

**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, 2025

OR

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

For the Transition Period From \_\_\_\_\_ to \_\_\_\_\_

Commission File Number: 001-36913

**Zevra Therapeutics, Inc.**

(Exact Name of Registrant as Specified in Its Charter)

Delaware

(State or Other Jurisdiction of Incorporation or Organization)

20-5894398

(I.R.S. Employer Identification No.)

1180 Celebration Boulevard, Suite 103, Celebration, FL

(Address of Principal Executive Offices)

34747

(Zip Code)

(321) 939-3416

(Registrant's Telephone Number, Including Area Code)

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

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

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

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§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	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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

As of November 3, 2025, the registrant had 56,297,535 shares of common stock outstanding.

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## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, including the section entitled “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” contains forward-looking statements regarding future events and our future results that are subject to the safe harbors created under the Securities Act of 1933, as amended (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, 2024, filed with the SEC on March 12, 2025 (the “Annual Report on Form 10-K”), 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, 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 our 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 and the expected amount or timing of any payment related thereto under any of our collaboration agreements;
- 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, if approved;
- 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 on commercially reasonable terms, or at all, in order to support our continued operations;
- senior leadership and board member transitions and refreshments; and
- other factors discussed elsewhere in this report.

The forward-looking statements made in this report relate only to events as of the date on which the statements are made. We have included or made reference to important factors in the cautionary statements included in this report, particularly in the section entitled “Risk Factors” where we make reference to Part I, Item 1A. “Risk Factors” of our Annual Report on Form 10-K, 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.

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## 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. 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.

On August 30, 2023, we entered into an Agreement and Plan of Merger (the “Merger Agreement”) with Acer Therapeutics Inc. (“Acer”). On November 17, 2023 (the “Closing Date”), Zevra completed the acquisition of Acer (the “Merger”). Pursuant to the Merger Agreement, on the Closing Date, Acer continued as the surviving entity and as a wholly-owned subsidiary of Zevra.

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**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, 2025	December 31, 2024
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 54,439	\$ 33,785
Securities at fair value, current	145,070	35,711
Accounts and other receivables	16,841	10,509
Prepaid expenses and other current assets	7,398	4,052
Inventories, current	1,367	1,970
Total current assets	225,115	86,027
Securities at fair value, noncurrent	30,865	6,010
Inventories, noncurrent	720	10,999
Property and equipment, net	488	356
Operating lease right-of-use assets	1,337	657
Goodwill	4,701	4,701
Intangible assets, net	6,737	68,993
Other long-term assets	153	384
Total assets	\$ 270,116	\$ 178,127
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 11,072	\$ 25,456
Current portion of operating lease liabilities	441	420
Current portion of discount and rebate liabilities	5,609	4,989
Other current liabilities	8,990	3,200
Total current liabilities	26,112	34,065
Long-term debt	61,310	59,504
Warrant liability	13,767	17,804
Income tax payable	17,464	14,431
Operating lease liabilities, less current portion	966	372
Discount and rebate liabilities, less current portion	13,337	7,655
Other long-term liabilities	3,995	4,630
Total liabilities	136,951	138,461
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, 2025, or December 31, 2024	—	—
Common stock, \$0.0001 par value, 250,000,000 shares authorized; 57,793,414 shares issued and 56,217,722 shares outstanding as of September 30, 2025; 55,246,401 shares issued and 53,670,709 shares outstanding as of December 31, 2024	6	5
Additional paid-in capital	580,783	555,302
Treasury stock, at cost	(10,983)	(10,983)
Accumulated deficit	(434,225)	(505,289)
Accumulated other comprehensive (loss) income	(2,416)	631
Total stockholders' equity	133,165	39,666
Total liabilities and stockholders' equity	\$ 270,116	\$ 178,127

*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,	
	2025	2024	2025	2024
Revenue, net	\$ 26,063	\$ 3,695	\$ 72,345	\$ 11,569
Cost of product revenue (excluding \$315 and \$1,545 in intangible asset amortization for the three months ended September 30, 2025, and 2024, respectively, and \$3,546 and \$4,619 in intangible asset amortization for the nine months ended September 30, 2025, and 2024, respectively, shown separately below)	1,238	2,303	14,962	6,051
Intangible asset amortization	315	1,545	3,546	4,619
Impairment of intangible assets	—	—	58,710	—
Operating expenses:				
Research and development	3,432	10,945	10,123	33,743
Selling, general and administrative	16,935	16,208	57,262	38,743
Total operating expenses	20,367	27,153	67,385	72,486
Income (loss) from operations	4,143	(27,306)	(72,258)	(71,587)
Other (expense) income:				
Gain on sale of PRV	—	—	148,325	—
Interest expense	(2,051)	(2,312)	(6,029)	(5,157)
Fair value adjustment related to warrant and CVR liability	(5,506)	(4,746)	(1,379)	4,660
Fair value adjustment related to investments	124	90	119	64
Interest and other income, net	2,313	1,049	5,234	2,248
Total other (expense) income	(5,120)	(5,919)	146,270	1,815
(Loss) income before income taxes	(977)	(33,225)	74,012	(69,772)
Income tax benefit (expense)	433	—	(2,948)	—
Net (loss) income	\$ (544)	\$ (33,225)	\$ 71,064	\$ (69,772)
Net (loss) income per share of common stock:				
Basic	\$ (0.01)	\$ (0.69)	\$ 1.20	\$ (1.59)
Diluted	\$ (0.01)	\$ (0.69)	\$ 1.16	\$ (1.59)
Weighted-average shares of common stock outstanding:				
Basic	55,951,572	47,808,817	54,949,483	43,843,851
Diluted	55,951,572	47,808,817	56,782,763	43,843,851

*See accompanying notes to unaudited condensed consolidated financial statements.*

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

	<b>Three months ended September 30,</b>		<b>Nine months ended September 30,</b>	
	<b>2025</b>	<b>2024</b>	<b>2025</b>	<b>2024</b>
Net (loss) income	\$ (544)	\$ (33,225)	\$ 71,064	\$ (69,772)
Other comprehensive income (loss):				
Foreign currency translation adjustment	12	(536)	(3,047)	(72)
Other comprehensive income (loss):	12	(536)	(3,047)	(72)
Comprehensive (loss) income	<u>\$ (532)</u>	<u>\$ (33,761)</u>	<u>\$ 68,017</u>	<u>\$ (69,844)</u>

*See accompanying notes to unaudited condensed consolidated financial statements.*

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

	Common Stock	Additional Paid-in Capital	Treasury Stock, at cost	Accumulated Deficit	Other Comprehensive Income (Loss)	Total Stockholders' Equity
Balance as of January 1, 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 options exercised or RSUs vested	—	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 options exercised or RSUs vested	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 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, 2024	\$ 4	\$ 472,664	\$ (10,983)	\$ (399,778)	\$ (43)	\$ 61,864
Net loss	—	—	—	(16,622)	—	(16,622)
Stock-based compensation expense	—	2,190	—	—	—	2,190
Issuance of common stock in exchange for consulting services	—	56	—	—	—	56
Issuance of common stock for options exercised	—	1,146	—	—	—	1,146
Other comprehensive income	—	—	—	—	184	184
Balance as of March 31, 2024	\$ 4	\$ 476,056	\$ (10,983)	\$ (416,400)	\$ 141	\$ 48,818
Net loss	—	—	—	(19,925)	—	(19,925)
Stock-based compensation expense	—	2,688	—	—	—	2,688
Issuance of common stock in exchange for consulting services	—	193	—	—	—	193
Issuance of common stock as part of the Employee Stock Purchase Plan	—	424	—	—	—	424
Other comprehensive income	—	—	—	—	280	280
Balance as of June 30, 2024	\$ 4	\$ 479,361	\$ (10,983)	\$ (436,325)	\$ 421	\$ 32,478
Net loss	—	—	—	(33,225)	—	(33,225)
Stock-based compensation expense	—	6,229	—	—	—	6,229
Issuance of common stock in public offering	1	64,516	—	—	—	64,517
Issuance of common stock in exchange for consulting services	—	150	—	—	—	150
Issuance of common stock for options exercised	—	157	—	—	—	157
Other comprehensive loss	—	—	—	—	(536)	(536)
Balance as of September 30, 2024	\$ 5	\$ 550,413	\$ (10,983)	\$ (469,550)	\$ (115)	\$ 69,770

*See accompanying notes to unaudited condensed consolidated financial statements.*

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

	Nine months ended September 30,	
	2025	2024
<b>Cash flows from operating activities:</b>		
Net income (loss)	\$ 71,064	\$ (69,772)
Adjustments to reconcile net income (loss) to net cash used in operating activities:		
Stock-based compensation expense	8,350	10,888
Impairment of intangible assets	58,710	—
Inventory obsolescence charge	11,681	5,244
Income tax expense	2,948	—
Depreciation and amortization expense	3,692	4,741
Non-cash interest expense	2,037	1,489
Fair value adjustment related to warrant and CVR liability	1,379	(4,660)
Accretion on investments	(2,271)	—
Fair value adjustment related to investments	(119)	(64)
Consulting fees paid in common stock	75	399
Loss on foreign currency exchange rates	126	147
Gain on sale of PRV	(148,325)	—
Change in assets and liabilities:		
Accounts and other receivables	(5,610)	9,587
Prepaid expenses and other current assets	(3,342)	(452)
Inventories	(780)	(4,159)
Operating lease right-of-use assets	476	389
Accounts payable and accrued expenses	(15,999)	(10,590)
Discount and rebate liabilities	4,517	4,824
Operating lease liabilities	(546)	(395)
Other liabilities	4,823	(1,031)
Net cash used in operating activities	(7,114)	(53,415)
<b>Cash flows from investing activities:</b>		
Purchases of property and equipment	(866)	—
Disposals of property and equipment	589	—
Purchases of investments	(222,823)	(41,488)
Maturities of investments	91,000	24,798
Proceeds from sale of PRV	150,000	—
Net cash provided by (used in) investing activities	17,900	(16,690)
<b>Cash flows from financing activities:</b>		
Proceeds from issuance of debt, net of lender fees	—	58,990
Payment of third-party debt issuance costs	—	(2,091)
Repayment of debt	—	(42,700)
Proceeds from insurance financing arrangements	—	1,082
Proceeds from Employee Stock Purchase Plan	413	643
Proceeds from issuance of stock	—	64,516
Proceeds from issuance of common stock for options exercised or RSUs vested	3,520	1,303
Proceeds from issuance of common stock for warrants exercised	5,957	—
Payments of principal on insurance financing arrangements	(372)	(431)
Net cash provided by financing activities	9,518	81,312
Effect of exchange rate changes on cash and cash equivalents	350	(217)
Net increase in cash and cash equivalents	20,654	10,990
Cash and cash equivalents, beginning of period	33,785	43,049
Cash and cash equivalents, end of period	\$ 54,439	\$ 54,039
<b>Supplemental cash flow information:</b>		
Cash paid for interest	\$ 3,992	\$ 3,668
Right-of-use assets obtained in exchange for lease liabilities	1,115	419

*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 purpose-driven, commercial-stage company focused on bringing life-changing therapeutics to people living with rare diseases. The Company has a diverse portfolio of products and product candidates, which includes pre-clinical, clinical, and commercial stage assets.

On September 20, 2024, the U.S. Food and Drug Administration (“FDA”) approved the New Drug Application (“NDA”) for MIPLYFFA<sup>®</sup> (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. MIPLYFFA has also been granted orphan medicinal product designation for the treatment of NPC by the European Commission. The Company's other commercial stage asset, OLPRUVA<sup>®</sup> (sodium phenylbutyrate) for oral suspension, is approved by the FDA for the treatment of certain urea cycle disorders (“UCDs”).

