April 19, 2023

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 of Development of Transformative Epigenetic Editing Medicines for the Treatment of a Type of Muscular Dystrophy at the ASGCT Annual Meeting

MDL-101 preclinical data support efficacy and safety of a differentiated precision medicine approach for LAMA2 Congenital Muscular Dystrophy (LAMA2-CMD)

Preclinical data showing that our CRISPR-based epigenetic editing technology regulates the expression of target genes in mammals, suggesting the possibility of clinical efficacy as a therapeutic approach for serious genetic disorders

19-Apr-2023 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® epigenetic editing technology, today announced that the abstract has been accepted for a presentation in the late-breaking session* at the 26th Annual Meeting of The American Society of Gene & Cell Therapy (ASGCT), being held in Los Angeles CA on May 16-20, 2023. The abstracts present preclinical data from the Company's LAMA2 Congenital Muscular Dystrophy (LAMA2-CMD).

Modalis presentations at ASGCT will include preclinical data demonstrating that:

- In LAMA-2 knockout mice (dyW disease model mice) and non-human primates (NHPs), a CRISPR-GNDM® based molecule (MDL-101) targeting the LAMA-1 gene introduced into a muscle-specific AAV vector raised LAMA-1 expression to levels that complement LAMA-2 function in the disease animals. This suggests that MDL-101 may have therapeutic potential in the clinic.

At ASGCT, we will present preclinical data from MDL-101 validating the efficacy and safety of our differentiated therapeutic strategy utilizing our CRISPR-GNDM® technology for the previously difficult-to-treat LAMA2-CMD. "Our proprietary and world-first CRISPR-based epigenetic editing technology, CRISPR-GNDM®, controls the expression levels of disease-causing genes and provides a disease-modifying treatment for genetic disorders," said Haru Morita, CEO of Modalis. "MDL-101 has raised its firepower as a potentially life-changing gene therapy for the treatment of LAMA2-CMD by employing a muscle-specific capsid that further increases the selectivity for muscle tissue. Development is currently on track for IND filing by the end of 2024. At this meeting, we will share data from our mice and NHPs studies that have shown efficacy and safety and discuss the potential implications for therapeutic efficacy in the clinic." stated.

* Late-Breaking Abstract session： Sessions established to present data recognized as high impact, groundbreaking, innovative, and newsworthy after the normal abstract submission deadline

The Modalis Therapeutics presentation is as below. The abstract will become available on the ASGCT website from May 12th and the presentations will be posted on the Modalis website during the conference.

Oral Presentation:

Title: Advancing Epigenetic Editing with CRISPR-GNDM: Novel Muscle-Tropic AAV Vectors Deliver Promising Single-Dose Treatment for LAMA2-CMD
Date and Time: 5/19/2023 10:45AM
Session Name: Late-breaking Abstracts 2

About MDL-101

MDL-101 is an experimental, epigenetic 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 epigenetic gene 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.