Press Release

Kubota Vision Announced End of Phase 3 Clinical Trial of Emixustat in Patients with Stargardt Disease

SEATTLE (June 23, 2022) — Kubota Vision Inc. ("Kubota Vision"), a clinical-stage ophthalmology company and wholly-owned subsidiary of Kubota Pharmaceutical Holdings Co., Ltd. (Tokyo 4596), announced today the completion of the phase 3 clinical trial investigating emixustat hydrochloride ("emixustat") in patients with macular atrophy secondary to Stargardt disease.

The study was a multi-center, randomized, double-masked, and placebo-controlled phase 3 clinical study in which subjects were randomly assigned to emixustat 10 mg or placebo (2:1 ratio) once daily for 24 months. The target total number of subjects was 162; however, due to high interest in the study, a total of 194 subjects were enrolled in this study across 29 sites in 11 countries worldwide. The last patient has completed the study. The primary objective of this study is to determine if emixustat reduces the rate of macular atrophy progression, in comparison to placebo, in subjects with Stargardt disease. Secondary objectives include assessing changes in visual function parameters such as BCVA (best-corrected visual acuity) letter score and reading speed. The database of this study will be locked in the third quarter 2022.

Ryo Kubota, MD, PhD, Chairman, President and CEO of Kubota Vision Inc., stated, "Despite Stargardt being a rare disease, we were able to enroll more patients than originally planned and feel grateful that the patients trusted our drug with great expectation and continued taking the drug for two years during the pandemic. We would like to sincerely thank all of the participants and their families for their cooperation and are very pleased that the long-term, large-scale clinical trial has been successfully completed. We are also looking forward to receiving the results of the trial in the coming months."

The FDA (U.S. Food and Drug Administration) and EMA (European Medicines Agency) granted orphan drug designation to emixustat for the treatment of Stargardt disease. (See January 5, 2017 press release titled "Acucela Receives Orphan Drug Designation from the FDA for the Treatment of Stargardt Disease" and June 9, 2019 press release titled "Acucela Receives Orphan Designation from the EMA for Emixustat for the Treatment of Stargardt Disease")

About Stargardt Disease

Stargardt disease is a rare, genetically inherited disease that directly affects the retina of the eye, often resulting in the slow progression of vision loss in children. It may also be referred to as Stargardt macular dystrophy or juvenile macular degeneration and affects approximately 1 in 8,000 - 10,000 individuals worldwide.[*1] The most common form of the disease is caused by a genetic mutation of the ABCA4 gene leading to the accumulation of toxic vitamin A byproducts (primarily A2E) in the retina, which results in the gradual deterioration of photoreceptors and vision. Symptoms of Stargardt disease typically appear during childhood or adolescence, but in some cases difficulty with eyesight and vision loss may not be identified until later in life.

Stargardt disease affects less than 150,000 patients in total in the U.S., Europe and Japan where it is recognized as an orphan disease. Currently, there are no known therapies that slow the advance of the disease, and it is recognized as a serious unmet medical need.

[*1] Facts About Stargardt Disease, National Eye Institute. https://nei.nih.gov/health/stargardt/star_facts, accessed on 14 September 2018.

About Emixustat Hydrochloride

Emixustat modulates the visual cycle by inhibiting a critical enzyme of this pathway, retinal pigment epithelium protein 65 (RPE65). The visual cycle is the process by which vitamin A is recycled in the eye; vitamin A is crucial to the visual process. Slowing the visual cycle reduces the availability of vitamin A derivatives (11-cis- and all-trans-retinal) to form precursors of toxic A2E and related compounds. In addition, reducing the availability of 11-cis-retinal decreases retinal metabolic demands under dark conditions. Emixustat when delivered orally was found to be generally well tolerated in human clinical studies with delayed dark adaptation being the most common adverse event. Kubota Vision is exploring emixustat's potential to stop or slow the progression of vision loss in patients diagnosed with Stargardt disease in an ongoing clinical study.

About Kubota Vision Inc.

Kubota Vision Inc. is a wholly owned subsidiary of Kubota Pharmaceutical Holdings Co., Ltd. (Tokyo 4596) committed to translating innovation into a diverse portfolio of drugs and devices to preserve and restore vision for millions of people worldwide. Kubota Pharmaceutical group's development pipeline includes drug candidates for the treatment of diabetic retinopathy and Stargardt disease. The company is also developing a handheld OCT device for the monitoring of neovascular retinal diseases, to be used directly by patients, and wearable device for myopia control. https://www.kubotavision.com/; https://www.kubotaholdings.co.jp/en/

Cautionary Statements

Certain statements contained in this press release are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934 and the Private Securities Litigation Reform Act of 1995. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. These forward-looking statements include statements regarding our expectations related to our development plans and ability to successfully develop and commercialize our product candidates and the potential efficacy, future development plans and commercial potential of our product candidates. These statements are based on current assumptions that involve risks, uncertainties and other factors that could cause the actual results, events or developments to differ materially from those expressed or implied by such forward-looking statements. These risks and uncertainties, many of which are beyond our control, include, but are not limited to: our investigational product candidates may not demonstrate the expected safety and efficacy; our pre-clinical development efforts may not yield additional product candidates; any of our or our collaborators' product candidates may fail in development, may not receive required regulatory approvals, or may be delayed to a point where they are not commercially viable; our clinical trials could be delayed; new developments in the intensely competitive ophthalmic pharmaceutical market may require changes in our clinical trial plans or limit the potential benefits of our investigational product candidates; the impact of expanded product development and clinical activities on operating expenses; adverse conditions in the general domestic and global economic markets; as well as the other risks identified in our filings with the Securities and Exchange Commission. These forward-looking statements speak only as of the date hereof and we assume no obligation to update these forward-looking statements, and readers are cautioned not to place undue reliance on such forward-looking statements. For a detailed discussion of the foregoing risks and other risk factors, please refer to our

filings with the Securities and Exchange Commission, which are available on Kubota Pharmaceutical Holdings (Kubota Vision's parent company) investor relations website (https://www.kubotaholdings.co.jp/en/ir/) and on the SEC's website (http://www.sec.gov).

Media and Investor Relations Contact:

Press and Media Inquiries
Phone: +81-3-6550-8928
Email: pr@kubotaholdings.co.jp

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