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September 24th, 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 Epigenome Editing Technology (CRISPR-GNDM<sup>®</sup>) and Data Supporting Development of Transformative Epigenome Editing Medicines for the Treatment of Muscular Dystrophy at the 5<sup>th</sup> Annual Genome Editing Therapeutics Summit**

24-Sep-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<sup>®</sup> epigenome editing technology, announced that the company has been accepted for an Oral Presentation at the 5<sup>th</sup> Annual Genome Editing Therapeutics Summit (Dec 3-5, 2024, Boston, USA), and the following research results will be presented by our CSO, Dr. Tetsuya Yamagata.

- *Lead program (MDL-101) demonstrating sufficient and durable gene upregulation in mouse disease models and NHPs for the treatment of LAMA2-CMD*
- *Regulatory-aligned path to a first-in-human trial with optimized biomanufacturing process*
- *Up/down gene regulation technology combined with tissue-tropic AAV capsids for non-hepatic (neuromuscular, cardiovascular, and CNS) disorders.*

Dr. Tetsuya Yamagata, CSO of Modalis said “In the 12 years since the debut of CRISPR/Cas9 technology, the development of various innovative genome editing therapeutics such as base editing, prime editing, and epigenome editing has progressed in addition to the conventional genome editing technology using CRISPR/Cas9. We are honored to be recognized as a leader in CRISPR-based epigenome editing company and to have the opportunity to present our CRISPR-GNDM<sup>®</sup> technology and development progress data on the clinical development of our lead program, MDL-101, at this conference, which brings together leading companies developing novel genome editing therapeutics”.

The Modalis Therapeutics presentation will be at the 5<sup>th</sup> Annual Genome Editing Therapeutics Summit (URL ;<https://genome-editing-therapeutics-summit.com>)

### **Oral Presentation:**

**Title:** Epigenome editing with CRISPR-GNDM<sup>®</sup> as a treatment platform for unmet medical conditions

**Date and Time:** 12/5/2024, 11AM EST

**Session Name:** Progressing Epigenome Editors into the Clinic

### **About the 5<sup>th</sup> Annual Genome Editing Therapeutics Summit**

The conference brings together the industry's leading players in the development, application, and delivery of novel genome-edited therapies. The conference will feature oral presentations, panel discussions, and workshops by industry leaders in genome editing therapeutics to share the latest data on cutting-edge therapies in the development stage, to overcome challenges faced by genome editing therapeutics, and to promote their industrial application.

### **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<sup>®</sup> 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](http://www.modalistx.com).