



## **KemPharm Issues Letter to Shareholders Detailing Strategic Focus on CNS/Rare Disease Indications and Updated Clinical Development Strategy**

January 19, 2022 9:21 PM EST

*KemPharm to Advance KP1077 for Idiopathic Hypersomnia as Lead SDX Product Candidate*

*Strong Balance Sheet with Cash and Cash Equivalents of \$127.8M as of December 31, 2021; Available Cash and Revenues Extend Cash Runway through 2025 and Beyond*

*AZSTARYS® National Launch Accelerating, Full Sales Team in Place by end of January 2022*

*Conference Call and Live Audio Webcast with Slide Presentation Scheduled for Today, January 19, 2022, at 4:30 p.m. ET*

CELEBRATION, Fla., Jan. 19, 2022 (GLOBE NEWSWIRE) -- KemPharm, Inc. (NasdaqGS: KMPH), a specialty pharmaceutical company focused on the discovery and development of proprietary prodrugs, today announced that Travis C. Mickle, Ph.D., President and CEO of KemPharm, has issued a Letter to Shareholders detailing the company's strategic focus on Central Nervous System (CNS)/Rare Disease indications, as well as an updated clinical development strategy.

The full text of the letter follows. Information about the conference call and live audio webcast can be found following the letter's conclusion.

### **A MESSAGE FROM OUR CHIEF EXECUTIVE OFFICER**

Dear Fellow Shareholders:

Today is an important milestone in the ongoing evolution of KemPharm and follows a series of transformative events for the company during the past 12 months highlighted by the U.S. commercial launch of AZSTARYS®, the uplisting of our stock to the Nasdaq Global Select Market and its addition to the Nasdaq Biotechnology Index, and the restructuring and recapitalization of our finances, culminating with the announcement of a \$50 million share repurchase program through 2023. These events have allowed us to build a strong foundation for the Company's future, and with that momentum, we have worked to thoughtfully refine our strategic focus to CNS/Rare Disease indications for our future product development efforts.

Given our significant expertise and a track record of product development success, our strategic focus on CNS/rare disease indications allows us to target high-value areas with significant unmet needs. Our evaluation of both internal and external product candidates is guided by criteria that include an assessment of the commercial opportunity, and understanding of clinical, development and regulatory risks, as well as time, cost and need, among other strategic considerations. Within CNS/rare disease, there are many potential indications where we believe our LAT® (Ligand Activated Therapy) platform technology and our product development expertise can provide multiple opportunities to drive growth for KemPharm. Some of the possible areas of interest include:

- Neurology and neurodegenerative diseases like Alzheimer's disease, Parkinson's disease and Huntington's disease;
- Psychiatric disorders, which could include indications focused on niche market opportunities like rare sleep disorders or psychedelics; and
- Rare diseases and other adjacent or related therapeutic categories, like gastroenterology, metabolic diseases or endocrinology.

While we continue to build and acquire new opportunities, serdexmethylphenidate (SDX), will be a key building block for our CNS/rare disease pipeline and represents the potential to continue driving near-term growth for KemPharm. For those new to KemPharm, SDX is our proprietary prodrug of d-methylphenidate (d-MPH). It is the primary active pharmaceutical ingredient (API) in AZSTARYS, a once-daily product for the treatment of attention deficit hyperactivity disorder (ADHD) in patients ages six years and older, and is also the sole API in two other development-stage products, KP1077 (Idiopathic Hypersomnia) and KP879 (Stimulant Use Disorder).

As a prodrug, SDX is specifically designed to be pharmacologically inactive until reaching the lower gastrointestinal tract, where, by design, SDX is gradually converted to d-MPH. For AZSTARYS, this property is key to the drug's ability to provide extended symptom control for up to 13 hours, and SDX's combined formulation with d-MPH differentiates AZSTARYS from other d-MPH-based ADHD treatments on the market. AZSTARYS is being commercialized in the U.S. by Corium, Inc.

