



## Zevra Reports Third Quarter 2025 Financial Results and Corporate Update

November 5, 2025

2025 EPS of \$(0.01)

Q3 2025 net revenue of \$26.1 million, driven by MIPLYFFA® net revenue of \$22.4 million

Company to host conference call and webcast TODAY, November 5, 2025, at 4:30 p.m. ET

CELEBRATION, Fla., Nov. 05, 2025 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage company focused on providing therapies for people living with rare disease, today reported its financial results for the three and nine months ended September 30, 2025.

"Zevra is well-positioned for continued growth, driven by the strong performance of MIPLYFFA and the meaningful impact we are delivering to patients with Niemann-Pick disease type C," said Neil F. McFarlane, Zevra's President and Chief Executive Officer. "We have generated strong revenue and a solid operating runway that enables us to execute on our strategic priorities and invest in building Zevra into a leading rare disease company."

### Commercial Highlights

- MIPLYFFA: Eight prescription enrollment forms during Q3 2025, bringing the total since product launch to 137. Market access has reached 66% of covered lives, which is in line with the Company's expectations one year into the launch.
- OLPRUVA: One prescription enrollment form during Q3 2025, bringing the total since product launch to 30. Market access has reached 81% of covered lives. As a data-driven organization, the Company has decided to scale back its sales and marketing efforts for OLPRUVA. While options for OLPRUVA are evaluated, Zevra will maintain product availability and support services for patients.

### Pipeline and Innovation Highlights

- Arimoclomol: A Marketing Authorisation Application for the evaluation of arimoclomol for the treatment of Niemann-Pick disease type C (NPC) has been validated and is under review by the European Medicines Agency (EMA). It has been designated an Orphan Medicinal Product by the EMA. Arimoclomol is currently marketed in the U.S. under the brand name, MIPLYFFA.
- Celiprolol: Enrolled five patients in the event-driven Phase 3 DiSCOVER trial for the treatment of Vascular Ehlers-Danlos Syndrome during Q3 2025, bringing the total number of enrolled patients to 44, and there has been one confirmed event.

### Publication Highlights

- Open-label extension data showing sustained long-term efficacy of MIPLYFFA for the treatment of NPC was published in the peer-reviewed journal of *Molecular Genetics and Metabolism*.
- MIPLYFFA data featured at the following conferences: National Niemann-Pick Disease Foundation (NNPDF) Conference, Southeastern Regional Genetics Group (SERGG), International Congress of Inborn Errors of Metabolism (ICIE), International Niemann-Pick Disease Alliance (INPDA) Meeting, and Child Neurology Society (CNS) Conference.
  - New data from a pre-specified analysis showed that patients on concomitant miglustat treatment who were on placebo in the Phase 2/3 pivotal, double-blind study and switched to MIPLYFFA in the open-label extension phase experienced a decline in annual disease progression.
  - New data from a multi-center pediatric substudy in patients younger than two years old showed that MIPLYFFA was well tolerated in this age group with no new safety signals observed.
  - Nomination for Best Poster Award received for poster highlighting MIPLYFFA's differentiated mechanism of action targeting the underlying pathology of NPC.

### Q3 2025 Financial Highlights

- **Revenue, Net:** \$26.1 million for Q3 2025, which includes \$22.4 million of MIPLYFFA net revenue, \$0.1 million of OLPRUVA net revenue, \$2.4 million in net reimbursements from the French EAP for arimoclomol, and \$1.2 million in royalties and other reimbursements under the AZSTARYS® license agreement. For Q3 2024, total net revenue was \$3.7 million. MIPLYFFA net revenue in Q3 was impacted by the redesign of Medicare Part D rebates, resulting in a gross-to-net adjustment of \$1.2 million.
- **Cost of Product Revenue:** \$1.2 million for Q3 2025, excluding non-cash intangible asset amortization. Cost of product revenue for Q3 2024 was \$2.3 million.

