Zevra Therapeutics Presents Positive New Real-World Data on MIPLYFFA® in Patients with Neimann-Pick Disease Type C (NPC) at the 22nd Annual WORLDSymposium™

February 4, 2026

Real-world and clinical trial data underscore long-term safety and effectiveness in pediatric and adult NPC patients

CELEBRATION, Fla., Feb. 04, 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 announced the presentation of four posters highlighting positive new data on MIPLYFFA® (arimoclomol) for the treatment of Niemann-Pick Disease Type C (NPC) at the 22nd Annual WORLD*Symposium* ™ .

"These data highlight MIPLYFFA's potential to meaningfully stabilize disease progression across a broad spectrum of NPC patients, including adults who have historically had limited clinical data," said Adrian Quartel, M.D., FFPM, Zevra's Chief Medical Officer. "As controlled clinical trial results are reinforced by long-term real-world experience, we continue to strengthen our understanding of MIPLYFFA's impact and advance our mission to deliver meaningful therapies for people living with rare diseases."

Key Data Highlights

- *"Real-world Safety and Effectiveness of Arimoclomol in Patients with NPC: Outcomes from the U.S. Early Access Program (EAP) Over a 4-Year Period"* (Podium Presentation/Poster 41)
  - Four years of real-world data from the U.S. EAP demonstrate that arimoclomol was well tolerated and stabilized disease progression in the overall cohort, with changes in clinical severity scores remaining below thresholds for clinically meaningful worsening, supporting sustained benefit across a broad patient population, including adults.
- *"Multi-year Subgroup Analyses of Niemann-Pick Disease Type C Participants Treated with Arimoclomol in the U.S. Early Access Program"* (Poster 193)
  - Long-term real-world evidence from clinical practice, including data from participants with up to four years of follow-up, demonstrates durable treatment effects of arimoclomol and supports sustained clinical benefit with continued use over time.
- *"Efficacy of Arimoclomol Combined with Miglustat at Months 3, 6, 9, and 12 of the Double-blind, Randomized, Placebo-controlled NPC002 Trial"* (Poster 250)
  - In the post hoc efficacy analysis of the randomized, placebo-controlled NPC002 trial, arimoclomol combined with miglustat demonstrated 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.
- *"Long-term Safety and Effectiveness of Arimoclomol in Adult and Pediatric Niemann-Pick disease type C Patients in the US Early Access Program (EAP)"* (Poster 273)
  - Adult NPC patients treated with arimoclomol in the U.S. EAP showed disease stabilization over four years while maintaining a favorable safety profile. This four-year dataset provides the most robust insights to date in this understudied adult NPC population and represents the first published evidence on the impact of arimoclomol in adult NPC patients.

For more information visit the Zevra team at booths #213 and #507.

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. 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 Zevra Therapeutics, Inc.

Zevra Therapeutics, Inc. is a purpose-driven, commercial-stage company focused on bringing life-changing therapeutics to people living with rare diseases. The company's commercialization of its lead product, marketed in the U.S. for Niemann-Pick disease type C (NPC), a rare, progressive neurodegenerative disorder, provides a strong corporate foundation and validates its ability to advance therapies. In addition, the company is broadening access through geographic expansion opportunities and has a pipeline of rare disease programs. Zevra is a patient-centric organization guided by our values of accountability, integrity, innovation and courage, with the goal of creating long-term value for patients, partners, and shareholders.

For more information, please visit www.zevra.com or follow us on X and LinkedIn.

Caution 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 Company's expanded access program. 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 nine months ended September 30, 2025, filed on November 5, 2025, as well as 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.

Investor Contact

Nichol Ochsner
+1 (732) 754-2545
nochsner@zevra.com

Media Contact

Julie Downs
+1 (508) 246-3230
jdowns@zevra.com