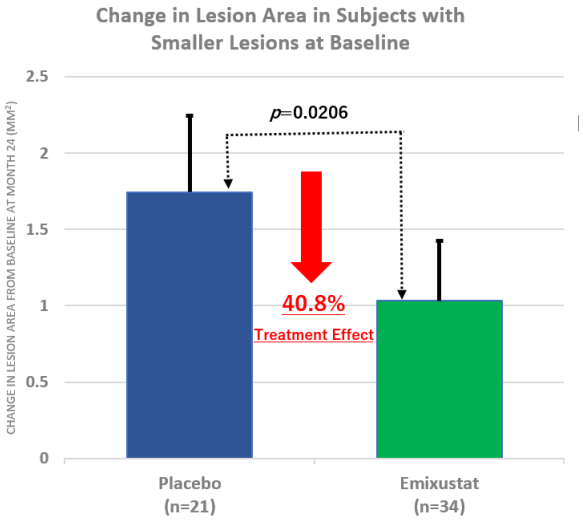
Press Release

Kubota Vision Announces Positive Post Hoc Analysis from Phase 3 Clinical Trial of Emixustat in Patients with Stargardt Disease

SEATTLE (October 2, 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 positive results from a post hoc analysis of the phase 3 clinical trial of the investigational visual cycle modulator emixustat hydrochloride (emixustat) in patients with Stargardt Disease.

The primary objective of this study was to determine if emixustat reduces the rate of macular atrophy progression, in comparison to placebo, in subjects with Stargardt disease. Previously announced top-line results showed that the study did not achieve its pre-planned primary or secondary endpoints. Prompted by planned subgroup analyses suggesting that emixustat subjects with smaller atrophic lesions at baseline had a reduced rate of progression compared to placebo subjects, post hoc analyses were performed to further investigate this finding. A multi-factor analysis was performed on the subgroup of subjects with smaller lesions at baseline, controlling for the baseline factors identified in univariate and multi-factor analyses to affect lesion progression in this subgroup. This analysis determined that emixustat treatment resulted in a 40.8% reduction in lesion progression compared to placebo at Month 24 (p=0.0206, emixustat n=34, placebo n=21).



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 total target 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.

Ryo Kubota, MD, PhD, Chairman, President, and CEO of Kubota Vision Inc., stated, "We are very excited to see strong efficacy in this subgroup. The fact that we have shown a positive effect in an earlier stage of a disease is very similar to what has been observed in drug development for other neurodegenerative diseases such as Alzheimer's disease; this offers the potential to treat these patients

proactively with the goal to preserve vision. We will move forward quickly to develop emixustat in this population and will seek a partner for this development."

The Company is carefully examining the impact on the consolidated earnings forecast for the fiscal year ending December 31, 2022 (January 1, 2022 to December 31, 2022) and will be promptly disclosed if a revision to the financial forecast becomes necessary.

About Funding for this Clinical Trial

As previously announced in August 2020, the FDA Office of Orphan Products Development (OOPD) awarded Kubota Vision an orphan products clinical trial grant to support this clinical trial. Specifically, this clinical trial was supported by the FDA of the U.S. Department of Health and Human Services (HHS) as part of a financial assistance award (FAIN: R01FD006849) totaling $1,633,917 with 6.7% funded by FDA/HHS and $22,858,571 (93.3%) funded by Kubota Vision. The contents of this press release are those of Kubota Vision and do not necessarily represent the official views of, nor an endorsement, by FDA/HHS, or the U.S. Government.

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. Stargardt disease market is expected to grow to US$1,600M in 2027.[*2]

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

[*2] WISEGUY RESEARCH CONSULTANTS PVT LTD Global Juvenile Degeneration (Stargardt Disease) Market Research Report-Forecast to 2027.

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.

The FDA and 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 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 pipeline includes a wearable device for myopia control using Kubota Glass™ technology and a handheld OCT device for the monitoring of neovascular retinal diseases, to be used directly by patients.
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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