Additionally, the Company has a pipeline of investigational product candidates, including celioprolol for the treatment of Vascular Ehlers-Danlos syndrome (“VEDS”) in patients with a confirmed type III collagen mutation and KP1077, the Company's clinical development product candidate being developed to treat idiopathic hypersomnia (“IH”), a rare neurological sleep disorder, and narcolepsy. The sole active pharmaceutical ingredient of KP1077 is serdexmethylphenidate (“SDX”), the Company's proprietary prodrug of d-methylphenidate (“d-MPH”). The FDA has granted KP1077 orphan drug designation for the treatment of IH.

***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 an aggregate of 2,269,721 shares of Zevra's common stock. On April 5, 2024, the Company filed an amendment to such registration statement, which was declared effective on April 8, 2024.

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

***August 2024 Offering***

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

### ***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, 2025, no shares have been issued or sold under the 2024 ATM Agreement.

### ***Reclassifications***

Certain reclassifications were made to historical period's unaudited condensed consolidated financial statements to conform to the classifications used in the current year. These reclassifications had no impact on the consolidated net (loss) income, changes in stockholder's 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 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, among others. The Company bases its estimates on historical experience and on various other assumptions that it believes to be reasonable, the results of which form the basis for making judgments about the carrying value of assets and liabilities.

### ***Investments***

The Company maintains investment securities that are classified as available-for-sale securities for which the Company has elected the fair value option under Financial Accounting Standards Board (“FASB”) Accounting Standards Codification (“ASC”) Topic 825, *Financial Instruments*. As such, these securities are carried at fair value with unrealized gains and losses included in fair value adjustment related to investments on the unaudited condensed consolidated statements of operations. The securities primarily consist of U.S. Treasury securities and corporate bonds and are included in securities at fair value in the unaudited condensed consolidated balance sheets. As of September 30, 2025, and December 31, 2024, the Company held securities with an aggregate fair value of \$175.9 million and \$41.7 million, respectively, that contained an aggregate unrealized gain of \$119,000 and \$18,000, respectively. For securities held at September 30, 2025, approximately \$145.1 million mature within one year and approximately \$30.9 million mature in one to three years. Applying fair value accounting to these debt securities more accurately represents the Company's investment strategy due to the fact that excess cash is currently being invested for the purpose of funding future operations. Interest income is recognized as earned using an effective yield method giving effect to the amortization of premium and accretion of discount and is based on the economic life of the securities. Interest income is included in interest and other income, net, in the unaudited condensed consolidated statements of operations.

### ***Variable Interest Entities***

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

To assess whether the Company has the power to direct the activities of the VIE that most significantly impact the VIE's economic performance, the Company considers all the facts and circumstances, including the Company's role in establishing the VIE and the

Company's ongoing rights and responsibilities. The assessment includes identifying the activities that most significantly impact the VIE's economic performance and identifying which party, if any, has the power to direct those activities. In general, the parties that make the most significant decisions affecting the VIE (management and 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. On August 30, 2023, the Company entered into an Agreement and Plan of Merger (the "Merger Agreement") with Acer Therapeutics Inc. ("Acer"). On November 17, 2023 (the "Closing Date"), Zevra completed the acquisition of Acer (the "Merger"). Pursuant to the Merger Agreement, on the Closing Date, Acer continued as the surviving entity and as a wholly-owned subsidiary of Zevra. As of September 30, 2025, and December 31, 2024, the Company identified 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 priority review voucher ("PRV") in conjunction with the FDA approval of MIPLYFFA. On February 27, 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 for 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 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 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.

### ***Arimoclomol French AC***

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

### ***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, 2025, or December 31, 2024. 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, 2025, and December 31, 2024, 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, 2025, the Company recognized charges of approximately \$0 and \$11.7 million, respectively, related to write-downs for unsaleable inventory. For the three and nine months ended September 30, 2024, the Company recognized charges of approximately \$2.0 million and \$5.2 million, respectively, 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 French AC, royalties or net sales milestone payments generated under the Collaboration and License Agreement with Commave Therapeutics SA (the "AZSTARYS<sup>®</sup> 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. As a condition to entering into the Merger Agreement, Acer and Relief Therapeutics SA ("Relief") entered into an exclusive license agreement on August 30, 2023 (the "Relief License Agreement"). Pursuant to the Relief License Agreement, Zevra was 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, the rights to this royalty were sold to Soleus Capital Management L.P.

In connection with the AZSTARYS License Agreement, the Company pays Aquestive Therapeutics, Inc. ("Aquestive") a royalty equal to 10% of all regulatory milestone and royalty payments.

### ***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.

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.

The Company files income tax returns in the United States for federal and various state jurisdictions. With few exceptions, the Company is no longer subject to U.S. federal and state and local income tax examinations for years prior to 2015, although carryforward attributes that were generated prior to 2017 may still be adjusted upon examination by the Internal Revenue Service if used in a future period. No income tax returns are currently under examination by taxing authorities.

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 and offset by net operating loss carryforwards in the U.S.

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. 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 2025.

### ***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.

### ***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 changes in 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 Black-Scholes-Merton (“BSM”) option pricing model.

### ***New Accounting Pronouncements Not Yet Adopted***

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

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

### **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 reimbursements received from product sales of MIPLYFFA and OLPRUVA, reimbursements received under the French AC, and royalties or net sales milestone payments generated under the AZSTARYS License Agreement. The accounting policies of the segment are the same as those described in Note B.

Zevra's CODM is the Company's Chief Executive Officer, Neil F. McFarlane. The CODM uses net income (loss), 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 following table presents the operating results of the Company's segment for the three and nine months ended September 30, 2025, and 2024:

	Three months ended September 30,		Nine months ended September 30,	
	2025	2024	2025	2024
Total revenues	\$ 26,063	\$ 3,695	\$ 72,345	\$ 11,569
Less significant segment expenses:				
Research and development directly identified to programs	1,780	4,916	6,480	18,369
Research and development not directly identified to programs	1,652	6,029	3,643	15,374
Selling, general and administrative directly identified to programs	5,144	4,864	19,144	12,560
Selling, general and administrative not directly identified to programs	11,791	11,344	38,118	26,183
Other segment items:				
Impairment of intangible assets	—	—	58,710	—
Income tax (benefit) expense	(433)	—	2,948	—
Interest income	(2,333)	(899)	(5,308)	(2,366)
Depreciation and amortization expense	392	1,617	3,692	4,741
Interest expense	2,051	2,312	6,029	5,157
Other expense (income), net (a)	6,563	6,737	(132,175)	1,323
Segment net (loss) income	\$ (544)	\$ (33,225)	\$ 71,064	\$ (69,772)

(a) Other expense, net, included in segment net (loss) income includes the gain on the sale of the PRV, foreign currency exchange gains and losses, cost of product revenue (excluding intangible asset amortization), fair value adjustment related to warrant and contingent value right (“CVR”) liabilities, fair value adjustment related to investments, and other overhead expenses.

The Company holds long-lived assets in the United States of \$2.2 million and \$13.4 million as of September 30, 2025, and December 31, 2024, respectively. The Company holds long-lived assets in Europe of \$0.4 million and \$0.5 million as of September 30, 2025, and December 31, 2024, respectively.

#### D. Inventories

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

	September 30, 2025	December 31, 2024
Raw materials	\$ —	\$ 7,928
Work in progress	1,224	3,260
Finished goods	863	1,781
Total inventories	\$ 2,087	\$ 12,969

#### 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 is divided into three tranches as follows: (i) \$60.0 million, which was funded in full on the Term Loans Closing Date; (ii) \$20.0 million, which 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 principal amount of the Term Loans outstanding (the “Outstanding Principal Amount”) will bear interest at a rate equal to 3-Month Term Secured Overnight Financing Rate (“SOFR”) plus 7.00% per annum. If the net product sales for the calendar year ending December 31, 2025 exceed \$100.0 million, the Outstanding Principal Amount will bear interest at 3-Month Term SOFR plus 6.00% per annum. If the net product sales for the calendar year ending December 31, 2025 do not exceed \$100.0 million, then for any subsequent period of four consecutive fiscal quarters ending on or after March 31, 2026, in which net product sales exceed \$125.0 million, the Outstanding Principal Amount will bear interest at 3-Month Term SOFR plus 6.50% per annum. In all cases, the 3-Month Term SOFR rate will be subject to a floor of 4.00% per annum. Interest will be payable quarterly in arrears on the last day of each calendar quarter. The Company has the option to pay up to 25% of the interest in-kind beginning on the Term Loans Closing Date, through and including June 30, 2026. The Company has recognized approximately \$2.7 million and \$1.4 million of interest-in-kind as of September 30, 2025, and December 31, 2024, which is included in long-term debt in the unaudited condensed consolidated balance sheets. The Term Loans will mature on the fifth anniversary of the Term Loans Closing Date. In connection with the Credit Agreement, the Company incurred approximately \$2.2 million of costs, which primarily consisted of underwriting, legal and other professional fees, and are included as a reduction to the carrying amount of the related debt liability and are deferred and amortized over the remaining life of the financing using the effective interest method.

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

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

	September 30, 2025	December 31, 2024
Notes payable	\$ 63,090	\$ 61,552
Unamortized original issue discount	(801)	(921)
Less: debt issuance costs	(979)	(1,127)
	<u>\$ 61,310</u>	<u>\$ 59,504</u>

Future minimum principal payments under the Term Loans as of September 30, 2025, were as follows (in thousands):

<b>Year Ended December 31,</b>	
2025 (excluding the nine months ended September 30, 2025)	\$ —
2026	—
2027	—
2028	—
2029	63,090
Total minimum payments	63,090
Less: unamortized debt discount, debt issuance costs and paid in kind interest	(1,780)
Long-term debt	<u>\$ 61,310</u>

#### F. Revenue, net

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. For the three and nine months ended September 30, 2024, the Company recorded \$3.7 million and \$11.6 million, respectively, of revenue.

#### Product Revenues, Net

On December 27, 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 UCs. For the three and nine months ended September 30, 2025, sales of OLPRUVA were \$0.1 million and \$0.5 million, respectively. For the three and nine months ended September 30, 2024, sales of OLPRUVA were de minimis.

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, 2025, sales of MIPLYFFA were \$22.4 million and \$61.0 million, respectively.

The Company currently utilizes a single specialty pharmacy provider as its sole distributor for both 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. To commercialize MIPLYFFA and OLPRUVA in the U.S., 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.

Adjustments to product revenue recognized as a result of changes in estimates for discounts and allowances for products shipped in previous periods were 4% and 1% of revenue, net, on the unaudited condensed consolidated statements of operations during the three and nine months ended September 30, 2025, respectively. No adjustments to product revenue were recognized as a result of changes in estimates during the three and nine months ended September 30, 2024.

#### Arimoclomol French AC

For the three and nine months ended September 30, 2025, the Company recognized revenue related to the French AC of \$2.4 million and \$7.3 million, respectively, net of a clawback liability of \$1.5 million and \$4.5 million, respectively, and other gross to net adjustments. For the three and nine months ended September 30, 2024, the Company recognized revenue related to the French AC of

\$2.6 million and \$8.0 million, respectively, net of a clawback liability of \$1.4 million and \$4.4 million, respectively, and other gross to net adjustments.

