



Zevra Reports Second Quarter 2025 Financial Results and Corporate Update

August 12, 2025

Q2 2025 net revenue of \$25.9 million, driven by product net revenue of \$21.8 million

Completed sale of PRV for \$150.0 million, bolstering the balance sheet as the Company executes on its commercial launches and development programs

Submitted a Marketing Authorisation Application to the European Medicines Agency for the evaluation of arimoclomol for the treatment of Niemann-Pick Disease Type C

Company to host conference call and webcast TODAY, August 12, 2025, at 4:30 p.m. ET

CELEBRATION, Fla., Aug. 12, 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 six months ended June 30, 2025.

"This is an exciting time of growth for Zevra," said Neil F. McFarlane, Zevra's President and Chief Executive Officer. "Following approval late last year, we are focused on executing the U.S. launch of MIPLYFFA®, and we believe the remarkable performance in the second quarter sets a strong foundation for continued momentum. As the only treatment that has been shown to halt disease progression by addressing its underlying pathology, we are invigorated by the meaningful impact MIPLYFFA is bringing to people diagnosed with Niemann-Pick disease type C (NPC). Now, with the recent submission of the Marketing Authorisation Application (MAA) in the E.U., we seek to expand these differentiated benefits to greater numbers of patients with this debilitating disease."

Commercial Highlights

- MIPLYFFA: 7 prescription enrollment forms during Q2 2025, bringing the total since product launch to 129. Market access has reached 52% of covered lives, which is in line with expectations at this stage of the launch.
- OLPRUVA: 1 prescription enrollment form during Q2 2025, bringing the total since product launch to 29. Market access has increased to 79% of covered lives.

Pipeline and Innovation Highlights

- Recently filed the arimoclomol MAA with the European Medicines Agency (EMA) for the treatment of NPC, meeting the Company's guidance for submission in second half of 2025. Arimoclomol has been designated an Orphan Medicinal Product by the EMA. Arimoclomol is currently marketed in the U.S. under the brand name MIPLYFFA®.
- Enrolled 7 patients in the Phase 3 DiSCOVER trial of celiprolol for the treatment of Vascular Ehlers-Danlos Syndrome during Q2 2025, bringing the total number of enrolled patients to 39.

Corporate Highlights

- On April 1, 2025, Zevra closed the sale of its Pediatric Rare Disease Priority Review Voucher ("PRV") for gross proceeds of \$150.0 million, bolstering the Company's balance sheet with non-dilutive capital.

Q2 2025 Financial Highlights

- **Revenue, Net:** \$25.9 million for Q2 2025, which includes \$21.5 million of MIPLYFFA net revenue, \$0.3 million of OLPRUVA net revenue, \$2.6 million in net reimbursements from the French EAP for arimoclomol, \$1.2 million in royalties and other reimbursements under the AZSTARYS® license agreement, and an upfront payment of \$0.3 million from the out-license of our dextrophan prodrug. For Q2 2024, total net revenue was \$4.4 million.
- **Cost of Product Revenue:** \$14.0 million for Q2 2025, which includes \$1.6 million of non-cash intangible asset amortization for the period, and inventory obsolescence expense of \$11.7 million. Based on our quarterly assessment of the recoverability of certain intangible assets and inventory related to OLPRUVA in accordance with U.S. GAAP, the prevailing trend for enrollments, paid authorizations, as well as market and competitive landscape dynamics, we have also recognized a non-cash charge of \$58.7 million for impairment of intangible assets as of June 30, 2025.

