Zevra Reports Third Quarter 2026 Financial Results and Corporate Update

November 3, 2026

$37.8 million net revenue for Q3 2026, driven by $34.0 million in revenue from MIPLYFFA

45% increase in total net revenue over Q3 2025

Operational execution fueled strong cash position of $270.9 million

Company to host conference call and webcast TODAY, November 3, 2026, at 4:30 p.m. ET

BOSTON, Mass., Nov. 03, 2026 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage company focused on bringing life-changing therapeutics to people living with rare diseases, today reported its financial results for the third quarter ended September 30, 2026.

“In the third quarter, we made continued progress across our business, building on MIPLYFFA's commercial performance in the U.S. and advancing our pipeline in support of our mission to redefine what’s possible in bringing life-changing therapies to people living with rare disease,” said Neil F. McFarlane, Zevra's President and Chief Executive Officer. “We remain focused on reaching more people living with Niemann-Pick disease type C, advancing celiprolol for vascular Ehlers-Danlos syndrome, and pursuing European access to arimoclomol. Our strong financial position enables us to advance these priorities while continuing to make arimoclomol available to eligible patients through our global Expanded Access Program.”

MIPLYFFA® (arimoclomol) Highlights

- U.S.: Received 13 MIPLYFFA prescription enrollment forms for Niemann-Pick disease type C (NPC) during Q3 2026, bringing the total to 197 since product launch.
- EU: The Company requested a re-examination of its Marketing Authorisation Application for arimoclomol for the treatment of NPC and is awaiting the outcome of the process.
- Global Expanded Access Program (EAP): As of September 30, 2026, 134 patients were enrolled in the global EAP.
- Following the publication of its pediatric data in *Molecular Genetics and Metabolism Reports,* Zevra met with the U.S. Food and Drug Administration (FDA) to discuss a potential supplemental New Drug Application (sNDA) for MIPLYFFA in infants less than 24 months of age. Company anticipates filing sNDA in Q1 2027.

Pipeline and Innovation Highlights

- Enrolled six patients in the event-driven Phase 3 DiSCOVER trial of celiprolol for the treatment of Vascular Ehlers-Danlos Syndrome during Q3 2026, bringing the total number of enrolled patients to 72, with a total of four confirmed events. The Company recently engaged with the FDA for a Type C meeting to discuss a potential submission strategy for celiprolol.

Q3 2026 Financial Highlights

- **Revenue, Net:** $37.8 million for Q3 2026, which includes $34.0 million of MIPLYFFA net revenue, $0.2 million of OLPRUVA net revenue, and $3.6 million in net reimbursements from our global EAP. This was an increase in total net revenue of $11.7 million compared to $26.1 million in Q3 2025.
- **Cost of Product Revenue:** $1.8 million for Q3 2026, excluding non-cash intangible asset amortization. Cost of product revenue for Q3 2025 was $1.2 million, excluding non-cash intangible asset amortization.
- **Operating Expenses:** $24.1 million for Q3 2026, which includes non-cash stock compensation expense of $2.7 million. Total operating expenses for Q3 2025 were $20.4 million.
    - R&D expense was $4.9 million for Q3 2026, which was an increase of $1.5 million compared to $3.4 million for Q3 2025, due primarily to our ongoing investment in arimoclomol efforts and in the celiprolol development program.
    - SG&A expense was $19.1 million for Q3 2026, which was an increase of $2.2 million compared to $16.9 million for Q3 2025, due primarily to commercial and infrastructure related costs.
- **Net income (loss):** Net income of $19.2 million, or $0.31 per basic and $0.26 diluted share for Q3 2026, compared to a

net (loss) of $(0.5) million, or $(0.01) per basic and diluted share for Q3 2025.

    - In Q3 2026, the Company recognized $5.1 million in noncash income due to fair value adjustments related to warrant and contingent value rights (CVR) liability compared to $(5.5) million of noncash expense in Q3 2025.

- **Cash Position:** Cash, cash equivalents and investments were $270.9 million as of September 30, 2026. Based on its current operating forecast, the Company believes available financial resources are sufficient to execute on its strategic priorities.
- **Common and Fully Diluted Shares O/S:** As of September 30, 2026, total shares of common stock outstanding were 59,439,959, and fully diluted common shares were 68,968,909, which included 6,999,571 shares issuable from outstanding awards under equity incentive plans, and 2,529,379 shares issuable upon exercise of warrants.

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 2026.

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. A replay of the webcast will be available for 90 days beginning at approximately 5:30 p.m. ET on November 3, 2026.

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

- (800) 274-8461 (United States)
- +1 (203) 518-9814 (International)
- Conference ID: ZVRAQ326

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 more than 5 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 (≥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. toll-free at 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.

For more information, please see the full Prescribing Information, including Instructions for Use.

