

SECURITIES AND EXCHANGE COMMISSION

FORM 10-Q

Quarterly report pursuant to sections 13 or 15(d)

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FILER

ZEVRA THERAPEUTICS, INC.

CIK: **1434647** | IRS No.: **205894398** | State of Incorpor.: **DE** | Fiscal Year End: **1231**
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SIC: **2834** Pharmaceutical preparations

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

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2024

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the Transition Period From _____ to _____

Commission File Number: 001-36913

Zevra Therapeutics, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware
(State or Other Jurisdiction of Incorporation or Organization)

20-5894398
(I.R.S. Employer Identification No.)

1180 Celebration Boulevard, Suite 103, Celebration, FL
(Address of Principal Executive Offices)

34747
(Zip Code)

(321) 939-3416

(Registrant's Telephone Number, Including Area Code)

(Former Name, Former Address, and Former Fiscal Year if Changed Since Last Report)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, \$0.0001 par value per share	ZVRA	The Nasdaq Stock Market LLC (Nasdaq Global Select Market)

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of November 8, 2024, the registrant had 53,375,932 shares of common stock outstanding.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, including the section entitled “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” contains forward-looking statements regarding future events and our future results that are subject to the safe harbors created under the Securities Act of 1933, as amended, or the Securities Act, and the Securities Exchange Act of 1934, as amended, or the Exchange Act. Forward-looking statements relate to future events or our future financial performance. We generally identify forward-looking statements by terminology such as “may,” “will,” “would,” “should,” “expects,” “plans,” “anticipates,” “could,” “intends,” “target,” “projects,” “contemplates,” “believes,” “estimates,” “predicts,” “assume,” “intend,” “potential,” “continue” or other similar words or the negative of these terms. We have based these forward-looking statements largely on our current expectations about future events and financial trends that we believe may affect our business, financial condition and results of operations. The outcome of the events described in these forward-looking statements is subject to risks, uncertainties and other factors described in Part II, Item 1A. “Risk Factors” and elsewhere in this Quarterly Report on Form 10-Q and Part I, Item 1A. “Risk Factors” of our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, filed with the SEC on April 1, 2024. Accordingly, you should not place undue reliance upon these forward-looking statements. We cannot assure you that the events and circumstances reflected in the forward-looking statements will be achieved or occur; the timing of events and circumstances and actual results could differ materially from those anticipated in the forward-looking statements. Forward-looking statements contained in this report include, but are not limited to, statements about:

- *our ability to integrate Acer (as defined below) into our business successfully or realize the anticipated synergies and related benefits of the Merger (as defined below);*
- *the progress of, outcome or and timing of any regulatory approval for any of our product candidates and the expected amount or timing of any payment related thereto under any of our collaboration agreements;*
- *our ability to continue as a going concern;*
- *the progress of, timing of and expected amount of expenses associated with our research, development and commercialization activities;*
- *our ability to raise additional funds on commercially reasonable terms, or at all, in order to support our continued operations;*
- *the sufficiency of our cash resources to fund our operating expenses and capital investment requirements for any period;*
- *the expected timing of our clinical trials for our product candidates and the availability of data and results of those trials;*
- *our expectations regarding federal, state and foreign regulatory requirements;*
- *the potential therapeutic benefits and effectiveness of our products and product candidates;*
- *the size and characteristics of the markets that may be addressed by our products and product candidates;*
- *our intention to seek to establish, and the potential benefits to us from, any strategic collaborations or partnerships for the development or sale of our products and product candidates; if approved;*
- *our expectations as to future financial performance, expense levels and liquidity sources;*
- *the timing of commercializing our products and product candidates, if approved;*
- *senior leadership and board member transitions and refreshments; and*
- *other factors discussed elsewhere in this report.*

The forward-looking statements made in this report relate only to events as of the date on which the statements are made. We have included or made reference to important factors in the cautionary statements included in this report, particularly in the section entitled

"Risk Factors" where we make reference to Part I, Item 1A. "Risk Factors" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, filed with the SEC on April 1, 2024, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make. Except as required by law, we do not assume any intent to update any forward-looking statements after the date on which the statement is made, whether as a result of new information, future events or circumstances or otherwise.

NOTE REGARDING COMPANY REFERENCE

Unless the context otherwise requires, we use the terms "Zevra," "Company," "we," "us" and "our" in this Quarterly Report on Form 10-Q to refer to Zevra Therapeutics, Inc., formerly known as KemPharm, Inc. prior to February 21, 2023. We have proprietary rights to a number of trademarks used in this Quarterly Report on Form 10-Q that are important to our business, including LAT® and the Zevra logo. All other trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners. Solely for convenience, the trademarks and trade names in this Quarterly Report on Form 10-Q are referred to without the ® and ™ symbols, 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.

On August 30, 2023, the Company and Aspen Z Merger Sub, Inc., an indirect wholly-owned subsidiary of Zevra ("Merger Sub") entered into an Agreement and Plan of Merger (the "Merger Agreement") with Acer Therapeutics Inc. ("Acer"). On November 17, 2023 (the "Closing Date"), we completed the acquisition of Acer. Pursuant to the Merger Agreement, on the Closing Date, Merger Sub was merged with and into Acer (the "Merger"), with Acer continuing as the surviving entity and as a wholly-owned subsidiary of Zevra.

PART I — FINANCIAL INFORMATION

ITEM 1. UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

ZEVRA THERAPEUTICS, INC. UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS (in thousands, except share and par value amounts)

	September 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	\$ 54,039	\$ 43,049
Securities at fair value, current	35,337	24,688
Accounts and other receivables	7,790	17,377
Prepaid expenses and other current assets	2,276	1,824
Total current assets	99,442	86,938
Inventories	8,756	9,841
Securities at fair value, long-term	6,105	—
Property and equipment, net	607	736
Operating lease right-of-use assets	820	790
Goodwill	4,701	4,701
Intangible assets, net	70,608	69,227
Other long-term assets	512	94
Total assets	<u>\$ 191,551</u>	<u>\$ 172,327</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	\$ 24,464	\$ 28,403
Line of credit payable	—	37,700
Current portion of operating lease liabilities	540	543
Current portion of discount and rebate liabilities	8,547	4,550
Other current liabilities	930	2,524
Total current liabilities	34,481	73,720
Long-term debt	58,904	5,066
Warrant liability	13,902	16,100
Operating lease liabilities, less current portion	483	456
Discount and rebate liabilities, less current portion	8,490	7,663
Other long-term liabilities	5,521	7,458
Total liabilities	<u>121,781</u>	<u>110,463</u>
Commitments and contingencies (Note D)		
Stockholders' equity:		
Preferred stock:		
Undesignated preferred stock, \$0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of September 30, 2024, or December 31, 2023	—	—
Common stock, \$0.0001 par value, 250,000,000 shares authorized, 54,803,056 shares issued and 53,227,364 shares outstanding as of September 30, 2024; 43,110,360 shares issued and 41,534,668 shares outstanding as of December 31, 2023	5	4
Additional paid-in capital	550,413	472,664
Treasury stock, at cost	(10,983)	(10,983)
Accumulated deficit	(469,550)	(399,778)
Accumulated other comprehensive income (loss)	(115)	(43)
Total stockholders' equity	<u>69,770</u>	<u>61,864</u>

Total liabilities and stockholders' equity	\$ 191,551	\$ 172,327
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See accompanying notes to unaudited condensed consolidated financial statements.

ZEVRA THERAPEUTICS, INC.
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share amounts)

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Revenue, net	\$ 3,695	\$ 2,895	\$ 11,569	\$ 14,541
Cost of product revenue (excluding \$1,545 and \$4,619 in intangible asset amortization for the three and nine months ended September 30, 2024, respectively shown separately below)	2,303	144	6,051	946
Intangible asset amortization	1,545	—	4,619	—
Operating expenses:				
Research and development	10,945	12,297	33,743	28,385
Selling, general and administrative	16,208	5,818	38,743	19,657
Total operating expenses	27,153	18,115	72,486	48,042
Loss from operations	(27,306)	(15,364)	(71,587)	(34,447)
Other (expense) income:				
Interest expense	(2,312)	(366)	(5,157)	(745)
Fair value adjustment related to warrant and CVR liability	(4,746)	3,678	4,660	4,253
Fair value adjustment related to investments	90	124	64	451
Interest and other income (expense), net	1,049	1,738	2,248	4,331
Total other (expense) income	(5,919)	5,174	1,815	8,290
Loss before income taxes	(33,225)	(10,190)	(69,772)	(26,157)
Income tax expense	—	(177)	—	—
Net loss	<u>\$ (33,225)</u>	<u>\$ (10,367)</u>	<u>\$ (69,772)</u>	<u>\$ (26,157)</u>
Basic and diluted net loss per share of common stock:				
Net loss	<u>\$ (0.69)</u>	<u>\$ (0.30)</u>	<u>\$ (1.59)</u>	<u>\$ (0.76)</u>
Weighted average number of shares of common stock outstanding:				
Basic and diluted	<u>47,808,817</u>	<u>34,724,614</u>	<u>43,843,851</u>	<u>34,364,075</u>

See accompanying notes to unaudited condensed consolidated financial statements.

ZEVRA THERAPEUTICS, INC.
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(in thousands)

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Net loss	\$ (33,225)	\$ (10,367)	\$ (69,772)	\$ (26,157)
Other comprehensive (loss) income:				
Foreign currency translation adjustment	(536)	5	(72)	(333)
Other comprehensive (loss) income	(536)	5	(72)	(333)
Comprehensive loss	<u>\$ (33,761)</u>	<u>\$ (10,362)</u>	<u>\$ (69,844)</u>	<u>\$ (26,490)</u>

See accompanying notes to unaudited condensed consolidated financial statements.

ZEVRA THERAPEUTICS, INC.
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
(in thousands)

	Common Stock	Additional Paid-in Capital	Treasury Stock, at cost	Accumulated Deficit	Other Comprehensive Income (Loss)	Total Stockholders' Equity
Balance as of January 1, 2024	\$ 4	\$ 472,664	\$ (10,983)	\$ (399,778)	\$ (43)	\$ 61,864
Net loss	—	—	—	(16,622)	—	(16,622)
Stock-based compensation expense	—	2,119	—	—	—	2,119
Issuance of common stock in exchange for consulting services	—	56	—	—	—	56
Issuance of common stock as part of the Employee Stock Purchase Plan	—	71	—	—	—	71
Issuance of common stock for options exercised	—	1,146	—	—	—	1,146
Other comprehensive income	—	—	—	—	184	184
Balance as of March 31, 2024	\$ 4	\$ 476,056	\$ (10,983)	\$ (416,400)	\$ 141	\$ 48,818
Net loss	—	—	—	(19,925)	—	(19,925)
Stock-based compensation expense	—	2,632	—	—	—	2,632
Issuance of common stock in exchange for consulting services	—	193	—	—	—	193
Issuance of common stock as part of the Employee Stock Purchase Plan	—	480	—	—	—	480
Other comprehensive income	—	—	—	—	280	280
Balance as of June 30, 2024	\$ 4	\$ 479,361	\$ (10,983)	\$ (436,325)	\$ 421	\$ 32,478
Net loss	—	—	—	(33,225)	—	(33,225)
Stock-based compensation expense	—	6,137	—	—	—	6,137
Issuance of common stock in public offering	1	64,516	—	—	—	64,517
Issuance of common stock in exchange for consulting services	—	150	—	—	—	150
Issuance of common stock as part of the Employee Stock Purchase Plan	—	92	—	—	—	92
Issuance of common stock for options exercised	—	157	—	—	—	157
Other comprehensive loss	—	—	—	—	(536)	(536)
Balance as of September 30, 2024	\$ 5	\$ 550,413	\$ (10,983)	\$ (469,550)	\$ (115)	\$ 69,770

See accompanying notes to unaudited condensed consolidated financial statements.

ZEVRA THERAPEUTICS, INC.
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY,
CONTINUED
(in thousands)

	Common Stock	Additional Paid-in Capital	Treasury Stock, at cost	Accumulated Deficit	Other Comprehensive Income (Loss)	Total Stockholders' Equity
Balance as of January 1, 2023	\$ 3	\$ 436,269	\$ (7,536)	\$ (353,729)	\$ 113	\$ 75,120
Net loss	—	—	—	(13,217)	—	(13,217)
Stock-based compensation expense	—	945	—	—	—	945
Shares repurchased as part of the Share Repurchase Program	—	—	(3,447)	—	—	(3,447)
Issuance of common stock in exchange for consulting services	—	42	—	—	—	42
Other comprehensive loss	—	—	—	—	(176)	(176)
Balance as of March 31, 2023	\$ 3	\$ 437,256	\$ (10,983)	\$ (366,946)	\$ (63)	\$ 59,267
Net income	—	—	—	(2,573)	—	(2,573)
Stock-based compensation expense	—	2,151	—	—	—	2,151
Issuance of common stock in exchange for consulting services	—	25	—	—	—	25
Issuance of common stock as part of the Employee Stock Purchase Plan	—	165	—	—	—	165
Other comprehensive loss	—	—	—	—	(162)	(162)
Balance as of June 30, 2023	\$ 3	\$ 439,597	\$ (10,983)	\$ (369,519)	\$ (225)	\$ 58,873
Net loss	—	—	—	(10,367)	—	(10,367)
Stock-based compensation expense	—	1,440	—	—	—	1,440
Issuance of common stock in connection with the Merger (Note L)	—	11,500	—	—	—	11,500
Issuance of common stock in exchange for consulting services	—	71	—	—	—	71
Other comprehensive income	—	—	—	—	5	5
Balance as of September 30, 2023	\$ 3	\$ 452,608	\$ (10,983)	\$ (379,886)	\$ (220)	\$ 61,522

See accompanying notes to unaudited condensed consolidated financial statements.

ZEVRA THERAPEUTICS, INC.
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Nine months ended September 30,	
	2024	2023
Cash flows from operating activities:		
Net loss	\$ (69,772)	\$ (26,157)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	10,888	4,482
Inventory obsolescence charge	5,244	—
Non-cash interest expense	1,489	—
Depreciation and amortization expense	4,741	219
Fair value adjustment related to warrant and CVR liability	(4,660)	(4,159)
Fair value adjustment related to investments	(64)	(451)
Loss on sublease and disposal of property and equipment	—	157
Consulting fees paid in common stock	399	138
Loss (gain) on foreign currency exchange	147	(30)
Change in assets and liabilities:		
Accounts and other receivables	9,587	(1,628)
Prepaid expenses and other assets	(452)	27
Inventories	(4,159)	190
Operating lease right-of-use assets	389	243
Other long-term assets	—	(93)
Accounts payable and accrued expenses	(10,590)	5,400
Discount and rebate liability	4,824	3,895
Operating lease liabilities	(395)	(326)
Other liabilities	(1,031)	716
Net cash used in operating activities	(53,415)	(17,377)
Cash flows from investing activities:		
Purchases of property and equipment	—	(224)
Purchases of investments	(41,488)	(45,821)
Purchases of secured corporate notes	-	(25,426)
Maturities of investments	24,798	43,496
Net cash used in investing activities	(16,690)	(27,975)
Cash flows from financing activities:		
Proceeds from issuance of debt, net of lender fees	58,990	38,801
Payment of third-party debt issuance costs	(2,091)	—
Repayment of debt	(42,700)	(12,800)
Proceeds from insurance financing arrangements	1,082	1,256
Proceeds from Employee Stock Purchase Plan	643	219
Payments of principal on insurance financing arrangements	(431)	(564)
Payments to repurchase shares as part of the Share Repurchase Program	—	(3,447)
Proceeds from issuance of stock	65,819	—
Repayment of principal on finance lease liabilities	—	(5)
Net cash provided by financing activities	81,312	23,460
Effect of exchange rate changes on cash and cash equivalents	(217)	(305)
Net increase (decrease) in cash and cash equivalents	10,990	(22,197)
Cash and cash equivalents, beginning of period	43,049	65,466
Cash and cash equivalents, end of period	\$ 54,039	\$ 43,269
Supplemental cash flow information:		
Cash paid for interest	\$ 3,668	\$ 456

See accompanying notes to unaudited condensed consolidated financial statements.

ZEVRA THERAPEUTICS, INC.
NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

A. Description of Business, Basis of Presentation, and Significant Transactions

Organization

Zevra Therapeutics, Inc. (the "Company") is a commercial-stage company focused on addressing unmet needs for the treatment of rare diseases. The Company has a diverse portfolio of products and product candidates, which includes clinical stage pipeline and commercial stage assets. On September 20, 2024, the U.S. Food and Drug Administration ("FDA") approved the New Drug Application ("NDA") for MIPLYFFA™ (arimoclomol), an orally-delivered treatment for Niemann-Pick disease type C ("NPC"), which is an ultra-rare and progressive neurodegenerative disease. MIPLYFFA, the first FDA-approved treatment for NPC, is indicated for use in combination with miglustat for the treatment of neurological manifestations of NPC in adult and pediatric patients two years of age and older. In addition, the Company received a transferable rare pediatric disease priority review voucher in conjunction with the approval of MIPLYFFA. MIPLYFFA has also been granted orphan medicinal product designation for the treatment of NPC by the European Commission. OLPRUVA® (sodium phenylbutyrate) for oral suspension is approved by the FDA for the treatment of urea cycle disorders ("UCDs"). The Company also has a pipeline of investigational product candidates, including celiprolol for the treatment of vascular Ehlers-Danlos syndrome in patients with a confirmed type III collagen mutation. KP1077 is the Company's clinical development product candidate which is being developed as a treatment for idiopathic hypersomnia ("IH"), a rare neurological sleep disorder, and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate ("SDX"), the Company's proprietary prodrug of d-methylphenidate ("d-MPH"). The FDA has granted KP1077 orphan drug designation for the treatment of IH.

The Company changed its name from KemPharm, Inc. to Zevra Therapeutics, Inc. effective as of February 21, 2023. On March 1, 2023, following its name change, the Company's common stock began trading on the Nasdaq Global Select Market under the ticker symbol "ZVRA".

Going Concern

The accompanying unaudited condensed consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business. During the nine months ended September 30, 2024, and 2023, the Company incurred a net loss of \$69.8 million and \$26.2 million, respectively, and as of September 30, 2024, has an accumulated deficit of \$469.6 million. The Company has sustained operating losses for the majority of its corporate history and expects to continue to incur operating losses and negative operating cash flows until revenues reach a level sufficient to support ongoing operations.

The Company's ability to continue operating as a going concern is contingent upon its ability to generate revenue from approved products or obtain product candidate regulatory approvals, which would generate revenue, milestones, and cash flow sufficient to support ongoing operations and the satisfaction of financial covenants. These factors raise substantial doubt as to the Company's ability to continue as a going concern for at least one year from the date the unaudited condensed consolidated financial statements are being issued. The unaudited condensed consolidated financial statements do not include any adjustments that might result from the outcome of these uncertainties.

The Company's liquidity needs will be largely determined by the success of operations through the progression of its products and product candidates in the future. The Company also may consider other sources to fund operations including: (1) out-licensing rights to certain of its technologies and product candidates, pursuant to which the Company would receive cash royalties and milestones; (2) raising additional capital through equity or debt financings or from other sources; (3) obtaining product candidate regulatory approvals, which would generate revenue, milestones and cash flow; (4) reducing spending on one or more research and development programs, including by discontinuing development; and/or (5) restructuring operations to change its overhead structure. The Company is in the early stages of its commercialization effort for OLPRUVA and MIPLYFFA and does not yet have a substantial basis to project future earnings, and its other sources of revenue are not sufficient to sustain its present activities on their own. Accordingly, the Company's ability to continue as a going concern may require it to obtain additional financing to fund its operations.

Basis of Presentation

The Company prepared the unaudited condensed consolidated financial statements in accordance with U.S. GAAP and the rules and regulations of the Securities and Exchange Commission ("SEC") and, in the Company's opinion, reflect all adjustments, including normal recurring items that are necessary.

Merger

On August 30, 2023, the Company and Aspen Z Merger Sub, Inc., a wholly-owned subsidiary of Zevra ("Merger Sub"), entered into an Agreement and Plan of Merger (the "Merger Agreement") with Acer Therapeutics Inc. ("Acer"), a pharmaceutical company focused on development and commercialization of therapies for rare and life-threatening diseases. On November 17, 2023 (the "Closing Date"), the Company completed the acquisition of Acer. Pursuant to the Merger Agreement, on the Closing Date, Merger Sub was merged with and into Acer (the "Merger"), with Acer continuing as the surviving entity and as a wholly-owned subsidiary of Zevra. In connection therewith, Zevra also purchased Acer's secured debt from Nantahala Capital Management, LLC ("NCM"), certain of its affiliates and certain other parties (collectively with NCM, "Nantahala") through a series of transactions and Zevra agreed to provide Acer with a bridge loan facility for up to \$18.0 million ("Bridge Loan"), subject to certain terms and conditions. The Merger expanded Zevra's rare disease portfolio, as well as increased and diversified its revenues with the addition of a U.S. commercial asset, OLPRUVA, indicated for the treatment of UCDs. See Note L for further discussion related to the Merger.

Registration Statements on Form S-3

In connection with the Merger, Zevra and Nantahala concurrently entered into a registration rights agreement, pursuant to which Zevra agreed to file a resale registration statement with respect to the resale of the Zevra common stock issuable to Nantahala. On February 5, 2024, Zevra filed a registration statement on Form S-3 (File No. 333-276856) registering an aggregate of 2,269,721 shares of Zevra's common stock. On April 5, 2024, we filed an amendment to the registration statement on Form S-3 (File No. 333-250945) covering the issuance of the shares of our common stock issuable upon the exercise of warrants issued in connection with the Merger (Note L) and remaining unexercised as of the date of the amendment, which was declared effective on April 8, 2024.

On June 4, 2024, the Company filed a registration statement on Form S-3 (File No. 333-279941) (the "June 2024 Registration Statement") under which the Company may sell securities, including as may be issuable upon conversion, redemption, repurchase, exchange or exercise of securities, in one or more offerings up to a total aggregate offering price of \$350.0 million, \$75.0 million of which was allocated to the sale of the shares of common stock issuable under the 2024 ATM Agreement (as described further below). The registration statement was declared effective on June 13, 2024.

August 2024 Offering

On August 8, 2024, the Company entered into an underwriting agreement (the "Underwriting Agreement") with Cantor Fitzgerald & Co. and William Blair & Company, L.L.C., as representatives of the several underwriters named therein (collectively, the "Underwriters"), in connection with the offering, issuance and sale by the Company of 9,230,770 shares of the Company's common stock at a public offering price of \$6.50 per share, pursuant to the June 2024 Registration Statement and a related prospectus supplement dated August 8, 2024 filed with the SEC (the "August 2024 Offering"). Under the terms of the Underwriting Agreement, the Company also granted the Underwriters an option exercisable for 30 days to purchase up to an additional 1,384,615 shares of its Common Stock at the public offering price, less underwriting discounts and commissions, which the Underwriters exercised in full on August 9, 2024. The August 2024 Offering closed on August 12, 2024. Total shares issued were 10,615,385. Net proceeds from the offering were approximately \$64.5 million, after deducting underwriting discounts and commissions and estimated offering expenses payable by the Company. The Company intends to use the net proceeds of the offering to support the commercial launch activities for MIPLYFFA, continued commercial support for OLPRUVA and the continued development of celiprolol and KP1077 through potential NDA filings and other general corporate purposes.

Based on the planned use of proceeds, the Company believes that the net proceeds from the August 2024 Offering and its existing cash and cash equivalents will be sufficient to fund its operating expenses and capital expenditure requirements into 2027, subject to continuing compliance with the Company's debt covenants.

Entry into 2024 ATM Agreement

On July 12, 2024, the Company entered into an equity distribution agreement (the "2024 ATM Agreement") with Citizens JMP Securities LLC ("Citizens JMP") under which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock having an aggregate offering price of up to \$75.0 million through Citizens JMP as its sales agent. The issuance and sale, if any, of common stock by the Company under the 2024 ATM Agreement will be made pursuant to the June 2024 Registration Statement, the accompanying prospectus, and the related prospectus supplement dated July 12, 2024. Citizens JMP may sell the common stock by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415 of the Securities Act. Citizens JMP will use commercially reasonable efforts to sell the common stock from time to time, based upon instructions from the Company (including any price, time or size limits or other customary parameters or conditions the Company may impose). The Company will pay Citizens JMP a commission equal to 3.0% in the aggregate of the gross sales proceeds of any common stock sold through Citizens JMP under the 2024 ATM Agreement. As of September 30, 2024, no shares have been issued or sold under the 2024 ATM Agreement.

Termination of 2021 ATM Agreement

On July 2, 2021, the Company entered into an equity distribution agreement (the "2021 ATM Agreement") with JMP Securities LLC ("JMP") and RBC Capital Markets, LLC ("RBCCM") under which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock having an aggregate offering price of up to \$75.0 million through JMP and RBCCM as its sales agents. The issuance and sale, if any, of common stock by the Company under the 2021 ATM Agreement will be made pursuant to the registration statement on Form S-3 (File No. 333-257661) which was declared effective on July 12, 2021, the accompanying prospectus, and the related prospectus supplement dated July 12, 2021. JMP and RBCCM may sell the common stock by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415 of the Securities Act. JMP and RBCCM will use commercially reasonable efforts to sell the common stock from time to time, based upon instructions from the Company (including any price, time

or size limits or other customary parameters or conditions the Company may impose). The Company will pay JMP and RBCCM a commission equal to 3.0% in the aggregate of the gross sales proceeds of any common stock sold through JMP and RBCCM under the 2021 ATM Agreement. The 2021 ATM Agreement terminated on July 11, 2024, and no shares had been issued or sold under the 2021 ATM Agreement as of the termination date.

Share Repurchase Program

On December 20, 2021, the Company initiated a share repurchase program (the "Share Repurchase Program") pursuant to which the Company was able to repurchase up to \$50 million of shares of its common stock through December 31, 2023. On December 31, 2023, the Share Repurchase Program ended, and the Company had repurchased 1,575,692 shares of its common stock for approximately \$11.0 million.

B. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires the Company to make estimates and assumptions that affect the amounts reported in the unaudited condensed consolidated financial statements and accompanying notes. Actual results could differ from those estimates.

On an ongoing basis, the Company evaluates its estimates and assumptions, including those related to revenue recognition, the useful lives of property and equipment, the recoverability of long-lived assets, the incremental borrowing rate for leases, and assumptions used for purposes of determining stock-based compensation, income taxes, the fair value of investments and the fair value of the warrant liabilities, contingent value right ("CVR") liabilities and discount and rebate liabilities, among others. The Company bases its estimates on historical experience and on various other assumptions that it believes to be reasonable, the results of which form the basis for making judgments about the carrying value of assets and liabilities.

Investments

The Company maintains investment securities that are classified as available-for-sale securities for which the Company has elected the fair value option under Accounting Standards Codification ("ASC") 825, *Financial Instruments* ("ASC 825"). As such, these securities are carried at fair value with unrealized gains and losses included in fair value adjustment related to investments on the unaudited condensed consolidated statements of operations. The securities primarily consist of U.S. Treasury securities and corporate bonds and are included in securities at fair value in the unaudited condensed consolidated balance sheets. The Company's investments in corporate bonds mature within one to five years. As of September 30, 2024, and December 31, 2023, the Company held securities with an aggregate fair value of \$41.4 million and \$24.7 million, respectively, that contained aggregate unrealized gains of approximately \$0.1 million and \$0.6 million, respectively. Applying fair value accounting to these debt securities more accurately represents the Company's investment strategy due to the fact that excess cash is currently being invested for the purpose of funding future operations. Interest income is recognized as earned using an effective yield method giving effect to the amortization of premium and accretion of discount and is based on the economic life of the securities. Interest income is included in interest and other income, net in the unaudited condensed consolidated statements of operations.

Variable Interest Entities

The primary beneficiary of a variable interest entity ("VIE") is required to consolidate the assets and liabilities of the VIE. When the Company obtains a variable interest in another entity, it assesses at the inception of the relationship and upon occurrence of certain significant events whether the entity is a VIE, and if so, whether the Company is the primary beneficiary of the VIE based on its power to direct the activities of the VIE that most significantly impact the VIE's economic performance and the Company's obligation to absorb losses or the rights to receive benefits from the VIE that could potentially be significant to the VIE.

To assess whether the Company has the power to direct the activities of the VIE that most significantly impact the VIE's economic performance, the Company considers all the facts and circumstances, including the Company's role in establishing the VIE and the Company's ongoing rights and responsibilities. The assessment includes identifying the activities that most significantly impact the VIE's economic performance and identifying which party, if any, has the power to direct those activities. In general, the parties that make the most significant decisions affecting the VIE (management and representation on the Board of Directors) are deemed to have the power to direct the activities of a VIE.

To assess whether the Company has the obligation to absorb losses of the VIE or the rights to receive benefits from the VIE that could potentially be significant to the VIE, the Company considers all of its economic interests that are deemed to be variable interests in the VIE.

This assessment requires judgement in determining whether these interests, in the aggregate, are considered potentially significant to the VIE. As of September 30, 2024, and December 31, 2023, the Company identified Acer to be the Company's sole interest in a VIE (Note L). As Zevra is the final decision maker for all of Acer's research, development, and commercialization of drug candidates that it is producing, the Company directs the activities of Acer that most significantly impact its performance. Therefore, the Company is the primary beneficiary of this VIE for accounting purposes.

Revenue Recognition

The Company recognizes revenue in accordance with the provisions of ASC 606, *Revenue from Contracts with Customers* ("ASC 606") and, as a result, follows the five-step model when recognizing revenue: 1) identifying a contract; 2) identifying the performance obligations; 3) determining the transaction price; 4) allocating the price to performance obligations; and 5) recognizing revenue when the performance obligations have been fulfilled.

Licensing Agreements

The terms of the Company's licensing agreements typically include one or more of the following: (i) upfront fees; (ii) milestone payments related to the achievement of development, regulatory, or commercial goals; and (iii) royalties on net sales of licensed products. Each of these payments may result in licensing revenues.

As part of the accounting for these agreements, the Company must develop estimates and assumptions that require judgment to determine the underlying stand-alone selling price for each performance obligation which determines how the transaction price is allocated among the performance obligations. Generally, the estimation of the stand-alone selling price may include such estimates as independent evidence of market price, forecasted revenues or costs, development timelines, discount rates, and probability of regulatory success. The

Company evaluates each performance obligation to determine if they can be satisfied at a point in time or over time, and it measures the services delivered to the licensee which are periodically reviewed based on the progress of the related program. The effect of any change made to an estimated input component and, therefore revenue or expense recognized, would be recorded as a change in estimate. In addition, variable consideration (e.g., milestone payments) must be evaluated to determine if it is constrained and, therefore, excluded from the transaction price.

Up-front Fees: If a license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenues from the transaction price allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time.

Milestone Payments: At the inception of each arrangement that includes milestone payments (variable consideration), the Company evaluates whether the milestones are considered probable of being reached and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's or the licensee's control, such as non-operational developmental and regulatory approvals, are generally not considered probable of being achieved until those approvals are received. At the end of each reporting period, the Company re-evaluates the probability of achievement of milestones that are within its or the licensee's control, such as operational developmental milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenues and earnings in the period of adjustment. Revisions to the Company's estimate of the transaction price may also result in negative licensing revenues and earnings in the period of adjustment.

Product Revenues, Net

Net revenues from product sales is recognized at the transaction price when the customer obtains control of the Company's products, which occurs at a point in time, typically upon receipt of the product by the customer. Our current single customer is a specialty pharmacy provider.

The Company's net revenues represent total revenues adjusted for discounts and allowances, including estimated cash discounts, chargebacks, rebates, returns, copay assistance, data fees and wholesaler fees for services. These adjustments represent variable consideration under ASC 606 and are recorded as a reduction of revenue. These adjustments are established by management as its best estimate based on available information and will be adjusted to reflect known changes in the factors that impact such allowances. Adjustments for variable consideration are determined based on the contractual terms with customers, historical trends, communications with customers and the levels of inventory remaining in the distribution channel, as well as expectations about the market for the product and anticipated introduction of competitive products.

Acquired IPR&D and Milestones Expenses

In an asset acquisition, payments incurred prior to regulatory approval to acquire rights to in-process R&D projects are expensed as acquired IPR&D and milestones expense in the consolidated statements of earnings unless the project has an alternative future use. These costs include upfront and development milestone payments related to R&D collaborations, licensing arrangements, or other asset acquisitions that provide rights to develop, manufacture and/or sell pharmaceutical products. Where contingent development milestone payments are due to third parties, prior to regulatory approval, the payment obligations are expensed when the milestone results are achieved. Regulatory and commercial milestone payments made to third parties subsequent to regulatory approval are capitalized as intangible assets and amortized to cost of products sold over the remaining useful life of the related product.

Inventories

The value of inventory is recorded at its net realizable value. The Company determines the cost of its other inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis.

The Company may scale-up and make commercial quantities of its product candidates prior to the date it anticipates that such product will receive final regulatory approval. The scale-up and commercial production of pre-launch inventory involves the risk that such products may not be approved for marketing on a timely basis, or ever. This risk notwithstanding, the Company may scale-up and build pre-launch inventory of product that have not received final regulatory approval when the Company believes such action is appropriate in relation to the commercial value of the product launch opportunity. Inventory manufactured prior to regulatory approval is recorded as research and development expense until regulatory approval for the product is obtained. Inventory used in clinical trials is also expensed as research and development expense, when selected for such use. Inventory that can be used in either the production of clinical or commercial products is expensed as research and development costs when identified for use in a clinical manufacturing campaign. The cost of finished goods inventory that is shipped to a customer to support the Company's patient assistance programs is expensed when those shipments take place. As of September 30, 2024, and December 31, 2023, the Company did not have pre-launch inventory that qualified for capitalization.

The Company performs an assessment of the recoverability of capitalized inventory during each reporting period and writes down any excess and obsolete inventory to its net realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded as a component of cost of product revenue in the statements of operations and comprehensive loss. The determination of whether inventory costs will be realizable requires the use of estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required. Additionally, the Company's product is subject to strict quality control and monitoring that it performs throughout the manufacturing process. In the event that certain batches or units of product do not meet quality specifications, the Company will record a charge to cost of product revenue, to write-down any unsaleable inventory to its estimated net realizable value. For the three and nine months ended September 30, 2024, the Company recognized a charge of approximately \$2.0 million and \$5.2 million, respectively, related to write-downs for unsaleable inventory. No such write downs were recognized for the three and nine months ended September 30, 2023.

Foreign currency

Assets and liabilities are translated into the reporting currency using the exchange rates in effect on the unaudited condensed consolidated balance sheet dates. Equity accounts are translated at historical rates, except for the change in accumulated deficit during the year,

which is the result of the income statement translation process. Revenue and expense accounts are translated using the weighted average exchange rate during the period. The cumulative translation adjustments associated with the net assets of foreign subsidiaries are recorded in accumulated other comprehensive income/loss in the accompanying unaudited condensed consolidated statements of stockholders' equity.

Debt Issuance Costs

Debt issuance costs incurred in connection with financing arrangements are recorded as a reduction of the related debt on the balance sheet and amortized over the life of the respective financing arrangement using the effective interest method.

Warrants

The Company accounts for warrants as either equity-classified or liability-classified instruments based on an assessment of the warrant's specific terms and applicable authoritative guidance in FASB ASC Topic 480, *Distinguishing Liabilities from Equity* ("ASC 480") and FASB ASC Topic 815, *Derivatives and Hedging* ("ASC 815"). The assessment considers whether the warrants are freestanding financial instruments pursuant to ASC 480, meet the definition of a liability pursuant to ASC 480, and whether the warrants meet all of the requirements for equity classification under ASC 815, including whether the warrants are indexed to our own stock and whether the warrant holders could potentially require "net cash settlement" in a circumstance outside of the company's control, among other conditions for equity classification. This assessment, which requires the use of professional judgment, is conducted at the time of warrant issuance and as of each subsequent quarterly period end date while the warrants are outstanding.

For warrants that meet all criteria for equity classification, the warrants are required to be recorded as a component of additional paid-in capital, on the unaudited condensed consolidated statement of stockholders' deficit at the time of issuance. For warrants that do not meet all the criteria for equity classification, the warrants are required to be recorded at their initial fair value on the date of issuance, and on each balance sheet date thereafter. Changes in the estimated fair value of the warrants are recognized as a non-cash gain or loss in other expense, net, on the unaudited condensed consolidated statement of operations. The fair value of the warrants was estimated using the Black-Scholes option pricing model.

New Accounting Pronouncements Not Yet Adopted

Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures

In November 2023, the FASB issued Accounting Standards Update ("ASU") No. 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*, which modifies the disclosure and presentation requirements of reportable segments. The amendments in the update require the disclosure of significant segment expenses that are regularly provided to the chief operating decision maker ("CODM") and included within each reported measure of segment profit and loss. The amendments also require disclosure of all other segment items by reportable segment and a description of its composition. Additionally, the amendments require disclosure of the title and position of the CODM and an explanation of how the CODM uses the reported measure(s) of segment profit or loss in assessing segment performance and deciding how to allocate resources. This update is effective for annual periods beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024. Early adoption is permitted. The Company is currently evaluating the impact that this guidance will have on the presentation of its consolidated financial statements and accompanying notes.

