Zevra Reports First Quarter 2025 Financial Results and Corporate Update

May 13, 2025

Q1 2025 net revenue of $20.4 million, driven by product net revenue of $17.2 million

Completed sale of PRV for gross proceeds of $150 million, positioning balance sheet to drive the Company's commercial launches and development programs

Company to host conference call and webcast today, May 13, 2025, at 4:30 p.m. ET

CELEBRATION, Fla., May 13, 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 first quarter ended Mar. 31, 2025.

"We are continuing Zevra's transformation into a patient-centric, commercial-stage rare disease therapeutics company," said **Neil F. McFarlane, Zevra's President and Chief Executive Officer**. "With our bolstered cash balance, we are well-positioned to support our strategic priorities, including establishing MIPLYFFA® as the cornerstone of treatment for NPC globally, maximizing our commercial opportunity with OLPRUVA®, and supporting our growth through our development pipeline."

Commercial Highlights

- All active patients in the U.S. Expanded Access Program (EAP) for arimoclomol, have been enrolled to receive MIPLYFFA. The EAP has since closed, including locking the database and deactivating study sites.
- MIPLYFFA: 13 new prescription enrollment forms during Q1 2025, marking 122 total through the end of the quarter. In line with expectations at this stage of launch, market access is 38% of covered lives.
- OLPRUVA: 5 new patient enrollment forms were received in Q1 2025, bringing the total from initial product availability in July 2023, and including Zevra's promotion of OLPRUVA since late January 2024, to 28. Market access has increased to 78% of covered lives.

Pipeline and Innovation Highlights

- Filing of the MIPLYFFA MAA with the European Medicines Agency remains on track for the second half of 2025 as reinforced by recent conversations with European authorities.
- Enrolled 5 additional patients in the Phase 3 DiSCOVER trial of celiprolol for the treatment of Vascular Ehlers-Danlos Syndrome, bringing the total number of enrolled patients to 32.
- The company out-licensed IP related to a pre-clinical prodrug that had been deprioritized. Zevra is entitled to receive a nominal upfront cash payment, potential future regulatory milestones, and mid-single-digit royalties on net sales.

Corporate Highlights and Upcoming Milestones

- On April 7, 2025, Zevra announced closing of the sale of its Pediatric Rare Disease Priority Review Voucher ("PRV") to an undisclosed buyer for gross proceeds of $150 million.

Q1 2025 Financial Highlights

- **Revenue, Net**: $20.4 million for Q1 2025, which includes $17.1 million of MIPLYFFA net revenue, $0.1 million of OLPRUVA net revenue, $2.3 million in net reimbursements from the French EAP for arimoclomol, and $0.9 million in royalties and other reimbursements under the AZSTARYS® license agreement. This was an increase in total net revenue of $17.0 million compared to $3.4 million in Q1 2024.
- **Cost of Goods Sold**: $3.0 million for Q1 2025, which includes $1.6 million of non-cash intangible asset amortization.
- **Operating Expenses**: $22.8 million for Q1 2025, which includes non-cash stock compensation expense of $3.1 million.
    - R&D expense was $3.3 million for Q1 2025, which was a decrease of $9.0 million compared to Q1 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 $19.5 million for Q1 2025, which was an increase of $9.6 million compared to Q1 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**: ($3.1) million, or ($0.06) per basic and diluted share for Q1 2025, compared to a net loss of ($16.6) million, or ($0.40) per basic and diluted share in Q1 2024.

- **Cash Position**: Cash, cash equivalents and investments were $68.7 million as of Mar. 31, 2025. Combined with the net proceeds of $148.3 million from the sale of the PRV received just after the end of the quarter on Apr. 1, 2025, cash, cash equivalents and investments would be $217.0 million. Based on the Company's current operating forecast and adding the non-dilutive capital from the PRV sale, we have ample resources and financial flexibility to execute on our strategic priorities independent from the capital markets.