SDX also possesses several pharmacokinetic (PK) properties that distinguish it from d-MPH and other stimulant drug therapies. As highlighted at the recent APSARD 2022 Annual Conference, research has demonstrated that SDX produces dose-proportional d-MPH exposure, is not impacted by the presence or lack of food, and is fully absorbed, metabolized and excreted following oral administration. These properties, we believe, are instrumental to the prodrug's ability to enable a consistent and smooth release of d-MPH.

Lastly, and perhaps most importantly, SDX is the only methylphenidate-based compound designated as a Schedule IV controlled substance by the U.S. Drug Enforcement Agency (DEA). This is a significant distinction as it could indicate to prescribers that SDX has a lower potential for abuse when compared to d-MPH, a Schedule II controlled substance.

Given the many unique properties of SDX, we believe there is a substantial opportunity to develop multiple SDX-based drug candidates that potentially address disease indications underserved by current therapeutic options. Based on this promise, we conducted a clinical trial exploring the safety and

PK of SDX delivered at doses higher than those studied as part of the AZSTARYS development program. The intent of the trial was to determine if higher doses of SDX could be administered safely and produce pharmacodynamic effects consistent with the dosing and in alignment with disease indications that we believe could benefit from the unique properties of SDX.

As reported in December, 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, with d-MPH plasma concentrations demonstrating a gradual increase followed by a slow decline. Additionally, data suggested that the higher SDX doses produced targeted biological effects that potentially align with the treatment of idiopathic hypersomnia and other sleep disorders, as well as stimulant use disorder. Specifically, increased wakefulness, alertness, excitability, and insomnia effects were observed in the study.

In short, the results were exactly what we were hoping to achieve, allowing us to finalize our SDX development plan.

### **KP1077 for Idiopathic Hypersomnia – Lead Development Candidate**

Buoyed by the favorable SDX data, we conducted an analysis of potential development opportunities to determine how to best prioritize our SDX-based product candidates in order to maximize shareholder value. The process included:

1. A risk assessment analyzing the clinical, development and regulatory challenges for each product;
2. An evaluation of each product's commercial opportunity, including physician, payor, and competitive landscape research; and
3. A calculation of the projected time, cost, and additional needs required to ensure regulatory success with the FDA, from IND (Investigational New Drug application) to NDA (New Drug Application)

From this, it was clear that KP1077 as a treatment for idiopathic hypersomnia (IH) should be our lead development candidate.

For those unfamiliar with IH, it is a rare neurological sleep disorder affecting approximately 37,000 patients in the U.S. However, anecdotal evidence suggests the actual number of IH patients could be higher.

The cardinal feature of IH is excessive daytime sleepiness (EDS), characterized by daytime lapses into sleep, or an irrepressible need to sleep that persists even with adequate or prolonged nighttime sleep. Additionally, those with IH have extreme difficulty waking, otherwise known as "sleep inertia," severe brain fog, and may fall asleep unintentionally or at inappropriate times, otherwise known as narcolepsy. Moreover, people with IH often report memory problems, difficulty maintaining focus, and depression.

IH is a serious, debilitating condition that can impact every moment of a person's life, even making the mundane a challenge. As a result, both the medical and pharmaceutical communities have begun to direct more resources at treating the condition. Xywav<sup>®</sup>, developed by Jazz Pharmaceuticals, was approved by the FDA in August for the treatment of IH, and recently, Harmony Biosciences announced that it would initiate a Phase 3 trial of Wakix<sup>®</sup>, currently approved for EDS or cataplexy in adult patients with narcolepsy, in patients with IH. Additionally, prescribers utilize narcolepsy medications and various methylphenidate/stimulant products "off-label" to treat IH symptoms, with methylphenidate being one of the most commonly used stimulants for treating IH.

