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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, 2026

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	**20-5894398**
(State or Other Jurisdiction of Incorporation or Organization)	**(I.R.S. Employer Identification No.)**
101 Federal Street, Boston, MA	**02110**
(Address of Principal Executive Offices)	**(Zip Code)**

(888) 958-1253
(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 (§232.405 of this chapter) 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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

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

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 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 October 30, 2026, the registrant had 59,504,621 shares of common stock outstanding.

ZEVRA THERAPEUTICS, INC.
FORM 10-Q

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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 (the "Securities Act"), and the Securities Exchange Act of 1934, as amended (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," "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 outcomes of the events described in these forward-looking statements are subject to risks, uncertainties and other factors described in "Risk Factors" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2025, filed with the SEC on March 9, 2026 (the "Annual Report on Form 10-K"), as supplemented in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, filed with the SEC on May 6, 2026 (the "Q1 2026 Form 10-Q"), and elsewhere in this report. 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, and 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 commercialize and the timing of commercializing our products and product candidates, if approved;*
- *the potential therapeutic benefits and effectiveness of our products and product candidates;*
- *the progress of, timing of, and expected amount of expenses associated with our commercialization, research, and development activities;*
- *the size and characteristics of the markets that may be addressed by our products and product candidates;*
- *the expected timing of clinical trials for our product candidates and the availability of data and results of those trials;*
- *the progress of, outcome of and timing of any regulatory approval for any of our product candidates;*
- *our expectations regarding federal, state and foreign tax, legal and regulatory requirements;*
- *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;*
- *our expectations as to future financial performance, expense levels and liquidity sources;*
- *the sufficiency of our cash resources to fund our operating expenses and capital investment requirements for any period;*
- *our ability to raise additional funds if needed on commercially reasonable terms, or at all, in order to support our continued operations; 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 as supplemented in the Q1 2026 Form 10-Q, 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 developments, including 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. and its subsidiaries. We have proprietary rights to a number of trademarks and service marks used in this Quarterly Report on Form 10-Q that are important to our business, including MIPLYFFA® and its related logo, OLPRUVA® and its related logo, LAT®, and the Zevra companies' logos. In addition, Zevra Therapeutics® and Zevra® are both registered trademarks of the Company. 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.

PART I — FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

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

	September 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 122,148	$ 62,406
Investments, current	73,420	128,605
Accounts and other receivables	19,943	23,258
Prepaid expenses and other current assets	7,257	6,998
Inventories, current	3,070	1,740
Total current assets	225,838	223,007
Investments, noncurrent	75,323	47,879
Inventories, noncurrent	—	879
Property and equipment, net	816	489
Operating lease right-of-use assets	1,423	1,212
Goodwill	4,701	4,701
Intangible assets, net	5,474	6,421
Other long-term assets	—	143
Total assets	$ 313,575	$ 284,731
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 13,672	$ 11,598
Current portion of operating lease liabilities	626	419
Current portion of discount and rebate liabilities	7,223	12,188
Current portion of income tax payable	18,532	13,710
Other current liabilities	1,316	1,362
Total current liabilities	41,369	39,277
Long-term debt	—	61,928
Warrant liability	7,712	9,575
Income tax payable	7,304	7,029
Operating lease liabilities, less current portion	602	859
Discount and rebate liabilities, less current portion	14,792	9,693
Other long-term liabilities	2,873	1,713
Total liabilities	74,652	130,074
Commitments and contingencies (Note M)		
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, 2026, or December 31, 2025	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized; 61,015,651 shares issued and 59,439,959 shares outstanding as of September 30, 2026; 58,338,319 shares issued and 56,854,781 shares outstanding as of December 31, 2025	6	6
Additional paid-in capital	609,618	588,458
Treasury stock, at cost	(10,983)	(10,983)
Accumulated deficit	(356,210)	(422,060)
Accumulated other comprehensive loss	(3,508)	(764)
Total stockholders' equity	238,923	154,657
Total liabilities and stockholders' equity	$ 313,575	$ 284,731

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,	
	2026	2025	2026	2025
Revenue, net	$ 37,756	$ 26,063	$ 113,639	$ 72,345
Cost of product revenue (excluding $315 and $315 in intangible asset amortization for the three months ended September 30, 2026, and 2025, respectively, and $947 and $3,546 in intangible asset amortization for the nine months ended September 30, 2026, and 2025, respectively, shown separately below)	(1,794)	(1,238)	(5,216)	(14,962)
Intangible asset amortization	(315)	(315)	(947)	(3,546)
Impairment of intangible assets	—	—	—	(58,710)
Gain on sale of future royalties, intellectual property, and other assets, net	—	—	43,314	—
Operating expenses:				
Research and development	(4,937)	(3,432)	(13,815)	(10,123)
Selling, general and administrative	(19,130)	(16,935)	(56,472)	(57,262)
Total operating expenses	(24,067)	(20,367)	(70,287)	(67,385)
Income (loss) from operations	11,580	4,143	80,503	(72,258)
Other income (expense):				
Gain on sale of PRV	—	—	—	148,325
Loss on extinguishment of debt	—	—	(2,756)	—
Loss on derivative liability	—	—	(7,216)	—
Interest expense	—	(2,051)	(1,711)	(6,029)
Fair value adjustment related to warrant and CVR liability	5,143	(5,506)	(297)	(1,379)
Fair value adjustment related to investments	(39)	124	(395)	119
Interest and other income, net	3,034	2,313	9,189	5,234
Total other income (expense)	8,138	(5,120)	(3,186)	146,270
Income (loss) before income taxes	19,718	(977)	77,317	74,012
Income tax (expense) benefit	(510)	433	(11,467)	(2,948)
Net income (loss)	$ 19,208	$ (544)	$ 65,850	$ 71,064
Net income (loss) per share of common stock:				
Basic	$ 0.31	$ (0.01)	$ 1.07	$ 1.20
Diluted	$ 0.26	$ (0.01)	$ 1.03	$ 1.16
Weighted-average shares of common stock outstanding:				
Basic	59,407,392	55,951,572	58,993,342	54,949,483
Diluted	61,732,366	55,951,572	61,103,394	56,782,763

See accompanying notes to unaudited condensed consolidated financial statements.

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

	Three months ended September 30,		Nine months ended September 30,	
	2026	2025	2026	2025
Net income (loss)	$ 19,208	$ (544)	$ 65,850	$ 71,064
Other comprehensive (loss) income:				
Foreign currency translation adjustment	(618)	12	(1,967)	(3,047)
Net unrealized loss on available-for-sale securities	(673)	—	(777)	—
Other comprehensive (loss) income	(1,291)	12	(2,744)	(3,047)
Comprehensive income (loss)	$ 17,917	$ (532)	$ 63,106	$ 68,017

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, 2026	$ 6	$ 588,458	$ (10,983)	$ (422,060)	$ (764)	$ 154,657
Net income	—	—	—	37,890	—	37,890
Stock-based compensation expense	—	3,105	—	—	—	3,105
Issuance of common stock for stock awards	—	25	—	—	—	25
Issuance of common stock for stock warrants exercised	—	11,160	—	—	—	11,160
Other comprehensive loss	—	—	—	—	(1,033)	(1,033)
Balance as of March 31, 2026	$ 6	$ 602,748	$ (10,983)	$ (384,170)	$ (1,797)	$ 205,804
Net income	—	—	—	8,752	—	8,752
Stock-based compensation expense	—	2,969	—	—	—	2,969
Issuance of common stock as part of the Employee Stock Purchase Plan	—	428	—	—	—	428
Issuance of common stock for stock awards	—	119	—	—	—	119
Other comprehensive loss	—	—	—	—	(420)	(420)
Balance as of June 30, 2026	$ 6	$ 606,264	$ (10,983)	$ (375,418)	$ (2,217)	$ 217,652
Net income	—	—	—	19,208	—	19,208
Stock-based compensation expense	—	2,672	—	—	—	2,672
Issuance of common stock for stock awards	—	682	—	—	—	682
Other comprehensive loss	—	—	—	—	(1,291)	(1,291)
Balance as of September 30, 2026	$ 6	$ 609,618	$ (10,983)	$ (356,210)	$ (3,508)	$ 238,923

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, 2025	$ 5	$ 555,302	$ (10,983)	$ (505,289)	$ 631	$ 39,666
Net loss	—	—	—	(3,099)	—	(3,099)
Stock-based compensation expense	—	3,115	—	—	—	3,115
Issuance of common stock in exchange for consulting services	—	75	—	—	—	75
Issuance of common stock for stock awards	—	1,979	—	—	—	1,979
Other comprehensive loss	—	—	—	—	(713)	(713)
Balance as of March 31, 2025	$ 5	$ 560,471	$ (10,983)	$ (508,388)	$ (82)	$ 41,023
Net income	—	—	—	74,707	—	74,707
Stock-based compensation expense	—	2,464	—	—	—	2,464
Issuance of common stock as part of the Employee Stock Purchase Plan	—	413				413
Issuance of common stock for stock awards	1	968	—	—	—	969
Other comprehensive loss	—	—	—	—	(2,346)	(2,346)
Balance as of June 30, 2025	$ 6	$ 564,316	$ (10,983)	$ (433,681)	$ (2,428)	$ 117,230
Net loss	—	—	—	$ (544)	—	(544)
Stock-based compensation expense	—	2,771	—	—	—	2,771
Issuance of common stock for options exercised or RSUs vested	—	573	—	—	—	573
Issuance of common stock for stock warrants exercised	—	13,123	—	—	—	13,123
Other comprehensive income	—	—	—	—	12	12
Balance as of September 30, 2025	$ 6	$ 580,783	$ (10,983)	$ (434,225)	$ (2,416)	$ 133,165

