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Company name: Modalis Therapeutics Corporation

Stock exchange listing: Tokyo Stock Exchange

Ticker/Code number: 4883

URL: <https://www.modalistx.com/en/>

Corporate Representative: Haruhiko Morita

Modalis Receives Japanese Patent for DUX4 Gene-Targeted Therapy for Facioscapulohumeral Muscular Dystrophy

Modalis Therapeutics (Tokyo, Japan & Waltham, MA; President and CEO: Haru Morita, “Modalis”) today announced that the Japan Patent Office (JPO) has granted the company’s patent application entitled “Method for Treating Facioscapulohumeral Muscular Dystrophy by Targeting the DUX4 Gene” (Application No. JP2023-513722).

This patent covers a novel therapeutic approach for muscular dystrophy utilizing Modalis’ proprietary CRISPR-GNDM® epigenome editing technology to target the DUX4 gene. The inventors include Dr. Tetsuya Yamagata, Dr. Yuanbo Qin, and other members of Modalis’ research and development team.

Facioscapulohumeral muscular dystrophy (FSHD) is the third most common form of muscular dystrophy worldwide, with an estimated prevalence of approximately one million people globally, including about 38,000 in the United States. FSHD is characterized by progressive muscle weakness in the face, shoulder blades, and upper arms, and approximately 20% of patients require a wheelchair by the age of 50. Currently, there are no approved therapies that address the root genetic cause of the disease.

Modalis’ development candidate, MDL-103, leverages the company’s unique CRISPR-GNDM® technology to selectively and durably suppress expression of the DUX4 gene — the root cause of FSHD — in muscle tissue without introducing double-stranded breaks in DNA. This mechanism is expected to improve muscle function. The newly granted patent claims gene therapy products that inhibit the expression of the DUX4 gene and its toxic protein products, thereby offering a potential disease-modifying treatment for FSHD.

The program is being advanced with support from the SolveFSHD Foundation and the XPRIZE Foundation. With this patent grant, Modalis has secured intellectual property protection for its product in Japan.

Modalis will continue to drive the research and development of innovative gene therapies for FSHD and other diseases with high unmet medical needs.

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*Epigenome editing: The control of gene expression through modification of DNA, histones, non-coding RNA, etc., to turn genes on or off while leaving the gene sequence intact.

dCas9: An enzyme from which the cleavage activity of Cas9, a genome-editing cleavage enzyme, has been removed. It can be used for base substitution and epigenome editing by linking with transcription factors and other elements.