



Modalis Therapeutics Reports Operational Highlights and First Quarter 2024 Financial Results

08-May-2024 TOKYO & Waltham, Mass.- Modalis Therapeutics Corporation ("Modalis" 4883.T TSE), a pioneer in developing cutting-edge solutions for rare genetic diseases through its proprietary CRISPR-GNDM® epigenetic gene modification technology, today announced its financial results for the first quarter ended March 31, 2024, alongside recent operational milestones.

“In 2023, Modalis announced the prioritization of its pipeline programs that support our focus as a neuromuscular-focused epigenome editing company,” said Haru Morita, Chief Executive Officer of Modalis. “Modalis further clarified our focus program and shifted to a development centered on MDL-101. We continued to advance its pre-clinical pipelines this quarter and our MDL-101 program has completed process development, which is one of the biggest hurdles for a gene therapy program before entering clinical trial, and as a result has reached a level of productivity, yield, and quality that makes it feasible to proceed to clinical trials in the production of engineered capsid-based AAV molecules, alongside preparations for the phase 1/2 clinical trial expected to commence next year. In the current environment, careful choices must be made when deciding how many programs to pursue at any one time. To that end, we are pleased to announce that we are narrowing our strategic focus and prioritizing our investments in the most promising programs. To this end, we have made the difficult but necessary decision to pull back from some of our preclinical assets, downsize some of our infrastructure, and reallocate our investments to maximize the potential of the programs we believe have the most value.”

Recent Preclinical and Business Highlights

- **MDL-101 is advancing to IND**
 - Established manufacturing process. Achieved reasonable productivity, yield, and quality that can reach clinical trials. Further development of analytical methods for the product.
 - At the same time, discussions were held on establishing protocols for conducting clinical trials, networking with patient groups, and building assay systems.
- **MDL-202 for DM1**
 - Disease model mice study and analysis of pilot NHP study ongoing.
 - In alignment with the announced strategic transformation, Modalis slows down the development of non-core programs including the DM1 program and continues to seek a potential collaboration partner or external investment.
- **Research collaboration**
 - Began collaboration with Company JCR Pharma in the area of CNS, validating the approach to CNS targets by combining BBB trans-passing capsid, JBC-AAV, and our CRISPR-GNDM technology.
 - In April, we entered into a partnership agreement with Ginkgo Bioworks. to combine Ginkgo's synthetic biology technology and biofoundry capabilities with our epigenome editing technology and to develop cross-promotion.
- **Conference and presentation**
 - 4th Annual Next Generation Gene Therapy Vectors Summit (June 12-14)
 - Cell and Gene Therapy Summit 2024 (July 8-10)

First Year 2024 Financial Results:

- **Cash Position:** Cash and deposits as of December 31, 2024, was ¥1,413 million, compared to ¥1,883 million as of December 31, 2023, a decrease of ¥470 million. The decrease in Cash and deposits was primarily due to R&D Expenses and G&A Expenses.
- **Research & Development (R&D) Expenses:** R&D expenses were ¥422 million for the three ended March 31, 2024, compared to ¥443 million for March 31, 2023, a decrease of ¥20 million. Decreases in R&D expenses were primarily due to decreases in development costs associated with the advancement of the Company's proprietary GNDM platform and product candidates.
- **General & Administrative (G&A) Expenses:** G&A expenses were ¥67 million for the three ended March 31, 2024, compared to ¥71 million for March 31, 2023, a Decrease of ¥4 million. Decreases in G&A expenses were primarily due to Decreased personnel costs.
- **Net Loss:** Net Loss was ¥457 million for the three ended March 31, 2024, compared to ¥532 million for March 31, 2023.
- Please refer to 1Q Consolidated Financial Results disclosed according to Japanese accounting rules in the English version below.

About Modalis:

Modalis Therapeutics is developing precision genetic medicines through epigenome editing. Modalis is pursuing therapies for orphan genetic diseases using its proprietary CRISPR-GNDM technology which enables the locus-specific modulation of gene expression or histone modification without the need for double-stranded DNA cleavage, gene editing, or base editing. Modalis focuses primarily on genetic disorders caused by loss of gene regulation – resulting in excess or insufficient protein production – which includes more than 660 genes that are currently estimated to cause human disease due to haploinsufficiency. Headquartered in Tokyo with laboratories and facilities in Waltham, Massachusetts. For additional information, visit www.modalistx.com.

Forward-Looking Statements:

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Contacts

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Consolidated Financial Results
for the Three Months Ended March 31, 2024
[Japanese GAAP]



May 8, 2024

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
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 Scheduled date of filing quarterly securities report: May 14, 2024
 Scheduled date of commencing dividend payments: -
 Availability of supplementary briefing material on quarterly financial results: Available
 Schedule of quarterly financial results briefing session: Scheduled (for individual investors)

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

1. Consolidated Financial Results for the Three Months Ended March 31, 2024 (January 1, 2024 to March 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	%
Three months ended								
March 31, 2024	-	-	(490)	-	(457)	-	(457)	-
March 31, 2023	-	(100)	(514)	-	(509)	-	(532)	-

(Note) Comprehensive income: Three months ended March 31, 2024: ¥ (458) million [- %]
 Three months ended March 31, 2023: ¥ (531) million [- %]

	Basic earnings per share	Diluted earnings per share
	Yen	Yen
Three months ended		
March 31, 2024	(13.57)	-
March 31, 2023	(17.86)	-

(Notes)

The amount shown as “business revenue” in the consolidated statements of income has been shown as “Operating revenue” in this document.

Diluted net income per share is not shown in the above table, because net income per share was negative although there are residual shares.

(2) Consolidated Financial Position

	Total assets	Net assets	Capital adequacy ratio
	Million yen	Million yen	%
As of March 31, 2024	1,559	1,011	62.9
As of March 31, 2023	2,025	1,380	66.8

(Reference) Equity: As of March 31, 2024: ¥981 million
 As of March 31, 2023: ¥1,353 million