Income Taxes (Topic 740): Improvements to Income Tax Disclosures

In December 2023, the FASB issued ASU No. 2023- 09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*, which expands disclosures in an entity's income tax rate reconciliation table and disclosures regarding cash taxes paid both in the U.S. and foreign jurisdictions. The update will be effective for annual periods beginning after December 15, 2024. The Company is currently evaluating the impact that this guidance will have on the presentation of its consolidated financial statements and accompanying notes.

C. Inventories

	September 30, 2024	December 31, 2023
Raw materials	\$ 4,549	\$ 2,938
Work in progress	3,084	1,884
Finished goods	1,123	5,019
Total inventory	<u>\$ 8,756</u>	<u>\$ 9,841</u>

D. Debt Obligations

Long-term debt consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Notes payable	\$ 61,035	\$ —
Secured promissory note	—	5,000
Unamortized original issue (discount) premium	(959)	148
Less: debt issuance costs	(1,172)	(82)
	<u>\$ 58,904</u>	<u>\$ 5,066</u>

Secured Promissory Note

In connection with the Merger (Note L), on August 30, 2023, the Company and Nantahala, entered into a secured promissory note payable by Zevra to Nantahala in the original principal amount of \$5.0 million (the "Nantahala Note"). The Nantahala Note initially bore interest at 9.0% per annum, payable quarterly in arrears in cash. The interest rate increased to 12.0% per annum effective March 1, 2024, as the Nantahala Note remained unpaid six months from its issue date. The additional 3.0% interest would have been paid in shares of Zevra's common stock based on the volume weighted average trading price ("VWAP") of Zevra's common stock during the twenty consecutive trading days ending on the date before such interest payment date. Beginning on the first interest payment date following the second anniversary of the Nantahala Note, and on each interest payment date thereafter, Zevra was required to make \$0.6 million amortization payments on the Nantahala Note until it was paid in full. All principal and unpaid interest on the Nantahala Note

would have been due on August 30, 2026, the third anniversary of the Nantahala Note. Zevra was entitled to prepay the Nantahala Note at any time without penalty. The Nantahala Note was secured by Zevra's interest in (i) the loan assets under the Loan Purchase Agreement described in Note L; (ii) the note assets under the Note Purchase Agreement described in Note L; (iii) the Bridge Loan described in Note L; and (iv) the proceeds therefrom. The Company used the proceeds from the Nantahala Note, along with \$12.0 million in cash and 98,683 shares of Zevra's common stock, to acquire Acer's term loans, as more fully described in Note L. In April 2024, the Nantahala Note was repaid in full and terminated. At the time of repayment, Nantahala elected to receive a cash payment in lieu of shares of Zevra's common stock in exchange for the additional 3.0% interest accrued for the period from March 1, 2024, through April 5, 2024.

Line of Credit

On January 26, 2023, the Company and Wells Fargo, as lender, entered into a revolving margin account agreement. The Company's investments were used as collateral for the loan and the amount the Company was able to borrow was limited to 80-90% of its outstanding investment balance held with Wells Fargo. The margin account bore interest at the Prime rate minus 225 basis-points. As of December 31, 2023, \$37.7 million was outstanding under the margin account. In April 2024, the Company repaid the outstanding balance under the margin account with Wells Fargo, and upon such repayment, the margin capabilities were removed from the account.

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Term Loans

On April 5, 2024 (the "Term Loans Closing Date"), the Company entered into a credit agreement (the "Credit Agreement") with HCR Stafford Fund II, L.P., HCR Potomac Fund II, L.P., and Perceptive Credit Holdings IV, LP (collectively, the "Lenders"), and Alter Domus (US) LLC, as administrative agent (the "Administrative Agent").

Under the terms of the Credit Agreement, the Lenders provided a senior secured loan facility to the Company in the aggregate principal amount of \$100.0 million, which is divided into three tranches as follows: (i) \$60.0 million which was funded in full on the Term Loans Closing Date; (ii) \$20.0 million which is available to the Company in up to two drawings, each in an amount not to exceed \$10.0 million, at the Company's option until 18 months following the Term Loans Closing Date; and (iii) \$20.0 million which is available to the Company upon approval by the FDA of the NDA for MIPLYFFA for the treatment of NPC, at the Company's option until December 31, 2024 (collectively, the "Term Loans").

The principal amount of the Term Loans outstanding (the "Outstanding Principal Amount") will bear interest at a rate equal to 3-Month Term SOFR *plus* 7.00% per annum. If the net product sales for the calendar year ending December 31, 2025, exceed \$100.0 million, the Outstanding Principal Amount will bear interest at 3-Month Term SOFR *plus* 6.00% per annum. If the net product sales for the calendar year ending December 31, 2025, do not exceed \$100.0 million, then for any subsequent period of four consecutive fiscal quarters ending on or after March 31, 2026, in which net product sales exceed \$125.0 million, the Outstanding Principal Amount will bear interest at 3-Month Term SOFR *plus* 6.50% per annum. In all cases, the 3-Month Term SOFR rate will be subject to a floor of 4.00% per annum. Interest will be payable quarterly in arrears on the last day of each calendar quarter. The Company has the option to pay up to 25% of the interest in-kind beginning on the Term Loans Closing Date, through and including June 30, 2026. The Company has recognized approximately \$0.9 million of interest-in-kind as of September 30, 2024, which is included in long-term debt in the unaudited condensed consolidated balance sheet. The Term Loans will mature on the fifth anniversary of the Term Loans Closing Date. In connection with the Credit Agreement, the Company incurred approximately \$2.2 million of costs, which primarily consisted of underwriting, legal and other professional fees, and are included as a reduction to the carrying amount of the related debt liability and are deferred and amortized over the remaining life of the financing using the effective interest method.

The Credit Agreement contains customary affirmative and negative covenants by the Company, which among other things, will require the Company to provide certain financial reports to the Lenders within 60 days after the end of each of the first three fiscal quarters of each fiscal year and 105 days after the end of each fiscal year, meet certain minimum net product sales amounts, and limit the ability of the Company to incur or guarantee additional indebtedness, engage in certain transactions, and effect a consolidation or merger without consent. In addition, as long as the line of credit remains active, the Company must maintain a minimum cash balance of \$20.0 million to ensure the Company can meet its immediate capital needs. The obligations of the Company under the Credit Agreement may be accelerated upon customary events of default, including non-payment of principal, interest, fees and other amounts, covenant defaults, insolvency, material judgments, or inaccuracy of representations and warranties. The Term Loans are secured by a first priority perfected lien on, and security interest in, substantially all current and future assets of the Company. The proceeds of the Term Loans were used to refinance certain existing indebtedness of the Company and its subsidiaries. The Company will use the remaining proceeds to pay fees and expenses related to the debt financing and fund the development and commercialization of OLPRUVA and MIPLYFFA.

Future minimum principal payments under the Term Loans as of September 30, 2024, were as follows (in thousands):

Year Ending December 31,	
2024 (excluding the nine months ending September 30, 2024)	\$ —
2025	—
2026	—
2027	—
2028	—
Thereafter	60,919
Total minimum payments	60,919
Less: unamortized debt discount, debt issuance costs and paid in kind interest	(2,015)
Note payable	\$ 58,904

E. Revenue, net

AZSTARYS License Agreement

The Company entered into a Collaboration and License Agreement (the "AZSTARYS License Agreement") with Commave Therapeutics SA (formerly known as Boston Pharmaceutical S.A.) ("Commave"), an affiliate of Gurnet Point Capital, L.P., dated September 3, 2019. Under the AZSTARYS License Agreement, as amended, the Company granted to Commave an exclusive, worldwide license to develop, manufacture and commercialize the Company's product candidates containing SDX and d-MPH, including AZSTARYS, or any other product candidates containing SDX and developed to treat ADHD or any other central nervous system disorder. Corium Inc. was tasked by Commave to lead all commercialization activities for AZSTARYS under the AZSTARYS License Agreement. Pursuant to the AZSTARYS License Agreement, Commave agreed to pay milestone payments up to an aggregate of \$590.0 million upon the occurrence of specified regulatory milestones related to AZSTARYS, additional fixed payments upon the achievement of specified U.S. sales milestones, and quarterly, tiered royalty payments based on a range of percentages of net sales (as defined in the AZSTARYS License Agreement). Commave is obligated to make such royalty payments on a product-by-product basis until expiration of the royalty term for the applicable product.

The Company concluded that these regulatory milestones, sales milestones and royalty payments each contain a significant uncertainty associated with a future event. As such, these milestone and royalty payments are constrained at contract inception and are not included in the transaction price as the Company could not conclude that it is probable a significant reversal in the amount of cumulative revenue recognized will not occur surrounding these milestone payments. At the end of each reporting period, the Company updates its assessment of whether the milestone and royalty payments are constrained by considering both the likelihood and magnitude of the potential revenue reversal. For the three and nine months ended September 30, 2024, the Company recognized \$1.1 million and \$3.6 million of revenue under the AZSTARYS License Agreement, respectively, primarily related to royalties. For the three and nine months ended September 30, 2023, the Company recognized revenue under the AZSTARYS License Agreement of \$0.9 million and \$7.2 million, respectively, which includes recognition of a \$5.0 million net sales milestone that was met in June 2023. There was no deferred revenue related to this agreement as of September 30, 2024, or December 31, 2023.

In accordance with the terms of the Company's Termination Agreement with Aquestive Therapeutics ("Aquestive") dated March 20, 2012, Aquestive has the right to receive an amount equal to 10% of any royalty or milestone payments made to the Company related to AZSTARYS or KP1077 under the AZSTARYS License Agreement.

The AZSTARYS License Agreement is within the scope of ASC 606, as the transaction represents a contract with a customer where the participants function in a customer / vendor relationship and are not exposed equally to the risks and rewards of the activities contemplated under the AZSTARYS License Agreement.

Relief Exclusive License Agreement

As a condition to entering into the Merger Agreement, Acer and Relief Therapeutics SA ("Relief") entered into an exclusive license agreement on August 30, 2023 (the "Relief License Agreement"). Pursuant to the Relief License Agreement, Relief will hold exclusive development and commercialization rights for OLPRUVA in the European Union, Liechtenstein, San Marino, Vatican City, Norway, Iceland, Principality of Monaco, Andorra, Gibraltar, Switzerland, United Kingdom, Albania, Bosnia, Kosovo, Montenegro, Serbia and North Macedonia ("Geographical Europe"). The Company will have the right to receive a royalty of up to 10% of the net sales of OLPRUVA in Geographical Europe. For the three and nine months ended September 30, 2024, the Company did not recognize any revenue under the Relief License Agreement. There was no deferred revenue related to this agreement as of September 30, 2024, and December 31, 2023. For further discussion of the Relief License Agreement, see Note L.

Product Revenues, Net

Expanded Access Program

Net revenue includes revenue from the sale of arimoclomol for the treatment of NPC under the remunerated expanded access compassionate use program in France ("French nATU"). An expanded access compassionate use program is a program giving specific patients access to a drug, which is not yet approved for commercial sale. Only drugs targeting serious or rare indications and for which there is currently no appropriate treatment are considered for expanded access compassionate use programs. Further, to be considered for the expanded access compassionate use program, the drug must have proven efficacy and safety and must either be undergoing price negotiations or seeking marketing approval.

In accordance with ASC 606, the Company recognizes revenue when fulfilling its performance obligation under the Expanded Access Program ("EAP") by transferring control of promised goods or services to its customer, in an amount that reflects the consideration that the Company expects to receive in exchange for those goods or services. In determining when the customer obtains control of the product, the Company considers certain indicators, including whether the Company has a present right to payment from the customer, whether title and/or significant risks and rewards of ownership have transferred to the customer and whether the customer acceptance has been received. Revenue is recognized net of sales deductions, including discounts, rebates, applicable distributor fees, and revenue-based taxes.

The French Health Authorities and the manufacturer have agreed to a price for sales during the French nATU, but the final transaction price depends on the terms and conditions in the contracts with the French Health Authorities and is subject to price negotiations with the French Health Authorities following market approval. Any excess in the price charged the manufacturer compared to the price agreed with the health authorities once the drug product is approved in France must be repaid. The repayment is considered in the clawback liability (rebate). An estimate of net revenue and clawback liability are recognized using the 'expected value' method. Accounting for net revenue and clawback liability requires determination of the most appropriate method for the expected final transaction price. This estimate also requires assumptions with respect to inputs into the method, including current pricing of comparable marketed products within the rare disease area in France. Management has considered the expected final sales price as well as the price of similar drug products. The Company is operating within a rare disease therapeutic area where there is unmet treatment need and hence a limited number of comparable commercialized drugs products. The limited available relevant market information for directly comparable commercialized drugs within rare disease increases the uncertainty in management's estimate.

For the three and nine months ended September 30, 2024, the Company recognized revenue related to the EAP in France of \$2.6 million and \$8.0 million, respectively, which is net of a clawback liability of \$1.4 million and \$4.4 million, respectively, and other gross to net adjustments. For the three and nine months ended September 30, 2023, the Company recognized revenue related to the EAP in France of \$2.0 million and \$7.1 million, respectively, which is net of a clawback liability of \$1.1 million and \$4.0 million, respectively, and other gross to net adjustments. The total estimated reserve liability as of September 30, 2024, and December 31, 2023, was \$17.0 million and \$12.2 million, respectively. As of September 30, 2024, and December 31, 2023, this estimated reserve liability is recorded as discount and rebate liabilities in the unaudited condensed consolidated balance sheets and is separated into current and long-term based upon the timing of the expected payment to the French regulators.

Product Sales

On December 27, 2022, the FDA approved OLPRUVA (sodium phenylbutyrate), a prescription medicine used along with certain therapy, including changes in diet, for the long-term management of adults and children with UCDs weighing 44 pounds (20 kg) or greater and

with a body surface area of 1.2m² or greater. On November 17, 2023, the Company acquired OLPRUVA in connection with the Merger (Note L). For the three and nine months ended September 30, 2024, sales of OLPRUVA were de minimis.

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On September 20, 2024, the NDA for MIPLYFFA, an orally-delivered treatment for NPC, which is an ultra-rare and progressive neurodegenerative disease, was approved. For the three and nine months ended September 30, 2024, sales of MIPLYFFA were de minimis. In May 2011, Orphazyme entered into an asset purchase agreement with LadRx Corporation, which was assigned to XOMA (US) LLC, a wholly-owned subsidiary of XOMA Corporation ("XOMA"), in June 2023 ("XOMA License Agreement"). Under the XOMA License Agreement, XOMA is entitled to certain net sales and regulatory milestone payments in addition to a mid-single digit royalty with respect to sales of MIPLYFFA. For the three and nine months ended September 30, 2024, the Company recognized a regulatory milestone payment of \$6.0 million due to XOMA under the XOMA License Agreement, which is included in intangible assets, net in the unaudited condensed consolidated balance sheet.

The Company currently utilizes a single specialty pharmacy provider as its sole distributor for both OLPRUVA and MIPLYFFA. The Company also enters into arrangements with health care providers and payors that provide for government mandated and/or privately negotiated rebates with respect to the purchase of its products. To commercialize OLPRUVA for oral suspension and MIPLYFFA in the U.S., the Company has built marketing, sales, medical affairs, distribution, managerial and other non-technical capabilities or is making arrangements with third parties to perform these services.

Accounts and Other Receivables

Accounts and other receivables consist of receivables from product sales, receivables under the AZSTARYS License Agreement and EAP, as well as income tax receivables and other receivables due to the Company. Receivables under the AZSTARYS License Agreement are recorded for amounts due to the Company related to reimbursable third-party costs and royalties on product sales. Receivables under the EAP are recorded for product sales under the French nATU. These receivables are evaluated to determine if any reserve or allowance should be established at each reporting date. As of September 30, 2024, the Company had receivables related to the EAP of \$4.4 million, AZSTARYS License Agreement of \$1.1 million, and other receivables of \$2.3 million. As of December 31, 2023, the Company had receivables related to the EAP of \$4.7 million, AZSTARYS License Agreement of \$11.4 million, and other receivables of \$1.3 million. As of September 30, 2024, and December 31, 2023, no reserve or allowance for doubtful accounts had been established.

F. Commitments and Contingencies

From time to time, the Company is involved in various legal proceedings arising in the normal course of business. For some matters, a liability is not probable, or the amount cannot be reasonably estimated and, therefore, an accrual has not been made. However, for such matters when it is probable that the Company has incurred a liability and can reasonably estimate the amount, the Company accrues and discloses such estimates.

Stockholder Litigation Related to the Merger

On October 12, 2023, Brodsky & Smith, purporting to act as counsel for Jerry Beavee, who was asserted to be a stockholder of Acer, filed a complaint entitled *Jerry Beavee v. Acer Therapeutics Inc., et al.*, No. 1:23-cv-08995 in the United States District Court for the Southern District of New York (the "Action") alleging that defendants violated Section 14(a) and 20(a) of the Securities Exchange Act of 1934 by filing the Preliminary Merger Registration Statement which allegedly omitted certain information that such counsel asserts is material to Acer's required disclosure. On October 30, 2023, Acer filed with the SEC a Schedule DEF14A that contained additional information regarding the Merger, which mooted the disclosure claims alleged in the Action. On December 8, 2023, Jerry Beavee filed with the court a notice of dismissal of the Action without prejudice.

On October 20, 2023, Long Law, LLC and Acocelli Law, PLLC, purporting to act as counsel for Kevin Turner, who was asserted to be a stockholder of Acer, filed a complaint entitled *Kevin Turner v. Acer Therapeutics Inc., et al.*, No. 1:23-cv-01185 in the United States District Court for the District of Delaware alleging that defendants violated Section 14(a) and 20(a) of the Securities Exchange Act of 1934 as well as SEC Rule 14a-9 by filing the Definitive Proxy Statement which allegedly omitted certain information that such counsel asserts is material to Acer's required disclosure. The complaint prays that, if asserted omissions are not adequately corrected, then Turner will seek to enjoin Acer from holding a stockholder meeting to approve the Merger and, if the Merger closes, will seek to rescind it and seek an award of damages.

On October 20, 2023, Long Law, LLC, purporting to act as counsel for Matthew Jones, who was asserted to be a stockholder of Acer, filed a complaint entitled *Matthew Jones v. Acer Therapeutics Inc., et al.*, No. 1:23-cv-01186 in the United States District Court for the District of Delaware alleging that defendants violated Section 14(a) and 20(a) of the Securities Exchange Act of 1934 as well as SEC

Rule 14a-9 by filing the Definitive Proxy Statement which allegedly omitted certain information that such counsel asserts is material to the Acer's required disclosure. The complaint prays that, if asserted omissions are not adequately corrected, then Jones will seek to enjoin Acer from holding a stockholder meeting to approve the Merger and, if the Merger closes, will seek to rescind it and seek an award of damages.

On June 19, 2024, the Company entered into a confidential fee agreement (the "Fee Agreement") to settle the above stockholder litigation matters related to the Merger. The parties to the Fee Agreement were entitled to an aggregate payment of approximately \$0.3 million, which was paid during the second quarter of 2024.

Litigation Related to the AZSTARYS License Agreement

In September 2024, the Company became engaged in a legal dispute regarding the AZSTARYS License Agreement. The litigation is in its early stages. We cannot predict with certainty the timing or ultimate outcome of this litigation or its potential impact on our business, financial condition, or results of operations. At this time, we have not recorded any accrual for contingent liability associated with this matter. The AZSTARYS License Agreement remains in effect during this litigation, and both parties continue to perform their respective obligations thereunder. However, there can be no assurance that this dispute will not have an adverse impact on our relationship with Commave or on the Company's business. The Company will continue to monitor developments in this matter and will assess the potential impact on our financial statements in future periods. The Company expects to incur significant legal expenses in connection with this litigation, which may materially affect its results of operations in future periods.

As of September 30, 2024, and December 31, 2023, no accruals were made related to commitments and contingencies.

G. Stock and Warrants

Authorized, Issued, and Outstanding Common Shares

As of September 30, 2024, and December 31, 2023, the Company had authorized shares of common stock of 250,000,000 shares. Of the authorized shares, 54,803,056 and 43,110,360 shares of common stock were issued as of September 30, 2024, and December 31, 2023, respectively, and 53,227,364 and 41,534,668 shares of common stock were outstanding as of September 30, 2024, and December 31, 2023, respectively.

As of September 30, 2024, and December 31, 2023, the Company had reserved authorized shares of common stock for future issuance as follows:

	September 30, 2024	December 31, 2023
Outstanding awards under equity incentive plans	8,987,997	8,023,142
Outstanding common stock warrants	5,483,537	5,603,729
Possible future issuances under equity incentive plans	4,536,410	1,728,885
Possible future issuances under employee stock purchase plans	1,242,425	1,340,172
Total common shares reserved for future issuance	<u>20,250,369</u>	<u>16,695,928</u>

Common Stock Activity

The following table summarizes common stock activity for the nine months ended September 30, 2024:

	Shares of Common Stock
Balance as of January 1, 2024	41,534,668
Common stock issued as compensation to third parties	9,000
Common stock issued as a result of stock options exercised	306,826
Balance as of March 31, 2024	41,850,494
Common stock issued as a result of the Employee Stock Purchase Plan	97,747
Common stock issued as compensation to third parties	41,820
Common stock issued as a result of stock options exercised	1,403
Balance as of June 30, 2024	41,991,464
Common stock issued as compensation to third parties	32,820
Common stock issued in connection with the Public Offering (Note B)	10,615,385
Common stock issued as a result of stock options exercised	39,750
Common stock issued in connection with vesting of performance-based awards	547,945
Balance as of September 30, 2024	<u>53,227,364</u>

Authorized, Issued, and Outstanding Preferred Stock

As of September 30, 2024, and December 31, 2023, the Company had 10,000,000 shares of authorized preferred stock, none of which were designated, issued, or outstanding.

Warrants to Purchase Common Stock

The Company has issued warrants to purchase common stock to various third parties, of which 5,483,537 remain outstanding as of September 30, 2024, and are immediately exercisable. These warrants qualify as participating securities under ASC Topic 260, *Earnings per Share*, and are treated as such in the net loss per share calculation (Note J). The Company may be required to redeem these warrants for a cash amount equal to the Black-Scholes value of the portion of the warrants to be redeemed (the "Put Option").

In connection with the Merger (Note L), in November 2023, the Company directly issued to certain investors an aggregate of 1,382,489 shares of its common stock, par value \$0.0001 per share, and accompanying warrants to purchase up to 1,382,489 shares of its common stock (the "2023 Warrants") at a combined offering price of \$4.34 per share of common stock and the Warrants and an aggregate of 917,934 shares of its common stock in exchange for the cancellation of a warrant to purchase 2,920,306 shares of common stock of Acer. The Warrants are immediately exercisable and expire on November 22, 2028. The Company used the net proceeds of approximately \$6.0 million from the offering for general corporate purposes. These warrants are separately exercisable by the warrant holders. While the warrants are outstanding (but unexercised), the warrant holders will participate in any dividend or other distribution of the Company's assets to its common stockholders by way of return of capital or otherwise. As of September 30, 2024, and December 31, 2023, none of the warrants have been exercised. The warrants have been evaluated to determine the appropriate accounting and classification pursuant to ASC 480 and ASC 815. Generally, freestanding warrants should be classified as (i) liabilities if the warrant terms allow settlement of the warrant exercise in cash and (ii) equity if the warrant terms only allow settlement in shares of common stock.

The Company determined that its outstanding warrants and the Put Option should be recorded as a liability and stated at fair value at each reporting period. Changes to the fair value of the warrant liability are recorded through the unaudited condensed consolidated statements of operations as a fair value adjustment related to warrant and CVR liability. As of September 30, 2024, and December 31, 2023, the fair value of the liability associated with these warrants and the Put Option was approximately \$13.9 million and \$16.1 million, respectively. The fair value adjustment related to these warrants and the Put Option was approximately \$6.0 million of loss and \$2.2 million of income for the three and nine months ended September 30, 2024, respectively. The fair value adjustment related to these warrants and the Put Option was approximately \$3.7 million and \$4.3 million of income for the three and nine months ended September 30, 2023, respectively.

H. Stock-Based Compensation

The Company maintains a stock-based compensation plan (the “Incentive Stock Plan”) that governs stock awards made to employees and directors prior to completion of the IPO.

In November 2014, the Board of Directors of the Company ("the Board"), and in April 2015, the Company’s stockholders, approved the Company’s 2014 Equity Incentive Plan (the “2014 Plan”), which became effective in April 2015. The 2014 Plan provides for the grant of stock options, other forms of equity compensation, and performance cash awards. In June 2021, the Company's stockholders approved an Amended and Restated 2014 Equity Incentive Plan (the "A&R 2014 Plan"), following its adoption by the Board in April 2021, which among other things added 4,900,000 shares to the maximum number of shares of common stock to be issued under the plan and extended the annual automatic increases (discussed further below) until January 1, 2031 and eliminated individual grant limits that applied under the 2014 Plan to awards that were intended to comply with the exemption for "performance-based compensation" under Code Section 162(m). The maximum number of shares of common stock that may be issued under the A&R 2014 Plan is 9,932,883 as of September 30, 2024. The number of shares of common stock reserved for issuance under the A&R 2014 Plan will automatically increase on January 1 of each year, until and including January 1, 2031, by 4% of the total number of shares of the Company’s capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by the Board. Pursuant to the terms of the 2014 Plan, on January 1, 2024, the common stock reserved for issuance under the 2014 Plan automatically increased by 1,661,386 shares.

During the three and nine months ended September 30, 2024, 27,750, and 761,500 stock options were exercised, respectively. During the three and nine months ended September 30, 2023, no stock options were exercised.

In June 2021, the Company's stockholders approved an Employee Stock Purchase Plan (the "ESPP"), following its adoption by the Board in April 2021. The maximum number of shares of common stock that may be issued under the ESPP is 1,500,000. The first offering period under the ESPP began on October 1, 2021, and the first purchase date occurred on May 31, 2022. As of September 30, 2024, 257,575 shares have been issued under the ESPP.

In January 2023, the Board approved the 2023 Employment Inducement Award Plan (the "2023 Plan"). The maximum number of shares of common stock that were initially available for issuance under the 2023 Plan was 1,500,000. In February 2024, the Board approved an amendment to the 2023 Plan to increase the aggregate number of shares of common stock available for issuance under the 2023 Plan from 1,500,000 to 4,500,000 shares.

In May 2023, the Board approved the Ninth Amended and Restated Non-Employee Director Compensation Policy (the “Non-Employee Director Compensation Policy”). The equity compensation made pursuant to the Non-Employee Director Compensation Policy will be granted under the A&R 2014 Plan.

Stock-based compensation expense recorded under the Incentive Stock Plan, A&R 2014 Plan, ESPP and 2023 Plan is included in the following line items in the accompanying unaudited condensed consolidated statements of operations (in thousands):

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Research and development	\$ 1,441	\$ 720	\$ 3,322	\$ 2,023
Selling, general and administrative	4,696	667	7,566	1,058
Total stock-based compensation expense	\$ 6,137	\$ 1,387	\$ 10,888	\$ 3,081

During the three and nine months ended September 30, 2024, the Company recognized approximately \$2.5 million in stock-based compensation expense related to performance-based awards. There was no stock-based compensation expense related to performance-based awards recognized during the nine months ended September 30, 2023.

I. Fair Value of Financial Instruments

The accounting standard for fair value measurements provides a framework for measuring fair value and requires disclosures regarding fair value measurements. Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date, based on the Company's principal or, in absence of a principal, most advantageous market for the specific asset or liability.

The Company uses a three-tier fair value hierarchy to classify and disclose all assets and liabilities measured at fair value on a recurring basis, as well as assets and liabilities measured at fair value on a non-recurring basis, in periods subsequent to their initial measurement. The hierarchy requires the Company to use observable inputs when available, and to minimize the use of unobservable inputs, when determining fair value. The three tiers are defined as follows:

- Level 1—Observable inputs that reflect quoted market prices (unadjusted) for identical assets or liabilities in active markets;
- Level 2—Observable inputs other than quoted prices in active markets that are observable either directly or indirectly in the marketplace for identical or similar assets and liabilities; and
- Level 3—Unobservable inputs that are supported by little or no market data, which require the Company to develop its own assumptions.

The carrying amounts of certain financial instruments, including cash and cash equivalents, investments, and accounts payable and accrued expenses, approximate their respective fair values due to the short-term nature of such instruments.

Assets and Liabilities Measured at Fair Value on a Recurring Basis

The Company evaluates its financial assets and liabilities subject to fair value measurements on a recurring basis to determine the appropriate level in which to classify them for each reporting period. This determination requires significant judgments to be made. The following table summarizes the conclusions reached regarding fair value measurements as of September 30, 2024, and December 31, 2023 (in thousands):

	Balance as of September 30, 2024	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
CVR liability (Note L)	\$ 4,800	\$ —	\$ —	\$ 4,800
Warrant liabilities	13,902	—	—	13,902
Total liabilities	\$ 18,702	\$ —	\$ —	\$ 18,702
Securities:				
U.S. Treasury securities	\$ 35,337	\$ 35,337	\$ —	\$ —
Corporate bonds	6,105	—	6,105	—
Total assets	\$ 41,442	\$ 35,337	\$ 6,105	\$ —

	Balance as of December 31, 2023	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
CVR liability (Note L)	\$ 7,262	\$ —	\$ —	\$ 7,262

Warrant liabilities	16,100	—	—	16,100
Total liabilities	<u>\$ 23,362</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 23,362</u>
Securities:				
U.S. Treasury securities	\$ 24,688	\$ 24,688	\$ —	\$ —
Total assets	<u>\$ 24,688</u>	<u>\$ 24,688</u>	<u>\$ —</u>	<u>\$ —</u>

The common stock warrant liabilities were recorded at fair value using the Black-Scholes option pricing model. The following assumptions were used in determining the fair value of the warrant liabilities valued using the Black-Scholes option pricing model as of September 30, 2024, and December 31, 2023:

	September 30, 2024	December 31, 2023
Risk-free interest rate	3.51% - 3.81%	3.76% - 4.12%
Volatility	61.76% - 82.44%	62.01% - 92.42%
Dividend yield	0%	0%
Expected term (years)	1.27 - 4.14	2.0 - 4.9
Weighted average fair value	\$ 2.54	\$ 2.94

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The following table is a reconciliation for the common stock warrant liabilities measured at fair value using Level 3 unobservable inputs (in thousands):

Balance as of December 31, 2023	\$ 16,100
Change in fair value measurement	(2,198)
Balance as of September 30, 2024	<u>\$ 13,902</u>

For the nine months ended September 30, 2024, and 2023, the changes in fair value of the warrant liabilities primarily resulted from the volatility of the Company's common stock and the change in the risk-free interest rates.

J. Net Loss Per Share

For all periods presented herein, the Company did not use the two-class method to compute net loss per share of common stock, even though it had issued securities, other than common stock, that contractually entitled the holders to participate in dividends and earnings, because these holders are not obligated to participate in a loss. The two-class method requires earnings for the period to be allocated between common stock and participating securities based upon their respective rights to receive distributed and undistributed earnings.

Under the two-class method, for periods with net income, basic net income per share of common stock is computed by dividing the undistributed net income by the weighted average number of shares of common stock outstanding during the period. Undistributed net income is computed by subtracting from net income the portion of current period earnings that participating securities would have been entitled to receive pursuant to their dividend rights had all of the period's earnings been distributed and subtracting the actual or deemed dividends declared. No such adjustment to earnings is made during periods with a net loss as the holders of the participating securities have no obligation to fund losses. Diluted net income per share of common stock is computed under the two-class method by using the weighted average number of shares of common stock outstanding plus the potential dilutive effects of stock options, warrants and other outstanding convertible securities. In addition to analyzing under the two-class method, the Company analyzes the potential dilutive effect of stock options and warrants, under the treasury-stock method and other outstanding convertible securities under the if-converted method when calculating diluted income (loss) per share of common stock, in which it is assumed that the stock options, warrants and other outstanding convertible securities convert into common stock at the beginning of the period or date of issuance, if the stock option, warrant or other outstanding convertible security was issued during the period. The Company reports the more dilutive of the approaches (two-class or treasury-stock/if-converted) as its diluted net income (loss) of common stock during the period.

As noted above, for all periods presented herein, the Company did not utilize the two-class approach as the Company was in a net loss position and the holders of the participating securities have no obligation to fund losses. The Company did analyze diluted net loss per share of common stock under the treasury-stock/if-converted method and noted that all outstanding stock options and warrants were anti-dilutive for the periods presented. For all periods presented, basic net loss per share of common stock was the same as diluted net loss per share of common stock.

The following securities, presented on a common stock equivalent basis, have been excluded from the calculation of weighted average number of shares of common stock outstanding because their effect is anti-dilutive:

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Awards under equity incentive plans	8,987,997	7,262,039	8,987,997	7,262,039
Common stock warrants	5,483,537	4,252,490	5,483,537	4,252,490
Total securities excluded from the calculation of weighted average number of shares of common stock outstanding	<u>14,471,534</u>	<u>11,514,529</u>	<u>14,471,534</u>	<u>11,514,529</u>

A reconciliation from net loss to basic and diluted net loss per share of common stock for the three and nine months ended September 30, 2024, and 2023, is as follows (in thousands):

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023

**Basic and diluted net loss per share of
common stock:**

Net loss, basic and diluted	\$	(33,225)	\$	(10,367)	\$	(69,772)	\$	(26,157)
Weighted average number of shares of common stock outstanding, basic and diluted		47,809		34,725		43,844		34,364
Basic and diluted net loss per share of common stock	\$	(0.69)	\$	(0.30)	\$	(1.59)	\$	(0.76)

K. Leases

The Company has operating and finance leases for office space, laboratory facilities and various laboratory equipment, furniture and office equipment and leasehold improvements. The Company determines if an arrangement is a lease at contract inception. Lease assets and lease liabilities are recognized based on the present value of lease payments over the lease term at the commencement date. The Company does not separate lease and non-lease components. Leases with a term of twelve (12) months or less at commencement are not recorded on the unaudited condensed consolidated balance sheets. Lease expense for these arrangements is recognized on a straight-line basis over the lease term. The Company's leases have remaining lease terms of less than 1 year to approximately 3 years, some of which include options to extend the leases for up to 5 years, and some which include options to terminate the leases within 1 year.

Effective June 1, 2021, the Company agreed to sublease office space in Florida, comprised of one of the two contiguous suites, under a non-cancelable operating lease, which expires in February 2026.

The components of lease expense were as follows (in thousands):

Lease Cost	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Finance lease cost:				
Amortization of right-of-use assets	\$ 12	\$ 26	\$ 36	\$ 90
Interest on lease liabilities	—	—	—	—
Total finance lease cost	12	26	36	90
Operating lease cost	119	113	330	340
Short-term lease cost	59	55	177	165
Variable lease cost	26	13	39	39
Less: sublease income	(50)	(39)	(128)	(117)
Total lease costs	<u>\$ 166</u>	<u>\$ 168</u>	<u>\$ 454</u>	<u>\$ 517</u>

Supplemental cash flow information related to leases was as follows (in thousands):

	Nine months ended September 30,	
	2024	2023
Cash paid for amounts included in the measurement of lease liabilities:		
Financing cash flows from finance leases	\$ —	\$ 5
Operating cash flows from operating leases	405	426
Operating cash flows from short-term leases	177	165
Operating cash flows from variable lease costs	39	39
Right-of-use assets obtained in exchange for lease liabilities:		
Finance leases	\$ —	\$ —
Operating leases	419	—

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Supplemental balance sheet information related to leases was as follows (in thousands, except weighted average remaining lease term and weighted average discount rate):

	September 30, 2024	December 31, 2023
Finance Leases		
Property and equipment, at cost	\$ 1,031	\$ 1,031
less: accumulated depreciation and amortization	(917)	(882)
Property and equipment, net	\$ 114	\$ 149
Other current liabilities	\$ —	\$ —
Other long-term liabilities	—	—
Total finance lease liabilities	\$ —	\$ —
Operating Leases		
Operating lease right-of-use assets	\$ 820	\$ 790
Total operating lease right-of-use assets	\$ 820	\$ 790
Current portion of operating lease liabilities	\$ 540	\$ 543
Operating lease liabilities, less current portion	483	456
Total operating lease liabilities	\$ 1,023	\$ 999
Weighted Average Remaining Lease Term		
Operating leases (in years)	2	2
Weighted Average Discount Rate		
Finance leases	0.0%	14.3%
Operating leases	9.6%	7.6%

Maturities of lease liabilities were as follows (in thousands):

Year Ending December 31,	Finance Leases	Operating Leases
2024 (excluding the nine months ended September 30, 2024)	\$ —	\$ 167
2025	—	536
2026	—	146
2027	—	136
2028	—	161
Thereafter	—	49
Total lease payments	—	1,195
Less: future interest expense	—	(172)
Lease liabilities	\$ —	\$ 1,023

L. Merger

On August 30, 2023, in connection with the Merger Agreement with Acer, the following transactions occurred prior to Closing:

- *Bridge Loan* - Zevra and Acer entered into a bridge loan agreement (the “Bridge Loan Agreement”), providing for Zevra to make loans to Acer up to an aggregate principal amount of \$16.5 million. The Bridge Loan was provided to Acer to support its termination agreement with Relief Therapeutics Holding SA and to provide Acer with working capital, including for payments of accounts payable to support the commercial launch of OLPRUVA and the development of celirolol pending the Merger’s closure. On October 31, 2023, the Company and Acer entered into an amendment to the Bridge Loan Agreement, which increased the aggregate principal amount available under the loan from \$16.5 million to \$18.0 million.
- *Purchase of Acer’s Term Loans* - Zevra purchased certain indebtedness of Acer held by Nantahala. Under the loan purchase with Nantahala, Zevra purchased (i) an original senior secured term loan facility made available to Acer in an aggregate amount of \$6.5 million and funded on March 14, 2022, and (ii) an additional senior secured term loan made to Acer in an aggregate amount of \$7.0 million in a single borrowing which funded on January 31, 2023 for (1) \$12.0 million in cash; (2) 98,683 shares of Zevra Common Stock; and (3) a secured Promissory Note payable by Zevra to Nantahala in the original principal amount \$5.0 million. These were recorded as receivables from Acer and were treated as a settlement of a preexisting relationship in connection with the closing of the transaction and recorded as a component of purchase consideration.
- *Purchase of Acer’s Convertible Notes* (“Marathon Convertible Notes”) - Under the Note Purchase Agreement with Nantahala, Zevra purchased the Marathon Convertible Notes that Nantahala had acquired on June 16, 2023. Zevra acquired the Marathon Convertible Notes in exchange for the issuance of 2,171,038 shares of Zevra Common Stock at \$5.0667 per share for a total purchase price of \$11.0 million.
- *Amendment to IP License Agreement and IP Termination Agreement* - As a condition to entering into the Merger Agreement, Acer and Relief entered into the Exclusive License Agreement and the Termination Agreement terminating the collaboration and license agreement, dated March 19, 2021, by and between Acer and Relief. Pursuant to the Exclusive License Agreement, Relief holds exclusive development and commercialization rights for OLPRUVA in Geographical Europe. Acer has the right to receive a royalty of up to 10.0% of the net sales of OLPRUVA in Geographical Europe. In accordance with the terms of the Termination Agreement, Relief received an upfront payment from Acer of \$10.0 million (which payment was funded with the Bridge Loan described above) with an additional payment of \$1.5 million due on the first-year anniversary of the \$10.0 million payment. Acer also agreed to pay a 10.0% royalty on net sales of OLPRUVA worldwide, excluding Geographical Europe, and 20.0% of any value received by Acer from certain third parties relating to OLPRUVA licensing or divestment rights, all of the foregoing which are capped at \$45.0 million, for total payments to Relief of up to \$56.5 million.