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,	
	2026	2025
Cash flows from operating activities:		
Net income (loss)	$ 65,850	$ 71,064
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:		
Stock-based compensation expense	8,746	8,350
Impairment of intangible assets	—	58,710
Inventory obsolescence charge	485	11,681
Income tax (expense) benefit	5,606	2,948
Depreciation and amortization expense	1,110	3,692
Non-cash lease expense	413	—
Non-cash interest expense	342	2,037
Fair value adjustment related to warrant and CVR liability	297	1,379
Accretion on investments	(1,202)	(2,271)
Fair value adjustment related to investments	395	(119)
Loss on extinguishment of debt	2,756	—
Loss on derivative liability	7,216	—
Cash paid to settle derivative instrument and payoff premium	(9,016)	—
Settlement of royalties to Aquestive	(4,893)	—
Paid-in-kind interest	(3,134)	—
Gain on sale of future royalties, intellectual property, and other assets, net	(43,314)	—
Consulting fees paid in common stock	—	75
(Gain) loss on foreign currency exchange rates	(2,705)	126
Gain on sale of PRV	—	(148,325)
Change in assets and liabilities:		
Accounts and other receivables	2,841	(5,610)
Prepaid expenses and other current assets	(260)	(3,342)
Inventories	(962)	(780)
Other long-term assets	4	—
Operating lease right-of-use assets	(162)	476
Accounts payable and accrued expenses	1,453	(15,999)
Discount and rebate liabilities	666	9,949
Operating lease liabilities	(501)	(546)
Other liabilities	1,190	(609)
Net cash provided by (used in) operating activities	33,221	(7,114)
Cash flows from investing activities:		
Sale of future royalties, intellectual property, and other assets, net	48,935	—
Purchases of property and equipment	(490)	(866)
Disposals of property and equipment	—	589
Purchases of investments	(160,449)	(222,823)
Sales and maturities of investments	188,222	91,000
Proceeds from sale of PRV	—	150,000
Net cash provided by investing activities	76,218	17,900
Cash flows from financing activities:		
Repayment of debt	(60,092)	—
Payments for employee taxes related to stock awards	(1,514)	—
Proceeds from Employee Stock Purchase Plan	434	413
Proceeds from issuance of common stock for options exercised	2,333	3,520
Proceeds from issuance of common stock for warrants exercised	9,171	5,957
Payments of principal on insurance financing arrangements	—	(372)
Net cash (used in) provided by financing activities	(49,668)	9,518
Effect of exchange rate changes on cash and cash equivalents	(29)	350
Net increase in cash and cash equivalents	59,742	20,654
Cash and cash equivalents, beginning of period	62,406	33,785
Cash and cash equivalents, end of period	$ 122,148	$ 54,439
Supplemental cash flow information:		
Cash paid for interest	$ 1,370	$ 3,992
Right-of-use assets obtained in exchange for lease liabilities	460	1,115
Cash paid for income taxes	5,860	—

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" or "Zevra") is a commercial-stage company with a late-stage pipeline committed to redefining what is possible in bringing life-changing therapeutics to people living with rare diseases. The Company is focused on broadening access through geographic expansion opportunities, progressing its pipeline toward key milestones, and delivering meaningful therapeutics.

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. The Company's other commercial stage asset, OLPRUVA® (sodium phenylbutyrate) for oral suspension, is approved by the FDA for the treatment of certain urea cycle disorders ("UCDs").

Arimoclomol has been granted orphan medicinal product designation for the treatment of NPC by the European Commission. The Company is pursuing regulatory approval in the European Union ("E.U.") and filed a Marketing Authorization Application ("MAA") with the European Medicines Agency ("EMA") in July 2025. In July 2026, the Committee for Medicinal Products for Human Use (CHMP) of the EMA adopted a negative opinion regarding the MAA. Under European regulatory procedures, the Company has requested a re-examination of the CHMP opinion.

Additionally, the Company is currently conducting a Phase 3 clinical trial of celiprolol for the treatment of Vascular Ehlers-Danlos syndrome ("VEDS") in patients with a confirmed type III collagen mutation.

On March 13, 2026, the Company entered into an Asset Purchase and Settlement Agreement (the "Commave Settlement Agreement") with Commave Therapeutics SA ("Commave") to sell certain assets of the Company to Commave and to resolve pending litigation related to claims arising under the Collaboration and License Agreement between the parties dated September 3, 2019, as amended (the "AZSTARYS License Agreement").

Pursuant to the Commave Settlement Agreement, Commave agreed to pay a total of $50.0 million, and the Company agreed to sell to Commave all of the Company's rights to certain assets relating to the Company's serdexmethylphenidate ("SDX") portfolio, including AZSTARYS and KP1077. Under the March 2012 termination agreement with Aquestive Therapeutics, Inc. ("Aquestive") (the "Aquestive Termination Agreement"), Aquestive has the right to receive an amount equal to 10% of any value generated by AZSTARYS and any product candidates containing SDX, including the sales proceeds under the Commave Settlement Agreement. Commave paid an aggregate of $45.0 million during the first quarter of 2026, consisting of $40.5 million paid to the Company and $4.5 million paid directly to Aquestive on behalf of the Company. In April 2026, Commave paid the remaining $5.0 million, consisting of $4.5 million to the Company and $0.5 million directly to Aquestive on behalf of the Company. In accordance with ASC 610-20, *Gains and Losses from the Derecognition of Nonfinancial Assets*, the net proceeds from the sale were recorded as a gain on sale of future royalties, intellectual property, and other assets, net of $43.3 million in the Company's unaudited condensed consolidated statements of operations for the nine months ended September 30, 2026. In addition, under the Commave Settlement Agreement, the Company and Commave have agreed to terminate the AZSTARYS License Agreement in its entirety.

Basis of Presentation

The Company prepared the unaudited condensed consolidated financial statements in accordance with accounting principles generally accepted in the United States of America ("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. All significant intercompany accounts and transactions have been eliminated in consolidation.

Registration Statements on Form S-3

On February 5, 2024, Zevra filed a registration statement on Form S-3 (File No. 333-276856) registering the resale of an aggregate of 2,269,721 shares of Zevra's common stock by certain stockholders (the "Resale Registration Statement"). The Resale Registration Statement 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 June 2024 Registration Statement was declared effective on June 13, 2024.

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, 2026, no shares have been issued or sold under the 2024 ATM Agreement.

Reclassifications

Certain reclassifications were made to historical periods' unaudited condensed consolidated financial statements to conform to the classifications used in the current year. These reclassifications had no impact on the consolidated net income (loss), changes in stockholders' equity, or cash flows previously reported.

B. Summary of Significant Accounting Policies

Use of Estimates

The preparation of these unaudited condensed consolidated 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 the warrant liability and discount and rebate liabilities, the valuation of embedded derivatives, and the net realizable value of inventory, 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.

Concentration of Credit Risk

Financial instruments that potentially expose the Company to concentrations of credit risk consist principally of cash on deposit and investments with multiple financial institutions, the balances of which frequently exceed insured limits, and accounts receivable, which are concentrated amongst a limited number of customers.

Investments

The Company maintains investment securities that are classified as available-for-sale securities, primarily consisting of U.S. Treasury securities and corporate bonds. In accordance with Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 825, *Financial Instruments,* the Company has historically elected the fair value option based on the Company's liquidity needs. For purchases of debt securities in previous periods, applying fair value accounting most accurately represented the Company's investment strategy due to the fact that excess cash was being invested for the purpose of funding future operations. In such circumstances, 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. As of September 30, 2026, and December 31, 2025, the Company held securities under the fair value option with an aggregate fair value of $33.0 million and $176.5 million, respectively, that contained an aggregate unrealized loss of $(0.4) million and an aggregate unrealized gain of $0.1 million, respectively, which is included on the unaudited condensed consolidated statements of operations.

The Company did not elect to apply the fair value option to individual securities purchased in 2026 due to its strengthening financial position and decreased need for immediate liquidity. For such securities, unrealized gains and losses are included in net unrealized gains (losses) on available-for-sale securities on the unaudited condensed consolidated statements of comprehensive income (loss). 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. As of September 30, 2026, securities for which the fair value option was not elected had an aggregate fair value and amortized cost basis of $115.7 million, with an aggregate unrealized loss of $(0.8) million included in the unaudited condensed consolidated statements of comprehensive income (loss). The fair value of these securities, along with those for which the fair value option was elected, are included in the investments, current, and investments, noncurrent, lines on the unaudited condensed consolidated balance sheets. We determined that none of these recently purchased available-for-sale securities were other-than-temporarily impaired as of September 30, 2026. Therefore, we believe that it is more likely than not that the investments will be held until maturity or a forecasted recovery of fair value.

For securities held at September 30, 2026, approximately $73.4 million mature within one year and approximately $75.3 million mature in one to three years.

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 members of 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 judgment in determining whether these interests, in the aggregate, are considered potentially significant to the VIE. As of September 30, 2026, and December 31, 2025, the Company identified Acer Therapeutics, Inc. (“Acer”) to be the Company's sole interest in a VIE. 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 and consolidates the assets and liabilities of the VIE.

Gain on Sale of PRV

The Company received a transferable rare pediatric disease priority review voucher (“PRV”) in conjunction with the FDA approval of MIPLYFFA. On February 26, 2025, the Company and its subsidiary, Zevra Denmark A/S, entered into an asset purchase agreement with a buyer, pursuant to which the Company agreed to sell the PRV to the buyer for aggregate proceeds of $150.0 million, payable in cash, upon the closing of the sale. On April 1, 2025, the asset sale was consummated and title of the PRV transferred to the buyer, resulting in net proceeds of $148.3 million to the Company. The PRV did not have a carrying value at the time of sale. In accordance with ASC 610-20, *Gains and Losses from the Derecognition of Nonfinancial Assets,* the net proceeds from the sale were recorded as a gain on sale of PRV in the Company's unaudited condensed consolidated statements of operations in the nine months ended September 30, 2025.

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 the performance obligations; and 5) recognizing revenue when the performance obligations have been fulfilled.

Product Revenues, net

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

In accordance with ASC 606, the Company recognizes revenue when fulfilling its performance obligation 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 customer acceptance has been received. 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. All estimated reserve liabilities related to commercial products are recorded within the current portion of discount and rebate liabilities in the unaudited condensed consolidated balance sheets.

Expanded Access Program

Net revenue includes revenue from the sale of arimoclomol provided for the treatment of NPC under an expanded access program (“EAP”) in France and in select territories outside Europe. An EAP is a program giving specific patients access to a drug that 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 programs. Further, to be considered for the expanded access 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 global 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 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 reimbursements under the EAP in France, but the final price depends on the terms and conditions negotiated with the French Health Authorities, following market authorization. Any excess in the price charged by the manufacturer compared to the price agreed with the health authorities once the medicinal product is authorized in France must be repaid. The potential repayment is considered in the clawback liability. 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 estimating the expected final 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 medicinal 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 medicinal products. The limited available relevant market information for directly comparable commercialized medicines within rare disease increases the uncertainty in management's estimate.

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 it 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.

Upfront 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.

Acquired IPR&D and Milestones Expenses

In an asset acquisition, payments incurred prior to regulatory approval to acquire rights to in-process research and development ("IPR&D") projects are expensed as acquired IPR&D and milestones expense in the unaudited condensed consolidated statements of operations 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 intangible asset amortization over the remaining useful life of the related product.

Embedded Derivative

The Company evaluates its financial instruments to determine if such instruments are derivatives or contain features that qualify as embedded derivatives. For derivative instruments that are bifurcated from a host contract, the Company recognizes them as either assets or liabilities in the unaudited condensed consolidated balance sheets and measures them at fair value. Embedded derivatives are remeasured at each reporting date, with changes in fair value recognized in earnings within loss on derivative liability in the unaudited condensed consolidated statements of operations. The Company initially assesses the probability of triggering events for bifurcated embedded derivatives in determining fair value. The probability is assessed at each reporting period.

