KemPharm Announces Strategic Acquisition of Arimoclomol from Orphazyme, Expanding its Rare CNS Diseases Pipeline

May 15, 2022 at 8:00 PM EDT

Arimoclomol is an NDA-stage, revenue-generating investigational drug candidate being developed for the treatment of Niemann-Pick disease type C (NPC), a rare progressive neurodegenerative disease

KemPharm plans to refile the New Drug Application (NDA) for arimoclomol in NPC with the U.S. Food and Drug Administration (FDA) as early as the First Quarter of 2023

Conference call and live audio webcast with slide presentation is scheduled for tomorrow, May 16, 2022, at 8:30 a.m., EDT

CELEBRATION, Fla., May 15, 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 a definitive agreement with Orphazyme A/S (in reconstruction) (ORPHA.CO; ORPH) (Orphazyme) to acquire arimoclomol, an orally-delivered, first-in-class heat shock protein (HSP) amplifier being developed as a treatment for Niemann-Pick disease type C (NPC). NPC is a rare progressive neurodegenerative disease that impacts children, adolescents, and adults, and is characterized by an inability of the body to transport cholesterol and lipids inside of cells, which leads to the abnormal accumulation of these substances within various tissues of the body, including the brain. Arimoclomol is currently being made available to NPC patients in the U.S., France and Germany under Orphazyme's Early Access Programs (EAP).

Under the terms of the agreement, KemPharm will purchase substantially all of the assets and operations of Orphazyme, including arimoclomol, for a cash payment of USD $12.8 million. The Company expects to finance the cash payment with a revolving line of credit secured by KemPharm's balance sheet. KemPharm intends to retain the majority of Orphazyme's current employees. In addition, KemPharm has agreed to assume an estimated reserve liability equal to approximately USD $5.2 million, which is an estimated future rebate due to the French regulatory authorities based on the revenue generated from the EAP in France. For the year ending December 31, 2022, the EAP is expected to generate at least USD $12 million in revenue based upon enrollment in France as of March 2022. The EAP is expected to remain in place until arimoclomol becomes commercially available in each of the current EAP markets. The transaction is expected to close on or before June 1, 2022, subject to customary closing conditions and approval by Orphazyme's creditors and the Danish bankruptcy court. Canaccord Genuity LLC acted as a strategic advisor to KemPharm for the transaction.

"This strategic acquisition of arimoclomol is a transformative event that significantly expands our rare CNS disease development pipeline, bringing to KemPharm an NDA-stage, revenue-generating product upon which we intend to build commercial capabilities that allow KemPharm to create and retain value for the benefit of shareholders," stated Richard Pascoe, Executive Chairman of KemPharm. "Moreover, the financial structure of the acquisition combined with the revenue currently being generated by arimoclomol from the early access program in France affords us the opportunity to acquire the asset in a capital efficient manner that has the potential to create positive cash flow, while incurring no shareholder dilution."

Arimoclomol is administered orally and has been studied in ten Phase 1, four Phase 2, and three pivotal Phase 2/3 trials. Arimoclomol has received Orphan Drug Designation (ODD) for NPC in the United States and the European Union. Arimoclomol has received Fast-Track Designation (FTD), Breakthrough Therapy Designation (BTD), and Rare Pediatric Disease Designation (RPDD) from the FDA for NPC. If approved in the U.S., arimoclomol would also be eligible to receive a

Pediatric Priority Review Voucher. On June 17, 2021, Orphazyme received a Complete Response Letter (CRL) from the FDA regarding its NDA for arimoclomol for the treatment of NPC. Orphazyme also withdrew its European Marketing Authorisation Application (MAA) for arimoclomol for the treatment of NPC ahead of a final vote and opinion by the Committee for Medicinal Products for Human Use (CHMP).

“The acquisition of arimoclomol aligns perfectly with our strategy to build KemPharm’s value via the advancement and commercialization of novel treatments that address rare CNS conditions, including our lead clinical candidate, KP1077 in idiopathic hypersomnia,” stated Travis Mickle, Ph.D., President and Chief Executive Officer of KemPharm. “We have carefully evaluated the CRL issued by the FDA and the minutes from the subsequent Type A meeting, as well as the data that has been generated from the development work performed to date. We believe the efficacy signal for arimoclomol in NPC is convincing and that there is a viable regulatory path that could enable a successful NDA resubmission. KemPharm has significant experience with challenging regulatory situations, having successfully led or participated in three FDA product approvals, two of which followed an initial CRL. We welcome the opportunity to work with the FDA on the resubmission of the NDA for arimoclomol in NPC, which we expect to file as early as the first quarter of 2023.”

NPC is a rare progressive lysosomal storage disorder characterized by an inability of the body to transport cholesterol and lipids inside of cells. This leads to dysfunction in organs such as the brain, spleen and liver. NPC can range from a fatal disorder within the first few months after birth (neonatal period) to a late onset, chronic progressive disorder that remains undiagnosed well into adulthood. Disease progression is irreversible in all patients, and loss of neuro-cognitive function adversely impacts their daily life. The mean age of death is 13 years (Bianconi, 2019), and there are no approved treatments for NPC in the United States.

“NPC is an ultra-rare, inherited neurodegenerative disease that affects people of all ages from infancy to adulthood, and leads to progressive impairment of mobility, cognition, speech, and swallowing, culminating in premature death,” said Marc Patterson, MD, Professor of Neurology, Pediatrics and Medical Genetics at Mayo Clinic. “Therapies to treat NPC are desperately needed, and there is hope that a treatment such as arimoclomol could provide a solution to patients around the world who are living daily with the disease. It is encouraging that there is an opportunity to continue the regulatory process for arimoclomol with the FDA.”

Conference Call Information:

KemPharm will host a conference call and live audio webcast with a slide presentation today at 8:30 a.m., EDT. Interested participants and investors may access the conference call by dialing either:

- (833) 793-7231 (U.S.)
- (614) 999-1675 (international)
- Conference ID: 7880862

An audio webcast with slide presentation will be accessible via the Investor Relations section of the Company’s website, http://investors.kempharm.com/. An archive of the webcast and presentation will be available for 90 days beginning at approximately 9:30 a.m. EDT, on May 16, 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 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 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 expected closing of KemPharm’s acquisition of arimoclomol, including the timing and financing thereof, the acquisition’s impact on KemPharm’s operations and financial results, the expected revenue from the EAP and the timing or results of an NDA resubmission for arimoclomol. 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 March 31, 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.

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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