In connection with the closing of the Merger on November 17, 2023, each share of common stock of Acer was converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value \$0.0001 per share, and (ii) one non-transferable CVR to be issued by Zevra, which will represent the right to receive one or more contingent payments up to an additional \$76 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer’s OLPRUVA and celirolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer’s early-stage program ACER-2820 (emetine).

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The assets acquired and liabilities assumed were recorded based on their acquisition date fair values. Consideration for the Merger was \$72.6 million and consists of (i) approximately 2.96 million shares of Zevra common stock valued at \$12.8 million, (ii) the Bridge Loan advances of \$17.8 million, (iii) \$12.0 million in cash paid to Nantahala; (iv) 2.27 million shares of Zevra Common Stock issued to Nantahala valued at \$11.5 million based on the VWAP of shares of Zevra Common Stock during the 20 consecutive trading days ending on the trading date prior to August 30, 2023; (v) a secured promissory note payable by Zevra to Nantahala in the original principal amount of \$5.0 million, as disclosed in Note C, (vi) \$8.5 million in the estimated fair value of contingent consideration related to the CVRs, (vii) approximately 0.9 million shares of Zevra Common Stock issued to a former holder of Acer warrants valued at \$4.0 million based on Zevra's common stock price on the Effective Date and (viii) \$1.0 million in notes payable paid by the Company on Acer's behalf. In addition, effective as of immediately prior to the Effective Time, all of the outstanding and unexercised Acer stock options were automatically cancelled and ceased to exist without any cash or other consideration being paid or provided in respect thereof. The following purchase price allocation reflects the preliminary estimates of and assumptions related to the fair values of assets acquired and liabilities assumed:

Assets	
Cash	\$ 575
Prepaid expenses	278
Other current assets	11
Inventory	9,376
Property, plant, and equipment	35
Other noncurrent assets	209
Approved product - OLPRUVA	68,000
IPR&D - celiprolol	2,000
Goodwill acquired	4,701
	<u>85,185</u>
Liabilities	
Accounts payable and accrued expenses	\$ 10,881
Deferred collaboration funding	1,500
Operating lease liabilities	175
	<u>12,556</u>
Fair Value of Net Assets Acquired	<u>\$ 72,629</u>

The preliminary fair values assigned to the tangible and intangible assets acquired and liabilities assumed were determined using an income approach based on management's estimates and assumptions, as well as other information compiled by management, including third-party valuations that utilize customary valuation procedures and techniques. These preliminary fair values are subject to change within the one-year measurement period. The estimated fair values were developed by discounting future net cash flows to their present value at market-based rates of return. The goodwill acquired represents the excess of the purchase price and related costs over the value assigned to the net tangible and identifiable intangible assets of the business acquired. The useful lives of the intangible assets for amortization purposes were determined by considering the period of expected cash flows used to measure the fair values of the intangible assets adjusted as appropriate for entity-specific factors including legal, regulatory, contractual, competitive, economic and other factors that may limit the useful life. The marketed product asset is amortized on a straight-line basis over its estimated useful life. As of September 30, 2024, the in-process research and development ("IPR&D") project had not been completed or abandoned and, therefore, the IPR&D intangible asset is not currently subject to amortization.

The results of operations and changes in stockholders' equity for Acer were included in the Company's consolidated financial statements beginning November 18, 2023.

The following pro forma combined results of operations present the acquisition as if it had occurred on January 1, 2023. The pro forma combined results of operations do not necessarily represent the Company's consolidated results of operations had the acquisition occurred on the date assumed, nor are these results necessarily indicative of the Company's future consolidated results of operations. The Company expects to realize certain benefits from integrating Acer into the Company and to incur certain one-time costs. The pro forma combined results of operations do not reflect these benefits or costs.

	Three Months Ended September 30, 2023	Nine Months Ended September 30, 2023
Pro forma revenue	\$ 3,267	\$ 14,913
Pro forma net loss	(25,900)	(54,763)

Cancellation of Acer Warrant

On November 22, 2023, the Company sold an aggregate of 917,934 shares of its common stock to a healthcare focused investment fund (the "Investor") to cancel a warrant held by the Investor to purchase 2,920,306 shares of common stock of Acer. The shares of common stock were offered and sold to the Investor in a registered direct offering without an underwriter or placement agent.

Contingent Consideration

Contingent consideration liabilities relate to our liabilities arising in connection with the CVRs issued as a result of the Merger. The contingent consideration is classified as Level 3 in the fair value hierarchy. The fair value is measured based on a Monte Carlo simulation or a scenario-based method, depending on the earn-out achievement objectives, utilizing projections about future performance. Significant inputs include volatility and projected financial information, including projections representative of a market participant's view of the expected cash payments associated with the agreed upon regulatory milestones based on probabilities of technical success, timing of the potential milestone events for the compounds, and estimated discount rates.

The following table provides a reconciliation of the beginning and ending balances related to the contingent consideration liabilities for the CVRs (dollars in thousands):

Balance at December 31, 2023	\$ 7,262
Change in fair value recognized in earnings	(2,462)
Balance at September 30, 2024	<u>\$ 4,800</u>

For the nine months ended September 30, 2024, the Company recorded a \$2.5 million gain on the change in fair value of contingent consideration, primarily due to changes in market data and revenue projections.

M. Goodwill & Intangible Assets

The Company's goodwill balance was \$4.7 million as of September 30, 2024, and December 31, 2023.

As of September 30, 2024, and December 31, 2023, non-amortizable intangible assets include IPR&D of \$2.0 million

As of September 30, 2024, and December 31, 2023, the Company had a definite-lived intangible asset, net related to the acquisition of OLPRUVA as a result of the Merger of \$62.6 million and \$67.2 million, respectively. This is amortized on a straight-line basis over the OLPRUVA patent life of thirteen years and is reviewed periodically for impairment. Amortization expense is recorded as intangible asset amortization in the unaudited condensed consolidated statements of operations and was \$1.5 million and \$4.6 million for the three and nine months ended September 30, 2024. No amortization expense related to definite-lived intangible assets was recognized for the three and nine months ended September 30, 2023.

In accordance with its policy, the Company reviews the estimated useful lives of its intangible assets on an ongoing basis. This review indicated that based on recent facts and circumstances, including detailed analysis on sales experience and prospective product strategy, the actual life of the definite-lived intangible asset related to OLPRUVA was longer than the estimated useful life previously established at the acquisition of the asset. As a result, effective September 30, 2024, the Company changed its estimate of the useful life of this intangible asset to better reflect the estimated periods during which this asset will remain in service. The estimated useful life was previously 11 years and was increased to approximately 13 years. The change in estimate had no effect on depreciation and amortization expense, net income, or basic and diluted earnings per share for the three and nine months ended September 30, 2024.

In connection with the XOMA License Agreement, the Company owed XOMA a royalty payment of \$6.0 million upon approval of MIPLYFFA in September 2024, which is included in intangible assets, net in the unaudited condensed consolidated balance sheet as of September 30, 2024.

This definite-lived intangible asset is amortized on a straight-line basis over the MIPLYFFA patent life of approximately five years and is reviewed periodically for impairment. Amortization expense is recorded as intangible asset amortization in the unaudited condensed

consolidated statements of operations and was de minimis for the three and nine months ended September 30, 2024 . No amortization expense related to definite-lived intangible assets was recognized for the three and nine months ended September 30, 2023 .

For intangible assets subject to amortization, estimated amortization expense for the five fiscal years subsequent to September 30, 2024, is expected to be \$6.5 million per year.

N. Subsequent Events

The Company evaluated events and transactions occurring subsequent to September 30, 2024, through November 13, 2024, the date the accompanying unaudited condensed consolidated financial statements were issued. During this period, there were no subsequent events that required recognition in the accompanying unaudited condensed consolidated financial statements, nor were there any additional non-recognized subsequent events that required disclosure.

ITEM 2. 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 our unaudited condensed consolidated financial statements and related notes thereto included elsewhere in this Quarterly Report on Form 10-Q. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, 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 Part II, Item 1A. "Risk Factors" of this Quarterly Report on Form 10-Q and Part I, Item 1A. "Risk Factors" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, filed with the SEC on April 1, 2024, 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.

Overview

We are a commercial-stage company focused on addressing unmet needs for the treatment of rare diseases. Our mission is to bring life changing therapeutics to people living with rare diseases. With unique, data-driven development and commercialization strategies, we are overcoming complex drug development challenges to make new therapies available to the rare disease community. We have a diverse portfolio of products and product candidates, which includes a clinical stage pipeline and commercial stage assets. Our team has specialized expertise and a track record of success in advancing promising therapies that face complex clinical and regulatory challenges with an approach that balances science and data with patient need.

Following the U.S. approval of AZSTARYS® (further described below) in March 2021, we undertook a strategic process to evaluate how to leverage and potentially augment the Company's existing capabilities while also considering where to invest in our pipeline to generate long-term shareholder value. With a track record of drug development success leading to approvals for products which had either difficult pathways to approval or where approvals were won following a complete response letter ("CRL") from the U.S. Food and Drug Administration ("FDA"), the Company determined to focus its expertise on rare disease indications, as well as seeking value-creating opportunities by building and directly commercializing product candidates in lieu of an out-licensing model. We are executing on this balanced approach by building a culture that is patient-focused and driven by our commitment to developing and making available therapies which address the myriad unmet needs within the rare disease community.

As part of our commitment to serving the rare disease community, in February 2023, we changed our name to Zevra Therapeutics, Inc. Our name, Zevra, is the Greek word for zebra, which is the internationally recognized symbol for rare disease. This name reflects our intense focus and dedication to developing transformational, patient-focused therapies for rare diseases with limited or no treatment options available, or treatment areas with significant unmet needs.

In May 2022, we purchased all of the assets and operations of Orphazyme A/S ("Orphazyme") related to arimoclomol, settled all of Orphazyme's actual outstanding liabilities to its creditors with a cash payment of \$12.8 million, and agreed to assume an estimated reserve clawback liability of \$5.2 million related to revenue generated from Orphazyme's Expanded Access Program in France (the "EAP").

On November 17, 2023, Zevra completed the acquisition of Acer Therapeutics, Inc. ("Acer"). Pursuant to the Merger Agreement, Acer continues as a wholly-owned subsidiary of Zevra (the "Merger"). The Merger included the acquisition of OLPRUVA® (sodium phenylbutyrate) for oral suspension, which was approved by the FDA on December 27, 2022, for the treatment of certain urea cycle disorders ("UCDs"). Acer also had a pipeline of investigational product candidates, including celiprolol for the treatment of Vascular Ehlers-Danlos syndrome ("VEDS") in patients with a confirmed type III collagen (COL3A1) mutation. At the effective time of the Merger (the "Effective Time"), each share of common stock of Acer, par value \$0.0001 per share, issued and outstanding immediately prior to the Effective Time (excluding cancelled shares and any shares held by holders who have exercised their appraisal rights) were converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value \$0.0001 per share, and (ii) one non-transferable contingent value right ("CVR") issued by Zevra, which represents the right to receive one or more contingent payments up to an additional \$76.0 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer's OLPRUVA and celiprolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer's early-stage program ACER-2820 (emetine).

On September 20, 2024, the FDA approved the New Drug Application ("NDA") for MIPLYFFA™ (arimoclomol), an orally-delivered treatment for Niemann-Pick disease type C ("NPC"), which is an ultra-rare and progressive neurodegenerative disease. MIPLYFFA, the first FDA-approved treatment for NPC, is indicated for use in combination with miglustat for the treatment of neurological manifestations

of NPC in adult and pediatric patients two years of age and older. In addition, we received a transferable rare pediatric disease priority review voucher in conjunction with the approval. MIPLYFFA has also been granted orphan medical product designation for the treatment of NPC by the European Commission.

Now focused on late-stage clinical development and commercial opportunities, Zevra has discontinued its in-house drug discovery activities and is closing its laboratory facilities in Iowa and Virginia at the end of the third quarter. Future early research and development activities will be outsourced.

To accomplish our mission, we are seeking to further expand our pipeline through both internal development and through our business development activities to collaborate, partner, and potentially acquire additional assets. We intend to target assets that will allow us to leverage the expertise and infrastructure that we have built to help mitigate risk and enhance our probability of success. In addition, we may consider external opportunities within neurology and neurodegenerative diseases, psychiatric disorders, and other rare diseases, along with adjacent or related therapeutic categories. If we are successful, expanding our pipeline could be accretive to our value proposition and has the potential to create incremental long-term value for stockholders.

Our recurring operating losses and negative cash flows from operations raise substantial doubt about our ability to continue as a going concern. The Company's ability to continue operating as a going concern is contingent upon its ability to generate revenue from approved products or obtain product candidate regulatory approvals, which would generate revenue, milestones, and cash flow sufficient to support ongoing operations and the satisfaction of financial covenants. We are early in our commercialization effort for OLPRUVA and MIPLYFFA and do not yet have a substantial basis to project future earnings, and our other sources of revenue are not sufficient to sustain our present activities on their own. Accordingly, our ability to continue as a going concern may require us to obtain additional financing to fund our operations. The perception of our inability to continue as a going concern may make it more difficult for us to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or debt, the terms of these securities may restrict our ability to operate. 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. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or altogether cease our research and development programs or future commercialization efforts.

Our Product Candidates and Approved Products

We have built a diverse portfolio of products and product candidates through a combination of internal development and strategic investments through acquisition. For example, we have employed our proprietary Ligand Activated Technology ("LAT") platform to develop approved products (e.g., AZSTARYS), and clinical development candidate (KP1077IH and KP1077N). Through our business development efforts, we have added commercial products (OLPRUVA and MIPLYFFA), and a clinical development candidate (celiprolol). We furthermore have a variety of product candidates and compounds that are clinical-stage and designed to address a variety of rare diseases and other indications.

Currently active commercial products and development assets are summarized in the table below:

Active Zevra Commercial and Development Assets

Parent Drug	Indication	Product / Candidate	Development Status	Next Milestone(s)
Sodium phenylbutyrate	Urea Cycle Disorders (UCD)	OLPRUVA	FDA Approved	Tracking Commercial Progress
Arimoclomol	Niemann Pick disease type C (NPC)	MIPLYFFA	FDA Approved	Tracking Commercial Progress
Celiprolol	Vascular Ehlers Danlos Syndrome (VEDS)	Celiprolol	Clinical - Phase 3	Phase 3 ongoing
Serdexmethylphenidate	Idiopathic Hypersomnia (IH)	KP1077IH	Clinical - Phase 2	Evaluation of potential Phase 3 Trial
Serdexmethylphenidate	Narcolepsy	KP1077N	Clinical - Phase 1/2	Evaluation of next steps based on potential Phase 3 Trial for KP1077IH
Serdexmethylphenidate and dexamethylphenidate	Attention Deficit and Hyperactivity Disorder (ADHD)	AZSTARYS	FDA Approved and Partnered	Collecting royalties and milestones

These anticipated milestones are based on information currently available to us. Our current plans and expectations are subject to a number of uncertainties, risks and other important factors that could materially impact our plans, including risks which are not solely within our control. See Part I, Item 1A. "Risk Factors" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, filed with the SEC on April 1, 2024, as updated by Part II, Item 1A. "Risk Factors" of this Quarterly Report on Form 10-Q.

OLPRUVA

OLPRUVA (sodium phenylbutyrate) for oral suspension is approved in the U.S. as adjunctive therapy to standard of care, which includes dietary management, for the chronic management of UCDs involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), orargininosuccinic acid synthetase (AS). OLPRUVA is a proprietary and novel formulation of sodium phenylbutyrate powder, packaged in pre-measured single-dose envelopes, that has shown bioequivalence to existing sodium phenylbutyrate powder but with a pH-sensitive polymer coating that is designed to minimize dissolution of the coating for up to five minutes after preparation.

UCDs are a group of rare, genetic disorders that can cause harmful ammonia to build up in the blood, potentially resulting in brain damage and neurocognitive impairments, if ammonia levels are not controlled. Any increase in ammonia over time is serious. Therefore, it is important to adhere to any dietary protein restrictions and have alternative medication options to help control ammonia levels. Approximately 1 in 100,000 people have UCD, and there are an estimated 800 patients who are actively treated with nitrogen scavenging therapy in the U.S. While there are therapies currently approved for the treatment of UCDs - specifically RAVICTI®, marketed by Amgen, Inc. (formerly Horizon Therapeutics) and PHEBURANE®, marketed by Medunik USA - there remain unmet needs for this community of patients. OLPRUVA offers benefits over other UCD treatments by eliminating issues with palatability, offering improved portability with its single-dose envelopes, and it comes in a dosage that is personalized to the patient based on weight.

To commercialize OLPRUVA for oral suspension in the U.S. we have built marketing, sales, medical affairs, distribution, managerial and other non-technical capabilities or is making arrangements with third parties to perform these services. During the quarter ended

December 31, 2023, we began generating revenue from the sale of OLPRUVA in the U.S. For additional information regarding the Merger, see Note L of our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q. Zevra has a partnership with Relief Therapeutics who has rights to commercialize OLPRUVA in various European Union [EU] countries, if approved. In addition, Zevra pays royalties of 10% of U.S. net sales plus milestones to Relief Therapeutics.

During the first half of 2024, we initiated the commercial launch of OLPRUVA in the U.S. We have focused our initial efforts on the approximately 40 metabolic treatment centers of excellence across the United States which treat the majority of UCD patients to build awareness with physicians regarding the benefits of OLPRUVA. In the months since launch, our team has been able to engage with more than 90% of our customers. We have seen meaningful growth in reimbursement coverage, which was approximately 55% of U.S. covered lives at the time of acquisition, to now approximately 76%. Zevra is refining its commercial strategy for OLPRUVA to focus on specific patient segments where there are fewer access barriers, and, to increase awareness through targeted patient education campaigns.

OLPRUVA summary:

- ***OLPRUVA is available in the U.S for the treatment of UCD.*** OLPRUVA is an adjunctive therapy for long-term management of adults and children weighing 20kg or greater with UCD from deficiencies of CPS, OTC, or AS.
- ***OLPRUVA is differentiated from currently available forms of phenylbutyrate.*** OLPRUVA is formulated to improve palatability while providing patients with a portable and discrete pre-measured dose.
- ***Zevra has assembled a team to support OLPRUVA and additional future commercial products.*** We have established an efficient commercial team which is designed to fully service the patients and prescribers within the rare disease indications we are pursuing.

MIPLYFFA (arimoclomol)

On September 20, 2024, the FDA approved the NDA for MIPLYFFA, an orally-delivered treatment for NPC, which is an ultra-rare and progressive neurodegenerative disease. MIPLYFFA, the first FDA-approved treatment for NPC, is indicated for use in combination with miglustat for the treatment of neurological manifestations of NPC in adult and pediatric patients two years of age and older. In addition, we received a transferable rare pediatric disease priority review voucher (“PRV”) in conjunction with the approval. MIPLYFFA has also been granted orphan medical product designation for the treatment of NPC by the European Commission.

NPC is characterized by an inability of the body to transport cholesterol and lipids inside of cells. Symptoms of NPC include a progressive impairment of mobility, cognition, speech, and swallowing, often culminating in premature death. The incidence of NPC is estimated to be one in 100,000 to 130,000 live births. We estimate that there are approximately 1,800 individuals with NPC in the U.S. and Europe, of these, approximately 300 have been diagnosed in the U.S. However, diagnostic challenges may affect the number of potential patients, and we believe that the availability of treatment options could increase awareness of the disease and assist in more accurately identifying patients. Effective therapies to treat NPC are desperately needed, and for this reason, MIPLYFFA is currently being made available to NPC patients in France, Germany, and other EU member states under various expanded access programs (“EAPs”).

Zevra holds global rights to develop and commercialize MIPLYFFA. We intend to seek regulatory approval in the E.U., and we are currently evaluating the potential to commercialize outside of the U.S., and/or to seek additional regulatory approvals to support future global commercialization opportunities.

MIPLYFFA summary:

- ***Demonstrated halting of disease progression.*** MIPLYFFA in combination with miglustat has demonstrated a halting of progression of the disease through 12 months of treatment.
- ***Ease of flexible administration as an oral treatment.*** MIPLYFFA is administered as an oral capsule that can be swallowed whole, opened and contents mixed with foods or liquids, or delivered through a feeding tube.
- ***Extensive clinical experience with favorable safety data.*** No significant safety findings have been reported with more than 600 patients treated in various clinical trials and through our EAPs.
- ***Advantageous regulatory designations.*** MIPLYFFA has been granted orphan medical product designation for the treatment of NPC by the European Commission. We received a transferable PRV upon approval of MIPLYFFA.

Celiprolol

The Merger with Acer included the acquisition of celiprolol. We are advancing celiprolol as an investigational product candidate for the treatment of VEDS in patients with a confirmed type III collagen (*COL3A1*) mutation. Celiprolol is a selective adrenergic modulator (“SAM”) and, if we receive the first approval in the U.S. for celiprolol, we believe it would be deemed a new chemical entity (“NCE”) in the U.S. Celiprolol is currently approved in the EU for the treatment of hypertension and angina.

Ehlers-Danlos Syndrome is an inherited disorder caused by mutations in the genes responsible for the structure, production, or processing of collagen, an important component of the connective tissues in the human body, or proteins that interact with collagen. VEDS causes abnormal fragility in blood vessels, which can give rise to aneurysms, abnormal connections between blood vessels known as arteriovenous fistulas, arterial dissections, and spontaneous vascular ruptures, all of which can be potentially life-threatening. The incidence of VEDS is estimated to be one in 50,000 to 200,000 people. There are approximately 7,500 patients in the U.S.

Currently, there are no approved therapies anywhere in the world for VEDS. However, celiprolol, prescribed off label, has become the standard of care therapy for VEDS in some European countries. Medical intervention for VEDS focuses on surgery, symptomatic treatment, genetic counseling, and prophylactic measures, such as avoiding intense physical activity, scuba diving, and violent sports. Therefore, patients must adopt a “watch and wait” approach following any confirmed diagnosis. Unfortunately, many of these arterial events have high mortality associated with them, and thus, a pharmacologic intervention that reduces the rate of events would be clinically meaningful.

Celiprolol received orphan drug designation from the FDA for the treatment of VEDS in 2015. In October 2018, a new celiprolol NDA was submitted to the FDA by Acer based on data obtained from the BBEST trial and was subsequently accepted by the FDA in October 2018 with priority review status. Following FDA review, Acer received a CRL from the FDA stating that it will be necessary

to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with VEDS. Subsequently, Acer appealed the FDA decision, and while the FDA denied the appeal, it described possible paths forward toward approval. In a May 2021 Type B meeting with the FDA, Acer discussed the conduct of an U.S.-based prospective, randomized, double-blind, placebo-controlled, decentralized clinical trial in patients with *COL3A1* positive VEDS, and sought the FDA's opinion on various proposed design features of the study.

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Based on FDA's feedback during the Type B meeting, we adopted a decentralized (virtual) event-based clinical trial design and use of an independent centralized adjudication committee with a primary endpoint based on clinical events associated with disease outcome. In April 2022, the FDA granted celiprolol Breakthrough Therapy designation (“BTD”) in the U.S. for the treatment of patients with COL3A1-positive VEDS.

In July 2022, Acer initiated enrollment in a Phase 3 long-term event-driven clinical trial designed based on the discussions from the May 2021 Type B meeting with the FDA, also known as the DiSCOVER trial. The DiSCOVER trial intends to enroll 150 VEDS patients, with 100 patients receiving celiprolol and 50 patients receiving placebo. Recruitment in the Phase-3 trial was restarted earlier this year after a brief hiatus and the trial is actively enrolling patients. We believe that celiprolol could address significant unmet needs as there are currently no approved treatments for VEDS in the U.S.

Celiprolol summary:

- **Currently, no approved treatments for VEDS in the U.S.** There are currently no approved treatments of VEDS in the U.S. and we believe that celiprolol, if approved, could be a significant innovation in the treatment of VEDS in the U.S. where current treatment options are focused primarily on surgical intervention.
- **Unique pharmacological profile.** Mechanism of action in VEDS patients is thought to be through vascular dilatation and smooth muscle relaxation, the effect of which is to reduce the mechanical stress on collagen fibers in the arterial wall, and thereby potentially less incidence of vascular ruptures.
- **Evidence of efficacy in the EU and extensive clinical experience from multiple trials.** Celiprolol has become the primary treatment for VEDS patients in several European countries. BBEST Clinical Trial data showed 76% reduction in risk of arterial events observed in COL3A1+ subpopulation, with additional data from a long-term observational study in France.
- **Regulatory designations.** Celiprolol for VEDS would be considered an NCE in the U.S. and has been granted Orphan Drug designation and Breakthrough Therapy designation.
- **Solid patent protection through 2038.** Celiprolol is generally protected by U.S. patents that will expire, after utilizing all appropriate patent term adjustments but excluding possible term extensions, in 2038.

KP1077

KP1077 is being developed and evaluated for the treatment of IH and narcolepsy. IH is a rare neurological sleep disorder affecting approximately 37,000 patients in the United States. The cardinal feature of IH is excessive daytime sleepiness (“EDS”), characterized by daytime lapses into sleep, or an irrepressible need to sleep that persists even with adequate or prolonged nighttime sleep. Additionally, those with IH have extreme difficulty waking, otherwise known as “sleep inertia,” suffer from severe and debilitating brain fog, and may fall asleep unintentionally or at inappropriate times, also known as narcolepsy. These symptoms often further lead to reported memory problems, difficulty maintaining focus, and depression.

There is currently only one approved product for the treatment of IH, XYWAV®, developed by Jazz Pharmaceuticals. A second product, WAKIX®, developed by Harmony Biosciences and originally approved for the treatment of EDS or cataplexy in adult patients with narcolepsy, but in October 2023, Harmony announced that the difference in outcome for EDS when comparing WAKIX and placebo in its Phase 3 trial with IH patients did not reach statistical significance. Prescribers also utilize narcolepsy medications and various stimulant products “off-label” to treat IH symptoms, with methylphenidate, a stimulant which has been classified by the DEA as a Schedule II controlled substance, being one of the most commonly used stimulants for treating IH. While each of these medications can help to address certain IH symptoms, there are also potential shortcomings, including dosing inconvenience, serious adverse events, such as elevated blood pressure and heart rate, and significant drug-to-drug interactions (“DDIs”), including with medications used to manage contraception and depression. In addition, patients have indicated that the effectiveness of their current medication was poor.

Narcolepsy is a rare, chronic, debilitating neurologic disorder of sleep-wake state instability that impacts up to 200,000 Americans and is primarily characterized by EDS and cataplexy (sudden loss of muscle tone while a person is awake) along with other manifestations of rapid eye movement (“REM”), sleep dysregulation, which intrude into wakefulness. In most patients, narcolepsy is caused by the loss of hypocretin, a neuropeptide in the brain that supports sleep-wake state stability. Typical symptom onset occurs in adolescence or young adulthood, but it can take up to a decade to be properly diagnosed. Although there are several approved medications for narcolepsy, we believe a treatment option based on serdexmethylphenidate (“SDX”), our proprietary prodrug of d-MPH which has

previously been classified as a Schedule IV controlled substance, with superior exposure/duration characteristics and low abuse potential may be beneficial.

We reported top-line data from a Phase 1 proof-of-concept study of SDX in the fourth quarter of 2021 and final data for the Phase 1 proof-of-concept study of SDX in the first quarter of 2022. The proof-of-concept study was a dose-escalation study to evaluate the pharmacokinetics, pharmacodynamic stimulant effects, and safety of single oral doses of SDX in subjects with a history of high-dose stimulant use. In the trial, 240 mg and 360 mg doses of SDX were observed to be well-tolerated and produced d-MPH exposure that appeared to increase proportionally with dose. Mean d-MPH plasma concentrations showed a gradual increase after SDX administration, reaching a broad peak from eight to twelve hours post-dose, followed by a shallow decline thereafter. Increased wakefulness, alertness, hypervigilance, and insomnia effects were reported by study participants, which we believe suggests that SDX produced targeted pharmacodynamic effects that have the potential to benefit patients with IH and other sleep disorders. In November 2022, we announced that the FDA has granted the orphan drug designation to SDX for the treatment of IH.

In January 2022, we announced that we had selected KP1077 for the treatment of IH and narcolepsy as our lead clinical development candidate. KP1077 utilizes SDX, our prodrug of d-MPH, as its API. During the first quarter of 2022, we initiated a Phase 1 clinical trial comparing the cardiovascular safety of SDX to immediate-release and long-acting formulations of RITALIN®, a commonly prescribed central nervous system ("CNS") stimulant. In September 2022, we announced topline data from our exploratory Phase 1 clinical trial, which showed the potential for higher dose formulations of SDX to be safe and well tolerated while avoiding the potential for greater cardiovascular safety risk compared to immediate-release and long-acting formulations of Ritalin.

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Based on the data, in December 2022, we announced the initiation of a double-blind, placebo-controlled, randomized-withdrawal, dose-optimizing, multi-center Phase 2 clinical trial evaluating the efficacy and safety of KP1077 for the treatment of IH. The trial concluded in March 2024 and provided meaningful information of the optimal dose and dosing regimen to inform Phase 3 trial design.

We enrolled 48 adult patients with IH in more than 30 centers in the United States. Part 1 of the trial consisted of a five-week open-label titration phase during which patients were optimized to one of four doses of SDX (80, 160, 240, or 320 mg/day). Part 2 of the trial entailed a two-week randomized, double-blind, withdrawal phase, during which two-thirds of the trial participants will continue to receive their optimized dose while the remaining one-third will receive placebo. Participants were further assigned into two evenly divided cohorts. The first cohort received a single daily dose just before bedtime, and the second cohort received half the daily dose shortly after awakening and half the daily dose prior to bedtime.

Clinically meaningful improvements were observed across all studied endpoints. The trial was not powered for statistical significance, and this was not the primary endpoint. The exploratory endpoints of sleep inertia and brain fog performed in-line with expectations and were stable when compared across a variety of other endpoints. Symptom improvements in patients receiving KP1077 were similar after both once-per-day, and twice-per-day dosing.

In the Phase 2 trial, KP1077 was observed to be well-tolerated at all dose levels and both dosing regimens, with adverse events that are typical for stimulants and mostly mild in severity. These results are consistent with data from the Phase 1 trial with SDX that indicated no greater cardiovascular safety risk despite higher overall exposure levels when compared to both immediate and long-acting methylphenidate products currently used off-label for the treatment of IH. The trial concluded in March 2024 and provided meaningful information of the optimal dose and dosing regimen to inform Phase 3 trial design. On June 3, 2024, we announced final results from the Phase 2 Clinical Trial of KP1077 for IH. The proof-of-concept study was designed to demonstrate safety and tolerability and was not powered to demonstrate statistical significance. However, the trial included several important secondary and exploratory endpoints, such as the change in Epworth Sleepiness Scale ("ESS") total score, the IH Severity Scale ("IHSS"), the Sleep Inertia Visual Analog Scale ("SIVAS"), and a new scale to assess the symptoms and severity of brain fog. These data gathered from the secondary endpoints will help inform the study design for a potential Phase 3 clinical trial of KP1077. We held the end-of-Phase 2 meeting with the FDA in September 2024, to discuss the design of a pivotal Phase 3 trial to study KP1077 in IH.

Key Takeaways from Phase 2 Clinical Trial of KP 1077 for Idiopathic Hypersomnia include:

- KP1077 was well tolerated at all dose levels evaluated in the trial, including the highest dose of 320 mg daily, regardless of the dosing regimen: once daily (QD) or twice daily (BID).
 - Adverse events (AEs) were similar to other methylphenidate products
 - Most common AEs included insomnia, headache, anxiety, decreased appetite, and nausea
 - Most AEs occurred during the titration period, were mild, and did not lead to early discontinuation
- KP1077 produced clinically meaningful improvements in EDS as assessed by change from baseline in the ESS during both the 5-week open-label (OL) titration period which was maintained during the 2-week double-blind withdrawal period for both dosing regimens.
 - Mean total ESS scores decreased by approximately 9 points after 5 weeks of OL treatment.
- At the end of 7 weeks of treatment, patients administered KP1077 showed clinically meaningful benefits in change from baseline for the ESS, IHSS, SIVAS, and Brain Fog Scale (BFS):
 - Mean total ESS score decreased by 9.4 (QD) and 8.8 (BID)
 - Mean total IHSS score decreased by 16.1 (QD) and 12.3 (BID)
 - Mean SIVAS score decreased by 25.9 (QD) and 17.2 (BID)
 - Mean total BFS symptom score decreased by 23.8 (QD) and 22.3 (BID)
- The study successfully fulfilled the objectives of providing key information for the design of a pivotal efficacy trial, and the results of the secondary efficacy endpoints were supportive of initiating a Phase 3 trial of KP1077.

We completed a Phase 1 clinical trial in healthy volunteers to assess proposed dosing regimen for the narcolepsy indication. We are leveraging the data from the study and the IH program in evaluating the potential to initiate a Phase 3 trial in narcolepsy.

As discussed above, we recently completed an End-of-Phase 2 meeting with the FDA who indicated that a single pivotal study with appropriate confirmatory evidence will be sufficient to submit a new NDA. We are evaluating strategic alternatives to advance clinical development toward NDA submission and later, commercialization.

KP1077 is subject to the terms and conditions of the AZSTARYS License Agreement (as defined below) but is not currently licensed to Commave thereunder.

KP1077 Summary:

- **No drug-to-drug interactions.** We have not observed drug-to-drug interactions in clinical drug-drug interaction studies.
- **Potential for reduced abuse potential as a Schedule IV controlled substance.** All other methylphenidate-based products have been designated as Schedule II controlled substances, which indicates stricter control over the prescribing and use of such products. KP1077 is based on SDX, which has been designated a Schedule IV controlled substance.
- **No currently approved generic equivalent product.** KP1077 contains SDX, our proprietary prodrug of d-methylphenidate, also known as the new chemical name, serdexmethylphenidate, by the U.S. Adopted Names Council of the American Medical Association (“USAN”), which means that there may be no generic equivalent product for KP1077 in most U.S. states, making drug-equivalent substitution potentially difficult at the pharmacy.
- **Orphan drug designation.** Because small size of the IH patient population, the FDA has granted KP1077 orphan drug designation for the treatment of IH. We believe KP1077 may potentially be eligible for fast-track and breakthrough therapy designation, which may provide various regulatory benefits for the development program.

AZSTARYS (Partnered product)

AZSTARYS contains d-MPH and our prodrug of dexamethylphenidate, SDX. On March 2, 2021, the FDA approved AZSTARYS as a once-daily treatment for attention deficit hyperactivity disorder (ADHD), in patients age six years and older. AZSTARYS is currently being marketed in the U.S. under our September 2019 collaboration and license agreement, or the AZSTARYS License Agreement, with Commave. Under the AZSTARYS License Agreement, we granted to Commave an exclusive, worldwide license, to develop, manufacture, and commercialize AZSTARYS and any of our product candidates containing SDX and used to treat ADHD or any other CNS disease. In July 2020, we entered into the consulting agreement with Corium, Inc. ("Corium") (the "Corium Consulting Agreement"), under which Corium and Commave, respectively, engaged us to guide the product development and regulatory activities for certain current and potential future products in their portfolio, as well as continue supporting preparation for the potential commercial launch of AZSTARYS.

