Zevra Therapeutics Celebrates and Supports Global Niemann-Pick Disease Awareness Day on October 19th and Niemann-Pick Disease Awareness Month Throughout October

October 19, 2023

CELEBRATION, Fla., Oct. 19, 2023 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA), a rare disease therapeutics company, proudly supports the Niemann-Pick community on Global Niemann-Pick Disease Awareness Day and throughout Niemann-Pick Disease Awareness Month. Patient organizations like the International Niemann-Pick Disease Alliance (INPDA) and the National Niemann-Pick Disease Foundation (NNPDF) provide essential support for people living with Niemann-Pick disease type C (NPC) and their families and advocate for needed treatments.

“So many members of the advocacy community around the world work tirelessly to support people who live with NPC and raise awareness of this devastating disease. At NNPDF, we welcome the opportunities to collaborate with industry partners like Zevra that are advancing promising research that can lead to new treatments that drive change and hope for the NPC community,” said Joslyn Crowe, Executive Director at NNPDF and Vice President of INPDA. “Throughout the year and especially during October where we observe Global Niemann-Pick Awareness Day and Niemann-Pick Awareness Month, we join with leaders from industry and our colleagues from the International Niemann-Pick Disease Alliance to help others understand the challenges associated with the condition and the future potential to provide needed relief to patients and families.”

“NPC remains a condition with no approved treatment options in the U.S.,” said Daniel Gallo, Ph.D., Senior Vice President of Medical Affairs and Advocacy at Zevra. “It is an ultra-rare, genetic, relentlessly progressive neurodegenerative disease impacting approximately 1 in 100,000 live births. At Zevra we are focused on working closely with the advocacy community for the benefit of people living with NPC and their families.”

Zevra remains committed to working with INPDA, NNPDF and the full NPC community to advance therapeutic options for this rare, neurodegenerative disease and devastating condition. Zevra also remains committed to patients receiving access to the Company’s investigational treatment, arimoclomol, through the ongoing Expanded Access Program (EAP), which is currently active in the United States and other European countries.

About Niemann-Pick Disease Type C (NPC):
Niemann-Pick disease type C (NPC) is an ultra-rare, 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. 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 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.

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 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, and which can be identified by the use of words such as "may," "will," "expect," "project," "estimate," "anticipate," "plan," "believe," "potential," "should," "continue," "could," "intend," "target," "predict," or the negative versions of those words or other comparable words or expressions, although not all forward-looking statements contain these identifying words or expressions. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements include without limitation statements regarding senior leadership and board member transitions and refreshment, or the timing thereof, and our strategic and product development objectives, the potential sale of the Priority Review Voucher, the promise and potential impact of our preclinical or clinical trial data, including without limitation the initiation, timing and results of any clinical trials or readouts, the content, information used for, timing or results of any IND applications and NDA submissions or resubmissions for arimoclomol, KP1077, or any other product candidates for any specific disease indication or at any dosage, the potential achievement of commercial sales or revenue milestones for AZSTARYS and the timing thereof, the sufficiency of our cash, cash equivalents and investments to fund our operating activities for any specific period of time, 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, 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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