



KemPharm Issues Letter to Shareholders

January 24, 2023

CELEBRATION, Fla., Jan. 24, 2023 (GLOBE NEWSWIRE) -- KemPharm, Inc. (NasdaqGS: KMPH) (KemPharm, or the Company), a rare disease therapeutics company focused on the development of treatments for rare central nervous system (CNS) disorders, neurodegenerative diseases, lysosomal storage disorders and related treatment areas, today announced that Richard W. Pascoe, Chief Executive Officer of KemPharm, has issued a Letter to Shareholders. The letter provides an update on recent events and outlook for 2023.

The full text of the letter follows.

A MESSAGE FROM RICH PASCOE, OUR CHIEF EXECUTIVE OFFICER

Dear Fellow Shareholders:

This is a very exciting time in the growth of KemPharm and our strategic transformation into a leading rare disease company. The numerous accomplishments in 2022 and prior have set the stage for us to achieve multiple milestones during 2023 as we advance our arimoclomol and KP1077 programs towards key inflection points that have the potential to position us for success in fulfilling our mission to become a commercially-focused rare disease company.

Since the beginning of the year, we have made several enhancements to the leadership team that are squarely focused on enhancing shareholder value as we seek to secure regulatory approval for our pipeline assets, build top-tier commercial capabilities, and enhance our pipeline through targeted business development transactions. With the senior team firmly in place and focused on achieving our key objectives, I am confident that we will deliver value in 2023 and beyond.

KemPharm is a company founded on solid science and this will continue to be a pillar upon which we stand. However, solid science alone will not be enough to propel KemPharm into its next stage of growth and create the value that our shareholders deserve. We must accelerate our pipeline expansion efforts and thoughtfully begin building our commercial capabilities so that we are well-positioned to not only create, but also retain a much larger portion of the value that we have developed from the investments entrusted to us by you, the shareholders of KemPharm. Underscored by a strong balance sheet, we will accomplish this task by leveraging our internal capabilities as well as identifying targeted business development opportunities exemplified by our Orphazyme transaction in 2022.

As KemPharm's new CEO, I bring more than 30 years of biotechnology industry experience to the company. Over these years, there have been successes and setbacks, and learning from what went right and what went wrong in each situation is important to building a successful, thriving company. I am eager to leverage these experiences, combined with the collective experiences of our senior leadership team, to successfully navigate the next chapter in KemPharm's evolution.

Our goals as a company, and as a leadership team, are two-fold. First, we seek to improve the lives of patients by developing novel therapeutics for rare diseases where there are no treatments, or where current treatment options are insufficient. Second, we must drive value for our shareholders. I believe these are two sides of the same coin. If we are successful in these endeavors, which I firmly believe we will be, and we do so by carefully managing our resources, then we will have delivered on our mission to provide value to both patients and shareholders.

Arimoclomol – A Unique and Potentially Game-Changing Product Opportunity

KemPharm's acquisition of arimoclomol in May 2022, as well as substantially all of the assets and operations of Orphazyme, was a transformative event for the company that is now central to our strategy to build KemPharm's value through the advancement and commercialization of novel treatments that address rare diseases.

Arimoclomol is an orally-delivered, first-in-class investigational product candidate being developed as a treatment for Niemann-Pick disease type C (NPC), a rare neurodegenerative disease characterized by an inability of the body to transport cholesterol and lipids inside of cells. Symptoms of NPC include a progressive impairment of mobility, cognition, speech, and swallowing, often culminating in premature death. Therapies to treat NPC are desperately needed, and for this reason, arimoclomol is currently being made available to NPC patients in the U.S., France, Germany and other European countries under various Early Access Programs (EAPs).

KemPharm's regulatory team continues to make progress with the updated New Drug Application (NDA) for arimoclomol as a treatment for NPC, which we expect to file as early as the third quarter of 2023. Our aim is to submit an NDA filing that presents meaningful evidence of safety and efficacy and provides the highest likelihood of approval. To that end, we are continuing to work

diligently to characterize the substantial data repository generated since the prior Complete Response Letter, including the recently completed four-year open-label safety trial.

Adding to our enthusiasm for arimoclomol is the fact that the drug has received Orphan Drug Designation for NPC in the U.S. and the E.U., as well as Fast-Track Designation, Breakthrough Therapy Designation, and Rare Pediatric Disease Designation from the U.S. Food and Drug Administration (FDA) for NPC. If approved in the U.S., arimoclomol will also be eligible to receive a Pediatric Priority Review Voucher.

For 2023, KemPharm is focused on four key areas for arimoclomol:

1. The resubmission of the NDA to the FDA targeted to be filed as early as the third quarter of 2023;
2. Supporting the continuation of the EAPs in the various countries where it has been available;
3. Identifying a regulatory path forward with the European Medicines Agency; and
4. Building the commercial infrastructure needed to support an ultra-rare disease product.

