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April 30th, 2024

Company name: Modalis Therapeutics Corporation
Stock exchange listing: Tokyo Stock Exchange
Code number: 4883
URL: https://www.modalistx.com/en/
Representative: Haruhiko Morita

Modalis Therapeutics to Present Data Supporting Development of Transformative Epigenome Editing Medicines for the Treatment of Muscular Dystrophy at The Global Cell & Gene Therapy Summit

30-Apr-2024 TOKYO & Waltham, Mass – Modalis Therapeutics Corporation (Tokyo Stock Exchange: 4883), a pioneering company developing innovative products for the treatment of rare genetic diseases utilizing its proprietary CRISPR-GNDM® epigenome editing technology, announced that the company has been accepted for an Oral Presentation at the Global Cell & Gene Therapy Summit (July 8-10, 2024, Boston, USA), and the following research results will be presented by our CSO, Dr. Tetsuya Yamagata.

- *Product concept and rationale for MDL-101, a differentiated precision medicine for LAMA2-congenital muscular dystrophy (LAMA2-CMD)*
- *Preclinical data supports efficacy in LAMA-2 knockout mice (dyW disease model mice) and target engagement in non-human primates (NHPs).*
- *The possibility that our world-leading CRISPR-based Epigenome editing technology may show clinical efficacy as a therapeutics approach to treat serious genetic diseases.*

At the Global Cell & Gene Therapy Summit, Modalis will present preclinical data from MDL-101 validating the efficacy and safety of our differentiated therapeutic strategy utilizing our CRISPR-GNDM® technology for the difficult-to-treat LAMA2-CMD. "Our proprietary and first-in-class CRISPR-based epigenome editing technology, CRISPR-GNDM®, controls the expression levels of disease-causing genes and provides a disease-modifying treatment for genetic disorders," said Dr. Tetsuya Yamagata, CSO of Modalis. "MDL-101 has raised the probability of technical success as a potentially life-changing gene therapy for the treatment of LAMA2-CMD and is currently on track for IND filing. At this meeting, we will share data from our mouse and NHP studies that demonstrate efficacy, safety, and manufacturability, and discuss the potential implications for therapeutic efficacy in the clinic." stated.

The Modalis Therapeutics presentation will be at the Global Cell & Gene Therapy Summit （URL；https://cgtsummit.com）

Oral Presentation:

Title: Epigenetic Editing with CRISPR-GNDM®: Novel Muscle-tropic AAV Vectors Deliver Promising Single-dose Treatment for LAMA2-CMD
Date and Time: 7/8/2024, 10-12M EST (TBD)
Session Name: Gene Editing Breakthroughs & Therapeutics Approaches

About The Global Cell & Gene Therapy Summit

Cell & Gene therapy is rapidly developing as transformative novel therapy that can address unmet medical needs for a variety of conditions. However, Cell & Gene therapy is more expensive and requires more precise control than existing modalities, which poses challenges in terms of patient accessibility and industrial use. Therefore, this conference will bring together global leaders from academia and industry to exchange knowledge and views, including the latest technological developments, market strategies, social implications, and moral conditions, in order to promote partnerships between academia and industry to achieve the commercialization of Cell & Gene therapy.

About MDL-101

MDL-101 is an experimental, epigenome modulation therapy under investigation for the treatment of LAMA2-Congenital Muscular Dystrophy (LAMA2-CMD). MDL-101 is comprised of guide nucleotide targeting LAMA-1 gene, a highly homologous sister gene of the disease-causing gene LAMA-2, enzyme-null Cas9 (dCas9) fused with trans-activating domain driven by a muscle-specific promoter and coded in a muscle-specific AAV vector. MDL-101 upregulates LAMA-1 gene products in patients' muscle tissue to compensate for loss-of-function caused by mutation of LAMA-2, and therefore has the potential to provide a one-time, durable treatment benefit for people living with LAMA2-CMD.
About Modalis:

Modalis Therapeutics develops precision genetic medicines using epigenome editing technology. Modalis is pursuing therapies for orphan genetic diseases using its proprietary CRISPR-GNDM® technology which enables the gene/locus-specific modulation of gene expression or epigenetic editing without the need for DNA cleavage or altering DNA sequence. Headquartered in Tokyo with laboratories and facilities in Waltham Massachusetts, the company is listed on Tokyo Stock Exchange's Growth market. For additional information, visit www.modalistx.com.