Combangio, Inc., a Wholly Owned Subsidiary of Kala Pharmaceuticals, Awarded $15 Million by California Institute for Regenerative Medicine to Support Ongoing KPI-012 Program for the Treatment of PCED

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MENLO PARK, Calif. and ARLINGTON, Mass., April 28, 2023 (GLOBE NEWSWIRE) -- Kala Pharmaceuticals, Inc. (NASDAQ:KALA), a clinical-stage biopharmaceutical company dedicated to the research, development and commercialization of innovative therapies for rare and severe diseases of the eye, today announced that the California Institute for Regenerative Medicine (CIRM) has awarded Combangio, Inc., a wholly owned subsidiary of Kala, a $15 million grant to support its ongoing KPI-012 program for the treatment of persistent corneal epithelial defect (PCED). The grant includes funding for the CHASE (**C**orneal **H**ealing **A**fter **SE**cretome therapy) Phase 2b clinical trial as well as product and process characterization and analytical development for the program.

"Consistent with CIRM's mission to accelerate the development of potentially transformative medicines to improve the care of patients globally, we are excited to support the KPI-012 program for the treatment of PCED," said Maria T. Millan, M.D., President and CEO of CIRM. "KPI-012's differentiated product profile has the potential to deliver rapid and sustained wound healing with broad-based application across all underlying PCED etiologies. We look forward to partnering with the company to bring this regenerative therapy to the thousands of patients in need."

KPI-012 is a human mesenchymal stem cell secretome (MSC-S), being developed for the treatment of PCED. Based on its multifactorial mechanism of action and preclinical and clinical data generated to-date, Kala believes KPI-012 may represent a significant advancement in the treatment of PCED and could become the first approved treatment for PCED across all its various etiologies.

The CHASE Phase 2b clinical trial includes two patient cohorts. In March 2023, the company reported positive safety data from the first patient cohort, evaluating the safety of the high dose of KPI-012 (3 U/mL) dosed topically four times per day (QID). The second patient cohort is currently enrolling in a multicenter, randomized, double-masked, vehicle-controlled, parallel-group study to evaluate the safety and efficacy of two doses of KPI-012 ophthalmic solution (3 U/mL and 1 U/mL) versus vehicle dosed topically QID for 56 days in approximately 90 patients. The primary endpoint of the trial is the complete healing of the PCED as measured by corneal fluorescein staining. Topline safety and efficacy data is targeted in the first quarter of 2024. If the results are positive, and subject to discussion with regulatory authorities, the company believes this trial could serve as the first of two pivotal trials required to support the submission of a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA).

"We are honored that CIRM has recognized KPI-012's potential to correct impaired corneal healing," said Mark Iwicki, Chief Executive Officer and Chairman of Kala Pharmaceuticals. "We believe this grant not only serves as further validation for KPI-012 and our MSC-S platform, but will provide us with important funding to advance our ongoing Phase 2b trial, which could serve as the first of two pivotal trials needed to support the submission of a BLA to the FDA."

About KPI-012 for Persistent Corneal Epithelial Defect (PCED)

Persistent corneal epithelial defect, which is defined as a persistent non-healing corneal defect or wound that is refractory to conventional treatments, is a rare disease with an estimated incidence in the United States of 100,000 cases per year and 238,000 cases per year in the United States, European Union and Japan combined. PCED can have various etiologies, including neurotrophic keratitis, surgical epithelial debridement, microbial/viral keratitis, corneal transplant, limbal stem cell deficiency and mechanical and chemical trauma and, if left untreated, can lead to infection, corneal ulceration or perforation, scarring, opacification and significant vision loss.

Based on a multifactorial mechanism of action and preclinical and clinical data generated to-date, Kala believes KPI-012 may represent a significant advancement in the treatment of PCED and could become the first approved treatment for PCED across all its various etiologies.

About Kala Pharmaceuticals

Kala is a clinical-stage biopharmaceutical company dedicated to the research, development and commercialization of innovative therapies for rare and severe diseases of the eye. Kala's biologics-based investigational therapies utilize Kala's proprietary mesenchymal stem cell secretome (MSC-S) platform. Kala's lead product candidate, KPI-012, is a human MSC-S, which contains numerous human-derived biofactors, such as growth factors, protease inhibitors, matrix proteins and neurotrophic factors that can potentially correct the impaired corneal healing that is an underlying etiology of multiple severe ocular diseases. KPI-012 is currently in clinical development for the treatment of persistent corneal epithelial defect (PCED), a rare disease of impaired corneal healing, for which it has received Orphan Drug and Fast Track designations from the U.S. Food and Drug Administration. Kala is also targeting the potential development of KPI-012 for the treatment of Limbal Stem Cell Deficiency and ocular manifestations of moderate-to-severe Sjögren's and has initiated preclinical studies to evaluate the potential utility of its MSC-S platform for retinal degenerative diseases, such as Retinitis Pigmentosa and Stargardt Disease. For more information on Kala, please visit www.kalarx.com.

About The California Institute for Regenerative Medicine (CIRM)

CIRM was created by the people of California to accelerate regenerative medicine to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission. To meet this challenge, CIRM's team of highly trained and experienced professionals actively partner with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising regenerative medicine technologies. With $5.5 billion in funding and more than 150 active stem cell programs in its portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality. For more information on CIRM, please visit www.cirm.ca.gov.

Forward Looking Statements:

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 that involve substantial risks and uncertainties. Any statements in this press release about Kala's future expectations, plans and prospects, including but not limited to statements about the potential benefits and future operation of the CIRM award; Kala's expectations with respect to potential advantages of KPI-012 and its MSC-S platform; the clinical utility of KPI-012 for PCED; anticipated timelines to report topline data for the CHASE Phase 2b clinical trial of KPI-012; Kala's belief that the Chase Phase 2b trial could serve as the first of two pivotal trials required to support the submission of a BLA to the FDA; Kala's plans to pursue research and development of KPI-012 and its MSC-S platform for other indications; and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "likely," "will," "would," "could," "should," "continue," and similar expressions constitute forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: Kala's ability to comply with the requirements under the CIRM award; uncertainties inherent in the initiation and conduct of preclinical studies and clinical trials; uncertainties regarding availability and timing of data from clinical trials; whether results of early clinical trials or trials in different disease indications will be indicative of the results of ongoing or future trials; whether results of the Phase 1b clinical trial of KPI-012 will be indicative of results for any future clinical trials and studies of KPI-012, including the CHASE Phase 2b clinical trial; whether interim data from a clinical trial will be predictive of the results of the trial; uncertainties associated with regulatory review of clinical trials and applications for marketing approvals; Kala's ability to retain and hire key personnel; the impact of extraordinary external events, such as the current pandemic health event resulting from the coronavirus (COVID-19), and their collateral consequences; the sufficiency of cash resources and need for additional financing and other important factors, any of which could cause Kala's actual results to differ from those contained in the forward-looking statements, discussed in the "Risk Factors" section of Kala's Annual Report on Form 10-K and other filings Kala makes with the Securities and Exchange Commission. These forward-looking statements represent Kala's views as of the date of this press release and should not be relied upon as representing Kala's views as of any date subsequent to the date hereof. Kala does not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

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