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 company with a late-stage pipeline committed to redefining what is possible in bringing life-changing therapies to people living with rare diseases. The Company is focused on broadening access through geographic expansion opportunities, progressing its pipeline toward key milestones, and delivering meaningful therapeutics. The commercialization of its lead product, marketed in the U.S. for Niemann-Pick disease type C (NPC), a rare, progressive neurodegenerative disease, provides a strong corporate foundation and validates its ability to advance therapies from development to market. Zevra's vision is realized through disciplined execution of its strategic plan and core values — patient centricity, integrity, accountability, innovation, and courage — which guide its efforts to deliver long-term value.

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 expanding access to MYPLYFFA, including with respect to the requested re-examination of the MAA for arimoclomol in the EU and the continued availability of our global EAP; the potential to accelerate development of the Company's treatment for Vascular Ehlers-Danlos Syndrome and a potential submission strategy; and the sufficiency of the Company's available financial resources to execute on its strategic priorities. 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, 2025, filed on March 9, 2026, 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,	
	2026	2025	2026	2025
Revenue, net	$ 37,756	$ 26,063	$ 113,639	$ 72,345
Cost of product revenue (excluding $315 and $315 in intangible asset amortization for the three months ended September 30, 2026, and 2025, respectively, and $947 and $3,546 in intangible asset amortization for the nine months ended September 30, 2026, and 2025, respectively, shown separately below)	(1,794)	(1,238)	(5,216)	(14,962)
Intangible asset amortization	(315)	(315)	(947)	(3,546)
Impairment of intangible assets	—	—	—	(58,710)
Gain on sale of future royalties, intellectual property, and other assets, net	—	—	43,314	—
Operating expenses:				
Research and development	(4,937)	(3,432)	(13,815)	(10,123)
Selling, general and administrative	(19,130)	(16,935)	(56,472)	(57,262)
Total operating expenses	(24,067)	(20,367)	(70,287)	(67,385)
Income (loss) from operations	11,580	4,143	80,503	(72,258)
Other income (expense):				
Gain on sale of PRV	—	—	—	148,325
Loss on extinguishment of debt	—	—	(2,756)	—
Loss on derivative liability	—	—	(7,216)	—
Interest expense	—	(2,051)	(1,711)	(6,029)
Fair value adjustment related to warrant and CVR liability	5,143	(5,506)	(297)	(1,379)
Fair value adjustment related to investments	(39)	124	(395)	119
Interest and other income, net	3,034	2,313	9,189	5,234
Total other income (expense)	8,138	(5,120)	(3,186)	146,270
Income (loss) before income taxes	19,718	(977)	77,317	74,012
Income tax (expense) benefit	(510)	433	(11,467)	(2,948)

Net income (loss)	$ 19,208	$ (544)	$ 65,850	$ 71,064
Net income (loss) per share of common stock:				
Basic	$ 0.31	$ (0.01)	$ 1.07	$ 1.20
Diluted	$ 0.26	$ (0.01)	$ 1.03	$ 1.16
Weighted-average shares of common stock outstanding:				
Basic	59,407,392	55,951,572	58,993,342	54,949,483
Diluted	61,732,366	55,951,572	61,103,394	56,782,763

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

	September 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 122,148	$ 62,406
Investments, current	73,420	128,605
Accounts and other receivables	19,943	23,258
Prepaid expenses and other current assets	7,257	6,998
Inventories, current	3,070	1,740
Total current assets	225,838	223,007
Investments, noncurrent	75,323	47,879
Inventories, noncurrent	—	879
Property and equipment, net	816	489
Operating lease right-of-use assets	1,423	1,212
Goodwill	4,701	4,701
Intangible assets, net	5,474	6,421
Other long-term assets	—	143
Total assets	$ 313,575	$ 284,731
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 13,672	$ 11,598
Current portion of operating lease liabilities	626	419
Current portion of discount and rebate liabilities	7,223	12,188
Current portion of income tax payable	18,532	13,710
Other current liabilities	1,316	1,362
Total current liabilities	41,369	39,277
Long-term debt	—	61,928
Warrant liability	7,712	9,575
Income tax payable	7,304	7,029
Operating lease liabilities, less current portion	602	859
Discount and rebate liabilities, less current portion	14,792	9,693
Other long-term liabilities	2,873	1,713
Total liabilities	74,652	130,074
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, 2026, or December 31, 2025	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized; 61,015,651 shares issued and 59,439,959 shares outstanding as of September 30, 2026; 58,338,319 shares issued and 56,854,781 shares outstanding as of December 31, 2025	6	6
Additional paid-in capital	609,618	588,458
Treasury stock, at cost	(10,983)	(10,983)
Accumulated deficit	(356,210)	(422,060)
Accumulated other comprehensive loss	(3,508)	(764)
Total stockholders' equity	238,923	154,657
Total liabilities and stockholders' equity	$ 313,575	$ 284,731