Zevra Therapeutics to Present at the 21st Annual WORLDSymposium™

January 30, 2025

Zevra to receive a 2025 New Treatment Award for MIPLYFFA ™ at the 21st Annual WORLDSymposium

Eight abstracts discussing data and clinical experience associated with MIPLYFFA accepted for poster presentation, with one poster selected for oral presentation by Dr. Eugen Mengel

CELEBRATION, Fla., Jan. 30, 2025 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage rare disease therapeutics company, today announced that eight abstracts discussing data and clinical experience associated with MIPLYFFA™ (MY-PLY-FAH) (arimoclomol) have been accepted for poster presentation at the 21st Annual WORLD*Symposium* ™. One poster discussing efficacy results from a 12-month double-blind randomized trial of arimoclomol on the re-scored 4-domain Niemann-Pick disease type C (NPC) Clinical Severity Score (R4DNPCCSS) has been selected for oral presentation by Dr. Eugen Mengel, MD. 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.

In addition, the Company will receive a 2025 New Treatment Award for MIPLYFFA. The New Treatment Award recognizes important achievements in advancing the treatment of lysosomal diseases for products receiving regulatory approval.

"The recent FDA approval of MIPLYFFA underscores the persistence required to overcome regulatory challenges and to deliver treatments to people living with rare and ultra-rare diseases," said Adrian Quartel, MD, FFPM, Zevra's Chief Medical Officer. "We are honored to receive a 2025 New Treatment Award. WORLD*Symposium*'s recognition of MIPLYFFA is a testament to the unwavering devotion of physicians, researchers, patient advocacy groups, caregivers, and people living with NPC who have heroically championed MIPLYFFA."

Featured Presentation Details

Poster Number:	228
Title:	*Efficacy Results from A 12-month Double-blind Randomised Trial of Arimoclomol for Treatment of Niemann-Pick Disease Type C – Presenting A Rescored 4-domain NPC Clinical Severity Scale*
Date/Time:	Thursday, Feb. 6, 2025, 3:30 p.m. – 5:30 p.m. PT, and oral presentation on Friday, Feb. 7, 2025, 8:30 a.m. PT
Presenter:	Eugen Mengel, MD, SphinCS GmbH, Institute of Clinical Science for LSD, Hochheim, Germany

Poster Presentation Details

Poster Number:	317
Title:	*Arimoclomol Upregulates Expression of Genes Belonging to the Coordinated Lysosomal Expression and Regulation (CLEAR) Network*
Date/Time:	Wednesday, Feb. 5, 2025, 3:30 p.m. – 5:30 p.m. PT
Presenter:	Hadeel Shammas, PhD, Assistant Director of Research and Scientific Affairs, Zevra Therapeutics, Celebration, FL, USA

Poster Number:	031
Title:	*Arimoclomol for the Treatment of Niemann-pick Disease Type C In a Real-world Setting: Long-term Data from an Expanded Access Program in the United States*
Date/Time:	Thursday, Feb. 6, 2025, 3:30 p.m. – 5:30 p.m. PT
Presenter:	Elizabeth Berry-Kravis, MD, PhD, Professor of Pediatrics, Neurological Sciences, and Biochemistry at Rush University Medical Center in Chicago

Poster Number:	135

Title:	*Perseverance Is Key for Regulatory Success in Ultra-Rare Diseases – Key Learnings from Arimoclomol's Regulatory Journey*
Date/Time:	Thursday, Feb. 6, 2025, 3:30 p.m. – 5:30 p.m. PT
Presenter:	Louise Himmelstrup, MSc Pharmacy, Head of Global Regulatory Affairs, Zevra Therapeutics, Celebration, FL, USA

Poster Number:	065
Title:	*Safety of Arimoclomol in a Pediatric Sub-Study of Niemann-pick Disease Type C Patients Aged 6 To <24 Months at Study Enrollment*
Date/Time:	Thursday, Feb. 6, 2025, 3:30 p.m. – 5:30 p.m. PT
Presenter:	Christine í Dali, MD, Child Neurologist, Vice President, Clinical Science, Zevra Therapeutics, Celebration, FL, USA

Poster Number:	229
Title:	*Long-term Efficacy and Safety Evaluation of Arimoclomol Treatment in Patients with Niemann-Pick Type C – Data From 48 Months Open Label Trial*
Date/Time:	Thursday, Feb. 6, 2025, 3:30 p.m. – 5:30 p.m. PT
Presenter:	Eugen Mengel, MD, SphinCS GmbH, Institute of Clinical Science for LSD, Hochheim, Germany

Poster Number:	094
Title:	*Arimoclomol Safety Profile in the Treatment of Niemann-pick Disease Type C In a Real-world Setting: Long-term Safety Data from an Expanded Access Program in the United States*
Date/Time:	Thursday, Feb. 6, 2025, 3:30 p.m. – 5:30 p.m. PT
Presenter:	Can Ficicioglu, MD, PhD, Director of the Newborn Metabolic Screening Program and the Lysosomal Storage Diseases Program, and Clinical Director of the Metabolic Disease Program at Children's Hospital of Philadelphia.

Poster Number:	032
Title:	*Qualitative Assessment of the Validity and Standardization of the Swallow Domain in the 5-domain Niemann-Pick Disease Type C (NPC) Clinical Severity Scale (5DNPCCSS) And Analysis in an NPC Clinical Trial Data Set*
Date/Time:	Thursday, Feb. 6, 2025, 3:30 p.m. – 5:30 p.m. PT
Presenter:	Elizabeth Berry-Kravis, MD, PhD, Professor of Pediatrics, Neurological Sciences, and Biochemistry at Rush University Medical Center in Chicago

E-Posters will be available to all registered attendees via the WORLD*Symposium* mobile app beginning at 05:00 PST on Tuesday, Feb. 4, 2025, and will remain accessible throughout the live meeting. On-Demand registered attendees can access e-Posters from Feb. 12 to Mar. 14, 2025.

Members of Zevra's team will be available at the meeting which takes place Feb. 3-7, 2025, in San Diego, CA; attendees are invited to visit Zevra at its commercial and medical booth (#206).

About the 21st Annual WORLD*Symposium* ™

WORLD*Symposium* is designed for basic, translational and clinical researchers, patient advocacy groups, clinicians, and all others who are interested in learning more about the latest discoveries related to lysosomal diseases and the clinical investigation of these advances. Each year, WORLD*Symposium* presents the latest information from basic science, translational research, and clinical trials for lysosomal diseases.

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.

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