# Zevra Therapeutics Announces U.S. Commercial Availability of MIPLYFFA ™ (arimoclomol) for Treatment of Niemann-Pick Disease Type C

November 21, 2024

*MIPLYFFA, the first FDA-approved treatment for Niemann-Pick disease type C, is available at Zevra's specialty pharmacy for dispense*

*AmplifyAssist™, Zevra's comprehensive patient support program, in place to address access barriers*

CELEBRATION, Fla., Nov. 21, 2024 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage rare disease therapeutics company, today announced that MIPLYFFA™ (MY-PLY-FAH) (arimoclomol), the first treatment approved by the U.S. Food and Drug Administration (FDA) for the treatment of Nieman Pick disease type C (NPC), is now commercially available for dispense. 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.

"Early adoption of MIPLYFFA has exceeded our expectations, and we are pleased to report that product is now available for shipment to NPC patients. This is in line with the shorter timeframe of our guidance of eight to 12 weeks post-approval," said Josh Schafer, Chief Commercial Officer and Executive Vice President of Business Development, Zevra Therapeutics. "We continue to receive prescription enrollments through our patient services program, *AmplifyAssist*, and are supporting patients as they navigate the benefits verification process to obtain MIPLYFFA."

*AmplifyAssist* is Zevra's comprehensive patient support program designed to support the individual needs of eligible patients and those who care for them. Available resources include personalized insurance coverage education and support, copay and alternate funding identification assistance for eligible patients' product needs, disease state information and therapy management counseling, and ongoing interactions to address barriers while facilitating timely prescription refills. Information about the program is available at MIPLYFFA.com or via telephone. The AmplifyAssist team can be reached toll-free at (888) 668-4198 from 8 a.m. CT to 6 p.m. CT Monday through Friday.

Patients and caregivers should speak with their physician to get started with MIPLYFFA. Physicians can fill out an enrollment form at MIPLYFFA.com, order the prescription, and when approved, the medication will be mailed to their patient's home.

Healthcare providers and patients/caregivers should refer to the Full Prescribing Information and Instructions for Use for information on the proper administration of MIPLYFFA.

**AmplifyAssist™ —Comprehensive Support for Patients**

Zevra is committed to assisting those whose lives are affected by NPC to overcome the barriers and challenges that may impact their treatment journey. *AmplifyAssist* is Zevra's comprehensive patient support program. The mission of the program is to support the individual needs of eligible patients and those who care for them. Available resources include personalized insurance coverage education and support, copay and alternate funding identification assistance for eligible patients' product needs, disease state information and therapy management counseling, and ongoing interactions to address barriers while facilitating timely prescription refills. Information about the program is available at MIPLYFFA.com or via telephone. The AmplifyAssist team can be reached toll-free at (888) 668-4198 from 8 a.m. CT to 6 p.m. CT Monday through Friday. Healthcare providers who want to submit prescriptions can visit MIPLYFFA.com to complete the prescription enrollment form that initiates the process for accessing the treatment.

**About MIPLYFFA™ (arimoclomol)**

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 was granted Breakthrough Therapy designation, Rare Pediatric Disease designation, Orphan Drug designation, and Fast Track designation by the FDA for the treatment of NPC. MIPLYFFA was further granted 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.

**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 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 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.

Expanded access programs are made available by Zevra Therapeutics, Inc. and its affiliates and are subject to the Company's Expanded Access Program (EAP) policy, as published on its website. Participation in these programs is subject to the laws and regulations of each jurisdiction under which each respective program is operated. Eligibility for participation in any such program is

at the treating physician's discretion.

For more information, please visit www.zevra.com or follow us on X (formerly Twitter) 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 potential benefits of any of our products or product candidates for any specific disease or at any dosage; our strategic and product development objectives; prescription enrollments; our ability to support patients as they navigate the benefits verification process to obtain MIPLYFFA; and availability of and access to MIPLYFFA. 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, 2023, Zevra's quarterly report for the three and nine months ended September 30, 2024, 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.

---

**Zevra Contact**

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

**Russo Partners Contacts**

David Schull
+1 (858) 717-2310
david.schull@russopartnersllc.com

Ignacio Guerrero-Ros, Ph.D.
+1 (646) 942-5604
ignacio.guerrero-ros@russopartnersllc.com