Zevra Therapeutics Executes Distribution Agreement to Broaden Access to MIPLYFFA® for the Treatment of Niemann-Pick Disease Type C (NPC)

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CELEBRATION, Fla., Dec. 29, 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 that the Company has executed an exclusive expanded access distribution agreement with Uniphar, an Ireland-based pharmaceutical services provider with a proven record of success in global warehousing, distribution and supply chain management. The agreement enables Niemann-Pick Disease Type C (NPC) patients to access MIPLYFFA® (arimoclomol), for reimbursed named patient supply in select territories outside of Europe.

NPC is an ultra-rare, relentlessly progressive, genetic disorder that leads to premature mortality. MIPLYFFA, used in conjunction with miglustat, is the only treatment shown to halt disease progression by addressing the underlying pathology of NPC with improvement seen at the first evaluation at week 12, and durable effect for more than five years.

“There remains a clear unmet need within the rare disease community and this distribution agreement enables us to further our mission by expanding access and supporting a greater number of patients and families living with NPC,” said Neil F. McFarlane, Zevra's President and Chief Executive Officer. “By leveraging Uniphar’s leadership and infrastructure, we have an opportunity to initially address a select patient population, while continuing to prioritize our U.S. commercial launch and prepare for a potential EU approval.”

Commenting on the agreement, Brian O’Shaunnessy, Chief Commercial Officer at Uniphar, said, “Uniphar is proud to partner with Zevra to expand global access to MIPLYFFA. We believe our proven expertise in global distribution and supply chain management combined with Zevra's innovative approach to rare disease therapies will help make a life-changing difference to patients living with NPC.”

MIPLYFFA is approved by the U.S. Food and Drug Administration and is commercially available in the U.S. A Marketing Authorisation Application for the evaluation of arimoclomol for the treatment of NPC has been validated and is under review by the European Medicines Agency (EMA).

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. 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 focused on addressing unmet needs for the treatment of rare diseases. 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.

About Uniphar

Uniphar is a trusted global partner to pharma, medtech and biotech, delivering solutions that connect medicines and technologies with patients worldwide. With over 57 years of experience and partnerships with 200+ multinational clients, Uniphar combines deep expertise, infrastructure and market insight to meet the evolving needs of the life sciences sector.

Uniphar Pharma integrates Development, Clinical, Access, Medical, Commercial, Distribution and Global Sourcing to support the full product lifecycle – from early-stage research through to commercialization and beyond. Uniphar’s unified platform spans medical affairs, regulatory strategy, market access, patient engagement, commercial services, distribution and supply chain. With a team of 3,500+ across 180 countries, Uniphar bridges manufacturers, healthcare providers and patients, ensuring innovative therapies reach those who need them – driving value, access and better outcomes worldwide. From molecule to market and beyond.

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 Company’s expanded access program. 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, Quarterly Report on Form 10-Q for the three and nine months ended September 30, 2025, filed on November 5, 2025, 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.

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