- **Operating Expenses:** \$20.4 million for Q3 2025, which includes non-cash stock compensation expense of \$2.8 million. Total operating expenses for Q3 2024 were \$27.2 million.
  - R&D expense was \$3.4 million for Q3 2025, which was a decrease of \$7.5 million compared to \$10.9 million for Q3 2024 due primarily to a decrease in third-party costs upon completion of the KP1077 Phase 2 trial, combined with a decrease in personnel-related costs.
  - SG&A expense was \$16.9 million for Q3 2025, which was an increase of \$0.7 million compared to \$16.2 million for Q3 2024. Period-over-period increase was primarily related to an increase in personnel-related costs, professional fees, and other expenses associated with our commercial, medical and launch activities.
- **Net Loss:** \$(0.5) million, or \$(0.01) per basic and diluted share for Q3 2025, compared to a net loss of \$(33.2) million, or \$(0.69) per basic and diluted share, in Q3 2024. Q3 2025 net loss includes non-cash warrant fair value adjustment expense of \$5.5 million, non-cash stock-based compensation expense of \$2.8 million, and non-cash intangible asset amortization expense of \$0.3 million.
- **Cash Position:** Cash, cash equivalents and securities were \$230.4 million as of September 30, 2025, which included \$6.0 million in cash received in exchange for 1,046,890 warrants exercised at a weighted average exercise price of \$5.69 per share, and \$0.6 million in cash received in exchange for 86,628 options exercised at a weighted average exercise price of \$6.62, during the three months ended September 30, 2025. Based on its current operating forecast, the Company believes available financial resources are sufficient to execute on its strategic priorities independent from the capital markets.
- **Common and Fully Diluted Shares O/S:** As of September 30, 2025, total shares of common stock outstanding were 56,217,722, and fully diluted common shares were 67,882,914, which included 7,228,555 issuable from outstanding awards under equity incentive plans, and 4,436,637 shares issuable upon exercise of warrants.

## Inducement Awards

The Company granted options to purchase an aggregate of 93,000 shares of the Company's common stock (the "Inducement Awards") to 8 new employees pursuant to the Company's 2023 Employment Inducement Award Plan (as amended and/or restated, the "Inducement Award Plan"). Each Inducement Award vests over four years, with 25% vesting on the first anniversary of the employee's start date, and the remainder vesting in three equal annual installments thereafter (subject to each such employee's continued employment on each vesting date).

Each Inducement Award was approved by the Compensation Committee of the Board of Directors and granted as an inducement material to the individual entering into employment with Zevra, in accordance with Nasdaq Listing Rule 5635(c)(4). The Inducement Award Plan is used exclusively for the grant of equity awards to individuals who were not previously employees of Zevra, or following a bona fide period of non-employment, as an inducement material to such individuals entering into employment with Zevra.

## Conference Call Information

Zevra will host a conference call and audio webcast TODAY at 4:30 p.m. ET to discuss its corporate update and financial results for the third quarter 2025.

A link to the audio webcast is accessible on the "Events & Presentations" page in the Investor Relations section of the Zevra's website at [investors.zevra.com](https://investors.zevra.com). A replay of the webcast will be available for 90 days beginning at approximately 5:30 p.m. ET on November 5, 2025.

Additionally, interested participants and investors may access the conference call by dialing either:

- (800) 579-2543 (United States)
- +1 (785) 424-1789 (International)
- Conference ID: ZVRAQ325

## About MIPLYFFA® (arimoclomol)

MIPLYFFA (arimoclomol) is Zevra's approved therapy for the treatment of Niemann-Pick disease type C (NPC). Approved by the U.S. Food and Drug Administration on Sep. 20, 2024, MIPLYFFA (arimoclomol) increases the activation of the transcription factors EB (TFEB) and E3 (TFE3) resulting in the upregulation of coordinated lysosomal expression and regulation (CLEAR) genes. MIPLYFFA has also been shown to reduce unesterified cholesterol in the lysosomes of human NPC fibroblasts. The clinical significance of these findings is not fully understood. In the pivotal phase 3 trial, MIPLYFFA halted disease progression compared to placebo over the one-year duration of the trial when measured by the only validated disease progression measurement tool, the NPC Clinical Severity Scale. MIPLYFFA has also received Orphan Medicinal Product designation by the European Medicines Agency (EMA) for the treatment of NPC. The extensive data generated for MIPLYFFA has shown long-term, meaningful clinical outcomes with 5 and in some patients 7 years of patient experience across more than 270 NPC patients worldwide through a Phase 2/3 clinical trial, Open-Label Extension (OLE) study, Expanded Access Programs (EAP), and a pediatric sub-study, which is the most expansive clinical development program in NPC to date. Zevra has submitted a Marketing Authorization Application to the European Medicines Agency for the evaluation of arimoclomol for the treatment of Niemann-Pick disease type C.

