Zevra Therapeutics to Participate in the 2023 NNPDF Family Support and Medical Conference

July 19, 2023

CELEBRATION, Fla., July 19, 2023 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company, formerly KemPharm, Inc.), a rare disease therapeutics company, today announced their support of the upcoming National Niemann-Pick Disease Foundation's (NNPDF) Family Support and Medical Conference. Members of the Zevra team will attend this event which is being held in Orlando, Florida, from July 20 through July 22, 2023.

In addition to providing a corporate update, Zevra plans to lead a family working group session to gain insights from caregivers on important topics facing the rare disease community. Two research posters will also be presented during the event, with a focus on previously published information regarding the validity of the 5-Domain NPC Clinical Severity Scale (5D NPCCSS) as a clinical outcomes assessment measure, and the correlation between the 5D NPCCSS and corresponding items of the Scale for the Assessment and Rating of Ataxia (SARA).

"NNPDF's Family Support and Medical Conference is a significant event for the NPC community that brings together patients, caregivers, clinicians and researchers to promote much needed awareness, understanding and information about services and support available and education about potential treatments for this devastating disease," said Christal Mickle, President, interim Chief Executive Officer and Chief Development Officer of Zevra. "The Zevra team is proud to sponsor the NNPDF and their work supporting the patients and their families who continue to inspire us with their strength and hope. We look forward to being a part of this year's event."

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 age of onset, rate of progression and presents differently in each person. 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 Niemann-Pick disease type C (NPC), has been granted orphan drug designation, Fast Track designation, Breakthrough Therapy designation and rare pediatric disease designation for NPC by the U.S. Food and Drug Administration (FDA), and orphan medicinal product designation for the treatment of NPC by the European Medicines Agency (EMA). The arimoclomol New Drug Application (NDA) is currently being prepared for a resubmission to the FDA.

About Zevra:

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, Inc. 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 March 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.

Contacts:

Nichol Ochsner
+1 (732) 754-2545
nochsner@zevra.com

Janine Bogris
+1 (201) 245-6838
janine.bogris@evokegroup.com

ZEVRA THERAPEUTICS