Zevra Therapeutics Announces Arimoclomol Research Featured in Two Poster Presentations at the 19th Annual WORLDSymposium™ 2023

February 24, 2023

Interim data analysis of four-year, open-label extension from Phase 2/3 clinical trial suggests arimoclomol may reduce long-term progression of Niemann-Pick Disease Type C (NPC)

CELEBRATION, Fla., Feb. 24, 2023 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: KMPH) ("Zevra" or the "Company," formerly known as KemPharm, Inc.), today announced that arimoclomol, the Company's orally-delivered, first-in-class investigational product candidate being developed as a treatment for Niemann-Pick disease type C (NPC), is being featured in two poster presentations during the 19th Annual WORLD*Symposium* ™ 2023, the annual research conference dedicated to lysosomal diseases being held February 22-26, 2023 in Orlando, Florida, USA.

Research presented at WORLD*Symposium* ™ 2023 included an interim analysis from the ongoing four-year open-label extension of the Phase 2/3 clinical trial of arimoclomol. Results from this analysis, based on up to four years of continuous treatment, suggest that arimoclomol may reduce the long-term progression of NPC. Zevra plans to include these data as part of the updated New Drug Application (NDA) for arimoclomol, which the Company expects to resubmit to the U.S. Food and Drug Administration (FDA) as early as the third quarter of 2023.

"We are pleased to share this important research of arimoclomol at WORLD *Symposium* ™ 2023, which expands our understanding of the potential for arimoclomol to decrease the long-term progression of NPC," said Travis C. Mickle, Ph.D., President of Zevra. "We are particularly encouraged by the top-line efficacy data from the four-year open label study of arimoclomol given the significant need to develop improved therapies to treat NPC, a devastating lysosomal disease characterized by progressive impairment of mobility, cognition, speech and swallowing before a premature death. We look forward to including these data which further characterize the potential clinical benefit of arimoclomol in NPC, along with a 48-month safety analysis, in our updated NDA for arimoclomol."

"I am encouraged that the extension studies have provided further data supporting a beneficial effect of arimoclomol in patients with Niemann-Pick disease, type C, for whom safe and effective therapies are urgently needed," said Marc C. Patterson, M.D., Neurology, Pediatrics and Medical Genetics, Mayo Clinic, Rochester, Minnesota, USA.

The first poster (#277), titled, "Evaluation of the Long-Term Effect of Arimoclomol in NPC," reported 48-month data from the open-label extension (OLE) of the Phase 2/3 clinical trial of arimoclomol in NPC (NPC-002). Upon fulfilling the randomized double-blinded portion of the phase 2/3 clinical trial, both placebo- and arimoclomol-treated patients were given the option to continue into the four-year (48 month) OLE phase of the study with arimoclomol treatment provided in addition to their current standard of care. Progression of NPC disease through the DB and OLE phases was assessed utilizing the five-domain NPC Clinical Severity Scale (5DNPCCSS) and compared with an estimated progression calculated from the combination of untreated patients from the NPC-001 observational trial and placebo patients from the NPC-002 Phase 2/3 trial. As presented at WORLD*Symposium* ™ 2023, results from the data analysis indicated that long-term progression of NPC may be reduced in patients treated with arimoclomol. There are no approved treatments for NPC in the US currently; miglustat is approved for the treatment of NPC in the EU.

The second poster (#83), titled, "Association Between NPC Severity Score Domains and Corresponding Items of the Performance-based Scale for the Assessment and Rating of Ataxia (SARA)," reported research investigating correlations between relevant 5DNPCCSS domains and corresponding SARA test items to potentially provide further supportive evidence for 5DNPCCSS validity as a tool for evaluating NPC progression. The SARA test evaluates impairment related to cerebellar ataxia, which was a secondary endpoint in the Phase 2/3 clinical trial of arimoclomol in NPC (NPC progression based on the 5DNPCCSS was the primary endpoint). Based on a comparative analysis of both measurements, it was determined that individual 5DNPCCSS domains and relevant performance-based SARA test items showed strong associations and alignment between the two instruments for all analysis methods used. These results provide further support that the evaluated 5DNPCCSS domains are appropriately standardized to allow for reliable and reproducible scoring of disease severity in NPC. This analysis is also intended to be included in the NDA resubmission.

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

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 the treatment of 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").

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 discretion of the treating physician.

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 potential of arimoclomol to reduce the long-term progression of NPC, the timing, content 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, our participation in and the presentation of our product candidates at research conferences, 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) Annual Report on Form 10-K for the year ended December 31, 2021, as updated by Zevra's (formerly KemPharm) Quarterly Report on Form 10-Q for the three months ended September 30, 2022, and Zevra's (formerly KemPharm) 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.

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