Modalis Therapeutics Reports Continued Progress Across Lead Programs and Strengthening of Intellectual Property Portfolio in the Third Quarter of Fiscal Year 2025

07-November-2025 TOKYO & WALTHAM, Mass.- Modalis Therapeutics Corporation ("Modalis" 4883.T TSE), a pioneer in developing innovative solutions for rare genetic diseases through its proprietary CRISPR-GNDM® epigenetic gene editing technology, today announced its financial results for the third quarter ended September 30, 2025, alongside recent operational milestones.

“We are very pleased to report that during the third quarter of 2025, our lead program MDL-101 for LAMA2-CMD has entered a critical stage, with GLP toxicology studies in non-human primates now in progress and GMP manufacturing of the investigational product well underway,” said CEO Morita. “At the same time, our MDL-201 program for Duchenne muscular dystrophy has demonstrated robust efficacy at a remarkably low dose in animal models, and our MDL-103 program for FSHD continues to yield encouraging preclinical data under grants from the XPRIZE Foundation and the Solve FSHD Foundation. Furthermore, the registration of two new patents in Japan — one for DUX4-targeted FSHD therapy and another for tauopathies such as Alzheimer’s disease — clearly demonstrates the expanding potential of our CRISPR-GNDM® platform. Together, these achievements represent tangible progress toward realizing our vision of gene control therapeutics that transform patients’ lives.”

R&D Highlights

- MDL-101 (LAMA2-Congenital Muscular Dystrophy Type 1A)

The Company's lead program, MDL-101, targeting LAMA2-CMD, is progressing smoothly toward a clinical trial application planned for mid-2026. Both GLP-compliant toxicity studies in non-human primates and GMP manufacturing of the investigational product are underway, representing key milestones in the transition to clinical development. No events have been identified that would materially affect the current timeline, and site preparation activities continue to ensure a prompt trial launch following regulatory approval.

- MDL-201 (Duchenne Muscular Dystrophy)

For MDL-201, Modalis conducted comparative evaluations in a DMD disease-model mouse against a benchmark therapy mimicking an existing gene therapy. The data confirmed equivalent or superior efficacy in both treadmill endurance and extensor digitorum longus (EDL) muscle strength tests at one-tenth the benchmark dose. These findings validate the therapeutic concept of MDL-201 and further demonstrate the potential of the CRISPR-GNDM® platform beyond LAMA2-CMD.

- MDL-103 (Facioscapulohumeral Muscular Dystrophy)

For MDL-103, targeting FSHD, Modalis received grants from the XPRIZE Foundation and the Solve FSHD Foundation to accelerate preclinical studies. Collaborative animal studies have since generated promising data demonstrating that the CRISPR-GNDM molecule effectively targets the Dux4 gene

and significantly suppresses its downstream expression. These results provide a solid foundation for continued development.

IP highlights

In September 2025, Modalis strengthened its intellectual property portfolio with two new patent registrations in Japan:

- JP 7736329 - Therapeutic approach targeting the DUX4 gene for facioscapulohumeral muscular dystrophy (FSHD)
- JP 7749244 - Therapeutic approach targeting the tau protein for the treatment of tauopathies, including Alzheimer's disease

These patents expand Modalis' therapeutic scope beyond neuromuscular diseases to include neurodegenerative conditions, demonstrating the broad applicability of the CRISPR-GNDM® epigenome editing platform.

Outlook and Collaborations

Modalis' pipeline - including MDL-101, MDL-201, and MDL-103 - is progressing synergistically under a unified technology platform for gene regulation therapeutics. Through strategic alliances such as its joint research with JCR Pharmaceuticals Co., Ltd., the Company is expanding its access to innovative delivery systems and advancing its mission to bring transformative gene-control therapies to patients worldwide.

Third Quarter 2025 Financial Results:

- **Cash Position**: Cash and deposits as of September 30, 2025, was ¥3,307million, compared to ¥3,575 million as of December 31, 2024, a decrease of ¥267 million. The decrease in Cash and deposits was primarily due to payments for research and development expenses.
- **Revenues**: No revenues in the nine months ended September 30, 2025.
- **Research & Development (R&D) Expenses:** R&D expenses were ¥1,632 million in the nine months ended September 30, 2025, compared to ¥882 million in the nine months ended September 30, 2024, an increase of ¥750 million.
- **General & Administrative (G&A) Expenses**: G&A expenses were ¥182 million in the nine months ended September 30, 2025, compared to ¥180 million in the nine months ended September 30, 2024, an increase of ¥2 million.
- **Net Loss**: Net loss was ¥1,801 million in the nine months ended September 30, 2025, compared to ¥1,060 million in the nine months ended September 30, 2024.
- Please refer to 3Q Consolidated Financial Results disclosed according to Japanese accounting rules in the English version below.

About Modalis:

Modalis Therapeutics is developing precision genetic medicines through the use of epigenome editing. Modalis is developing treatments for rare genetic disorders using its proprietary CRISPR-GNDM technology, which allows for the specific modulation of gene expression or histone modification without the necessity for double-stranded DNA cleavage, gene editing, or base editing. Modalis' primary focus is on genetic disorders caused by loss of gene regulation, resulting in excess or insufficient protein production. This includes multiple neuromuscular disorders. The company is

headquartered in Tokyo with laboratories and facilities in Waltham, Massachusetts. For additional information, visit www.modalistx.com.

Forward-Looking Statements:

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Consolidated Financial Results for the Nine Months Ended September 30, 2025 [Japanese GAAP]

November 7, 2025

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Stock exchange listing: Tokyo Stock Exchange
Code number: 4883
URL: https://www.modalistx.com/en/
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Scheduled date of filing quarterly securities report: -
Scheduled date of commencing dividend payments: -
Preparation of supplementary material on financial results: Yes
Holding of financial results briefing: Scheduled (for individual investors)

(Amounts of less than one million yen are rounded down.)

1. Consolidated Financial Results for the Nine Months Ended September 30, 2025 (January 1, 2025, to September 30, 2025)

(1) Consolidated Operating Results (% indicates changes from the previous corresponding period.)

	Operating revenue		Operating income		Ordinary income		Profit attributable to owners of parent	
Nine months ended	Million yen	%	Million yen	%	Million yen	%	Million yen	%
September 30, 2025	-	-	(1,815)	-	(1,798)	-	(1,801)	-
September 30, 2024	-	-	(1,062)	-	(1,059)	-	(1,060)	-

(Note) Comprehensive income: Nine months ended September 30, 2025: ¥(1,810) million [-%]
Nine months ended September 30, 2024: ¥(1,059) million [-%]

	Basic earnings per share	Diluted earnings per share
Nine months ended	Yen	Yen
September 30, 2025	(23.91)	-
September 30, 2024	(27.21)	-

(Notes)
For diluted earnings per share, the figure is not presented as the company recorded basic loss per share although the company has dilutive shares.

(2) Consolidated Financial Position

	Total assets	Net assets	Capital adequacy ratio
	Million yen	Million yen	%
As of September 30, 2025	3,423	2,693	77.7
As of December 31, 2024	3,691	3,548	95.5

(Reference) Equity: As of September 30, 2025: ¥2,659million
As of December 31, 2024: ¥3,526million