Zevra Therapeutics Announces Multiple Datasets on MIPLYFFA® (arimoclomol) to be Presented at the International Congress of Inborn Errors of Metabolism (ICIEM)

August 28, 2025

CELEBRATION, Fla., Aug. 28, 2025 (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 four posters on MIPLYFFA® (MY-PLY-FAH) (arimoclomol) will be presented at the International Congress of Inborn Errors of Metabolism (ICIEM), taking place September 2-6, 2025, in Kyoto, Japan.

MIPLYFFA is approved in the U.S. for the treatment of Niemann-Pick disease type C (NPC). A poster (# BP-19) detailing that MIPLYFFA's mechanism of action uniquely targets the underlying pathophysiology of NPC was selected for a Best Poster nomination. Additionally, positive, new data from a multi-center pediatric substudy in patients younger than two years old, as well as a new prespecified efficacy analysis of patients on routine clinical care with miglustat who switched from placebo to MIPLYFFA in their treatment regimen, will be presented.

Poster Details

Title: *Safety and efficacy of arimoclomol in a pediatric substudy of Niemann-Pick disease type C patients aged 6 to <24 months at study enrollment*
Presentation No: P-261
Date/Time: Wednesday, September 3, 2025; 6:00pm JST
Presenter: Laila Arash-Kaps, M.D.; SpinCS, Clinical Science for LSD

Title: *Arimoclomol upregulates expression of genes belonging to the coordinated lysosomal expression and regulation (CLEAR) network*

Best Poster Nomination

Presentation No: BP-19
Date/Time: Thursday, September 4, 2025; 5:30pm JST
Presenter: Hadeel Shammas, Ph.D.; Zevra Therapeutics

Title: *Arimoclomol for the treatment of Niemann-Pick disease type C in a real-world setting: long-term outcomes from an expanded access program in the United States*
Presentation No: P-76
Date/Time: Thursday, September 4, 2025; 5:30pm JST
Presenter: Caroline Hastings, M.D.; UCSF Benioff Children's Hospitals

Title: *Efficacy results across a 12-month double-blind randomized trial and an open-label extension phase of arimoclomol for treatment of Niemann-Pick disease type C in patients treated with miglustat*
Presentation No: P-264
Date/Time: Thursday, September 4, 2025; 5:30pm JST
Presenter: Laila Arash-Kaps, M.D.; SpinCS, Clinical Science for LSD

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. A Marketing Authorization Application for the evaluation of arimoclomol for the treatment of Niemann-Pick disease type C has been validated and is under review by the European Medicines Agency.

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 commercial-stage company combining science, data and patient need to create transformational therapies for rare diseases with limited or no treatment options. Our mission is to bring life-changing therapeutics to people living with rare diseases. With unique, data-driven development and commercialization strategies, the Company is overcoming complex drug development challenges to make new therapies available to the rare disease community.

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 promise and potential impact of our preclinical or clinical trial data; or the potential benefits of any of our products or product candidates for any specific disease or at any dosage. 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, and Zevra's Quarterly Report on Form 10-Q for the quarter ended June 30, 2025, filed on August 12, 2025, and Zevra's other filings with the SEC. 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.

Zevra Contact

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