Zevra Therapeutics Announces FDA Acceptance of IND Application for KP1077 in Narcolepsy

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Zevra plans to expand KP1077 clinical program to address multiple rare sleep disorders with trials initiating in 2023

CELEBRATION, Fla., May 03, 2023 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) ("Zevra" or the "Company" and formerly KemPharm, Inc.), a rare disease therapeutics company, today announced the acceptance of an Investigational New Drug ("IND") application by the U.S. Food and Drug Administration ("FDA") to begin a Phase 1 clinical trial of KP1077 in narcolepsy which is expected to provide additional information regarding the optimal dosing regimen. Zevra plans to initiate the first of several Phase 1 clinical trials of KP1077 in narcolepsy this year. These trials will support both the narcolepsy and idiopathic hypersomnia ("IH") clinical development programs. Serdexmethylphenidate ("SDX") is the sole active pharmaceutical ingredient in KP1077.

"The FDA's acceptance of KP1077's IND for narcolepsy is an important step forward in the advancement of this novel therapy, creating a meaningful opportunity to expand our development efforts into this high-need indication," said Christal M.M. Mickle, Chief Product Development Officer of Zevra. "We have conducted extensive research with SDX, and we believe that KP1077 has the potential to effectively treat both IH and narcolepsy types 1 and 2 with a differentiated safety profile."

Zevra filed the IND for KP1077 for the treatment of narcolepsy in March 2023. Zevra also 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). Data from that study have the potential to support the advancement of KP1077 into a pivotal Phase 3 study in IH, as well as provide support in determining whether to initiate a Phase 3 trial in narcolepsy. During a related pre-IND meeting with the FDA, Zevra received confirmation that additional non-clinical studies were not needed to advance KP1077 for the treatment of idiopathic hypersomnia into clinical development due to the abundance of data already available on serdexmethylphenidate .

About Narcolepsy
Narcolepsy is a chronic debilitating central disorder of hypersomnolence. The primary symptom of narcolepsy is excessive daytime sleepiness characterized by daily episodes of an irrepressible need to sleep or daytime lapses into sleep. People with narcolepsy have abnormal rapid eye movement (REM) sleep which can cause disrupted nighttime sleep, sleep paralysis and sleep-related hallucinations around sleep-wake transitions. Narcolepsy has severe personal, social, and economic consequences. People with narcolepsy experience substantial impairment of their mental and physical wellbeing, and depression and anxiety are common. Cognitive dysfunction, such as difficulty with focus, concentration and memory (also referred to as 'brain fog') is often reported. All of which contribute to overall disease burden and poor quality of life in people with narcolepsy.

Narcolepsy is categorized as narcolepsy type 1 (NT1) and type 2 (NT2). NT1 is considered a distinct entity characterized by loss of hypocretin neurons. When narcolepsy presents without cataplexy and CSF hypocretin-1 concentration that do not meet the type 1 criterion it is categorized as NT2.
The worldwide prevalence for both types of narcolepsy together has been estimated to be approximately 25 to 50 per 100,000 people. Epidemiological studies using well-defined criteria for estimating the prevalence of narcolepsy (both NT1 and NT2) in the United States show incidence rates ranging from 31 to 79 per 100,000 people, suggesting that there may be from 100,000 to 260,000 Americans with the disease.

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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