Zevra Therapeutics to Present at the 52nd Child Neurology Society Annual Meeting

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Presentation to focus on the arimoclomol program for the treatment of Niemann-Pick Disease type C and related early access programs

CELEBRATION, Fla., Oct. 05, 2023 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a rare disease therapeutics company, today announced that it will present data related to clinical study outcomes and its early access programs via both oral and poster presentations at the upcoming 52nd Child Neurology Society (CNS) Annual Meeting. This meeting will be held October 4-7, 2023, in Vancouver, Canada. The presentations will focus on arimoclomol, Zevra's late-stage pipeline program in development for the treatment of Niemann-Pick Disease type C (NPC), including 48 months of efficacy and safety data captured through the double-blind and open-label extension phases of the registrational trial, as well as methodology related to establishing an early access program with real-world data collection capabilities.

"Our team is eager to highlight our arimoclomol program at the 52 nd CNS Annual Meeting and engage with the key child neurologists who are treating devastating conditions like NPC where few therapeutic options exist," said Daniel Gallo, PhD, Senior Vice President of Medical Affairs and Advocacy for Zevra. "The arimoclomol data demonstrate the importance of understanding the long-term efficacy and treatment approach for this potential new therapy for NPC."

Oral Presentation Details:

Title:	Evaluation of the Long-Term Effect of Arimoclomol in NPC – 48 Months Data from CT-ORZY-NPC-002
Presenter:	Marc Patterson, MD, Professor of Neurology, Pediatrics, and Medical Genetics, Mayo Clinic College of Medicine and Science, Rochester, MN
Session:	Platform 3
Time:	Friday, October 6th at 2:15 - 4:00 p.m.
Location:	Ballroom BC

Poster Presentation Details:

Title:	Real World Data Collection in Niemann-Pick Disease Type C – Data from Expanded Access Program with Arimoclomol
Presenter:	Elizabeth Berry Kravis, MD, PhD, Professor of Pediatrics, Neurological Sciences, and Biochemistry, Rush Medical College, Rush University Medical Center, Chicago, IL
Poster:	#100
Poster Review:	Thursday, Oct 5, 2023, at 12:30 - 2:00 p.m. and 5:30 - 7:30 p.m.

The Zevra team will also be on site throughout the event and invites attendees to connect at Booth #518 to learn more about how Zevra is supporting the child neurology community.

About Niemann-Pick disease type C (NPC):

Niemann-Pick disease type C (NPC) is an ultra-rare and progressive, 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 and is an autosomal recessive trait. 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 for NPC by the FDA, and orphan medicinal product designation for the treatment of NPC by the European Medicines Agency (EMA). The arimoclomol NDA is currently being prepared for resubmission to the FDA.

About Zevra Therapeutics:

Zevra Therapeutics is a rare disease company melding science, data, and patient need to create transformational therapies for diseases with limited or no treatment options. With unique, data-driven clinical, regulatory, and commercialization strategies, the Company is overcoming complex drug development challenges to bring much-needed therapies to patients. With both regulatory and clinical stage product candidates, the Company is building its commercial capability to make new therapies available to the rare disease community.

Early access programs are made available by Zevra Therapeutics and its affiliates and are subject to the Company's Early Access Program (EAP) policy as published on its website at 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.

Caution 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 upcoming events or Zevra's participation at such events. 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, 2022, as updated in Zevra's Quarterly Report on Form 10-Q for the quarter ended June 30, 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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