July 10th, 2024

Company name: Modalis Therapeutics Corporation
Stock exchange listing: Tokyo Stock Exchange
Code number: 4883
URL: https://www.modalistx.com/en/
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Modalis and GENIXCURE enter into Memorandum of Understanding (MOU) for Research Collaboration to Develop Next-Generation Therapeutics for Alzheimer's Disease

Modalis-GENIXCURE Sign MOU

July 10th, 2024
TOKYO & WALTHAM, Mass. Modalis Therapeutics Corporation (Modalis) (Tokyo Stock Exchange: 4883), a biotechnology company developing novel genetic medicines, today announced that it has entered into a Memorandum of Understanding (MOU) with GENIXCURE Inc. (genix-cure.com), which develops novel adeno-associated virus (AAV) vectors and therapeutics using a proprietary AI system. Based on this MOU, Modalis will provide MDL-104, generated with its proprietary epigenome editing technology, CRISPR-GNDM® (Guide Nucleotide-Directed Modulation), to GENIXCURE who will use MDL-104 with their proprietary CNS tropic AAV capsid, XOB031, to research and develop a novel gene therapy for the treatment of Alzheimer's disease.

GENIXCURE, founded in 2019, has its headquarters and R&D center in Suwon, South Korea, and develops gene therapies using its proprietary AAV vectors from the AI-based AAV Capsid Engineering Platform, CARE™ (Cell-specific AAV Research Engine), It also develops AAV gene therapies for the treatment of CNS disease such as Alzheimer's disease, ALS, Lafora Disease, and Frontotemporal Dementia. Being equipped with proprietary facilities and SOPs for varieties of serotypes, it provides one-stop CMO services covering AAV manufacturing, purification, and analysis. As a whole, the company leverages CARE™ for both proprietary and partnered therapeutic programs with its own CMC know-how, for all-in-one preclinical development of AAV gene therapy..

Modalis was founded in 2016, based on technology from Univ. of Tokyo, and conducts its research and development in Waltham, Massachusetts, USA. The Company develops therapeutics for patients suffering from serious genetic disorders such as muscle diseases, CNS diseases, and cardiomyopathies.

MDL-104, which uses naturally occurring AAV9 capsid as a drug delivery technology, has demonstrated significant suppression of the expression of intracellular Tau RNA and protein

in target tissues and cells within the CNS upon intra-cisterna magna (ICM) administration in mouse disease models. Through a collaborative relationship under this MOU, Modalis and GENIXCURE will conduct joint research and development with the aim of transducing high levels of MDL-104 to the brain, with limited exposure to non-targeted tissues, and demonstrating long-term efficacy and safety in murine and large animal species, including NHPs, using a minimally invasive intravenous injection (IV) administration method. The combined expertise of both companies will leverage their experience in AAV engineering and epigenetic modulation to develop best-in-class, IV administered therapies for Alzheimer's Disease and other Tauopathies.

About Lafora disease

Lafora disease is one of the causative agents of progressive myoclonic epilepsy (PME), a progressive neurodegenerative disorder with autosomal recessive inheritance that presents with myoclonus, seizures, cerebellar ataxia, and intellectual disability. It is diagnosed by the deposition of periodic acid Schiff (PAS)-stain-positive aggregates in neurons and myocardial cells, and was previously diagnosed by demonstrating Lafora bodies in skin and nerve biopsies. In recent years, the causative gene has been identified, and genetic diagnosis is now being performed at some specialized facilities.

Contacts
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