The total estimated reserve liability as of September 30, 2025, and December 31, 2024, was \$18.9 million and \$12.6 million, respectively. As of September 30, 2025, and December 31, 2024, 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**

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

The Company concluded that these regulatory milestones, sales milestones and royalty payments each contain a significant uncertainty associated with a future event. As such, these milestone and royalty payments are constrained at contract inception and are not included in the transaction price, as the Company could not conclude that it is probable a significant reversal in the amount of cumulative revenue recognized will not occur surrounding these milestone payments. At the end of each reporting period, the Company updates its assessment of whether the milestone and royalty payments are constrained by considering both the likelihood and magnitude of the potential revenue reversal. For the three and nine months ended September 30, 2025, the Company recognized revenue under the AZSTARYS License Agreement of \$1.2 million and \$3.3 million, respectively. For the three and nine months ended September 30, 2024, the Company recognized revenue under the AZSTARYS License Agreement of \$1.1 million and \$3.6 million, respectively. There was no deferred revenue related to this agreement as of September 30, 2025, or December 31, 2024. All revenues recognized under this agreement were derived in the United States.

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**

Pursuant to the Relief License Agreement, Relief will hold exclusive development and commercialization rights for OLPRUVA in the EU, Liechtenstein, San Marino, Vatican City, Norway, Iceland, Principality of Monaco, Andorra, Gibraltar, Switzerland, United Kingdom, Albania, Bosnia, Kosovo, Montenegro, Serbia and North Macedonia (“Geographical Europe”). The Company 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, 2025, and 2024, the Company did not recognize any revenue under the Relief License Agreement. There was no deferred revenue related to this agreement as of September 30, 2025, and December 31, 2024.

### **Accounts and Other Receivables**

Accounts and other receivables consist of receivables from product sales of MIPLYFFA and OLPRUVA, reimbursements received under the French AC, royalties generated under the AZSTARYS License Agreement, and other receivables due to the Company. Receivables under the AZSTARYS License Agreement are recorded for amounts due to the Company related to reimbursable third-party costs as well as milestones and royalties on product sales. Receivables are recorded for commercial sales of MIPLYFFA and OLPRUVA to a single specialty pharmacy and product sales under the French AC. 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, 2025	December 31, 2024
Commercial accounts receivable	\$ 8,770	\$ 4,010
Receivables related to product reimbursements	6,056	5,380
Royalties accounts receivable	1,200	786
Other receivables	815	333
Total receivables	<u>\$ 16,841</u>	<u>\$ 10,509</u>

As of September 30, 2025, and December 31, 2024, no reserve or allowance for doubtful accounts had been established.

## G. Stock and Warrants

### *Authorized, Issued, and Outstanding Common Shares*

As of September 30, 2025, and December 31, 2024, the Company had authorized shares of common stock of 250,000,000 shares. Of the authorized shares, 57,793,414 and 55,246,401 shares of common stock were issued as of September 30, 2025, and December 31, 2024, respectively, and 56,217,722 and 53,670,709 shares of common stock were outstanding as of September 30, 2025, and December 31, 2024, respectively.

As of September 30, 2025, and December 31, 2024, the Company had reserved authorized shares of common stock for future issuance as follows:

	September 30, 2025	December 31, 2024
Outstanding awards under equity incentive plans	7,228,555	7,789,658
Outstanding common stock warrants	4,436,637	5,483,537
Possible future issuances under equity incentive plans	6,422,637	5,383,165
Possible future issuances under employee stock purchase plans	1,055,685	1,148,012
Total common shares reserved for future issuance	<u>19,143,514</u>	<u>19,804,372</u>

### *Common Stock Activity*

The following table summarizes common stock activity for the three and nine months ended September 30, 2025:

	Shares of Common Stock
Balance as of January 1, 2025	53,670,709
Common stock issued as compensation to third parties	9,306
Common stock issued as a result of stock options exercised or RSUs vested	999,338
Common stock issued as a result of stock warrants exercised	10
Balance as of March 31, 2025	<u>54,679,363</u>
Common stock issued as a result of stock options exercised or RSUs vested	312,514
Common stock issued as a result of the Employee Stock Purchase Plan	92,327
Balance as of June 30, 2025	<u>55,084,204</u>
Common stock issued as a result of stock options exercised or RSUs vested	86,628
Common stock issued as a result of stock warrants exercised	1,046,890
Balance as of September 30, 2025	<u>56,217,722</u>

### ***Authorized, Issued, and Outstanding Preferred Stock***

As of September 30, 2025, and December 31, 2024, the Company had 10,000,000 shares of authorized preferred stock, none of which were designated, issued, or outstanding.

### ***Warrants to Purchase Common Stock***

The Company has issued warrants to purchase common stock to various third parties, of which 4,436,637 remain outstanding as of September 30, 2025, 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. 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, 2025, 1,046,900 of the warrants had been exercised. No warrants had been exercised as of December 31, 2024. 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, 2025, and December 31, 2024, the fair value of the liability associated with these warrants was approximately \$13.8 million and \$17.8 million, 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. The fair value adjustment related to these warrants for the three and nine months ended September 30, 2024 was \$6.0 million of loss and \$2.2 million of income, 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 12,079,711 as of September 30, 2025. 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, 2025, the common stock reserved for issuance under the A&R 2014 Plan automatically increased by 2,146,828 shares.

During the three and nine months ended September 30, 2025, 86,628 and 1,162,133 stock options were exercised, respectively. During the three and nine months ended September 30, 2024, 27,750 and 761,500 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, 2025, 444,315 shares have been issued under the ESPP.

In January 2023, the Board approved the 2023 Employment Inducement Award Plan (as amended, the "2023 Plan"). The maximum number of shares of common stock that may be issued under the 2023 Plan was 4,500,000 as of September 30, 2025.

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,	
	2025	2024	2025	2024
Research and development	\$ 250	\$ 1,441	\$ 721	\$ 3,322
Selling, general and administrative	2,521	4,696	7,629	7,566
Total stock-based compensation expense	\$ 2,771	\$ 6,137	\$ 8,350	\$ 10,888

There was no stock-based compensation expense related to performance-based awards recognized during the three and nine months ended September 30, 2025. During the three and nine months ended September 30, 2024, the Company recognized approximately \$2.5 million in stock-based compensation expense related to performance-based awards.

## 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, 2025, and December 31, 2024 (in thousands):

	Balance as of September 30, 2025	Quoted Prices in		
		Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
CVR liability	\$ 1,750	\$ —	\$ —	\$ 1,750
Warrant liabilities	13,767	—	—	13,767
Total liabilities	\$ 15,517	\$ —	\$ —	\$ 15,517
Securities:				
U.S. Treasury securities	\$ 107,083	\$ 107,083	\$ —	\$ —
Corporate bonds	68,852	—	68,852	—
Total assets	\$ 175,935	\$ 107,083	\$ 68,852	\$ —

	Balance as of December 31, 2024	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
CVR liability	\$ 3,500	\$ —	\$ —	\$ 3,500
Warrant liabilities	17,804	—	—	17,804
Total liabilities	<u>\$ 21,304</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 21,304</u>
<b>Securities:</b>				
U.S. Treasury securities	\$ 35,711	\$ 35,711	\$ —	\$ —
Corporate bonds	6,010	—	6,010	—
Total assets	<u>\$ 41,721</u>	<u>\$ 35,711</u>	<u>\$ 6,010</u>	<u>\$ —</u>

### **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, 2025, and December 31, 2024:

	September 30, 2025	December 31, 2024
Risk-free interest rate	3.55% - 3.92%	4.08% - 4.23%
Volatility	60.61% - 63.88%	62.14% - 68.68%
Dividend yield	— %	— %
Expected term (years)	0.27 - 3.15	1.02 - 3.89
Weighted average fair value	\$ 3.10	\$ 3.25

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, 2024	\$ 17,804
Change in fair value measurement of warrant liabilities	3,129
Warrants exercised	(7,166)
Balance as of September 30, 2025	<u>\$ 13,767</u>

For the nine months ended September 30, 2025, the changes in fair value of the warrant liabilities primarily resulted from changes in the discount rate.

### **Contingent Consideration**

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

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

Balance as of December 31, 2024	\$ 3,500
Change in fair value measurement of contingent consideration liabilities	(1,750)
Balance as of September 30, 2025	<u>\$ 1,750</u>

For the nine months ended September 30, 2025, the changes in fair value of contingent consideration primarily resulted from changes in market data and revenue projections.

#### 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.

Diluted net loss per share of common stock is the same as basic net loss per share of common stock for the three and nine months ended September 30, 2024, because the effects of potentially dilutive items were anti-dilutive for the respective periods. 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,	
	2025	2024	2025	2024
Awards under equity incentive plans	7,049,222	8,987,997	1,771,663	8,987,997
Common stock warrants	4,436,637	5,483,537	—	5,483,537
Total securities excluded from the calculation of weighted average number of shares of common stock outstanding	<u>11,485,859</u>	<u>14,471,534</u>	<u>1,771,663</u>	<u>14,471,534</u>

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

	<b>Three Months Ended September 30, 2025</b>	<b>Nine Months Ended September 30, 2025</b>
<b>Basic net (loss) income per share of common stock:</b>		
Net (loss) income	\$ (544)	\$ 71,064
Earnings allocated to participating securities	—	(5,309)
<b>Net (loss) income attributable to shares of common stock</b>	<b>(544)</b>	<b>65,755</b>
Less: Dividends declared or accumulated	—	—
<b>Undistributed net (loss) income attributable to shares of common stock, basic</b>	<b>(544)</b>	<b>65,755</b>
Weighted-average shares of common stock outstanding	55,952	54,949
<b>Basic net (loss) income per share of common stock</b>	<b>\$ (0.01)</b>	<b>\$ 1.20</b>
<b>Diluted net (loss) income per share of common stock:</b>		
Net (loss) income attributable to shares of common stock	\$ (544)	\$ 65,755
Less: Fair value adjustment income related to warrant liability	—	—
<b>Net (loss) income attributable to shares of common stock, diluted</b>	<b>(544)</b>	<b>65,755</b>
Weighted-average number of shares of common stock outstanding	55,952	54,949
Dilutive effect of outstanding stock options (as converted to common stock)	—	1,834
<b>Weighted-average shares of common stock outstanding, diluted</b>	<b>55,952</b>	<b>56,783</b>
<b>Diluted net (loss) income per share of common stock</b>	<b>\$ (0.01)</b>	<b>\$ 1.16</b>

## K. Leases

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

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

Lease Cost	<b>Three months ended September 30,</b>		<b>Nine months ended September 30,</b>	
	<b>2025</b>	<b>2024</b>	<b>2025</b>	<b>2024</b>
<b>Finance lease cost:</b>				
Amortization of right-of-use assets	\$ 1	\$ 12	\$ 4	\$ 36
Total finance lease cost	1	12	4	36
Operating lease cost	205	119	612	330
Short-term lease cost	32	59	127	177
Variable lease cost	10	26	36	39
Less: sublease income	(27)	(50)	(105)	(128)
<b>Total lease costs</b>	<b>\$ 221</b>	<b>\$ 166</b>	<b>\$ 674</b>	<b>\$ 454</b>

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

	Nine months ended September 30,	
	2025	2024
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	\$ 688	\$ 405
Operating cash flows from short-term leases	127	177
Operating cash flows from variable lease costs	36	39
Right-of-use assets obtained in exchange for lease liabilities:		
Operating leases	\$ 1,115	\$ 419

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

	September 30, 2025	December 31, 2024
<b>Finance Leases</b>		
Property and equipment, at cost	\$ 1,031	\$ 1,031
Less: accumulated depreciation and amortization	(1,026)	(1,023)
Property and equipment, net	\$ 5	\$ 8
<b>Operating Leases</b>		
Operating lease right-of-use assets	\$ 1,337	\$ 657
Current portion of operating lease liabilities	\$ 441	\$ 420
Operating lease liabilities, less current portion	966	372
Total operating lease liabilities	\$ 1,407	\$ 792
<b>Weighted Average Remaining Lease Term</b>		
Operating leases (in years)	3	3
<b>Weighted Average Discount Rate</b>		
Operating leases	12.9%	9.9%

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

Year Ended December 31,	Operating Leases
2025 (excluding the nine months ended September 30, 2025)	\$ 172
2026	552
2027	542
2028	393
2029	39
Total lease payments	1,698
Less: future interest expense	(291)
Lease liabilities	\$ 1,407

**L. Goodwill & Intangible Assets**

The Company's goodwill balance was \$4.7 million as of September 30, 2025, and December 31, 2024.

