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July 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 Data on Establishing Manufacturing Process of Muscle Tropic Engineered AAV Vectors Supporting Development of Transformative Epigenome Editing Medicines for the Treatment of Muscular Dystrophy at 16th Annual Bioprocessing Summit

24-July-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[®] epigenome editing technology, announced that the company has been accepted for an Oral Presentation at 16th Annual Bioprocessing Summit (Aug 19-22, 2024, Boston, USA), and the following research results will be presented by its Senior Director, Bioprocess Development, Dr. Seth Levy.

- *Development of upstream and downstream manufacturing process and analytical methods to achieve stable production and quality control of muscle tropic engineered AAV vectors for use in clinical trial.*
- *Comparison on the manufacturing process and outcome between natural occurring AAV9 vectors and muscle tropic engineered AAV vectors*

While research on engineered capsid is progressing and delivering remarkable results in many groups, manufacturing for practical use is still under development. As one of the leading companies in the area, Modalis is pleased to announce the results of the progress at the conference.

Oral Presentation:

Title: In-Process Stability Testing with Novel AAV Capsid Variants

Date and Time: 8/20/2024, 12-12:30PM EST

Session Name: Gene Therapy CMC & Analytics: Potency Assays, In-Process Testing

About 16th Annual Bioprocessing Summit

It is more important than ever for the bioprocessing industry to come together to share experiences and expertise to explore bioprocessing opportunities and overcome new challenges in new therapeutics modalities such as Cell and Gene Therapy. This conference brings together global leaders in the bioprocessing industry to share their experiences and expertise in the latest analytical method development, quality control, assurance, formulation, and stability improvement in the upstream and downstream strategy of bioprocessing. The conference is expected to attract approximately 1,500 attendees from all over the world, 300 oral presentations, and 100 poster presentation. (<https://www.bioprocessingsummit.com>)

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[®] 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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