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April 23rd, 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 MDL-202, a gene therapy for Myotonic Dystrophy Type 1 (DM1) using CRISPR-GNDM® epigenome editing technology at 2025 MDF Conference

23-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 utilizin4g its proprietary CRISPR-GNDM® epigenome editing technology, announced that it has been invited give an oral presentation at the 2025 MDF Conference (May 1-4, 2025, Indianapolis, USA). The following research results will be presented by the Company's Director, Molecular Biology and Genomics, Dr. Keith Connolly. Myotonic dystrophy type 1 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*

Oral Presentation:

Title: Myotonic dystrophy type 1 (DM1) treatment by CRISPR-GNDM® mediated suppression of DMPK mRNA

Date and Time: 5/1/2025, 3:20PM EST & 5/3/2025, 2:35-3:45PM EST
Session Name: Pharma Day and Industry Updates

https://www.myotonic.org/sites/default/files/2025-04/MDF-2025-Conference-Program.pdf

About 2025 MDF Conference

The Myotonic Dystrophy Foundation (MDF) is the leading global organization dedicated to connecting patients with myotonic dystrophy (DM) and their families with healthcare professionals and others to provide resources and support for the DM community. The MDF Conference is the largest event for DM patients and their families, researchers, clinicians, pharmaceutical, and biotech industry professionals in the DM community, with over 500 attendees. The Pharma Day, which takes place during the MDF Conference, will feature presentations on cutting-edge drug development in DM. (2025 MDF Conference | Myotonic Dystrophy Foundation)

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.

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