Zevra Therapeutics to Present New Long-Term Real-World Data on MIPLYFFA® in Niemann-Pick Disease Type C (NPC) at the 2026 SSIEM Annual Symposium

August 25, 2026

Presentations from U.S. and German early access programs highlight sustained disease stabilization and a consistent safety profile across diverse NPC patient populations

BOSTON, Aug. 25, 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 announced the presentation of two posters featuring long-term real-world data on MIPLYFFA® (arimoclomol) for the treatment of Niemann-Pick disease type C (NPC) at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium.

“These presentations further expand the growing body of evidence supporting the long-term safety and effectiveness of arimoclomol in NPC,” said Caroline Hastings, MD, Professor of Pediatrics, UCSF Benioff Children’s Hospital Oakland. “The data demonstrate sustained disease stabilization across a broad range of patients, including adults, while providing valuable insight from up to eight years of real-world treatment experience.”

Key Data Highlights

- ***“Subgroup Analyses of U.S. EAP Participants with Niemann-Pick Disease Type C Treated with Arimoclomol”*** **(Poster P-290)**
  Presenter: Caroline Hastings, M.D.
  - Durable disease stabilization through 4 years: minimal changes in disease severity over time, scores remained essentially unchanged from baseline at year 1 (11.2 vs. 11.1; n=78) and year 4 (11.6 vs. 11.6; n=20).
  - Consistent stabilization across age groups: at year 3, scores remained stable in children (10.7 vs. 10.8; n=16) and adults (10.7 vs. 10.2; n=22).
  - Sustained stabilization in children: among children ages 2–19 receiving arimoclomol plus miglustat, scores remained stable at year 1 (9.7 vs. 9.1; n=27) and year 4 (10.0 vs. 10.3; n=4), consistent with results from the pivotal trial.
  - Pivotal trial showed a 65% reduction in disease progression: average disease severity scores increased by 0.76 with arimoclomol vs. 2.15 with placebo over 12 months.

- ***“Real-World Use of Arimoclomol in Niemann-Pick Type C: Findings from a German Compassionate Use Programme”*** **(Poster P-100)**
  Presenter: Dr. med. Simone Harmeling

  - Long-term treatment experience of up to 8 years: 48 people with NPC received arimoclomol and mean treatment exposure of 3.0 years; 46 (95.8%) received arimoclomol plus miglustat.
  - Broad patient population: treatment included people ranging from 2 to 63 years old, with 45.8% starting treatment as adults.
  - No discontinuations due to treatment-related side effects: 21 participants discontinued treatment, primarily due to clinical trial eligibility requirements (n=10) or death (n=5); none were due to arimoclomol-related adverse events.
  - Clinical deterioration following treatment discontinuation: some participants experienced rapid clinical deterioration after stopping arimoclomol and subsequently restarted treatment, supporting the importance of continued treatment.

Both posters will be featured during the dedicated poster walk on Wednesday, August 26, 2026, from 5:30–6:30 CEST. For more information visit the Zevra team at booth S12 in exhibition hall 5.

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 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.

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 long-term safety and effectiveness of arimoclomol in NPC, and our ability to advance therapies

from development to market and deliver long-term value. 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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