Press Release

Kubota Vision Announces Publication of Emixustat's Pharmacodynamic Effects in Patients with Stargardt disease

Seattle (November 23, 2020) — Kubota Vision Inc. ("Kubota Vision"), a clinical-stage ophthalmology company and wholly-owned subsidiary of Kubota Pharmaceutical Holdings Co., Ltd. (Tokyo 4596), today announced the publication of results from a phase 2a clinical trial of emixustat hydrochloride (emixustat) in patients with macular atrophy (MA) secondary to Stargardt disease (STGD) in the medical journal British Journal of Ophthalmology.

The published article, "Randomized study evaluating the pharmacodynamics of emixustat hydrochloride in subjects with macular atrophy secondary to Stargardt disease," can be accessed at: https://bjo.bmj.com/content/early/2020/11/18/bjophthalmol-2020-317712. For additional information about the British Journal of Ophthalmology, please visit: https://bjo.bmj.com.

In this study, 23 subjects with MA secondary to STGD were randomized to once-daily 2.5 mg, 5 mg, or 10 mg emixustat and treated for one month to assess the effects of emixustat on the recovery rate of the rod photoreceptor b-wave on electroretinography after photobleaching, an indirect measure of inhibition of emixustat's target enzyme, RPE65. Subjects randomized to 10 mg demonstrated near complete suppression of this recovery rate, indicating robust RPE65 inhibition, while those in the 5 mg group demonstrated moderate suppression. No effects were seen in the 2.5 mg group. Adverse events observed were consistent with prior clinical trials and consisted primarily of ocular adverse events. The results of this study informed the dose selection for the ongoing 24-month phase 3 clinical trial (SeaSTAR Study; clinicaltrials.gov identifier: NCT03772665) in subjects with MA secondary to STGD.

The U.S. Food and Drug Administration (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") Also, the 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. (See August 20, 2020 press release titled "Kubota Vision Receives Orphan Products Clinical Trials Grants to Emixustat for Stargardt Disease")

Ryo Kubota, MD, PhD, Chairman, President and CEO of Kubota Vision Inc., stated, "We are so grateful being recognized and acknowledged for our innovation not only by the FDA, who recently announced to award grant to support our ongoing clinical trial, but also in an peer-reviewed ophthalmology scientific journal. Emixustat has a first-in-class technology delivered orally to treat eye diseases. We continue striving to pursue our innovative drug development and bring the drug to patients in need."

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 includes 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).

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