# KemPharm, Inc. Announces Top-Line Results from Clinical Trial Evaluating the Safety and Pharmacokinetics of “Higher-Dose SDX”

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*Data reveal serdexmethylphenidate (SDX) delivered at doses higher than those studied with AZSTARYS® is well-tolerated, yields dose-proportional d-MPH exposure, and produces targeted biological effects*

CELEBRATION, Fla., Dec. 14, 2021 (GLOBE NEWSWIRE) -- KemPharm, Inc. (NASDAQ: KMPH), a specialty pharmaceutical company focused on the discovery and development of proprietary prodrugs, announced today top-line results from its clinical trial exploring the safety and pharmacokinetics of serdexmethylphenidate (SDX) delivered at doses higher than those studied as part of the AZSTARYS® development program. AZSTARYS is a once-daily product approved for the treatment of attention deficit hyperactivity disorder (ADHD) in patients ages six years and older which is being commercialized in the U.S. by Corium, Inc., a portfolio company of Gurnet Point Capital (GPC). SDX is KemPharm’s proprietary prodrug of d-methylphenidate (d-MPH) and the primary active pharmaceutical ingredient in AZSTARYS. The U.S. Drug Enforcement Agency (DEA) has classified SDX as a Schedule IV controlled substance, which is a lower schedule than all other currently available methylphenidate-based products.

The dose-ascending Phase 1 clinical trial enrolled 14 subjects who were administered up to four increasing single oral doses of SDX, each at least 14 days apart. Doses ranged from 240 mg to 600 mg. Of those individual subjects administered more than one dose of SDX, 10 received 360 mg, seven received 480 mg, and two received 600 mg. Doses greater than 240 mg were above those studied under the AZSTARYS development program. Data from the study indicated that the 240 mg and 360 mg doses of SDX were well-tolerated and produced d-MPH exposure generally proportional to the dose. Consistent with previous studies, after dosing d-MPH plasma concentrations demonstrated a gradual increase followed by a slow decline resulting in prototypical broad d-MPH exposure peak observed after oral administration of SDX. Additionally, data suggested that the higher SDX doses produced targeted biological effects that potentially align with the treatment of idiopathic hypersomnia (IH) and other sleep disorders, as well as stimulant use disorder (SUD). Specifically, increased wakefulness, alertness, excitability and insomnia effects were observed in the study. Modest increases in Drug Liking, which was expected given the SDX’s status as a Schedule IV controlled substance, coupled with the stable PK profile predicted at “steady-state,” are factors thought to be predictive of a potentially successful maintenance therapy for SUD and related disorders.

Based on these results, KemPharm is assessing the development programs, approval pathways and commercial potential of two product candidates based on SDX, KP1077 for the treatment of IH, and KP879 for the treatment of SUD, and expects to provide an update on its plans to expand its pipeline in early Q1 2022. KemPharm is also exploring other disease indications that may benefit from SDX-based treatments.

“The intent of this clinical trial was to determine if higher doses of SDX could be administered safely and produce biological effects consistent with the dosing and in alignment with disease indications that we believe could benefit from the unique properties of SDX. In short, the results were exactly what we were hoping to achieve, and we now expect to finalize our SDX development plan and commercial value assessment in early 2022,” said Travis Mickle, Ph.D., President and Chief Executive Officer of KemPharm. “We believe the status of SDX as a Schedule IV controlled substance, combined with the unique pharmacokinetic profile of SDX, offers multiple treatment opportunities in a variety of disease indications, which has the potential to be the basis for a portfolio of SDX-based products.”

**About AZSTARYS®:**

AZSTARYS is an FDA-approved, once-daily product for the treatment of attention deficit hyperactivity disorder (ADHD) in patients age six years or older. AZSTARYS consists of SDX, KemPharm’s prodrug of d-methylphenidate (d-MPH), co-formulated with immediate release d-MPH. Corium, Inc., a portfolio company of Gurnet Point Capital, is leading all commercialization efforts for AZSTARYS in the U.S.

The complete approved prescribing information for AZSTARYS may be downloaded in PDF format here: https://kempharm.com/wp-content/uploads/2021/03/AZSTARYS-Master-Label-Final_20210302.pdf

**About KemPharm:**

KemPharm is a specialty pharmaceutical company focused on the discovery and development of proprietary prodrugs to treat serious medical conditions through its proprietary LAT® (Ligand Activated Therapy) technology. KemPharm utilizes its proprietary LAT® 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 attention deficit hyperactivity disorder, or ADHD, stimulant use disorder (SUD) and CNS rare diseases, including idiopathic hypersomnia (IH). 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), and APADAZ®, an immediate-release combination product containing benzhydrocodone, KemPharm’s prodrug of hydrocodone, and acetaminophen. 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 made in reliance upon the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Forward-looking statements include all statements that do not relate solely to historical or current facts and can be identified by the use of words such as “may,” “will,” “expect,” “project,” “estimate,” “anticipate,” “plan,” “believe,” “potential,” “should,” “continue” or the negative versions of those words or other comparable words. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements, including the continued commercialization of AZSTARYS® and the further development of KemPharm’s pipeline of product candidates, or the suitability of SDX for any specific disease indication, are based on information currently available to KemPharm and its current plans or expectations and are subject to a number of uncertainties and risks that could significantly affect current plans. Risks concerning KemPharm’s business are described in detail in KemPharm’s Quarterly Report on Form 10-Q for

the quarter ended September 30, 2021, and KemPharm's other filings with the Securities and Exchange Commission. KemPharm is under no obligation to, and expressly disclaims any such obligation to, update or alter its forward-looking statements, whether as a result of new information, future events or otherwise.

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