As of September 30, 2025, and December 31, 2024, non-amortizable intangible assets include \$2.0 million related to in-process research and development associated with the Merger.

The definite-lived intangible assets that are subject to amortization have been reviewed for impairment whenever events or changes in circumstances indicate 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 is not recoverable. Future cash flows specific to OLPRUVA, which most significantly includes an estimate of forecasted revenues, are based on reasonable and supportable assumptions regarding the cash flows expected to result from the use of the asset and its eventual disposition. 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 of \$0 and \$58.7 million, respectively, in the unaudited condensed consolidated statement of operations for the three and nine months ended September 30, 2025. As of December 31, 2024, the Company had a definite-lived intangible asset, net, related to the acquisition of OLPRUVA of \$61.3 million.

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 and \$2.6 million for the three and nine months ended September 30, 2025, respectively. There was \$1.5 million and \$4.6 million of amortization expense related to this intangible asset for the three and nine months ended September 30, 2024, 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 U.S., 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 and \$0.9 million for the three and nine months ended September 30, 2025, respectively. Amortization expense related to this intangible asset was de minimis for the three and nine months ended September 30, 2024.

For intangible assets subject to amortization, estimated amortization expense for the five fiscal years subsequent to September 30, 2025, is expected to be as follows:

2025 (excluding the nine months ended September 30, 2025)	\$	316
2026		1,263
2027		1,263
2028		1,263
2029		632

**M. Commitments and Contingencies**

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

*Litigation Related to the AZSTARYS License Agreement*

In September 2024, the Company became engaged in a legal dispute regarding the AZSTARYS License Agreement. The litigation is currently in the discovery phase. The Company cannot predict with certainty the timing or ultimate outcome of this litigation or its potential impact on the Company's business, financial condition, or results of operations. At this time, the Company has not recorded any accrual for contingent liability associated with this matter. The AZSTARYS License Agreement remains in effect during this litigation, and both parties continue to perform their respective obligations thereunder. However, there can be no assurance that this

dispute will not have an adverse impact on the Company's relationship with Commave or on the Company's business. The Company will continue to monitor developments in this matter and will assess the potential impact on the Company's financial statements in future periods. The Company expects to incur significant legal expenses in connection with this litigation, which may materially affect its results of operations in future periods.

As of September 30, 2025, and December 31, 2024, no accruals were made related to commitments and contingencies.

#### **N. Subsequent Events**

The Company evaluated events and transactions occurring subsequent to September 30, 2025, through November 5, 2025, 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, 2024, filed with the SEC on March 12, 2025, 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 purpose-driven, commercial-stage company focused on bringing life-changing therapeutics to people living with rare diseases. With a data-driven scientific approach and patient-first perspective, we are overcoming complex challenges to deliver therapies to the rare disease community. We have a diverse portfolio of products and product candidates, which includes pre-clinical, clinical, and commercial stage assets. Our team has specialized expertise and a track record of success in advancing promising therapies that face complex clinical, regulatory, and commercial challenges with an approach that balances science with patient need.

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

Our five-year strategic plan is focused on the continued transformation of Zevra into a leading rare-disease company. In addition to the commercialization of OLPRUVA and the approval and subsequent launch of MIPLYFFA, in the third quarter of 2024 we discontinued our in-house drug discovery activities and closed our laboratory facilities in Iowa and Virginia to prioritize near-term resources for our late-stage clinical development and commercial opportunities. In the future, we plan to outsource our discovery and early development activities and further expand our pipeline through both internal development and our business development activities to collaborate, partner, and potentially acquire additional assets. We intend to target 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. If we are successful, expanding our pipeline could be accretive to our value proposition and has the potential to create incremental long-term value for stockholders.

We continue to optimize and curate our IP portfolio through a variety of avenues to extract value for the benefit of stockholders. As part of our ongoing IP portfolio review, we licensed certain IP related to our pre-clinical stage prodrug of dextrophan in April 2025 to an undisclosed party for an upfront payment of \$250,000, potential future regulatory milestones of up to \$8.45 million and single-digit royalties on net sales. This licensing revenue is included in revenue, net, on the unaudited condensed consolidated statements of operations for the nine months ended September 30, 2025.

On September 20, 2024, the FDA approved the New Drug Application ("NDA") for MIPLYFFA (arimoclomol), for use in combination with miglustat for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adult and pediatric patients 2 years of age and older. MIPLYFFA is an orally-delivered treatment for NPC, which is an ultra-rare and progressive neurodegenerative disease. 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. MIPLYFFA has also been granted orphan medicinal product designation for the treatment of NPC by the European Commission. In November 2024, MIPLYFFA became commercially available for dispense in the United States.

On August 30, 2023, we entered into an Agreement and Plan of Merger (the "Merger Agreement") with Acer Therapeutics Inc. ("Acer"). On November 17, 2023 (the "Closing Date"), we completed the acquisition of Acer (the "Merger"). Pursuant to the Merger Agreement, Acer continues as a wholly-owned subsidiary of Zevra. The Merger included the acquisition of OLPRUVA (sodium phenylbutyrate) for oral suspension, which was approved by the FDA on December 27, 2022, for the treatment of certain urea cycle disorders (UCDs). 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.

In May 2022, we purchased all of the assets and operations of Orphazyme A/S ("Orphazyme") related to arimoclomol and agreed to assume an estimated reserve clawback liability of \$5.2 million related to revenue generated from the sale of arimoclomol for the treatment of NPC under the remunerated expanded access program in France, Accès Compassionnel ("French AC").

## Our Product Candidates and Approved Products

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

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	Development Status	Next Milestone(s)
Arimocloamol	Niemann-Pick disease type C (NPC)	MIPLYFFA	FDA Approved European Expanded Access Program (“EAP”) Marketing Authorisation Application (“MAA”) Submitted to European Medicines Agency (“EMA”) July 2025	Tracking U.S. Commercial Progress EMA Decision
Sodium phenylbutyrate	Urea Cycle Disorders (UCD)	OLPRUVA	FDA Approved	Tracking Commercial Progress
Celiprolol	Vascular Ehlers Danlos Syndrome (VEDS)	Celiprolol	Clinical - Phase 3	Ongoing Phase 3 Trial Fully Enrolled
Serdexmethylphenidate	Idiopathic Hypersomnia (IH)	KP1077IH	Clinical - Phase 2	Phase 3 Trial Ready; Seeking Strategic Alternatives
Serdexmethylphenidate	Narcolepsy	KP1077N	Clinical - Phase 1/2	Phase 3 Trial Potential; Seeking Strategic Alternatives
Serdexmethylphenidate and dexmethylphenidate	Attention Deficit and Hyperactivity Disorder (ADHD)	AZSTARYS	FDA Approved and Partnered	Collecting Royalties and Milestones

These anticipated milestones are based on information currently available to us. Our current plans and expectations are subject to a number of uncertainties, risks and other important factors that could materially impact our plans, including risks which are not solely within our control. See Part I, Item 1A. “Risk Factors” of our Annual Report on Form 10-K for the fiscal year ended December 31, 2024, filed with the SEC on March 12, 2025, as updated by Part II, Item 1A. “Risk Factors” of this Quarterly Report on Form 10-Q.

### MIPLYFFA

NPC is characterized by an inability of the body to transport cholesterol and lipids inside of cells. Symptoms of NPC include a progressive impairment of mobility, cognition, speech, and swallowing, often culminating in premature death. The incidence of NPC is estimated to be one in 100,000 to 130,000 live births. We estimate that there are approximately 2,000 individuals with NPC in the U.S. and Europe combined, of which approximately 900 are in the U.S. 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 U.S. However, low diagnostic rates may affect the number of potential patients, and we believe that the availability of treatment options in the U.S. 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, in combination with miglustat, for NPC, which is an ultra-rare and progressive neurodegenerative disease. MIPLYFFA, the first FDA-approved treatment for NPC, is indicated for use in combination with miglustat for the treatment of neurological manifestations of NPC in adult and pediatric patients two years of age and older. In addition, we received a transferable rare pediatric disease PRV in conjunction with the approval. On April 1, 2025, we completed the previously disclosed sale of the PRV (“Asset Sale”). The Asset Sale was completed pursuant to the terms of an asset purchase agreement, dated February 26, 2025 (the “PRV Transfer Agreement”). Pursuant to the PRV Transfer Agreement, we received net proceeds of \$148.3 million from the buyer upon the closing of the Asset Sale.

Effective therapies to treat NPC are desperately needed, and, for this reason, MIPLYFFA is currently being made available to NPC patients in France, Germany, and other EU member states under various EAPs. MIPLYFFA has also been granted orphan medicinal product designation for the treatment of NPC by the European Commission.

As of September 30, 2025, there were a total of 137 enrollments to receive MIPLYFFA. Our commercial plans will focus on continuing to raise awareness among people who are living with NPC that are diagnosed and untreated, or undiagnosed. 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. Soon after approval of MIPLYFFA, the FDA approved a second therapy for NPC, AQNEURSA, which is approved for the treatment of neurological manifestations of NPC in adults and pediatric patients weighing  $\geq 15$  kg and is marketed by IntraBio, Inc.

To commercialize both of our commercial products, MIPLYFFA and OLPRUVA, in the U.S., we have built, or are making 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. We continue to evaluate the potential to obtain regulatory approval for and to commercialize MIPLYFFA outside of the U.S. We are currently focusing on seeking regulatory approval in Europe, and filed an MAA in July 2025.

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 halting 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 NPC EAPs, with no safety findings of concern found.
- ***Advantageous regulatory designations.*** MIPLYFFA has been granted orphan medicinal product designation for the treatment of NPC by the European Commission. On April 1, 2025, we completed the sale of the PRV, which we received upon approval of MIPLYFFA.

## ***OLPRUVA***

OLPRUVA (sodium phenylbutyrate) for oral suspension is approved in the U.S. as adjunctive therapy to standard of care, which includes dietary management, for the chronic management of UCIDs 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.

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

In the fourth quarter of 2023, we began generating revenue from the sale of OLPRUVA in the U.S. Zevra has a partnership with Relief Therapeutics SA (“Relief”), which has rights to commercialize OLPRUVA in various European countries, if approved.

In the second quarter of 2025, we 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 that the carrying amount of the OLPRUVA asset group may not be fully recoverable. We performed an undiscounted cash flow analysis over the OLPRUVA asset group and determined that the carrying value of the asset group is not recoverable. We then estimated the fair value of the asset group to measure the impairment loss for the period. Significant assumptions used to determine this fair value measurement included 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. We recorded an intangible asset impairment charge of \$58.7 million in the unaudited condensed consolidated statement of operations for the nine months ended September 30, 2025.

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.

