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Aug 13th, 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 Functional Evaluation and Optimization Methodology for AAV Vector Genomes Using Nanopore Sequencing at Nanopore Community Meeting 2024, 1st BioPharma Day

13-Aug-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 invited for an Oral Presentation at Nanopore Community Meeting 2024, 1st BioPharma Day (Sep 16-18, 2024, Boston, USA), and the following research results will be presented by its Director, Molecular Biology and Genomics, Dr. Keith Connolly.

- *A method to accurately and non-biasedly evaluate and analyze the distribution of gene lengths loaded on AAV vectors using a nanopore sequencer.*
- *Based on such analysis, a methodology to remove problematic sequences from the candidate constructs to be loaded onto the AAV vector and to optimize them.*

AAV vectors are a promising and effective tool of delivering transgenes safely and selectively to target tissues and cells. Research and development of various AAV vectors is actively being conducted worldwide to improve their functions. On the other hand, what has become clearer as technology advances is that AAV containing incomplete-length genes sequences are generated as byproducts during the manufacturing process, which may lead to quality and ultimately, reduced efficacy and increased safety issues. In parallel with the development of our pipeline, Modalis, a leading company in AAV-based epigenetic editing therapy, has been working on the development of such basic technologies, and at this conference, we plan to present a novel method for analyzing and evaluating the strand length distribution of genes carried in AAV vectors using the latest nanopore sequencer that we have developed.

Oral Presentation:

Title: Nanopore sequencing and functional screening of AAV genomes for optimal production and function

Date and Time: 9/18/2024, 13:55-14:55PM EST

Session Name: Use of nanopore sequencing in analytical development

About Nanopore Community Meeting 2024 1st BioPharma Day

The conference will bring together leaders from academia and industry to discuss how nanopore sequencing technology is transforming the biopharmaceutical industry. With dedicated tracks on Research and Development and Quality Control, featuring plenary talks and panels by industry leaders, this exclusive event offers unique insights and networking opportunities. This event to be at the forefront of cutting-edge genomic advancements in nanopore technology([Nanopore Community Meeting 2024 - Boston \(nanoporetech.com\)](https://nanoporetech.com))

About Nanopore Sequencing

Nanopore sequencing is one of DNA or RNA sequencing technologies. DNA and RNA are passed through small nano scale holes made by protein one molecule at a time. The electric current that passes through the hole is measured. The technique determines the base sequence by measuring the electric current as the DNA or RNA passes through a small hole.

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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