Zevra Therapeutics Announces Submission of IND for KP1077 in Narcolepsy

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Zevra seeks to expand KP1077 clinical program to address multiple rare sleep disorders

CELEBRATION, Fla., April 04, 2023 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) ("Zevra" or the "Company" and formerly KemPharm, Inc.), a rare disease therapeutics company, today announced the submission of an Investigational New Drug (IND) application seeking authorization from the U.S. Food and Drug Administration ("FDA") to begin a Phase 1 clinical trial of KP1077 in narcolepsy. Once the clinical investigation plan proposed in the IND has been cleared to proceed by the FDA, Zevra plans to initiate its first of several Phase 1 clinical trials of KP1077 as early as the second quarter of 2023.

KP1077 is also currently being evaluated in a Phase 2 clinical trial for the treatment of idiopathic hypersomnia ("IH"). Those data have the potential to support not only the advancement of KP1077 into a pivotal Phase 3 study in IH, but also a Phase 3 trial in narcolepsy.

"Our research of KP1077 in IH laid the groundwork for us to expand our clinical studies to examine its efficacy in narcolepsy, a chronic neurological sleep disorder," said Richard W. Pascoe, Chief Executive Officer of Zevra. "We are committed to advancing therapies for rare disorders, and our work to address narcolepsy and the treatment of excessive daytime sleepiness with KP1077 is an essential part of our commitment. Expanding existing trials in ways that support the advancement of therapies that the rare disease community needs is the core of Zevra's mission."

Zevra filed an IND application in May 2022 for the treatment of IH with KP1077 and subsequently initiated a Phase 2 IH study in December 2022 (NCT05668754). During a pre-IND meeting with the FDA, Zevra received confirmation that additional non-clinical studies were not needed to advance KP1077 into clinical development due to the abundance of data already available on serdexmethylphenidate ("SDX"). SDX is the sole active pharmaceutical ingredient in KP1077.

To learn more about Zevra Therapeutics, visit zevra.com.

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 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").

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 U.S. 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, 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, expansion or 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 KP1077 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, 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.

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