Zevra Therapeutics Announces MIPLYFFA® (arimoclomol) Featured in Presentations at the National Niemann Pick Disease Foundation Conference

July 11, 2025

CELEBRATION, Fla., July 11, 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 an oral presentation and two poster presentations on MIPLYFFA® (MY-PLY-FAH) (arimoclomol) are being featured at the National Niemann Pick Disease Foundation (NNPDF) Conference, taking place July 10-13, 2025, in Concord, North Carolina. The presentations review data on MIPLYFFA, the first treatment approved by the U.S. Food and Drug Administration (FDA) for the treatment of Niemann-Pick disease type C (NPC). MIPLYFFA is indicated for use in combination with miglustat for the treatment of neurological manifestations of NPC in adult and pediatric patients 2 years of age and older.

"I am excited to be presenting an overview of MIPLYFFA and its role in the treatment of Niemann-Pick Disease Type C," said Barbara K. Burton, M.D., Professor of Pediatrics at Northwestern University Feinberg School of Medicine and Attending Physician in the Division of Genetics, Genomics and Metabolism at the Ann & Robert H. Lurie Children's Hospital of Chicago. "NPC is a lysosomal storage disease, and MIPLYFFA's unique mechanism improves lysosomal function, thereby addressing the underlying pathophysiology or cause of the disease. With data from a pivotal study demonstrating that MIPLYFFA, in combination with miglustat, halted disease progression at 12 months, I believe it's one of the most important therapies available for patients with NPC."

Presentation Details

Title:	*Advances in Niemann-Pick Disease Type C Treatment: The Role of Arimoclomol*
Date/Time:	Friday, July 11, 2025; 4:00pm ET
Presenter:	Barbara K. Burton, M.D., Professor of Pediatrics at Northwestern University Feinberg School of Medicine and Attending Physician in the Division of Genetics, Genomics and Metabolism at the Ann & Robert H. Lurie Children's Hospital of Chicago

Poster Details

Title:	*Long-Term Effectiveness and Safety Evaluation of Arimoclomol Treatment in Patients With Niemann-Pick Disease Type C – Data From the Pivotal Study and Open-Label Extension*
Authors:	Eugen Mengel, Sven Guenther, Lauren Hitchins, Christine í Dali
Summary:	In the pivotal trial, arimoclomol in combination with miglustat halted disease progression through 12 months compared with placebo as measured by the R4DNPCCSS. The effectiveness and safety of arimoclomol with miglustat was further confirmed in a 48-month open-label extension. Arimoclomol was generally well tolerated with no new safety signals observed during the OLE.

Title:	*Arimoclomol Upregulates Expression of Genes Belonging to the Coordinated Lysosomal Expression and Regulation (CLEAR) Network*
Authors:	Hadeel Shammas, Nikolaj Havnsøe Torp Petersen, Pontus Klein, Anja Koustrup, Marianne Terndrup Pedersen, Anne Sigaard Bie, Travis Mickle, Cathrine Kolster Fog, Thomas Kirkegaard Jensen, Sven Guenther
Summary:	The presented in vitro data provide mechanistic evidence of how arimoclomol can target NPC through multiple mechanistic pathways making it relevant in NPC. Increased translocation of the transcription factors TFE3 and TFEB from the cytosol to the nucleus is a crucial step that results in upregulation of a series of downstream processes that may improve lysosomal function and cell viability. Overall, the data support that arimoclomol does not only upregulate expression of certain CLEAR genes and specifically NPC1 at the transcriptional level, but also that this overexpression results in amplification of NPC1 protein levels and more successful NPC1 processing ultimately leading to increased cholesterol clearance from the lysosomal compartments.

	The effects of arimoclomol in mutant NPC cells found across the in vitro studies are consistent, and downstream effects expected to result from the activation of a specific process in one study could be confirmed in another study to provide an understanding of the mechanism of action of arimoclomol.

About MIPLYFFA® (arimoclomol)

MIPLYFFA (arimoclomol) is Zevra's approved therapy for use in combination with miglustat 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.

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.

Call your doctor for medical advice about side effects. You may report side effects to Zevra at 1-844-600-2237, or to the 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 Niemann-Pick Disease Type C (NPC)

Niemann-Pick disease type C (NPC) is an ultra-rare, progressive, and neurodegenerative lysosomal storage disorder characterized by an inability of the body to transport cholesterol and other lipids within the cell, leading to an accumulation of these substances in various cell types, including neurons. The disease is caused by mutations in the NPC1 or NPC2 genes, which are responsible for making the NPC1 and NPC2 lysosomal proteins. Both children and adults can be affected by NPC with varying clinical presentations. Those living with NPC can lose independence due to physical and cognitive limitations, with key neurological impairments presenting in speech, cognition, swallowing, ambulation, and fine motor skills. Disease diagnosis can often take years, with disease progression being irreversible and often leading to early mortality.

About Zevra Therapeutics, Inc.

Zevra Therapeutics, Inc. is a commercial-stage company combining science, data, and patient needs 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 upcoming events or Zevra's participation at such events. 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 March 31, 2025, filed on May 13, 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.

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