KemPharm Announces Submission of Investigational New Drug Application for Phase 2 Trial of KP1077 in Idiopathic Hypersomnia (IH)

May 5, 2022

Upon IND clearance, KemPharm anticipates initiating KP1077 Phase 2 IH trial as early as the second half of 2022

CELEBRATION, Fla., May 05, 2022 (GLOBE NEWSWIRE) -- KemPharm, Inc. (NasdaqGS: KMPH) (KemPharm, or the Company), a specialty pharmaceutical company focused on the discovery and development of novel treatments for rare central nervous system (CNS) diseases, today announced the submission of an Investigational New Drug (IND) application seeking authorization from the U.S. Food and Drug Administration (FDA) to initiate a Phase 2 clinical trial evaluating KP1077 as a treatment for idiopathic hypersomnia (IH), a rare neurological sleep disorder.

KemPharm successfully completed the pre-IND meeting process with the FDA in February 2022. During these interactions, KemPharm 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 (API) in KP1077.

"We are excited to begin the clinical development process for KP1077 as a potential treatment 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. "Our optimism surrounding KP1077 is based on the preclinical and clinical data regarding SDX, which includes the recent results from a Phase 1 clinical trial exploring the safety and pharmacokinetics (PK) of SDX administered in doses higher than currently approved. The data suggest that SDX produces a smoother, more gradual release of d-MPH that may avoid the adverse events associated with large and rapid exposure fluctuations that may be experienced with other stimulant-based therapies. 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 (EDS), 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."

Once the clinical investigation proposed in the IND has been cleared to proceed by the FDA, KemPharm plans to initiate its Phase 2 clinical trial of KP1077 in IH as early as the second half of 2022.

Additionally, KemPharm continues to advance a Phase 1 clinical trial designed to assess the relative cardiovascular safety of SDX compared to immediate-release and long-acting formulations of Ritalin® (racemic methylphenidate), a commonly prescribed CNS stimulant. Topline data from that study is expected as early as the third quarter of 2022.

About KemPharm:

KemPharm is a specialty pharmaceutical company focused on the discovery and development of novel treatments for rare central nervous system (CNS) diseases through its proprietary LAT® (Ligand Activated Therapy) platform technology. KemPharm utilizes its proprietary LAT® platform technology to generate improved prodrug versions of FDA-approved drugs as well as to generate prodrug versions of existing compounds that may have applications for new disease indications. KemPharm's prodrug product candidate pipeline is focused on the high need areas of idiopathic hypersomnia (IH) and other CNS/rare diseases. 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., and 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 prodrug product candidates visit www.kempharm.com or connect with us on Twitter, LinkedIn, Facebook and YouTube.

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 results of any IND applications, the potential benefits of KP1077, SDX or any other product candidates for any specific disease indication, or the potential benefits of any of KemPharm's product candidates,. 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, 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.

This press release also may contain estimates and other statistical data made by independent parties and by us relating to market size and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

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