### ***Celiprolol***

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

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

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

Celiprolol received orphan drug designation from the FDA for the treatment of VEDS in 2015. In October 2018, a new celiprolol NDA was submitted to the FDA by Acer based on data obtained from the BBEST trial and was subsequently accepted by the FDA in October 2018 with priority review status. Following FDA review, Acer received a CRL from the FDA stating that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with VEDS. Subsequently, Acer appealed the FDA decision. While the FDA denied the appeal, it described possible paths forward toward approval. In a May 2021 Type B meeting with the FDA, Acer discussed the conduct of an U.S.-based prospective, randomized, double-blind, placebo-controlled, decentralized clinical trial in patients with COL3A1 positive VEDS and sought the FDA’s opinion on various proposed design features of the study.

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

In July 2022, Acer initiated enrollment in a Phase 3 long-term event-driven clinical trial designed based on the discussions from the May 2021 Type B meeting with the FDA, also known as the DiSCOVER trial. The DiSCOVER trial intends to enroll 150 VEDS patients, with 100 patients receiving celiprolol and 50 patients receiving placebo. Recruitment in the Phase-3 trial was restarted mid-2024, and the trial has 44 enrolled participants as of September 30, 2025. 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. This outreach is ongoing with significant interest and participation.

Celiprolol summary:

- **Currently, no approved treatments for VEDS in the U.S.** There are currently no approved treatments for VEDS in the U.S., and we believe that celiprolol, if approved, could be a significant innovation in the treatment of VEDS in the U.S. where current treatment options are focused primarily on surgical intervention.
- **Unique pharmacological profile.** Mechanism of action in VEDS patients is thought to be through vascular dilatation and smooth muscle relaxation, the effect of which is to reduce the mechanical stress on collagen fibers in the arterial wall, and thereby potentially less incidence of vascular ruptures.
- **Evidence of efficacy in the EU and extensive clinical experience from multiple trials.** Celiprolol has become the primary treatment for VEDS patients in several European countries. BBEST Clinical Trial data showed 76% reduction in risk of arterial events observed in COLA3A1+ 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 U.S. if approved.
- **Solid patent protection through 2038.** Celiprolol is generally protected by U.S. patents that will expire, after utilizing all appropriate patent term adjustments but excluding possible term extensions, in 2038.

#### **KP1077**

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

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

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

We reported final data for the Phase 1 proof-of-concept study of SDX in the first quarter of 2022. Increased wakefulness, alertness, hypervigilance, and insomnia effects were reported by study participants, which we believe suggests that SDX produced targeted pharmacodynamic effects that have the potential to benefit patients with IH and other sleep disorders. In November 2022, we announced that the FDA has granted the orphan drug designation to SDX for the treatment of IH.

KP1077 utilizes SDX, our prodrug of d-MPH, as its active pharmaceutical ingredient. During the first quarter of 2022, we initiated a Phase 1 clinical trial comparing the cardiovascular safety of SDX to immediate-release and long-acting formulations of RITALIN, a commonly prescribed central nervous system (“CNS”) stimulant. In September 2022, we announced topline data from our exploratory Phase 1 clinical trial, which showed the potential for higher dose formulations of SDX to be safe and well tolerated, while avoiding the potential for greater cardiovascular safety risk compared to immediate-release and long-acting formulations of RITALIN.

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

Clinically meaningful improvements were observed across all endpoints studied. The exploratory endpoints of sleep inertia and brain fog performed in-line with expectations and were stable when compared across a variety of other endpoints. Symptom improvements in patients receiving KP1077 were similar after both once-per-day and twice-per-day dosing.

KP1077 Summary:

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

### ***AZSTARYS (Partnered product)***

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

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

Pursuant to the AZSTARYS License Agreement, Commave agreed to pay up to \$63.0 million in milestone payments upon the occurrence of specified regulatory milestones related to AZSTARYS, including FDA approval and specified conditions with respect to the final approval label. In addition, Corium agreed to make additional payments upon the achievement of specified U.S. sales milestones of up to \$420.0 million in the aggregate. Further, Commave will pay us quarterly, tiered royalty payments based on a percentage of net sales on a product-by-product basis. Corium also agreed to be responsible for and reimburse us for all of development, commercialization and regulatory expenses for any products or product candidates containing SDX, subject to certain limitations as set forth in the AZSTARYS License Agreement, including consultation fees to be paid to us for services provided to Corium in performing such activities.

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

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

### ***Other Third-Party Agreements***

#### **Distributor Agreement**

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

#### **MIPLYFFA License Agreements**

Prior to our acquisition of the assets of 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.

#### **OLPRUVA License Agreement**

Pursuant to the Relief License Agreement, Zevra was obligated to pay royalties of 10% of U.S. net sales 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, the rights to this royalty were sold to Soleus Capital Management L.P.

#### **Aquestive Termination Agreement**

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

## Relief Exclusive License Agreement

As a condition to entering into the Merger Agreement, Acer and Relief entered into an exclusive license agreement on August 30, 2023 (the “Relief License Agreement”). Pursuant to the Relief License Agreement, Relief will hold exclusive development and commercialization rights for OLPRUVA in the EU, Liechtenstein, San Marino, Vatican City, Norway, Iceland, Principality of Monaco, Andorra, Gibraltar, Switzerland, United Kingdom, Albania, Bosnia, Kosovo, Montenegro, Serbia and North Macedonia (“Geographical Europe”). 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, 2025, and 2024 (in thousands):*

	Three months ended September 30,		Period-to- Period Change
	2025	2024	
Revenue, net	\$ 26,063	\$ 3,695	\$ 22,368
Cost of product revenue (excluding \$315 and \$1,545 in intangible asset amortization for the three months ended September 30, 2025, and 2024, respectively, shown separately below)	1,238	2,303	(1,065)
Intangible asset amortization	315	1,545	(1,230)
Operating expenses:			
Research and development	3,432	10,945	(7,513)
Selling, general and administrative	16,935	16,208	727
Total operating expenses	20,367	27,153	(6,786)
Income (loss) from operations	4,143	(27,306)	31,449
Other expense:			
Interest expense	(2,051)	(2,312)	261
Fair value adjustment related to warrant and CVR liability	(5,506)	(4,746)	(760)
Fair value adjustment related to investments	124	90	34
Interest and other income, net	2,313	1,049	1,264
Total other expense	(5,120)	(5,919)	799
Loss before income taxes	(977)	(33,225)	32,248
Income tax benefit	433	—	433
Net loss	\$ (544)	\$ (33,225)	\$ 32,681

### *Net loss*

Net loss for the three months ended September 30, 2025, was \$0.5 million, compared to a net loss of \$33.2 million for the three months ended September 30, 2024, an increase to net loss of \$32.7 million. The increase was primarily attributable to an increase of \$22.4 million in revenue, a decrease of \$7.5 million in research and development expense, an increase of \$1.3 million in interest and other income, net, and a decrease of \$1.2 million in intangible asset amortization.

### *Revenue, net*

Revenue for the three months ended September 30, 2025, was \$26.1 million, compared to revenue of \$3.7 million for the three months ended September 30, 2024, an increase of approximately \$22.4 million. The increase was primarily due to an increase in product sales of MIPLYFFA of \$22.4 million.

### *Cost of product revenue*

Cost of product revenue for the three months ended September 30, 2025, decreased by \$1.1 million compared to the cost of product revenue for the three months ended September 30, 2024. This decrease was primarily due to the inventory write-down of \$2.0 million in the prior year with no comparable occurrence in the current year, partially offset by royalty costs related to product sales of MIPLYFFA.

### Intangible asset amortization

Intangible asset amortization for the three months ended September 30, 2025, decreased by \$1.2 million compared to the intangible asset amortization for the three months ended September 30, 2024, primarily due to definite-lived intangible assets acquired in the Merger 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 decreased by \$7.5 million, from \$10.9 million for the three months ended September 30, 2024, to \$3.4 million for the three months ended September 30, 2025. The decrease was primarily driven by a decrease in spending for the Phase 2 clinical study in KP1077 and a decrease in personnel-related costs.

### Selling, general and administrative

Selling, general and administrative expenses increased by \$0.7 million, from \$16.2 million for the three months ended September 30, 2024, to \$16.9 million for the three months ended September 30, 2025. The period-over-period increase was primarily related to an increase in personnel-related costs, professional fees, and other expenses as we continue to build our commercial organization.

### Other expense

Other expense for the three months ended September 30, 2025, was \$5.1 million compared to other expense of \$5.9 million for the three months ended September 30, 2024. The decrease was primarily attributable to an increase of \$1.3 million in interest and other income, net, partially offset by a decrease in the fair value adjustment related to warrant and CVR liability of \$0.8 million.

### Comparison of the nine months ended September 30, 2025, and 2024 (in thousands):

	Nine months ended September 30,		Period-to-
	2025	2024	Period Change
Revenue, net	\$ 72,345	\$ 11,569	\$ 60,776
Cost of product revenue (excluding \$3,546 and \$4,619 in intangible asset amortization for the nine months ended September 30, 2025, and 2024, respectively, shown separately below)	14,962	6,051	8,911
Intangible asset amortization	3,546	4,619	(1,073)
Impairment of intangible assets	58,710	—	58,710
Operating expenses:			
Research and development	10,123	33,743	(23,620)
Selling, general and administrative	57,262	38,743	18,519
Total operating expenses	67,385	72,486	(5,101)
Loss from operations	(72,258)	(71,587)	(671)
Other income:			
Gain on sale of PRV	148,325	—	148,325
Interest expense	(6,029)	(5,157)	(872)
Fair value adjustment related to warrant and CVR liability	(1,379)	4,660	(6,039)
Fair value adjustment related to investments	119	64	55
Interest and other income, net	5,234	2,248	2,986
Total other income	146,270	1,815	144,455
Income (loss) before income taxes	74,012	(69,772)	143,784
Income tax expense	(2,948)	—	(2,948)
Net income (loss)	\$ 71,064	\$ (69,772)	\$ 140,836

*Net income (loss)*

Net income for the nine months ended September 30, 2025, was \$71.1 million, compared to net loss of \$69.8 million for the nine months ended September 30, 2024, an increase in net income of approximately \$140.8 million. The increase was primarily attributable to the gain on sale of the PRV of \$148.3 million and an increase of \$60.8 million in revenue, partially offset by \$58.7 million in impairment of intangible assets and \$11.7 million in inventory obsolescence.

*Revenue, net*

Revenue for the nine months ended September 30, 2025, was \$72.3 million, compared to revenue of \$11.6 million for the nine months ended September 30, 2024, an increase of approximately \$60.8 million. The increase was primarily due to an increase in product sales of MIPLYFFA of \$61.0 million.

*Cost of product revenue*

Cost of product revenue for the nine months ended September 30, 2025, increased by approximately \$8.9 million compared to the cost of product revenue for the nine months ended September 30, 2024. The increase was primarily due to \$11.7 million in inventory obsolescence for the nine months ended September 30, 2025, compared to \$5.2 million in inventory obsolescence for the nine months ended September 30, 2024, as well as royalty costs related to product sales of MIPLYFFA.

*Intangible asset amortization*

Intangible asset amortization for the nine months ended September 30, 2025, decreased by \$1.1 million compared to the intangible asset amortization for the nine months ended September 30, 2024, primarily due to definite-lived intangible assets acquired in the Merger no longer being amortized as a result of the impairment.

*Impairment of intangible assets*

Impairment of intangible assets for the nine months ended September 30, 2025, was \$58.7 million and was composed of the impairment loss on the OLPRUVA definite-lived intangible asset. There was no comparable impairment in the prior year.

