# Zevra Reports Fourth Quarter and Full Year 2025 Financial Results

March 9, 2026

*Q4 net revenue of $34.1 million, representing 31% growth quarter-over-quarter*

*FY 2025 net revenue of $106.5 million, driven by growth in MIPLYFFA® net revenue to $87.4 million*

*2025 EPS of $1.40 basic and $1.35 diluted*

*Company to host conference call and webcast TODAY, March 9, 2026, at 4:30 p.m. ET*

BOSTON, March 09, 2026 (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 fourth quarter and full year ended December 31, 2025.

“MIPLYFFA is making a meaningful difference for patients with Niemann-Pick disease type C, and its strong, accelerating performance reinforces our confidence in its long-term potential,” said Neil F. McFarlane, Zevra's President and Chief Executive Officer. “We have officially relocated our global corporate headquarters to Boston, enhancing our access to a deep and highly specialized talent pool. Looking ahead, we are focused on executing against multiple near-term growth opportunities in 2026, and believe we are well-positioned to execute on our strategic priorities to create meaningful value for the rare disease community and our shareholders.”

**MIPLYFFA® (arimoclomol) Highlights**

- U.S.: Received 24 MIPLYFFA prescription enrollment forms for Niemann-Pick disease type C (NPC) during Q4 2025, bringing the total to 52 in 2025 and 161 since product launch. Market access has reached 68% of covered lives, supporting broad access to MIPLYFFA.
- EU: A Marketing Authorisation Application for the evaluation of arimoclomol for the treatment of NPC is under review by the European Medicines Agency (EMA). Arimoclomol has been designated an Orphan Medicinal Product by the EMA. The review process is progressing as expected, and at year-end, the EMA sent their 120-day list of questions, which the Company is prepared to respond to within the regulatory 90-day clock stop period.
- Global Expanded Access Program (EAP): In Q4 2025, the Company executed a distribution agreement to broaden access to arimoclomol to select territories beyond Europe. As of December 31, 2025, 113 patients were enrolled in the global EAP.

**Pipeline and Innovation Highlights**

- Enrolled eight patients in the event-driven Phase 3 DiSCOVER trial for the treatment of Vascular Ehlers-Danlos Syndrome during Q4 2025, bringing the total number of enrolled patients at year-end to 52, with a total of one confirmed event. The Company has recently engaged the Food and Drug Administration (FDA) in a Type C meeting to discuss regulatory options to accelerate the development program.

**Publication Highlights**

- Four posters were presented at the 22nd Annual WORLD Symposium™ including new data for MIPLYFFA.
  - Four-year real-world data from the U.S. EAP demonstrated that it was well tolerated and stabilized disease progression in the overall cohort, and new data for the subgroup of adults in the EAP showed consistent results.
  - In the post hoc efficacy analysis of the randomized, placebo-controlled study, there was a statistically significant slowing of disease progression compared to placebo as early as three months after treatment initiation, with sustained and increasing benefit through 12 months, highlighting early onset of clinical effect in patients with NPC.
- Abstract titled “Analysis of NPC1 Genotypes: Findings from the U.S. Arimoclomol Expanded Access Program for Niemann-Pick Type C” (#P214) was accepted for poster presentation at the Annual Clinical Genetics Meeting on Friday, March 13, 2026.

