KemPharm Announces Positive Topline Data from Phase 1 Clinical Trial Evaluating Cardiovascular Safety of Serdexmethylphenidate (SDX)

September 28, 2022 11:30 AM EDT

SDX administered at single doses of 80 mg and 200 mg was well-tolerated

CELEBRATION, Fla., Sept. 28, 2022 (GLOBE NEWSWIRE) -- KemPharm, Inc. (NasdaqGS: KMPH) (KemPharm, or the Company), a specialty pharmaceutical company focused on the discovery, development and commercialization of novel treatments for rare central nervous system (CNS), neurodegenerative and lysosomal storage diseases, today announced topline data from its exploratory Phase 1 clinical trial confirming the relative cardiovascular effects and pharmacokinetics 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, a potential treatment for idiopathic hypersomnia (IH), a rare sleep disorder.

Based on the data, KemPharm believes the initial dosing strengths for the planned Phase 2 clinical trial of KP1077 will be well-tolerated while providing higher overall exposures to d-MPH compared to other methylphenidate products that are often used off-label as a treatment for IH. 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 are pleased with the initial results from the Phase 1 cardiovascular safety trial of SDX, which demonstrated the potential for 'higher dose' formulations of SDX to be safe and well-tolerated. These data add to our confidence that the planned doses for the upcoming Phase 2 trial of KP1077 in IH will provide higher exposures to d-MPH while avoiding the potential for greater cardiovascular safety risk as compared to current methylphenidate products being used off-label," stated Travis Mickle, Ph.D., President and Chief Executive Officer of KemPharm. "Overall, the data suggest that SDX can be safely dosed at levels higher than current products, which is expected to result in improved efficacy. We believe this could position KP1077 as an advancement in the treatment of IH."

The Phase 1 open-label trial enrolled 15 volunteers, with 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 separated 5 hours between dosing, and a single dose of 80 mg of Ritalin LA®, with each dosing period 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 while the 200 mg SDX dose contains roughly 2.5 times the amount of d-MPH as 80 mg Ritalin LA.

Maximal plasma concentrations (C_{max}) of d-MPH were similar for both Ritalin treatments that were administered at equal doses of 80 mg. The d-MPH C_{max} values for both SDX treatments were dose proportional with the C_{max} of 200 mg SDX being approximately half of the values for Ritalin. Total plasma exposure to d-MPH was highest in the 200 mg SDX treatment group due to the extended-release profile that is unique to the prodrug, which produced prolonged exposure to d-MPH compared to both Ritalin treatment groups.

Data from the study also indicated that the maximum drug-related change from baseline in the mean curves for heart rate and systolic blood pressure correlated strongly with the maximum d-MPH exposure during the same time period. As a result, maximum changes from baseline in heart rate and systolic blood pressure were higher for both Ritalin treatments compared to the SDX treatments. Overall, the pharmacokinetic and pharmacodynamic data confirmed the two doses of SDX released d-MPH as designed and in a manner that is expected to be well-suited for the treatment of IH.

About KemPharm:

KemPharm is a specialty pharmaceutical company focused on the discovery, development and commercialization of novel treatments for rare central nervous system (CNS), neurodegenerative and lysosomal storage diseases. 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., 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 product candidates visit www.kempharm.com or connect with us on Twitter, LinkedIn, Facebook and YouTube.

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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 potential uses or benefits of 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, our plans 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 June 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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