Zevra Therapeutics Announces Two Abstracts Accepted for Presentation at the 19th Annual WORLDSymposium™ 2023

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CELEBRATION, Fla., Feb. 23, 2023 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: KMPH) (Zevra, or the Company, formerly KemPharm, Inc.), a rare disease therapeutics company, has announced that two abstracts involving clinical research of arimoclomol for the treatment of Niemann-Pick disease type C (NPC), including advancing understanding of NPC disease progression have been accepted for poster presentations at the 19th Annual WORLD*Symposium* ™ 2023, an annual research conference dedicated to lysosomal diseases. WORLD*Symposium* 2023 is held February 22-26, 2023, in Orlando, Florida.

Arimoclomol is an orally-delivered, first-in-class investigational product candidate being developed as a treatment for NPC, a rare neurodegenerative lysosomal disease characterized by an inability of the body to transport cholesterol and lipids inside of cells. Arimoclomol has been studied in ten Phase 1, four Phase 2, and three pivotal Phase 2/3 trials. Zevra is currently preparing an updated New Drug Application (NDA) for arimoclomol as a treatment for NPC, which the Company expects to file as early as the third quarter of 2023.

Details of the presentations are as follows:

Poster Number:	277
Title:	Evaluation of the long-term effect of arimoclomol in NPC
Poster Session:	Friday, February 24, 2023, 4:00 – 5:00 PM, ET
Presenter:	Marc Patterson, MD, Professor of Neurology, Pediatrics, and Medical Genetics, Mayo Clinic Children's Center in Rochester, MN

Poster Number:	83
Title:	Association between NPC severity score domains and corresponding items of the performance-based Scale for the Assessment and Rating of Ataxia (SARA)
Poster Session:	Saturday, February 25, 2023, 3:00 – 4:00 PM, ET
Presenter:	Christine í Dali, MD, Child Neurologist, Chief Medical Officer, Zevra Therapeutics (formerly KemPharm), Celebration, FL, USA

Additional information regarding the WORLD*Symposium* presentations can be found at: https://worldsymposia.org/.

Along with the new name, Zevra Therapeutics, the Company has recently adopted a new logo and launched a new corporate website. Visit www.zevra.com to learn more.

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.

Arimoclomol, Zevra's orally-delivered, first-in-class investigational product candidate for the treatment of Neimann-Pick type C ("NPC"), has been granted orphan drug designation, Fast Track designation and rare pediatric disease designation for NPC by the US Food and Drug Administration ("FDA") and the European Medicines Agency ("EMA").

KP1077 is Zevra's lead clinical candidate being developed to treat idiopathic hypersomnia ("IH") and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate ("SDX"), Zevra's proprietary prodrug of d-methylphenidate ("d-MPH"). The FDA has granted KP1077 orphan drug designation for the treatment of IH, and the US Drug Enforcement Agency ("DEA") has classified SDX as a Schedule IV controlled substance based on evidence suggesting SDX has a lower potential for abuse when compared to d-MPH, a Schedule II controlled substance.

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 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 statements regarding: 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 timing or results of any Investigational New Drug (IND) applications and New Drug Application (NDA) submissions for arimoclomol or any other product candidates for any specific disease indication or at any dosage, and our strategic and product development objectives. These forward-looking statements are based on information currently available to Zevra and its current plans or expectations and are subject to a number of 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 (formerly KemPharm's) Annual

Report on Form 10-K for the year ended December 31, 2021, as updated by Zevra's (formerly KemPharm's) Quarterly Report on Form 10-Q for the three months ended September 30, 2022, and Zevra's (formerly KemPharm'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 can give no assurance that such expectations will prove to be correct. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

Contacts:

Nichol Ochsner
nochsner@zevra.com

Jennifer Arcure
+1 (917) 603-0681
Jennifer.arcure@evokegroup.com

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