While each of these medications can help to address certain IH symptoms, each has critical shortcomings, including dosing inconvenience (Xywav requires two doses at night with one dose occurring four hours after bedtime), serious adverse events, such as elevated blood pressure and heart rate, and significant drug-drug interactions (DDIs), including with medications used to manage contraception and depression. In addition to the shortcomings, patients have indicated that their current medication effectiveness was poor<sup>1</sup>.

The market potential for IH is also highly compelling. With limited treatment options available for this small, underserved patient segment, there is the potential for KP1077 to be designated as an orphan drug, as well as possible eligibility for fast-track review status and for designation as a breakthrough treatment. In addition, we believe that if differentiated from other treatment options, pricing KP1077 in between products like Teva Pharmaceuticals' Provigil<sup>®</sup> (approximately \$24,000 per year at the highest dose) and Jazz Pharmaceuticals' Xywav (approximately \$187,000 per year at the highest dose) could lead to significant market share. Moreover, since the patient population is relatively small and well-monitored, given the debilitating nature of the disease, we believe it would be possible for KemPharm to field its own small specialty sales force to commercialize the product, and therefore keep a larger portion of the economics instead of out-licensing commercial rights to a third party.

Given these factors, we see a substantial opportunity to advance KP1077 as a treatment for IH, and initial market research suggests that KP1077 could capture a large share of the IH market based on the following potential treatment, regulatory and commercial benefits:

- Dosing flexibility – either one dose or two doses – designed to address the two primary issues associated with IH:
  - Nighttime dose would address sleep inertia
  - Morning dose would address daytime brain fog
- No drug-drug interaction potential especially with hormonal contraceptives and antidepressants
- Reduced abuse potential with a Schedule IV designation
- Orphan drug designation potential, as well as fast-track and breakthrough designation

eligibility

- No generic equivalent
- Composition of matter patents extending to 2037 with additional applications potentially extending the IP time horizon

In addition to these potential benefits, we anticipate an efficient and robust development program for KP1077. At present, we are targeting an Investigational New Drug (IND) application filing for KP1077 in IH by mid-year 2022. We then anticipate initiating a Phase 2 trial of KP1077 in IH in the third quarter of 2022 with top-line data by the second quarter of 2023.

We are also planning a parallel development program for KP1077 in narcolepsy. The IND filing for this additional program is expected during the second half of 2022, with a Phase 2/3 trial expected to begin by the end of 2022 or early 2023.

While KP1077 will be our lead SDX development candidate, we plan to continue the development of KP879 as an extended-duration, agonist replacement therapy for the treatment of Stimulant Use Disorder (SUD). However, through our evaluation of the clinical, regulatory and development risks for pursuing the SUD indication, we recognized that KP879 will require a more challenging and lengthier development program that will likely necessitate partnership with government, academia, or other strategic partners to successfully advance the program toward approval.

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<sup>1</sup> <https://www.sleepcountshcp.com/diopathic-hypersomnia-treatment-options>

### **Creating a CNS/Rare Disease Franchise with SDX and Other Opportunities**

Our strategic focus on CNS/Rare Disease indications will also guide our ongoing business development efforts to expand our development pipeline and accelerate value creation. As already outlined, we are considering external opportunities within neurology and neurodegenerative diseases, psychiatric disorders, and other rare diseases, along with adjacent or related therapeutic categories. We are seeking assets in Phase 2 or later, subject to our evaluation criteria, that we can in-license or acquire.

If we are successful, new product candidates that fit this focus could be accretive to KemPharm's value proposition by potentially adding new clinical data catalysts and the potential to create incremental long-term value for shareholders. In addition, we believe that a multi-channel development program with multiple product candidates addressing various CNS/rare disease indications will diversify risk and create the potential for multiple commercial-stage products in the future.

