Zevra Therapeutics Presents New Data on the Long-Term Safety and Efficacy of Arimoclomol as a Treatment for Niemann-Pick Disease Type C at the SIMD 45th Annual Meeting

April 15, 2024

The oral presentation highlighted data from Zevra's Expanded Access Program demonstrating a consistent safety profile

CELEBRATION, Fla., April 15, 2024 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a rare disease therapeutics company, today announced that new long-term real-world data from the arimoclomol expanded access program (EAP: NCT04316637) for the treatment of Niemann-Pick disease type C (NPC) was featured in an oral presentation during the Society for Inherited Metabolic Disorders (SIMD) 45th Annual Meeting taking place at the Sheraton/Le Meridien Charlotte Hotel Complex in Charlotte, NC, from April 14-17, 2024.

"These real-world data collected in the arimoclomol US EAP represent the first evidence to support the use of arimoclomol in adults with NPC, demonstrating a clinically meaningful slowing of disease progression," noted Kristina Julich, M.D., Assistant Professor, Department of Neurology, at The University of Texas at Austin, Dell Medical School. "NPC presents a therapeutic challenge, lacking approved treatment options in the U.S. and invariably leading to progressive loss of independence due to physical and cognitive impairments. We are very pleased with the positive new data that brings hope for a community with a high unmet medical need."

"These observed long-term clinical benefits add to the body of evidence for arimoclomol that supports its tolerability and effectiveness in the treatment of NPC, bringing us one step closer to helping patients suffering from this relentless and fatal disease," remarked Neil F. McFarlane, President and Chief Executive Officer of Zevra. "We remain committed to continue collaborating closely with the FDA as it reviews arimoclomol's NDA ahead of our PDUFA date on September 21, 2024."

Highlights from the Presentation:

The results were presented by Kristina Julich, M.D., Chief, Neurogenetics Center, and Assistant Professor of Pediatric Neurosciences at Dell Medical School from The University of Texas.

The first patient in the EAP was enrolled in June 2020. As of July 19, 2023, 41 participants from 11 U.S. centers were ≥18 years at arimoclomol initiation; 26 had baseline Physician-reported 5-domain NPC Clinical Severity Scale (5DNPCCS) assessments with ≥1 year of follow-up. Among these 26 adults, mean (standard deviation, SD) age at NPC diagnosis and arimoclomol initiation was 23.7 (9.0) and 28.5 (6.5) years, respectively. Patients continued arimoclomol treatment for a mean of 21 months (range: 12-32) and 69% (18/26) had recorded miglustat use. Adults treated with arimoclomol, including those with and without miglustat use, generally had a stable disease course over two years of treatment and follow-up and the safety profile was consistent with that observed in the Phase 2/3 study where no new safety adverse events were identified.

In addition to this scientific presentation, Marc C. Patterson, MD, Professor of Pediatrics at the Mayo Clinic College of Medicine and Science, also presented the data at the 2024 American Academy of Neurology Annual Meeting (AANAM), during the Society Spotlight: Child Neurology Society session. Dr. Patterson's talk was entitled "Evaluation of the Long-Term Effect of Arimoclomol in NPC - 48 Months Data from CT-ORZY-NPC-002."

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 tissue areas, including brain tissue. The disease is caused by mutations in the NPC1 or NPC2 genes, which are responsible for making lysosomal proteins. Both children and adults can be affected by NPC with varying clinical presentations. Those living with NPC lose independence due to physical and cognitive limitations, with key neurological impairments presenting in speech, cognition, swallowing, ambulation, and fine motor skills. Disease progression is irreversible and can be fatal within months or take years to be diagnosed and advance in severity.

About Arimoclomol

Arimoclomol, Zevra's orally delivered, first-in-class investigational product candidate for the treatment of NPC, has been granted Orphan Drug designation, Fast Track designation, Breakthrough Therapy designation, and Rare Pediatric Disease designation by the FDA, and Orphan Medicinal Product designation for the treatment of NPC by the European Medicines Agency (EMA). The FDA has accepted the resubmission of the NDA for arimoclomol and has set a user fee goal date (PDUFA date) of September 21,

2024.

About Zevra Therapeutics

Zevra Therapeutics is a 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 and its affiliates and are subject to the Company's Expanded Access Program (EAP) policy as published on its website at www.zevra.com. 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 presentation of data at conferences, the promise and potential impact of our preclinical or clinical trial data, the initiation, timing and results of any clinical trials or readouts, the content, information used for, timing or results of any NDA submissions or resubmissions for arimoclomol or any other product candidates for any specific disease indication or at any dosage, the potential launch or commercialization of any of product candidates or products, and our strategic and product development objectives, including with respect to becoming a leading, commercially focused rare disease company. 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, 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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