## INDICATIONS AND USAGE

MIPLYFFA is indicated 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.

## **IMPORTANT SAFETY INFORMATION**

### **Hypersensitivity Reactions:**

Hypersensitivity reactions such as urticaria and angioedema have been reported in patients treated with MIPLYFFA during Trial 1: two patients reported both urticaria and angioedema (6%) and one patient (3%) experienced urticaria alone within the first two months of treatment. Discontinue MIPLYFFA in patients who develop severe hypersensitivity reactions. If a mild or moderate hypersensitivity reaction occurs, stop MIPLYFFA and treat promptly. Monitor the patient until signs and symptoms resolve.

### **Embryofetal Toxicity:**

MIPLYFFA may cause embryofetal harm when administered during pregnancy based on findings from animal reproduction studies. Advise pregnant females of the potential risk to the fetus and consider pregnancy planning and prevention for females of reproductive potential.

### **Increased Creatinine without Affecting Glomerular Function:**

Across clinical trials of MIPLYFFA, mean increases in serum creatinine of 10% to 20% compared to baseline were reported. These increases occurred mostly in the first month of MIPLYFFA treatment and were not associated with changes in glomerular function.

During MIPLYFFA treatment, use alternative measures that are not based on creatinine to assess renal function. Increases in creatinine reversed upon MIPLYFFA discontinuation.

**The most common adverse reactions** in Trial 1 ( $\geq 15\%$ ) in MIPLYFFA-treated patients who also received miglustat were upper respiratory tract infection, diarrhea, and decreased weight.

Three (6%) of the MIPLYFFA-treated patients had the following adverse reactions that led to withdrawal from Trial 1: increased serum creatinine (one patient), and progressive urticaria and angioedema (two patients). Serious adverse reactions reported in MIPLYFFA-treated patients were hypersensitivity reactions including urticaria and angioedema.

**To report SUSPECTED ADVERSE REACTIONS, contact Zevra Therapeutics, Inc. at toll-free phone 1-844-600-2237 or FDA at 1 800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).**

### **Drug Interaction(s):**

Arimoclomol is an inhibitor of the organic cationic transporter 2 (OCT2) transporter and may increase the exposure of drugs that are OCT2 substrates. When MIPLYFFA is used concomitantly with OCT2 substrates, monitor for adverse reactions and reduce the dosage of the OCT2 substrate.

### **Use in Females and Males of Reproductive Potential:**

Based on animal findings, MIPLYFFA may impair fertility and may increase post-implantation loss and reduce maternal, placental, and fetal weights.

### **Renal Impairment:**

The recommended dosage of MIPLYFFA, in combination with miglustat, in patients with an eGFR  $\geq 15$  mL/minute to  $< 50$  mL/minute is lower than the recommended dosage (less frequent dosing) in patients with normal renal function.

MIPLYFFA capsules for oral use are available in the following strengths: 47 mg, 62 mg, 93 mg, and 124 mg.

### **About OLPRUVA®**

OLPRUVA (sodium phenylbutyrate) is Zevra's approved treatment for the treatment of certain UCIDs. OLPRUVA (sodium phenylbutyrate) for oral suspension is a prescription medicine used along with certain therapies, including changes in diet, for the long-term management of adults and children weighing 44 pounds (20 kg) or greater and with a body surface area (BSA) of 1.2 m<sup>2</sup> or greater, with UCIDs, involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS). OLPRUVA is not used to treat rapid increase of ammonia in the blood (acute hyperammonemia), which can be life-threatening and requires emergency medical treatment. For more information, please visit [www.OLPRUVA.com](http://www.OLPRUVA.com).