- **Operating Expenses:** \$24.2 million for Q2 2025, which includes non-cash stock compensation expense of \$2.5 million. For Q2 2024, total operating expenses were \$23.1 million.
 - R&D expense was \$3.4 million for Q2 2025, which was a decrease of \$7.1 million compared to Q2 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 \$20.8 million for Q2 2025, which was an increase of \$8.2 million compared to Q2 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.
- **Other Income:** \$147.9 million for Q2 2025, which includes \$148.3 million of net proceeds from the sale of the PRV which closed on April 1, 2025, and offset by \$0.4 million in non-cash fair value adjustment.
- **Net Income (Loss):** \$74.7 million, or \$1.24 per basic share and \$1.21 per diluted share for Q2 2025, compared to a net loss of \$(19.9) million, or \$(0.48) per basic and diluted share, in Q2 2024.

Excluding \$148.3 million of net proceeds from the one-time PRV sale, the \$58.7 million non-cash impairment charge related to intangible assets, and the \$11.7 million inventory obsolescence charge recognized during the quarter, adjusted net loss was \$(3.2) million, or \$(0.06) adjusted net loss per basic and diluted share for Q2 2025¹.

- **Cash Position:** Cash, cash equivalents and securities were \$217.7 million as of June 30, 2025. Based on the Company's current operating forecast, we believe we have sufficient resources and financial flexibility to execute on our strategic priorities independent from the capital markets.
- **Common and Fully Diluted Shares O/S:** As of June 30, 2025, total shares of common stock outstanding were 55,084,204, and fully diluted common shares were 67,962,774, which included 7,395,043 issuable from outstanding awards under equity incentive plans, and 5,483,527 shares issuable upon exercise of warrants.

¹Adjusted net loss and adjusted net loss per share are non-GAAP financial measures. Management believes that adjusted net loss and adjusted net loss per share provide useful information for investors, and management uses these supplemental measures to assess the Company's operating performance. Adjusted net loss and adjusted net loss per share have limitations as analytical tools because they do not reflect all of the amounts associated with our results of operations as determined in accordance with U.S. GAAP. Additionally, they may not be comparable to similarly titled measures of other companies, including in our industry, limiting the usefulness of those measures for comparative purposes. Because of these limitations, these non-GAAP financial measures should be considered along with other operating and financial performance measures presented in accordance with U.S. GAAP. The presentation of these non-GAAP financial measures are not intended to be considered in isolation or as a substitute for, or superior to, financial information prepared and presented in accordance with U.S. GAAP.

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 second quarter 2025.

The audio webcast will be accessible via the Investor Relations section of the Company's website, <http://investors.zevra.com/>. An archive of the audio webcast will be available for ninety (90) days beginning at approximately 5:30 p.m. ET on August 12, 2025.

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

- (800) 245-3047 (United States)
- +1 (203) 518-9765 (International)
- Conference ID: ZVRAQ225

Inducement Awards

Since August 19, 2024, the Company has granted inducement equity awards to 9 individuals totaling 54,875 shares of restricted stock units of Zevra's common stock, vesting over three years, with 33 1/3% vesting on the first anniversary of each respective individual's start date, and the remainder vesting in two equal annual installments thereafter, and 49,750 options to purchase Zevra's common stock, vesting over four years, with 25% vesting on the first anniversary of each respective individual's start date, and the remainder vesting in three equal annual installments thereafter. The inducement awards were granted under the Zevra Therapeutics, Inc. 2023 Employment Inducement Award Plan (as amended and/or restated, the Inducement Award Plan) and, in each case, an award agreement thereunder.

The stock option awards were granted as inducement material to the individuals entering into employment with Zevra in accordance with Nasdaq 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.

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.

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 ≥ 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 UCDs. 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 m² or greater, with UCDs, 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.

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 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 arimocloamol; promise and potential impact of our preclinical or clinical trial data; the initiation, timing and results of any clinical trials or readouts, the content, information used for, timing or results of any NDA submissions or resubmissions for any products or product candidates for any specific disease indication or at any dosage; 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 six months ended June 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.