Commave has tasked Corium, another affiliate of Gurnet Point Capital, L.P., to lead all commercialization activities for AZSTARYS in the U.S. Corium commercially launched AZSTARYS in the U.S. during the third quarter of 2021. In December 2021, Commave entered into a sublicense of commercialization rights for AZSTARYS in greater China to Shanghai Ark Biopharmaceutical Ltd.

Pursuant to the AZSTARYS License Agreement, Commave agreed to pay up to \$63.0 million in milestone payments upon the occurrence of specified regulatory milestones related to AZSTARYS, including FDA approval and specified conditions with respect to the final approval label. In addition, Corium agreed to make additional payments upon the achievement of specified U.S. sales milestones of up to \$420 million in the aggregate. Further, Commave will pay us quarterly, tiered royalty payments based on a percentage of net sales on a product-by-product basis. Corium also agreed to be responsible for and reimburse us for all of development, commercialization and regulatory expenses for any products or product candidates containing SDX, subject to certain limitations as set forth in the AZSTARYS License Agreement, including consultation fees to be paid to us for services provided to Corium in performing such activities.

In April 2021, we entered into the AZSTARYS Amendment. Pursuant to the AZSTARYS Amendment, we and Commave agreed to modify the compensation terms of the AZSTARYS License Agreement. Commave paid us \$10.0 million in connection with the execution of the AZSTARYS Amendment following the FDA approval of AZSTARYS in the United States. Corium also paid us \$10.0 million following the SDX scheduling determination by the DEA, which occurred on May 7, 2021. In addition, the AZSTARYS Amendment increased the total remaining future regulatory and sales milestone payments related to AZSTARYS up to an aggregate of \$590.0 million. The AZSTARYS License Agreement will continue on a product-by-product basis (i) until expiration of the royalty term for the applicable product candidate in the United States and (ii) perpetually for all other countries.

In May 2021, we announced that SDX, our proprietary prodrug of d-MPH and the primary active pharmaceutical ingredient ("API") in AZSTARYS, was classified as a Schedule IV controlled substance by the DEA. AZSTARYS is classified as a Schedule II controlled substance as its formulation includes a 70:30 mixture of SDX (Schedule IV) and d-MPH (Schedule II), respectively.

During the first half of 2023, annual net sales of AZSTARYS surpassed \$25 million, triggering the first annual net sales milestone payment of \$5.0 million under the AZSTARYS License Agreement, which was earned and recognized as revenue in the second quarter of 2023, and received after quarter-end. During the second half of 2023, annual net sales of AZSTARYS surpassed \$50 million, triggering the second net sales milestone payment of \$10.0 million under the AZSTARYS License Agreement, which was earned and recognized in the fourth quarter of 2023.

Other Third-Party Agreements

Aquestive Termination Agreement

Under our March 2012 termination agreement with Aquestive Therapeutics ("Aquestive") has the right to receive a royalty amount equal to 10% of any value generated by AZSTARYS and any product candidates containing SDX. In connection with the AZSTARYS License Agreement, we paid Aquestive a royalty equal to 10% of the quarterly royalty payments and of the regulatory and net sales milestones earned in 2020, 2021, and 2023.

Distributor Agreement

Our current single distributor for sales of our approved products, OLPRUVA and MIPLYFFA, is a specialty pharmacy provider, however, the Company may establish additional specialty distributors or other retail pharmacies and certain medical centers or hospitals. In addition to distribution agreements, we enter into arrangements with health care providers and payors that provide for government mandated and/or privately negotiated rebates with respect to the purchase of our products.

Relief Exclusive License Agreement

As a condition to entering into the Merger Agreement, Acer and Relief Therapeutics SA ("Relief") entered into an exclusive license agreement on August 30, 2023 (the "Relief License Agreement"). Pursuant to the Relief License Agreement, Relief will hold exclusive development and commercialization rights for OLPRUVA in the EU, Liechtenstein, San Marino, Vatican City, Norway, Iceland, Principality of Monaco, Andorra, Gibraltar, Switzerland, United Kingdom, Albania, Bosnia, Kosovo, Montenegro, Serbia and North Macedonia ("Geographical Europe"). The Company will have the right to receive a royalty of up to 10% of the net sales of OLPRUVA in Geographical Europe.

XOMA Agreement

In May 2011, Orphazyme entered into an asset purchase agreement with LadRx Corporation, which was assigned to XOMA (US) LLC, a wholly-owned subsidiary of XOMA Corporation ("XOMA"), in June 2023 ("Xoma License Agreement"). Under the XOMA License Agreement, XOMA is entitled to certain net sales and regulatory milestone payments in addition to a mid-single digit royalty with respect to sales of MIPLYFFA.

Results of Operations

Comparison of the three months ended September 30, 2024, and 2023 (in thousands):

	Three months ended September 30,		Period-to-
	2024	2023	Period Change
Revenue, net	\$ 3,695	\$ 2,895	\$ 800
Cost of product revenue (excluding \$1,545 in intangible asset amortization for the three months ended September 30, 2024, shown separately below)	2,303	144	2,159
Intangible asset amortization	1,545	—	1,545
Operating expenses:			
Research and development	10,945	12,297	(1,352)
Selling, general and administrative	16,208	5,818	10,390
Total operating expenses	27,153	18,115	9,038
Loss from operations	(27,306)	(15,364)	(11,942)
Other (expense) income:			
Interest expense	(2,312)	(366)	(1,946)
Fair value adjustment related to warrant and CVR liability	(4,746)	3,678	(8,424)

Fair value adjustment related to investments	90	124	(34)
Interest and other income (expense), net	1,049	1,738	(689)
Total other (expense) income	(5,919)	5,174	(11,093)
Loss before income taxes	(33,225)	(10,190)	(23,035)
Income tax expense	—	(177)	177
Net loss	<u>\$ (33,225)</u>	<u>\$ (10,367)</u>	<u>\$ (22,858)</u>

Net Loss

Net loss for the three months ended September 30, 2024, was \$33.2 million, compared to net loss of \$10.4 million for the three months ended September 30, 2023, an increase in net loss of \$22.8 million. The change was primarily attributable to an increase in loss from operations of \$11.9 million and a decrease in other income of \$11.1 million.

Revenue

Revenue for the three months ended September 30, 2024, was \$3.7 million, compared to revenue of \$2.9 million for the three months ended September 30, 2023, an increase of \$0.8 million. The increase was primarily due to an increase in royalties and other reimbursements under the AZATARYS License Agreement of \$0.3 million and an increase in French EAP reimbursements of \$0.5 million. OLPRUVA revenue was de minimis for the period.

Cost of product revenue

Cost of product revenue for the three months ended September 30, 2024, increased by approximately \$2.2 million compared to the cost of product revenue for the three months ended September 30, 2023, primarily due to recognition of \$2.0 million of inventory obsolescence.

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Intangible asset amortization

Intangible asset amortization for the three months ended September 30, 2024 , was due to \$1.5 million in amortization expense related to definite-lived intangible assets acquired in the Merger.

Research and Development

Research and development expenses decreased by\$1.4 million, from \$12.3 million for the three months endedSeptember 30, 2023, to \$10.9 million for the three months ended September 30, 2024. This decrease was primarily driven by a decrease in spending for the ongoing Phase 2 clinical study in KP1077, partially offset by an increase in personnel-related costs and an increase in third-party costs related to MIPLYFFA.

Selling, General and Administrative

Selling, general and administrative expenses increased by\$10.4 million, from \$5.8 million for the three months ended September 30, 2023, to \$16.2 million for the three months ended September 30, 2024. The period-over-period increase was primarily related to an increase in personnel costs due to an increase in headcount and an increase in other expenses as we built our commercial organization.

Other (Expense) Income

Other expense for the three months ended September 30, 2024, was \$5.9 million compared to other income of \$5.2 million for the three months ended September 30, 2023. This change was primarily attributable to a decrease in the fair value adjustment related to warrant and CVR liability of \$8.4 million, an increase in interest expense of \$1.9 million, and a decrease in interest and other income of \$0.7 million.

Comparison of the nine months ended September 30, 2024, and 2023 (in thousands):

	Nine months ended September 30,		Period-to-
	2024	2023	Period Change
Revenue, net	\$ 11,569	\$ 14,541	\$ (2,972)
Cost of product revenue (excluding \$4,619 in intangible asset amortization for the nine months ended September 30, 2024, shown separately below)	6,051	946	5,105
Intangible asset amortization	4,619	—	4,619
Operating expenses:			
Research and development	33,743	28,385	5,358
Selling, general and administrative	38,743	19,657	19,086
Total operating expenses	72,486	48,042	24,444
Loss from operations	(71,587)	(34,447)	(37,140)
Other (expense) income:			
Interest expense	(5,157)	(745)	(4,412)
Fair value adjustment related to warrant and CVR liability	4,660	4,253	407
Fair value adjustment related to investments	64	451	(387)
Interest and other income (expense), net	2,248	4,331	(2,083)
Total other (expense) income	1,815	8,290	(6,475)
Loss before income taxes	(69,772)	(26,157)	(43,615)
Income tax expense	—	—	—
Net loss	\$ (69,772)	\$ (26,157)	\$ (43,615)

Net Loss

Net loss for the nine months ended September 30, 2024, was \$69.8 million, compared to net loss of \$26.2 million for the nine months ended September 30, 2023, an increase in net loss of \$43.6 million. The change was primarily attributable to an increase in loss from operations of \$37.1 million and a decrease in other income of \$6.5 million.

Revenue

Revenue for the nine months ended September 30, 2024, was \$11.6 million, compared to revenue of \$14.5 million for the nine months ended September 30, 2023, a decrease of \$2.9 million. This decrease was primarily due to a decrease in net sales milestones under the AZSTARYS License Agreement of \$5.0 million and a decrease in consulting revenue of \$1.5 million, partially offset by an increase in royalties and other reimbursements under the AZSTARYS License Agreement of \$2.4 million and an increase in French EAP reimbursements of \$1.2 million. OLPRUVA revenue was de minimis for the nine months ended September 30, 2024.

Cost of product revenue

Cost of product revenue for the nine months ended September 30, 2024, increased by approximately \$5.1 million compared to cost of product revenue for the nine months ended September 30, 2023, primarily due to recognition of \$5.2 million of inventory obsolescence.

Intangible asset amortization

Intangible asset amortization for the nine months ended September 30, 2024, was due to \$4.6 million in amortization expense related to definite-lived intangible assets acquired in the Merger.

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Research and Development

Research and development expenses increased by \$5.3 million, from \$28.4 million for the nine months ended September 30, 2023, to \$33.7 million for the nine months ended September 30, 2024. This increase was primarily driven by an increase in personnel-related costs and an increase in third-party research and development costs related to celiprolol, partially offset by a decrease in research and development costs related to a product for which the NDA was withdrawn during the nine months ended September 30, 2023.

Selling, General and Administrative

Selling, general and administrative expenses increased by \$19.0 million, from \$19.7 million for the nine months ended September 30, 2023, to \$38.7 million for the nine months ended September 30, 2024. The period-over-period increase was primarily related to an increase in personnel costs and fees associated with our commercial and business development activities.

Other Income

Other income decreased by \$6.5 million, from \$8.3 million for the nine months ended September 30, 2023, to \$1.8 million for the nine months ended September 30, 2024. This decrease was primarily attributable to an increase in interest expense of \$4.4 million, a decrease in interest and other income of \$2.1 million, and a decrease in the fair value adjustment related to investments of \$0.4 million, partially offset by an increase in fair value adjustment related to warrant and CVR liability of \$0.4 million.

Liquidity and Capital Resources

Sources of Liquidity

Through September 30, 2024, we have funded our research and development and operating activities primarily through the issuance of debt and equity and from revenue received under the EAP, AZSTARYS License Agreement, OLPRUVA product sales and consulting arrangements. As of September 30, 2024, we had cash, cash equivalents and investments of \$95.5 million.

To date, we have generated revenue from the EAP, AZSTARYS License Agreement, reimbursement of out-of-pocket third-party costs, the performance of consulting services, and sales of OLPRUVA.

We have had recurring negative net operating cash flows and we anticipate that we may continue to incur minimal positive net cash flows from operations or negative net cash flows from operations for at least the next several years. We expect that our sources of revenue will be through payments arising from our license agreement with Commave, the EAP, sales of OLPRUVA and MIPLYFFA and other potential consulting arrangements and any other future arrangements related to one of our product candidates.

We filed a registration statement on Form S-3 covering the sale of the shares of our common stock up to \$350.0 million, \$75.0 million of which was allocated to the sales of the shares of common stock issuable under the Equity Distribution Agreement (described below). The Form S-3 was declared effective on July 12, 2021. As of September 30, 2024, no shares had been issued or sold under the Equity Distribution Agreement and this registration statement under S-3 expired on July 12, 2024.

On June 4, 2024, we filed a registration statement on Form S-3 (File No. 333-279941) (the "June 2024 Registration Statement") under which we may sell securities, including as may be issuable upon conversion, redemption, repurchase, exchange or exercise of securities, in one or more offerings up to a total aggregate offering price of \$350.0 million, \$75.0 million of which was allocated to the sales of the shares of common stock issuable under the 2024 ATM Agreement (as described further below). The registration statement was declared effective on June 13, 2024.

On August 8, 2024, we entered into an underwriting agreement (the "Underwriting Agreement") with Cantor Fitzgerald & Co. and William Blair & Company, L.L.C., as representatives of the several underwriters named therein (collectively, the "Underwriters"), in connection with the August offering. Under the terms of the Underwriting Agreement, we also granted the Underwriters an option exercisable for 30 days to purchase up to an additional 1,384,615 shares of our common stock at the public offering price, less underwriting discounts and commissions, which the Underwriters exercised in full on August 9, 2024. The August 2024 Offering closed on August 12, 2024. Total shares issued were 10,615,385. Net proceeds from the offering were approximately \$64.5 million, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds of the offering to support the commercial launch activities for MIPLYFFA, continued commercial support for OLPRUVA and the continued development of celiprolol and KP1077 through potential NDA filings and other general corporate purposes.

We have incurred operating losses since our inception and, as of September 30, 2024, had an accumulated deficit of \$469.6 million. Our recurring operating losses and negative cash flows from operations raise substantial doubt about our ability to continue as a going concern. The Company's ability to continue operating as a going concern is contingent upon its ability to generate revenue from approved products or obtain product candidate regulatory approvals, which would generate revenue, milestones, and cash flow sufficient to support ongoing operations and the satisfaction of financial covenants. We are early in our commercialization effort for OLPRUVA and MIPLYFFA and do not yet have a substantial basis to project future earnings, and our other sources of revenue are not sufficient to sustain our present activities on their own. Accordingly, our ability to continue as a going concern may require us to obtain additional financing to fund our operations. The perception of our inability to continue as a going concern may make it more difficult for us to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or debt, the terms of these securities may restrict our ability to operate. 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. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or altogether cease our research and development programs or future commercialization efforts.

Equity Distribution Agreements

On July 12, 2024, we entered into an equity distribution agreement (the "2024 ATM Agreement") with Citizens JMP Securities LLC ("Citizens JMP") under which we may offer and sell, from time to time in our sole discretion, shares of our common stock having an aggregate offering price of up to \$75.0 million through Citizens JMP as our sales agent. The issuance and sale, if any, of common stock by us under the 2024 ATM Agreement will be made pursuant to a registration statement on Form S-3. Citizens JMP may sell the common stock by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415 of the Securities Act. Citizens JMP will use commercially reasonable efforts to sell the common stock from time to time, based upon instructions from us (including any price, time or size limits or other customary parameters or conditions we may impose). We will pay Citizens JMP a commission equal to 3.0% in the aggregate of the gross sales proceeds of any common stock sold through Citizens JMP under the 2024 ATM Agreement. In connection with the 2024 ATM Agreement, we filed the June 2024 Registration Statement, the accompanying prospectus, and the related prospectus supplement dated July 12, 2024, covering the sale of the shares of our common stock up to \$350.0 million, \$75.0 million of which was allocated to the sales of the shares of common stock issuable under the 2024 ATM Agreement.

On July 2, 2021, we entered into an equity distribution agreement ("2021 ATM Agreement") with JMP Securities LLC ("JMP") and RBC Capital Markets, LLC ("RBCCM"), under which we had the ability to offer and sell shares of our common stock having an aggregate offering price of up to \$75.0 million through JMP and RBCCM as our sales agents. The 2021 ATM Agreement expired on July 12, 2024.

Merger Transactions and Documents

On August 30, 2023, in connection with the Merger Agreement with Acer, the following transactions occurred prior to Closing:

- *Bridge Loan* – Zevra and Acer entered into a bridge loan facility for up to \$18.0 million ("Bridge Loan"), providing for Zevra to make the Bridge Loan to Acer up to an aggregate principal amount of \$16.5 million. The Bridge Loan was provided to Acer to support its termination agreement with Relief and to provide Acer with working capital, including for payments of accounts payable to support the commercial launch of OLPRUVA and the development of celiprolol pending the Merger's closure. On October 31, 2023, the Company and Acer entered into an amendment to the Bridge Loan Agreement, which increased the aggregate principal amount available under the loan from \$16.5 million to \$18.0 million.
- *Purchase of Acer's Term Loans* – Zevra purchased certain indebtedness of Acer held by Nantahala Capital Management, LLC ("NCM"). Under the loan purchase with Nantahala, certain of its affiliates and certain other parties (collectively with Nantahala, "Nantahala Holders") Zevra purchased (i) an original senior secured term loan facility made available to Acer in an aggregate amount of \$6.5 million and funded on March 14, 2022, and (ii) an additional senior secured term loan made to Acer in an aggregate amount of \$7.0 million in a single borrowing which funded on January 31, 2023 for (1) \$12.0 million in cash; (2) 98,683 shares of Zevra Common Stock; and (3) a secured Promissory Note payable by Zevra to Nantahala in the original principal amount \$5.0 million (the "Nantahala Note"). These were recorded as receivables from Acer and were treated as a settlement of a preexisting relationship in connection with the closing of the transaction and recorded as a component of purchase consideration. In April 2024, the Nantahala Note was repaid in full and terminated.
- *Purchase of Acer's Convertible Notes* ("Marathon Convertible Notes") – Under the Note Purchase Agreement with the Nantahala Holders, Zevra purchased the Marathon Convertible Notes that Nantahala had acquired on June 16, 2023. Zevra acquired the Marathon Convertible Notes in exchange for the issuance of 2,171,038 shares of Zevra Common Stock at \$5.0667 per share for a total purchase price of \$11.0 million.
- *Amendment to IP License Agreement and IP Termination Agreement*: As a condition to entering into the Merger Agreement, Acer and Relief entered into the Exclusive License Agreement and the Termination Agreement terminating the collaboration and license agreement, dated March 19, 2021, by and between Acer and Relief. Pursuant to the Exclusive License Agreement, Relief holds exclusive development and commercialization rights for OLPRUVA in Geographical Europe. Acer has the right to receive a royalty of up to 10% of the net sales of OLPRUVA in Geographical Europe. In accordance with the terms of the Termination Agreement, Relief received an upfront payment from Acer of \$10.0 million (which payment was funded with the Bridge Loan described above) with an additional payment of \$1.5 million due on the first-year anniversary of the \$10.0 million payment. Acer has also agreed to pay a 10% royalty on net sales of OLPRUVA worldwide, excluding Geographical Europe, and 20.0% of any value received by Acer from certain third parties relating to OLPRUVA licensing or divestment rights, all of the foregoing which are capped at \$45.0 million, for total payments to Relief of up to \$56.5 million.

In connection with the closing of the Merger on November 17, 2023, each share of common stock of Acer was converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value \$0.0001 per share, and (ii) one non-

transferable CVR to be issued by Zevra, which will represent the right to receive one or more contingent payments up to an additional \$76 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer's OLPRUVA and celiprolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer's early-stage program ACER-2820 (emetine).

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Registration Rights Agreement

Zevra and Nantahala concurrently entered into a registration rights agreement (the “Registration Rights Agreement”), pursuant to which Zevra agreed to file a resale registration statement with respect to the resale of the Zevra common stock issuable under the Loan and Note Purchase Agreements and the Nantahala Note. On February 5, 2024, Zevra filed a registration statement on Form S-3 (File No. 333-276856) registering an aggregate of 2,269,721 shares of Zevra’s common stock that were issued pursuant to the Loan and Note Purchase Agreements. On April 5, 2024, we filed an amendment to the registration statement on Form S-3 (File No. 333-250945) covering the issuance of the shares of our common stock issuable upon the exercise of warrants issued in connection with the Merger and remaining unexercised as of the date of the amendment, which was declared effective on April 8, 2024.

Cancellation of Acer Warrant

On November 22, 2023, we sold an aggregate of 1,382,489 shares of our common stock and accompanying warrants to purchase up to 1,382,489 shares of our common stock at a price of \$4.34 per share to a healthcare focused investment fund (the “Investor”) for gross proceeds of approximately \$6.0 million and an aggregate of 917,934 shares of our common stock to cancel a warrant held by the Investor to purchase 2,920,306 shares of common stock of Acer. The shares of common stock and warrants were offered and sold to the Investor in a registered direct offering without an underwriter or placement agent.

Line of Credit

On January 26, 2023, we and Wells Fargo, as lender, entered into a margin account agreement. Our investments were used as collateral for the loan and the amount we were able to borrow was limited to 80-90% of our outstanding investment balance held with Wells Fargo. The margin account bore interest at the Prime Rate minus 225 basis-points. This facility was paid off on April 5, 2024, and the margin capability was removed from the account.

Term Loans

On April 5, 2024 (the “Term Loans Closing Date”), we entered into a credit agreement (the “Credit Agreement”) with HCR Stafford Fund II, L.P., HCR Potomac Fund II, L.P., and Perceptive Credit Holdings IV, LP (collectively, the “Lenders”), and Alter Domus (US) LLC, as administrative agent (the “Administrative Agent”).

Under the terms of the Credit Agreement, the Lenders provided a senior secured loan facility to us in the aggregate principal amount of \$100.0 million, which is divided into three tranches as follows: (i) \$60.0 million which was funded in full on the Term Loans Closing Date; (ii) \$20.0 million which is available to us in up to two drawings, each in an amount not to exceed \$10.0 million, at the Company’s option until 18 months following the Term Loans Closing Date; and (iii) \$20.0 million which is available to us upon approval by the FDA of the NDA for MIPLYFFA for the treatment of NPC, at our option until December 31, 2024 (collectively, the “Term Loans”).

The principal amount of the Term Loans outstanding (the “Outstanding Principal Amount”) will bear interest at a rate equal to 3-Month Term SOFR *plus* 7.00% per annum. If the net product sales for the calendar year ending December 31, 2025, exceed \$100.0 million, the Outstanding Principal Amount will bear interest at 3-Month Term SOFR *plus* 6.00% per annum. If the net product sales for the calendar year ending December 31, 2025, do not exceed \$100.0 million, then for any subsequent period of four consecutive fiscal quarters ending on or after March 31, 2026, in which net product sales exceed \$125.0 million, the Outstanding Principal Amount will bear interest at 3-Month Term SOFR *plus* 6.50% per annum. In all cases, the 3-Month Term SOFR rate will be subject to a floor of 4.00% per annum. Interest will be payable quarterly in arrears on the last day of each calendar quarter. We have the option to pay up to 25% of the interest in-kind beginning on the Term Loans Closing Date, through and including June 30, 2026. The Term Loans will mature on the fifth anniversary of the Term Loans Closing Date. In connection with the Credit Agreement, we incurred approximately \$2.2 million of costs, which primarily consisted of underwriting, legal and other professional fees, and are included as a reduction to the carrying amount of the related debt liability and are deferred and amortized over the remaining life of the financing using the effective interest method.

The Credit Agreement contains customary affirmative and negative covenants by us, which among other things, will require us to provide certain financial reports to the Lenders, meet certain minimum net product sales amounts, and limit our ability to incur or guarantee additional indebtedness, engage in certain transactions, and effect a consolidation or merger without consent. In addition, as long as the line of credit remains active, we must maintain a minimum cash balance of \$20.0 million to ensure that we can meet our immediate capital needs. Our obligations under the Credit Agreement may be accelerated upon customary events of default, including non-payment of principal, interest, fees and other amounts, covenant defaults, insolvency, material judgments, or inaccuracy of representations and warranties. The Term Loans are secured by a first priority perfected lien on, and security interest in, substantially all of our current and future assets. The proceeds of the Term Loans were used to refinance certain of our previously existing indebtedness. We will use

the remaining proceeds to pay fees and expenses related to the debt financing and fund the development and commercialization of OLPRUVA and MIPLYFFA.

Cash Flows

The following table summarizes our cash flows for the nine months ended September 30, 2024, and 2023 (in thousands):

	Nine months ended September 30,	
	2024	2023
Net cash used in operating activities	\$ (53,415)	\$ (17,377)
Net cash used in investing activities	(16,690)	(27,975)
Net cash provided by financing activities	81,312	23,460
Effect of exchange rates on cash and cash equivalents	(217)	(305)
Net increase (decrease) in cash and cash equivalents	\$ 10,990	\$ (22,197)

Operating Activities

For the nine months ended September 30, 2024, net cash used in operating activities of \$53.4 million consisted of a net loss of \$69.8 million, changes in working capital of \$1.8 million, partially offset by \$18.2 million in adjustments for non-cash items. Net loss was primarily attributable to our spending on research and development programs and operating costs; partially offset by revenue received under the AZSTARYS License Agreement, and the EAP. The changes in working capital consisted of \$10.6 million related to a change in accounts payable and accrued expenses, \$4.2 million change in inventories, \$0.4 million related to a change in operating lease liabilities, an increase of \$0.5 million in prepaids and other assets and \$1.0 million related to a change in other liabilities, partially offset by \$9.6 million related to a change in accounts and other receivables, \$0.4 million related to a change in operating lease right-of-use assets, and \$4.8 million related to a change in discount and rebate liabilities. The adjustments for non-cash items primarily consisted of stock-based compensation expense of \$10.9 million, an inventory obsolescence charge of \$5.2 million, interest expense of \$1.5 million, and \$5.3 million related to depreciation, amortization and other items, partially offset by a change in the fair value of warrant and CVR liability of \$4.7 million.

For the nine months ended September 30, 2023, net cash used in operating activities of \$17.4 million consisted of a net loss of \$26.2 million; partially offset by \$0.4 million in adjustments for non-cash items and \$8.4 million in changes in working capital. Net loss was primarily attributable to our spending on research and development programs and operating costs; partially offset by revenue received under the AZSTARYS License Agreement, Arimoclomol EAP and the Corium Consulting Agreement. The changes in working capital consisted of \$3.9 million related to a change in discount and rebate liabilities, \$5.4 million related to a change in accounts payable and accrued expenses, \$0.2 million related to a change in operating lease right-of-use assets, \$0.2 million related to a change in inventories, \$0.7 million related to a change in other liabilities; partially offset by \$1.6 million related to a change in accounts and other receivables and \$0.3 million related to a change in operating lease liabilities. The adjustments for non-cash items primarily consisted of stock-based compensation expense of \$3.1 million, non-cash severance expense of \$1.4 million, and \$0.5 million related to depreciation, amortization and other items; partially offset by a change in the fair value adjustment related to warrant and CVR liability of \$4.2 million and a change in fair value adjustment related to investments of \$0.5 million.

Investing Activities

For the nine months ended September 30, 2024, net cash used in investing activities was \$16.7 million, which was primarily attributable to purchases of investments of \$41.5 million, partially offset by \$24.8 million in maturities of investments.

For the nine months ended September 30, 2023, net cash used in investing activities was \$28.0 million, which was primarily attributable to purchases of investments of \$45.8 million, purchases of secured corporate notes of \$25.4 million, and purchases of property and equipment of \$0.2 million; partially offset by maturities of investments of \$43.4 million.

Financing Activities

For the nine months ended September 30, 2024, net cash provided by financing activities was \$81.3 million, which was primarily attributable to proceeds from the issuance of debt of \$59.0 million and proceeds from the issuance of stock of \$65.8 million, partially offset by repayments of debt of \$42.7 million.

For the nine months ended September 30, 2023, net cash provided by financing activities was \$23.5 million, which was primarily attributable to proceeds from the issuance of debt of \$38.8 million, proceeds from insurance financing programs of \$1.3 million and

proceeds from the Employee Stock Purchase Plan of \$0.2 million; partially offset by repayment of debt of \$12.8 million, payments to repurchase shares as part of the Share Repurchase Program of \$3.4 million, and payments of principal on insurance financing arrangements of \$0.5 million.

Future Funding Requirements

While under applicable accounting principles factors exist that raise substantial doubt about our ability to continue as a going concern, based on our current operating forecast and the net proceeds from the August 2024 Offering (as described above), we believe that our existing cash, cash equivalents and investments will be sufficient to fund our operations into 2027, subject to continuing compliance with our debt covenants. This estimate includes anticipated revenue from MIPLYFFA sales, reimbursements from the EAP, royalties under the AZSTARYS License Agreement, and accounts for continued investments into our development pipeline programs. This estimate does not include potential proceeds from a PRV sale. Certain of the milestones are associated with regulatory matters that are outside our control. In addition, we maintain the majority of our cash and cash equivalents in accounts with major U.S. and multi-national financial institutions, and our deposits at these institutions exceed insured limits. Market conditions can impact the viability of these institutions. In the event of a failure of any of the financial institutions where we maintain our cash and cash equivalents, there can be no assurance that we would be able to access uninsured funds in a timely manner or at all. Any inability to access or delay in accessing these funds could adversely affect our business and financial position.

Potential near-term sources of additional funding include:

- any royalties or net sales milestone payments generated under the AZSTARYS License Agreement;
- any product sales under the EAP;
- any product sales of OLPRUVA;
- any product sales of MIPLYFFA; and
- any consulting services revenue generated under other potential consulting agreements;

We cannot guarantee that we will be able to generate sufficient proceeds from any of these potential sources to fund our operating expenses. We anticipate that our expenses will fluctuate substantially as we:

- continue our ongoing clinical trials and our product development activities for our pipeline of product candidates;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- continue research and development and clinical trials of our product candidates;
- seek to discover and develop additional product candidates either internally or in partnership with other pharmaceutical companies;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio; and
- incur additional legal, accounting and other expenses in operating as a public company.

To date, we have generated revenue from the AZSTARYS License Agreement, reimbursements of out-of-pocket third-party costs, the performance of consulting services, OLPRUVA product sales, and product sales under the EAP. We expect that, for the foreseeable future, our only sources of revenues will be through payments arising from the AZSTARYS License Agreement, product sales of OLPRUVA and MIPLYFFA, through potential consulting arrangements and any other future arrangements related to one of our product candidates and product sales under the EAP. While we have entered into the AZSTARYS License Agreement to develop, manufacture and commercialize AZSTARYS, we cannot guarantee that this, or any strategy we adopt in the future, will be successful. For instance, we received milestone payments under the AZSTARYS License Agreement, but we cannot guarantee that we will earn any additional milestone or royalty payments under this agreement in the future. We also cannot guarantee that we will continue to generate revenue under the EAP or successfully commercialize OLPRUVA or MILEYFFA. We also expect to continue to incur additional costs associated with operating as a public company.

We have based our estimates of our cash needs and cash runway on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect and we cannot guarantee that we will be able to generate sufficient proceeds from the AZSTARYS License Agreement, product reimbursements under the EAP, product sales of OLPRUVA and MILYFFA, potential consulting arrangements or other funding transactions to fund our operating expenses. To meet any additional cash requirements, we may seek to sell additional equity or convertible securities that may result in dilution to our stockholders, issue additional debt or seek other third-party funding, including potential strategic transactions, such as licensing or collaboration arrangements. Because of the numerous risks and uncertainties associated with the development and commercialization of product candidates and products, we are unable to estimate the amounts of increased capital outlays and operating expenditures necessary to complete the commercialization and development of our partnered product or product candidates, should they obtain regulatory approval.

Critical Accounting Estimates

This management's discussion and analysis of our financial condition and results of operations is based on our unaudited condensed consolidated financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States. The preparation of our unaudited condensed consolidated financial statements requires us to make estimates that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of our unaudited condensed consolidated financial statements, as well as the reported revenues and expenses during the reported periods. We evaluate these estimates on an ongoing basis. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions.

Our critical accounting policies have not changed materially from those described in *Part II, Item 7 – Management's Discussion and Analysis of Financial Condition and Results of Operations* of our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, filed with the SEC on April 1, 2024.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Not applicable.

ITEM 4. CONTROLS AND PROCEDURES

Limitations on Effectiveness of Controls and Procedures

In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our chief executive officer and our chief financial officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2024. Based on the evaluation of our disclosure controls and procedures as of September 30, 2024, our chief executive officer and our chief financial officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control Over Financial Reporting

There was no change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) identified in connection with the evaluation required by Rules 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during our fiscal quarter ended September 30, 2024, that materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

From time to time, we may be involved in routine legal proceedings, as well as demands, claims and threatened litigation, which arise in the normal course of our business. In connection with the AZSTARYS License Agreement with Commave, a dispute has arisen with Commave concerning the interpretation of certain provisions under the AZSTARYS License Agreement. On September 4, 2024, Commave filed a complaint against Zevra in the Court of Chancery of the State of Delaware (Case No. 2024-0920-LWW) alleging breach of contract and seeking injunctive relief, specific performance, declaratory relief, and damages regarding the parties' respective rights and obligations under the Agreement.

We strongly disagree with Commave's allegations and believe this lawsuit is without merit. We filed a motion to dismiss the complaint on October 7, 2024. Commave's opposition to the motion to dismiss is due November 21, 2024, and Zevra's reply in support of the motion to dismiss is due December 20, 2024. All discovery in the litigation has been stayed pending resolution of Zevra's motion to dismiss.

The litigation is in its early stages. While we intend to vigorously defend against Commave's claims, the outcome of this matter is inherently uncertain. We cannot predict with certainty the timing or ultimate outcome of this litigation or its potential impact on our business, financial condition, or results of operations. At this time, we have not recorded any accrual for contingent liability associated with this matter.

The AZSTARYS License Agreement remains in effect during this litigation, and both parties continue to perform their respective obligations thereunder. However, there can be no assurance that this dispute will not have an adverse impact on our relationship with Commave or on Zevra's business.

We will continue to monitor developments in this matter and will assess the potential impact on our financial statements in future periods. We expect to incur significant legal expenses in connection with this litigation, which may materially affect our results of operations in future periods.

Other than disclosed herein, we believe there is no litigation pending that would reasonably be expected to, individually or in the aggregate, have a material adverse effect on our results of operations or financial condition.

ITEM 1A. RISK FACTORS

In addition to the other information set forth in this Quarterly Report on Form 10-Q, you should carefully consider all the risk factors and uncertainties described in Part I, Item 1A. "Risk Factors" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, filed with the SEC on April 1, 2024, before investing in our common stock. Other than as described below, there have been no material changes to the risk factors described in that report. If any of those risks materialize, our business, financial condition and results of operations could be seriously harmed. This Quarterly Report on Form 10-Q also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements because of the risk factors in our Annual Report on Form 10-K and below, and the other factors described in in this Quarterly Report on Form 10-Q.

If we experience additional material weaknesses or otherwise fail to maintain effective internal control over financial reporting and disclosure controls and procedures, we may not be able to accurately report our financial results or report them in a timely manner, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

Our management is required to report annually on the effectiveness of our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act, or Section 404. The rules governing the standards that must be met for our management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the Company's annual or interim financial statements will not be prevented or detected on a timely basis. We previously identified and disclosed a material weakness in our internal control over financial reporting in our Annual Report on Form 10-K for the year ended December 31, 2023, and our Quarterly Report on Form 10-Q for the three months ended March 31, 2024. This material weakness has since been remediated.

Our failure to certify the effectiveness of our internal control over financial reporting or our disclosure controls and procedures, or the identification of the material weakness, could subject us to regulatory scrutiny and a loss of public confidence, which could have a material adverse effect on our business and our stock price. In the future, we may identify additional material weaknesses or significant deficiencies, and we may not be able to remediate them in time to meet the deadline imposed by the Sarbanes-Oxley Act for compliance with the requirements of Section 404. In addition, we may encounter problems or delays in completing the implementation of any requested improvements and, if and when such a report is required, receiving a favorable attestation report from our independent registered public accounting firm. If we do not maintain adequate financial and management personnel, processes and controls, we may not be able to manage our business effectively or accurately report our financial performance on a timely basis, which could cause a decline in our common stock price and adversely affect our results of operations and financial condition.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

Recent Sales of Unregistered Securities

None.

Purchases of Equity Securities By the Issuer and Affiliated Purchasers

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

Not applicable.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

(a) Disclosure in lieu of reporting on a Current Report on Form 8-K.

None.

(b) Material changes to the procedures by which security holders may recommend nominees to the board of directors.

None.

(c) Insider Trading Arrangements and Policies.

On October 15, 2024, Timothy J. Sangiovanni, Senior Vice President, Finance and Corporate Controller, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense of Rule 10b5-1(c) for the sale of up to 3,000 shares of the Company's common stock until April 15, 2025.

On October 15, 2024, Sven Guenther, Ph.D., Chief Scientific Officer, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense of Rule 10b5-1(c) for the sale of up to 8,000 shares of the Company's common stock until April 15, 2025.

On October 15, 2024, Joshua Schafer, Chief Commercial Officer & Executive Vice President, Business Development, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense of Rule 10b5-1(c) for the sale of up to 10,500 shares of the Company's common stock until April 15, 2025.

On October 15, 2024, Christal M.M. Mickle, Chief Development Officer, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense of Rule 10b5-1(c) for the sale of up to 6,300 shares of the Company's common stock until April 15, 2025.

