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Company name: Modalis Therapeutics Corporation
Stock exchange listing: Tokyo Stock Exchange
Ticker/Code number: 4883
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U.S. Patent Granted for Muscular Dystrophy Therapy Targeting LAMA1

Modalis Therapeutics Corporation (Modalis) announced today that the United States Patent and Trademark Office (USPTO) has granted its patent (U.S. Application No. 17/635,608), titled “Method for Treating Muscular Dystrophy by Targeting LAMA1 Gene.” The patent was issued on July 9, 2025.

This patent covers a gene therapy approach for muscular dystrophy using a CRISPR-based epigenome modulation system targeting the LAMA1 gene. The patented invention was developed by Dr. Yuanbo Qin and the Modalis team is part of Modalis's broader pipeline of therapies addressing neuromuscular disorders through its proprietary CRISPR-GNDM® epigenome editing platform.

The LAMA2 gene is known to play a role in the structure and function of muscle tissue. Abnormal regulation or pathogenic expression of LAMA2 has been suggested to be associated with specific forms of muscular dystrophy. The scope of this patent claim relates to therapeutic compositions comprising guide RNA optimized for LAMA1, a sister gene of LAMA2, delivered by adeno-associated virus (AAV) under the control of a muscle-specific promoter, and methods for using such compositions.

CRISPR-GNDM® can be used to target large causative genes that cannot be approached by conventional gene therapy or other modalities, enabling selective induction of the LAMA1 gene in muscles and compensating for lost LAMA2 function, as demonstrated in animal studies.

Following the patent registration in Japan in February, the patent has now been granted in the US. This establishes important intellectual property rights in the US, a major market, for our LAMA1-targeted muscular dystrophy treatment, and lays an important foundation for future clinical development and patent registration in other regions.

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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.