KemPharm Partners with the Hypersomnia Foundation to Support Sleep Disorder Research and Advocacy

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KemPharm advancing multicenter Phase 2 clinical trial investigating the efficacy and safety of KP1077 for the treatment of idiopathic hypersomnia (IH)

CELEBRATION, Fla., Jan. 18, 2023 (GLOBE NEWSWIRE) -- KemPharm, Inc. (NasdaqGS: KMPH) (KemPharm, or the Company), a rare disease therapeutics company focused on the development of treatments for rare central nervous system (CNS) disorders, neurodegenerative diseases, lysosomal storage disorders and related treatment areas, announced its partnership with the Hypersomnia Foundation, which engages, informs and champions the global community to improve the lives of people with idiopathic hypersomnia (IH) and related sleep disorders.

IH is a chronic neurologic disorder marked by significant detrimental effects on nighttime sleep as well as daytime sleepiness/wakefulness. It is estimated that approximately 37,000 patients are currently diagnosed with IH and seeking treatment, although the total population may be much larger due to patients not seeking treatment or having not been diagnosed.

KemPharm is currently investigating KP1077 in a double-blind, placebo-controlled, randomized-withdrawal, dose-optimizing, multi-center Phase 2 clinical trial evaluating the efficacy and safety of KP1077 for the treatment of IH. KemPharm expects to enroll approximately 48 adult patients with IH in more than 30 centers in the United States. Additional details about the study, how to enroll, and available study sites can be found on www.clinicaltrials.gov (NCT05668754).

"We are pleased to be working in coordination with the Hypersomnia Foundation to increase awareness and knowledge of idiopathic hypersomnia for patients, caregivers and clinicians to better understand the condition and advance research, with the ultimate goal of faster diagnosis and improved outcomes," said Richard W. Pascoe, Chief Executive Officer of KemPharm. "IH is a rare sleep disorder for which few treatment options exist. We believe KP1077 has the potential to address the most debilitating symptoms of IH, and we look forward to advancing the recently initiated Phase 2 clinical trial of KP1077 in IH with support from patient groups, including the Hypersomnia Foundation."

"We are deeply grateful for the work KemPharm is doing to advance treatments for our community at such a time when pharmacological options for people with IH are so desperately lacking," said Claire Crisp, MFA, Chief Executive officer of the Hypersomnia Foundation. "Support from sponsors like KemPharm are key to our efforts to advocate for and support those dealing with debilitating sleep disorders."

About the Hypersomnia Foundation:

The Hypersomnia Foundation provides information in the hypersomnia community, increases awareness and knowledge of hypersomnias in order to reduce time to diagnosis and fosters relationships between the patient community and scientific, industrial and government organizations. The foundation also supports the discovery of scientific breakthroughs on central hypersomnias and translates new discoveries into improved outcomes and quality of life for people with hypersomnias. For more information, please visit www.hypersomniafoundation.org.

About KP1077:

KP1077 is KemPharm's lead clinical candidate being developed as a treatment for idiopathic hypersomnia (IH) and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate (SDX), KemPharm's proprietary prodrug of d-methylphenidate. SDX has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) 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.

About the Phase 2 Clinical Trial Investigating KP1077 in IH:

KemPharm is currently advancing a multicenter, dose-optimizing, double-blind, placebo-controlled, randomized-withdrawal Phase 2 clinical trial to evaluate safety and efficacy of KP1077 as a treatment for IH. KemPharm is seeking to enroll approximately 48 adult patients with IH in more than 30 centers in the United States. Part 1 of the trial will consist of a five-week open-label titration phase during which patients will be optimized to one of four doses of SDX (80, 160, 240, or 320 mg/day). Part 2 of the trial will entail a two-week randomized, double-blind, withdrawal phase, during which two-thirds of the trial participants will continue to receive their optimized dose while the remaining one-third will receive placebo. Participants will be further assigned into two evenly divided cohorts. The first cohort will receive a single daily dose just before bedtime, and the second cohort will receive half the daily dose shortly after awakening and half the daily dose prior to bedtime.

The primary endpoint is the safety and tolerability of SDX. The major secondary efficacy endpoint is the change in Epworth Sleepiness Scale (ESS) total score. Additional exploratory endpoints include the Patient Global Impression of Severity (PGI-S), the Clinical Global Impression of Severity (CGI-S), change in total score on the Idiopathic Hypersomnia Severity Scale (IHSS), and a new scale to assess the symptoms and severity of "Brain Fog".

Additional details about the study, how to enroll and available study sites can be found on www.clinicaltrials.gov (NCT05668754).

About KemPharm:

KemPharm is a rare disease therapeutics company focused on the discovery, development and commercialization of novel treatments for rare CNS and neurodegenerative diseases, lysosomal storage disorders and related treatment areas. KemPharm has a diverse product portfolio, combining a clinical-stage development pipeline with NDA-stage and commercial assets. The pipeline includes arimoclomol, an orally-delivered, first-in-class investigational product candidate for Niemann-Pick disease type C (NPC), and KP1077, which the Company is developing as a treatment for idiopathic hypersomnia (IH), a rare neurological sleep disorder, and narcolepsy. In addition, the U.S. Food and Drug Administration (FDA) has approved AZSTARYS®, a once-daily treatment for ADHD in patients age six years and older containing KemPharm's prodrug, serdexmethylphenidate (SDX), which is being commercialized by Corium, Inc. in the U.S. The FDA has also approved APADAZ®, an immediate-release combination product containing benzhydrocodone, KemPharm's prodrug of hydrocodone, and acetaminophen, which is being commercialized by KVK-Tech, Inc. in the U.S. For more information on KemPharm and its pipeline of product candidates, visit www.kempharm.com or connect with us on Twitter, LinkedIn, Facebook and YouTube.

Early access programs are made available by KemPharm, Inc. and its affiliates, and are subject to the Company's Early Access Program (EAP) policy as published on its website at www.kempharm.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 timing or results of any Investigational New Drug applications and New Drug Application (NDA) submissions, KP1077, SDX, or any other product candidates for any specific disease indication or at any dosage, the potential benefits of any of KemPharm's product candidates, and our strategic and product development objectives. These forward-looking statements are based on information currently available to KemPharm 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 KemPharm's Annual Report on Form 10-K for the year ended December 31, 2021, as updated by KemPharm's Quarterly Report on Form 10-Q for the three months ended September 30, 2022, and 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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