ACUCELA

Press Release

Acucela Provides Update on Emixustat Phase 3 Clinical Trial in Patients with Stargardt Disease

SEATTLE (February 12, 2020) — Acucela Inc. ("Acucela"), a clinical-stage ophthalmology company and wholly-owned subsidiary of Kubota Pharmaceutical Holdings Co., Ltd. (Tokyo 4596), announced today that the company has achieved over 65% subject enrollment in its ongoing phase 3 clinical trial investigating emixustat hydrochloride ("emixustat") in patients with macular atrophy secondary to Stargardt disease.

The study 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. For a rare disease like Stargardt, enrolling subjects into a clinical study can be challenging. A total of 108 patients have been enrolled in this study as of January 31, 2020, and the target total of 162 subjects are expected to be fully enrolled across 30 sites in 11 countries worldwide during 2020.

The FDA (U.S. Food and Drug Administration) and European Medicines Agency (EMA) granted orphan drug designation to emixustat for the treatment of Stargardt disease.

This projection has been included in the financial results of FY2019 announced today, February 13, 2020 in Japan.

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. Acucela 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 Acucela Inc.

Acucela 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. Acucela'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. https://www.acucela.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 (Acucela'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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