



## Zevra Therapeutics Announces MIPLYFFA® and OLPRUVA® Presentations at the Annual Meeting of the Southeastern Regional Genetics Group (SERGG)

July 18, 2025

CELEBRATION, Fla., July 18, 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, announced that three posters on MIPLYFFA® (MY-PLY-FAH) (arimoclomol) and one on OLPRUVA® (sodium phenylbutyrate) are being presented at the 42<sup>nd</sup> Annual Meeting of the Southeastern Regional Genetics Group (SERGG), taking place July 17-19, 2025, in Asheville, North Carolina. MIPLYFFA is approved in the U.S. for the treatment of Niemann-Pick disease type C (NPC). OLPRUVA is approved in the U.S. for the treatment of certain patients with urea cycle disorders (UCDs).

"We are pleased with our strong presence at this conference, alongside numerous others scheduled throughout the year -- underscoring our deep engagement with physician and patient communities and highlighting the continued excitement being driven by our data," said Adrian Quartel, M.D., FFPM, Zevra's Chief Medical Officer. "The datasets on MIPLYFFA illustrate that its distinct mechanism of action precisely targets the underlying pathology of NPC, and this disease-modifying activity has led to sustained disease stabilization in patients undergoing treatment for multiple years. For OLPRUVA, we are presenting data on the ability for it to be administered via a gastrostomy tube, as this can play an important role in the management of UCD for some patients."

### Poster Details

|          |                                                                                                                                                                                                                                                                                                                                                                                         |
|----------|-----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Title:   | <i>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</i>                                                                                                                                                                                                                    |
| Authors: | Elizabeth M. Berry-Kravis, Walla Al-Hertani, Marc Patterson, Can Ficicioglu, Loren Pena, Kristina Julich, Damara Ortiz, Paula Schleifer, Caroline Hastings, Paul Hillman, Ronan O'Reilly, Blair Orr, Daniel Gallo, Elena Buglo.                                                                                                                                                         |
| Summary: | Patients treated with MIPLYFFA in the U.S. EAP, including those with and without miglustat as a component of routine clinical care, experienced relatively stable disease through the up to 3 years of follow-up. Published natural history indicates that, on average, patients progress between ~1.0–2.0 points per year on the 5-domain Niemann-Pick type C Clinical Severity Scale. |

|          |                                                                                                                                                                                                                                                                                                       |
|----------|-------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Title:   | <i>Long-Term Efficacy and Safety Evaluation of Arimoclomol Treatment in Patients With Niemann-Pick Disease Type C – Data From a 48-Month Open-Label Trial</i>                                                                                                                                         |
| Authors: | Eugen Mengel, Marc Patterson, Sven Guenther, Christine í Dali, Elena Buglo.                                                                                                                                                                                                                           |
| Summary: | Patients were treated with MIPLYFFA in a 48-month open-label extension (OLE) study following the Phase 2/3, randomized, double-blind, placebo-controlled pivotal trial. The OLE data demonstrate that treated patients experienced a sustained reduction in disease progression for at least 5 years. |

|          |                                                                                                                                                                                                                      |
|----------|----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Title:   | <i>Arimoclomol Upregulates Expression of Genes Belonging to the Coordinated Lysosomal Expression and Regulation (CLEAR) Network</i>                                                                                  |
| 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, Elena Buglo. |
| Summary: | MIPLYFFA targets the pathophysiology of Niemann-Pick disease type C by two pathways to improve autophagy, reduce cholesterol accumulations and prevent cell death.                                                   |

|          |                                                                                                                                                                                      |
|----------|--------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Title:   | <i>Administration of a dual-coated sodium phenylbutyrate (OLPRUVA) suspension via gastrostomy tube</i>                                                                               |
| Authors: | Lauren Hitchins, Chirstopher Lauderback, Blair Orr, Adrian Quartel.                                                                                                                  |
| Summary: | Gastrostomy tube administration studies were completed with OLPRUVA showing it met the criteria with all the gastrostomy tubes at the evaluated doses and volumes of administration. |

## About SERGG

The Southeastern Regional Genetics Group (SERGG) is a non-profit 501c3 whose members include healthcare professionals involved in genetic services and newborn screening in Alabama, Florida, Georgia, Louisiana, Mississippi, North Carolina, South Carolina, and Tennessee.

One of the goals of SERGG is to address the inequities in genetic service and resources in the region and to expand existing regional capabilities and resources and to develop new regional systems to address these gaps. Another goal is to improve the existing regional communication infrastructure and to facilitate information sharing among providers of genetic services and consumers and to establish collaborative partnerships with other professional organizations.

## 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 ( $\geq 15\%$ ) 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](http://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  $\geq 15$  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 OLPRUVA®**

OLPRUVA (sodium phenylbutyrate) is Zevra's approved treatment for the treatment of certain UCDs. OLPRUVA (sodium phenylbutyrate) for oral suspension is a prescription medicine used along with certain therapies, including changes in diet, for the long-term management of adults and children weighing 44 pounds (20 kg) or greater and with a body surface area (BSA) of 1.2 m<sup>2</sup> or greater, with UCDs, involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS). OLPRUVA is not used to treat rapid increase of ammonia in the blood (acute hyperammonemia), which can be life-threatening and requires emergency medical treatment. For more information, please visit [www.OLPRUVA.com](http://www.OLPRUVA.com).

#### **Important Safety Information**

Certain medicines may increase the level of ammonia in your blood or cause serious side effects when taken during treatment with OLPRUVA. Tell your doctor about all the medicines **you or your** child take, especially if you or your child take corticosteroids, valproic acid, haloperidol, and/or probenecid.

OLPRUVA can cause serious side effects, including: 1) nervous system problems (neurotoxicity). Symptoms include sleepiness, tiredness, lightheadedness, vomiting, nausea, headache, confusion, 2) low potassium levels in your blood (hypokalemia) and 3) conditions related to swelling (edema). OLPRUVA contains salt (sodium), which can cause swelling from salt and water retention. Tell your doctor right away if you or your child get any of these symptoms. Your doctor may do certain blood tests to check for side effects during treatment with OLPRUVA. If you have certain medical conditions such as heart, liver or kidney problems, are pregnant/planning to get pregnant or breast-feeding, your doctor will decide if OLPRUVA is right for you.

The most common side effects of OLPRUVA include absent or irregular menstrual periods, decreased appetite, body odor, bad taste or avoiding foods you ate prior to getting sick (taste aversion). These are not all of the possible side effects of OLPRUVA. Call your doctor for medical advice about side effects. You may report side effects to U.S. FDA at 1-800-FDA-1088.

#### **About Zevra Therapeutics, Inc.**

Zevra Therapeutics, Inc. is a commercial-stage rare disease company combining science, data, and patient needs to create transformational therapies for 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](http://www.zevra.com) or follow us on [X](#) and [LinkedIn](#).

#### **Cautionary Note 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 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.

**Zevra Contact**

Nichol Ochsner  
+1 (732) 754-2545  
[nochsnr@zevra.com](mailto:nochsnr@zevra.com)