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July 15, 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 Epigenome Editing Technology, Differentiated Activation Programs, and Preclinical Data at the 6th Annual Genome Editing Therapeutics Summit

15-July-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 the company has been accepted for an Oral Presentation at the 6th Annual Genome Editing Therapeutics Summit (Sept 29th-Oct 1st, 2025, Boston, USA), and the following research results will be presented by our CSO, Dr. Tetsuya Yamagata.

- *Demonstrate the efficiency and durability of gene activation by Cas-9 mediated in vivo epigenome editing*
- *Understand the impact of using a muscle tropic AAV capsid delivery system*
- *Highlight that despite sustained Cas-9 expression and limited immunogenicity, favorable toxicology data support the therapeutic safety profile*

Dr. Tetsuya Yamagata, CSO of Modalis said “In recent years, the first patient cases treated with CRISPR-based genome editing therapies have been reported worldwide, and various *ex vivo* and *in vivo* technologies are steadily advancing toward clinical application. This progress significantly expands the potential of genome editing in treating genetic diseases. Among these approaches, epigenome editing is particularly promising due to its ability to regulate gene expression without cutting DNA, offering a high safety profile. Programs entering clinical stages have emerged, making epigenome editing a globally recognized next-generation gene regulation technology. In this context, we are honored to present our pioneering CRISPR-GNDM® technology and preclinical data on our MDL-101 and MDL-201 programs, which specializes in targeted gene activation, at the 6th Genome Editing Therapeutics Summit.”.

The Modalis Therapeutics presentation will be at the 6th Annual Genome Editing Therapeutics Summit （URL；https://genome-editing-therapeutics-summit.com）

Oral Presentation:

Title: Movement Toward the Clinic: Preclinical Development of Gene Activation programs with CRISPR-GNDM® platform
Date and Time: 10/1/2025, 11:45 AM EST
Session Name: Optimizing CRISPR Technologies to Progress Epigenome Editors

About the 6th 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 epigenome modulation therapy for the treatment of LAMA2-Congenital Muscular Dystrophy (LAMA2-CMD). MDL-101 is comprised of guide nucleotide targeting the LAMA1 gene, a highly homologous sister gene of the LAMA2, 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 LAMA1 gene products in patients' muscle tissue to compensate for loss-of-function caused by mutations of LAMA2, and therefore has the potential to provide a one-time, durable treatment benefit forpatients with LAMA2-CMD.

About MDL-201

MDL-201 is an epigenome modulation therapy under investigation for the treatment of Duchenne Muscular Dystrophy (DMD). MDL-201 is comprised of guide nucleotide targeting the Utrophin (UTRN) gene, a sister gene of Dystrophin, enzyme-null Cas9 (dCas9) fused with trans-activating domain driven by a muscle-specific promoter and coded in a muscle-specific AAV vector. MDL-201 upregulates UTRN gene products in patients' muscle tissue to compensate for loss-of-function caused by mutations in the Dystrophin gene, and therefore has the potential to provide a one-time, durable treatment benefit for patients with DMD.

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.