



Modalis Therapeutics Reports Operational Highlights and Fiscal Year End 2024 Financial Results

13-Feb-2024 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 fiscal year ended December 31, 2024, as well as recent operational milestones.

"We are pleased with our progress in the fourth quarter and the fiscal year 2024. Despite this difficult environment for Biotech, we were able to make progress in both development and finance and received both an Orphan Drug Designation (ODD) and a Rare Pediatric Disease Designation (RPDD) from the FDA for our lead program, MDL-101. MDL-101 has attracted the attention of the pharmaceutical industry as well as patients and their families who are waiting to enter clinical trials as a result of reports at several conferences following the publication of the paper in May," said CEO Morita. "With further progress in research and development with our partners, we are now stepping up our collaboration to a second phase."

Furthermore, we have restarted the development of MDL-201 for Duchenne muscular dystrophy (DMD), which is a validated market guaranteed by existing treatments, and we believe that we have the potential to provide a best-in-class gene therapy through a unique method of activating alternative genes using our proprietary CRISPR-GNDM® technology.

The warrants and the convertible bonds issued through third-party allotment were exercised and converted by the end of the last October, and the 15th warrants, which began to be exercised this year, have been exercised to about half. This has raised about 2.8 billion yen, and together with the funds on hand, we will be able to proceed with development centered on MDL-101.

Recent Preclinical and Business Highlights

- **MDL-101 is advancing to IND**
 - Pre-clinical trials are underway in preparation for IND filing.
 - Working on the technology transfer and manufacturing with CDMOs
 - RPDD (September) and ODD (October) from the U.S. regulatory authorities
- **Other programs**
 - MDL-201(DMD): replaced the capsid with a new version of muscle tropic ones and are currently conducting various evaluations with the new molecules.
- **Research collaboration**
 - JCR Collaboration is continuing on a central nervous system disease by combining our CRISPR-GNDM payload with JCR's AAV capsids that can penetrate the blood-brain barrier (BBB) and stepped up to the 2nd phase (announced in Jan 6, 2025)
 - Established a collaboration with Genixcure in AI-based capsid search for GC capsids for Alzheimer's disease and pursuing with GinkoBioworks in use of CRISPR base epigenome editing technology for various fields.
- **IP updates**
 - Patent related to MDL-101 (JP2022-509664) and MDL-202 (JP2022-518586) were granted in Japan in Jan 2025 and September 2024, respectively

- Patent for modified Cas9 (US18/058,832) co-filed with the University of Tokyo, was granted in the US (September)
- **Conference and presentation**
 - Publication of a paper on preclinical data for MDL-101 titled “Efficient and durable gene activation by Cas9-mediated” (May) <https://doi.org/10.1101/2024.05.03.592438>
 - epigenome editing in vivo
 - Cell and Gene Therapy Summit (Jul 8-10 in Boston)
 - Bioprocessing Summit (Aug 19-22 in Boston)
 - Gene Therapy Immunogenicity Summit (Aug 22 in Boston)
 - Nanopore Community Meeting Boston (Sep 16-17, in Boston)
 - 5th Genome Editing Therapeutics Summit (Dec 5 in Boston)

Fiscal Year 2024 Financial Results:

- **Cash Position:** Cash and deposits as of December 31, 2024, was ¥3,575 million, compared to ¥1,883 million as of December 31, 2023, an increase of ¥1,691 million. The increase in Cash and deposits was primarily due to Issuance of shares through the exercise of stock acquisition rights.
- **Revenues:** No revenue for the full year ended December 31, 2024.
- Decreases in R&D expenses were primarily due to a reorganization of our R&D structure. **Research & Development (R&D) Expenses:** R&D expenses were ¥1,092 million for full year ended December 31, 2024, compared to ¥2,102 million for December 31, 2023, a decrease of ¥1,010 million.
- **General & Administrative (G&A) Expenses:** G&A expenses were ¥245 million for the full year ended December 31, 2024, compared to ¥267 million for December 31, 2023, a decrease of ¥22 million. Decreases in G&A expenses were primarily due to decreased personnel costs.
- **Net Loss:** Net Loss was ¥1,317 million for the full year ended December 31, 2024, compared to ¥2,391 million for December 31, 2023.
- Please refer to 4Q 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:

