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Company name: Modalis Therapeutics Corporation
Stock exchange listing: Tokyo Stock Exchange
Code number: 4883
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Presentation of Data Supporting of Development of Transformative Epigenetic Editing Medicines for the Treatment of a Type of Muscular Dystrophy at the ASGCT Annual Meeting

22-May-2023 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® epigenetic editing technology, today announced that the American Society for Gene and Cell Therapy (ASGCT), we reported on the development data of our LAMA2-CMD gene therapy molecule, MDL-101. Our presentation was selected as a late-breaking abstract as one of 14 abstracts selected from more than 100 applications and attracted a great deal of attention.

Oral Presentation:

Title: Advancing Epigenetic Editing with CRISPR-GNDM: Novel Muscle-Tropic AAV Vectors Deliver Promising Single-Dose Treatment for LAMA2-CMD

Summary

- Candidate CRISPR-GNDM® vectors demonstrated robust efficacy in LAMA2-CMD disease mouse model with single AAV vector administration.
- Systemic administration of our lead candidates with novel muscle-tropic AAV capsids in NHPs resulted in widespread vector distribution and enhanced GNDM expression in muscle tissue.
- Our study is one of the **first** to demonstrate successful epigenetic editing in NHPs, as evidenced by significant target engagement and LAMA1 gene upregulation across muscle tissues.
- Our study also showed systemic Cas9 expression in NHPs is safe and well tolerated.
- Epigenetic editing using CRISPR-GNDM® technology holds promise as a next-generation gene therapy platform for a variety of neuromuscular and other genetic disorders.

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Please click the link below to view our presentation materials used in the conference presentation.

https://www.dropbox.com/s/i4fvz5s6s4ecs4o/2023%20ASGCT%20Modalis.pdf?dl=0

Those who have registered for the conference can also view an archive of the presentation videos on the conference website.

* Late-Breaking Abstract session： Sessions established to present data recognized as high impact, groundbreaking, innovative, and newsworthy after the normal abstract submission deadline

About MDL-101

MDL-101 is an experimental, epigenetic 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 epigenetic gene 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.