**FY 2025 Financial Highlights**

- **Revenue, Net:** $106.5 million, which includes $87.4 million of MIPLYFFA net revenue, $0.8 million of OLPRUVA net revenue, $13.0 million in net reimbursements from our EAP, and $5.0 million in royalties and other reimbursements under the AZSTARYS® license agreement. For full year 2024, net revenue was $23.6 million, which was primarily driven by $10.1 million in MIPLYFFA net revenue, $0.1 million in OLPRUVA net revenue, $9.1 million in net reimbursements from our EAP, and $4.3 million in royalties and other reimbursements under the AZSTARYS® license agreement.
- **Cost of Product Revenue:** $16.5 million, excluding non-cash intangible asset amortization. Cost of product revenue for the year ended December 31, 2024 was $7.4 million.
- **Operating Expenses:** $90.4 million, which includes non-cash compensation expense of $12.6 million. Total operating expenses for the year ended December 31, 2024 were $97.0 million.
    - R&D expense was $12.7 million, which was a decrease of $29.4 million compared to $42.1 million for FY 2024 due primarily to a decrease in personnel-related costs, combined with a decrease in third-party costs.
    - SG&A expense was $77.6 million, which was an increase of $22.7 million compared to $54.9 million for FY 2024 due primarily to an increase in third-party costs related to MIPLYFFA, combined with an increase in personnel-related costs, professional fees, and other expenses associated with our commercial, medical and launch activities.
- **Net Income (Loss):** Net income of $83.2 million, or $1.40 per basic and $1.35 per diluted share for 2025, compared to a net loss of $(105.5) million, or $(2.28) per basic and diluted share for 2024. 2025 net income includes non-cash fair value adjustment of $2.2 million, non-cash stock-based compensation expense of $12.6 million, and non-cash intangible asset amortization expense of $3.9 million.
- **Cash Position:** Cash, cash equivalents and securities were $238.9 million as of December 31, 2025. Based on the Company's current operating forecast, the Company believes it has sufficient resources and financial flexibility to execute on its strategic priorities independent from the capital markets.
- **Common and Fully Diluted Shares O/S:** As of December 31, 2025, total shares of common stock outstanding were 56,854,781, and fully diluted common shares were 67,939,395, which included 7,060,457 issuable from outstanding awards under equity incentive plans, and 4,024,157 shares issuable upon exercise of warrants.

**Q4 2025 Financial Highlights**

- **Revenue, Net:** $34.1 million for Q4 2025, which includes $26.4 million of MIPLYFFA net revenue, $0.4 million of OLPRUVA net revenue, $5.6 million in net reimbursements from the EAP, and $1.8 million in royalties and other reimbursements under the AZSTARYS® license agreement. For Q4 2024, total net revenue was $12.0 million, which includes $10.1 million of MIPLYFFA net revenue, $0.1 million of OLPRUVA net revenue, $1.1 million in net reimbursements from the EAP, and $0.7 million in royalties and other reimbursements under the AZSTARYS® license agreement.
- **Cost of Product Revenue:** $1.5 million for Q4 2025, excluding non-cash intangible asset amortization. Cost of product revenue for Q4 2024 was $1.4 million.
- **Operating Expenses:** $23.0 million for Q4 2025, which includes non-cash stock compensation of $4.3 million. Total operating expenses for Q4 2024 were $24.5 million.
    - R&D expense was $2.6 million for Q4 2025, which was a decrease of $5.8 million compared to $8.4 million for Q4 2024 due primarily to a decrease in personnel-related costs, combined with a decrease in third-party costs.
    - SG&A expense was $20.4 million for Q4 2025, which was an increase of $4.3 million compared to $16.1 million for Q4 2024, due primarily to an increase in third-party costs incurred, mainly due to higher spend on commercial products, combined with an increase in personnel-related costs.
- **Net Income (Loss):** Net income of $12.2 million, or $0.20 per basic and $0.19 per diluted share for Q4 2025, compared to a net loss of $(35.7) million, or $(0.67) per basic and diluted share for Q4 2024.

**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 fourth quarter and full year 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. A replay of the webcast will be available for 90 days beginning at approximately 5:30 p.m. ET on March 9, 2026.

