



Kubota Vision Receives Orphan Products Clinical Trials Grants to Emixustat for Stargardt Disease

SEATTLE (Aug 19, 2020) — Kubota Vision Inc. (“Kubota Vision”), a clinical-stage ophthalmology company and wholly-owned subsidiary of Kubota Pharmaceutical Holdings Co., Ltd. (Tokyo 4596), announced today that the U.S. Food and Drug Administration (FDA) Office of Orphan Products Development (OOPD) has awarded an orphan products clinical trial grant to support the ongoing phase 3 study of emixustat in Stargardt disease (Clinicaltrials.gov identifier: NCT03772665).

The FDA OOPD was established in 1983 to advance the evaluation and development of drugs, biologics, medical devices and medical foods that demonstrate promise for the diagnosis and/or treatment of rare diseases and conditions. Orphan product clinical trial grants support well-controlled studies that will either result in market approval of these products or substantially contribute to the essential data needed for medical product development that will ultimately meet the needs of rare disease patients. The total fund Kubota Vision receives in three years will be up to \$1.6 million in total.

The study, initiated on November 7, 2018, is a multi-center, randomized, double-masked, and placebo-controlled phase 3 clinical study in which subjects are randomly assigned to emixustat 10 mg or placebo (2:1 ratio) once daily for 24 months. After all subjects have completed the study, the database will be locked and top-line data readout will begin.

Ryo Kubota, MD, PhD, Chairman, President and CEO of Kubota Vision Inc., stated, “It is a great pleasure and honor to receive this grant from the FDA for our ongoing phase 3 study of emixustat in Stargardt disease. The FDA awards this grant to approximately 15% of all applicants each year, whom the FDA recognizes as a great potential to treat rare diseases. We will continue our dedication to advancing this program and enforce our commitment to finding a treatment for this disease.”

The FDA and European Medicines Agency (EMA) 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 U.S., Europe and Japan where it is recognized as an orphan disease. Currently, there are no known therapies that exist to 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 include drug candidates for the treatment of diabetic retinopathy, Stargardt disease, and optogenetics-based gene therapy for the treatment of retinitis pigmentosa. 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>).

“Kubota Vision”, the Kubota Vision logo and “Kubota” are registered trademarks or trademarks of Kubota Vision Inc. or Kubota Pharmaceutical Holdings in various jurisdictions.

Media and Investor Relations Contact:

Hiroki Maekawa
Chief Financial Officer
Phone: +81-3-6550-8928
Email: pr@kubotaholdings.co.jp

###