



国立大学法人
東京医科歯科大学
TOKYO MEDICAL AND DENTAL UNIVERSITY

December 24, 2018

Rena Therapeutics Inc.

Tokyo Medical and Dental University

Rena Therapeutics and Ionis Pharmaceuticals Agree to
a Non-Exclusive License Agreement for Heteroduplex Oligonucleotide (HDO) Patents

Rena Therapeutics Inc. (President & CEO: Jun Sasaki, headquarters: Chiyoda-ku, Tokyo, hereinafter referred to as “Rena”), a bio-venture company authorized by Tokyo Medical and Dental University (hereinafter referred to as “TMDU”), announced today that it has executed a non-exclusive license agreement with Ionis Pharmaceuticals, Inc. (Chairman and CEO: Dr. Stanley T. Crooke, headquarters: Carlsbad, California, U.S.A., hereinafter referred to as “Ionis”) to research, develop, manufacture and commercialize novel nucleic acid drugs using HDO.

Ionis, the leader in RNA-targeted drug discovery and development, has created an efficient, broadly applicable, proprietary antisense technology platform with the potential to treat diseases where no other therapeutic approaches have proven effective. Utilizing its novel technology, Ionis discovered and developed SPINRAZA[®] (nusinersen)*, the first and only approved treatment for spinal muscular atrophy (SMA), and TEGSEDI[™] (inotersen), the world’s first RNA-targeted therapeutic approved for the treatment of polyneuropathy of hereditary transthyretin (TTR) amyloidosis (ATTR) in adults. Ionis has a pipeline of over 40 drugs with the potential to treat neurological, infectious and rare diseases and cancer. Several first-in-class or best-in-class therapies, including treatments for cardiovascular disease, Huntington’s Disease, and amyotrophic lateral sclerosis (ALS) are nearing pivotal trials.

Through the agreement with Ionis, Rena hopes to accelerate the research and development of novel nucleic acid drugs using HDO. Rena will receive a licensing fee as well as milestone payments and royalties based on the progress of development and commercial sales of HDO drugs. The parties agreed not to disclose financial terms.

Antisense Oligonucleotide (hereinafter referred to as "ASO") and siRNA (Short-interfering RNA) therapeutics are capable of treating a range of diseases by controlling specific genes. HDO is a third nucleic acid pharmaceutical platform with a new molecular structure and mechanism of action which is different from the nucleic acid pharmaceutical platforms such as ASO and siRNA.

Rena is committed and responsible for creating novel nucleic acid drugs using HDO, the third nucleic acid pharmaceutical platform and providing them to patients. Rena believes it is important to cooperate with companies that can share its commitment. The execution of this agreement is consistent with our commitment.

[About Rena]

Rena is a Japanese corporation established in 2015, a bio-venture company authorized by TMDU, having its office at Otemachi Financial City Grand Cube 3F, 9-2, Otemachi 1-chome, Chiyoda-ku, Tokyo 100-0004, Japan.

Nucleic acid drugs have been recognized issues such as delivery to a disease site, side effects and stability in blood after administration, but on the other hand, they have advantage to be able to approach to diseases in which they were difficult to apply therapy using low-molecular-weight compounds, antibodies, etc.

HDO has a structure combining an antisense strand (= active chain) (double stranded upper part) functioning as an active body and a carrier chain (lower part) containing a ligand for carrying an antisense strand to a disease site. By adopting such a structure, it is expected that the antisense effect can be implemented more effectively in the target organ than the conventional single-stranded antisense nucleic acid.



Rena has a business model that provides solutions and services based on HDO technology as a platform to customers and receives consideration.

After establishing in January 2015, Rena has raised funds twice. As a result, we will strengthen the HDO technology, we have already signed collaborative research agreements with two major pharmaceutical companies already, and will continue to increase the number of contracts with pharmaceutical companies and will further strengthen our business foundation.



国立大学法人
東京医科歯科大学
TOKYO MEDICAL AND DENTAL UNIVERSITY

Rena is committed and responsible for creating novel nucleic acid drugs using HDO in order to respond to unmet medical needs such as cancer, neurodegenerative diseases, hereditary diseases and other difficult diseases.

*Marketed by Biogen

(For inquiries about this announcement)

Contact Manager: Shoichi Iwamoto, General Manager

Rena Therapeutics Inc.

Phone number +81-3-4243-6081

E-mail: info@renatherapeutics.com

(Inquiries concerning research contents)

Professor Takanori Yokota

Department of Neurology and Neurological Science,

Graduate School of Medical and Dental Sciences,

Tokyo Medical and Dental University (TMDU)

Phone number +81-3-5803-5233

E-mail: yokonuro@tmd.ac.jp

End of announcement