ACUCELA

Press Release

Acucela and SIRION Biotech Set Sights on Gene Delivery Tools for Ocular Gene Therapy

SEATTLE (January 8, 2018) — Acucela Inc. ("Acucela"), a clinical-stage ophthalmology company and 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, announced today that it signed a two year development agreement with gene delivery expert SIRION Biotech GmbH ("SIRION") to establish optimized AAV vectors for clinical applications in ocular gene therapy. With this cooperative effort, Acucela bolsters its move into the fast growing gene therapy market to find a genetic cure for retinitis pigmentosa (RP). Key terms of the agreement include milestone payments to SIRION, with additional royalties to be paid on net sales from resulting products or therapies.

RP is the most common form of inherited retinal degeneration. Onset is usually in childhood and progresses to complete blindness by around age 40. It is estimated that approximately 1 in 4000 people are affected in their lifetime[(1)], and approximately 1.5 million people worldwide are affected by the disease[(2)]. RP describes a family of slowly progressing diseases that are caused by a variety of autosomal dominant, autosomal recessive and x-linked mutations that lead to photoreceptor degeneration. It is a complex indication with over 100 pathogenic mutations identified[(3)]. To date, no pharmacological treatment is available.

Recombinant adeno associated virus (rAAV) vectors are considered the best and most promising gene delivery system for therapeutic applications and have been shown in preclinical experiments to successfully deliver human rhodopsin (hRho) to the retina. Acucela, together with SIRION and an academic partner consortium, aims to develop the next generation of rAAV vectors. The goal is to secure new and modified AAV capsids that will ensure that the therapeutic viral particles exhibit a safe product profile with improved specificity for therapeutic protein delivery, over wild type vectors, that is needed to effectively restore light sensitivity to patients.

SIRION relies on 10 years of experience in viral vector engineering and leverages close ties to leading academics in Europe that will play a pivotal role in this project. World renowned experts in AAV biology, Prof Grimm from the Unversitätsklinikum Heidelberg and Prof. Büning of the Medizinische Hochschule Hannover as well as PD Dr. Michalakis from the Ludwig-Maximilians Universität München will contribute their individual expertise to this program.

"The academic acumen of our partners, together with our strong viral vector specialization and experience will empower our client to enter clinical trials with an efficient, safe and scalable product. For us it is the chance to participate in a fundamental step for gene therapies that can help millions of patients worldwide win back their eyesight. This is what we started SIRION Biotech for," said Dr. Christian Thirion, founder and CEO of the company.

Additionally, Dr. Ryo Kubota, MD, PhD, Chairman, President and CEO of Acucela stated, "We are pleased to advance our optogenetic genetherapy program in partnership with SIRION Biotech to bring a new therapeutic approach to RP patients suffering from this devastating disease."

(1) Genetics Home Reference, retinitis pigmentosa. https://ghr.nlm.nih.gov/condition/retinitis-pigmentosa. Retrieved Nov 7, 2016.
(2) Vaidya P, Vaidaya A. Retinitis Pigmentosa: Disease Encumbrance in the Eurozone. Int J Ophthalmol Clin Res. 2:030 (2015).
(3) National Human Genome Research Institute. Leaning About Retinitis Pigmentosa. https://www.genome.gov/13514348/. Retrieved Nov 7, 2016.

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 and therapeutics for the treatment of retinitis pigmentosa, proliferative diabetic retinopathy, diabetic macular edema, AMD, Stargardt disease, cataracts and presbyopia. The company is also developing a monitoring device for neovascular retinal diseases, to be used directly by patients. http://www.acucela.com; http://www.kubotaholdings.co.jp/en/

About SIRION Biotech GmbH

SIRION Biotech provides custom engineering services of viral vector strategies for research and development in the life sciences and industry. The Germany based technology experts provides small to large-scale projects to customers worldwide, with over 200 a year. SIRION Biotech's unique focus on gene delivery is why it is the only company able to master customization of all 3 major virus systems that are used regularly for therapeutic and research purposes, to genetically modify mammalian cells.
The company has a strong customer base in cancer research, neurosciences, regenerative medicines, gene therapy and immuno-oncology.

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 is available on the SEC's website (http://www.sec.gov).

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