Inventories

The value of inventories is recorded at net realizable value. The Company determines the cost of its inventories, which include amounts related to materials and manufacturing overhead, on a first-in, first-out basis. Inventories that are not expected to be sold within 12 months are classified as inventories, noncurrent.

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 products 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. We capitalize inventory costs associated with our products prior to regulatory approval when, based on management's judgment, future commercialization is considered probable and the future economic benefit is expected to be realized; otherwise, such costs are expensed as research and development. The determination to capitalize inventory costs is based on various factors, including status and expectations of the regulatory approval process, any known safety or efficacy concerns, potential labeling restrictions, and any other impediments to obtaining regulatory approval. The Company had no pre-approval inventory on its unaudited condensed consolidated balance sheets as of September 30, 2026, or December 31, 2025. Inventory used in clinical trials is also expensed as research and development expense when selected for such use. Inventory that can be used in the production of either 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, 2026, and December 31, 2025, 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 unaudited condensed consolidated statements of operations. 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 products are subject to strict quality control and monitoring, which is performed 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, 2026, the Company recognized $0.0 million and $0.5 million, respectively, related to write-downs for unsaleable inventory. For the three and nine months ended September 30, 2025, the Company recognized a charge of approximately $11.7 million related to write-downs for unsaleable inventory.

Cost of Product Revenue

The components of cost of product revenue are royalties and expenses directly attributable to revenue. To date, the Company has generated revenue from product sales of MIPLYFFA and OLPRUVA, reimbursements received under the global EAP, royalties or net sales milestone payments generated under the AZSTARYS License Agreement, and consulting agreements.

Prior to our acquisition of the assets of Orphazyme A/S ("Orphazyme") in May 2022, Orphazyme had 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 a mid-single digit percentage royalty with respect to net sales of MIPLYFFA as well as milestone payments based on future potential sales and regulatory milestones, including a $4.0 million regulatory milestone payment upon approval in the E.U. and a $5.0 million sales milestone payment upon annual sales of $100.0 million. XOMA was acquired by Ligand Pharmaceuticals in July 2026.

On August 28, 2023, Acer and Relief Therapeutics SA ("Relief") entered into a termination agreement (the "Relief Termination Agreement"). Pursuant to the Relief Termination Agreement, Zevra is obligated to pay royalties of 10% of U.S. net sales of OLPRUVA up to a maximum of $45.0 million, plus specified regulatory milestones, for total payments to Relief of up to $56.5 million. In April 2025, Relief sold the rights to this royalty to Soleus Capital Management L.P., and in December 2025, Relief merged with NeuroX Group SA, and the combined company now operates as MindMaze Therapeutics Holding SA.

Accounts and Other Receivables

Accounts and other receivables consist of receivables from MIPLYFFA and OLPRUVA product sales, receivables under the AZSTARYS License Agreement, the global EAP, and other receivables due to the Company. Receivables under the AZSTARYS License Agreement are recorded for amounts due to the Company related to royalties on product sales. Receivables under the global EAP are recorded for revenue from arimoclomol in France and select territories outside of Europe. The Company provides reserves against receivables for estimated losses that may result from a customer's inability to pay. Receivables are evaluated to determine if any reserve or allowance should be recorded based on consideration of the current economic environment, expectations of future economic conditions, specific circumstances and the Company's own historical collection experience. Amounts determined to be uncollectible are charged or written-off against the reserve.

Research and Development

Major components of research and development include costs of clinical trials and related clinical manufacturing costs, cash compensation, stock-based compensation, costs of drug development, costs of materials and supplies, overhead costs, regulatory and compliance costs, and fees paid to consultants and other entities that conduct certain research and development activities on the Company's behalf. Costs incurred in research and development are expensed as incurred.

The Company records nonrefundable advance payments it makes for future research and development activities as prepaid expenses. Prepaid expenses are recognized as expense in the unaudited condensed consolidated statements of operations as the Company receives the related goods or services.

The Company enters into contractual agreements with third-party vendors who provide research and development, manufacturing, and other services in the ordinary course of business. Some of these contracts are subject to milestone-based invoicing and services are completed over an extended period of time. The Company records liabilities under these contractual commitments when an obligation has been incurred. This accrual process involves reviewing open contracts and purchase orders, communicating with the applicable

personnel to identify services that have been performed and estimating the level of service performed and the associated cost when the Company has not yet been invoiced or otherwise notified of actual cost. The majority of the service providers invoice the Company monthly in arrears for services performed. The Company makes estimates of the accrued expenses as of each balance sheet date based on the facts and circumstances known. The Company periodically confirms the accuracy of the estimates with the service providers and makes adjustments, if necessary.

Patent Costs

Patent costs, including related legal costs, are expensed as incurred and recorded within general and administrative expenses on the unaudited condensed consolidated statements of operations.

Income Taxes

The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the financial reporting and tax basis of assets and liabilities, as well as for operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using the tax rates that are expected to apply to taxable income for the years in which those tax assets and liabilities are expected to be realized or settled. Valuation allowances are recorded to reduce deferred tax assets to the amount the Company believes is more likely than not to be realized.

The Company is subject to taxation in the United States (including federal, state, and local jurisdictions) and the Kingdom of Denmark. Generally, the Company is subject to examination by tax jurisdictions from 2021 to 2024 tax years as the statute of limitations (excluding net operating loss carryforwards) are 3 to 4 years in the United States and Kingdom of Denmark. Tax liabilities may arise from interpretations and judgments made by the Company with regard to transfer pricing in the application of the relevant statutes, regulations, tax rulings and case law across the various jurisdictions. The Company uses significant judgment in (1) determining whether the technical merits of tax positions taken in the various jurisdictions are more-likely-than-not to be sustained based on applicable tax law and (2) measuring the related amount of tax liability that qualifies for recognition. The United States federal and state tax jurisdictions can audit the net operating loss carryforwards from the tax years in which the statute of limitations has expired but can only adjust the net operating loss carryforwards. No income tax returns are currently under examination by United States taxing authorities.

Uncertain tax positions are recognized only when the Company believes it is more likely than not that the tax position will be upheld on examination by the taxing authorities based on the merits of the position. The Company recognizes interest and penalties, if any, related to unrecognized income tax uncertainties in income tax expense.

On December 22, 2017, the U.S. government enacted H.R. 1, “An Act to provide for reconciliation pursuant to titles II and V of the concurrent resolution on the budget for fiscal year 2018” (“Tax Act”). Effective January 1, 2018, the Tax Act provides for a new global intangible low-taxed income (“GILTI”) provision. Under the GILTI provision, certain foreign subsidiary earnings in excess of an allowable return on the foreign subsidiary’s tangible assets are included in U.S. taxable income. The Company has not recorded any deferred taxes for future GILTI inclusions as any future inclusions are expected to be treated as a period expense.

On July 4, 2025, the One Big Beautiful Bill Act (the “OBBB”), which includes a broad range of tax reform provisions, was signed into law in the United States. This includes a net Controlled Foreign Corporation (“CFC”) tested income (“NCTI”) provision, which is an update of the GILTI provision that was effective under H.R. 1. The Company has not recorded any deferred taxes for future NCTI inclusions as any future inclusions are expected to be treated as a period expense. The Company continues to assess the impact of the OBBB but currently does not expect the OBBB to have a material impact on the Company's estimated annual effective tax rate in 2026.

Stock-Based Compensation

The Company measures and recognizes compensation expense for all stock-based payment awards made to employees, officers and directors based on the estimated fair values of the awards as of the grant date. The Company records the value of the portion of the award that is ultimately expected to vest as expense over the requisite service period. The Company also accounts for equity instruments issued to non-employees using a fair value approach under ASC subtopic 505-50. The Company values equity instruments and stock options granted using the Black-Scholes-Merton (“BSM”) option pricing model.

Earnings per Share

The Company uses the two-class method to compute net income (loss) per common share because the Company has issued securities, other than common stock, that contractually entitle the holders to participate in dividends and earnings of the Company. 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. Holders of each series of the Company's convertible preferred stock, if applicable, and select warrants are entitled to participate in distributions, when and if declared by the board of directors, that are made to common stockholders and, as a result, are considered participating securities.

Segment and Geographic Information

Operating segments are defined as components of an enterprise (business activity from which it earns revenue and incurs expenses) for which discrete financial information is available and regularly reviewed by the chief operating decision maker ("CODM") in deciding how to allocate resources and in assessing performance. The Company's CODM is its Chief Executive Officer. The Company views its operations and manages its business as a single operating and reporting segment. See Note C for further information.

Foreign Currency

Assets and liabilities are translated into the reporting currency using the exchange rates in effect on the balance sheet dates. Equity accounts are translated at historical rates, except for the change in retained earnings 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 unaudited condensed consolidated balance sheets 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 the Company's own stock and whether the warrant holders could potentially require "net cash settlement" in a circumstance outside of the issuing 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 statements of changes in stockholders' equity 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 statements of operations. The fair value of the warrants was estimated using the BSM option pricing model.

New Accounting Pronouncements Recently Adopted

In December 2023, the FASB issued Accounting Standards Update ("ASU") No. 2023-09, Income Taxes ("Topic 740"): *Improvements to Income Tax Disclosures*. ASU 2023-09 establishes new income tax disclosure requirements in addition to modifying and eliminating certain existing requirements. Under the new guidance, entities must consistently categorize and provide greater disaggregation of information in the reconciliation of the effective tax rate to the statutory tax rate and must also further disaggregate income taxes paid. The Company adopted ASU 2023-09 for the year ended December 31, 2025 using a retrospective approach and included the required disclosures in the Notes to the Consolidated Financial Statements for income taxes. This standard update did not affect the Company's results of operations.

New Accounting Pronouncements Not Yet Adopted

In November 2024, the FASB issued ASU No. 2024-03, Income Statement: Reporting Comprehensive Income-Expense Disaggregation Disclosures ("Subtopic 220-40"): *Disaggregation of Income Statement Expenses*. The new standard requires disclosure of specified information about certain costs and expenses. ASU 2024-03 is effective for fiscal years beginning after December 15, 2026 and for interim periods within fiscal years beginning after December 15, 2027. Early adoption is permitted. The

Company is currently evaluating this guidance to determine the impact it may have on its consolidated financial statements disclosures.

C. Segment Information

Zevra manages its business activities on a consolidated basis and operates as a single operating segment dedicated to the research and development, manufacturing, commercialization and sale of innovative medicines and therapies. The Company primarily derives its revenue from MIPLYFFA and OLPRUVA product sales and reimbursements received under the global EAP. For the nine months ended September 30, 2026, and 2025, the Company's revenue included royalties generated under the AZSTARYS License Agreement.

Zevra's CODM is the Company's Chief Executive Officer, Neil F. McFarlane. The CODM uses net income, as reported in the Company's unaudited condensed consolidated statements of operations, in evaluating performance of its segment and determining how to allocate resources of the Company as a whole, including investing in its research and development, commercialization efforts, and acquisition strategy. The CODM does not review assets in evaluating the results of the segment, and, therefore, such information is not presented. The accounting policies of the segment are the same as those described in Note B.