KP1077 – Advancing Clinical Trials in Idiopathic Hypersomnia and Narcolepsy

As announced in December 2022, KemPharm initiated the Phase 2 clinical trial investigating KP1077 as a treatment for idiopathic hypersomnia (IH). This was a significant clinical milestone for KemPharm and provides a springboard for several value creating events during the year, including:

1. Interim Phase 2 KP1077 IH efficacy and safety data as early as the third quarter of 2023 and full Phase 2 data by year end 2023; and
2. Potential to advance KP1077 directly into phase 3 studies in narcolepsy as early as prior to year-end 2023 based on previous Phase 1 studies of serdexmethylphenidate (SDX), the sole pharmaceutical agent in KP1077, and learnings from the ongoing Phase 2 study in IH.

We believe there is great potential for KP1077 in IH based on preclinical and clinical data involving SDX, our prodrug of d-methylphenidate (d-MPH), and the sole active pharmaceutical ingredient in KP1077. This includes the results from a Phase 1 clinical trial comparing the cardiovascular safety of SDX to immediate-release and long-acting formulations of Ritalin® (Methylphenidate HCl), a commonly prescribed CNS stimulant. Based on the data, we believe the initial dosing strengths for the 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. This is expected to result in improved efficacy in treating several of the debilitating symptoms of IH, which could differentiate KP1077 as an advancement in the treatment of IH.

The recently initiated Phase 2 clinical trial is designed to investigate this hypothesis as well as evaluate KP1077's effect on several symptoms associated with IH, including excessive daytime sleepiness, extreme difficulty waking, and severe brain fog. Additionally, this study provides an opportunity to explore both the optimal SDX dose range and dosing regimen(s) – nighttime dosing or twice daily – which will inform the design of future Phase 3 studies for the IH indication and the potential to expand the indication to include narcolepsy.

KemPharm – Many Reasons for Optimism in 2023 and Beyond

We believe KemPharm is stronger today than it has been at any point since its inception. We possess a diverse product pipeline led by arimoclomol and KP1077, and supported by the ongoing commercialization of AZSTARYS® by our partner, Corium, Inc.

Supporting our strategic and pipeline development efforts is a strong financial foundation, bolstered by \$102.9 million in cash, cash equivalents and long-term investments as of December 31, 2022. Unlike many development-stage biopharmaceutical companies, we believe our balance sheet provides the ability to pursue our strategic and product development objectives while also seeking external business development opportunities aligned with our strategic pivot to a fully integrated commercial company. Given this, and based on our current operating forecast, we expect available capital will allow us to pursue our development plans and extend our cash runway into 2026.

In closing, we believe the numerous milestone opportunities anticipated for 2023 and beyond will position KemPharm for continued growth as we focus on bringing much-needed therapies to patients with rare diseases. Since inception, we have enjoyed product development success in a risky industry. Our move to rare diseases is an opportunity to build upon what is good at KemPharm and make it great. We seek to build upon our legacy of solid science and position ourselves to realize a greater portion of the value potential of the assets we have developed, and thereby create greater value for our shareholders.

We look forward to your participation in the 2023 Annual Meeting of Stockholders, which will be held virtually at 8:00 a.m. ET on April 25, 2023. Details for how to join the meeting will be provided in the upcoming weeks via our annual proxy statement.

On behalf of our board of directors, thank you for your loyalty to KemPharm and for placing your trust in me and our senior team. I know that we must earn your trust each and every day.

Sincerely,

Richard W. Pascoe

Shareholder and Chief Executive Officer
KemPharm, Inc.

End of the shareholder letter text.

About KemPharm:

KemPharm is a rare disease therapeutics company focused on the discovery, development and commercialization of novel treatments for rare CNS and neurodegenerative diseases, lysosomal storage disorders and related treatment areas. KemPharm has a diverse product portfolio, combining a clinical-stage development pipeline with NDA-stage and commercial assets. The pipeline includes arimocloamol, 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. The FDA has also approved 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](#).

Early access programs are made available by KemPharm, Inc. and its affiliates, and are subject to the Company's Early Access Program (EAP) policy as published on its website at www.kempharm.com. Participation in these programs is subject to the laws and regulations of each jurisdiction under which each respective program is operated. Eligibility for participation in any such program is at the discretion of the treating physician.

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 initiation, timing and results of any clinical trials or readouts, the timing or results of any Investigational New Drug (IND) applications and New Drug Application (NDA) submissions, KP1077, SDX, or any other product candidates for any specific disease indication or at any dosage, our cash, cash equivalents and long-term investments and the sufficiency of our cash reserves or our ability to fund our operating and development activities for any specific length of time; 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 KemPharm's Quarterly Report on Form 10-Q for the three months ended September 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.

Trademarks are held by their respective owners.

Financial Disclosure Advisory

The cash, cash equivalents and long-term investments information provided in this letter is based on preliminary unaudited information and management estimates for the year ended December 31, 2022, is not a comprehensive statement of the Company's financial results as of and for the fiscal year ended December 31, 2022 and is subject to completion of the Company's financial closing procedures. The Company's independent registered public accounting firm has not conducted an audit or review of and does not express an opinion or any other form of assurance with respect to this preliminary estimate.

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