Kala Pharmaceuticals to Present Clinical Data for KPI-012, its Mesenchymal Stem Cell Secretome Product, for the Treatment of PCED at the 2022 ARVO Annual Meeting

May 1, 2022

-- Data Demonstrate Rapid and Complete Wound Healing, with Six of Eight (75%) Patients Achieving Complete Healing within Four Weeks; All Remained Healed Through End of Follow-Up --
-- All Patients with Pain at Baseline Reported Zero Pain by Week 3 --
-- KPI-012 was Well-Tolerated with No Significant Safety Issues Observed --
-- On Track to Submit Investigational New Drug Application (IND) and Initiate Phase 2/3 Trial in 4Q 2022 --

ARLINGTON, Mass., May 01, 2022 (GLOBE NEWSWIRE) -- Kala Pharmaceuticals, Inc. (NASDAQ:KALA), a commercial-stage biopharmaceutical company focused on the discovery, development and commercialization of innovative therapies for diseases of the eye, today reported clinical data from a Phase 1b trial of KPI-012, its novel, cell-free secretome therapy for the treatment of severe ocular diseases driven by impaired healing. As previously disclosed, treatment with KPI-012 was well tolerated and resulted in significant improvements in patients with various persistent corneal epithelial defect (PCED) etiologies, with complete healing of the PCED in six of eight evaluable patients. The data will be presented on Tuesday, May 3 in a poster session at the 2022 Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting.

“The clinical activity observed to date with KPI-012 is encouraging,” said Valeria Sánchez-Huerta, M.D. FACS, Medical Director at Asociación para Evitar la Ceguera en México (Association to Prevent Blindness in Mexico) and an investigator in the Phase 1b trial. “PCED is a disease of impaired corneal healing which, if left untreated, can lead to infection, corneal perforation and irreversible vision loss. Achieving rapid and complete wound healing, as well as a reduction in PCED-related pain, and an improvement in visual acuity and corneal opacity, in patients with a range of underlying etiologies is remarkable, particularly after such a short treatment duration. Based on these early data, I believe KPI-012 could become the first treatment to address PCED across all etiologies and I look forward to further evaluating its potential in later-stage studies.”

PCED, 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. 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 can lead to corneal ulceration, perforation, stromal scarring, secondary infections and significant vision loss.

Healing after corneal injury follows a highly coordinated process involving growth factors, cell signaling, proliferation, migration and extracellular matrix remodeling. In patients with PCED, there is an imbalance of key biomolecules, including growth factors and cytokines, which results in significant inflammation, impaired innervation and disruption of the protective corneal epithelial and stromal layers. KPI-012 was designed specifically to address this imbalance: it is a novel, human bone marrow-derived mesenchymal stem cell (MSC) secretome containing numerous human biomolecules, including protease inhibitors, matrix proteins, growth factors and neurotrophic factors, that provide a multifactorial mechanism of action to address impaired corneal healing across numerous etiologies.

“We are pleased to present these exciting data from the first KPI-012 clinical trial,” said Kim Brazzell, Ph.D., Head of Research and Development and Chief Medical Officer at Kala Pharmaceuticals. “These data, which served as the foundation for our acquisition of Combangio last year, highlight the potential of KPI-012 to deliver a novel approach to treating PCED, as well as other rare front and back of the eye diseases. Our goal remains to advance KPI-012 into a Phase 2/3 trial for PCED later this year, as we aim to deliver new and better options to people living with severe ocular surface diseases.”

The poster presentation is now available on the Kala Pharmaceuticals website at https://investors.kalarx.com/presentations.

Highlights from the ARVO Presentation

The single-arm, prospective, open-label Phase 1b clinical trial enrolled 12 patients, including three who were enrolled in a safety lead-in cohort and nine enrolled in an efficacy cohort. Within the efficacy cohort, patients presented with PCED of various etiologies and durations ranging from 15 to 871 days. Patients were treated with twice daily KPI-012 for up to four weeks, with follow-up occurring at two, four and 12 weeks after their last dose of therapy. The key efficacy endpoint was complete healing of the corneal defects evaluated by corneal staining. Other efficacy endpoints included reduction in defect size, visual acuity, and corneal opacity. Safety measures included tolerability/pain, intraocular pressure and adverse events.

Eight patients were evaluable for efficacy assessment; one participant was ineligible due to a non-treatment related adverse event. Improvement was seen in seven of the eight evaluable patients, with six of the eight achieving complete healing by the end of Week 4, including four patients who were healed by the end of Week 1 and one patient who was healed by the end of Week 2. All six healed patients remained healed through the end of the follow-up period. In addition, improvement in PCED lesion size was observed in both patients who did not experience full wound healing. Across all eight patients, the mean improvement in lesion size from baseline to end of treatment was -16.23 mm. KPI-012 was well-tolerated in the trial.

Clinical Development Plans

Kala plans to file an investigational new drug (IND) application with the U.S. Food and Drug Administration (FDA) and, subject to regulatory clearance, initiate a Phase 2/3 clinical trial of KPI-012 in PCED patients in the fourth quarter of 2022. Kala believes this trial could serve as the first of two required pivotal trials. The FDA has granted KPI-012 Orphan Designation for the treatment of PCED and the Company believes it could also meet the criteria for fast-track and breakthrough designations.

In addition, Kala believes the multifactorial mechanism of action of KPI-012 also makes it a platform technology and is evaluating KPI-012 for potential

expansion to indications for rare front of the eye diseases, such as limbal stem cell deficiency and Sjogren's Syndrome, as well as select rare back of the eye diseases, such as retinitis pigmentosa and optic neuritis.

About Kala Pharmaceuticals, Inc.

Kala is a commercial-stage biopharmaceutical company focused on the discovery, development, and commercialization of innovative therapies for diseases of the eye. Kala has applied its AMPPLIFY® mucus-penetrating particle (MPP) Drug Delivery Technology to two ocular therapies, EYSUVIS® (loteprednol etabonate ophthalmic suspension) 0.25% and INVELTYS® (loteprednol etabonate ophthalmic suspension) 1%. The Company also has a pipeline of development programs including a clinical-stage secretome product candidate, KPI-012, initially targeting persistent corneal epithelial defects (PCED) and multiple proprietary new chemical entity (NCE) preclinical development programs targeted to address unmet medical needs, including both front and back of the eye diseases. For more information on Kala, please visit www.kalarx.com.

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 Kala's expectations with respect to KPI-012, the future development or commercialization of KPI-012, conduct and timelines of clinical trials, Kala's plans to progress its pipeline of preclinical development programs targeted to address front and back of the eye diseases, 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 those discussed in the "Risk Factors" section of Kala's Annual Report on Form 10-K, most recently filed Quarterly Report on Form 10-Q and other filings Kala makes with the Securities and Exchange Commission. These forward-looking statements represent the Company's views as of the date of this release and should not be relied upon as representing the 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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