*Research and development*

Research and development expenses decreased by \$23.6 million, from \$33.7 million for the nine months ended September 30, 2024, to \$10.1 million for the nine months ended September 30, 2025. The decrease was primarily driven by a decrease in spending for the Phase 2 clinical study in KP1077 and a decrease in personnel-related costs.

*Selling, general and administrative*

Selling, general and administrative expenses increased by approximately \$18.5 million, from \$38.7 million for the nine months ended September 30, 2024, to \$57.3 million for the nine months ended September 30, 2025. The period-over-period increase was primarily related to an increase in personnel-related costs, professional fees, and other expenses as we continue to build our commercial organization.

*Other income*

Other income for the nine months ended September 30, 2025, was \$146.3 million compared to other income of \$1.8 million for the nine months ended September 30, 2024. The increase was primarily attributable to the gain on sale of the PRV of \$148.3 million and an increase in interest and other income, net of \$3.0 million, partially offset by a decrease in fair value adjustment related to warrant and CVR liability of \$6.0 million.

**Liquidity and Capital Resources**

***Sources of Liquidity***

Through September 30, 2025, 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 French AC, royalties or net sales milestone payments generated under the AZSTARYS License Agreement, our PRV sale consummated on April 1, 2025, and consulting agreements. As of September 30, 2025, we had cash, cash equivalents and investments of \$230.4 million.

On February 26, 2025, we entered into the PRV Transfer Agreement, pursuant to which we agreed to sell the PRV to the buyer, subject to customary closing conditions. Pursuant to the PRV Transfer Agreement, the buyer agreed to pay us \$150.0 million, payable in cash, upon the closing of the sale. On April 1, 2025, the asset sale was consummated, resulting in net proceeds of \$148.3 million.

We have had recurring negative net operating cash flows throughout our operating history, and we cannot guarantee or predict when we may begin to consistently generate positive net cash flows from operations, or if at all. We expect that our sources of revenue will be from product sales of MIPLYFFA and OLPRUVA, reimbursements received under the French AC, royalties or net sales milestone payments generated under the AZSTARYS License Agreement, and any other future arrangements related to one or more of our products or product candidates.

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 Statements on Form S-3***

In connection with the Merger, we and Nantahala Capital Management, LLC (“Nantahala”) concurrently entered into a registration rights agreement, pursuant to which we agreed to file a resale registration statement with respect to the resale of our common stock issuable to Nantahala. On February 5, 2024, we filed a registration statement on Form S-3 (File No. 333-276856) registering an aggregate of 2,269,721 shares of our common stock. On April 5, 2024, we filed an amendment to such registration statement, which was declared effective on April 8, 2024.

On June 4, 2024, we filed a registration statement on Form S-3 (File No. 333-279941) (the “June 2024 Registration Statement”) under which we 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. The June 2024 Registration Statement was declared effective on June 13, 2024.

### ***August 2024 Offering***

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

### ***Entry into 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 its 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, 2025, no shares have been issued or sold under the 2024 ATM Agreement.

## ***Merger***

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

In connection with the closing of the Merger on November 17, 2023, each share of common stock of Acer was converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value \$0.0001 per share, and (ii) one non-transferable CVR to be issued by Zevra, which will represent the right to receive one or more contingent payments up to an additional \$76.0 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer's OLPRUVA and celiprolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer's early-stage program ACER-2820 (emetine).

## ***Stockholders Agreement***

In connection with the Merger, a certain stockholder of Acer entered into, and Acer agreed to use its reasonable best efforts to cause certain other stockholders to enter into joinders to, a stockholders agreement with Zevra (the "Stockholders Agreement"). Pursuant to the Stockholders Agreement, the stockholders party thereto agreed to, or would agree to, among other things, vote all of their shares in Zevra that they own in favor of each nominee included in the Zevra board of director's slate of nominees for each election of directors and in favor of each matter approved by the Zevra board of directors and submitted to stockholders of Zevra for the approval of stockholders following the closing of the Merger and until the second anniversary of the Closing Date of the Merger (the "Trigger Date"). In addition, the stockholders party to the Stockholders Agreement will be subject to customary standstill provisions, subject to certain exceptions, until the Trigger Date.

## ***Term Loans***

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

Under the terms of the Credit Agreement, the Lenders provided a senior secured loan facility to us in the aggregate principal amount of \$100.0 million, which is divided into three tranches as follows: (i) \$60.0 million, which was funded in full on the Term Loans Closing Date; (ii) \$20.0 million, which 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 opt to draw down the amounts described in (ii) and (iii) above prior to their applicable expiration dates.

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

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

## Cash Flows

The following table summarizes our cash flows for the nine months ended September 30, 2025, and 2024 (in thousands):

	Nine months ended September 30,	
	2025	2024
Net cash used in operating activities	\$ (7,114)	\$ (53,415)
Net cash provided by (used in) investing activities	17,900	(16,690)
Net cash provided by financing activities	9,518	81,312
Effect of exchange rate changes on cash and cash equivalents	350	(217)
Net increase in cash and cash equivalents	\$ 20,654	\$ 10,990

## Operating Activities

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 French AC, 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.

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

## Investing Activities

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.

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

### ***Financing Activities***

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.

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

### ***Future Funding Requirements***

We believe our available cash and cash equivalents, together with our ability to generate operating cash flow and our access to short-term and long-term borrowings, 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 and cash equivalents in accounts with major U.S. and multi-national financial institutions, and our deposits at these institutions exceed insured limits. Market conditions can impact the viability of these institutions. In the event of a failure of any of the financial institutions where we maintain our cash and cash equivalents, there can be no assurance that we would be able to access uninsured funds in a timely manner or at all. Any inability to access or delay in accessing these funds could adversely affect our business and financial position.

Potential near-term sources of additional funding include:

- any product sales of MIPLYFFA;
- any product sales of OLPRUVA;
- any reimbursements received for arimoclomol under the French AC; and
- any royalties or net sales milestone payments generated under the AZSTARYS License Agreement.

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

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

To date, we have generated revenue from product sales of MIPLYFFA and OLPRUVA, reimbursements received under the French AC, royalties or net sales milestone payments generated under the AZSTARYS License Agreement, our PRV sale consummated on April 1, 2025 and consulting agreements. We expect that, for the foreseeable future, our sources of revenues will be from product sales of MIPLYFFA and OLPRUVA, reimbursements received under the French AC, royalties or net sales milestone payments generated under the AZSTARYS License Agreement, and any other future arrangements related to one or more of our products or product candidates. We cannot guarantee that our current commercialization strategies, or any strategy we adopt in the future, will be successful. For instance, we received milestone payments under the AZSTARYS License Agreement, but we cannot guarantee that we will earn any additional milestone or royalty payments under this agreement in the future. We also cannot guarantee that we will continue to receive reimbursements under the French AC or the extent of our success in commercializing MIPLYFFA or OLPRUVA. We also expect to continue to incur significant additional costs associated with operating as a public company.

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

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, 2024, filed with the SEC on March 12, 2025.

### **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, 2025. Based on the evaluation of our disclosure controls and procedures as of September 30, 2025, 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, 2025, that materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## PART II OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

From time to time, we may be involved in routine legal proceedings, as well as demands, claims and threatened litigation, which arise in the normal course of our business. In connection with the AZSTARYS License Agreement with Commave, a dispute has arisen with Commave concerning the interpretation of certain provisions under the AZSTARYS License Agreement.

On September 4, 2024, Commave filed a complaint against Zevra in the Court of Chancery of the State of Delaware (Case No. 2024-0920-LWW) alleging breach of contract and seeking injunctive relief, specific performance, declaratory relief, and damages regarding the parties' respective rights and obligations under the Agreement.

On February 12, 2025, our motion to dismiss was denied and the case is now in the discovery phase. On July 17, 2025, Zevra and Commave filed cross motions for partial summary judgment as to certain of Commave's claims, and the court held oral argument on the parties' cross motions on September 22, 2025. We strongly disagree with Commave's allegations and believe this lawsuit is without merit.

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

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

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

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

### ITEM 1A. RISK FACTORS

In addition to the other information set forth in this Quarterly Report on Form 10-Q, you should carefully consider all the risk factors and uncertainties described in Part I, Item 1A. "Risk Factors" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2024, filed with the SEC on March 12, 2025, before investing in our common stock. Other than as described below, there have been no material changes to the risk factors described in that report. If any such risks materialize, our business, financial condition and results of operations could be seriously harmed. This Quarterly Report on Form 10-Q also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements because of the risk factors in our Annual Report on Form 10-K and below, and the other factors described in this Quarterly Report on Form 10-Q.

***Significant political, trade, regulatory developments, and other circumstances beyond our control could delay, prevent or impair our development or commercialization efforts.***

Trade policies, geopolitical disputes and other international conflicts can result in tariffs, sanctions and other measures that restrict international trade, and can materially adversely affect our business, particularly if these measures affect regions where manufacturing and product development activities take place or raw materials are sourced. For example, the U.S. government has recently imposed tariffs on certain foreign goods, and some foreign governments have threatened or instituted retaliatory tariffs on certain U.S. goods and have indicated a willingness to impose additional tariffs on U.S. products, which could increase the cost of goods needed to commercialize our products and continue development of our product candidates. The extent and duration of any tariffs and the resulting impact on general economic conditions and on our business are uncertain and depend on various factors, such as negotiations between the United States and other countries, the response of such countries, and exemptions or exclusions that may be granted. Countries may also adopt other measures, such as controls on imports or exports of goods, technology or data, that could adversely impact supply chains. As these tensions continue to rise, more targeted approaches on certain products, industries or companies could significantly impact our development and commercialization efforts. Further, such actions by the U.S. could result in other retaliatory

actions by those countries which could impact our ability to profitably commercialize our products in those jurisdictions. As a result, our business, operations, and financial condition could be materially harmed.

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

We are highly dependent on the management, research and development, clinical, financial and business development expertise of our senior leadership team, as well as the other members of our scientific and clinical teams. Although we have employment agreements with each of our executive officers, these agreements do not obligate them to continue working for our company and they may terminate their employment with us at any time. Our future performance will depend, in part, on our ability to retain senior level executives who will provide continuity of leadership among the larger workforce. If we are unable to avoid excessive or disruptive turnover of our executive officers, that could be viewed negatively by our customers, employees, investors, and other third-party partners, and could have an adverse impact on our business and results of operations.

Our success depends in part on our ability to attract and retain the best available personnel with the necessary qualifications, which in a competitive global market means that our recruitment efforts should not be limited entirely to the U.S. To the extent U.S. immigration-related policies, laws, rules, proclamations or orders restrict our ability to attract, or increase the cost of attracting, international talent, this could adversely impact our business and results of operations. For example, the current U.S. administration's decision, announced September 19, 2025, that, subject to limited exceptions, petitions for H-1B visas should be approved only if they are accompanied by a payment of \$100,000, could hamper our recruitment efforts for any new personnel who require such a visa. Additional changes in U.S. immigration policies, rules, laws or orders could further increase our recruitment costs or delay key projects that require specialized expertise. In addition, restrictions or delays in the issuance of other visas or in immigration processing could adversely affect our ability to collaborate effectively with our employees outside the U.S. and to retain the qualified personnel needed for our operations.

Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our product candidate pipeline toward scaling up for commercialization, manufacturing and sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize our prodrug product candidates. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

***We recognized an impairment charge related to intangible assets. If our remaining assets become impaired in the future, we would incur additional impairment charges, which would negatively affect our operating results.***

We recognized impairment charges of \$58.7 million related to definite-lived intangible assets during the quarter ended June 30, 2025. If our remaining assets become impaired in the future, we would incur additional impairment charges, which would negatively affect our results of operations. There is significant judgment required in the analysis of a potential impairment of identified intangible assets and other long-lived assets. Impairment may result from, among other things, significant changes in the manner of use of the acquired assets, negative industry or economic trends and/or significant underperformance relative to historic or projected operating results. For additional information, see Note L of our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

***Current and future healthcare reform legislation or regulation may increase the difficulty and cost for us to obtain marketing approval of our product candidates and increase the cost to commercialize our approved products, and may also create similar difficulty for any of our product candidates that may be approved in the future, which may have a negative impact on our business and results of operations.***

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect the ability to profitably sell our approved products and our ability to profitably sell any of our product candidates for which we obtain marketing approval.

Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. For example, in 2010, the ACA was signed into law. The ACA substantially changed the way healthcare is financed by both governmental and private insurers, and significantly affects the U.S. pharmaceutical industry.

Among the provisions of the ACA of importance to our business, including our ability to commercialize and the prices we may obtain for our product candidates that are approved for sale are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for non-compliance;
- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;
- expansion of the list of entity types eligible for participation in the Public Health Service 340B drug pricing program (the "340B program") to include certain free-standing cancer hospitals, critical access hospitals, rural referral centers, and sole community hospitals, but exempting "orphan drugs," such as MIPLYFFA, from the 340B ceiling price requirements for these covered entities; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Since its enactment, there have been executive, judicial and congressional challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Thus, the ACA remains in effect in its current form.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, included aggregate reductions to Medicare payments to providers, which went into effect in April 2013, and, due to subsequent legislative amendments, will stay in effect through 2032. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. In addition, on March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminated the statutory Medicaid drug rebate cap, beginning January 1, 2024. The rebate was previously capped at 100% of a drug's average manufacturer price.

On August 16, 2022, the Inflation Reduction Act of 2022 (the "IRA") was signed into law. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023), and replaces the Part D coverage gap discount program with a new manufacturer discounting program (which began in 2025). The IRA permits the Secretary of the Department of Health and Human Services ("HHS") to implement many of these provisions through guidance, as opposed to regulation, for the initial years. CMS has published the negotiated prices for the initial ten drugs, which will first be effective in 2026, and has published the list of the subsequent 15 drugs that will be subject to negotiation, although the drug price negotiation program is currently subject to legal challenges. The impact of the IRA on us and the pharmaceutical industry cannot yet be fully determined, but is likely to be significant.

The One Big Beautiful Bill Act, which was enacted in July 2025, imposes significant reductions in the funding of the Medicaid program. Such reductions are expected to decrease the number of persons enrolled in Medicaid and reduce the services covered by Medicaid, which could adversely affect our sales of MIPLYFFA®, OLPRUVA® or any other product candidate that we may commercialize.

The Trump administration has also issued executive orders that address the pricing of pharmaceuticals in the U.S. and propose a so-called most favored nation pricing policy, which would tie the price of drugs in the U.S. to the lowest price in a group of other countries. While it is unclear whether and how these proposals will be implemented, the Trump policies are likely to have a negative impact on the pharmaceutical industry. Even proposals or executive actions that are ultimately deemed unlawful could negatively impact the U.S. pharmaceutical sector and our business, for example by causing uncertainty and delaying development and commercialization efforts.

Further, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drugs.

At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure, drug price increase reporting and other transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Some states have enacted legislation creating so-called prescription drug affordability boards, which ultimately may attempt to impose price limits on certain drugs in these states, while some states are also seeking to implement general, across the board price caps for pharmaceuticals, or are seeking to regulate drug distribution. These new laws may result in additional reductions in Medicare and other healthcare funding, which could negatively impact customers for our product candidates, if approved, and, accordingly, our financial operations.

In the EU, on December 13, 2021, Regulation No 2021/2282 on Health Technology Assessment (“HTA”) amending Directive 2011/24/EU was adopted. The Regulation entered into force in January 2022 and has been applicable since January 2025, with phased implementation based on the type of product, i.e. oncology and advanced therapy medicinal products as of 2025, orphan medicinal products as of 2028, and all other medicinal products by 2030. The Regulation intends to boost cooperation among EU member states in assessing health technologies, including new medicinal products, and provide the basis for cooperation at the EU level for joint clinical assessments in these areas. It will permit EU member states to use common HTA tools, methodologies, and procedures across the EU, working together in four main areas, including joint clinical assessment of the innovative health technologies with the highest potential impact for patients, joint scientific consultations whereby developers can seek advice from HTA authorities, identification of emerging health technologies to recognize promising technologies early, and continuing voluntary cooperation in other areas. Individual EU member states will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technology, and for making decisions on pricing and reimbursement.

We expect that the healthcare reform measures that have been adopted and may be adopted in the future may, among other things, result in more rigorous coverage criteria as well as additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain or maintain profitability, or commercialize our product candidates.

Legislative and regulatory proposals and enacted statutes have been made to expand post-approval requirements and restrict sales and promotional activities for drugs. For instance, the Drug Supply Chain Security Act imposes obligations on manufacturers of pharmaceutical products, among others, related to product tracking and tracing. Among the requirements of this new legislation, manufacturers are required to provide specified information regarding the drug products they produce to individuals and entities to which product ownership is transferred, label drug products with a product identifier and keep specified records regarding the drug products. The transfer of information to subsequent product owners by manufacturers will eventually be required to be done electronically. Manufacturers are also required to verify that purchasers of products are appropriately licensed. Further, under this legislation, manufacturers have drug product investigation, quarantine, disposition and FDA and trading-partner notification responsibilities related to counterfeit, diverted, stolen and intentionally adulterated products, as well as products that are the subject of fraudulent transactions or which are otherwise unfit for distribution, such that they would be reasonably likely to result in serious health consequences or death.

In the EU, the EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission’s proposal for revision of several legislative instruments related to medicinal products (potentially reducing the duration of regulatory data protection, revising the eligibility for expedited pathways, etc.) was published on April 26, 2023. The proposed revisions remain to be agreed and adopted by the European Parliament and European Council, and the proposals may therefore be substantially revised before adoption, which is not anticipated before early 2026. The revisions may, however, have a significant impact on the pharmaceutical industry and our business in the long term.

We cannot be sure whether additional legislative changes will be enacted, or whether the FDA’s or foreign regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA’s approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

***If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in which we participate, we could be subject to additional reimbursement requirements, penalties, sanctions and fines, which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.***

Medicaid is a joint federal and state program administered by the states for low income and disabled beneficiaries. We participate in and have certain price reporting obligations under the Medicaid Drug Rebate Program (the “MDRP”) as a condition of having covered outpatient drugs payable under Medicaid and, if applicable, under Medicare Part B. The MDRP requires us to pay a rebate to state Medicaid programs every quarter for each unit of our covered outpatient drugs dispensed to Medicaid beneficiaries and paid for by a state Medicaid program. The rebate is based on pricing data that we must report on a monthly and quarterly basis to the Centers for Medicare & Medicaid Services (“CMS”), the federal agency that administers the MDRP and other governmental healthcare programs. These data include the average manufacturer price (“AMP”) for each drug and, in the case of innovator products, the best price, which in general represents the lowest price available from the manufacturer to certain entities in the U.S. in any pricing structure, calculated to include all sales and associated rebates, discounts and other price concessions. The Medicaid rebate consists of two components, the basic rebate and the additional rebate, which is triggered if the AMP for a drug increases faster than inflation. If we become aware that our MDRP government price reporting submission for a prior quarter was incorrect or has changed as a result of recalculation of the pricing data, we must resubmit the corrected data for up to three years after those data originally were due. If we fail to provide information timely or are found to have knowingly submitted false information to the government, we may be subject to civil monetary penalties and other sanctions, including termination from the MDRP. In the event that CMS terminates our rebate agreement pursuant to which we participate in the MDRP, no federal payments would be available under Medicaid or Medicare Part B for our covered outpatient drugs. Our failure to comply with our MDRP price reporting and rebate payment obligations could negatively impact our financial results.

The IRA imposes rebates under Medicare Part B and Medicare Part D that are triggered by price increases that outpace inflation (starting in 2023), as described under the risk factor *“Current and future healthcare reform legislation or regulation may increase the difficulty and cost for us to obtain marketing approval of our product candidates and increase the cost to commercialize our approved products, and may also create similar difficulty for any of our product candidates that may be approved in the future, which may have a negative impact on our business and results of operations,”* above. The Medicare Part D rebate will be calculated on the basis of the AMP figures we report pursuant to the MDRP.

Federal law requires that any company that participates in the MDRP also participate in the Public Health Service’s 340B drug pricing program in order for federal funds to be available for the manufacturer’s drugs under Medicaid and, if applicable, Medicare Part B. We participate in the 340B program, which is administered by the Health Resources and Services Administration (“HRSA”), and requires us to charge statutorily defined covered entities no more than the 340B “ceiling price” for our covered outpatient drugs. These 340B covered entities include a variety of community health clinics and other entities that receive health services grants from the Public Health Service, as well as hospitals that serve a disproportionate share of low-income patients. The ACA expanded the list of covered entities to include certain free-standing cancer hospitals, critical access hospitals, rural referral centers and sole community hospitals, but exempts “orphan drugs,” such as OLPRUVA, from the ceiling price requirements for these covered entities. The 340B ceiling price is calculated using a statutory formula based on the AMP and rebate amount for the covered outpatient drug as calculated under the MDRP, and, in general, products subject to Medicaid price reporting and rebate liability are also subject to the 340B ceiling price calculation and discount requirement. We must report 340B ceiling prices to HRSA on a quarterly basis, and HRSA publishes those prices to 340B covered entities. In addition, HRSA has finalized regulations regarding the calculation of the 340B ceiling price and the imposition of civil monetary penalties on manufacturers that knowingly and intentionally overcharge covered entities for 340B-eligible drugs. HRSA has also finalized a revised regulation implementing an administrative dispute resolution process through which 340B covered entities may pursue claims against participating manufacturers for overcharges, and through which manufacturers may pursue claims against 340B covered entities for engaging in unlawful diversion or duplicate discounting of 340B drugs. Our failure to comply with 340B program requirements could negatively impact our financial results. Any additional future changes to the definition of average manufacturer or and the Medicaid rebate amount under legislation or regulation could affect our 340B ceiling price calculations and also negatively impact our financial results.

In order for OLPRUVA or any product candidates, if approved, to be paid for with federal funds under the Medicaid and Medicare Part B programs and purchased by certain federal agencies and grantees, we also participate in the U.S. Department of Veterans Affairs (“VA”) Federal Supply Schedule (“FSS”) pricing program. As part of this program, we are required to make our products available for procurement on an FSS contract under which we must comply with standard government terms and conditions and charge a price that is no higher than the statutory Federal Ceiling Price (“FCP”), to four federal agencies (VA, U.S. Department of Defense (“DOD”), Public Health Service, and U.S. Coast Guard). The FCP is based on the Non-Federal Average Manufacturer Price (“Non-FAMP”), which we must calculate and report to the VA on a quarterly and annual basis. Pursuant to applicable law, knowing provision of false information in connection with a Non-FAMP filing can subject a manufacturer to significant civil monetary penalties for each item of false information. The FSS pricing and contracting obligations also contain extensive disclosure and certification requirements.