On October 15, 2024, R. LaDuane Clifton, Chief Financial Officer and Treasurer, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense of Rule 10b5-1(c) for the sale of up to 11,000 shares of the Company's common stock until April 15, 2025.

On October 15, 2024, Neil F. McFarlane, Chief Executive Officer, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense of Rule 10b5-1(c) for the sale of up to 91,817 shares of the Company's common stock until April 15, 2025.

Other than as disclosed above, during the three months ended September 30, 2024, no director or officer of the Company adopted or terminated a “Rule 10b5-1 trading arrangement” or “non-Rule 10b5-1 trading arrangement,” as each term is defined in Item 408(a) of Regulation S-K.

ITEM 6. EXHIBITS

The following is a list of exhibits filed as part of this Form 10-Q (the SEC file number for all items incorporated by reference herein from reports on Forms 10-K, 10-Q, and 8-K is 001-36913):

Exhibit No.	Description
3.1	Amended and Restated Certificate of Incorporation of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on April 21, 2015).
3.1.1	Certificate of Amendment of Amended and Restated Certificate of Incorporation of the Registrant, effective as of December 23, 2020 (incorporated herein by reference to Registrant's Current Report on Form 8-K as filed with the SEC on December 23, 2020).
3.1.2	Certificate of Amendment of Amended and Restated Certificate of Incorporation of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 24, 2023).
3.2	Amended and Restated Bylaws, as currently in effect, of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 28, 2024).
4.1	Specimen stock certificate evidencing shares of Common Stock (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 12, 2021).
31.1*	Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as amended.
31.2*	Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as amended.
32.1**	Certification of the Principal Executive Officer pursuant to Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, and 18. U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2**	Certification of the Principal Financial Officer pursuant to Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, and 18. U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS*	Inline XBRL Instance Document
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104**	Cover page Interactive Data File (embedded within the Inline XBRL and combined in Exhibit 101)

* Filed herewith

** Furnished herewith

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Zevra Therapeutics, Inc.

Date: November 13, 2024

By: /s/ Neil F. McFarlane
Neil F. McFarlane
President and Chief Executive Officer
(Principal Executive Officer)

Date: November 13, 2024

By: /s/ R. LaDuane Clifton
R. LaDuane Clifton, MBA, CPA
Chief Financial Officer and Treasurer
(Principal Financial Officer)

CERTIFICATION

I, Neil F. McFarlane, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Zevra Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

November 13, 2024

/s/ Neil F. McFarlane

Name: Neil F. McFarlane

Title: President and Chief Executive Officer
(Principal Executive Officer)

CERTIFICATION

I, R. LaDuane Clifton, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Zevra Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

November 13, 2024

/s/ R. LaDuane Clifton

Name: R. LaDuane Clifton, MBA, CPA

Title: Chief Financial Officer and Treasurer
(Principal Financial Officer)

**CERTIFICATION OF THE PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report on Form 10-Q of Zevra Therapeutics, Inc., (the "Company") for the quarterly period ended September 30, 2024, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Neil F. McFarlane, Principal Executive Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

November 13, 2024

/s/ Neil F. McFarlane

Name: Neil F. McFarlane

Title: President and Chief Executive Officer
(Principal Executive Officer)

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350, is not being "filed" by the Company as part of the Report or as a separate disclosure document and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.

**CERTIFICATION OF THE PRINCIPAL FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report on Form 10-Q of Zevra Therapeutics, Inc., (the "Company") for the quarterly period ended September 30, 2024, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, R. LaDuane Clifton, Principal Financial Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

November 13, 2024

/s/ R. LaDuane Clifton

Name: R. LaDuane Clifton, MBA, CPA

Title: Chief Financial Officer and Treasurer
(Principal Financial Officer)

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350, is not being "filed" by the Company as part of the Report or as a separate disclosure document and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.

**Document And Entity
Information - shares**

**9 Months Ended
Sep. 30, 2024**

Nov. 13, 2024

Document Information [Line Items]

<u>Entity Central Index Key</u>	0001434647	
<u>Entity Registrant Name</u>	ZEVRA THERAPEUTICS, INC.	
<u>Amendment Flag</u>	false	
<u>Current Fiscal Year End Date</u>	--12-31	
<u>Document Fiscal Period Focus</u>	Q3	
<u>Document Fiscal Year Focus</u>	2024	
<u>Document Type</u>	10-Q	
<u>Document Quarterly Report</u>	true	
<u>Document Period End Date</u>	Sep. 30, 2024	
<u>Document Transition Report</u>	false	
<u>Entity File Number</u>	001-36913	
<u>Entity Incorporation, State or Country Code</u>	DE	
<u>Entity Tax Identification Number</u>	20-5894398	
<u>Entity Address, Address Line One</u>	1180 Celebration Boulevard, Suite 103	
<u>Entity Address, City or Town</u>	Celebration	
<u>Entity Address, State or Province</u>	FL	
<u>Entity Address, Postal Zip Code</u>	34747	
<u>City Area Code</u>	321	
<u>Local Phone Number</u>	939-3416	
<u>Title of 12(b) Security</u>	Common Stock, \$0.0001 par value per share	
<u>Trading Symbol</u>	ZVRA	
<u>Security Exchange Name</u>	NASDAQ	
<u>Entity Current Reporting Status</u>	Yes	
<u>Entity Interactive Data Current</u>	Yes	
<u>Entity Filer Category</u>	Non-accelerated Filer	
<u>Entity Small Business</u>	true	
<u>Entity Emerging Growth Company</u>	false	
<u>Entity Shell Company</u>	false	
<u>Entity Common Stock, Shares Outstanding</u>		53,375,932

**Unaudited Condensed
Consolidated Balance Sheets**
- USD (\$)
\$ in Thousands

**Sep. 30, Dec. 31,
2024 2023**

Assets

Cash and cash equivalents	\$ 54,039	\$ 43,049
Securities at fair value, current	35,337	24,688
Accounts and other receivables	7,790	17,377
Prepaid expenses and other current assets	2,276	1,824
Total current assets	99,442	86,938
Inventories	8,756	9,841
Securities at fair value, long-term	6,105	0
Property and equipment, net	607	736
Operating lease right-of-use assets	820	790
Goodwill acquired	4,701	4,701
Intangible assets, net	70,608	69,227
Other long-term assets	512	94
Total assets	191,551	172,327

Liabilities and stockholders' equity

Accounts payable and accrued expenses	24,464	28,403
Line of credit payable	0	37,700
Current portion of operating lease liabilities	540	543
Current portion of discount and rebate liabilities	8,547	4,550
Other current liabilities	930	2,524
Total current liabilities	34,481	73,720
Long-term debt	58,904	5,066
Warrant liability	13,902	16,100
Operating lease liabilities, less current portion	483	456
Discount and rebate liabilities, less current portion	8,490	7,663
Other long-term liabilities	5,521	7,458
Total liabilities	121,781	110,463

Commitments and contingencies (Note D)

Stockholders' equity:

Common stock, \$0.0001 par value, 250,000,000 shares authorized, 54,803,056 shares issued and 53,227,364 shares outstanding as of September 30, 2024; 43,110,360 shares issued and 41,534,668 shares outstanding as of December 31, 2023	5	4
Additional paid-in capital	550,413	472,664
Treasury stock, at cost	(10,983)	(10,983)
Accumulated deficit	(469,550)	(399,778)
Accumulated other comprehensive income (loss)	(115)	(43)
Total stockholders' equity	69,770	61,864
Total liabilities and stockholders' equity	191,551	172,327
Undesignated Preferred Stock [Member]		

Stockholders' equity:

<u>Undesignated preferred stock, \$0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of September 30, 2024, or December 31, 2023</u>	\$ 0	\$ 0
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**Unaudited Condensed
Consolidated Balance Sheets**

Sep. 30, 2024 Dec. 31, 2023

(Parentheticals) - \$ / shares

<u>Preferred stock, shares authorized (in shares)</u>	10,000,000	10,000,000
<u>Common stock, par value (in dollars per share)</u>	\$ 0.0001	\$ 0.0001
<u>Common stock, shares authorized (in shares)</u>	250,000,000	250,000,000
<u>Common stock, shares issued (in shares)</u>	54,803,056	43,110,360
<u>Common stock, shares outstanding (in shares)</u>	53,227,364	41,534,668
<u>Undesignated Preferred Stock [Member]</u>		
<u>Preferred stock, par value (in dollars per share)</u>	\$ 0.0001	\$ 0.0001
<u>Preferred stock, shares authorized (in shares)</u>	10,000,000	10,000,000
<u>Preferred stock, shares issued (in shares)</u>	0	0
<u>Preferred stock, shares outstanding (in shares)</u>	0	0

Unaudited Condensed Consolidated Statements of Operations - USD (\$) \$ in Thousands	3 Months Ended		9 Months Ended	
	Sep. 30, 2024	Sep. 30, 2023	Sep. 30, 2024	Sep. 30, 2023
<u>Revenue, net</u>	\$ 3,695	\$ 2,895	\$ 11,569	\$ 14,541
<u>Cost of product revenue (excluding \$1,545 and \$4,619 in intangible asset amortization for the three and nine months ended September 30, 2024, respectively shown separately below)</u>	2,303	144	6,051	946
<u>Intangible asset amortization</u>	1,545	0	4,619	0
<u>Operating expenses:</u>				
<u>Research and development</u>	10,945	12,297	33,743	28,385
<u>Selling, general and administrative</u>	16,208	5,818	38,743	19,657
<u>Total operating expenses</u>	27,153	18,115	72,486	48,042
<u>Loss from operations</u>	(27,306)	(15,364)	(71,587)	(34,447)
<u>Other (expense) income:</u>				
<u>Interest expense</u>	(2,312)	(366)	(5,157)	(745)
<u>Fair value adjustment related to warrant and CVR liability</u>	(4,746)	3,678	4,660	4,253
<u>Fair value adjustment related to investments</u>	90	124	64	451
<u>Interest and other income (expense), net</u>	1,049	1,738	2,248	4,331
<u>Total other (expense) income</u>	(5,919)	5,174	1,815	8,290
<u>Loss before income taxes</u>	(33,225)	(10,190)	(69,772)	(26,157)
<u>Income tax expense</u>	0	(177)	0	0
<u>Net loss</u>	\$ (33,225)	\$ (10,367)	\$ (69,772)	\$ (26,157)
<u>Basic and diluted net loss per share of common stock:</u>				
<u>Net loss (in dollars per share)</u>	\$ (0.69)	\$ (0.3)	\$ (1.59)	\$ (0.76)
<u>Weighted average number of shares of common stock outstanding:</u>				
<u>Basic and diluted (in shares)</u>	47,808,817	34,724,614	43,843,851	34,364,075

Unaudited Condensed Consolidated Statements of Operations (Parentheticals) - USD (\$) \$ in Thousands	3 Months Ended		9 Months Ended	
	Sep. 30, 2024	Sep. 30, 2023	Sep. 30, 2024	Sep. 30, 2023
Amortization of intangible assets	\$ 1,545	\$ 0	\$ 4,619	\$ 0

Unaudited Condensed Consolidated Statements of Comprehensive Loss - USD (\$) \$ in Thousands	3 Months Ended		9 Months Ended	
	Sep. 30, 2024	Sep. 30, 2023	Sep. 30, 2024	Sep. 30, 2023
<u>Net loss</u>	\$ (33,225)	\$ (10,367)	\$ (69,772)	\$ (26,157)
<u>Other comprehensive (loss) income:</u>				
<u>Foreign currency translation adjustment</u>	(536)	5	(72)	(333)
<u>Other comprehensive (loss) income</u>	(536)	5	(72)	(333)
<u>Comprehensive loss</u>	\$ (33,761)	\$ (10,362)	\$ (69,844)	\$ (26,490)

Unaudited Condensed Consolidated Statements of Changes in Stockholders' Equity - USD (\$) \$ in Thousands	Previously Reported [Member] Common Stock [Member]	Previously Reported [Member] Additional Paid-in Capital [Member]	Previously Reported [Member] Treasury Stock, Common [Member]	Previously Reported [Member] Retained Earnings [Member]	Previously Reported [Member] AOCI Attributable to Parent [Member]	Previously Reported [Member]	Common Stock [Member]	Additional Paid-in Capital [Member]	Treasury Stock, Common [Member]	Retained Earnings [Member]	AOCI Attributable to Parent [Member]	Total
Balance at Dec. 31, 2022	\$ 3	\$ 436,269	\$ (7,536)	\$ (353,729)	\$ 113	\$ 75,120						
Net Income (loss)							\$ 0	\$ 0	\$ 0	\$ (13,217)	\$ 0	\$ (13,217)
Stock-based compensation expense							0	945	0	0	0	945
Issuance of common stock in exchange for consulting services							0	42	0	0	0	42
Other comprehensive income							0	0	0	0	(176)	(176)
Net loss							0	0	0	(13,217)	0	(13,217)
Balance at Mar. 31, 2023	3	437,256	(10,983)	(366,946)	(63)	59,267						
Shares repurchased as part of the Share Repurchase Program							0	0	(3,447)	0	0	(3,447)
Balance at Dec. 31, 2022	3	436,269	(7,536)	(353,729)	113	75,120						
Net Income (loss)												(26,157)
Other comprehensive income												(333)
Net loss												(26,157)
Balance at Sep. 30, 2023							3	452,608	(10,983)	(379,886)	(220)	61,522
Balance at Mar. 31, 2023							3	437,256	(10,983)	(366,946)	(63)	59,267
Net Income (loss)							0	0	0	(2,573)	0	(2,573)
Stock-based compensation expense							0	2,151	0	0	0	2,151
Issuance of common stock in exchange for consulting services							0	25	0	0	0	25
Issuance of common stock as part of the Employee Stock Purchase Plan							0	165	0	0	0	165
Other comprehensive income							0	0	0	0	(162)	(162)
Net loss							0	0	0	(2,573)	0	(2,573)
Balance at Jun. 30, 2023							3	439,597	(10,983)	(369,519)	(225)	58,873
Net Income (loss)							0	0	0	(10,367)	0	(10,367)
Stock-based compensation expense							0	1,440	0	0	0	1,440
Issuance of common stock in exchange for consulting services							0	71	0	0	0	71
Other comprehensive income							0	0	0	0	5	5
Net loss							0	0	0	(10,367)	0	(10,367)
Balance at Sep. 30, 2023							3	452,608	(10,983)	(379,886)	(220)	61,522
Issuance of common stock in connection with the Merger (Note L)							0	11,500	0	0	0	11,500
Balance at Dec. 31, 2023	4	472,664	(10,983)	(399,778)	(43)	61,864						
Net Income (loss)							0	0	0	(16,622)	0	(16,622)
Stock-based compensation expense							0	2,119	0	0	0	2,119
Issuance of common stock in exchange for consulting services							0	56	0	0	0	56
Issuance of common stock as part of the Employee Stock Purchase Plan							0	71	0	0	0	71
Issuance of common stock for options exercised							0	1,146	0	0	0	1,146
Other comprehensive income							0	0	0	0	184	184
Net loss							0	0	0	(16,622)	0	(16,622)
Balance at Mar. 31, 2024							4	476,056	(10,983)	(416,400)	141	48,818
Balance at Dec. 31, 2023	\$ 4	\$ 472,664	\$ (10,983)	\$ (399,778)	\$ (43)	\$ 61,864						
												61,864

Net Income (loss)						(69,772)
Other comprehensive income						(72)
Net loss						(69,772)
Balance at Sep. 30, 2024	5	550,413	(10,983)	(469,550)	(115)	69,770
Balance at Mar. 31, 2024	4	476,056	(10,983)	(416,400)	141	48,818
Net Income (loss)	0	0	0	(19,925)	0	(19,925)
Stock-based compensation expense	0	2,632	0	0	0	2,632
Issuance of common stock in exchange for consulting services	0	193	0	0	0	193
Issuance of common stock as part of the Employee Stock Purchase Plan	0	480	0	0	0	480
Other comprehensive income	0	0	0	0	280	280
Net loss	0	0	0	(19,925)	0	(19,925)
Balance at Jun. 30, 2024	4	479,361	(10,983)	(436,325)	421	32,478
Net Income (loss)	0	0	0	(33,225)	0	(33,225)
Stock-based compensation expense	0	6,137	0	0	0	6,137
Issuance of common stock in exchange for consulting services	0	150	0	0	0	150
Issuance of common stock as part of the Employee Stock Purchase Plan	0	92	0	0	0	92
Issuance of common stock for options exercised	0	157	0	0	0	157
Other comprehensive income	0	0	0	0	(536)	(536)
Net loss	0	0	0	(33,225)	0	(33,225)
Issuance of common stock in public offering	1	64,516	0	0	0	64,517
Balance at Sep. 30, 2024	\$ 5	\$ 550,413	\$ (10,983)	\$ (469,550)	\$ (115)	\$ 69,770

**Unaudited Condensed
Consolidated Statements of
Cash Flows - USD (\$)
\$ in Thousands**

9 Months Ended
Sep. 30, 2024 Sep. 30, 2023

Cash flows from operating activities:

Net loss	\$ (69,772)	\$ (26,157)
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Adjustments to reconcile net loss to net cash used in operating activities:

Stock-based compensation expense	10,888	4,482
Inventory obsolescence charge	5,244	0
Non-cash interest expense	1,489	0
Depreciation and amortization expense	4,741	219
Fair value adjustment related to warrant and CVR liability	(4,660)	(4,159)
Fair value adjustment related to investments	(64)	(451)
Loss on sublease and disposal of property and equipment	0	157
Consulting fees paid in common stock	399	138
Loss (gain) on foreign currency exchange	147	(30)

Change in assets and liabilities:

Accounts and other receivables	9,587	(1,628)
Prepaid expenses and other assets	(452)	27
Inventories	(4,159)	190
Operating lease right-of-use assets	389	243
Other long-term assets	0	(93)
Accounts payable and accrued expenses	(10,590)	5,400
Discount and rebate liability	4,824	3,895
Operating lease liabilities	(395)	(326)
Other liabilities	(1,031)	716
Net cash used in operating activities	(53,415)	(17,377)

Cash flows from investing activities:

Purchases of property and equipment	0	(224)
Purchases of investments	(41,488)	(45,821)
Purchases of secured corporate notes	0	(25,426)
Maturities of investments	24,798	43,496
Net cash used in investing activities	(16,690)	(27,975)

Cash flows from financing activities:

Proceeds from issuance of debt, net of lender fees	58,990	38,801
Payment of third-party debt issuance costs	(2,091)	0
Repayment of debt	(42,700)	(12,800)
Proceeds from insurance financing arrangements	1,082	1,256
Proceeds from Employee Stock Purchase Plan	643	219
Payments of principal on insurance financing arrangements	(431)	(564)
Payments to repurchase shares as part of the Share Repurchase Program	0	(3,447)
Proceeds from issuance of stock	65,819	0
Repayment of principal on finance lease liabilities	0	(5)
Net cash provided by financing activities	81,312	23,460

<u>Effect of exchange rate changes on cash and cash equivalents</u>	(217)	(305)
<u>Net increase (decrease) in cash and cash equivalents</u>	10,990	(22,197)
<u>Cash and cash equivalents, beginning of period</u>	43,049	65,466
<u>Cash and cash equivalents, end of period</u>	54,039	43,269
<u>Supplemental cash flow information:</u>		
<u>Cash paid for interest</u>	\$ 3,668	\$ 456

**Note A - Description of
Business, Basis of
Presentation and Significant
Transactions**

9 Months Ended

Sep. 30, 2024

**Notes to Financial
Statements**

**Business Description and
Basis of Presentation [Text
Block]**

A. Description of Business, Basis of Presentation, and Significant Transactions

Organization

Zevra Therapeutics, Inc. (the "Company") is a commercial-stage company focused on addressing unmet needs for the treatment of rare diseases. The Company has a diverse portfolio of products and product candidates, which includes clinical stage pipeline and commercial stage assets. On September 20, 2024, the U.S. Food and Drug Administration ("FDA") approved the New Drug Application ("NDA") for MIPLYFFA™ (arimoclomol), an orally-delivered treatment for Niemann-Pick disease type C ("NPC"), which is an ultra-rare and progressive neurodegenerative disease. MIPLYFFA, the first FDA-approved treatment for NPC, is indicated for use in combination with miglustat for the treatment of neurological manifestations of NPC in adult and pediatric patients two years of age and older. In addition, the Company received a transferable rare pediatric disease priority review voucher in conjunction with the approval of MIPLYFFA. MIPLYFFA has also been granted orphan medicinal product designation for the treatment of NPC by the European Commission. OLPRUVA® (sodium phenylbutyrate) for oral suspension is approved by the FDA for the treatment of urea cycle disorders ("UCDs"). The Company also has a pipeline of investigational product candidates, including celiprolol for the treatment of vascular Ehlers-Danlos syndrome in patients with a confirmed type III collagen mutation. KP1077 is the Company's clinical development product candidate which is being developed as a treatment for idiopathic hypersomnia ("IH"), a rare neurological sleep disorder, and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate ("SDX"), the Company's proprietary prodrug of d-methylphenidate ("d-MPH"). The FDA has granted KP1077 orphan drug designation for the treatment of IH.

The Company changed its name from KemPharm, Inc. to Zevra Therapeutics, Inc. effective as of February 21, 2023. On March 1, 2023, following its name change, the Company's common stock began trading on the Nasdaq Global Select Market under the ticker symbol "ZVRA".

Going Concern

The accompanying unaudited condensed consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business. During the nine months ended September 30, 2024, and 2023, the Company incurred a net loss of \$69.8 million and \$26.2 million, respectively, and as of September 30, 2024, has an accumulated deficit of \$469.6 million. The Company has sustained operating losses for the majority of its corporate history and expects to continue to incur operating losses and negative operating cash flows until revenues reach a level sufficient to support ongoing operations.

The Company's ability to continue operating as a going concern is contingent upon its ability to generate revenue from approved products or obtain product candidate regulatory approvals, which would generate revenue, milestones, and cash flow sufficient to support ongoing operations and the satisfaction of financial covenants. These factors raise substantial doubt as to the Company's ability to continue as a going concern for at least one year from the date the unaudited condensed consolidated financial statements are being issued. The unaudited condensed consolidated financial statements do not include any adjustments that might result from the outcome of these uncertainties.

The Company's liquidity needs will be largely determined by the success of operations through the progression of its products and product candidates in the future. The Company also may consider other sources to fund operations including: (1) out-licensing rights to certain of its technologies and product candidates, pursuant to which the Company would receive cash royalties and milestones; (2) raising additional capital through equity or debt financings or from other sources; (3) obtaining product candidate regulatory approvals, which would generate revenue, milestones and cash flow; (4) reducing spending on one or more research and development programs, including by discontinuing development; and/or (5) restructuring operations to change its overhead structure. The Company is in the early stages of its commercialization effort for OLPRUVA and MIPLYFFA and does not yet have a substantial basis to project future earnings, and its other sources of revenue are not sufficient to sustain its present activities on their own. Accordingly, the Company's ability to continue as a going concern may require it to obtain additional financing to fund its operations.

Basis of Presentation

The Company prepared the unaudited condensed consolidated financial statements in accordance with U.S. GAAP and the rules and regulations of the Securities and Exchange Commission ("SEC") and, in the Company's opinion, reflect all adjustments, including normal recurring items that are necessary.

Merger

On August 30, 2023, the Company and Aspen Z Merger Sub, Inc., a wholly-owned subsidiary of Zevra ("Merger Sub"), entered into an Agreement and Plan of Merger (the "Merger Agreement") with Acer Therapeutics Inc. ("Acer"), a pharmaceutical company focused on development and commercialization of therapies for rare and life-threatening diseases. On November 17, 2023 (the "Closing Date"), the Company completed the acquisition of Acer. Pursuant to the Merger Agreement, on the Closing Date, Merger Sub was merged with and into Acer (the "Merger"), with Acer continuing as the surviving entity and as a wholly-owned subsidiary of Zevra. In connection therewith, Zevra also purchased Acer's secured debt from Nantahala Capital Management, LLC ("NCM"), certain of its affiliates and certain other parties (collectively with NCM, "Nantahala") through a series of transactions and Zevra agreed to provide Acer with a bridge loan facility for up to \$18.0 million ("Bridge Loan"), subject to certain terms and conditions. The Merger expanded Zevra's rare disease portfolio, as well as increased and diversified its revenues with the addition of a U.S. commercial asset, OLPRUVA, indicated for the treatment of UCDs. See Note L for further discussion related to the Merger.

Registration Statements on Form S-3

In connection with the Merger, Zevra and Nantahala concurrently entered into a registration rights agreement, pursuant to which Zevra agreed to file a resale registration statement with respect to the resale of the Zevra common stock issuable to Nantahala. On February 5, 2024, Zevra filed a registration statement on Form S-3 (File No. 333-276856) registering an aggregate of 2,269,721 shares of Zevra's common stock. On April 5, 2024, we filed an amendment to the registration statement on Form S-3 (File No. 333-250945) covering the issuance of the shares of our common stock issuable upon the exercise of warrants issued in connection with the Merger (Note L) and remaining unexercised as of the date of the amendment, which was declared effective on April 8, 2024.

On June 4, 2024, the Company filed a registration statement on Form S-3 (File No. 333-279941) (the "June 2024 Registration Statement") under which the Company may sell securities, including as may be issuable upon conversion, redemption, repurchase, exchange or exercise of securities, in one or more offerings up to a total aggregate offering price of \$350.0 million, \$75.0 million of which was allocated to the sale of the shares of common stock issuable under the 2024 ATM Agreement (as described further below). The registration statement was declared effective on June 13, 2024.

August 2024 Offering

On August 8, 2024, the Company entered into an underwriting agreement (the “Underwriting Agreement”) with Cantor Fitzgerald & Co. and William Blair & Company, L.L.C., as representatives of the several underwriters named therein (collectively, the “Underwriters”), in connection with the offering, issuance and sale by the Company of 9,230,770 shares of the Company’s common stock at a public offering price of \$6.50 per share, pursuant to the June 2024 Registration Statement and a related prospectus supplement dated August 8, 2024 filed with the SEC (the “August 2024 Offering”). Under the terms of the Underwriting Agreement, the Company also granted the Underwriters an option exercisable for 30 days to purchase up to an additional 1,384,615 shares of its Common Stock at the public offering price, less underwriting discounts and commissions, which the Underwriters exercised in full on August 9, 2024. The August 2024 Offering closed on August 12, 2024. Total shares issued were 10,615,385. Net proceeds from the offering were approximately \$64.5 million, after deducting underwriting discounts and commissions and estimated offering expenses payable by the Company. The Company intends to use the net proceeds of the offering to support the commercial launch activities for MIPLYFFA, continued commercial support for OLPRUVA and the continued development of celiprolol and KP1077 through potential NDA filings and other general corporate purposes.

Based on the planned use of proceeds, the Company believes that the net proceeds from the August 2024 Offering and its existing cash and cash equivalents will be sufficient to fund its operating expenses and capital expenditure requirements into 2027, subject to continuing compliance with the Company's debt covenants.

Entry into 2024 ATM Agreement

On July 12, 2024, the Company entered into an equity distribution agreement (the “2024 ATM Agreement”) with Citizens JMP Securities LLC (“Citizens JMP”) under which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock having an aggregate offering price of up to \$75.0 million through Citizens JMP as its sales agent. The issuance and sale, if any, of common stock by the Company under the 2024 ATM Agreement will be made pursuant to the June 2024 Registration Statement, the accompanying prospectus, and the related prospectus supplement dated July 12, 2024. Citizens JMP may sell the common stock by any method permitted by law deemed to be an “at the market offering” as defined in Rule 415 of the Securities Act. Citizens JMP will use commercially reasonable efforts to sell the common stock from time to time, based upon instructions from the Company (including any price, time or size limits or other customary parameters or conditions the Company may impose). The Company will pay Citizens JMP a commission equal to 3.0% in the aggregate of the gross sales proceeds of any common stock sold through Citizens JMP under the 2024 ATM Agreement. As of September 30, 2024, no shares have been issued or sold under the 2024 ATM Agreement.

Termination of 2021 ATM Agreement

On July 2, 2021, the Company entered into an equity distribution agreement (the “2021 ATM Agreement”) with JMP Securities LLC (“JMP”) and RBC Capital Markets, LLC (“RBCCM”) under which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock having an aggregate offering price of up to \$75.0 million through JMP and RBCCM as its sales agents. The issuance and sale, if any, of common stock by the Company under the 2021 ATM Agreement will be made pursuant to the registration statement on Form S-3 (File No. 333-257661) which was declared effective on July 12, 2021, the accompanying prospectus, and the related prospectus supplement dated July 12, 2021. JMP and RBCCM may sell the common stock by any method permitted by law deemed to be an “at the market offering” as defined in Rule 415 of the Securities Act. JMP and RBCCM will use commercially reasonable efforts to sell the common stock from time to time, based upon instructions from the Company (including any price, time or size limits or other customary parameters or conditions the Company may impose). The Company will pay JMP and RBCCM a commission equal to 3.0% in the aggregate of the gross sales proceeds of any common stock sold through JMP and RBCCM under the 2021 ATM Agreement. The 2021 ATM Agreement terminated on July 11, 2024, and no shares had been issued or sold under the 2021 ATM Agreement as of the termination date.

Share Repurchase Program

On December 20, 2021, the Company initiated a share repurchase program (the "Share Repurchase Program") pursuant to which the Company was able to repurchase up to \$50 million of shares of its common stock through December 31, 2023. On December 31, 2023, the Share Repurchase Program ended, and the Company had repurchased 1,575,692 shares of its common stock for approximately \$11.0 million.

**Note B - Summary of
Significant Accounting
Policies**

9 Months Ended

Sep. 30, 2024

[Notes to Financial](#)

[Statements](#)

[Significant Accounting](#)

[Policies \[Text Block\]](#)

B. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires the Company to make estimates and assumptions that affect the amounts reported in the unaudited condensed consolidated financial statements and accompanying notes. Actual results could differ from those estimates.

On an ongoing basis, the Company evaluates its estimates and assumptions, including those related to revenue recognition, the useful lives of property and equipment, the recoverability of long-lived assets, the incremental borrowing rate for leases, and assumptions used for purposes of determining stock-based compensation, income taxes, the fair value of investments and the fair value of the warrant liabilities, contingent value right ("CVR") liabilities and discount and rebate liabilities, among others. The Company bases its estimates on historical experience and on various other assumptions that it believes to be reasonable, the results of which form the basis for making judgments about the carrying value of assets and liabilities.

Investments

The Company maintains investment securities that are classified as available-for-sale securities for which the Company has elected the fair value option under Accounting Standards Codification ("ASC") 825, *Financial Instruments* ("ASC 825"). As such, these securities are carried at fair value with unrealized gains and losses included in fair value adjustment related to investments on the unaudited condensed consolidated statements of operations. The securities primarily consist of U.S. Treasury securities and corporate bonds and are included in securities at fair value in the unaudited condensed consolidated balance sheets. The Company's investments in corporate bonds mature within one to five years. As of September 30, 2024, and December 31, 2023, the Company held securities with an aggregate fair value of \$41.4 million and \$24.7 million, respectively, that contained aggregate unrealized gains of approximately \$0.1 million and \$0.6 million, respectively. Applying fair value accounting to these debt securities more accurately represents the Company's investment strategy due to the fact that excess cash is currently being invested for the purpose of funding future operations. Interest income is recognized as earned using an effective yield method giving effect to the amortization of premium and accretion of discount and is based on the economic life of the securities. Interest income is included in interest and other income, net in the unaudited condensed consolidated statements of operations.

Variable Interest Entities

The primary beneficiary of a variable interest entity ("VIE") is required to consolidate the assets and liabilities of the VIE. When the Company obtains a variable interest in another entity, it assesses at the inception of the relationship and upon occurrence of certain significant events whether the entity is a VIE, and if so, whether the Company is the primary beneficiary of the VIE based on its power to direct the activities of the VIE that most significantly impact the VIE's economic performance and the Company's obligation to absorb losses or the rights to receive benefits from the VIE that could potentially be significant to the VIE.

To assess whether the Company has the power to direct the activities of the VIE that most significantly impact the VIE's economic performance, the Company considers all the facts and circumstances, including the Company's role in establishing the VIE and the Company's ongoing rights and responsibilities. The assessment includes identifying the activities that most

significantly impact the VIE's economic performance and identifying which party, if any, has the power to direct those activities. In general, the parties that make the most significant decisions affecting the VIE (management and representation on the Board of Directors) are deemed to have the power to direct the activities of a VIE.

To assess whether the Company has the obligation to absorb losses of the VIE or the rights to receive benefits from the VIE that could potentially be significant to the VIE, the Company considers all of its economic interests that are deemed to be variable interests in the VIE.

This assessment requires judgement in determining whether these interests, in the aggregate, are considered potentially significant to the VIE. As of September 30, 2024, and December 31, 2023, the Company identified Acer to be the Company's sole interest in a VIE (Note L). As Zevra is the final decision maker for all of Acer's research, development, and commercialization of drug candidates that it is producing, the Company directs the activities of Acer that most significantly impact its performance. Therefore, the Company is the primary beneficiary of this VIE for accounting purposes.

Revenue Recognition

The Company recognizes revenue in accordance with the provisions of ASC 606, *Revenue from Contracts with Customers* ("ASC 606") and, as a result, follows the five-step model when recognizing revenue: 1) identifying a contract; 2) identifying the performance obligations; 3) determining the transaction price; 4) allocating the price to performance obligations; and 5) recognizing revenue when the performance obligations have been fulfilled.

Licensing Agreements

The terms of the Company's licensing agreements typically include one or more of the following: (i) upfront fees; (ii) milestone payments related to the achievement of development, regulatory, or commercial goals; and (iii) royalties on net sales of licensed products. Each of these payments may result in licensing revenues.

As part of the accounting for these agreements, the Company must develop estimates and assumptions that require judgment to determine the underlying stand-alone selling price for each performance obligation which determines how the transaction price is allocated among the performance obligations. Generally, the estimation of the stand-alone selling price may include such estimates as independent evidence of market price, forecasted revenues or costs, development timelines, discount rates, and probability of regulatory success. The Company evaluates each performance obligation to determine if they can be satisfied at a point in time or over time, and it measures the services delivered to the licensee which are periodically reviewed based on the progress of the related program. The effect of any change made to an estimated input component and, therefore revenue or expense recognized, would be recorded as a change in estimate. In addition, variable consideration (e.g., milestone payments) must be evaluated to determine if it is constrained and, therefore, excluded from the transaction price.

Up-front Fees: If a license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenues from the transaction price allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time.

Milestone Payments: At the inception of each arrangement that includes milestone payments (variable consideration), the Company evaluates whether the milestones are considered probable of being reached and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's or the licensee's control, such as non-operational developmental and

regulatory approvals, are generally not considered probable of being achieved until those approvals are received. At the end of each reporting period, the Company re-evaluates the probability of achievement of milestones that are within its or the licensee's control, such as operational developmental milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenues and earnings in the period of adjustment. Revisions to the Company's estimate of the transaction price may also result in negative licensing revenues and earnings in the period of adjustment.

Product Revenues, Net

Net revenues from product sales is recognized at the transaction price when the customer obtains control of the Company's products, which occurs at a point in time, typically upon receipt of the product by the customer. Our current single customer is a specialty pharmacy provider.

The Company's net revenues represent total revenues adjusted for discounts and allowances, including estimated cash discounts, chargebacks, rebates, returns, copay assistance, data fees and wholesaler fees for services. These adjustments represent variable consideration under ASC 606 and are recorded as a reduction of revenue. These adjustments are established by management as its best estimate based on available information and will be adjusted to reflect known changes in the factors that impact such allowances. Adjustments for variable consideration are determined based on the contractual terms with customers, historical trends, communications with customers and the levels of inventory remaining in the distribution channel, as well as expectations about the market for the product and anticipated introduction of competitive products.

Acquired IPR&D and Milestones Expenses

In an asset acquisition, payments incurred prior to regulatory approval to acquire rights to in-process R&D projects are expensed as acquired IPR&D and milestones expense in the consolidated statements of earnings unless the project has an alternative future use. These costs include upfront and development milestone payments related to R&D collaborations, licensing arrangements, or other asset acquisitions that provide rights to develop, manufacture and/or sell pharmaceutical products. Where contingent development milestone payments are due to third parties, prior to regulatory approval, the payment obligations are expensed when the milestone results are achieved. Regulatory and commercial milestone payments made to third parties subsequent to regulatory approval are capitalized as intangible assets and amortized to cost of products sold over the remaining useful life of the related product.

Inventories

The value of inventory is recorded at its net realizable value. The Company determines the cost of its other inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis.

The Company may scale-up and make commercial quantities of its product candidates prior to the date it anticipates that such product will receive final regulatory approval. The scale-up and commercial production of pre-launch inventory involves the risk that such products may not be approved for marketing on a timely basis, or ever. This risk notwithstanding, the Company may scale-up and build pre-launch inventory of product that have not received final regulatory approval when the Company believes such action is appropriate in relation to the commercial value of the product launch opportunity. Inventory manufactured prior to regulatory approval is recorded as research and development expense until regulatory approval for the product is obtained. Inventory used in clinical trials is also expensed as research and development expense, when selected for such use. Inventory that can be used in either the production of clinical or commercial products is expensed as research and development costs when identified for use in a clinical manufacturing campaign. The cost of finished goods inventory that is shipped to a customer to support the Company's patient assistance programs is expensed when those shipments take place. As of September 30, 2024, and December 31, 2023, the Company did not have pre-launch inventory that qualified for capitalization.