### **Important Safety Information**

Certain medicines may increase the level of ammonia in your blood or cause serious side effects when taken during treatment with OLPRUVA. Tell your doctor about all the medicines **you or your** child take, especially if you or your child take corticosteroids, valproic acid, haloperidol, and/or probenecid.

OLPRUVA can cause serious side effects, including: 1) nervous system problems (neurotoxicity). Symptoms include sleepiness, tiredness, lightheadedness, vomiting, nausea, headache, confusion, 2) low potassium levels in your blood (hypokalemia) and 3)

conditions related to swelling (edema). OLPRUVA contains salt (sodium), which can cause swelling from salt and water retention. Tell your doctor right away if you or your child get any of these symptoms. Your doctor may do certain blood tests to check for side effects during treatment with OLPRUVA. If you have certain medical conditions such as heart, liver or kidney problems, are pregnant/planning to get pregnant or breast-feeding, your doctor will decide if OLPRUVA is right for you.

The most common side effects of OLPRUVA include absent or irregular menstrual periods, decreased appetite, body odor, bad taste or avoiding foods you ate prior to getting sick (taste aversion). These are not all of the possible side effects of OLPRUVA. Call your doctor for medical advice about side effects. You may report side effects to U.S. FDA at 1-800-FDA-1088.

### **About Celiprolol**

Celiprolol is Zevra's investigational clinical candidate for the treatment of Vascular Ehlers-Danlos Syndrome (VEDS). Celiprolol has been granted Orphan Drug and Breakthrough Therapy designations by the U.S. FDA. Zevra recently restarted enrollment in the DiSCOVER trial, a Phase 3 trial being conducted under a Special Protocol Assessment (SPA) agreement with the U.S. FDA. Celiprolol's mechanism of action is designed to reduce the mechanical stress on collagen fibers within the arterial wall through vascular dilation and smooth muscle relaxation.

### **About Zevra Therapeutics, Inc.**

Zevra Therapeutics, Inc. is a commercial-stage company focused on addressing unmet needs for the treatment of rare diseases. Our mission is to bring life-changing therapeutics to people living with rare diseases. With unique, data-driven development and commercialization strategies, the Company is overcoming complex drug development challenges to make new therapies available to the rare disease community.

Expanded access programs are made available by Zevra Therapeutics, Inc. and its affiliates and are subject to the Company's Expanded Access Program (EAP) policy, as published on its website. Participation in these programs is subject to the laws and regulations of each jurisdiction under which each respective program is operated. Eligibility for participation in any such program is at the treating physician's discretion.

For more information, please visit [www.zevra.com](http://www.zevra.com) or follow us on X and LinkedIn.

### **Cautionary Note Concerning Forward-Looking Statements**

This press release may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements that do not relate solely to historical or current facts, including without limitation statements regarding our growth; the U.S. launch and potential global expansion of MIPLYFFA®; submissions to, review by, and discussions with the EMA regarding arimoclomol; promise and potential impact of our preclinical or clinical trial data; the initiation, timing and results of any clinical trials or readouts; the potential benefits of any of our products or product candidates for any specific disease or at any dosage; future research and development activities; our strategic and product development objectives, including with respect to becoming a leading, commercially focused rare disease company; our financial position, including our cash balance and anticipated cash runway; and the timing of any of the foregoing. Forward-looking statements are based on information currently available to Zevra and its current plans or expectations. They are subject to several known and unknown uncertainties, risks, and other important factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by the forward-looking statements. These and other important factors are described in detail in the "Risk Factors" section of Zevra's Annual Report on Form 10-K for the year ended December 31, 2024, filed on March 12, 2025, Quarterly Report on Form 10-Q for the three and nine months ended September 30, 2025, to be filed with the SEC, as well as and Zevra's other filings with the Securities and Exchange Commission. While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we cannot assure that such expectations will prove correct. These forward-looking statements should not be relied upon as representing our views as of any date after the date of this press release.

### **Zevra Contact**

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**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

**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		
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