The following table presents the operating results of the Company's segment for the three and nine months ended September 30, 2026 and 2025 (in thousands):

	Three months ended September 30,		Nine months ended September 30,	
	2026	2025	2026	2025
Total revenues	$ 37,756	$ 26,063	$ 113,639	$ 72,345
Less significant segment expenses:				
Research and development directly identified to programs	3,002	1,780	8,140	6,480
Research and development not directly identified to programs	1,935	1,652	5,675	3,643
Selling, general and administrative directly identified to programs	5,572	5,144	15,136	19,144
Selling, general and administrative not directly identified to programs	13,558	11,791	41,336	38,118
Other segment items:				
Impairment of intangible assets	—	—	—	58,710
Income tax expense (benefit)	510	(433)	11,467	2,948
Interest income	(2,149)	(2,333)	(6,498)	(5,308)
Depreciation and amortization expense	316	392	1,110	3,692
Interest expense	—	2,051	1,711	6,029
Other (income) expense, net (a)	(4,196)	6,563	(30,288)	(132,175)
Segment net income (loss)	$ 19,208	$ (544)	$ 65,850	$ 71,064

(a) Other (income) expense, net, included in segment net income (loss) includes the gain on sale of the PRV, cost of product revenue, intangible asset amortization, gain on sale of future royalties, intellectual property, and other assets, net, loss on extinguishment of debt, loss on derivative liability, fair value adjustment related to warrant and contingent value right (“CVR”) liabilities, fair value adjustments related to investments, and other overhead expenses.

The Company holds long-lived assets in the United States of $2.0 million and $2.2 million as of September 30, 2026, and December 31, 2025, respectively. The Company holds long-lived assets in Europe of $0.2 million and $0.4 million as of September 30, 2026, and December 31, 2025, respectively.

D. Inventories

The components of inventory are summarized as follows (in thousands):

	September 30, 2026	December 31, 2025
Raw materials	$ 23	$ 7
Work in progress	1,002	1,780
Finished goods	2,045	832
Total inventories	$ 3,070	$ 2,619

E. Debt Obligations

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 was 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 was 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 October 5, 2025; and; (iii) $20.0 million, which was 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 Company did not draw down the amounts described in (ii) and (iii) above prior to their applicable expiration dates. The proceeds of the Term Loans were used to refinance certain existing indebtedness of the Company and its subsidiaries. The Company used the remaining proceeds to pay fees and expenses related to the debt financing and commercialization of MIPLYFFA and OLPRUVA, and to further the development of its other product candidates.

The Company evaluated the contractual terms of the Term Loans in accordance with ASC 815, *Derivatives and Hedging,* to determine whether any embedded features require bifurcation from the host debt instrument. The embedded features identified in the Term Loans included a mandatory prepayment provision upon the occurrence of certain triggering events. Based on its assessment, the Company determined that the mandatory repayment feature is not clearly and closely related to the host debt instrument and therefore requires bifurcation and separate accounting as a derivative instrument. The embedded derivative is not designated as a hedging instrument and is accounted for separately from the host debt instrument. The derivative is remeasured at each reporting date, with changes in fair value recognized in the unaudited consolidated statements of operations. As of December 31, 2025, the embedded derivative had no value as the Company determined that the probability of the associated triggering event was remote. The Company recognized a loss on derivative liability of $0.0 million and $7.2 million in the unaudited condensed consolidated statements of operations for the three and nine months ended September 30, 2026, respectively. The embedded derivative was eliminated upon repayment of the Term Loans as described below.

On March 12, 2026, the Company repaid in full all outstanding obligations under the Credit Agreement. As of the date of repayment, the aggregate principal amount outstanding under the Credit Agreement was approximately $63.1 million (which includes accrued paid-in-kind interest of approximately $3.1 million), plus accrued and unpaid cash interest of $1.4 million. Under the terms of the Credit Agreement, the Company was also required to pay a final payment premium of $1.8 million, a make-whole amount and prepayment premium of $7.2 million and legal fees of approximately $0.1 million. The Term Loans were secured by a first priority perfected lien on, and security interest in, substantially all current and future assets of the Company and certain of its subsidiaries that were guarantors thereunder. Upon repayment, the Credit Agreement and all related loan documents were terminated, and all liens and security interests granted thereunder were released. The Company recognized a loss on extinguishment of debt of $0.0 million and $2.8 million in the unaudited condensed consolidated statements of operations during the three and nine months ended September 30, 2026.

Prior to the repayment, the principal amount of the Term Loans outstanding (the "Outstanding Principal Amount") bore interest at a rate equal to 3-Month Term Secured Overnight Financing Rate ("SOFR") plus 7.00% per annum. The 3-Month Term SOFR rate was subject to a floor of 4.00% per annum. Interest was payable quarterly in arrears on the last day of each calendar quarter. The Company had the option to pay up to 25% of the interest in-kind beginning on the Term Loans Closing Date, through and including March 31, 2026. During the three months ended March 31, 2026, the Company repaid approximately $3.1 million of interest-in-kind. The Company had recognized approximately $3.1 million of interest-in-kind as of December 31, 2025, which is included in long-term debt in the unaudited condensed consolidated balance sheet. The Term Loans would have matured on April 5, 2029, the fifth anniversary of the Term Loans Closing date.

Long-term debt consisted of the following at December 31, 2025 (in thousands):

	December 31, 2025
Notes payable	$ 63,608
Unamortized original issue discount	(755)
Less: debt issuance costs	(925)
	$ 61,928

F. Revenue, net

For the three and nine months ended September 30, 2026, the Company recorded $37.8 million and $113.6 million, respectively, of revenue. For the three and nine months ended September 30, 2025, the Company recorded $26.1 million and $72.3 million, respectively, of revenue. Included in revenue for the nine months ended September 30, 2025, is a de minimis amount related to the licensing of certain IP.

Product Revenues, net

On September 20, 2024, the FDA approved MIPLYFFA (arimoclomol), an orally-delivered treatment for NPC, which is an ultra-rare and progressive neurodegenerative disease, for treatment in combination with miglustat. For the three and nine months ended September 30, 2026, sales of MIPLYFFA were $34.0 million and $88.9 million, respectively. For the three and nine months ended September 30, 2025, sales of MIPLYFFA were $22.4 million and $61.0 million, respectively.

On December 22, 2022, the FDA approved OLPRUVA (sodium phenylbutyrate), a prescription medicine used along with certain therapy, including changes in diet, for the chronic management of adults and children with certain UCDs. For the three and nine months ended September 30, 2026, sales of OLPRUVA were $0.2 million and $0.7 million, respectively. For the three and nine months ended September 30, 2025, sales of OLPRUVA were $0.1 million and $0.5 million, respectively.

The Company currently utilizes a single specialty pharmacy provider as its sole distributor for MIPLYFFA and OLPRUVA. 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. All estimated reserve liabilities related to commercial products are recorded within the current portion of discount and rebate liabilities in the unaudited condensed consolidated balance sheets. To commercialize MIPLYFFA and OLPRUVA in the United States, the Company has built marketing, sales, medical affairs, distribution, managerial and other non-technical capabilities or has made arrangements with third parties to perform these services. All revenues derived from sales of MIPLYFFA and OLPRUVA are in the United States.

Expanded Access Program

For the three and nine months ended September 30, 2026, the Company recognized revenue related to the global EAP of $3.6 million and $22.7 million, respectively, net of a clawback liability and other adjustments of $2.5 million and $11.5 million, respectively. For the three and nine months ended September 30, 2025, the Company recognized revenue related to the global EAP of $2.4 million and $7.3 million, net of a clawback liability and other adjustments of $1.8 million and $5.5 million, respectively.

The total estimated clawback reserve liability as of September 30, 2026, and December 31, 2025, was $14.8 million and $15.3 million, respectively. As of September 30, 2026, and December 31, 2025, 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.

AZSTARYS License Agreement

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 was 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, 2026, the Company recognized revenue under the AZSTARYS License Agreement of $0.0 million and $1.4 million, respectively, and all revenues recognized under this agreement were derived in the United States. For the three and nine months ended September 30, 2025, the Company recognized revenue under the AZSTARYS License Agreement of $1.2 million and $3.3 million, respectively. There was no deferred revenue related to this agreement as of September 30, 2026, or December 31, 2025. As further discussed in Note A, the Company and Commave agreed to terminate the AZSTARYS License Agreement during the first quarter of 2026, and therefore the Company will not recognize revenue in future periods under the 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

On August 28, 2023, Acer and Relief Therapeutics SA ("Relief") entered into an exclusive license agreement (the "Relief License Agreement"). Pursuant to the Relief License Agreement, Relief holds exclusive development and commercialization rights for OLPRUVA in the European Union ("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 has 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, 2026, and 2025, 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, 2026, and December 31, 2025.

Accounts and Other Receivables

Accounts and other receivables consist of receivables from MIPLYFFA and OLPRUVA product sales, the global EAP, and other receivables due to the Company. Receivables under the global EAP are recorded for product sales of MIPLYFFA in France and select territories outside of Europe. The Company provides reserves against receivables for estimated losses that may result from a customer's inability to pay. Receivables are evaluated to determine if any reserve or allowance should be recorded based on consideration of the current economic environment, expectations of future economic conditions, specific circumstances and the Company's own historical collection experience. Amounts determined to be uncollectible are charged or written-off against the reserve.

Accounts and other receivables consist of the following (in thousands):

	September 30, 2026	December 31, 2025
Commercial accounts receivable	$ 13,247	$ 9,876
Receivables related to product reimbursements	6,381	10,998
Royalties accounts receivable	—	1,786
Other receivables	315	598
Total receivables	$ 19,943	$ 23,258

As of September 30, 2026, and December 31, 2025, no reserve or allowance for doubtful accounts had been established.

G. Stock and Warrants

Authorized, Issued, and Outstanding Common Shares

As of September 30, 2026, and December 31, 2025, the Company had 250,000,000 shares of authorized common stock. Of the authorized shares, 61,015,651 and 58,338,319 shares of common stock were issued as of September 30, 2026, and December 31, 2025, respectively, and 59,439,959 and 56,854,781 shares of common stock were outstanding as of September 30, 2026, and December 31, 2025, respectively.

