



Zevra Therapeutics Reports Third Quarter 2024 Financial Results and Corporate Updates

November 12, 2024

FDA approval of MIPLYFFA™ and product launch cap third quarter filled with multiple commercial and clinical development milestones

Zevra received rare pediatric disease Priority Review Voucher

Company ends quarter with cash, cash equivalents and investments of \$95.5 million

Company to host conference call and webcast today, Nov. 12, 2024, 4:30 p.m. ET

CELEBRATION, Fla., Nov. 12, 2024 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage company focused on addressing unmet needs for the treatment of rare diseases, today reported its financial results for the third quarter ended Sept. 30, 2024, and provided corporate updates.

"The third quarter was one of the most exciting and transformational periods in Zevra's journey," said **Neil F. McFarlane, Zevra's President and Chief Executive Officer**. "After years of tireless effort, our team has achieved a major milestone with FDA approval of MIPLYFFA™ (arimoclomol), and we're celebrating with the Niemann-Pick disease type C (NPC) community. We intend to achieve our goals with a high-performing team committed to execute, focus and innovate to drive our continued growth and long-term transformation."

Program Highlights

- On Sept. 20, the U.S. Food and Drug Administration (FDA) approved MIPLYFFA capsules as an orally delivered treatment for NPC. MIPLYFFA, the first NPC drug approved by the FDA, is indicated for use in combination with miglustat for the treatment of neurological manifestations of NPC in adult and pediatric patients 2 years of age and older. With the approval of MIPLYFFA, Zevra received a rare pediatric disease Priority Review Voucher (PRV) which the Company could monetize to help fund its growth.
- As of Oct. 31, Zevra has received 90 prescription enrollment forms for MIPLYFFA of which 30% are approved for reimbursement and are ready for fulfillment upon drug availability, consistent with the Company's prior guidance of eight to 12 weeks post launch.
- Zevra has launched [AmplifyAssist™](#), a comprehensive support program for caregivers and those eligible living with NPC or UCDS.
- OLPRUVA® had three new patient enrollments in the third quarter, and reimbursement coverage is at approximately 76% of covered lives. Improving OLPRUVA's formulary position is a primary objective to make it the preferred treatment for UCDS.
- Zevra is refining its commercial strategy for OLPRUVA to focus on specific patient segments where there are fewer access barriers, and, together with The National Urea Cycle Disorders Foundation, launched a targeted patient education campaign highlighting the importance of prompt and accurate testing of blood ammonia levels, which, when elevated, may be a sign of a UCD. Campaign materials are available at www.checkammonia.com.
- The Company recently completed an End-of-Phase 2 meeting with the FDA for KP1077. The Agency indicated that a single pivotal study with appropriate confirmatory evidence will be sufficient to submit a new drug application (NDA). Zevra is evaluating strategic alternatives to advance clinical development and later, commercialization.
- The Phase 3 DiSCOVER trial of celiprolol for the treatment of Vascular Ehler-Danlos Syndrome (VEDS) is underway, and the Company has achieved its objective to restart enrollment and to begin dosing new patients, reporting that a total of 19 patients are now in the trial as of the end of the third quarter. Zevra is continuing its efforts to expand its partnerships with patient advocacy groups, treatment centers of excellence, and medical key opinion leaders.

Corporate Highlights and Upcoming Milestones

- In August, Zevra completed an underwritten public offering of 10,615,385 shares of its common stock at an offering price of \$6.50 per share for total net proceeds of approximately \$64.5 million, after deducting underwriting discounts and

commissions and offering expenses. Cantor and William Blair were joint book-running managers for the offering. Citizens JMP and H.C. Wainwright & Co. were co-managers.

- The Company completed a thorough portfolio assessment and developed a strategic plan for 2025 that builds from its 2024 accomplishments and furthers Zevra's transformation towards becoming a leading rare disease company with a five-year vision to create value for patients and shareholders by organizing our priorities on four key pillars: **Commercial Excellence, Pipeline and Innovation, Talent and Culture, and Corporate Foundation.**

Now focused on late-stage clinical development and commercial opportunities, Zevra has discontinued its in-house drug discovery activities and is closing its laboratory facilities in Iowa and Virginia. Future early research and development activities will be outsourced.