The Company performs an assessment of the recoverability of capitalized inventory during each reporting period and writes down any excess and obsolete inventory to its net realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded as a component of cost of product revenue in the statements of operations and comprehensive loss. The determination of whether inventory costs will be realizable requires the use of estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required. Additionally, the Company's product is subject to strict quality control and monitoring that it performs throughout the manufacturing process. In the event that certain batches or units of product do not meet quality specifications, the Company will record a charge to cost of product revenue, to write-down any unsaleable inventory to its estimated net realizable value. For the three and nine months ended September 30, 2024, the Company recognized a charge of approximately \$2.0 million and \$5.2 million, respectively, related to write-downs for unsaleable inventory. No such write downs were recognized for the three and nine months ended September 30, 2023.

Foreign currency

Assets and liabilities are translated into the reporting currency using the exchange rates in effect on the unaudited condensed consolidated balance sheet dates. Equity accounts are translated at historical rates, except for the change in accumulated deficit during the year, which is the result of the income statement translation process. Revenue and expense accounts are translated using the weighted average exchange rate during the period. The cumulative translation adjustments associated with the net assets of foreign subsidiaries are recorded in accumulated other comprehensive income/loss in the accompanying unaudited condensed consolidated statements of stockholders' equity.

Debt Issuance Costs

Debt issuance costs incurred in connection with financing arrangements are recorded as a reduction of the related debt on the balance sheet and amortized over the life of the respective financing arrangement using the effective interest method.

Warrants

The Company accounts for warrants as either equity-classified or liability-classified instruments based on an assessment of the warrant's specific terms and applicable authoritative guidance in FASB ASC Topic 480, *Distinguishing Liabilities from Equity* ("ASC 480") and FASB ASC Topic 815, *Derivatives and Hedging* ("ASC 815"). The assessment considers whether the warrants are freestanding financial instruments pursuant to ASC 480, meet the definition of a liability pursuant to ASC 480, and whether the warrants meet all of the requirements for equity classification under ASC 815, including whether the warrants are indexed to our own stock and whether the warrant holders could potentially require "net cash settlement" in a circumstance outside of the company's control, among other conditions for equity classification. This assessment, which requires the use of professional judgment, is conducted at the time of warrant issuance and as of each subsequent quarterly period end date while the warrants are outstanding.

For warrants that meet all criteria for equity classification, the warrants are required to be recorded as a component of additional paid-in capital, on the unaudited condensed consolidated statement of stockholders' deficit at the time of issuance. For warrants that do not meet all the criteria for equity classification, the warrants are required to be recorded at their initial fair value on the date of issuance, and on each balance sheet date thereafter. Changes in the estimated fair value of the warrants are recognized as a non-cash gain or loss in other expense, net, on the unaudited condensed consolidated statement of operations. The fair value of the warrants was estimated using the Black-Scholes option pricing model.

New Accounting Pronouncements Not Yet Adopted

Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures

In November 2023, the FASB issued Accounting Standards Update ("ASU") No. 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*, which modifies the disclosure and presentation requirements of reportable segments. The amendments in the update require the disclosure of significant segment expenses that are regularly provided to the chief operating decision maker ("CODM") and included within each reported measure of segment profit and loss. The amendments also require disclosure of all other segment items by reportable segment and a description of its composition. Additionally, the amendments require disclosure of the title and position of the CODM and an explanation of how the CODM uses the reported measure(s) of segment profit or loss in assessing segment performance and deciding how to allocate resources. This update is effective for annual periods beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024. Early adoption is permitted. The Company is currently evaluating the impact that this guidance will have on the presentation of its consolidated financial statements and accompanying notes.

Income Taxes (Topic 740): Improvements to Income Tax Disclosures

In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*, which expands disclosures in an entity's income tax rate reconciliation table and disclosures regarding cash taxes paid both in the U.S. and foreign jurisdictions. The update will be effective for annual periods beginning after December 15, 2024. The Company is currently evaluating the impact that this guidance will have on the presentation of its consolidated financial statements and accompanying notes.

Note C - Inventories

**9 Months Ended
Sep. 30, 2024**

[Notes to Financial Statements](#)

[Inventory Disclosure \[Text Block\]](#) C.

Inventories

	September 30, 2024	December 31, 2023
Raw materials	\$ 4,549	\$ 2,938
Work in progress	3,084	1,884
Finished goods	1,123	5,019
Total inventory	<u>\$ 8,756</u>	<u>\$ 9,841</u>

Note D - Debt Obligations

9 Months Ended
Sep. 30, 2024

[Notes to Financial
Statements](#)

[Debt Disclosure \[Text Block\]](#)

D. Debt Obligations

Long-term debt consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Notes payable	\$ 61,035	\$ —
Secured promissory note	—	5,000
Unamortized original issue (discount) premium	(959)	148
Less: debt issuance costs	(1,172)	(82)
	<u>\$ 58,904</u>	<u>\$ 5,066</u>

Secured Promissory Note

In connection with the Merger (Note L), on August 30, 2023, the Company and Nantahala, entered into a secured promissory note payable by Zevra to Nantahala in the original principal amount of \$5.0 million (the "Nantahala Note"). The Nantahala Note initially bore interest at 9.0% per annum, payable quarterly in arrears in cash. The interest rate increased to 12.0% per annum effective March 1, 2024, as the Nantahala Note remained unpaid six months from its issue date. The additional 3.0% interest would have been paid in shares of Zevra's common stock based on the volume weighted average trading price ("VWAP") of Zevra's common stock during the twenty consecutive trading days ending on the date before such interest payment date. Beginning on the first interest payment date following the second anniversary of the Nantahala Note, and on each interest payment date thereafter, Zevra was required to make \$0.6 million amortization payments on the Nantahala Note until it was paid in full. All principal and unpaid interest on the Nantahala Note would have been due on August 30, 2026, the third anniversary of the Nantahala Note. Zevra was entitled to prepay the Nantahala Note at any time without penalty. The Nantahala Note was secured by Zevra's interest in (i) the loan assets under the Loan Purchase Agreement described in Note L; (ii) the note assets under the Note Purchase Agreement described in Note L; (iii) the Bridge Loan described in Note L; and (iv) the proceeds therefrom. The Company used the proceeds from the Nantahala Note, along with \$12.0 million in cash and 98,683 shares of Zevra's common stock, to acquire Acer's term loans, as more fully described in Note L. In April 2024, the Nantahala Note was repaid in full and terminated. At the time of repayment, Nantahala elected to receive a cash payment in lieu of shares of Zevra's common stock in exchange for the additional 3.0% interest accrued for the period from March 1, 2024, through April 5, 2024.

Line of Credit

On January 26, 2023, the Company and Wells Fargo, as lender, entered into a revolving margin account agreement. The Company's investments were used as collateral for the loan and the amount the Company was able to borrow was limited to 80-90% of its outstanding investment balance held with Wells Fargo. The margin account bore interest at the Prime rate minus 225 basis-points. As of December 31, 2023, \$37.7 million was outstanding under the margin account. In April 2024, the Company repaid the outstanding balance under the margin account with Wells Fargo, and upon such repayment, the margin capabilities were removed from the account.

Term Loans

On April 5, 2024 (the "Term Loans Closing Date"), the Company entered into a credit agreement (the "Credit Agreement") with HCR Stafford Fund II, L.P., HCR Potomac Fund II, L.P., and

Perceptive Credit Holdings IV, LP (collectively, the “Lenders”), and Alter Domus (US) LLC, as administrative agent (the “Administrative Agent”).

Under the terms of the Credit Agreement, the Lenders provided a senior secured loan facility to the Company in the aggregate principal amount of \$100.0 million, which is divided into three tranches as follows: (i) \$60.0 million which was funded in full on the Term Loans Closing Date; (ii) \$20.0 million which is available to the Company in up to two drawings, each in an amount not to exceed \$10.0 million, at the Company’s option until 18 months following the Term Loans Closing Date; and (iii) \$20.0 million which is available to the Company upon approval by the FDA of the NDA for MIPLYFFA for the treatment of NPC, at the Company’s option until December 31, 2024 (collectively, the “Term Loans”).

The principal amount of the Term Loans outstanding (the “Outstanding Principal Amount”) will bear interest at a rate equal to 3-Month Term SOFR *plus* 7.00% per annum. If the net product sales for the calendar year ending December 31, 2025, exceed \$100.0 million, the Outstanding Principal Amount will bear interest at 3-Month Term SOFR *plus* 6.00% per annum. If the net product sales for the calendar year ending December 31, 2025, do not exceed \$100.0 million, then for any subsequent period of four consecutive fiscal quarters ending on or after March 31, 2026, in which net product sales exceed \$125.0 million, the Outstanding Principal Amount will bear interest at 3-Month Term SOFR *plus* 6.50% per annum. In all cases, the 3-Month Term SOFR rate will be subject to a floor of 4.00% per annum. Interest will be payable quarterly in arrears on the last day of each calendar quarter. The Company has the option to pay up to 25% of the interest in-kind beginning on the Term Loans Closing Date, through and including June 30, 2026. The Company has recognized approximately \$0.9 million of interest-in-kind as of September 30, 2024, which is included in long-term debt in the unaudited condensed consolidated balance sheet. The Term Loans will mature on the fifth anniversary of the Term Loans Closing Date. In connection with the Credit Agreement, the Company incurred approximately \$2.2 million of costs, which primarily consisted of underwriting, legal and other professional fees, and are included as a reduction to the carrying amount of the related debt liability and are deferred and amortized over the remaining life of the financing using the effective interest method.

The Credit Agreement contains customary affirmative and negative covenants by the Company, which among other things, will require the Company to provide certain financial reports to the Lenders within 60 days after the end of each of the first three fiscal quarters of each fiscal year and 105 days after the end of each fiscal year, meet certain minimum net product sales amounts, and limit the ability of the Company to incur or guarantee additional indebtedness, engage in certain transactions, and effect a consolidation or merger without consent. In addition, as long as the line of credit remains active, the Company must maintain a minimum cash balance of \$20.0 million to ensure the Company can meet its immediate capital needs. The obligations of the Company under the Credit Agreement may be accelerated upon customary events of default, including non-payment of principal, interest, fees and other amounts, covenant defaults, insolvency, material judgments, or inaccuracy of representations and warranties. The Term Loans are secured by a first priority perfected lien on, and security interest in, substantially all current and future assets of the Company. The proceeds of the Term Loans were used to refinance certain existing indebtedness of the Company and its subsidiaries. The Company will use the remaining proceeds to pay fees and expenses related to the debt financing and fund the development and commercialization of OLPRUVA and MIPLYFFA.

Future minimum principal payments under the Term Loans as of September 30, 2024, were as follows (in thousands):

Year Ending December 31,	
2024 (excluding the nine months ending September 30, 2024)	\$ —
2025	—
2026	—
2027	—
2028	—

Thereafter	60,919
Total minimum payments	60,919
Less: unamortized debt discount, debt issuance costs and paid in kind interest	(2,015)
Note payable	<u>\$ 58,904</u>

Note E - Revenue, Net

**9 Months Ended
Sep. 30, 2024**

Notes to Financial Statements

Revenue from Contract with Customer [Text Block]

E. Revenue, net

Licensing Agreements

AZSTARYS License Agreement

The Company entered into a Collaboration and License Agreement (the "AZSTARYS License Agreement") with Commave Therapeutics SA (formerly known as Boston Pharmaceutical S.A.) ("Commave"), an affiliate of Gurnet Point Capital, L.P., dated September 3, 2019. Under the AZSTARYS License Agreement, as amended, the Company granted to Commave an exclusive, worldwide license to develop, manufacture and commercialize the Company's product candidates containing SDX and d-MPH, including AZSTARYS, or any other product candidates containing SDX and developed to treat ADHD or any other central nervous system disorder. Corium Inc. was tasked by Commave to lead all commercialization activities for AZSTARYS under the AZSTARYS License Agreement. Pursuant to the AZSTARYS License Agreement, Commave agreed to pay milestone payments up to an aggregate of \$590.0 million upon the occurrence of specified regulatory milestones related to AZSTARYS, additional fixed payments upon the achievement of specified U.S. sales milestones, and quarterly, tiered royalty payments based on a range of percentages of net sales (as defined in the AZSTARYS License Agreement). Commave is obligated to make such royalty payments on a product-by-product basis until expiration of the royalty term for the applicable product.

The Company concluded that these regulatory milestones, sales milestones and royalty payments each contain a significant uncertainty associated with a future event. As such, these milestone and royalty payments are constrained at contract inception and are not included in the transaction price as the Company could not conclude that it is probable a significant reversal in the amount of cumulative revenue recognized will not occur surrounding these milestone payments. At the end of each reporting period, the Company updates its assessment of whether the milestone and royalty payments are constrained by considering both the likelihood and magnitude of the potential revenue reversal. For the three and nine months ended September 30, 2024, the Company recognized \$1.1 million and \$3.6 million of revenue under the AZSTARYS License Agreement, respectively, primarily related to royalties. For the three and nine months ended September 30, 2023, the Company recognized revenue under the AZSTARYS License Agreement of \$0.9 million and \$7.2 million, respectively, which includes recognition of a \$5.0 million net sales milestone that was met in June 2023. There was no deferred revenue related to this agreement as of September 30, 2024, or December 31, 2023.

In accordance with the terms of the Company's Termination Agreement with Aquestive Therapeutics ("Aquestive") dated March 20, 2012, Aquestive has the right to receive an amount equal to 10% of any royalty or milestone payments made to the Company related to AZSTARYS or KP1077 under the AZSTARYS License Agreement.

The AZSTARYS License Agreement is within the scope of ASC 606, as the transaction represents a contract with a customer where the participants function in a customer / vendor relationship and are not exposed equally to the risks and rewards of the activities contemplated under the AZSTARYS License Agreement.

Relief Exclusive License Agreement

As a condition to entering into the Merger Agreement, Acer and Relief Therapeutics SA ("Relief") entered into an exclusive license agreement on August 30, 2023 (the "Relief License Agreement"). Pursuant to the Relief License Agreement, Relief will hold exclusive development and commercialization rights for OLPRUVA in the European Union, Liechtenstein, San Marino, Vatican City, Norway, Iceland, Principality of Monaco, Andorra, Gibraltar, Switzerland, United Kingdom, Albania, Bosnia, Kosovo, Montenegro, Serbia and North Macedonia ("Geographical Europe"). The Company will have the right to receive a royalty of up to 10% of the net sales of OLPRUVA in Geographical Europe. For the three and nine months ended September 30, 2024, the Company did not recognize any revenue under the Relief License Agreement. There was no deferred revenue related to this agreement as of September 30, 2024, and December 31, 2023. For further discussion of the Relief License Agreement, see Note L.

Product Revenues, Net

Expanded Access Program

Net revenue includes revenue from the sale of arimoclomol for the treatment of NPC under the remunerated expanded access compassionate use program in France ("French nATU"). An expanded access compassionate use program is a program giving specific patients access to a drug, which is not yet approved for commercial sale. Only drugs targeting serious or rare indications and for which there is currently no appropriate treatment are considered for expanded access compassionate use programs. Further, to be considered for the expanded access compassionate use program, the drug must have proven efficacy and safety and must either be undergoing price negotiations or seeking marketing approval.

In accordance with ASC 606, the Company recognizes revenue when fulfilling its performance obligation under the Expanded Access Program ("EAP") by transferring control of promised goods or services to its customer, in an amount that reflects the consideration that the Company expects to receive in exchange for those goods or services. In determining when the customer obtains control of the product, the Company considers certain indicators, including whether the Company has a present right to payment from the customer, whether title and/or significant risks and rewards of ownership have transferred to the customer and whether the customer acceptance has been received. Revenue is recognized net of sales deductions, including discounts, rebates, applicable distributor fees, and revenue-based taxes.

The French Health Authorities and the manufacturer have agreed to a price for sales during the French nATU, but the final transaction price depends on the terms and conditions in the contracts with the French Health Authorities and is subject to price negotiations with the French Health Authorities following market approval. Any excess in the price charged the manufacturer compared to the price agreed with the health authorities once the drug product is approved in France must be repaid. The repayment is considered in the clawback liability (rebate). An estimate of net revenue and clawback liability are recognized using the 'expected value' method. Accounting for net revenue and clawback liability requires determination of the most appropriate method for the expected final transaction price. This estimate also requires assumptions with respect to inputs into the method, including current pricing of comparable marketed products within the rare disease area in France. Management has considered the expected final sales price as well as the price of similar drug products. The Company is operating within a rare disease therapeutic area where there is unmet treatment need and hence a limited number of comparable commercialized drugs products. The limited available relevant market information for directly comparable commercialized drugs within rare disease increases the uncertainty in management's estimate.

For the three and nine months ended September 30, 2024, the Company recognized revenue related to the EAP in France of \$2.6 million and \$8.0 million, respectively, which is net of a clawback liability of \$1.4 million and \$4.4 million, respectively, and other gross to net adjustments. For the three and nine months ended September 30, 2023, the Company recognized revenue related to the EAP in France of \$2.0 million and \$7.1 million, respectively, which is net of a clawback liability of \$1.1 million and \$4.0 million, respectively, and other gross to net adjustments. The total estimated reserve liability as of September 30, 2024, and December 31, 2023, was \$17.0 million and \$12.2

million, respectively. As of September 30, 2024, and December 31, 2023, this estimated reserve liability is recorded as discount and rebate liabilities in the unaudited condensed consolidated balance sheets and is separated into current and long-term based upon the timing of the expected payment to the French regulators.

Product Sales

On December 27, 2022, the FDA approved OLPRUVA (sodium phenylbutyrate), a prescription medicine used along with certain therapy, including changes in diet, for the long-term management of adults and children with UCDs weighing 44 pounds (20 kg) or greater and with a body surface area of 1.2m² or greater. On November 17, 2023, the Company acquired OLPRUVA in connection with the Merger (Note L). For the three and nine months ended September 30, 2024, sales of OLPRUVA were de minimis.

On September 20, 2024, the NDA for MIPLYFFA, an orally-delivered treatment for NPC, which is an ultra-rare and progressive neurodegenerative disease, was approved. For the three and nine months ended September 30, 2024, sales of MIPLYFFA were de minimis. In May 2011, Orphazyme entered into an asset purchase agreement with LadRx Corporation, which was assigned to XOMA (US) LLC, a wholly-owned subsidiary of XOMA Corporation ("XOMA"), in June 2023 ("XOMA License Agreement"). Under the XOMA License Agreement, XOMA is entitled to certain net sales and regulatory milestone payments in addition to a mid-single digit royalty with respect to sales of MIPLYFFA. For the three and nine months ended September 30, 2024, the Company recognized a regulatory milestone payment of \$6.0 million due to XOMA under the XOMA License Agreement, which is included in intangible assets, net in the unaudited condensed consolidated balance sheet.

The Company currently utilizes a single specialty pharmacy provider as its sole distributor for both OLPRUVA and MIPLYFFA. The Company also enters into arrangements with health care providers and payors that provide for government mandated and/or privately negotiated rebates with respect to the purchase of its products. To commercialize OLPRUVA for oral suspension and MIPLYFFA in the U.S., the Company has built marketing, sales, medical affairs, distribution, managerial and other non-technical capabilities or is making arrangements with third parties to perform these services.

Accounts and Other Receivables

Accounts and other receivables consist of receivables from product sales, receivables under the AZSTARYS License Agreement and EAP, as well as income tax receivables and other receivables due to the Company. Receivables under the AZSTARYS License Agreement are recorded for amounts due to the Company related to reimbursable third-party costs and royalties on product sales. Receivables under the EAP are recorded for product sales under the French nATU. These receivables are evaluated to determine if any reserve or allowance should be established at each reporting date. As of September 30, 2024, the Company had receivables related to the EAP of \$4.4 million, AZSTARYS License Agreement of \$1.1 million, and other receivables of \$2.3 million. As of December 31, 2023, the Company had receivables related to the EAP of \$4.7 million, AZSTARYS License Agreement of \$11.4 million, and other receivables of \$1.3 million. As of September 30, 2024, and December 31, 2023, no reserve or allowance for doubtful accounts had been established.

**Note F - Commitments and
Contingencies**

**9 Months Ended
Sep. 30, 2024**

**Notes to Financial
Statements**

**Commitments and
Contingencies Disclosure**

[Text Block]

F. Commitments and Contingencies

From time to time, the Company is involved in various legal proceedings arising in the normal course of business. For some matters, a liability is not probable, or the amount cannot be reasonably estimated and, therefore, an accrual has not been made. However, for such matters when it is probable that the Company has incurred a liability and can reasonably estimate the amount, the Company accrues and discloses such estimates.

Stockholder Litigation Related to the Merger

On October 12, 2023, Brodsky & Smith, purporting to act as counsel for Jerry Beavee, who was asserted to be a stockholder of Acer, filed a complaint entitled Jerry Beavee v. Acer Therapeutics Inc., et al., No. 1:23-cv-08995 in the United States District Court for the Southern District of New York (the "Action") alleging that defendants violated Section 14(a) and 20(a) of the Securities Exchange Act of 1934 by filing the Preliminary Merger Registration Statement which allegedly omitted certain information that such counsel asserts is material to Acer's required disclosure. On October 30, 2023, Acer filed with the SEC a Schedule DEF14A that contained additional information regarding the Merger, which mooted the disclosure claims alleged in the Action. On December 8, 2023, Jerry Beavee filed with the court a notice of dismissal of the Action without prejudice.

On October 20, 2023, Long Law, LLC and Acocelli Law, PLLC, purporting to act as counsel for Kevin Turner, who was asserted to be a stockholder of Acer, filed a complaint entitled Kevin Turner v. Acer Therapeutics Inc., et al., No. 1:23-cv-01185 in the United States District Court for the District of Delaware alleging that defendants violated Section 14(a) and 20(a) of the Securities Exchange Act of 1934 as well as SEC Rule 14a-9 by filing the Definitive Proxy Statement which allegedly omitted certain information that such counsel asserts is material to Acer's required disclosure. The complaint prays that, if asserted omissions are not adequately corrected, then Turner will seek to enjoin Acer from holding a stockholder meeting to approve the Merger and, if the Merger closes, will seek to rescind it and seek an award of damages.

On October 20, 2023, Long Law, LLC, purporting to act as counsel for Matthew Jones, who was asserted to be a stockholder of Acer, filed a complaint entitled Matthew Jones v. Acer Therapeutics Inc., et al., No. 1:23-cv-01186 in the United States District Court for the District of Delaware alleging that defendants violated Section 14(a) and 20(a) of the Securities Exchange Act of 1934 as well as SEC Rule 14a-9 by filing the Definitive Proxy Statement which allegedly omitted certain information that such counsel asserts is material to the Acer's required disclosure. The complaint prays that, if asserted omissions are not adequately corrected, then Jones will seek to enjoin Acer from holding a stockholder meeting to approve the Merger and, if the Merger closes, will seek to rescind it and seek an award of damages.

On June 19, 2024, the Company entered into a confidential fee agreement (the "Fee Agreement") to settle the above stockholder litigation matters related to the Merger. The parties to the Fee Agreement were entitled to an aggregate payment of approximately \$0.3 million, which was paid during the second quarter of 2024.

Litigation Related to the AZSTARYS License Agreement

In September 2024, the Company became engaged in a legal dispute regarding the AZSTARYS License Agreement. The litigation is in its early stages. We cannot predict with certainty the timing or ultimate outcome of this litigation or its potential impact on our business, financial condition, or results of operations. At this time, we have not recorded any accrual for contingent liability

associated with this matter. The AZSTARYS License Agreement remains in effect during this litigation, and both parties continue to perform their respective obligations thereunder. However, there can be no assurance that this dispute will not have an adverse impact on our relationship with Commave or on the Company's business. The Company will continue to monitor developments in this matter and will assess the potential impact on our financial statements in future periods. The Company expects to incur significant legal expenses in connection with this litigation, which may materially affect its results of operations in future periods.

As of September 30, 2024, and December 31, 2023, no accruals were made related to commitments and contingencies.

Note G - Stock and Warrants

**9 Months Ended
Sep. 30, 2024**

[Notes to Financial Statements](#)

[Equity \[Text Block\]](#)

G. Stock and Warrants

Authorized, Issued, and Outstanding Common Shares

As of September 30, 2024, and December 31, 2023, the Company had authorized shares of common stock of 250,000,000 shares. Of the authorized shares, 54,803,056 and 43,110,360 shares of common stock were issued as of September 30, 2024, and December 31, 2023, respectively, and 53,227,364 and 41,534,668 shares of common stock were outstanding as of September 30, 2024, and December 31, 2023, respectively.

As of September 30, 2024, and December 31, 2023, the Company had reserved authorized shares of common stock for future issuance as follows:

	September 30, 2024	December 31, 2023
Outstanding awards under equity incentive plans	8,987,997	8,023,142
Outstanding common stock warrants	5,483,537	5,603,729
Possible future issuances under equity incentive plans	4,536,410	1,728,885
Possible future issuances under employee stock purchase plans	1,242,425	1,340,172
Total common shares reserved for future issuance	<u>20,250,369</u>	<u>16,695,928</u>

Common Stock Activity

The following table summarizes common stock activity for the nine months ended September 30, 2024:

	Shares of Common Stock
Balance as of January 1, 2024	41,534,668
Common stock issued as compensation to third parties	9,000
Common stock issued as a result of stock options exercised	306,826
Balance as of March 31, 2024	41,850,494
Common stock issued as a result of the Employee Stock Purchase Plan	97,747
Common stock issued as compensation to third parties	41,820
Common stock issued as a result of stock options exercised	1,403
Balance as of June 30, 2024	41,991,464
Common stock issued as compensation to third parties	32,820
Common stock issued in connection with the Public Offering (Note B)	10,615,385

Common stock issued as a result of stock options exercised	39,750
Common stock issued in connection with vesting of performance-based awards	547,945
Balance as of September 30, 2024	<u>53,227,364</u>

Authorized, Issued, and Outstanding Preferred Stock

As of September 30, 2024, and December 31, 2023, the Company had 10,000,000 shares of authorized preferred stock, none of which were designated, issued, or outstanding.

Warrants to Purchase Common Stock

The Company has issued warrants to purchase common stock to various third parties, of which 5,483,537 remain outstanding as of September 30, 2024, and are immediately exercisable. These warrants qualify as participating securities under ASC Topic 260, *Earnings per Share*, and are treated as such in the net loss per share calculation (Note J). The Company may be required to redeem these warrants for a cash amount equal to the Black-Scholes value of the portion of the warrants to be redeemed (the "Put Option").

In connection with the Merger (Note L), in November 2023, the Company directly issued to certain investors an aggregate of 1,382,489 shares of its common stock, par value \$0.0001 per share, and accompanying warrants to purchase up to 1,382,489 shares of its common stock (the "2023 Warrants") at a combined offering price of \$4.34 per share of common stock and the Warrants and an aggregate of 917,934 shares of its common stock in exchange for the cancellation of a warrant to purchase 2,920,306 shares of common stock of Acer. The Warrants are immediately exercisable and expire on November 22, 2028. The Company used the net proceeds of approximately \$6.0 million from the offering for general corporate purposes. These warrants are separately exercisable by the warrant holders. While the warrants are outstanding (but unexercised), the warrant holders will participate in any dividend or other distribution of the Company's assets to its common stockholders by way of return of capital or otherwise. As of September 30, 2024, and December 31, 2023, none of the warrants have been exercised. The warrants have been evaluated to determine the appropriate accounting and classification pursuant to ASC 480 and ASC 815. Generally, freestanding warrants should be classified as (i) liabilities if the warrant terms allow settlement of the warrant exercise in cash and (ii) equity if the warrant terms only allow settlement in shares of common stock.

The Company determined that its outstanding warrants and the Put Option should be recorded as a liability and stated at fair value at each reporting period. Changes to the fair value of the warrant liability are recorded through the unaudited condensed consolidated statements of operations as a fair value adjustment related to warrant and CVR liability. As of September 30, 2024, and December 31, 2023, the fair value of the liability associated with these warrants and the Put Option was approximately \$13.9 million and \$16.1 million, respectively. The fair value adjustment related to these warrants and the Put Option was approximately \$6.0 million of loss and \$2.2 million of income for the three and nine months ended September 30, 2024, respectively. The fair value adjustment related to these warrants and the Put Option was approximately \$3.7 million and \$4.3 million of income for the three and nine months ended September 30, 2023, respectively.

Note H - Stock-based Compensation

**9 Months Ended
Sep. 30, 2024**

[Notes to Financial Statements](#)

[Share-Based Payment Arrangement \[Text Block\]](#)

H. Stock-Based Compensation

The Company maintains a stock-based compensation plan (the "Incentive Stock Plan") that governs stock awards made to employees and directors prior to completion of the IPO.

In November 2014, the Board of Directors of the Company ("the Board"), and in April 2015, the Company's stockholders, approved the Company's 2014 Equity Incentive Plan (the "2014 Plan"), which became effective in April 2015. The 2014 Plan provides for the grant of stock options, other forms of equity compensation, and performance cash awards. In June 2021, the Company's stockholders approved an Amended and Restated 2014 Equity Incentive Plan (the "A&R 2014 Plan"), following its adoption by the Board in April 2021, which among other things added 4,900,000 shares to the maximum number of shares of common stock to be issued under the plan and extended the annual automatic increases (discussed further below) until January 1, 2031 and eliminated individual grant limits that applied under the 2014 Plan to awards that were intended to comply with the exemption for "performance-based compensation" under Code Section 162(m). The maximum number of shares of common stock that may be issued under the A&R 2014 Plan is 9,932,883 as of September 30, 2024. The number of shares of common stock reserved for issuance under the A&R 2014 Plan will automatically increase on January 1 of each year, until and including January 1, 2031, by 4% of the total number of shares of the Company's capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by the Board. Pursuant to the terms of the 2014 Plan, on January 1, 2024, the common stock reserved for issuance under the 2014 Plan automatically increased by 1,661,386 shares.

During the three and nine months ended September 30, 2024, 27,750, and 761,500 stock options were exercised, respectively. During the three and nine months ended September 30, 2023, no stock options were exercised.

In June 2021, the Company's stockholders approved an Employee Stock Purchase Plan (the "ESPP"), following its adoption by the Board in April 2021. The maximum number of shares of common stock that may be issued under the ESPP is 1,500,000. The first offering period under the ESPP began on October 1, 2021, and the first purchase date occurred on May 31, 2022. As of September 30, 2024, 257,575 shares have been issued under the ESPP.

In January 2023, the Board approved the 2023 Employment Inducement Award Plan (the "2023 Plan"). The maximum number of shares of common stock that were initially available for issuance under the 2023 Plan was 1,500,000. In February 2024, the Board approved an amendment to the 2023 Plan to increase the aggregate number of shares of common stock available for issuance under the 2023 Plan from 1,500,000 to 4,500,000 shares.

In May 2023, the Board approved the Ninth Amended and Restated Non-Employee Director Compensation Policy (the "Non-Employee Director Compensation Policy"). The equity compensation made pursuant to the Non-Employee Director Compensation Policy will be granted under the A&R 2014 Plan.

Stock-based compensation expense recorded under the Incentive Stock Plan, A&R 2014 Plan, ESPP and 2023 Plan is included in the following line items in the accompanying unaudited condensed consolidated statements of operations (in thousands):

Three months ended September 30,	Nine months ended September 30,
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	2024	2023	2024	2023
Research and development	\$ 1,441	\$ 720	\$ 3,322	\$ 2,023
Selling, general and administrative	4,696	667	7,566	1,058
Total stock-based compensation expense	\$ 6,137	\$ 1,387	\$ 10,888	\$ 3,081

During the three and nine months ended September 30, 2024, the Company recognized approximately \$2.5 million in stock-based compensation expense related to performance-based awards. There was no stock-based compensation expense related to performance-based awards recognized during the nine months ended September 30, 2023.

**Note I - Fair Value of
Financial Instruments**

**9 Months Ended
Sep. 30, 2024**

**Notes to Financial
Statements**

**Fair Value Disclosures [Text
Block]**

I. Fair Value of Financial Instruments

The accounting standard for fair value measurements provides a framework for measuring fair value and requires disclosures regarding fair value measurements. Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date, based on the Company's principal or, in absence of a principal, most advantageous market for the specific asset or liability.

The Company uses a three-tier fair value hierarchy to classify and disclose all assets and liabilities measured at fair value on a recurring basis, as well as assets and liabilities measured at fair value on a non-recurring basis, in periods subsequent to their initial measurement. The hierarchy requires the Company to use observable inputs when available, and to minimize the use of unobservable inputs, when determining fair value. The three tiers are defined as follows:

- Level 1—Observable inputs that reflect quoted market prices (unadjusted) for identical assets or liabilities in active markets;
- Level 2—Observable inputs other than quoted prices in active markets that are observable either directly or indirectly in the marketplace for identical or similar assets and liabilities; and
- Level 3—Unobservable inputs that are supported by little or no market data, which require the Company to develop its own assumptions.

The carrying amounts of certain financial instruments, including cash and cash equivalents, investments, and accounts payable and accrued expenses, approximate their respective fair values due to the short-term nature of such instruments.

Assets and Liabilities Measured at Fair Value on a Recurring Basis

The Company evaluates its financial assets and liabilities subject to fair value measurements on a recurring basis to determine the appropriate level in which to classify them for each reporting period. This determination requires significant judgments to be made. The following table summarizes the conclusions reached regarding fair value measurements as of September 30, 2024, and December 31, 2023 (in thousands):

	Balance as of September 30, 2024	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
CVR liability (Note L)	\$ 4,800	\$ —	\$ —	\$ 4,800
Warrant liabilities	13,902	—	—	13,902
Total liabilities	<u>\$ 18,702</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 18,702</u>
Securities:				
U.S. Treasury securities	\$ 35,337	\$ 35,337	\$ —	\$ —
Corporate bonds	6,105	—	6,105	—
Total assets	<u>\$ 41,442</u>	<u>\$ 35,337</u>	<u>\$ 6,105</u>	<u>\$ —</u>

	Balance as of December 31, 2023	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
CVR liability (Note L)	\$ 7,262	\$ —	\$ —	\$ 7,262
Warrant liabilities	16,100	—	—	16,100
Total liabilities	\$ 23,362	\$ —	\$ —	\$ 23,362
Securities:				
U.S. Treasury securities	\$ 24,688	\$ 24,688	\$ —	\$ —
Total assets	\$ 24,688	\$ 24,688	\$ —	\$ —

The common stock warrant liabilities were recorded at fair value using the Black-Scholes option pricing model. The following assumptions were used in determining the fair value of the warrant liabilities valued using the Black-Scholes option pricing model as of September 30, 2024, and December 31, 2023:

	September 30, 2024	December 31, 2023
Risk-free interest rate	3.51% - 3.81%	3.76% - 4.12%
Volatility	61.76% - 82.44%	62.01% - 92.42%
Dividend yield	0%	0%
Expected term (years)	1.27 - 4.14	2.0 - 4.9
Weighted average fair value	\$ 2.54	\$ 2.94

The following table is a reconciliation for the common stock warrant liabilities measured at fair value using Level 3 unobservable inputs (in thousands):

Balance as of December 31, 2023	\$ 16,100
Change in fair value measurement	(2,198)
Balance as of September 30, 2024	\$ 13,902

For the nine months ended September 30, 2024, and 2023, the changes in fair value of the warrant liabilities primarily resulted from the volatility of the Company's common stock and the change in the risk-free interest rates.

Note J - Net Loss Per Share

**9 Months Ended
Sep. 30, 2024**

[Notes to Financial Statements](#)

[Earnings Per Share \[Text Block\]](#)

J. Net Loss Per Share

For all periods presented herein, the Company did not use the two-class method to compute net loss per share of common stock, even though it had issued securities, other than common stock, that contractually entitled the holders to participate in dividends and earnings, because these holders are not obligated to participate in a loss. The two-class method requires earnings for the period to be allocated between common stock and participating securities based upon their respective rights to receive distributed and undistributed earnings.

Under the two-class method, for periods with net income, basic net income per share of common stock is computed by dividing the undistributed net income by the weighted average number of shares of common stock outstanding during the period. Undistributed net income is computed by subtracting from net income the portion of current period earnings that participating securities would have been entitled to receive pursuant to their dividend rights had all of the period's earnings been distributed and subtracting the actual or deemed dividends declared. No such adjustment to earnings is made during periods with a net loss as the holders of the participating securities have no obligation to fund losses. Diluted net income per share of common stock is computed under the two-class method by using the weighted average number of shares of common stock outstanding plus the potential dilutive effects of stock options, warrants and other outstanding convertible securities. In addition to analyzing under the two-class method, the Company analyzes the potential dilutive effect of stock options and warrants, under the treasury-stock method and other outstanding convertible securities under the if-converted method when calculating diluted income (loss) per share of common stock, in which it is assumed that the stock options, warrants and other outstanding convertible securities convert into common stock at the beginning of the period or date of issuance, if the stock option, warrant or other outstanding convertible security was issued during the period. The Company reports the more dilutive of the approaches (two-class or treasury-stock/if-converted) as its diluted net income (loss) of common stock during the period.

As noted above, for all periods presented herein, the Company did not utilize the two-class approach as the Company was in a net loss position and the holders of the participating securities have no obligation to fund losses. The Company did analyze diluted net loss per share of common stock under the treasury-stock/if-converted method and noted that all outstanding stock options and warrants were anti-dilutive for the periods presented. For all periods presented, basic net loss per share of common stock was the same as diluted net loss per share of common stock.

