



Modalis Therapeutics Reports Operational Highlights and Fiscal Year End 2023 Financial Results

14-Feb-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 fiscal year ended December 31, 2023, alongside recent operational milestones.

" While challenges are prevalent in the industry, we take immense gratification in witnessing the advancement of MDL-101, navigating a clear-cut trajectory toward its Investigational New Drug (IND) status. The imminent prospect of its introduction into clinical trials as the inaugural CRISPR-based epigenome-editing therapeutic raises enthusiasm within our ranks," said Haru Morita, Chief Executive Officer of Modalis. Morita further elaborated, "Our enthusiasm is compounded by the strategic collaboration we forged with JCR Pharma (JCR) in December, which is focused on central nervous system disorders. JCR's JBC technology, which enables genetic cargos to traverse the blood-brain barrier (BBB), aligns seamlessly with our CRISPR-GNDM® platform. We believe the synergy between these technologies will catalyze paradigm-shifting gene therapies, enabling us to precisely deliver GNDM to discrete neuronal targets."

Recent Preclinical and Business Highlights

- **MDL-101 is advancing to IND**
 - Received encouraging responses from FDA to our PreIND submission, elucidating a clear path to IND filing. Our GLP-tox plans were aligned with FDA guidance and studies have been initiated.
 - Process development has been completed to enable GMP manufacturing.
- **MDL-207 targeting Dravet Syndrome has been added to the pipeline**
 - Our innovative technology for epigenetic gene modulation is being explored as a potential long-term treatment of this serious condition by increasing the expression of the SCN1a gene transcripts from surviving alleles.
 - Animal proof of concept has been established in a murine Dravet disease model.
- **Research collaboration is established with JCR Pharma**
 - We have initiated a research collaboration with JCR Pharma to develop a novel therapeutic for an undisclosed CNS target utilizing JCR's JBC-AAV technology and Modalis' CRISPR-GNDM technology.
- **Three key patents have been granted or issued**
 - Progress continues to be made with patents for elemental technologies being approved in various countries.
 1. miniCas9 patent issued in Japan
 2. PAM-flex SpCas9 patent granted in China
 3. miniVR (small transcription activator) patent issued in Russia
 - Modalis also maintains its right and license to fundamental CRISPR patents, which Editas has recently licensed to a third party for treatment for beta-thalassemia

Fiscal Year 2023 Financial Results:

- **Cash Position:** Cash and deposits as of December 31, 2023, was ¥1,883 million, compared to ¥2,933 million as of December 31, 2022, a decrease of ¥1,050 million. The decrease in Cash and deposits was primarily due to R&D Expenses and G&A Expenses.

- **Revenues:** No revenue for the full year ended December 31, 2023.
- **Research & Development (R&D) Expenses:** R&D expenses were ¥2,103 million for full year ended December 31, 2023, compared to ¥1,862 million for December 31, 2022, an increase of ¥241 million. Increases in R&D expenses were primarily due to increases in development costs associated with the advancement of the Company's proprietary GNDM platform, product candidates and yen depreciation against US dollar.
- **General & Administrative (G&A) Expenses:** G&A expenses were ¥268 million for the full year ended December 31, 2023, compared to ¥242 million for December 31, 2022, an increase of ¥26 million. Increases in G&A expenses were primarily due to increased personnel costs.
- **Net Loss:** Net Loss was ¥2,352 million for the full year ended December 31, 2023, compared to ¥1,996 million for December 31, 2022.
- 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 epigenetic gene modulation. 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

Modalis Therapeutics

Sawako Nakamura

media@modalistx.com

Consolidated Financial Results for the Fiscal Year Ended December 31, 2023 [Japanese GAAP]



Feb 14, 2024

Company name: Modalis Therapeutics Corporation

Stock exchange listing: Tokyo Stock Exchange

Code number: 4883

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

Representative: Haruhiko Morita, President and Representative Director

Contact: Naoki Kobayashi, CFO and Executive Officer

Phone: +81-3-6822-4584

Scheduled date of filing quarterly securities report: March 26, 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 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, 2023 (January 1, 2023 to December 31, 2023)

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

	Operating revenue		Operating income		Ordinary income		Profit attributable to owners of parent	
Fiscal Year ended	Million yen	%	Million yen	%	Million yen	%	Million yen	%
December 31, 2023	-	(100)	(2,370)	-	(2,351)	-	(2,391)	-
December 31, 2022	40	-	(2,063)	-	(1,995)	-	(2,702)	-

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

Fiscal Year ended December 31, 2022:: ¥ (2,691) million [- %]

	Basic earnings 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, 2023	(77.24)	-	(111.9)	(91.2)	-
December 31, 2022	(92.85)	-	(63.8)	(43.4)	-

(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	Net assets per share
	Million yen	Million yen	%	Yen
As of December 31, 2023	2,025	1,380	66.8	40.59
As of December 31, 2022	3,129	2,941	93.4	99.53

(Reference) Equity: As of December 31, 2023: ¥1,353 million

As of December 31, 2022: ¥2,922 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, 2023	(2,254)	(39)	1,216	1,883
As of December 31, 2022	(1,895)	(185)	63	2,933