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

	Three months ended June 30,		Six months ended June 30,	
	2025	2024	2025	2024
Revenue, net	\$ 25,881	\$ 4,449	\$ 46,282	\$ 7,874
Cost of product revenue (excluding \$1,616 and \$1,546 in intangible asset amortization for the three months ended June 30, 2025, and 2024, respectively, and \$3,231 and \$3,074 in intangible asset amortization for the six months ended June 30, 2025, and 2024, respectively, shown separately below)	12,379	3,573	13,724	3,748
Intangible asset amortization	1,616	1,546	3,231	3,074
Impairment of intangible assets	58,710	—	58,710	—
Operating expenses:				
Research and development	3,433	10,521	6,691	22,798
Selling, general and administrative	20,782	12,604	40,327	22,535
Total operating expenses	<u>24,215</u>	<u>23,125</u>	<u>47,018</u>	<u>45,333</u>
Loss from operations	<u>(71,039)</u>	<u>(23,795)</u>	<u>(76,401)</u>	<u>(44,281)</u>
Other income (expense):				
Gain on sale of PRV	148,325	—	148,325	—
Interest expense	(2,009)	(2,110)	(3,978)	(2,845)
Fair value adjustment related to warrant and CVR liability	(747)	5,779	4,127	9,406
Fair value adjustment related to investments	(2)	1	(5)	(26)
Interest and other income, net	2,378	270	2,921	1,199
Total other income	<u>147,945</u>	<u>3,940</u>	<u>151,390</u>	<u>7,734</u>
Income (loss) before income taxes	76,906	(19,855)	74,989	(36,547)
Income tax expense	(2,199)	(70)	(3,381)	—
Net income (loss)	<u>\$ 74,707</u>	<u>\$ (19,925)</u>	<u>\$ 71,608</u>	<u>\$ (36,547)</u>
Net income (loss) per share of common stock:				
Basic	\$ 1.24	\$ (0.48)	\$ 1.20	\$ (0.87)
Diluted	\$ 1.21	\$ (0.48)	\$ 1.16	\$ (0.87)
Weighted-average shares of common stock outstanding:				
Basic	54,780,938	41,899,087	54,440,100	41,839,582
Diluted	56,324,903	41,899,087	56,062,443	41,839,582

ZEVRA THERAPEUTICS, INC. UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS

(in thousands, except share and par value amounts)

	June 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	\$ 47,712	\$ 33,785
Securities at fair value, current	154,899	35,711
Accounts and other receivables	18,266	10,509
Prepaid expenses and other current assets	4,352	4,052
Inventories, current	1,108	1,970
Total current assets	226,337	86,027
Securities at fair value, noncurrent	15,089	6,010
Inventories, noncurrent	782	10,999
Property and equipment, net	599	356
Operating lease right-of-use assets	1,496	657
Goodwill	4,701	4,701
Intangible assets, net	7,053	68,993
Other long-term assets	220	384
Total assets	\$ 256,277	\$ 178,127
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	\$ 15,842	\$ 25,456
Current portion of operating lease liabilities	525	420
Current portion of discount and rebate liabilities	5,599	4,989
Other current liabilities	6,866	3,200
Total current liabilities	28,832	34,065
Long-term debt	60,692	59,504
Warrant liability	15,807	17,804
Income tax payable	17,479	14,431
Operating lease liabilities, less current portion	1,059	372
Discount and rebate liabilities, less current portion	11,862	7,655
Other long-term liabilities	3,316	4,630
Total liabilities	139,047	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 June 30, 2025, or December 31, 2024	—	—
Common stock, \$0.0001 par value, 250,000,000 shares authorized; 56,659,896 shares issued and 55,084,204 shares outstanding as of June 30, 2025; 55,246,401 shares issued and 53,670,709 shares outstanding as of December 31, 2024	6	5
Additional paid-in capital	564,316	555,302
Treasury stock, at cost	(10,983)	(10,983)
Accumulated deficit	(433,681)	(505,289)
Accumulated other comprehensive (loss) income	(2,428)	631
Total stockholders' equity	117,230	39,666
Total liabilities and stockholders' equity	\$ 256,277	\$ 178,127