation exists.

(ii) In the absence of such markets for the Common Stock, the Fair Market Value will be determined by the Board in good faith in compliance with applicable laws and regulations and in a manner that complies with Sections 409A of the Code

(iii) Notwithstanding the foregoing, for any Offering that commences on the IPO Date, the Fair Market Value of the shares of Common Stock on the Offering Date will be the price per share at which shares are first sold to the public in the Company' s initial public offering as specified in the final prospectus for that initial public offering.

(p) "***IPO Date***" means the date of the underwriting agreement between the Company and the underwriters managing the initial public offering of the Common Stock, pursuant to which the Common Stock is priced for the initial public offering.

(q) "***Offering***" means the grant to Eligible Employees of Purchase Rights, with the exercise of those Purchase Rights automatically occurring at the end of one or more Purchase Periods. The terms and conditions of an Offering will generally be set forth in the "***Offering Document***" approved by the Board for that Offering.

(r) "***Offering Date***" means a date selected by the Board for an Offering to commence.

(s) "***Officer***" means a person who is an officer of the Company or a Related Corporation within the meaning of Section 16 of the Exchange Act.

(t) "***Participant***" means an Eligible Employee who holds an outstanding Purchase Right.

(u) "***Plan***" means this Prevail Therapeutics Inc. 2019 Employee Stock Purchase Plan.

(v) "***Purchase Date***" means one or more dates during an Offering selected by the Board on which Purchase Rights will be exercised and on which purchases of shares of Common Stock will be carried out in accordance with such Offering.

(w) "***Purchase Period***" means a period of time specified within an Offering, generally beginning on the Offering Date or on the first Trading Day following a Purchase Date, and ending on a Purchase Date. An Offering may consist of one or more Purchase Periods.

(x) "***Purchase Right***" means an option to purchase shares of Common Stock granted pursuant to the Plan.

(y) "***Related Corporation***" means any "parent corporation" or "subsidiary corporation" of the Company whether now or subsequently established, as those terms are defined in Sections 424(e) and (f), respectively, of the Code.

(z) "***Securities Act***" means the Securities Act of 1933, as amended.

(aa) "***Trading Day***" means any day on which the exchange(s) or market(s) on which shares of Common Stock are listed, including but not limited to the NYSE, Nasdaq Global Select Market, the Nasdaq Global Market, the Nasdaq Capital Market or any successors thereto, is open for trading.

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the reference to our firm under the caption "Experts" and to the use of our report dated March 26, 2019, except for the section entitled "Forward Stock Split" in Note 17, as to which the date is June 10, 2019, in Amendment No. 1 to the Registration Statement (Form S-1 No. 333-231754) and related Prospectus of Prevail Therapeutics Inc. for the registration of 8,455,950 shares of its common stock.

/s/ Ernst & Young LLP

New York, New York

June 10, 2019