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: ZVRAQ425

**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 (≥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 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 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, 2025, to be filed with the Securities and Exchange Commission, 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.**

**CONSOLIDATED STATEMENTS OF OPERATIONS**

**(in thousands, except share and per share amounts)**

| | Year Ended December 31, | |
|---|---|---|
| | **2025** | **2024** |
| Revenue, net | $ 106,470 | $ 23,612 |
| Cost of product revenue (excluding $3,862 and $6,235 in intangible asset amortization for the years ended December 31, 2025, and 2024, respectively, shown separately below) | 16,482 | 7,417 |
| Intangible asset amortization | 3,862 | 6,235 |
| Impairment of intangible assets | 58,710 | — |
| Operating expenses: | | |
| Research and development | 12,743 | 42,095 |
| Selling, general and administrative | 77,616 | 54,868 |
| Total operating expenses | 90,359 | 96,963 |
| Loss from operations | (62,943) | (87,003) |
| Other income (expense): | | |
| Gain on sale of PRV | 148,325 | — |
| Interest expense | (7,977) | (7,351) |
| Fair value adjustment related to warrant and CVR liability | 2,178 | 2,057 |
| Fair value adjustment related to investments | 149 | (18) |
| Interest and other income, net | 6,946 | 2,175 |
| Total other income (expense) | 149,621 | (3,137) |
| Income (loss) before income taxes | 86,678 | (90,140) |
| Income tax expense | (3,449) | (15,371) |
| Net income (loss) | $ 83,229 | $ (105,511) |
| | | |
| Net income (loss) per share of common stock: | | |
| Basic | $ 1.40 | $ (2.28) |
| Diluted | $ 1.35 | $ (2.28) |
| | | |
| Weighted-average shares of common stock outstanding: | | |
| Basic | 55,311,308 | 46,251,239 |
| Diluted | 57,262,715 | 46,251,239 |

**CONSOLIDATED BALANCE SHEETS**

**(in thousands, except share and par value amounts)**

| | December 31, 2025 | December 31, 2024 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 62,406 | $ 33,785 |
| Securities at fair value, current | 128,605 | 35,711 |
| Accounts and other receivables | 23,258 | 10,509 |
| Prepaid expenses and other current assets | 6,998 | 4,052 |
| Inventories, current | 1,740 | 1,970 |
| Total current assets | 223,007 | 86,027 |
| Securities at fair value, noncurrent | 47,879 | 6,010 |
| Inventories, noncurrent | 879 | 10,999 |
| Property and equipment, net | 489 | 356 |
| Operating lease right-of-use assets | 1,212 | 657 |
| Goodwill | 4,701 | 4,701 |
| Intangible assets, net | 6,421 | 68,993 |
| Other long-term assets | 143 | 384 |
| Total assets | $ 284,731 | $ 178,127 |
| **Liabilities and stockholders' equity** | | |
| Current liabilities: | | |
| Accounts payable and accrued expenses | $ 11,598 | $ 25,456 |
| Current portion of operating lease liabilities | 419 | 420 |
| Current portion of discount and rebate liabilities | 12,188 | 5,929 |
| Current portion of income tax payable | 13,710 | — |
| Other current liabilities | 1,362 | 2,260 |
| Total current liabilities | 39,277 | 34,065 |
| Long-term debt | 61,928 | 59,504 |
| Warrant liability | 9,575 | 17,804 |
| Income tax payable | 7,029 | 14,431 |
| Operating lease liabilities, less current portion | 859 | 372 |
| Discount and rebate liabilities, less current portion | 9,693 | 7,655 |
| Other long-term liabilities | 1,713 | 4,630 |
| Total liabilities | 130,074 | 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 December 31, 2025, or December 31, 2024 | — | — |
| Common stock, $0.0001 par value, 250,000,000 shares authorized; 58,338,319 shares issued and 56,854,781 shares outstanding as of December 31, 2025; 55,246,401 shares issued and 53,670,709 shares outstanding as of December 31, 2024 | 6 | 5 |
| Additional paid-in capital | 588,458 | 555,302 |
| Treasury stock, at cost | (10,983) | (10,983) |
| Accumulated deficit | (422,060) | (505,289) |
| Accumulated other comprehensive (loss) income | (764) | 631 |
| Total stockholders' equity | 154,657 | 39,666 |
| Total liabilities and stockholders' equity | $ 284,731 | $ 178,127 |