The following securities, presented on a common stock equivalent basis, have been excluded from the calculation of weighted average number of shares of common stock outstanding because their effect is anti-dilutive:

	Three months ended		Nine months ended	
	September 30,		September 30,	
	2024	2023	2024	2023
Awards under equity incentive plans	8,987,997	7,262,039	8,987,997	7,262,039
Common stock warrants	5,483,537	4,252,490	5,483,537	4,252,490
Total securities excluded from the calculation of weighted average number of shares of common stock outstanding	14,471,534	11,514,529	14,471,534	11,514,529

A reconciliation from net loss to basic and diluted net loss per share of common stock for the three and nine months ended September 30, 2024, and 2023, is as follows (in thousands):

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Basic and diluted net loss per share of common stock:				
Net loss, basic and diluted	\$ (33,225)	\$ (10,367)	\$ (69,772)	\$ (26,157)
Weighted average number of shares of common stock outstanding, basic and diluted	47,809	34,725	43,844	34,364
Basic and diluted net loss per share of common stock	\$ (0.69)	\$ (0.30)	\$ (1.59)	\$ (0.76)

Note K - Leases

**9 Months Ended
Sep. 30, 2024**

[Notes to Financial Statements](#)

[Lease Disclosure \[Text Block\]](#) **K. Leases**

The Company has operating and finance leases for office space, laboratory facilities and various laboratory equipment, furniture and office equipment and leasehold improvements. The Company determines if an arrangement is a lease at contract inception. Lease assets and lease liabilities are recognized based on the present value of lease payments over the lease term at the commencement date. The Company does not separate lease and non-lease components. Leases with a term of twelve (12) months or less at commencement are not recorded on the unaudited condensed consolidated balance sheets. Lease expense for these arrangements is recognized on a straight-line basis over the lease term. The Company's leases have remaining lease terms of less than 1 year to approximately 3 years, some of which include options to extend the leases for up to 5 years, and some which include options to terminate the leases within 1 year.

Effective June 1, 2021, the Company agreed to sublease office space in Florida, comprised of one of the two contiguous suites, under a non-cancelable operating lease, which expires in February 2026.

The components of lease expense were as follows (in thousands):

Lease Cost	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Finance lease cost:				
Amortization of right-of-use assets	\$ 12	\$ 26	\$ 36	\$ 90
Interest on lease liabilities	—	—	—	—
Total finance lease cost	12	26	36	90
Operating lease cost	119	113	330	340
Short-term lease cost	59	55	177	165
Variable lease cost	26	13	39	39
Less: sublease income	(50)	(39)	(128)	(117)
Total lease costs	\$ 166	\$ 168	\$ 454	\$ 517

Supplemental cash flow information related to leases was as follows (in thousands):

	Nine months ended September 30,	
	2024	2023
Cash paid for amounts included in the measurement of lease liabilities:		
Financing cash flows from finance leases	\$ —	\$ 5
Operating cash flows from operating leases	405	426
Operating cash flows from short-term leases	177	165
Operating cash flows from variable lease costs	39	39

Right-of-use assets obtained in exchange for lease liabilities:

Finance leases	\$	—	\$	—
Operating leases		419		—

Supplemental balance sheet information related to leases was as follows (in thousands, except weighted average remaining lease term and weighted average discount rate):

	September 30, 2024	December 31, 2023
Finance Leases		
Property and equipment, at cost	\$ 1,031	\$ 1,031
less: accumulated depreciation and amortization	(917)	(882)
Property and equipment, net	<u>\$ 114</u>	<u>\$ 149</u>
Other current liabilities	\$ —	\$ —
Other long-term liabilities	—	—
Total finance lease liabilities	<u>\$ —</u>	<u>\$ —</u>
Operating Leases		
Operating lease right-of-use assets	\$ 820	\$ 790
Total operating lease right-of-use assets	<u>\$ 820</u>	<u>\$ 790</u>
Current portion of operating lease liabilities	\$ 540	\$ 543
Operating lease liabilities, less current portion	483	456
Total operating lease liabilities	<u>\$ 1,023</u>	<u>\$ 999</u>
Weighted Average Remaining Lease Term		
Operating leases (in years)	2	2
Weighted Average Discount Rate		
Finance leases	0.0%	14.3%
Operating leases	9.6%	7.6%

Maturities of lease liabilities were as follows (in thousands):

Year Ending December 31,	Finance Leases	Operating Leases
2024 (excluding the nine months ended September 30, 2024)	\$ —	\$ 167
2025	—	536
2026	—	146
2027	—	136
2028	—	161
Thereafter	—	49
Total lease payments	—	1,195
Less: future interest expense	—	(172)
Lease liabilities	<u>\$ —</u>	<u>\$ 1,023</u>

Note L - Merger

**9 Months Ended
Sep. 30, 2024**

Notes to Financial Statements

Mergers, Acquisitions and Dispositions Disclosures [Text Block]

L. Merger

On August 30, 2023, in connection with the Merger Agreement with Acer, the following transactions occurred prior to Closing:

- *Bridge Loan* - Zevra and Acer entered into a bridge loan agreement (the “Bridge Loan Agreement”), providing for Zevra to make loans to Acer up to an aggregate principal amount of \$16.5 million. The Bridge Loan was provided to Acer to support its termination agreement with Relief Therapeutics Holding SA and to provide Acer with working capital, including for payments of accounts payable to support the commercial launch of OLPRUVA and the development of celiprolol pending the Merger’s closure. On October 31, 2023, the Company and Acer entered into an amendment to the Bridge Loan Agreement, which increased the aggregate principal amount available under the loan from \$16.5 million to \$18.0 million.
- *Purchase of Acer’s Term Loans* - Zevra purchased certain indebtedness of Acer held by Nantahala. Under the loan purchase with Nantahala, Zevra purchased (i) an original senior secured term loan facility made available to Acer in an aggregate amount of \$6.5 million and funded on March 14, 2022, and (ii) an additional senior secured term loan made to Acer in an aggregate amount of \$7.0 million in a single borrowing which funded on January 31, 2023 for (1) \$12.0 million in cash; (2) 98,683 shares of Zevra Common Stock; and (3) a secured Promissory Note payable by Zevra to Nantahala in the original principal amount \$5.0 million. These were recorded as receivables from Acer and were treated as a settlement of a preexisting relationship in connection with the closing of the transaction and recorded as a component of purchase consideration.
- *Purchase of Acer’s Convertible Notes* (“Marathon Convertible Notes”) - Under the Note Purchase Agreement with Nantahala, Zevra purchased the Marathon Convertible Notes that Nantahala had acquired on June 16, 2023. Zevra acquired the Marathon Convertible Notes in exchange for the issuance of 2,171,038 shares of Zevra Common Stock at \$5.0667 per share for a total purchase price of \$11.0 million.
- *Amendment to IP License Agreement and IP Termination Agreement* - As a condition to entering into the Merger Agreement, Acer and Relief entered into the Exclusive License Agreement and the Termination Agreement terminating the collaboration and license agreement, dated March 19, 2021, by and between Acer and Relief. Pursuant to the Exclusive License Agreement, Relief holds exclusive development and commercialization rights for OLPRUVA in Geographical Europe. Acer has the right to receive a royalty of up to 10.0% of the net sales of OLPRUVA in Geographical Europe. In accordance with the terms of the Termination Agreement, Relief received an upfront payment from Acer of \$10.0 million (which payment was funded with the Bridge Loan described above) with an additional payment of \$1.5 million due on the first-year anniversary of the \$10.0 million payment. Acer also agreed to pay a 10.0% royalty on net sales of OLPRUVA worldwide, excluding Geographical Europe, and 20.0% of any value received by Acer from certain third parties relating to OLPRUVA licensing or divestment rights, all of the foregoing which are capped at \$45.0 million, for total payments to Relief of up to \$56.5 million.

In connection with the closing of the Merger on November 17, 2023, each share of common stock of Acer was converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value \$0.0001 per share, and (ii) one non-transferable CVR to be issued by Zevra, which will represent the right to receive one or more contingent payments up to an additional \$76 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer's OLPRUVA and celiprolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer's early-stage program ACER-2820 (emetine).

The assets acquired and liabilities assumed were recorded based on their acquisition date fair values. Consideration for the Merger was \$72.6 million and consists of (i) approximately 2.96 million shares of Zevra common stock valued at \$12.8 million, (ii) the Bridge Loan advances of \$17.8 million, (iii) \$12.0 million in cash paid to Nantahala; (iv) 2.27 million shares of Zevra Common Stock issued to Nantahala valued at \$11.5 million based on the VWAP of shares of Zevra Common Stock during the 20 consecutive trading days ending on the trading date prior to August 30, 2023; (v) a secured promissory note payable by Zevra to Nantahala in the original principal amount of \$5.0 million, as disclosed in Note C, (vi) \$8.5 million in the estimated fair value of contingent consideration related to the CVRs, (vii) approximately 0.9 million shares of Zevra Common Stock issued to a former holder of Acer warrants valued at \$4.0 million based on Zevra's common stock price on the Effective Date and (viii) \$1.0 million in notes payable paid by the Company on Acer's behalf. In addition, effective as of immediately prior to the Effective Time, all of the outstanding and unexercised Acer stock options were automatically cancelled and ceased to exist without any cash or other consideration being paid or provided in respect thereof. The following purchase price allocation reflects the preliminary estimates of and assumptions related to the fair values of assets acquired and liabilities assumed:

Assets	
Cash	\$ 575
Prepaid expenses	278
Other current assets	11
Inventory	9,376
Property, plant, and equipment	35
Other noncurrent assets	209
Approved product - OLPRUVA	68,000
IPR&D - celiprolol	2,000
Goodwill acquired	4,701
	<u>85,185</u>
Liabilities	
Accounts payable and accrued expenses	\$10,881
Deferred collaboration funding	1,500
Operating lease liabilities	175
	<u>12,556</u>
Fair Value of Net Assets Acquired	<u><u>\$72,629</u></u>

The preliminary fair values assigned to the tangible and intangible assets acquired and liabilities assumed were determined using an income approach based on management's estimates and assumptions, as well as other information compiled by management, including third-party valuations that utilize customary valuation procedures and techniques. These preliminary fair values are subject to change within the one-year measurement period. The estimated fair values were developed by discounting future net cash flows to their present value at market-based rates of return. The goodwill acquired represents the excess of the purchase price and related costs over the value assigned to the net tangible and identifiable intangible assets of the business

acquired. The useful lives of the intangible assets for amortization purposes were determined by considering the period of expected cash flows used to measure the fair values of the intangible assets adjusted as appropriate for entity-specific factors including legal, regulatory, contractual, competitive, economic and other factors that may limit the useful life. The marketed product asset is amortized on a straight-line basis over its estimated useful life. As of September 30, 2024, the in-process research and development ("IPR&D") project had not been completed or abandoned and, therefore, the IPR&D intangible asset is not currently subject to amortization.

The results of operations and changes in stockholders' equity for Acer were included in the Company's consolidated financial statements beginning November 18, 2023.

The following pro forma combined results of operations present the acquisition as if it had occurred on January 1, 2023. The pro forma combined results of operations do not necessarily represent the Company's consolidated results of operations had the acquisition occurred on the date assumed, nor are these results necessarily indicative of the Company's future consolidated results of operations. The Company expects to realize certain benefits from integrating Acer into the Company and to incur certain one-time costs. The pro forma combined results of operations do not reflect these benefits or costs.

	Three Months Ended September 30, 2023		Nine Months Ended September 30, 2023	
Pro forma revenue	\$	3,267	\$	14,913
Pro forma net loss		(25,900)		(54,763)

Cancellation of Acer Warrant

On November 22, 2023, the Company sold an aggregate of 917,934 shares of its common stock to a healthcare focused investment fund (the "Investor") to cancel a warrant held by the Investor to purchase 2,920,306 shares of common stock of Acer. The shares of common stock were offered and sold to the Investor in a registered direct offering without an underwriter or placement agent.

Contingent Consideration

Contingent consideration liabilities relate to our liabilities arising in connection with the CVRs issued as a result of the Merger. The contingent consideration is classified as Level 3 in the fair value hierarchy. The fair value is measured based on a Monte Carlo simulation or a scenario-based method, depending on the earn-out achievement objectives, utilizing projections about future performance. Significant inputs include volatility and projected financial information, including projections representative of a market participant's view of the expected cash payments associated with the agreed upon regulatory milestones based on probabilities of technical success, timing of the potential milestone events for the compounds, and estimated discount rates.

The following table provides a reconciliation of the beginning and ending balances related to the contingent consideration liabilities for the CVRs (dollars in thousands):

Balance at December 31, 2023	\$ 7,262
Change in fair value	(2,462)

recognized in earnings	
Balance at September 30, 2024	<u>\$ 4,800</u>

For the nine months ended September 30, 2024, the Company recorded a \$2.5 million gain on the change in fair value of contingent consideration, primarily due to changes in market data and revenue projections.

**Note M - Goodwill &
Intangible Assets**

**9 Months Ended
Sep. 30, 2024**

**Notes to Financial
Statements**

**Goodwill and Intangible
Assets Disclosure [Text Block]**

M. Goodwill & Intangible Assets

The Company's goodwill balance was \$4.7 million as of September 30, 2024, and December 31, 2023.

As of September 30, 2024, and December 31, 2023, non-amortizable intangible assets include IPR&D of \$2.0 million

As of September 30, 2024, and December 31, 2023, the Company had a definite-lived intangible asset, net related to the acquisition of OLPRUVA as a result of the Merger of \$62.6 million and \$67.2 million, respectively. This is amortized on a straight-line basis over the OLPRUVA patent life of thirteen years and is reviewed periodically for impairment. Amortization expense is recorded as intangible asset amortization in the unaudited condensed consolidated statements of operations and was \$1.5 million and \$4.6 million for the three and nine months ended September 30, 2024. No amortization expense related to definite-lived intangible assets was recognized for the three and nine months ended September 30, 2023.

In accordance with its policy, the Company reviews the estimated useful lives of its intangible assets on an ongoing basis. This review indicated that based on recent facts and circumstances, including detailed analysis on sales experience and prospective product strategy, the actual life of the definite-lived intangible asset related to OLPRUVA was longer than the estimated useful life previously established at the acquisition of the asset. As a result, effective September 30, 2024, the Company changed its estimate of the useful life of this intangible asset to better reflect the estimated periods during which this asset will remain in service. The estimated useful life was previously 11 years and was increased to approximately 13 years. The change in estimate had no effect on depreciation and amortization expense, net income, or basic and diluted earnings per share for the three and nine months ended September 30, 2024.

In connection with the XOMA License Agreement, the Company owed XOMA a royalty payment of \$6.0 million upon approval of MIPLYFFA in September 2024, which is included in intangible assets, net in the unaudited condensed consolidated balance sheet as of September 30, 2024.

This definite-lived intangible asset is amortized on a straight-line basis over the MIPLYFFA patent life of approximately five years and is reviewed periodically for impairment. Amortization expense is recorded as intangible asset amortization in the unaudited condensed consolidated statements of operations and was de minimis for the three and nine months ended September 30, 2024 . No amortization expense related to definite-lived intangible assets was recognized for the three and nine months ended September 30, 2023 .

For intangible assets subject to amortization, estimated amortization expense for the five fiscal years subsequent to September 30, 2024, is expected to be \$6.5 million per year.

Note N - Subsequent Events

**9 Months Ended
Sep. 30, 2024**

[Notes to Financial
Statements](#)

[Subsequent Events \[Text
Block\]](#)

N. Subsequent Events

The Company evaluated events and transactions occurring subsequent to September 30, 2024, through November 13, 2024, the date the accompanying unaudited condensed consolidated financial statements were issued. During this period, there were no subsequent events that required recognition in the accompanying unaudited condensed consolidated financial statements, nor were there any additional non-recognized subsequent events that required disclosure.

**Insider Trading
Arrangements**

**3 Months Ended
Sep. 30, 2024
shares**

Trading Arrangements, by Individual

Non-Rule 10b5-1 Arrangement Adopted [Flag] false

Non-Rule 10b5-1 Arrangement Terminated [Flag] false

ZVRA_ Timothy J. Sangiovanni [Member]

Trading Arrangements, by Individual

Trading Arrangement Adoption Date October 15, 2024

Trading Arrangement, Individual Name Timothy J. Sangiovanni

Trading Arrangement, Individual Title Senior Vice President, Finance and Corporate Controller

Rule 10b5-1 Arrangement Adopted [Flag] true

Rule 10b5-1 Arrangement Terminated [Flag] true

Trading Arrangement, Securities Aggregate Available Amount 3,000

Trading Arrangement Termination Date April 15, 2025

ZVRA_ Sven Guenther, Ph.D [Member]

Trading Arrangements, by Individual

Trading Arrangement Adoption Date October 15, 2024

Trading Arrangement, Individual Name Sven Guenther, Ph.D.

Trading Arrangement, Individual Title Chief Scientific Officer

Rule 10b5-1 Arrangement Adopted [Flag] true

Rule 10b5-1 Arrangement Terminated [Flag] true

Trading Arrangement, Securities Aggregate Available Amount 8,000

Trading Arrangement Termination Date April 15, 2025

ZVRA_ Joshua Schafer [Member]

Trading Arrangements, by Individual

Trading Arrangement Adoption Date October 15, 2024

Trading Arrangement, Individual Name Joshua Schafer

Trading Arrangement, Individual Title Chief Commercial Officer & Executive Vice President, Business Development

Rule 10b5-1 Arrangement Adopted [Flag] true

Rule 10b5-1 Arrangement Terminated [Flag] true

Trading Arrangement, Securities Aggregate Available Amount 10,500

Trading Arrangement Termination Date April 15, 2025

ZVRA_ Christal M.M. Mickle [Member]

Trading Arrangements, by Individual

Trading Arrangement Adoption Date October 15, 2024

Trading Arrangement, Individual Name Christal M.M. Mickle

Trading Arrangement, Individual Title Chief Development Officer

Rule 10b5-1 Arrangement Adopted [Flag] true

Rule 10b5-1 Arrangement Terminated [Flag]	true
Trading Arrangement, Securities Aggregate Available Amount	6,300
Trading Arrangement Termination Date	April 15, 2025
ZVRA_ R. LaDuane Clifton [Member]	
Trading Arrangements, by Individual	
Trading Arrangement Adoption Date	October 15, 2024
Trading Arrangement, Individual Name	R. LaDuane Clifton
Trading Arrangement, Individual Title	Chief Financial Officer and Treasurer
Rule 10b5-1 Arrangement Adopted [Flag]	true
Rule 10b5-1 Arrangement Terminated [Flag]	true
Trading Arrangement, Securities Aggregate Available Amount	11,000
Trading Arrangement Termination Date	April 15, 2025
ZVRA_ Neil F. McFarlane [Member]	
Trading Arrangements, by Individual	
Trading Arrangement Adoption Date	October 15, 2024
Trading Arrangement, Individual Name	Neil F. McFarlane
Trading Arrangement, Individual Title	Chief Executive Officer
Rule 10b5-1 Arrangement Adopted [Flag]	true
Rule 10b5-1 Arrangement Terminated [Flag]	true
Trading Arrangement, Securities Aggregate Available Amount	91,817
Trading Arrangement Termination Date	April 15, 2025

Significant Accounting Policies (Policies)

9 Months Ended
Sep. 30, 2024

[Accounting Policies](#)

[\[Abstract\]](#)

[Use of Estimates, Policy](#)

[\[Policy Text Block\]](#)

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires the Company to make estimates and assumptions that affect the amounts reported in the unaudited condensed consolidated financial statements and accompanying notes. Actual results could differ from those estimates.

On an ongoing basis, the Company evaluates its estimates and assumptions, including those related to revenue recognition, the useful lives of property and equipment, the recoverability of long-lived assets, the incremental borrowing rate for leases, and assumptions used for purposes of determining stock-based compensation, income taxes, the fair value of investments and the fair value of the warrant liabilities, contingent value right ("CVR") liabilities and discount and rebate liabilities, among others. The Company bases its estimates on historical experience and on various other assumptions that it believes to be reasonable, the results of which form the basis for making judgments about the carrying value of assets and liabilities.

[Investment, Policy \[Policy Text Block\]](#)

Investments

The Company maintains investment securities that are classified as available-for-sale securities for which the Company has elected the fair value option under Accounting Standards Codification ("ASC") 825, *Financial Instruments* ("ASC 825"). As such, these securities are carried at fair value with unrealized gains and losses included in fair value adjustment related to investments on the unaudited condensed consolidated statements of operations. The securities primarily consist of U.S. Treasury securities and corporate bonds and are included in securities at fair value in the unaudited condensed consolidated balance sheets. The Company's investments in corporate bonds mature within one to five years. As of September 30, 2024, and December 31, 2023, the Company held securities with an aggregate fair value of \$41.4 million and \$24.7 million, respectively, that contained aggregate unrealized gains of approximately \$0.1 million and \$0.6 million, respectively. Applying fair value accounting to these debt securities more accurately represents the Company's investment strategy due to the fact that excess cash is currently being invested for the purpose of funding future operations. Interest income is recognized as earned using an effective yield method giving effect to the amortization of premium and accretion of discount and is based on the economic life of the securities. Interest income is included in interest and other income, net in the unaudited condensed consolidated statements of operations.

[Consolidation, Variable Interest Entity, Policy \[Policy Text Block\]](#)

Variable Interest Entities

The primary beneficiary of a variable interest entity ("VIE") is required to consolidate the assets and liabilities of the VIE. When the Company obtains a variable interest in another entity, it assesses at the inception of the relationship and upon occurrence of certain significant events whether the entity is a VIE, and if so, whether the Company is the primary beneficiary of the VIE based on its power to direct the activities of the VIE that most significantly impact the VIE's economic performance and the Company's obligation to absorb losses or the rights to receive benefits from the VIE that could potentially be significant to the VIE.

To assess whether the Company has the power to direct the activities of the VIE that most significantly impact the VIE's economic performance, the Company considers all the facts and circumstances, including the Company's role in establishing the VIE and the Company's ongoing rights and responsibilities. The assessment includes identifying the activities that most significantly impact the VIE's economic performance and identifying which party, if any, has the power to direct those activities. In general, the parties that make the most significant decisions

affecting the VIE (management and representation on the Board of Directors) are deemed to have the power to direct the activities of a VIE.

To assess whether the Company has the obligation to absorb losses of the VIE or the rights to receive benefits from the VIE that could potentially be significant to the VIE, the Company considers all of its economic interests that are deemed to be variable interests in the VIE.

This assessment requires judgement in determining whether these interests, in the aggregate, are considered potentially significant to the VIE. As of September 30, 2024, and December 31, 2023, the Company identified Acer to be the Company's sole interest in a VIE (Note L). As Zevra is the final decision maker for all of Acer's research, development, and commercialization of drug candidates that it is producing, the Company directs the activities of Acer that most significantly impact its performance. Therefore, the Company is the primary beneficiary of this VIE for accounting purposes.

[Revenue from Contract with Customer \[Policy Text Block\]](#)

Revenue Recognition

The Company recognizes revenue in accordance with the provisions of ASC 606, *Revenue from Contracts with Customers* ("ASC 606") and, as a result, follows the five-step model when recognizing revenue: 1) identifying a contract; 2) identifying the performance obligations; 3) determining the transaction price; 4) allocating the price to performance obligations; and 5) recognizing revenue when the performance obligations have been fulfilled.

Licensing Agreements

The terms of the Company's licensing agreements typically include one or more of the following: (i) upfront fees; (ii) milestone payments related to the achievement of development, regulatory, or commercial goals; and (iii) royalties on net sales of licensed products. Each of these payments may result in licensing revenues.

As part of the accounting for these agreements, the Company must develop estimates and assumptions that require judgment to determine the underlying stand-alone selling price for each performance obligation which determines how the transaction price is allocated among the performance obligations. Generally, the estimation of the stand-alone selling price may include such estimates as independent evidence of market price, forecasted revenues or costs, development timelines, discount rates, and probability of regulatory success. The Company evaluates each performance obligation to determine if they can be satisfied at a point in time or over time, and it measures the services delivered to the licensee which are periodically reviewed based on the progress of the related program. The effect of any change made to an estimated input component and, therefore revenue or expense recognized, would be recorded as a change in estimate. In addition, variable consideration (e.g., milestone payments) must be evaluated to determine if it is constrained and, therefore, excluded from the transaction price.

Up-front Fees: If a license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenues from the transaction price allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time.

Milestone Payments: At the inception of each arrangement that includes milestone payments (variable consideration), the Company evaluates whether the milestones are considered probable of being reached and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's or the licensee's control, such as non-operational developmental and regulatory approvals, are generally not considered probable of being achieved until those approvals

are received. At the end of each reporting period, the Company re-evaluates the probability of achievement of milestones that are within its or the licensee's control, such as operational developmental milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenues and earnings in the period of adjustment. Revisions to the Company's estimate of the transaction price may also result in negative licensing revenues and earnings in the period of adjustment.

Product Revenues, Net

Net revenues from product sales is recognized at the transaction price when the customer obtains control of the Company's products, which occurs at a point in time, typically upon receipt of the product by the customer. Our current single customer is a specialty pharmacy provider.

The Company's net revenues represent total revenues adjusted for discounts and allowances, including estimated cash discounts, chargebacks, rebates, returns, copay assistance, data fees and wholesaler fees for services. These adjustments represent variable consideration under ASC 606 and are recorded as a reduction of revenue. These adjustments are established by management as its best estimate based on available information and will be adjusted to reflect known changes in the factors that impact such allowances. Adjustments for variable consideration are determined based on the contractual terms with customers, historical trends, communications with customers and the levels of inventory remaining in the distribution channel, as well as expectations about the market for the product and anticipated introduction of competitive products.

[Research and Development Expense, Policy \[Policy Text Block\]](#)

Acquired IPR&D and Milestones Expenses

In an asset acquisition, payments incurred prior to regulatory approval to acquire rights to in-process R&D projects are expensed as acquired IPR&D and milestones expense in the consolidated statements of earnings unless the project has an alternative future use. These costs include upfront and development milestone payments related to R&D collaborations, licensing arrangements, or other asset acquisitions that provide rights to develop, manufacture and/or sell pharmaceutical products. Where contingent development milestone payments are due to third parties, prior to regulatory approval, the payment obligations are expensed when the milestone results are achieved. Regulatory and commercial milestone payments made to third parties subsequent to regulatory approval are capitalized as intangible assets and amortized to cost of products sold over the remaining useful life of the related product.

[Inventory, Policy \[Policy Text Block\]](#)

Inventories

The value of inventory is recorded at its net realizable value. The Company determines the cost of its other inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis.

The Company may scale-up and make commercial quantities of its product candidates prior to the date it anticipates that such product will receive final regulatory approval. The scale-up and commercial production of pre-launch inventory involves the risk that such products may not be approved for marketing on a timely basis, or ever. This risk notwithstanding, the Company may scale-up and build pre-launch inventory of product that have not received final regulatory approval when the Company believes such action is appropriate in relation to the commercial value of the product launch opportunity. Inventory manufactured prior to regulatory approval is recorded as research and development expense until regulatory approval for the product is obtained. Inventory used in clinical trials is also expensed as research and development expense, when selected for such use. Inventory that can be used in either the production of clinical or commercial products is expensed as research and development costs when identified for use in a clinical manufacturing campaign. The cost of finished goods inventory that is shipped to a customer to support the Company's patient assistance programs is expensed when those shipments take place. As of September 30, 2024, and December 31, 2023, the Company did not have pre-launch inventory that qualified for capitalization.

The Company performs an assessment of the recoverability of capitalized inventory during each reporting period and writes down any excess and obsolete inventory to its net realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded as a component of cost of product revenue in the statements of operations and comprehensive loss. The determination of whether inventory costs will be realizable requires the use of estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required. Additionally, the Company's product is subject to strict quality control and monitoring that it performs throughout the manufacturing process. In the event that certain batches or units of product do not meet quality specifications, the Company will record a charge to cost of product revenue, to write-down any unsaleable inventory to its estimated net realizable value. For the three and nine months ended September 30, 2024, the Company recognized a charge of approximately \$2.0 million and \$5.2 million, respectively, related to write-downs for unsaleable inventory. No such write downs were recognized for the three and nine months ended September 30, 2023.

[Foreign Currency Transactions and Translations Policy](#) *Foreign currency*
[Policy Text Block]

Assets and liabilities are translated into the reporting currency using the exchange rates in effect on the unaudited condensed consolidated balance sheet dates. Equity accounts are translated at historical rates, except for the change in accumulated deficit during the year, which is the result of the income statement translation process. Revenue and expense accounts are translated using the weighted average exchange rate during the period. The cumulative translation adjustments associated with the net assets of foreign subsidiaries are recorded in accumulated other comprehensive income/loss in the accompanying unaudited condensed consolidated statements of stockholders' equity.

[Debt, Policy](#) [Policy Text Block]

Debt Issuance Costs

Debt issuance costs incurred in connection with financing arrangements are recorded as a reduction of the related debt on the balance sheet and amortized over the life of the respective financing arrangement using the effective interest method.

[Warrants](#) [Policy Text Block]

Warrants

The Company accounts for warrants as either equity-classified or liability-classified instruments based on an assessment of the warrant's specific terms and applicable authoritative guidance in FASB ASC Topic 480, *Distinguishing Liabilities from Equity* ("ASC 480") and FASB ASC Topic 815, *Derivatives and Hedging* ("ASC 815"). The assessment considers whether the warrants are freestanding financial instruments pursuant to ASC 480, meet the definition of a liability pursuant to ASC 480, and whether the warrants meet all of the requirements for equity classification under ASC 815, including whether the warrants are indexed to our own stock and whether the warrant holders could potentially require "net cash settlement" in a circumstance outside of the company's control, among other conditions for equity classification. This assessment, which requires the use of professional judgment, is conducted at the time of warrant issuance and as of each subsequent quarterly period end date while the warrants are outstanding.

For warrants that meet all criteria for equity classification, the warrants are required to be recorded as a component of additional paid-in capital, on the unaudited condensed consolidated statement of stockholders' deficit at the time of issuance. For warrants that do not meet all the criteria for equity classification, the warrants are required to be recorded at their initial fair value on the date of issuance, and on each balance sheet date thereafter. Changes in the estimated fair value of the warrants are recognized as a non-cash gain or loss in other expense, net, on the unaudited condensed consolidated statement of operations. The fair value of the warrants was estimated using the Black-Scholes option pricing model.

New Accounting Pronouncements Not Yet Adopted

Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures

In November 2023, the FASB issued Accounting Standards Update ("ASU") No. 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*, which modifies the disclosure and presentation requirements of reportable segments. The amendments in the update require the disclosure of significant segment expenses that are regularly provided to the chief operating decision maker ("CODM") and included within each reported measure of segment profit and loss. The amendments also require disclosure of all other segment items by reportable segment and a description of its composition. Additionally, the amendments require disclosure of the title and position of the CODM and an explanation of how the CODM uses the reported measure(s) of segment profit or loss in assessing segment performance and deciding how to allocate resources. This update is effective for annual periods beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024. Early adoption is permitted. The Company is currently evaluating the impact that this guidance will have on the presentation of its consolidated financial statements and accompanying notes.

Income Taxes (Topic 740): Improvements to Income Tax Disclosures

In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*, which expands disclosures in an entity's income tax rate reconciliation table and disclosures regarding cash taxes paid both in the U.S. and foreign jurisdictions. The update will be effective for annual periods beginning after December 15, 2024. The Company is currently evaluating the impact that this guidance will have on the presentation of its consolidated financial statements and accompanying notes.

Note C - Inventories (Tables)

**9 Months Ended
Sep. 30, 2024**

[Notes Tables](#)

[Schedule of Inventory, Noncurrent \[Table Text Block\]](#)

	September 30, 2024	December 31, 2023
Raw materials	\$ 4,549	\$ 2,938
Work in progress	3,084	1,884
Finished goods	1,123	5,019
Total inventory	<u>\$ 8,756</u>	<u>\$ 9,841</u>

**Note D - Debt Obligations
(Tables)**

**9 Months Ended
Sep. 30, 2024**

Notes Tables

Schedule of Long-Term Debt Instruments

[Table Text Block]

	September 30, 2024	December 31, 2023
Notes payable	\$ 61,035	\$ —
Secured promissory note	—	5,000
Unamortized original issue (discount) premium	(959)	148
Less: debt issuance costs	(1,172)	(82)
	<u>\$ 58,904</u>	<u>\$ 5,066</u>

Schedule of Maturities of Long-Term Debt

[Table Text Block]

Year Ending December 31,	
2024 (excluding the nine months ending September 30, 2024)	\$ —
2025	—
2026	—
2027	—
2028	—
Thereafter	60,919
Total minimum payments	60,919
Less: unamortized debt discount, debt issuance costs and paid in kind interest	(2,015)
Note payable	<u>\$ 58,904</u>

**Note G - Stock and Warrants
(Tables)**

**9 Months Ended
Sep. 30, 2024**

Notes Tables

Schedule of Authorized Shares of Common Stock
Reserved for Future Issuance [Table Text Block]

	September 30, 2024	December 31, 2023
Outstanding awards under equity incentive plans	8,987,997	8,023,142
Outstanding common stock warrants	5,483,537	5,603,729
Possible future issuances under equity incentive plans	4,536,410	1,728,885
Possible future issuances under employee stock purchase plans	1,242,425	1,340,172
Total common shares reserved for future issuance	20,250,369	16,695,928

Schedule of Common Stock Outstanding Roll
Forward [Table Text Block]

	Shares of Common Stock
Balance as of January 1, 2024	41,534,668
Common stock issued as compensation to third parties	9,000
Common stock issued as a result of stock options exercised	306,826
Balance as of March 31, 2024	41,850,494
Common stock issued as a result of the Employee Stock Purchase Plan	97,747
Common stock issued as compensation to third parties	41,820
Common stock issued as a result of stock options exercised	1,403
Balance as of June 30, 2024	41,991,464
Common stock issued as compensation to third parties	32,820
Common stock issued in connection with the Public Offering (Note B)	10,615,385
Common stock issued as a result of stock options exercised	39,750
Common stock issued in connection with vesting of performance-based awards	547,945
Balance as of September 30, 2024	53,227,364

**Note H - Stock-based
Compensation (Tables)**

**9 Months Ended
Sep. 30, 2024**

Notes Tables

Share-Based Payment Arrangement, Expensed and
Capitalized, Amount [Table Text Block]

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Research and development	\$1,441	\$ 720	\$ 3,322	\$2,023
Selling, general and administrative	4,696	667	7,566	1,058
Total stock-based compensation expense	\$6,137	\$1,387	\$10,888	\$3,081

**Note I - Fair Value of
Financial Instruments
(Tables)**

**9 Months Ended
Sep. 30, 2024**

Notes Tables

[Schedule of Fair Value, Assets
and Liabilities Measured on
Recurring Basis \[Table Text
Block\]](#)

	Balance as of September 30, 2024	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
CVR liability (Note L)	\$ 4,800	\$ —	\$ —	\$ 4,800
Warrant liabilities	13,902	—	—	13,902
Total liabilities	\$ 18,702	\$ —	\$ —	\$ 18,702

Securities:

U.S. Treasury securities	\$ 35,337	\$ 35,337	\$ —	\$ —
Corporate bonds	6,105	—	6,105	—
Total assets	\$ 41,442	\$ 35,337	\$ 6,105	\$ —

	Balance as of December 31, 2023	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
CVR liability (Note L)	\$ 7,262	\$ —	\$ —	\$ 7,262
Warrant liabilities	16,100	—	—	16,100
Total liabilities	\$ 23,362	\$ —	\$ —	\$ 23,362

Securities:

U.S. Treasury securities	\$ 24,688	\$ 24,688	\$ —	\$ —
Total assets	\$ 24,688	\$ 24,688	\$ —	\$ —

[Fair Value Measurement
Inputs and Valuation
Techniques \[Table Text Block\]](#)

	September 30, 2024	December 31, 2023
Risk-free interest rate	3.51% - 3.81%	3.76% - 4.12%
Volatility	61.76% - 82.44%	62.01% - 92.42%
Dividend yield	0%	0%
Expected term (years)	1.27 - 4.14	2.0 - 4.9
Weighted average fair value	\$ 2.54	\$ 2.94
Balance as of December 31, 2023		\$ 16,100
Change in fair value measurement		(2,198)
Balance as of September 30, 2024		\$ 13,902

[Fair Value, Liabilities
Measured on Recurring Basis,
Unobservable Input](#)

[Reconciliation \[Table Text Block\]](#)

**Note J - Net Loss Per Share
(Tables)**

**9 Months Ended
Sep. 30, 2024**

Notes Tables

Schedule of Antidilutive Securities
Excluded from Computation of Earnings
Per Share [Table Text Block]

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Awards under equity incentive plans	8,987,997	7,262,039	8,987,997	7,262,039
Common stock warrants	5,483,537	4,252,490	5,483,537	4,252,490
Total securities excluded from the calculation of weighted average number of shares of common stock outstanding	14,471,534	11,514,529	14,471,534	11,514,529

Schedule of Earnings Per Share, Basic and Diluted [Table Text Block]

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Basic and diluted net loss per share of common stock:				
Net loss, basic and diluted	\$(33,225)	\$(10,367)	\$(69,772)	\$(26,157)
Weighted average number of shares of common stock outstanding, basic and diluted	47,809	34,725	43,844	34,364
Basic and diluted net loss per share of common stock	\$ (0.69)	\$ (0.30)	\$ (1.59)	\$ (0.76)

