Zevra Therapeutics Announces Publication of Positive MIPLYFFA® Data from Pediatric Substudy in Infants with Niemann-Pick Disease Type C (NPC)

June 23, 2026

Data published in Molecular Genetics and Metabolism Reports suggest MIPLYFFA (arimoclomol) was well tolerated in pediatric patients with no new safety signals observed

BOSTON, June 23, 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, announced the publication of “Arimoclomol in infants with Niemann-Pick disease type C: Results from the phase 2/3 open-label pediatric substudy” in *Molecular Genetics and Metabolism Reports*. This multicenter, open-label pediatric substudy evaluated arimoclomol, in addition to concomitant miglustat, in five children with Niemann-Pick disease type C (NPC) aged 12 to <24 months at enrollment, assessing safety, tolerability, and pharmacokinetics over up to 36 months of treatment.

“The publication of these data adds to the growing body of clinical evidence supporting MIPLYFFA across age groups in NPC,” said Christine i Dali, Zevra’s Vice President, Clinical Science and an author of the publication. “Understanding the safety and pharmacokinetic profile of arimoclomol in these young children is critical as we continue to explore the potential role of earlier treatment initiation for patients living with this devastating disease.”

Arimoclomol was generally well tolerated, with no new safety signals, and pharmacokinetic results were consistent with older pediatric populations. Developmental assessments showed variable delays, reflecting underlying phenotypic heterogeneity. Although limited by small sample size, the study provides preliminary evidence supporting initiation of MIPLYFFA in this age group.

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 potential use of arimoclomol in infants with NPC. 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, 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