As of September 30, 2026, and December 31, 2025, the Company had reserved authorized shares of common stock for future issuance as follows:

	September 30, 2026	December 31, 2025
Outstanding awards under equity incentive plans	6,999,571	7,060,457
Outstanding common stock warrants	2,529,379	4,024,157
Possible future issuances under equity incentive plans	7,842,154	6,327,569
Possible future issuances under employee stock purchase plan	925,230	1,011,962
Total common shares reserved for future issuance	18,296,334	18,424,145

Common Stock Activity

The following table summarizes common stock activity for the three and nine months ended September 30, 2026:

	Shares of Common Stock
Balance as of January 1, 2026	56,854,781
Common stock issued as a result of stock options exercised or RSUs vested	955,575
Common stock issued as a result of stock warrants exercised	1,304,494
Balance as of March 31, 2026	59,114,850
Common stock issued as a result of stock options exercised or RSUs vested	140,324
Common stock issued as a result of Employee Stock Purchase Plan	86,732
Balance as of June 30, 2026	59,341,906
Common stock issued as a result of stock options exercised or RSUs vested	98,053
Balance as of September 30, 2026	59,439,959

Authorized, Issued, and Outstanding Preferred Stock

As of September 30, 2026, and December 31, 2025, the Company had 10,000,000 shares of authorized, unallocated preferred stock. As of September 30, 2026, and December 31, 2025, no shares of preferred stock 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 2,529,379 remain outstanding as of September 30, 2026, 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 income (loss) per share calculation (Note J). The Company may be required to redeem these warrants for a cash amount equal to the BSM value of the portion of the warrants to be redeemed.

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, 2026, 2,954,158 of the warrants had been exercised or expired. As of December 31, 2025, 1,459,380 warrants had 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 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, 2026, and December 31, 2025, the fair value of the liability associated with these warrants was approximately $7.7 million and $9.6 million, respectively. The fair value adjustment related to these warrants for the three and nine months ended September 30, 2026 was $5.5 million of income and $0.1 million of loss, respectively. The fair value adjustment related to these warrants for the three and nine months ended September 30, 2025 was $5.1 million and $3.1 million of loss, respectively.

H. Stock-Based Compensation

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 was 14,353,902 as of September 30, 2026. The number of shares of common stock reserved for issuance under the A&R 2014 Plan automatically increases on January 1 of each year, beginning on January 1, 2016, and ending on 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 A&R 2014 Plan, on January 1, 2026, the common stock reserved for issuance under the A&R 2014 Plan automatically increased by 2,274,191 shares.

During the three and nine months ended September 30, 2026, 98,053 and 1,097,846 stock options were exercised, respectively. During the three and nine months ended September 30, 2025, 86,628 and 1,162,133 stock options were exercised, respectively.

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, 2026, 574,770 shares have been issued under the ESPP.

In January 2023, the Board approved the 2023 Employment Inducement Award Plan (as amended, the "2023 Plan") to make equity awards to newly hired employees as a material inducement to employment in accordance with Nasdaq Rule 5635(c)(4). The maximum number of shares of common stock that may be issued under the 2023 Plan was 4,500,000 as of September 30, 2026.

In February 2025, the Board approved the Tenth Amended and Restated Non-Employee Director Compensation Policy (the "Non-Employee Director Compensation Policy"). The equity compensation granted pursuant to the Non-Employee Director Compensation Policy is granted under the A&R 2014 Plan.

Stock-based compensation expense recorded under the 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,	
	2026	2025	2026	2025
Research and development	$ 209	$ 250	$ 658	$ 721
Selling, general and administrative	2,463	2,521	8,088	7,629
Total stock-based compensation expense	$ 2,672	$ 2,771	$ 8,746	$ 8,350

There was no stock-based compensation expense related to performance-based awards recognized during the three and nine months ended September 30, 2026, and 2025, as the probability criteria was not met.

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, accounts and other receivables, 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, 2026, and December 31, 2025 (in thousands):

	Balance as of September 30, 2026	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Warrant liabilities	$ 7,712	$ —	$ —	$ 7,712
CVR liability	1,710	—	—	1,710
Total liabilities	$ 9,422	$ —	$ —	$ 9,422
Securities:				
U.S. Treasury securities	$ 79,745	$ 79,745	$ —	$ —
Corporate bonds	68,998	—	68,998	—
Total assets	$ 148,743	$ 79,745	$ 68,998	$ —

	Balance as of December 31, 2025	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Warrant liabilities	$ 9,575	$ —	$ —	$ 9,575
CVR liability	1,540	—	—	1,540
Total liabilities	$ 11,115	$ —	$ —	$ 11,115
Securities:				
U.S. Treasury securities	$ 97,132	$ 97,132	$ —	$ —
Corporate bonds	79,352	—	79,352	—
Total assets	$ 176,484	$ 97,132	$ 79,352	$ —

Warrants

The common stock warrant liabilities were recorded at fair value using the BSM option pricing model. The following assumptions were used in determining the fair value of the warrant liabilities valued using the BSM option pricing model as of September 30, 2026, and December 31, 2025:

	September 30, 2026	December 31, 2025
Risk-free interest rate	4.12% - 4.79%	3.42% - 3.67%
Volatility	58.24% - 65.51%	57.28% - 66.70%
Dividend yield	— %	— %
Expected term (years)	0.25 - 2.15	0.02 - 2.89
Weighted average fair value	$ 3.05	$ 2.38

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, 2025	$ 9,575
Change in fair value measurement of warrant liabilities	127
Warrants exercised	(1,990)
Balance as of September 30, 2026	$ 7,712

For the nine months ended September 30, 2026, the changes in fair value of the warrant liabilities primarily resulted from changes in the discount rate and volatility.

Contingent Consideration

Contingent consideration liabilities relate to the Company's liabilities arising in connection with the CVRs. 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 (in thousands):

Balance as of December 31, 2025	$	1,540
Change in fair value measurement of contingent consideration liabilities		170
Balance as of September 30, 2026	$	1,710

For the nine months ended September 30, 2026, the changes in fair value of contingent consideration primarily resulted from changes in market data and revenue projections.

Embedded Derivative

The fair value of the embedded derivative is classified as Level 3 in the fair value hierarchy and had no value at September 30, 2026 and December 31, 2025. The liability associated with the embedded derivative represented the fair value of the mandatory redemption provision in the Credit Agreement. During the nine months ended September 30, 2026, all outstanding obligations under the Credit Agreement were paid off which represented a triggering event. The embedded derivative was then re-valued, and extinguished together with the debt (see Note E).

The fair value was determined using a Black-Derman-Toy ("BDT") binomial lattice model in conjunction with a discounted cash flow methodology. The valuation incorporates a discount rate derived from the risk-free rate, based on the US Treasury Daily Treasury Par Yield Curve. The quantitative information about the significant unobservable inputs used in the Level 3 fair value measurement for the embedded derivative includes a credit spread of 5.53%.

The following table provides a reconciliation of the beginning and ending balances related to the embedded derivative (in thousands):

Balance as of December 31, 2025	$	—
Change in fair value measurement of embedded derivative		7,216
Extinguishments		(7,216)
Balance as of September 30, 2026	$	—

For the nine months ended September 30, 2026, the changes in fair value of the embedded derivative resulted from a change in probability of a triggering event occurring.

J. Net Income (Loss) Per Share

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 attributable to common stockholders, basic net income attributable to common stockholders per share of common stock is computed by dividing the net income attributable to common stockholders by the weighted average number of shares of common stock outstanding during the period. Undistributed net income attributable to common stockholders 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. 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 attributable to common stockholders 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 and warrants. 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 when calculating diluted income (loss) attributable to common stockholders per share of common stock, in which it is assumed that the stock options and warrants convert into common stock at the beginning of the period or date of issuance, if the stock option or warrant 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) attributable to common stockholders per share of common stock during the period.

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,	
	2026	2025	2026	2025
Awards under equity incentive plans	2,224,345	7,049,222	2,224,345	1,771,663
Common stock warrants	1,529,379	4,436,637	—	—
Total securities excluded from the calculation of weighted average number of shares of common stock outstanding	3,753,724	11,485,859	2,224,345	1,771,663

A reconciliation from net income (loss) to basic net income (loss) attributable to common stockholders per share of common stock and diluted net income (loss) attributable to common stockholders per share of common stock for the three and nine months ended September 30, 2026, and 2025 is as follows (in thousands):

	Three months ended September 30,		Nine months ended September 30,	
	2026	2025	2026	2025
Basic net income (loss) per share of common stock:				
Net income (loss)	$ 19,208	$ (544)	$ 65,850	$ 71,064
Earnings allocated to participating securities	(784)	—	(2,707)	(5,309)
Net income (loss) attributable to shares of common stock	**18,424**	**(544)**	**63,143**	**65,755**
Less: Dividends declared or accumulated	—	—	—	—
Undistributed net income (loss) attributable to shares of common stock, basic	**18,424**	**(544)**	**63,143**	**65,755**
Weighted-average shares of common stock outstanding	59,407	55,952	58,993	54,949
Basic net income (loss) per share of common stock	**$ 0.31**	**$ (0.01)**	**$ 1.07**	**$ 1.20**
Diluted net income (loss) per share of common stock:				
Net income (loss) attributable to shares of common stock	$ 18,424	$ (544)	$ 63,143	$ 65,755
Plus: Fair value adjustment income related to warrant liability	(2,452)	—	—	—
Net income (loss) attributable to shares of common stock, diluted	**15,972**	**(544)**	**63,143**	**65,755**
Weighted-average number of shares of common stock outstanding	59,407	55,952	58,993	54,949
Dilutive effect of outstanding stock options (as converted to common stock)	2,325	—	2,110	1,834
Weighted-average shares of common stock outstanding, diluted	**61,732**	**55,952**	**61,103**	**56,783**
Diluted net income (loss) per share of common stock	**$ 0.26**	**$ (0.01)**	**$ 1.03**	**$ 1.16**

K. Leases

The Company has operating leases for office space and 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 12 months or less at commencement are not recorded on the unaudited condensed consolidated balance sheets. Lease expense for these arrangements is recognized on straight-line bases over the lease term. The Company's leases have remaining lease terms of less than one year and up to approximately three years, and some which include options to terminate the leases within one year. In March 2026, we entered into a lease related to our corporate headquarters to provide us with additional office space, which commenced in June 2026.

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

	Three months ended September 30,		Nine months ended September 30,	
	2026	2025	2026	2025
Operating lease cost	201	205	522	612
Short-term lease cost	51	32	119	127
Variable lease cost	—	10	—	36
Less: sublease income	—	(27)	(26)	(105)
Total lease costs	$ 252	$ 220	$ 615	$ 670

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

	Nine months ended September 30,	
	2026	2025
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 490	$ 688
Operating cash flows from short-term leases	119	127
Right-of-use assets obtained in exchange for lease liabilities:		
Operating leases	$ 460	$ 1,115

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

	September 30, 2026	December 31, 2025
Operating lease right-of-use assets	$ 1,423	$ 1,212
Current portion of operating lease liabilities	$ 626	$ 419
Operating lease liabilities, less current portion	602	859
Total operating lease liabilities	$ 1,228	$ 1,278
Weighted average remaining lease term	1.87	3.0
Weighted average discount rate	14.4%	13.0%

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

Year Ended December 31,	
2026 (excluding the nine months ended September 30, 2026)	$ 189
2027	761
2028	431
2029	21
Total lease payments	1,402
Less: future interest expense	(174)
Lease liabilities	$ 1,228

L. Goodwill & Intangible Assets

The Company's goodwill balance was $4.7 million as of September 30, 2026, and December 31, 2025.

