KemPharm Doses First Subject in Phase 1 Clinical Trial Evaluating Cardiovascular Safety of Serdexmethylphenidate (SDX)

April 19, 2022 11:30 AM EDT

Cardiovascular safety is a key component of the KP1077 product profile; Topline data comparing SDX to other stimulant treatments expected in Q3 2022

CELEBRATION, Fla., April 19, 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 that the first subject has been dosed in a Phase 1 clinical trial designed to assess the relative cardiovascular safety of serdexmethylphenidate (SDX) compared to immediate-release and long-acting formulations of Ritalin® (racemic methylphenidate), a commonly prescribed CNS stimulant. SDX, KemPharm's proprietary prodrug of d-methylphenidate (d-MPH), is the sole active pharmaceutical ingredient (API) in KP1077, which KemPharm is developing as a treatment for idiopathic hypersomnia (IH), a rare sleep disorder.

"The intent of this clinical trial is to collect additional data to support the ongoing development of our SDX-based product candidates, in particular, KP1077, and further differentiate SDX from other methylphenidate-based products," stated Travis Mickle, Ph.D., President and Chief Executive of KemPharm. "The off-label use of stimulant based treatments in IH and other sleep disorders is growing but with an number of significant limitations based on patient comorbidities and demographics as well as patient dissatisfaction due to inadequate efficacy. Regarding cardiovascular safety, it is estimated that roughly 50% of the US population suffers from high blood pressure, which is also a primary side-effect of stimulant treatment and as a result, many IH patients are either contraindicated, discontinued or limited in the dosage of stimulant that can be prescribed for treatment. We believe this limitation can be overcome by demonstrating an improved cardiovascular safety profile compared to current stimulants, which would potentially allow SDX to be dosed higher than current products resulting in improved efficacy. As we hope to demonstrate in this trial, we believe that observing limited cardiovascular side-effects represents the key product differentiator for both the safety and potential efficacy of KP1077 compared to existing stimulant treatments."

KemPharm anticipates filing an Investigational New Drug (IND) application for KP1077 with the U.S. Food and Drug Administration (FDA) as early as this quarter. Upon acceptance of the IND, KemPharm plans to initiate a Phase 2 clinical trial of KP1077 in patients with IH later this year and a second trial in patients with narcolepsy as early as the second half of 2022.

The Phase 1 open-label trial will enroll up to 15 volunteers, each randomly receiving a series of four oral treatments in a crossover design: single doses of 80 mg and 200 mg of SDX, two doses of 40 mg of immediate-release Ritalin, or a single dose of 80 mg of Ritalin LA®, with each dose spaced at least seven days apart. The immediate-release Ritalin total dose (2 x 40 mg), the 80 mg Ritalin LA and 80 mg of SDX represent approximately the same amount of d-MPH, the active ingredient of interest, in each dose. The primary objective of the study is to evaluate cardiovascular response including heart rate, blood pressure and electrocardiogram (ECG), and any potential correlation with the exposure to d-MPH after administration of each treatment. Secondary endpoints will include pharmacokinetics, and overall safety and tolerability of SDX. KemPharm expects to receive topline results from this cardiovascular safety trial 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 new once-daily treatment for ADHD in patents 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 timing or results of any IND applications, the potential benefits of KP1077, SDX or any other product candidates for any specific disease indication, the potential benefits of any of KemPharm's product candidates, the success or timing of the launch or commercialization of AZSTARYS® or any other products, 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, 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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