We also participate in the Tricare Retail Pharmacy program, under which we are required to pay quarterly rebates on utilization of innovator products that are dispensed through the Tricare Retail Pharmacy network to Tricare beneficiaries. The rebates are calculated as the difference between the annual Non-FAMP and FCP. We are required to list our innovator products on a Tricare Agreement in order for them to be eligible for DOD formulary inclusion. If we overcharge the government in connection with our FSS contract or Tricare Agreement, whether due to a misstated FCP or otherwise, we are required to refund the difference to the government. Failure to make necessary disclosures and/or to identify contract overcharges could result in allegations against us under the False Claims Act and other laws and regulations. Unexpected refunds to the government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

Individual states continue to consider and have enacted legislation to limit the growth of healthcare costs, including the cost of prescription drugs and combination products. A number of states have either implemented or are considering implementation of drug price transparency legislation. Requirements of pharmaceutical manufacturers under such laws include advance notice of planned price increases, reporting price increase amounts and factors considered in taking such increases, wholesale acquisition cost information disclosure to prescribers, purchasers and state agencies, and new product notice and reporting. Such legislation could limit the price or payment for certain drugs, and a number of states are authorized to impose civil monetary penalties or pursue other enforcement mechanisms against manufacturers who fail to comply with drug price transparency requirements, including the untimely, inaccurate or incomplete reporting of drug pricing information.

Pricing and rebate calculations vary among products and programs. The calculations are complex and are often subject to interpretation by us, governmental or regulatory agencies, and the courts. CMS, the Department of Health & Human Services Office of Inspector General, and other governmental agencies have pursued manufacturers that were alleged to have failed to report these data to the government in a timely or accurate manner. Governmental agencies may also make changes in program interpretations, requirements or conditions of participation, some of which may have implications for amounts previously estimated or paid. We cannot assure you that any submissions we are required to make under the MDRP, the 340B program, the VA/FSS program, the Tricare Retail Pharmacy Program, and other governmental drug pricing programs will not be found to be incomplete or incorrect.

## **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, 2025, no director or officer of the Company adopted or terminated a “Rule 10b5-1 trading arrangement” or “non-Rule 10b5-1 trading arrangement,” as each term is defined in Item 408(a) of Regulation S-K.

**ITEM 6. EXHIBITS**

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

<b>Exhibit No.</b>	<b>Description</b>
3.1	<a href="#">Amended and Restated Certificate of Incorporation of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on April 21, 2015).</a>
3.1.1	<a href="#">Certificate of Amendment of Amended and Restated Certificate of Incorporation of the Registrant, effective as of December 23, 2020 (incorporated herein by reference to Registrant's Current Report on Form 8-K as filed with the SEC on December 28, 2020).</a>
3.1.2	<a href="#">Certificate of Amendment of Amended and Restated Certificate of Incorporation of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 24, 2023).</a>
3.2	<a href="#">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).</a>
4.1	<a href="#">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).</a>
10.1*	<a href="#">Fourth Amendment to the Lease Agreement, by and between the Registrant and TMT-CELEBRATION OFFICE CENTER I &amp; II LLC, dated as of September 4, 2025.</a>
31.1*	<a href="#">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.</a>
31.2*	<a href="#">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.</a>
32.1**	<a href="#">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.</a>
32.2**	<a href="#">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.</a>
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 5, 2025

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

Date: November 5, 2025

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

## FOURTH AMENDMENT TO OFFICE LEASE AGREEMENT

**THIS FOURTH AMENDMENT TO OFFICE LEASE AGREEMENT** (this “**Amendment**”) is made and entered into as of the latest date set forth beneath the parties’ signatures below (the “**Amendment Date**”) by and between **TMT-CELEBRATION OFFICE CENTER I & II LLC., Florida Limited Liability Company** (“**Landlord**”) and **Zevra Therapeutics, Inc., a Foreign Profit Corporation** (“**Tenant**”).

## WITNESSETH:

Landlord (as successor to Celebration Office Investors, LLC., a Florida limited liability company) and Tenant are parties to a certain lease dated November 3, 2014 and that certain First Amendment dated April 21, 2015, and that certain Second Amendment dating January 8<sup>th</sup>, 2016, and that certain Third Amendment dated July 28<sup>th</sup>, 2016 (collectively the “**Lease**”);

The Amendment provides for an amendment to the Lease of Suites 103, 104 and 108 containing approximately 17,074 rentable square feet (the “**Premises**”) located in 1180 Celebration Boulevard, Celebration, FL 34747 (the “**Building**”).

Landlord and Tenant wish to enter into this Amendment to modify and extend the lease term and agree upon such other matters as set forth below.

**NOW, THEREFORE**, for and in consideration of the agreements set forth herein, the receipt and sufficiency of which consideration is hereby acknowledged, Landlord and Tenant amend the Lease as follows:

1. **Incorporation of Recitals.** The above recitals are true and correct and are incorporated herein as if set forth in full.
2. **General Provisions.** All defined terms in this Amendment shall have the same meaning as in the Lease, except if otherwise noted herein. As amended by this Amendment, all of the terms, covenants, conditions, and agreements of the Lease shall remain in full force and effect. In the event of any conflict between the provisions of the Lease and the provisions of this Amendment, this Amendment shall control.
3. **Extension of Lease Term.** The Lease Term solely for Suite 108, containing approximately 6,302 rentable square feet, is hereby extended for an additional six (6) months commencing on March 1, 2026, and expiring at 11:59 pm/E.T. on August 31, 2026 (the “**Expiration Date**”). The Lease Term for Suites 103 and 104 shall expire on August 31, 2025 at 11:59pm/E.T. and shall be hereby removed from the definition of “**Premises**”.
4. **Premises & Relocation.** Tenant shall continue occupying Suites 103 and 104 from this Amendment Date until August 31, 2025. On or before September 1, 2025 or as otherwise agreed in writing by and between Landlord and Tenant, Tenant shall vacate Suites 103 and 104 and relocate to Suite 108, containing approx. 6,302 rentable square feet, for the remainder of the Lease Term.
5. **Tenant’s Acceptance.** Tenant accepts the Premises, the Building, and the Common Areas in their “as is” “where is” “with all faults” condition, and Landlord shall not be required to make any improvements to the Premises, the Building, or the Common Areas or provide or pay any improvement allowances or payments to Tenant under the Lease for Tenant’s occupancy of the Premises, and Tenant acknowledges that all construction obligations and allowances and other amounts required to be paid from Landlord to Tenant under the Lease have been performed and paid in full.
6. **Fixed Annual Rent.** From the Third Amendment Date until the Expiration Date, Tenant shall continue paying Fixed Annual Rent, plus applicable taxes thereon, to Landlord in accordance with the Third Amendment. Tenant shall pay Fixed Annual Rent to Landlord pursuant to the rent chart set forth below, plus applicable taxes, as and when due under this Amendment.

PERIOD	FIXED ANNUAL RENT MONTHLY PAYMENT
September 1, 2025 – February 28, 2026	\$15,185.56
March 1, 2026 – August 31, 2026	\$15,655.22

7. Additional Rent. At all times, Tenant shall continue paying to Landlord Tenant's Proportionate Share of Operating Costs pursuant to their Original Lease Agreement or any amendment that may have modified that agreement, all Additional Rent, and any other sums due under the Lease, as and when due under the Lease.
8. Brokers. Landlord and Tenant represent and warrant that they have neither consulted nor negotiated with any broker or finder as to this Amendment, except **Streamline Realty Partners Florida, Inc.** ("Landlord's Broker") and **Cushman & Wakefield** ("Tenant's Broker"). Landlord's Broker shall be paid any commissions pursuant to the terms of separate written commission agreement between Landlord and Landlord's Broker. Landlord and Tenant shall indemnify, defend, and save the other harmless from and against any claims for fees or commissions from anyone other than the Landlord's Broker with whom they have dealt concerning the Premises or this Amendment including attorneys' fees incurred in the defense of any such claim.
9. Counterparts. This Amendment may be executed in counterparts with the same effect as if both parties hereto had executed the same document. Both counterparts shall be construed together and shall constitute a single Amendment. The parties hereto consent and agree that this Amendment may be signed and/or transmitted by facsimile, e-mail of a .pdf document or using electronic signature technology (e.g., via DocuSign or similar electronic signature technology), and that such signed electronic record shall be valid and as effective to bind the party so signing as a paper copy bearing such party's handwritten signature. The parties further consent and agree that (i) to the extent a party signs this Amendment using electronic signature technology, by clicking "SIGN", such party is signing this Amendment electronically, and (ii) the electronic signatures appearing on this Amendment shall be treated, for purposes of validity, enforceability and admissibility, the same as handwritten signatures.
10. Miscellaneous. In the event of any conflict between the provisions of the Lease and the provisions of this Amendment, this Amendment shall control. Tenant agrees that it and its partners, officers, directors, employees, agents, consultants, accountants, real estate brokers and sales persons and attorneys shall not disclose the terms of the Lease, this Amendment or any future amendments, without Landlord's prior written consent, except to any bankers or attorneys or other professional advisors of Tenant, any accountants of Tenant in connection with the preparation of Tenant's financial statements or tax returns, or to an entity or person to whom disclosure is required by applicable law or in connection with any action brought to enforce the Lease. This Amendment shall be binding upon and inure to the benefit of the parties to this Amendment, their legal representatives, successors, and permitted assigns. Tenant hereby acknowledges and affirms that, as of the date hereof, the Lease is in full force and effect and, to the best of Tenant's knowledge, there are no claims, offsets, or breaches of the Lease, or any action or causes of action by Tenant against Landlord directly or indirectly relating to the Lease. The parties agree that the normal rule of construction to the effect that any ambiguities are to be resolved against the drafting party shall not be employed in the interpretation of this Amendment. The parties agree that the Lease is in full force and effect and the parties ratify and confirm the terms of the Lease as modified by this Amendment and the Lease is incorporated herein by reference as if fully set forth herein. The Lease, as amended, contains the entire agreement between Landlord and Tenant as to the Premises, and there are no other agreements, oral or written, between Landlord and Tenant relating to the Premises. All prior understandings and agreements between the parties concerning these matters are merged into this Amendment, which alone fully and completely expresses their understanding.

**IN WITNESS WHEREOF**, Landlord and Tenant have duly executed this Amendment.

**LANDLORD:**

**TMT-CELEBRATION OFFICE CENTER I & II LLC.**

By: /s/ Joseph Maas

Name: Joseph Maas

Title: Chief Operating Officer

Date: September 4, 2025

**TENANT:**

**Zevra Therapeutics, Inc.**

By: /s/ R. LaDuane Clifton

Name: R. LaDuane Clifton

Title: Chief Financial Officer

Date: September 4, 2025

## 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 5, 2025

/s/ Neil F. McFarlane

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Name: Neil F. McFarlane  
Title: President and Chief Executive Officer  
(Principal Executive Officer)

## CERTIFICATION

I, R. LaDuane Clifton, certify that:

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

November 5, 2025

/s/ R. LaDuane Clifton

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Name: R. LaDuane Clifton, MBA, CPA  
Title: Chief Financial Officer and Treasurer  
(Principal Financial Officer)

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

In connection with the Quarterly Report on Form 10-Q of Zevra Therapeutics, Inc., (the "Company") for the quarterly period ended September 30, 2025, 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 5, 2025

/s/ Neil F. McFarlane

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Name: Neil F. McFarlane  
Title: President and Chief Executive Officer  
(Principal Executive Officer)

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

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

In connection with the Quarterly Report on Form 10-Q of Zevra Therapeutics, Inc., (the "Company") for the quarterly period ended September 30, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, R. LaDuane Clifton, Principal Financial Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

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

November 5, 2025

/s/ R. LaDuane Clifton

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Name: R. LaDuane Clifton, MBA, CPA  
Title: Chief Financial Officer and Treasurer  
(Principal Financial Officer)

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