Prior to June 30, 2025, the Company had a definite-lived intangible asset, net related to the acquisition of OLPRUVA of $60.0 million, which was reviewed for impairment whenever events or changes in circumstances indicated that their carrying amounts may not be recoverable.

In the second quarter of 2025, the Company assessed the results of its refined commercial efforts related to OLPRUVA. This was determined to be a triggering event that could result in a decrease in future expected cash flows, and thus indicated the carrying amount of the OLPRUVA asset group may not be fully recoverable. The Company performed an undiscounted cash flow analysis over the OLPRUVA asset group and determined that the carrying value of the asset group was not recoverable. The Company then estimated the fair value of the asset group to measure the impairment loss for the period. The fair value measurement was based on Level 3 inputs including projected sales driven by market share and product sales price estimates, associated expenses, growth rates, and the discount rate used to measure the fair value of the net cash flows associated with this asset group. The Company recorded an intangible asset impairment charge in the second quarter of 2025, which resulted in reducing the OLPRUVA intangible asset value to zero.

Prior to the impairment discussed above, the OLPRUVA definite-lived intangible asset was being amortized on a straight-line basis over the OLPRUVA patent life of 13 years. Amortization expense is recorded as intangible asset amortization in the unaudited condensed consolidated statements of operations and was $0.0 million and $2.6 million for the three and nine months ended September 30, 2025, respectively.

In connection with the XOMA License Agreement, a regulatory milestone payment of $6.0 million was due to XOMA upon approval of MIPLYFFA in the United States, which the Company paid in October 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 $0.3 million for the three months ended September 30, 2026, and 2025, respectively. Amortization expense was $0.9 million for the nine months ended September 30, 2026, and 2025, respectively.

For intangible assets subject to amortization, estimated amortization expense for the five fiscal years subsequent to September 30, 2026, is expected to be as follows (in thousands):

2026 (excluding the nine months ended September 30, 2026)	$	316
2027		1,263
2028		1,263
2029		632
2030		—

As of September 30, 2026, and December 31, 2025, non-amortizable intangible assets include IPR&D of $2.0 million.

M. Commitments and Contingencies

Legal Matters

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.

As of September 30, 2026, and December 31, 2025, no accruals were made related to commitments and contingencies.

N. Subsequent Events

The Company evaluated events and transactions occurring subsequent to September 30, 2026, through November 3, 2026, 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, 2025, filed with the SEC on March 9, 2026, as supplemented in Part II, Item 1A. "Risk Factors" of our Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, filed with the SEC on May 6, 2026, 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 with a late-stage pipeline committed to redefining what is possible in bringing life-changing therapeutics to people living with rare diseases. We are focused on broadening access through geographic expansion opportunities, progressing our pipeline toward key milestones, and delivering meaningful therapeutics. Our vision is realized through disciplined execution of our strategic plan and our core values — patient centricity, integrity, accountability, innovation, and courage — which guide our efforts to deliver long-term value. The commercialization of our lead product, MIPLYFFA (arimoclomol), marketed in the United States for Niemann-Pick disease type C ("NPC"), a rare, progressive neurodegenerative disorder, provides a strong corporate foundation and demonstrates our ability to advance therapies from development to market.

In February 2023, we changed our name to Zevra Therapeutics, Inc. 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.

Our strategic plan is focused on transforming Zevra into a leading rare-disease company. We are prioritizing the commercialization and global expansion of MIPLYFFA, while OLPRUVA remains commercially available. We are also advancing the development of our clinical stage asset, celiprolol, and plan to further expand our pipeline through inorganic growth. We intend to become the preferred partner for assets that we believe will allow us to leverage the expertise and infrastructure that we have built to help mitigate risk and enhance our probability of success.

On September 20, 2024, the U.S. Food and Drug Administration ("FDA") approved the New Drug Application ("NDA") for MIPLYFFA, 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, and MIPLYFFA became commercially available for dispense in the United States in November 2024. In connection with this approval, we received a transferable rare pediatric disease priority review voucher ("PRV"). On April 1, 2025, we consummated the sale of the PRV, resulting in net proceeds of $148.3 million to us. Arimoclomol has also been granted orphan medicinal product designation for the treatment of NPC by the European Commission. We are pursuing regulatory approval of arimoclomol in Europe and filed a Marketing Authorization Application ("MAA") with the European Medicines Agency ("EMA") in July 2025. In July 2026, we announced that the Committee for Medicinal Products for Human Use (CHMP) of the EMA adopted a negative opinion regarding the MAA. Under European regulatory procedures, we have requested a re-examination of the CHMP opinion.

On November 17, 2023, we completed the acquisition of Acer Therapeutics, Inc. ("Acer"), pursuant to which Acer became a wholly-owned subsidiary of Zevra. This included the acquisition of OLPRUVA (sodium phenylbutyrate) for oral suspension, which was approved by the FDA on December 22, 2022, for the treatment of certain urea cycle disorders ("UCDs"). In addition, we acquired Acer's 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.

On March 13, 2026, we entered into an Asset Purchase and Settlement Agreement (the "Commave Settlement Agreement") with Commave Therapeutics SA ("Commave") to sell all of our rights to certain assets relating to our SDX portfolio, including AZSTARYS and KP1077, to Commave and to resolve pending litigation related to claims arising under the Collaboration and License Agreement between the parties dated September 3, 2019, as amended (the "AZSTARYS License Agreement"). Under the Commave Settlement Agreement, the AZSTARYS License Agreement was terminated in its entirety.

While we have recently had several quarters of positive net cash flows from operations, we previously had recurring negative net operating cash flows, and we cannot guarantee that we can continue to generate positive net cash flows from operations. Net cash provided by (used in) operating activities for the nine months ended September 30, 2026, and 2025, was $33.2 million and $(7.1)

million, respectively. We expect to continue to incur significant expenses and minimal positive net cash flows from operations or negative net cash flows from operations for the near future, and those expenses and losses may fluctuate significantly from quarter-to-quarter and year-to-year. We anticipate that our expenses will fluctuate substantially as we:

- continue building and maintaining our ongoing commercial capabilities to support the commercialization of our approved products;
- continue clinical trials for celiprolol or initiate preclinical studies, clinical trials and product development activities for future product candidates;
- seek regulatory approvals for any product candidates that may successfully complete clinical trials;
- seek to discover, license or acquire, and develop additional product candidates;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio;
- incur additional legal, accounting and other expenses in operating as a public company; and
- add operational systems and personnel, if needed, to support any future commercialization efforts.

Our Product Candidates and Approved Products

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

Active Zevra Commercial and Active Development Assets

Parent Drug	Indication	Product / Candidate	Status and IP
Arimoclomol	Niemann-Pick disease type C (NPC)	MIPLYFFA	FDA Approval: Sep. 20, 2024; Orphan Drug Exclusivity (“ODE”) through 2031; IP through 2041
Arimoclomol	NPC	Arimoclomol	Global Expanded Access Program (“EAP”) Marketing Authorisation Application (“MAA”) for arimoclomol under review by EMA, Submitted July 28, 2025
Sodium phenylbutyrate	Urea Cycle Disorders (UCD)	OLPRUVA	FDA Approval: Dec. 22, 2022; IP through 2036
Celiprolol	Vascular Ehlers Danlos Syndrome (VEDS)	Celiprolol	Clinical - Phase 3 trial ongoing; IP potential through 2038

MIPLYFFA

NPC is an ultra-rare and progressive neurodegenerative disease 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 2,000 individuals with NPC in the United States and Europe combined, of which approximately 900 are in the United States and 1,100 are in Europe, where there is a mature market with an approved treatment available for NPC. Of this estimated population, approximately 300 to 350 people have been diagnosed in the United States. NPC is clinically heterogeneous, with significant variability in symptom presentation and rate of progression. Although it has traditionally been considered a pediatric disease due to its genetic origins, nearly half of the people treated with MIPLYFFA are adults. Low diagnostic rates may affect the number of potential patients, and we believe that the availability of treatment options in the United States could increase awareness of the disease and assist in more accurately identifying patients.

On September 20, 2024, the FDA approved the NDA for MIPLYFFA, an orally-delivered treatment, for NPC. 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 PRV in conjunction with the approval. On April 1, 2025, we completed the sale of the PRV and received net proceeds of $148.3 million.

Effective therapies to treat NPC are desperately needed, and, for this reason, arimoclomol is currently being made available to NPC patients in France, Germany, and other EU member states, along with select territories outside of Europe under our global EAP. Arimoclomol has also been granted orphan medicinal product designation for the treatment of NPC by the European Commission. In July 2025, we filed an MAA. In July 2026, we announced that the CHMP of the EMA adopted a negative opinion regarding the MAA. We have requested a re-examination of the CHMP opinion.

In June 2026, Zevra published positive results of an open-label pediatric substudy in the peer reviewed journal *Molecular Genetics and Metabolism Reports*. This multicenter substudy evaluated arimoclomol, in addition to concomitant miglustat, in patients with a confirmed diagnosis of NPC1 or NPC2 aged 6 to <24 months at study enrollment over up to 36 months of treatment. In the five patients enrolled, arimoclomol was generally well tolerated, with no new safety signals, and pharmacokinetic results were consistent with older pediatric populations. Developmental assessments showed variable delays, reflecting underlying phenotypic heterogeneity. Although limited by small sample size, the study provides preliminary evidence supporting initiation of MIPLYFFA in this age group.

As of September 30, 2026, there were a total of 197 enrollments to receive MIPLYFFA. For MIPLYFFA, an enrollment is a prescription submitted to our specialty pharmacy, initiating the benefits investigation process to determine reimbursement and can lead to a 30-day paid dispense of MIPLYFFA. Our commercial plans focus on raising awareness among people who are living with NPC that are diagnosed and untreated, or undiagnosed.

To commercialize MIPLYFFA in the United States, we have built in-house capabilities, and have arrangements with third parties, to perform marketing, sales, medical affairs, distribution, managerial and other non-technical capabilities. Zevra holds global rights to develop and commercialize MIPLYFFA.

MIPLYFFA summary:

- ***Demonstrated halting of disease progression.*** MIPLYFFA in combination with miglustat demonstrated a clinically significant improvement compared to placebo as early as 12 weeks and a halting of progression of the disease through 12 months of treatment. Data from the 48-month Open Label Extension study confirms the effectiveness of MIPLYFFA in slowing disease progression over multiple years.

- ***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.*** Over 600 patients have been treated with arimoclomol across various clinical trials and indications as well as through our global EAP, with limited safety findings. Further, in a pediatric sub-study of patients aged 6-24 months, arimoclomol was well tolerated following at least 12 months of treatment with no new safety concerns observed.