Q3 2024 Financial Highlights

- **Revenue, Net:** \$3.7 million, comprised of \$2.6 million in net reimbursements from the French expanded access program (EAP) for arimoclomol, \$1.1 million of royalties and other reimbursements under the AZSTARYS® license agreement, and de minimis OLPRUVA revenue.
- **Cost of Goods Sold:** \$2.3 million, which includes recognition of \$2.0 million in inventory obsolescence reserve expense related to OLPRUVA inventory that is nearing expiration.
- **R&D Expenses:** \$10.9 million, which represents a decrease of \$1.4 million compared to Q3 2023 due to the completion of the KP1077 phase 2 trial.
- **SG&A Expenses:** \$16.2 million for Q3 2024, compared to \$5.8 million in Q3 2023, which reflects the commercial team fully in place and actively engaged in commercial launch activities and patient services initiatives. This includes non-cash stock compensation of \$6.1 million during the quarter, of which \$2.5 million was performance-based upon the approval of MIPLYFFA.
- **Net Loss:** (\$33.2) million, or (\$0.69) per basic and diluted share for Q3 2024, compared to a net loss of (\$10.4) million, or (\$0.30) per basic and diluted share in Q3 2023.
- **Cash Position:** Cash, cash equivalents and investments were \$95.5 million as of Sept. 30, 2024.
- **Cash Runway Forecast:** Based on the Company's current operating plan, available cash, cash equivalents and investments are expected to extend Zevra's cash runway into 2027, subject to continuing compliance with debt covenants. Cash runway forecast includes anticipated revenue from MIPLYFFA sales, reimbursements from the French EAP for arimoclomol, royalties under the AZSTARYS license agreement, and continued investments into our development pipeline programs. It does not include potential proceeds from a PRV sale.
- **Common and Fully Diluted Shares O/S:** As of Sept. 30, 2024, total shares of common stock outstanding were 53,227,364, and fully diluted common shares were 67,698,898, which included 5,483,537 shares issuable upon exercise of warrants.

Upcoming Events

- Guggenheim Securities Healthcare Innovation Conference, with fireside chat on Wednesday, Nov. 13, 2024, at 4:00 p.m. ET. Management will be available for one-on-one meetings with registered attendees.

Conference Call Information - UPDATED

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 of 2024.

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 Nov. 12, 2024.

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

- (785) 838-9345 (United States) -- **updated dial-in**
- +1 (203) 518-9814 (International)
- Conference ID: ZVRAQ324

About MIPLYFFA

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. MIPLYFFA was granted Breakthrough Therapy designation, Rare Pediatric Disease designation, Orphan Drug designation, and Fast Track designation by the FDA for the treatment of NPC. MIPLYFFA was further granted 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 ($\geq 15\%$) 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.

Before prescribing MIPLYFFA, please read the full [Prescribing Information, including Instructions for Use](#).

Drug Interaction(s):

Arimoclocholol 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) was approved for the treatment of certain UCIDs in December 2022 and has recently been marketed under the brand name, OLPRUVA. 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 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.

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 FDA at 1-800-FDA-1088.

About KP1077

Serdexmethylphenidate (SDX) is Zevra's proprietary prodrug of d-methylphenidate (d-MPH) and is the sole active pharmaceutical ingredient (API) of KP1077. KP1077 has been granted Orphan Drug designation by the FDA, and by the European Commission, for the treatment of Idiopathic Hypersomnia, or IH. The U.S. Drug Enforcement Agency (DEA) has classified SDX as a Schedule IV controlled substance based on evidence suggesting SDX has a lower potential for abuse when compared to d-MPH, a Schedule II controlled substance. In addition, KP1077 has intellectual property protection through 2037.

