KemPharm Receives FDA Orphan Drug Designation for Serdexmethylphenidate (SDX) for the Treatment of Idiopathic Hypersomnia (IH)

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Phase 2 clinical trial investigating KP1077 in patients with IH expected to initiate prior to year-end 2022

CELEBRATION, Fla., Nov. 18, 2022 (GLOBE NEWSWIRE) -- KemPharm, Inc. (NasdaqGS: KMPH) (KemPharm, or the Company), a biotechnology company focused on the discovery, development and commercialization of novel treatments for rare central nervous system (CNS) and neurodegenerative diseases, lysosomal storage disorders and related treatment areas, today announced that the U.S. Food and Drug Administration (FDA) has granted the Orphan Drug Designation to serdexmethylphenidate (SDX), KemPharm's proprietary prodrug of d-methylphenidate (d-MPH), for the treatment of idiopathic hypersomnia (IH), a rare neurological sleep disorder.

SDX is the sole active pharmaceutical ingredient (API) in KP1077, KemPharm's lead clinical candidate being developed as a treatment for IH and narcolepsy. KemPharm expects to initiate a Phase 2 clinical trial of KP1077 in patients with IH prior to year-end 2022 and a second trial in patients with narcolepsy in 2023.

"We appreciate the FDA's decision to grant Orphan Drug Designation to SDX for IH, a rare sleep disorder characterized by multiple, debilitating symptoms for which few treatment options exist," stated Travis Mickle, Ph.D., President and Chief Executive Officer of KemPharm. "This regulatory milestone comes at an important juncture in the ongoing development of KP1077 as we prepare to initiate the Phase 2 clinical trial in IH before year-end 2022. We believe KP1077 could provide an improved treatment option for patients with IH and other sleep disorders by addressing the most debilitating symptoms of IH, including excessive daytime sleepiness, extreme difficulty waking up (sleep inertia), severe "brain fog," and falling asleep unintentionally and/or at inappropriate times, even after adequate or prolonged nighttime sleep."

FDA Orphan Drug Designation may be granted to investigational therapies addressing rare medical diseases or conditions that affect fewer than 200,000 people in the United States, or a patient population greater than 200,000 individuals in the United States and when there is no reasonable expectation that the cost of developing and making available the drug in the United States will be recovered from sales in the United States for that drug. If a product that has Orphan Drug Designation subsequently receives the first FDA approval for a particular active ingredient for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including a full NDA, to market the same drug for the same indication for seven years, except in limited circumstances. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Among the other benefits of Orphan Drug Designation are tax credits for certain research and a waiver of the NDA application user fee.

About KemPharm:

KemPharm is a biotechnology 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 timing and results of any clinical trials or readouts, the impact of Orphan Drug Designation, the potential uses or benefits of arimoclomol, 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 the 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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