- ***Advantageous regulatory designations.*** Arimoclomol has been granted orphan medicinal product designation for the treatment of NPC by the European Commission.

OLPRUVA

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 United States. While there are therapies currently approved for the treatment of UCDs, we believe that OLPRUVA offers benefits over other UCD treatments by eliminating issues with palatability, offering improved portability with its single-dose envelopes, and being provided in a dosage personalized to the patient based on weight.

OLPRUVA (sodium phenylbutyrate) for oral suspension is approved in the United States 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), or argininosuccinic acid synthetase (AS). OLPRUVA for oral suspension 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 to help minimize the unpleasant taste of the sodium phenylbutyrate powder.

In the fourth quarter of 2023, we began generating revenue from the sale of OLPRUVA in the United States. Zevra has a partnership with Relief Therapeutics SA ("Relief"), which has rights to commercialize OLPRUVA in various European countries, if approved. For the three and nine months ended September 30, 2026, we had $0.2 million and $0.7 million in revenue, respectively, from sales of OLPRUVA. We have made the decision to scale back our sales and marketing efforts for OLPRUVA as we evaluate the path forward and weigh strategic alternatives.

OLPRUVA summary:

- ***OLPRUVA is available in the U.S. for the treatment of certain types of UCDs.*** OLPRUVA is an adjunctive therapy for the chronic 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.

- ***Currently evaluating strategic alternatives.*** We have had limited success in commercializing OLPRUVA in the United States, and we are currently evaluating the path forward and weighing strategic alternatives.

Celiprolol

Ehlers-Danlos Syndrome (EDS) is a rare 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. Vascular Ehlers-Danlos Syndrome (VEDS) is the most severe of the 13 types of EDS and causes abnormal fragility in large to medium sized arteries and hollow organs, such as the uterus and colon. This fragility can result in aneurysms, abnormal connections between blood vessels known as arteriovenous fistulas, arterial dissections, and spontaneous vascular and organ 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 United States.

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 adrenoceptor modulator ("SAM") that would qualify as a new chemical entity ("NCE") if approved in the United States. Celiprolol is currently approved in certain EU states for the treatment of hypertension and angina.

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; 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 complete response letter ("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. 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 a 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.

Based on the FDA's feedback during the Type B meeting, Acer 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 in the United States 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 mid-2024, and the trial has 72 enrolled participants as of September 30, 2026. We believe that celiprolol could address significant

unmet needs, as there are currently no approved treatments for VEDS in the U.S. We have implemented a broad recruitment drive focusing on collaborating with medical clinics where most patients are being managed. In parallel, we actively engaged the FDA in a Type C meeting in the first quarter of 2026 to discuss regulatory options to potentially accelerate the development program and plan to continue the dialogue with regulators going forward with a follow-up meeting in the fourth quarter of 2026.

Celiprolol summary:

- ***Currently, no approved treatments for VEDS in the United States.*** There are currently no approved treatments for VEDS in the United States, and we believe that celiprolol, if approved, could be a significant innovation in the treatment of VEDS 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, potentially resulting in less incidence of vascular and hollow organ ruptures.

- ***Evidence of efficacy in Europe 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 has been granted Orphan Drug designation and Breakthrough Therapy designation and, we believe, would be deemed an NCE in the United States if approved before any other celiprolol product.

- ***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.

License Agreements

XOMA License Agreement (MIPLYFFA)

In May 2022, we purchased all the assets and operations of Orphazyme A/S ("Orphazyme") related to arimoclomol. Prior to this acquisition, Orphazyme had 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 a mid-single digit percentage royalty with respect to net sales of MIPLYFFA as well as milestone payments based on future potential sales and regulatory milestones, including a $4.0 million regulatory milestone payment upon approval in the E.U. and a $5.0 million sales milestone payment upon annual sales of $100.0 million. XOMA was acquired by Ligand Pharmaceuticals in July 2026.

Relief Termination Agreement and Relief License Agreement (OLPRUVA)

In connection with our acquisition of Acer, Acer and Relief entered into a termination agreement on August 28, 2023 (the "Relief Termination Agreement"). Pursuant to the Relief Termination Agreement, we are obligated to pay royalties of 10% of U.S. net sales of OLPRUVA up to a maximum of $45.0 million, plus specified regulatory milestones, for total payments to Relief of up to $56.5 million. On April 10, 2025, Relief sold the rights to this royalty to Soleus Capital Management L.P., and in December 2025, Relief merged with NeuroX Group SA, and the combined company now operates as MindMaze Therapeutics Holding SA.

Acer and Relief also entered into an exclusive license agreement on August 28, 2023 (the "Relief License Agreement"). Pursuant to the Relief License Agreement, Relief holds 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"). We have the right to receive a royalty of up to 10% of the net sales of OLPRUVA in Geographical Europe.

Results of Operations

Comparison of the three months ended September 30, 2026 and 2025 (in thousands):

	Three months ended September 30,		Period-to-
	2026	2025	Period Change
Revenue, net	$ 37,756	$ 26,063	$ 11,693
Cost of product revenue (excluding $315 and $315 in intangible asset amortization for the three months ended September 30, 2026, and 2025, respectively, shown separately below)	(1,794)	(1,238)	556
Intangible asset amortization	(315)	(315)	—
Impairment of intangible assets	—	—	—
Operating expenses:			
Research and development	(4,937)	(3,432)	1,505
Selling, general and administrative	(19,130)	(16,935)	2,195
Total operating expenses	(24,067)	(20,367)	3,700
Income from operations	11,580	4,143	7,437
Other income (expense):			
Interest expense	—	(2,051)	2,051
Fair value adjustment related to warrant and CVR liability	5,143	(5,506)	10,649
Fair value adjustment related to investments	(39)	124	(163)
Interest and other income, net	3,034	2,313	721
Total other income (expense)	8,138	(5,120)	13,258
Income (loss) before income taxes	19,718	(977)	20,695
Income tax (expense) benefit	(510)	433	(943)
Net income (loss)	$ 19,208	$ (544)	19,752

Net Income (Loss)

Net income for the three months ended September 30, 2026, was $19.2 million, compared to a net loss of $0.5 million for the three months ended September 30, 2025, an increase to net income of $19.8 million. The increase was primarily attributable to an increase in revenue of $11.7 million, an increase in fair value adjustment related to warrant and CVR liability of $10.6 million, an increase in interest and other income, net, of $0.7 million, and a decrease in interest expense of $2.1 million, partially offset by a decrease in tax benefit of $0.9 million.

Revenue, net

Revenue for the three months ended September 30, 2026, was $37.8 million, compared to revenue of $26.1 million for the three months ended September 30, 2025, an increase of $11.7 million. The increase was primarily attributable to an increase in product sales of MIPLYFFA of $11.6 million.

Cost of product revenue

Cost of product revenue for the three months ended September 30, 2026, was $1.8 million, an increase of $0.6 million compared to cost of product revenue of $1.2 million for the three months ended September 30, 2025. The increase was primarily due to an increase in royalty costs related to increased product sales of MIPLYFFA.

Intangible asset amortization

Intangible asset amortization for the three months ended September 30, 2026, of $0.3 million was consistent when compared to intangible asset amortization of $0.3 million for the three months ended September 30, 2025.

Research and development

Research and development expenses increased by $1.5 million, from $3.4 million for the three months ended September 30, 2025, to $4.9 million for the three months ended September 30, 2026. This increase was due primarily to our ongoing arimoclomol efforts and our continued investment in the celiprolol development program.

Selling, general and administrative

Selling, general and administrative expenses increased by approximately $2.2 million, from $16.9 million for the three months ended September 30, 2025, to $19.1 million for the three months ended September 30, 2026. This increase was primarily due to commercial related costs.

Other income (expense)

Other income (expense) increased from $5.1 million of expense for the three months ended September 30, 2025, to $8.1 million of income for the three months ended September 30, 2026. The increase in income was primarily attributable to an increase in fair value adjustment related to warrant and CVR liability of $10.6 million, a decrease in interest expense of $2.1 million, and an increase in interest and other income, net, of $0.7 million,

Income tax (expense) benefit

Income tax (expense) benefit increased by $0.9 million, from $0.4 million benefit for the three months ended September 30, 2025, to $0.5 million expense for the three months ended September 30, 2026, due to the tax provision associated with increased income in the current quarter.

Results of Operations

Comparison of the nine months ended September 30, 2026 and 2025 (in thousands):

	Nine months ended September 30,		Period-to-Period Change
	2026	2025	
Revenue, net	$ 113,639	$ 72,345	$ 41,294
Cost of product revenue (excluding $947 and $3,546 in intangible asset amortization for the nine months ended September 30, 2026, and 2025, respectively, shown separately below)	(5,216)	(14,962)	(9,746)
Intangible asset amortization	(947)	(3,546)	(2,599)
Impairment of intangible assets	—	(58,710)	(58,710)
Gain on sale of future royalties, intellectual property, and other assets, net	43,314	—	43,314
Operating expenses:			
Research and development	(13,815)	(10,123)	3,692
Selling, general and administrative	(56,472)	(57,262)	(790)
Total operating expenses	(70,287)	(67,385)	2,902
Income (loss) from operations	80,503	(72,258)	152,761
Other (expense) income:			
Gain on sale of PRV	—	148,325	(148,325)
Loss on extinguishment of debt	(2,756)	—	(2,756)
Loss on derivative liability	(7,216)	—	(7,216)
Interest expense	(1,711)	(6,029)	4,318
Fair value adjustment related to warrant and CVR liability	(297)	(1,379)	1,082
Fair value adjustment related to investments	(395)	119	(514)
Interest and other income, net	9,189	5,234	3,955
Total other (expense) income	(3,186)	146,270	(149,456)
Income before income taxes	77,317	74,012	3,305
Income tax expense	(11,467)	(2,948)	(8,519)
Net income	$ 65,850	$ 71,064	$ (5,214)

Net income

Net income for the nine months ended September 30, 2026, was $65.9 million, and was comprised of revenue of $113.6 million, a gain on sale of future royalties, intellectual property, and other assets, net, of $43.3 million under the Commave Settlement Agreement, interest and other income, net of $9.2 million, partially offset by cost of product revenue of $5.2 million, intangible asset amortization of $0.9 million, research and development expense of $13.8 million, selling, general and administrative costs of $56.5 million, loss on derivative liability of $7.2 million, loss on extinguishment of debt of $2.8 million, interest expense of $1.7 million, fair value adjustments of $0.7 million and income tax expense of $11.5 million.