About Celiprolol

Celiprolol is an investigational clinical candidate for the treatment of Vascular Ehlers-Danlos Syndrome (VEDS). Celiprolol has been granted Orphan Drug and Breakthrough Therapy designations by the FDA. Zevra recently restarted enrollment in the Phase 3 trial, known as the DiSCOVER trial being conducted under a Special Protocol Assessment (SPA) agreement with the 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 needs 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 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; the impact of meetings or communications with the FDA or any advisory committee; statements or decisions by the FDA or any other entity for any products or product candidates; our ability to monetize any PRV; 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; our corporate governance objectives; 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, 2023, Zevra's Quarterly Report on Form 10-Q for the three months ended Sept. 30, 2024, 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 September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Revenue, net	\$ 3,695	\$ 2,895	\$ 11,569	\$ 14,541
Cost of product revenue (excluding \$1,545 and \$4,619 in intangible asset amortization for the three and nine months ended September 30, 2024, respectively shown separately below)	2,303	144	6,051	946
Intangible asset amortization	1,545	—	4,619	—
Operating expenses:				
Research and development	10,945	12,297	33,743	28,385
Selling, general and administrative	16,208	5,818	38,743	19,657
Total operating expenses	27,153	18,115	72,486	48,042
Loss from operations	(27,306)	(15,364)	(71,587)	(34,447)
Other (expense) income:				
Interest expense	(2,312)	(366)	(5,157)	(745)
Fair value adjustment related to warrant and CVR liability	(4,746)	3,678	4,660	4,253
Fair value adjustment related to investments	90	124	64	451
Interest and other income (expense), net	1,049	1,738	2,248	4,331
Total other (expense) income	(5,919)	5,174	1,815	8,290
Loss before income taxes	(33,225)	(10,190)	(69,772)	(26,157)
Income tax expense	—	(177)	—	—
Net loss	\$ (33,225)	\$ (10,367)	\$ (69,772)	\$ (26,157)
Basic and diluted net loss per share of common stock:				
Net loss	\$ (0.69)	\$ (0.30)	\$ (1.59)	\$ (0.76)
Weighted average number of shares of common stock outstanding:				
Basic and diluted	47,808,817	34,724,614	43,843,851	34,364,075

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

	September 30, 2024	December 31, 2023
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Assets

Current assets:

Cash and cash equivalents	\$ 54,039	\$ 43,049
Securities at fair value, current	35,337	24,688
Accounts and other receivables	7,790	17,377
Prepaid expenses and other current assets	2,276	1,824
Total current assets	99,442	86,938
Inventories	8,756	9,841
Securities at fair value, long-term	6,105	—
Property and equipment, net	607	736
Operating lease right-of-use assets	820	790
Goodwill	4,701	4,701
Intangible assets, net	70,608	69,227
Other long-term assets	512	94
Total assets	<u>\$ 191,551</u>	<u>\$ 172,327</u>

Liabilities and stockholders' equity

Current liabilities:

Accounts payable and accrued expenses	\$ 24,464	\$ 28,403
Line of credit payable	—	37,700
Current portion of operating lease liabilities	540	543
Current portion of discount and rebate liabilities	8,547	4,550
Other current liabilities	930	2,524
Total current liabilities	34,481	73,720
Long-term debt	58,904	5,066
Warrant liability	13,902	16,100
Operating lease liabilities, less current portion	483	456
Discount and rebate liabilities, less current portion	8,490	7,663
Other long-term liabilities	5,521	7,458
Total liabilities	<u>121,781</u>	<u>110,463</u>

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, 2024, or December 31, 2023	—	—
Common stock, \$0.0001 par value, 250,000,000 shares authorized, 54,803,056 shares issued and 53,227,364 shares outstanding as of September 30, 2024; 43,110,360 shares issued and 41,534,668 shares outstanding as of December 31, 2023	5	4
Additional paid-in capital	550,413	472,664
Treasury stock, at cost	(10,983)	(10,983)
Accumulated deficit	(469,550)	(399,778)
Accumulated other comprehensive income (loss)	(115)	(43)
Total stockholders' equity	<u>69,770</u>	<u>61,864</u>
Total liabilities and stockholders' equity	<u>\$ 191,551</u>	<u>\$ 172,327</u>