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October 25th, 2024

Company name: Modalis Therapeutics Corporation
Stock exchange listing: Tokyo Stock Exchange
Code number: 4883
URL: https://www.modalistx.com/en/
Representative: Haruhiko Morita

MDL-101 for the Treatment of Congenital Muscular Dystrophy Type 1a (LAMA2-CMD) has been granted Orphan Drug Designation (ODD) in the US by the Food and Drug Administration (FDA)

25-Oct-2024 TOKYO & Waltham, Mass – Modalis Therapeutics Corporation (Tokyo Stock Exchange: 4883, CEO: Haru Morita) has received Orphan Drug Designation (ODD) from the U.S. Food and Drug Administration (FDA) for MDL-101, which Modalis is developing for the treatment of congenital muscular dystrophy type 1A (LAMA2-CMD).

The ODD is based on the Orphan Disease Act of 1983 and is a system for designating drugs that meet certain conditions, such as having fewer than 200,000 patients in the United States and being particularly in need of medical treatment. This system supports and promotes the development of orphan drugs with the aim of providing safe, high-quality drugs to the medical community as soon as possible, in light of the situation where research and development of orphan drugs are not progressing sufficiently due to the small number of patients despite the high medical need for such drugs.

This designation makes it possible to utilize various pharmaceutical and research cost support measures from the FDA, such as exemption from application fees for new drug applications in the US, reductions or exemptions from federal taxes related to clinical development, as well as preferential measures for development and promotion in the US, and after approval, it will be granted exclusive first-to-market sales rights in the US for seven years, which is a major step towards multi-national, multi-center clinical trials, which are important for accelerating the development of drugs for rare diseases. We believe that this is the result of the recognition of the high medical need for the gene therapy we are developing, as well as the Rare Pediatric Disease designation we received earlier.

LAMA2-CMD is a severe, early-onset congenital muscular dystrophy caused by the absence of the LAMA2 protein, which is made up of more than 3,000 amino acids. Because it cannot be loaded onto an AAV vector, it is thought that the conventional approach to gene therapy development is difficult. For this reason, there is currently no treatment, including gene therapy, that addresses the underlying cause of LAMA2-CMD.

Modalis' proprietary CRISPR-GNDM®, is capable of specific modulation of the expression of disease-relevant genes, without introducing double-strand DNA breaks, and our MDL-101 is potentially the first-in-class therapeutics to solve the challenge and provide life-changing therapeutics for the patients of LAMA2-CMD by inducing expression of the sister gene LAMA1 across muscle tissues to compensate for the deficient function of LAMA2.

Having the company mission "*Every life deserves attention*", Modalis has been working actively for the treatment to patients suffering from diseases for which there is no cure.

About MDL-101

MDL-101 is an experimental, epigenetic editing therapy being developed to treat LAMA2-Congenital Muscular Dystrophy (LAMA2-CMD). MDL-101 is comprised of a guide nucleotide targeting LAMA1 gene, a highly homologous sister gene of the disease-causing gene 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 mutation of LAMA2. It therefore has the potential to provide a one-time, durable treatment to benefit 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 epigenome editing without the need for DNA cleavage or altering DNA sequence. Headquartered in Tokyo with all research and development operations in Waltham Massachusetts, the company is listed on the Tokyo Stock Exchange's Growth market. For additional information, visit www.modalistx.com.

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