Note K - Leases (Tables)

9 Months Ended Sep. 30, 2024

[Notes Tables](#)

[Lease, Cost \[Table Text Block\]](#)

Lease Cost	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Finance lease cost:				
Amortization of right-of-use assets	\$ 12	\$ 26	\$ 36	\$ 90
Interest on lease liabilities	—	—	—	—
Total finance lease cost	12	26	36	90
Operating lease cost	119	113	330	340
Short-term lease cost	59	55	177	165
Variable lease cost	26	13	39	39
Less: sublease income	(50)	(39)	(128)	(117)
Total lease costs	\$ 166	\$ 168	\$ 454	\$ 517

[Schedule of Leases Cash Flow Information \[Table Text Block\]](#)

	Nine months ended September 30,	
	2024	2023
Cash paid for amounts included in the measurement of lease liabilities:		
Financing cash flows from finance leases	\$ —	\$ 5
Operating cash flows from operating leases	405	426
Operating cash flows from short-term leases	177	165
Operating cash flows from variable lease costs	39	39

Right-of-use assets obtained in exchange for lease liabilities:		
Finance leases	\$ —	\$ —
Operating leases	419	—

[Schedule of Leases Balance Sheet Information \[Table Text Block\]](#)

	September 30, 2024	December 31, 2023
Finance Leases		
Property and equipment, at cost	\$ 1,031	\$ 1,031
less: accumulated depreciation and amortization	(917)	(882)
Property and equipment, net	\$ 114	\$ 149
Other current liabilities	\$ —	\$ —
Other long-term liabilities	—	—
Total finance lease liabilities	\$ —	\$ —

Operating Leases

Operating lease right-of-use assets	\$	820	\$	790
Total operating lease right-of-use assets	\$	820	\$	790

Current portion of operating lease liabilities	\$	540	\$	543
Operating lease liabilities, less current portion		483		456
Total operating lease liabilities	\$	1,023	\$	999

Weighted Average Remaining Lease Term

Operating leases (in years)	2	2
-----------------------------	---	---

Weighted Average Discount Rate

Finance leases	0.0%	14.3%
Operating leases	9.6%	7.6%

[Operating and Finance Lease, Liability, Maturity \[Table Text Block\]](#)

Year Ending December 31,	Finance Leases	Operating Leases
2024 (excluding the nine months ended September 30, 2024)	\$ —	\$ 167
2025	—	536
2026	—	146
2027	—	136
2028	—	161
Thereafter	—	49
Total lease payments	—	1,195
Less: future interest expense	—	(172)
Lease liabilities	\$ —	\$ 1,023

Note L - Merger (Tables)

**9 Months Ended
Sep. 30, 2024**

Notes Tables

Schedule of Recognized Identified

Assets Acquired and Liabilities

Assumed [Table Text Block]

Assets	
Cash	\$ 575
Prepaid expenses	278
Other current assets	11
Inventory	9,376
Property, plant, and equipment	35
Other noncurrent assets	209
Approved product - OLPRUVA	68,000
IPR&D - celiprolol	2,000
Goodwill acquired	4,701
	<u>85,185</u>

Liabilities	
Accounts payable and accrued expenses	\$10,881
Deferred collaboration funding	1,500
Operating lease liabilities	175
	<u>12,556</u>

Fair Value of Net Assets Acquired	<u><u>\$72,629</u></u>
--	------------------------

Business Acquisition, Pro Forma Information [Table Text Block]

	Three Months Ended September 30, 2023	Nine Months Ended September 30, 2023
Pro forma revenue	\$ 3,267	\$ 14,913
Pro forma net loss	(25,900)	(54,763)

Schedule of Business Acquisitions by Acquisition, Contingent Consideration [Table Text Block]

Balance at December 31, 2023	\$ 7,262
Change in fair value recognized in earnings	(2,462)
Balance at September 30, 2024	<u><u>\$ 4,800</u></u>

Note A - Description of Business, Basis of Presentation and Significant Transactions (Details Textual) - USD (\$) \$/ shares in Units, \$ in Thousands	3 Months Ended								9 Months Ended											
	Aug. 12, 2024	Aug. 08, 2024	Jul. 11, 2024	Sep. 30, 2024	Jun. 30, 2024	Mar. 31, 2024	Sep. 30, 2023	Jun. 30, 2023	Mar. 31, 2023	Sep. 30, 2024	Sep. 30, 2023	Jul. 12, 2024	Jun. 04, 2024	Feb. 05, 2024	Dec. 31, 2023	Oct. 31, 2023	Aug. 30, 2023	Dec. 20, 2021	Jul. 02, 2021	
Net Income (Loss)				\$ (33,225)	\$	\$	\$	\$	\$	\$	\$									
Attributable to Parent					(19,925)	(16,622)	(10,367)	(2,573)	(13,217)	(69,772)	(26,157)									
Retained Earnings				\$						\$					\$					
(Accumulated Deficit)				(469,550)						(469,550)					(399,778)					
Common Stock Registered, Shares (in shares)														2,269,721						
Common Stock Registered, Maximum Offering Price													\$ 350,000							
Stock Issued During Period, Shares, New Issues (in shares)				10,615,385																
Stock Repurchase Program, Authorized Amount																		\$ 50,000		
Share Repurchase Program [Member]																				
Treasury Stock, Common, Shares (in shares)															1,575,692					
Treasury Stock, Common, Value															\$ 11,000					
ATM Agreement Sales [Member]																				
Common Stock Registered, Maximum Offering Price													\$ 75,000							
ATM Agreement Sales [Member] JMP [Member]																				
Stock Issued During Period, Shares, New Issues (in shares)										0										
Equity Distribution Agreement, Maximum Aggregate Offering Price												\$ 75,000								
Equity Distribution Agreement, Commission Fee, Percentage of Gross Sales												3.00%								
ATM Agreement Sales [Member] JMP and RBCCM [Member]																				
Stock Issued During Period, Shares, New Issues (in shares)			0																	
Equity Distribution Agreement, Maximum Aggregate Offering Price																		\$ 75,000		
Equity Distribution Agreement, Commission Fee, Percentage of Gross Sales																		3.00%		
Underwriting Agreement [Member]																				
Stock Issued During Period, Shares, New Issues (in shares)		9,230,770																		
Shares Issued, Price Per Share (in dollars per share)		\$ 6.5																		
Over-Allotment Option [Member] Maximum [Member]																				
Stock Issuable During the Period (in shares)		1,384,615																		
Underwriting Agreement and Over-allotment Option [Member]																				
Stock Issued During Period, Shares, New Issues (in shares)	10,615,385																			
Proceeds from Issuance of Common Stock, Net	\$ 64,500																			
Bridge Loan [Member]																				
Financing Receivable, Maximum Limit																\$ 18,000	\$ 18,000			

**Note B - Summary of
Significant Accounting
Policies (Details Textual) -
USD (\$)
\$ in Thousands**

	3 Months Ended		9 Months Ended		12 Months Ended
	Sep. 30, 2024	Sep. 30, 2023	Sep. 30, 2024	Sep. 30, 2023	Dec. 31, 2023
Investments, Fair Value Disclosure	\$ 41,400		\$ 41,400		\$ 24,700
Unrealized Gain (Loss) on Investments			100		600
Inventory Write-down	2,000	\$ 0	5,244	\$ 0	
Pre-launch Inventory that Qualified for Capitalization [Member]					
Inventory, Net	\$ 0		\$ 0		\$ 0

**Note C - Inventories -
Summary of Inventory
(Details) - USD (\$)
\$ in Thousands**

Sep. 30, 2024 Dec. 31, 2023

<u>Raw materials</u>	\$ 4,549	\$ 2,938
<u>Work in progress</u>	3,084	1,884
<u>Finished goods</u>	1,123	5,019
<u>Total inventory</u>	\$ 8,756	\$ 9,841

Note D - Debt Obligations (Details Textual) - USD (\$) \$ in Thousands						1 Months Ended	9 Months Ended				
	Apr. 05, 2024	Aug. 30, 2023	Jan. 31, 2023	Jan. 26, 2023		Nov. 30, 2023	Sep. 30, 2024	Sep. 30, 2023	Mar. 01, 2024	Dec. 31, 2023	Nov. 17, 2023
Stock Issued During Period, Shares,						1,382,489					
Acquisitions (in shares)											
Proceeds from Issuance of Debt							\$	\$			
							58,990	38,801			
Debt Issuance Costs, Net							1,172			\$ 82	
Wells Fargo Bank, N.A. [Member]											
Long-Term Line of Credit										\$	
										37,700	
Wells Fargo Bank, N.A. [Member]											
Prime Rate [Member]											
Debt Instrument, Basis Spread on Variable Rate					2.25%						
Wells Fargo Bank, N.A. [Member]											
Minimum [Member]											
Line of Credit Facility, Maximum Borrowing Capacity, Percentage of Investments					80.00%						
Wells Fargo Bank, N.A. [Member]											
Maximum [Member]											
Line of Credit Facility, Maximum Borrowing Capacity, Percentage of Investments					90.00%						
SWK Loans [Member]											
Payments to Acquire Notes Receivable		\$	\$								
		12,000	12,000								
Stock Issued During Period, Shares, Acquisitions (in shares)		98,683	98,683								
Nantahala Note [Member]											
Debt Instrument, Face Amount		\$ 5,000	\$							\$	
			5,000							5,000	
Debt Instrument, Interest Rate, Stated Percentage		9.00%									
Debt Instrument, Interest Rate, Stated Percentage, If Unpaid Within Six Months		12.00%									
Debt Instrument, Interest Rate, Increase in Stated Percentage, If Unpaid Within Six Months		3.00%							3.00%		

Debt Instrument, Interest, Shares, Consecutive Trading Days For Calculation of VWAP (Day)	20 days	
Debt Instrument, Periodic Payment, Interest	\$ 600	
Term Loans Under Credit Agreement [Member		
Debt Instrument, Face Amount	\$ 100,000	
Proceeds from Issuance of Debt	60,000	
Debt Instrument, Available Amount Until 18 Months Following Closing Date	20,000	
Debt Instrument, Available Amount Until 18 Months Following Closing Date, Maximum Each Drawing	10,000	
Debt Instrument, Available Amount Until December 31, 2024	20,000	
Debt Instrument, Net Product Sales Performance Benchmark	100,000	
Debt Instrument, Net Product Sales Performance Benchmark in Next Year	\$ 125,000	
Debt Instrument, Maximum Interest Payment, Percentage	25.00%	
Interest Payable		900
Debt Issuance Costs, Net		2,200
Debt Instrument, Minimum Cash Balance		\$ 20,000
Term Loans Under Credit Agreement [Member Secured Overnight Financing Rate (SOFR) [Member]		
Debt Instrument, Basis Spread on Variable Rate	7.00%	
Debt Instrument, Basis Spread on Variable Rate When Performance Benchmark Meet	6.00%	
Debt Instrument, Basis Spread on Variable Rate When Performance Benchmark Meet in Next Year	6.50%	
Debt Instrument, Floor Rate	4.00%	

**Note D - Debt Obligations -
Aggregate Principal Amount
(Details) - USD (\$)
\$ in Thousands**

Sep. 30, 2024 Dec. 31, 2023

Unamortized original issue (discount) premium	\$ (959)	\$ 148
Less: debt issuance costs	(1,172)	(82)
Notes Payable	58,904	5,066
Notes Payable Excluding Secured Promissory Note [Member]		
Notes payable	61,035	0
Nantahala Note [Member]		
Notes payable	\$ 0	\$ 5,000

**Note D - Debt Obligations -
Future Minimum Principal
Payments (Details) - USD (\$)
\$ in Thousands**

Sep. 30, 2024 Dec. 31, 2023

<u>Note payable</u>	\$ 58,904	\$ 5,066
<u>Term Loans Under Credit Agreement [Member</u>		
<u>2024 (excluding the nine months ending September 30, 2024)</u>	0	
<u>2025</u>	0	
<u>2026</u>	0	
<u>2027</u>	0	
<u>2028</u>	0	
<u>Thereafter</u>	60,919	
<u>Total minimum payments</u>	60,919	
<u>Less: unamortized debt discount, debt issuance costs and paid in kind interest</u>	(2,015)	
<u>Note payable</u>	\$ 58,904	

Note E - Revenue, Net
(Details Textual) - USD (\$)
\$ in Thousands

	Mar. 20, 2012	1 Months Ended Jun. 30, 2023	3 Months Ended Sep. 30, 2023	Sep. 30, 2024	9 Months Ended Sep. 30, 2023	Sep. 30, 2024	12 Months Ended Dec. 31, 2023	May 07, 2021
Accounts Receivable, Allowance for Credit Loss			\$ 0		\$ 0		\$ 0	
Arimoclomol Purchase Agreement [Member]								
Contract with Customer, Liability, Revenue Recognized			2,600	\$ 2,000	8,000	\$ 7,100		
Contract with Customer, Clawback Liability			1,400	1,100	4,400	4,000		
Asset Acquisition, Consideration Transferred, Estimated Reserve Liability					17,000		12,200	
XOMA License Agreement [Member]								
Accrued Royalties			6,000		6,000			
Consulting Services [Member]								
Accounts Receivable, after Allowance for Credit Loss, Current			2,300		2,300		1,300	
Commave Therapeutics SA [Member] License [Member] AZSTARYS License Agreement [Member]								
Revenue Recognition, Milestone Method, Additional Revenue to be Recognized								\$ 590,000
Relief Therapeutics [Member] License [Member]								
Contract with Customer, Liability, Revenue Recognized			0		0			
Contract with Customer, Liability			0		\$ 0		0	
Royalty Revenue, Percent					10.00%			
Aquestive Therapeutics [Member] License [Member] AZSTARYS License Agreement [Member]								
Contract with Customer, Liability, Revenue Recognized			\$ 5,000	1,100	\$ 900	\$ 3,600	\$ 7,200	
Royalty Revenue, Percent		10.00%						
Accounts Receivable, after Allowance for Credit Loss, Current			1,100		1,100		11,400	
Arimoclomol EAP [Member]								
Accounts Receivable, after Allowance for Credit Loss, Current			\$ 4,400		\$ 4,400		\$ 4,700	

Note F - Commitments and Contingencies (Details Textual) - USD (\$) \$ in Thousands	3 Months Ended		
	Jun. 30, 2024	Sep. 30, 2024	Dec. 31, 2023
Loss Contingency Accrual		\$ 0	\$ 0
Litigation Related to Merger [Member]			
Payments for Legal Settlements	\$ 300		
AZSTARYS License Agreement [Member]			
Loss Contingency Accrual			\$ 0

Note G - Stock and Warrants (Details Textual) - USD (\$) \$ / shares in Units, \$ in Thousands									
		1 Months Ended	2 Months Ended	3 Months Ended	9 Months Ended				
	Nov. 22, 2023	Nov. 30, 2023	Dec. 31, 2023	Sep. 30, 2024	Sep. 30, 2023	Sep. 30, 2024	Sep. 30, 2023	Jun. 30, 2024	Mar. 31, 2024
Common Stock, Shares Authorized (in shares)			250,000,000	250,000,000		250,000,000			
Common Stock, Shares, Issued (in shares)			43,110,360	54,803,056		54,803,056			
Common Stock, Shares, Outstanding (in shares)			41,534,668	53,227,364		53,227,364		41,991,464	41,850,494
Preferred Stock, Shares Authorized (in shares)			10,000,000	10,000,000		10,000,000			
Class of Warrant or Right, Outstanding (in shares)				5,483,537		5,483,537			
Stock Issued During Period, Shares, Acquisitions (in shares)		1,382,489							
Common Stock, Par or Stated Value Per Share (in dollars per share)		\$ 0.0001	\$ 0.0001	\$ 0.0001		\$ 0.0001			
Stock Issued During Period, Shares, New Issues (in shares)				10,615,385					
Derivative and Warrant Liability, Noncurrent			\$ 16,100	\$ 13,902		\$ 13,902			
Fair Value Adjustment of Put Option, Net of Tax				\$ 6,000	\$ 3,700	\$ 2,200	\$ 4,300		
Acer Warrants [Member] Proceeds from Warrant Exercises		\$ 6,000							
Class of Warrants or Right, Exercised During Period (in shares)			0			0			
Acer Warrants [Member] Common Stock [Member] Class of Warrant or Right, Number of Securities Called by Warrants or Rights (in shares)		1,382,489							
Shares and Warrants Issued, Price Per Share (in dollars per share)		\$ 4.34							
The Investor Warrant [Member] Class of Warrant or Right, Number of Securities Called by Warrants or Rights (in shares)	2,920,306	2,920,306							
Stock Issued During Period, Shares, New Issues (in shares)	917,934	917,934							

Note G - Stock and Warrants
- Reserved Authorized
Shares of Common Stock for
Future Issuance (Details) -
shares

Sep. 30, 2024 Dec. 31, 2023

Common shares reserved for future issuance (in shares) Warrant [Member]	20,250,369	16,695,928
Common shares reserved for future issuance (in shares) Share-Based Payment Arrangement [Member]	5,483,537	5,603,729
Common shares reserved for future issuance (in shares) Possible Future Issuances Under Equity Incentive Plans [Member]	8,987,997	8,023,142
Common shares reserved for future issuance (in shares) Possible Future Issuances Under Employee Stock Purchase Plans [Member]	4,536,410	1,728,885
Common shares reserved for future issuance (in shares)	1,242,425	1,340,172

Note G - Stock and Warrants
- Common Stock Activity
(Details) - shares

	3 Months Ended			9 Months Ended
	Sep. 30, 2024	Jun. 30, 2024	Mar. 31, 2024	Sep. 30, 2024
<u>Balance (in shares)</u>	41,991,464	41,850,494	41,534,668	41,534,668
<u>Common stock issued as compensation to third parties (in shares)</u>	32,820	41,820	9,000	
<u>Common stock issued as a result of stock options exercised (in shares)</u>	39,750	1,403	306,826	
<u>Common stock issued as a result of the Employee Stock Purchase Plan (in shares)</u>		97,747		257,575
<u>Common stock issued as compensation to third parties (in shares)</u>	32,820	41,820	9,000	
<u>Common stock issued in connection with the Public Offering (Note B) (in shares)</u>	10,615,385			
<u>Common stock issued in connection with vesting of performance-based awards (in shares)</u>	547,945			
<u>Balance (in shares)</u>	53,227,364	41,991,464	41,850,494	53,227,364

Note H - Stock-based Compensation (Details Textual) - USD (\$) \$ in Thousands	1 Months Ended		3 Months Ended			9 Months Ended				
	Jan. 01, 2024	Jun. 30, 2021	Sep. 30, 2024	Jun. 30, 2024	Mar. 31, 2024	Sep. 30, 2023	Sep. 30, 2024	Sep. 30, 2023	Feb. 29, 2024	Jan. 31, 2023
Share-Based Compensation Arrangement by Share-Based Payment Award, Options, Exercises in Period (in shares)			39,750	1,403	306,826					
Employee Stock Purchase Plan, Maximum Number of Issuable Shares (in shares)		1,500,000								
Stock Issued During Period, Shares, Employee Stock Purchase Plans (in shares)				97,747			257,575			
Share-Based Payment Arrangement, Expense Performance Shares [Member]			\$ 6,137			\$ 1,387	\$ 10,888	\$ 3,081		
Share-Based Payment Arrangement, Expense 2014 Equity Incentive Plan [Member]			\$ 2,500				\$ 2,500	\$ 0		
Share-based Compensation Arrangement by Share-based Payment Award, Number of Additional Shares Authorized (in shares)		4,900,000								
Share-Based Compensation Arrangement by Share-Based Payment Award, Number of Shares Authorized (in shares)			9,932,883				9,932,883			
Share-based Compensation Arrangement by Share-based Payment Award, Number of Shares Reserved for Issuance, Incremental Percentage of Capital Stock Outstanding Increase in Common Stock Reserved for Issuance (in shares)	1,661,386									
Share-Based Compensation Arrangement by Share-Based Payment Award, Options, Exercises in Period (in shares)			27,750			0	761,500	0		
The 2023 Plan [Member]										
Share-Based Compensation Arrangement by Share-Based									4,500,000	1,500,000

Payment Award, Number of
Shares Authorized (in shares)

Note H - Stock-based Compensation - Stock-based Compensation Expense (Details) - USD (\$) \$ in Thousands	3 Months Ended		9 Months Ended	
	Sep. 30, 2024	Sep. 30, 2023	Sep. 30, 2024	Sep. 30, 2023
Stock based compensation expense	\$ 6,137	\$ 1,387	\$ 10,888	\$ 3,081
Research and Development Expense [Member]				
Stock based compensation expense	1,441	720	3,322	2,023
General and Administrative Expense [Member]				
Stock based compensation expense	\$ 4,696	\$ 667	\$ 7,566	\$ 1,058

**Note I - Fair Value of
Financial Instruments -
Assets and Liabilities
Measured at Fair Value on
Recurring Basis (Details) -
Fair Value, Recurring
[Member] - USD (\$)
\$ in Thousands**

Sep. 30, 2024 Dec. 31, 2023

Total liabilities	\$ 18,702	\$ 23,362
Total assets	41,442	24,688
US Treasury Securities [Member]		
Total assets	35,337	24,688
Corporate Bond Securities [Member]		
Total assets	6,105	
Fair Value, Inputs, Level 1 [Member]		
Total liabilities	0	0
Total assets	35,337	24,688
Fair Value, Inputs, Level 1 [Member] US Treasury Securities [Member]		
Total assets	35,337	24,688
Fair Value, Inputs, Level 1 [Member] Corporate Bond Securities [Member]		
Total assets	0	
Fair Value, Inputs, Level 2 [Member]		
Total liabilities	0	0
Total assets	6,105	0
Fair Value, Inputs, Level 2 [Member] US Treasury Securities [Member]		
Total assets	0	0
Fair Value, Inputs, Level 2 [Member] Corporate Bond Securities [Member]		
Total assets	6,105	
Fair Value, Inputs, Level 3 [Member]		
Total liabilities	18,702	23,362
Total assets	0	0
Fair Value, Inputs, Level 3 [Member] US Treasury Securities [Member]		
Total assets	0	0
Fair Value, Inputs, Level 3 [Member] Corporate Bond Securities [Member]		
Total assets	0	
CVR Liability [Member]		
Total liabilities	4,800	7,262
CVR Liability [Member] Fair Value, Inputs, Level 1 [Member]		
Total liabilities	0	0
CVR Liability [Member] Fair Value, Inputs, Level 2 [Member]		
Total liabilities	0	0
CVR Liability [Member] Fair Value, Inputs, Level 3 [Member]		
Total liabilities	4,800	7,262
Warrants [Member]		

Total liabilities	13,902	16,100
Warrants [Member] Fair Value, Inputs, Level 1 [Member]		
Total liabilities	0	0
Warrants [Member] Fair Value, Inputs, Level 2 [Member]		
Total liabilities	0	0
Warrants [Member] Fair Value, Inputs, Level 3 [Member]		
Total liabilities	\$ 13,902	\$ 16,100

**Note I - Fair Value of
Financial Instruments - Fair
Value Assumptions for
Warrant Liabilities (Details)**

	9 Months Ended Sep. 30, 2024 \$ / shares	12 Months Ended Dec. 31, 2023 \$ / shares
Weighted average fair value (in dollars per share)	\$ 2.54	\$ 2.94
Measurement Input, Expected Dividend Rate [Member]		
Risk-free interest rate	0	0
Minimum [Member] Measurement Input, Risk Free Interest Rate [Member]		
Risk-free interest rate	0.0351	0.0376
Minimum [Member] Measurement Input, Price Volatility [Member]		
Risk-free interest rate	0.6176	0.6201
Minimum [Member] Measurement Input, Expected Term [Member]		
Risk-free interest rate	1.27	2
Maximum [Member] Measurement Input, Risk Free Interest Rate [Member]		
Risk-free interest rate	0.0381	0.0412
Maximum [Member] Measurement Input, Price Volatility [Member]		
Risk-free interest rate	0.8244	0.9242
Maximum [Member] Measurement Input, Expected Term [Member]		
Risk-free interest rate	4.14	4.9

**Note I - Fair Value of
Financial Instruments -
Reconciliation of Beginning
and Ending Balances for
Derivative and Warrant
Liability Measured at Fair
Value on Recurring Basis
(Details)
\$ in Thousands**

9 Months Ended

**Sep. 30, 2024
USD (\$)**

<u>Balance</u>	\$ 16,100
<u>Change in fair value measurement</u>	(2,198)
<u>Balance</u>	\$ 13,902

Note J - Net Loss Per Share - Anti-dilutive Securities (Details) - shares	3 Months Ended		6 Months Ended		9 Months Ended
	Sep. 30, 2024	Sep. 30, 2023	Jun. 30, 2024	Sep. 30, 2023	
Antidilutive securities (in shares)	14,471,534	11,514,529	14,471,534	11,514,529	
Share-Based Payment Arrangement [Member]					
Antidilutive securities (in shares)	8,987,997	7,262,039	8,987,997	7,262,039	
Warrants to Purchase Common Stock [Member]					
Antidilutive securities (in shares)	5,483,537	4,252,490	5,483,537	4,252,490	

Note J - Net Loss Per Share - Earnings Per Share (Details) - USD (\$) \$ / shares in Units, \$ in Thousands	3 Months Ended						9 Months Ended	
	Sep. 30, 2024	Jun. 30, 2024	Mar. 31, 2024	Sep. 30, 2023	Jun. 30, 2023	Mar. 31, 2023	Sep. 30, 2024	Sep. 30, 2023
Net Income (Loss) Attributable to Parent	\$ (33,225)	\$ (19,925)	\$ (16,622)	\$ (10,367)	\$ (2,573)	\$ (13,217)	\$ (69,772)	\$ (26,157)
Weighted average number of shares of common stock outstanding, basic and diluted (in shares)	47,808,817			34,724,614			43,843,851	34,364,075
Basic and diluted net loss per share of common stock (in dollars per share)	\$ (0.69)			\$ (0.3)			\$ (1.59)	\$ (0.76)

**Note K - Leases (Details
Textual)**

**9 Months Ended
Sep. 30, 2024**

Lessee, Lease, Option to Extend, Maximum Term (Year)	5 years
Lessee, Lease, Option to Terminate, Term (Year)	1 year
Minimum [Member]	
Lessee, Lease, Remaining Term of Contract (Year)	1 year
Maximum [Member]	
Lessee, Lease, Remaining Term of Contract (Year)	3 years

Note K - Leases - Lease Cost (Details) - USD (\$) \$ in Thousands	3 Months Ended		9 Months Ended	
	Sep. 30, 2024	Sep. 30, 2023	Sep. 30, 2024	Sep. 30, 2023
Amortization of right-of-use assets	\$ 12	\$ 26	\$ 36	\$ 90
Interest on lease liabilities	0	0	0	0
Total finance lease cost	12	26	36	90
Operating lease cost	119	113	330	340
Short-term lease cost	59	55	177	165
Variable lease cost	26	13	39	39
Less: sublease income	(50)	(39)	(128)	(117)
Total lease costs	\$ 166	\$ 168	\$ 454	\$ 517

**Note K - Leases -
Supplement Cash Flow
Information Related to
Leases (Details) - USD (\$)**

9 Months Ended

Sep. 30, 2024 Sep. 30, 2023

\$ in Thousands

Financing cash flows from finance leases	\$ (0)	\$ 5
Operating cash flows from operating leases	405	426
Operating cash flows from short-term leases	177	165
Operating cash flows from variable lease costs	39	39
Finance leases	0	0
Operating leases	\$ 419	\$ 0

Note K - Leases -
Supplement Balance Sheet
Information Related to
Lease (Details) - USD (\$)
\$ in Thousands

Sep. 30, 2024 Dec. 31, 2023

Property and equipment, net	\$ 114	\$ 149
Other current liabilities	0	0
Other long-term liabilities	0	0
Lease liabilities, finance leases	0	0
Operating lease right-of-use assets	820	790
Total operating lease right-of-use assets	820	790
Current portion of operating lease liabilities	540	543
Operating lease liabilities, less current portion	483	456
Total operating lease liabilities	\$ 1,023	\$ 999
Operating leases (in years) (Year)	2 years	2 years
Finance leases	0.00%	14.30%
Operating leases	9.60%	7.60%
Property and Equipment, At Cost [Member]		
Property and equipment, at cost	\$ 1,031	\$ 1,031
Accumulated Depreciation and Amortization [Member]		
less: accumulated depreciation and amortization	\$ (917)	\$ (882)

**Note K - Leases - Maturities
of Lease Liabilities (Details) -**

USD (\$)

Sep. 30, 2024 Dec. 31, 2023

\$ in Thousands

<u>2024 (excluding the three months ended March 31, 2024), finance leases</u>	\$ 0	
<u>2024 (excluding the three months ended March 31, 2024), operating leases</u>	167	
<u>2025, finance leases</u>	0	
<u>2025, operating leases</u>	536	
<u>2026, finance leases</u>	0	
<u>2026, operating leases</u>	146	
<u>2027, finance leases</u>	0	
<u>2027, operating leases</u>	136	
<u>2028, finance leases</u>	0	
<u>2028, operating leases</u>	161	
<u>Thereafter, finance leases</u>	0	
<u>Thereafter, operating leases</u>	49	
<u>Total lease payments, finance leases</u>	0	
<u>Total lease payments, operating leases</u>	1,195	
<u>Less: future interest expense, finance leases</u>	0	
<u>Less: future interest expense, operating leases</u>	(172)	
<u>Lease liabilities, finance leases</u>	0	\$ 0
<u>Lease liabilities, operating leases</u>	\$ 1,023	\$ 999

Note L - Merger (Details Textual) \$ / shares in Units, \$ in Thousands	Nov. 22, 2023 shares	Nov. 17, 2023 USD (\$) \$ / shares shares	Aug. 30, 2023 USD (\$) (\$) shares	Jun. 16, 2023 USD (\$) \$ / shares shares	Jan. 31, 2023 USD (\$) (\$) shares	Mar. 14, 2022 USD (\$)	1 Months Ended	3 Months Ended	9 Months Ended	Sep. 30, 2024 USD (\$) shares	Dec. 31, 2023 USD (\$)	Oct. 31, 2023 USD (\$)
							Nov. 30, 2023 shares	Sep. 30, 2024 USD (\$) shares	Sep. 30, 2024 USD (\$)			
Stock Issued During Period, Shares, Acquisitions (in shares) shares							1,382,489					
Stock Issued During Period, Shares, New Issues (in shares) shares								10,615,385				
Stock Issued During Period, Value, New Issues								\$ 64,517				
The Investor Warrant [Member]												
Stock Issued During Period, Shares, New Issues (in shares) shares	917,934						917,934					
Class of Warrant or Right, Number of Securities Called by Warrants or Rights (in shares) shares	2,920,306						2,920,306					
SWK Loans [Member]												
Payments to Acquire Notes Receivable			\$ 12,000		\$ 12,000							
Stock Issued During Period, Shares, Acquisitions (in shares) shares			98,683		98,683							
Marathon Convertible Notes [Member]												
Stock Issued During Period, Shares, Acquisitions (in shares) shares				2,171,038								
Asset Acquisition, Share Price (in dollars per share) \$ / shares				\$ 5.0667								
Asset Acquisition, Consideration Transferred				\$ 11,000								
SWK Loans and Marathon Notes [Member]												
Payments to Acquire Notes Receivable		\$ 12,000										
Stock Issued During Period, Shares, Acquisitions (in shares) shares	2,270,000											
Asset Acquisition, Consideration Transferred,		\$ 11,500										

Equity Interest Issued and Issuable			
VWAP, Consecutive Trading Days (Year)	20 years		
Nantahala Note [Member]			
Debt Instrument, Face Amount	\$ 5,000	\$ 5,000	\$ 5,000
Acer Therapeutics Inc [Member] License Agreement and Termination Agreement [Member] Relief [Member]			
Maximum Royalty Percentage	20.00%		
Upfront Payment	\$ 10,000		
Addiitonal Payment on First Anniversary	1,500		
Maximum Royalty Obligation	\$ 45,000		
Acer Therapeutics Inc [Member] License Agreement and Termination Agreement [Member] Relief [Member] Europe [Member]			
Maximum Royalty Percentage	10.00%		
Acer Therapeutics Inc [Member] License Agreement and Termination Agreement [Member] Relief [Member] Worldwide, Excluding Europe [Member]			
Maximum Royalty Percentage	10.00%		
Acer Therapeutics Inc [Member] SWK Loans [Member]			
Proceeds from Issuance of Long-Term Debt		\$ 7,000	\$ 6,500
Maximum [Member] Acer Therapeutics Inc [Member] License Agreement and Termination Agreement [Member] Relief [Member]			
Contractual Obligation	\$ 56,500		
Bridge Loan [Member]			
Financing Receivable, Maximum Limit	18,000		\$ 18,000
Bridge Loan [Member] Minimum [Member]			
Financing Receivable, Maximum Limit	16,500		

Acer Therapeutics Inc [Member] Business Combination, Consideration Transferred, Equity Interests Issued and Issuable, Conversion Ratio	0.121			
Business Acquisition, Share Price (in dollars per share) \$ / shares	\$ 0.0001			
Business Combination, Contingent Consideration Arrangements, Range of Outcomes, Value, High	\$ 76,000			
Business Combination, Consideration Transferred Business Acquisition, Equity Interest Issued or Issuable, Number of Shares (in shares) shares	\$ 72,600			
Business Combination, Consideration Transferred, Equity Interests Issued and Issuable	2,960,000			
Business Combination, Consideration Transferred, Equity Interests Issued and Issuable	\$ 12,800			
Business Combination, Contingent Consideration, Liability	8,500	\$ 4,800	\$ 4,800	\$ 7,262
Repayments of Notes Payable Gain (Loss) on Contingent Consideration, Change in Fair Value	\$ 1,000		\$ 2,500	
Acer Therapeutics Inc [Member] Acer Warrants [Member] Stock Issued During Period, Shares, New Issues (in shares) shares	900,000			
Stock Issued During Period, Value, New Issues	\$ 4,000			
Acer Therapeutics Inc [Member] Bridge Loan [Member] Business Combination, Consideration Transferred, Other	\$ 17,800	\$ 16,500		

**Note L - Merger - Estimates
and Assumptions Related to
Fair Value of Assets and
Liabilities Acquired (Details)
- USD (\$)
\$ in Thousands**

	Sep. 30, 2024	Dec. 31, 2023	Nov. 17, 2023
Cash			\$ 575
Prepaid expenses			278
Other current assets			11
Inventory			9,376
Property, plant, and equipment			35
Other noncurrent assets			209
Approved product - OLPRUVA			68,000
?IPR&D - celiprolol			2,000
Goodwill acquired	\$ 4,701	\$ 4,701	
Business Combination, Recognized Identifiable Assets Acquired and Liabilities Assumed, Assets			85,185
Accounts payable and accrued expenses			10,881
Deferred collaboration funding			1,500
Operating lease liabilities			175
Business Combination, Recognized Identifiable Assets Acquired and Liabilities Assumed, Liabilities			12,556
Fair Value of Net Assets Acquired			72,629
Acer Therapeutics Inc [Member]			
Goodwill acquired			\$ 4,701

Note L - Merger - Pro Forma 3 Months Ended 9 Months Ended
Combined Results of
Operations (Details) - USD
(\\$)
\$ in Thousands

	Sep. 30, 2023	Sep. 30, 2023
Pro forma revenue	\$ 3,267	\$ 14,913
Pro forma net loss	\$ (25,900)	\$ (54,763)

Note L - Merger - Reconciliation of Contingent Consideration Liabilities (Details) - Acer Therapeutics Inc [Member] \$ in Thousands	9 Months Ended Sep. 30, 2024 USD (\$)
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<u>Balance</u>	\$ 7,262
<u>Change in fair value recognized in earnings</u>	(2,462)
<u>Balance</u>	\$ 4,800

Note M - Goodwill & Intangible Assets (Details Textual) - USD (\$) \$ in Thousands	3 Months Ended		6 Months Ended	9 Months Ended		
	Sep. 30, 2024	Sep. 30, 2023	Sep. 30, 2023	Sep. 30, 2024	Sep. 30, 2023	Dec. 31, 2023
Goodwill	\$ 4,701			\$ 4,701		\$ 4,701
Indefinite-Lived Intangible Assets (Excluding Goodwill)	\$ 2,000			\$ 2,000		\$ 2,000
Finite-Lived Intangible Asset, Useful Life (Year)	13 years			13 years		11 years
Amortization of Intangible Assets	\$ 1,545	\$ 0		\$ 4,619	\$ 0	
Finite-Lived Intangible Asset, Expected Amortization, Remainder of Fiscal Year	6,500			6,500		
Finite-Lived Intangible Asset, Expected Amortization, Year One	6,500			6,500		
Finite-Lived Intangible Asset, Expected Amortization, Year Two	6,500			6,500		
Finite-Lived Intangible Asset, Expected Amortization, Year Three	6,500			6,500		
Finite-Lived Intangible Asset, Expected Amortization, Year Four	6,500			6,500		
XOMA License Agreement [Member] Accrued Royalties	\$ 6,000			\$ 6,000		
Patents [Member] Finite-Lived Intangible Asset, Useful Life (Year)	13 years			13 years		
Patents [Member] XOMA License Agreement [Member] Amortization of Intangible Assets			\$ 0		\$ 0	
OLPRUVA [Member] Finite-Lived Intangible Assets, Net	\$ 62,600			\$ 62,600		\$ 67,200