This document has been prepared by Modalis Therapeutics Corporation and Modalis Therapeutics Inc. (the “Companies”) solely for information purposes only. This document does not constitute or form part of and should not be construed as, an offer to sell or issue or the solicitation of an offer to buy or acquire securities of the Companies in Japan, the United States or any other jurisdictions. The information contained herein is based on current economic, regulatory, market trends and other conditions. The Companies make no representation or guarantee with respect to the credibility,

accuracy or completeness of the information herein. The information contained herein may change without prior notice. You may not publish or use this document and the contents thereof for any other purpose without a prior written consent of the Companies. Furthermore, the information on future business results are forward-looking statements. Forward-looking statements include but not limited to expressions such as "*believe*", "*expect*", "*plan*", "*strategic*", "*expect*", "*anticipate*", "*predict*" and "*possibility*", as well as other similar expressions to explain future business activities, achievements, events and future conditions. Forward-looking statements are predictions about the future that reflect management's judgment based on currently available information. As such, these forward-looking statements are subject to various risks and uncertainties that could cause actual results to differ materially from those expressed in or suggested by the forward-looking statements. Therefore, you may not rely entirely on forward-looking statements. The Companies do not assume any obligation to change or correct any forward-looking statements in light of new information, future events or other findings. This document and its contents are confidential and are being provided to you solely for your information and may not be retransmitted. This presentation is being furnished to you solely for your information and may not be reproduced or redistributed to any other person. In giving this presentation, the Companies do not undertake any obligation to provide the recipient with access to any additional information or to update this presentation or any additional information or to correct any inaccuracies in any such information which may become apparent. Information on companies other than the Companies and information provided from third parties are based on public information or sources. The Companies have not independently verified the accuracy and appropriateness of such data and indicators used herein, nor assume any responsibility for the accuracy and appropriateness of such data and indicators presented in this document.

Contacts

Modalis Therapeutics

Sawako Nakamura

media@modalistx.com

Consolidated Financial Results for the Fiscal Year Ended December 31, 2024 [Japanese GAAP]



February 13, 2025

Company name: Modalis Therapeutics Corporation

Stock exchange listing: Tokyo Stock Exchange

Code number: 4883

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

Representative: Haruhiko Morita, CEO and Representative Director

Contact: Yosuke Nakashima, Executive Officer

Phone: +81-3-6231-0456

Scheduled date of Ordinary General Meeting of Shareholders: March 27, 2025

Scheduled date of filing securities report: March 27, 2025

Scheduled date of commencing dividend payments: -

Availability of supplementary briefing material on financial results: Available

Schedule of financial results briefing session: Scheduled (for securities analysts and institutional investors)

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

1. Consolidated Financial Results for the Fiscal Year Ended December 31, 2024 (January 1, 2024, to December 31, 2024)

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

	Operating revenue		Operating income		Ordinary income		Profit attributable to owners of parent	
	Million yen	%	Million yen	%	Million yen	%	Million yen	%
Fiscal Year ended December 31, 2024	-	-	(1,337)	-	(1,303)	-	(1,317)	-
December 31, 2023	-	(100.0)	(2,370)	-	(2,351)	-	(2,391)	-

(Note) Comprehensive income: Fiscal Year ended December 31, 2024: ¥ (1,312) million [-%]

Fiscal Year ended December 31, 2023: ¥ (2,400) million [-%]

	Net income per share	Diluted earnings per share	Return of equity	Ordinary income to total assets	Operating income to operating revenue
Fiscal Year ended	Yen	Yen	%	%	%
December 31, 2024	(28.57)	-	(54.0)	(45.6)	-
December 31, 2023	(77.24)	-	(111.9)	(91.2)	-

(Notes)

For diluted earnings per share, the figure is not presented as the Company recorded basic loss per share, although it had dilutive shares, although the Company had dilutive shares.

(2) Consolidated Financial Position

	Total assets	Net assets	Equity ratio	Net assets per share
	Million yen	Million yen	%	Yen
As of December 31, 2024	3,691	3,548	95.5	50.81
As of December 31, 2023	2,025	1,380	66.8	40.59

(Reference) Equity: As of December 31, 2024: ¥3,534 million

As of December 31, 2023: ¥1.353 million

(3) Cash flow

	Cash flows from operating activities	Cash flows from investing activities	Cash flows from financing activities	Cash and cash equivalents at the end of period
	Million yen	Million yen	Million yen	Million yen
As of December 31, 2024	(1,432)	(0)	3,044	3,575
As of December 31, 2023	(2,254)	(39)	1,216	1,883