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April 28th, 2026

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 New Preclinical Data Demonstrating Robust Gene Suppression for MDL-202 (DM1) and MDL-103 (FSHD) at ASGCT 2026
— Next-generation CRISPR-GNDM[®] epigenome editing shows potential for best-in-class therapies —

TOKYO & WALTHAM, Mass., April 27, 2026 — Modalis Therapeutics Corporation (Tokyo Stock Exchange: 4883), a preclinical-stage biotechnology company developing precision gene therapies using its proprietary CRISPR-GNDM[®] epigenome editing technology, today announced that it will present new preclinical data demonstrating robust and sustained target gene suppression for MDL-202 (Myotonic Dystrophy Type 1, DM1) and MDL-103 (Facioscapulohumeral Muscular Dystrophy, FSHD) at the 29th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT), taking place May 11–15, 2026 in Boston, MA.

These results highlight the potential of CRISPR-GNDM[®] to achieve durable, muscle-selective gene suppression without inducing double-strand DNA breaks, supporting its differentiated safety and efficacy profile compared to conventional gene editing approaches.

Presentation Highlights

1. MDL-202 (Indication: DM1)

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.

The Company is developing MDL-202 using its proprietary CRISPR-GNDM[®] technology. This approach enables the muscle-selective and sustained suppression of the DMPK gene without causing double-strand DNA breaks, offering potential for significant functional improvement.

At this year's meeting, the Company will disclose latest data for its "Next-Generation MDL-202", which utilizes a muscle-tropic capsid (ATC-187) developed by its collaboration partner, Affinia Therapeutics. In mouse models, systemic administration of the Next-Gen MDL-202 achieved exceptional suppression of the DMPK gene, demonstrating its potential as a best-in-class therapeutics candidates.

2. MDL-103 (Indication: FSHD)

FSHD is the third most prevalent genetic dystrophy, affecting an estimated 1 million people worldwide, including approximately 38,000 in the United States. Characterized by progressive muscle wasting in the face, shoulders, and upper arms, approximately 20% of patients require a wheelchair by age 50. Currently, there are no approved therapies that address the root cause of FSHD.

MDL-103 leverages the CRISPR-GNDM[®] technology to achieve muscle-selective and sustained suppression of the Dux4 gene, the primary driver of FSHD pathology, without cutting the DNA.

The Company will present efficacy and safety results from studies using a Xenograft disease mouse model (human FSHD muscle cells transplanted into mice) developed by the University of Massachusetts. In this clinically relevant model, MDL-103 achieved significant suppression of Dux4 target genes at low doses, highlighting its potential as a best-in-class treatment for FSHD.

Poster Presentation:

ID 1492 Title: [Pairing AAV genome and capsid engineering of an epigenetic-editing modality to develop a best-in-class treatment for Myotonic Dystrophy Type 1 \(DM1\)](#)

Speaker: Keith Connolly, PhD

Date and Time: 5/12/2026 5:00-6:30 PM EST

ID 2314 Title: [Epigenetic suppression of Dux4 using CRISPR-GNDM technology as a therapeutic for FSHD](#)

Speaker: Alec M DeSimone, PhD

Date and Time: 5/13/2026, 5:00-6:30PM EST

About ASGCT 29th 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.

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