### **KemPharm –Poised for Significant Growth in 2022 and Beyond**

Foundational to the pursuit of our new strategic focus is the potential to begin realizing royalties and sales milestone payments from the ongoing commercialization of AZSTARYS. Corium, our U.S. commercial partner for AZSTARYS, continues to see prescriber momentum build, and we are pleased to report that AZSTARYS now has payor access to more than 100 million commercial and Medicaid patient lives as of January 1, 2022. Payor access for AZSTARYS has doubled since October 1, 2021, thanks to the efforts of the excellent team Corium has built for the commercial launch. This success is leading Corium to accelerate national expansion of the commercial roll-out, with full national staffing to be in place by end of January 2022. We are encouraged by this progress and expect 2022 to be a time of significant growth for AZSTARYS.

We also enter 2022 with a strong balance sheet, which as of December 31, 2021, included cash and cash equivalents totaling \$127.8 million. Unlike many development-stage biopharmaceutical companies, our balance sheet provides the ability to pursue our strategic and product development objectives while also seeking external opportunities. Available capital combined with anticipated revenues allows us to pursue our development plan and extend our cash runway through 2025 and beyond. In addition, our recently announced \$50 million share repurchase program, which will remain in place through 2023, provides a mechanism through which we can directly return value to shareholders as we are able to realize success in our business efforts.

In closing, the successes that we have experienced over the past year combined with the growth opportunities anticipated for 2022 and beyond have been made possible by the diligent efforts of our team and the support of our shareholders. Thanks to all of you.

Sincerely,

Travis C. Mickle, Ph.D.  
Shareholder, President, and Chief Executive Officer  
KemPharm, Inc.

*End of the shareholder letter text.*

### **Conference Call Information:**

KemPharm will host a conference call and live audio webcast with slide presentation on Wednesday, January 19, 2022, at 4:30 p.m. ET, to discuss its strategic focus on CNS/rare disease indications and its updated clinical development strategy.

**Telephone Access:** To access the conference call telephonically, interested participants and investors will be required to register via the following online form: <http://www.directeventreg.com/registration/event/6718737>

Once registered, all individuals will be provided with participant dial-in numbers, a passcode and a registrant ID, which can then be used to access the conference call.

Participants may register at any time. It is recommended that the registration process be completed at least 15 minutes prior to the start of the call.

**Webcast Access:** The live audio webcast with slide presentation will be accessible via the Investor Relations section of KemPharm's website, <http://investors.kempharm.com/>. An archive of the webcast and presentation will be available for 90 days beginning at approximately 5:30 p.m. ET, on January 19, 2022.

## About AZSTARYS®:

AZSTARYS is an FDA-approved, once-daily product for the treatment of attention deficit hyperactivity disorder (ADHD) in patients age six years and 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](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 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 patients 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](http://www.kempharm.com) or connect with us on [Twitter](#), [LinkedIn](#), [Facebook](#) and [YouTube](#).

## Financial Disclosure Advisory

The Company reports its financial results in accordance with U.S. generally accepted accounting principles (GAAP). The expected financial results discussed in this press release are preliminary and represent the most current information available to the Company's management, as financial closing procedures for the fourth quarter and fiscal year ended December 31, 2021, are not yet complete. These estimates are not a comprehensive statement of the Company's financial results for the fourth quarter and fiscal year ended December 31, 2021, and actual results may differ materially from these estimates as a result of the completion of year-end accounting procedures and adjustments, including the execution of the Company's internal control over financial reporting, the completion of the preparation and audit of the Company's financial statements and the subsequent occurrence or identification of events prior to the formal issuance of the audited financial statements for fiscal 2021.

## 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 continued commercialization and market outlook for AZSTARYS®, the potential benefits of AZSTARYS, KemPharm's pipeline of product candidates including the clinical development of KP879 and KP1077, the promise and potential impact of our preclinical or clinical trial data, upcoming milestones, including without limitation the timing and results of any clinical trials or readouts, the potential benefits of SDX or any other product candidates for any specific disease indication, or the potential benefits of any of KemPharm's product candidates or market assessments, KemPharm's forecasted cash runway, our strategic and product development objectives, and the execution and duration of our share repurchase program. 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, 2020, KemPharm's Quarterly Report for the quarter ended September 30, 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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