Net income for the nine months ended September 30, 2025 was $71.1 million, and was comprised of revenue of $72.3 million, a gain on sale of PRV of $148.3 million, interest and other income, net of $5.2 million, partially offset by cost of product revenue of $15.0 million (including inventory obsolescence charges of $11.7 million), intangible asset amortization of $3.5 million, impairment of intangible assets of $58.7 million, research and development of $10.1 million, selling, general and administrative costs of $57.3 million, interest expense of $6.0 million, fair value adjustments of $1.3 million and income tax expense of $2.9 million.

Revenue, net

Revenue for the nine months ended September 30, 2026, was $113.6 million, compared to revenue of $72.3 million for the nine months ended September 30, 2025, an increase of $41.3 million. The increase was primarily attributable to an increase in revenues under the global EAP of $15.4 million and an increase in product sales of MIPLYFFA of $27.9 million, partially offset by a decrease in royalty revenue of $1.9 million.

Cost of product revenue

Cost of product revenue for the nine months ended September 30, 2026, was $5.2 million, a decrease of $9.7 million compared to cost of product revenue of $15.0 million for the nine months ended September 30, 2025. The decrease was primarily due to a decrease in the write-down of unsaleable inventory, partially offset by an increase in royalty costs related to increased product sales of MIPLYFFA.

Intangible asset amortization

Intangible asset amortization for the nine months ended September 30, 2026, was $0.9 million, a decrease of $2.6 million compared to intangible asset amortization of $3.5 million for the nine months ended September 30, 2025. The decrease was a result of definite-lived intangible assets acquired in the acquisition of Acer no longer being amortized as a result of the impairment recorded as of and for the period ended June 30, 2025.

Research and development

Research and development expenses increased by $3.7 million, from $10.1 million for the nine months ended September 30, 2025, to $13.8 million for the nine months ended September 30, 2026. This increase was primarily driven by an increase in spending for ongoing arimoclomol research and development efforts and the celiprolol Phase 3 study.

Selling, general and administrative

Selling, general and administrative expenses decreased by approximately $0.8 million, from $57.3 million for the nine months ended September 30, 2025, to $56.5 million for the nine months ended September 30, 2026. This decrease was primarily related to a decrease in third party spending and professional fees, partially offset by an increase in personnel related costs.

Other (expense) income

Other expense for the nine months ended September 30, 2026 was $3.2 million and consisted of a loss on extinguishment of debt of $2.8 million, loss on derivative liability of $7.2 million, interest expense of $1.7 million, and fair value adjustments of $0.7 million, partially offset by interest and other income, net of $9.2 million.

Other income for the nine months ended September 30, 2025 was $146.3 million and was attributable to a gain on sale of PRV of $148.3 million, interest and other income, net of $5.2 million, partially offset by interest expense of $6.0 million, and fair value adjustments of $1.3 million.

Income tax expense

Income tax expense increased by $8.5 million, from $2.9 million for the nine months ended September 30, 2025, to $11.5 million for the nine months ended September 30, 2026, due to the tax provision associated with increased income in the current period excluding the gain on sale of PRV for which income tax expense was recognized in the fourth quarter of 2024.

Liquidity and Capital Resources

Sources of Liquidity

Through September 30, 2026, we have funded our research and development and operating activities primarily through the issuance of debt and equity and from product sales of MIPLYFFA and OLPRUVA, reimbursements received under the global EAP, royalties or net sales milestone payments generated under the AZSTARYS License Agreement, payments received under the Commave Settlement Agreement, our PRV sale consummated on April 1, 2025, and consulting agreements. As of September 30, 2026, we had cash, cash equivalents and investments of $270.9 million.

While we have recently had several quarters of positive net cash flows from operations, we cannot guarantee that we can continue to generate positive net cash flows from operations. We expect that our sources of revenue will be from product sales of approved products, product reimbursements received under the global EAP, and any other future arrangements related to one or more of our products or product candidates.

If needed, 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.

Registration Statement on Form S-3

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 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 June 2024 Registration Statement was declared effective on June 13, 2024.

2024 ATM Agreement

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 at 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 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 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. As of September 30, 2026, no shares have been issued or sold under the 2024 ATM Agreement.

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, 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 was available to us in up to two drawings, each in an amount not to exceed $10.0 million, at our option until October 5, 2025; and; (iii) $20.0 million, which was 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"). We did not draw down the amounts described in (ii) and (iii) above prior to their applicable expiration dates. The proceeds of the Term Loans were used to refinance certain existing indebtedness of us and our subsidiaries. We used the remaining proceeds to pay fees and expenses related to the debt financing and commercialization of MIPLYFFA and OLPRUVA, and to further the development of other product candidates.

On March 12, 2026, we repaid in full all outstanding obligations under the Credit Agreement. As of the date of repayment, the aggregate principal amount outstanding under the Credit Agreement was approximately $63.1 million (which includes accrued paid-in-kind interest of approximately $3.1 million), plus accrued and unpaid cash interest of $1.4 million. Under the terms of the Credit Agreement, we were also required to pay a final payment premium of $1.8 million, a make-whole amount and prepayment premium of $7.2 million and legal fees of approximately $0.1 million. The Term Loans were secured by a first priority perfected lien on, and security interest in, substantially all current and future assets of ours and certain subsidiaries that were guarantors thereunder. Upon repayment, the Credit Agreement and all related loan documents were terminated, and all liens and security interests granted thereunder were released. We recognized a loss on extinguishment of debt of $2.8 million in the unaudited condensed consolidated statements of operations during the nine months ended September 30, 2026.

Cash Flows

The following table summarizes our cash flows for the nine months ended September 30, 2026, and 2025 (in thousands):

	Nine months ended September 30,	
	2026	**2025**
Net cash provided by (used in) operating activities	$ 33,221	$ (7,114)
Net cash provided by investing activities	76,218	17,900
Net cash (used in) provided by financing activities	(49,668)	9,518
Effect of exchange rate changes on cash and cash equivalents	(29)	350
Net increase in cash and cash equivalents	$ 59,742	$ 20,654

Operating Activities

For the nine months ended September 30, 2026, net cash provided by operating activities of $33.2 million consisted of net income of $65.9 million, in addition to changes in working capital of $4.3 million, partially offset by $36.9 million in adjustments for non-cash items. Net income was primarily attributable to income generated under the Commave Settlement Agreement, as well as revenue received from approved product sales, and reimbursements received under the global EAP. This income was partially offset by ongoing operating expenses to support our commercial organization and the resulting gain from the Commave Settlement Agreement, as well as the debt payoff. In addition to the aforementioned transactions, the adjustments for non-cash items primarily consisted of income tax expense of $5.6 million, stock-based compensation expense of $8.7 million, fair value adjustment related to warrant and CVR liability of $0.3 million and a gain on foreign currency exchange rates of $2.7 million.

For the nine months ended September 30, 2025, net cash used in operating activities of $7.1 million consisted of net income of $71.1 million, offset by $61.7 million in adjustments for non-cash items and changes in working capital of $16.5 million. Net income was primarily attributable to the sale of the PRV, as well as revenue received from product sales of MIPLYFFA and OLPRUVA, royalties generated under the AZSTARYS License Agreement, and reimbursements received under the global EAP, partially offset by impairment and obsolescence charges and spend on R&D programs and operating costs. The adjustments for non-cash items primarily consisted of the gain on sale of PRV of $148.3 million, partially offset by impairment of intangible assets of $58.7 million, inventory obsolescence of $11.7 million, stock-based compensation expense of $8.4 million, $3.7 million of depreciation and amortization expense, and income tax expense of $2.9 million.

Investing Activities

For the nine months ended September 30, 2026, net cash provided by investing activities was $76.2 million, which was primarily attributable to sales and maturities of investments of $188.2 million and sale of future royalties, intellectual property, and other assets, net, of $48.9 million, partially offset by $160.4 million in investment purchases.

For the nine months ended September 30, 2025, net cash provided by investing activities was $17.9 million, which was primarily attributable to proceeds from the sale of the PRV of $150.0 million and maturities of investments of $91.0 million, partially offset by $222.8 million in purchases of investments.

Financing Activities

For the nine months ended September 30, 2026, net cash used in financing activities was $49.7 million, which was primarily attributable to the repayment of debt of $60.1 million and the payments for employee taxes related to stock awards of $1.5 million, partially offset by proceeds from the issuance of stock for warrants exercised of $9.2 million and proceeds from issuance of common stock for options exercised of $2.3 million.

For the nine months ended September 30, 2025, net cash provided by financing activities was $9.5 million, which was primarily attributable to proceeds from the issuance of stock for warrants exercised of $6.0 million and options exercised or RSUs vested of $3.5 million.

Future Funding Requirements

We believe our available cash, cash equivalents and investments, together with our ability to generate operating cash flow, are sufficient to fund our existing and planned capital requirements for at least the next twelve months and the foreseeable future.

We maintain the majority of our cash, cash equivalents and investments 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:

- sales of our approved products;
- reimbursements received for arimoclomol under the global EAP; and
- potential sales of common stock by us under the 2024 ATM Agreement or any other offering of securities made pursuant to the June 2024 Registration Statement.

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 building and maintaining our ongoing commercial capabilities to support the commercialization of our approved products;
- continue clinical trials for celiprolol or initiate preclinical studies, clinical trials and product development activities for future product candidates;
- seek regulatory approvals for any product candidates that may successfully complete clinical trials;
- seek to discover, license or acquire, and develop additional product candidates;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio;
- incur additional legal, accounting and other expenses in operating as a public company; and
- add operational systems and personnel, if needed, to support any future commercialization efforts.

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. In addition, we cannot guarantee that we will be able to generate sufficient proceeds from sales of approved products, reimbursements received under the global EAP, 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 that may be necessary.

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 and/or commercialization efforts.

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, 2025, filed with the SEC on March 9, 2026.

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 defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of September 30, 2026. Based on the evaluation of our disclosure controls and procedures as of September 30, 2026, 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 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, 2026, 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. 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, 2025, filed with the SEC on March 9, 2026, as supplemented in Part II, Item 1A. "Risk Factors" of our Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, filed with the SEC on May 6, 2026, before investing in our common stock. There have been no material changes to the risk factors described in those reports.

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.

During the three months ended September 30, 2026, no director or officer of Zevra 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	Restated Certificate of Incorporation of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 9, 2026).
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 3, 2026

By: /s/ Neil F. McFarlane

Neil F. McFarlane

President and Chief Executive Officer
(Principal Executive Officer)

Date: November 3, 2026

By: /s/ Justin Renz

Justin Renz

Chief Financial Officer and Treasurer
(Principal Financial Officer)

EXHIBIT 31.1

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 3, 2026

/s/ Neil F. McFarlane

Name: Neil F. McFarlane

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

EXHIBIT 31.2

CERTIFICATION

I, Justin Renz, 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 3, 2026

/s/ Justin Renz

Name: Justin Renz

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

EXHIBIT 32.1

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, 2026, 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 3, 2026

/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.

EXHIBIT 32.2

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, 2026, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Justin Renz, 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 3, 2026

/s/ Justin Renz

Name: Justin Renz

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.