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April 30th, 2025

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 Preclinical Data on MDL-202, a gene therapy for Myotonic Dystrophy Type 1 (DM1) using CRISPR-GNDM® epigenome editing technology at ASGCT 28th Annual Meeting

30-April-2025 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 preclinical data on MDL-202, a gene therapy drug for myotonic dystrophy type 1 (DM1), and the study on the functional evaluation and optimization of a genetically engineered AAV vector utilizing the Nanopore sequencer, have been accepted for oral and poster presentations respectively at ASGCT 28th Annual Meeting (May 13-17, 2025, New Orleans, USA). DM1 is the most common form of muscular dystrophy in adults, with an incidence rate of approximately 5 cases per 100,000 people worldwide and is characterized by myotonia and muscle atrophy. The pathogenesis of DM1 is attributed to the abnormal expansion of CTG repeats in the 3' untranslated region of the DMPK gene. This expansion leads to the sequestration of proteins, such as MBNL1, that are involved in RNA splicing, thereby disrupting normal splicing processes in muscle cells.

Data to be presented demonstrates:

- *Identification of optimized gRNA and MDL-202 molecule with durable suppression of the target DMPK gene*
- *In vitro activity of MDL-202 using DM1 patient-derived muscle cells*
- *In vivo efficacy of MDL-202 with a muscle tropic AAV in DM1 disease mouse model*
- *A method to accurately evaluate and analyze, in a non-biased manner, the distribution of gene lengths in AAV vectors, using a nanopore sequencer, and a method to optimize engineered AAV vectors*

Oral Presentation:

Title: Treatment of Myotonic Dystrophy Type 1 (DM 1) by GNDM-mediated Suppression of the DMPK Gene

Date and Time: 5/17/2025, 10:45-11:00AM CST
Session Name: Gene Therapy for Muscle Diseases

Poster Presentation:

Title: Nanopore Sequencing and Screening of AAV Genomes for Optimal Production and Function

Date and Time: 5/13/2025, 6:00-7:00PM CST
Session Name: Poster Reception

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About ASGCT 28th Annual Meeting

ASGCT is a prominent international conference in the field of gene and cell therapy. The annual meeting is convened each May in the US. Researchers, physicians, pharmaceuticals, biotech, and regulatory authorities from all over the world to attend and give presentations on a wide range of subjects, from basic science research to clinical trials and regulatory trends. In recent years, there has been a great interest in cutting-edge gene therapy using next-generation CRISPR technology in the treatment of genetic diseases and cancer. Furthermore, it has become an important global platform where the pioneers of CRISPR research.

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.

For media contacts
Modalis Therapeutics
Sawako Nakamura
media@modalistx.com