- **Common and Fully Diluted Shares O/S**: As of Mar. 31, 2025, total shares of common stock outstanding were 54,679,363, and fully diluted common shares were 67,897,631, which included 7,734,741 issuable from outstanding awards under equity incentive plans, and 5,483,527 shares issuable upon exercise of warrants.

Upcoming Events

- Zevra will participate in a fireside chat at the H.C. Wainwright 3rd Annual Bioconnect Investor Conference on Tuesday, May 20, 2025, at 12:30 p.m. ET. Management will be available for one-on-one meetings with registered attendees.

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 first 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 May 13, 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: ZVRAQ125

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.

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 (≥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 m^2 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 rare disease company combining science, data, and patient need to create transformational therapies for diseases with limited or no treatment options. 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 the sale of the PRV and anticipated proceeds therefrom; 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; the potential benefits of our debt facility; our financial position, including our cash balance and anticipated cash runway; potential revenues from MIPLYFFA sales; potential revenues from our arimoclomol expanded access program in France; the potential for royalty and milestone contributions, the presentation of data at conferences; 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 Dec. 31, 2024, filed on Mar. 12, 2025, 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 March 31,	
	2025	**2024**
Revenue, net	$ 20,401	$ 3,425
Cost of product revenue (excluding $1,615 and $1,528 in intangible asset amortization for the three months ended March 31, 2025, and March 31, 2024, respectively, shown separately below)	1,345	175
Intangible asset amortization	1,615	1,528
Operating expenses:		
Research and development	3,258	12,277
Selling, general and administrative	19,545	9,931
Total operating expenses	22,803	22,208
Loss from operations	(5,362)	(20,486)
Other income (expense):		
Interest expense	(1,969)	(735)
Fair value adjustment related to warrant and CVR liability	4,874	3,627
Fair value adjustment related to investments	(3)	(27)

Interest and other income, net	543	929
Total other income	3,445	3,794
Income (loss) before income taxes	(1,917)	(16,692)
Income tax (expense) benefit	(1,182)	70
Net loss	$ (3,099)	$ (16,622)
Basic and diluted net loss per share of common stock:		
Net loss	$ (0.06)	$ (0.40)
Weighted average number of shares of common stock outstanding:		
Basic and diluted	54,095,543	41,778,774

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

	March 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 37,340	$ 33,785
Securities at fair value, current	25,291	35,711
Accounts and other receivables	12,617	10,509
Prepaid expenses and other current assets	3,766	4,052
Inventories, current	2,559	1,970
Total current assets	81,573	86,027
Securities at fair value, noncurrent	6,091	6,010
Inventories, noncurrent	10,615	10,999
Property and equipment, net	422	356
Operating lease right-of-use assets	1,636	657
Goodwill	4,701	4,701
Intangible assets, net	67,377	68,993
Other long-term assets	293	384
Total assets	$ 172,708	$ 178,127
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 17,819	$ 25,456
Current portion of operating lease liabilities	589	420
Current portion of discount and rebate liabilities	5,167	4,989
Other current liabilities	3,401	3,200
Total current liabilities	26,976	34,065
Long-term debt	60,090	59,504
Warrant liability	13,030	17,804
Income tax payable	16,166	14,431
Operating lease liabilities, less current portion	1,158	372
Discount and rebate liabilities, less current portion	9,389	7,655
Other long-term liabilities	4,876	4,630
Total liabilities	131,685	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 March 31, 2025, or December 31, 2024	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 56,255,055 shares issued and 54,679,363 shares outstanding as of March 31, 2025; 55,246,401 shares issued and 53,670,709 shares outstanding as of December 31, 2024	5	5
Additional paid-in capital	560,471	555,302
Treasury stock, at cost	(10,983)	(10,983)
Accumulated deficit	(508,388)	(505,289)
Accumulated other comprehensive (loss) income	(82)	631
Total stockholders' equity	41,023	39,666
Total liabilities and stockholders' equity	$ 172,708	$ 178,127