



## Modalis Therapeutics Reports Operational Highlights and Fiscal Year End of 2022 Financial Result

13-Feb-2023 TOKYO & Waltham, Mass.- Modalis Therapeutics Corporation (Modalis), a leading company developing innovative products for the treatment of rare genetic diseases utilizing its proprietary CRISPR-GNDM® gene modulation technology, today reported financial results for the Fiscal Year ended December 31, 2022, as well as recent operational highlights.

"Although 2022 was a rough year, we were able to continue to make steady progress," said Haru Morita, Chief Executive Officer of Modalis. "The emerging market environment has been challenging, and gene therapy has also thrown up safety concerns after the honeymoon period. What is different, however, compared to the dark era of gene therapy in the 2000s, is that a lot of resources have been invested, and as a result, solutions to the challenges continue to be created. Modalis has adapted to environmental changes and new technological trends to survive and made significant progress toward the clinic. While we are very sad that our collaboration with Eisai has come to an end, we are very pleased with the remarkable results achieved during the research period and are grateful for the financial and technical support we have received during this time. We continue to be committed to developing therapeutics so that we can deliver the best products to the patients as quickly as possible."

### Recent Preclinical and Business Highlights

- **MDL-101 goes through a confirmatory NHP study**
  - As the Company implemented the change in the vector for MDL-101, it developed new versions of molecules and conducted an NHP study to confirm efficacy and safety.
  - As soon as the analysis of the above-mentioned NHP study is completed, the company will file a pre-IND request to FDA to confirm the IND-enabling study design and manufacturing plan for IND.
- **MDL-104 is also being tested in NHP**
  - As the company has decided to give MDL-104 a high priority, candidate molecules are being tested in NHP study to see biodistribution.
  - Concurrent to the NHP study, the molecules were tested in two tauopathy disease models which hold the human Tau gene together with its regulatory element. A robust suppression of human Tau was confirmed both in mRNA and protein levels.
  - For the potential clinical trials, the company had meetings with multiple key opinion leaders in the field primarily to discuss potential indication(s) in tauopathy. Alzheimer's disorder and Frontotemporal dementia are the provisional candidates based on several factors including patient population, disease progression, and disease-causing sites in the brain.
- **Modalis is regaining the rights of MDL-205**
  - Upon completion of the research collaboration with Eisai, Modalis is regaining rights of MDL-205 which has demonstrated robust animal data. The detail will be announced together with the development plan after signing the agreement with Eisai.
- **MDL-105 for dilated cardiomyopathy (DCM)**
  - To explore opportunities of applying CRISPR-GNDM® technology in the cardiovascular space, the company has developed candidate molecules targeting Titin truncate variants (TTNtv) which is the most frequent genetic defect of DCM. Having access to a TTNtv animal model. The company is evaluating the molecules.
- **The other R&D and Corporate Updates**

- In this fiscal year, the patent jointly filed by Modalis and our partner, Astellas, titled " Method for treating muscular dystrophy by targeting utrophin gene" has been issued in Japan (May) and in the US (October)
- In December, A PAM-Flex Cas9 patent was also granted in Japan which was exclusively licensed from the University of Tokyo.

#### **Fiscal Year 2022 Financial Results:**

- **Cash Position:** Cash and deposits as of December 31, 2022, was ¥2,933 million, compared to ¥4,936 million as of December 31, 2021, a decrease of ¥2,003 million. Decrease in Cash and deposits were primarily due to a decrease in R&D Expense and G&A Expense.
- **Revenues:** Revenues were ¥40 million for full year ended December 31, 2022, respectively. Increases in Operating Revenue were primarily due to ongoing collaborations.
- **Research & Development (R&D) Expenses:** R&D expenses were ¥1,861 million for full year ended December 31, 2022, compared to ¥1,009 million for December 31, 2021, an increase of ¥852 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. In addition, increased expenses for conducting clinical trials of MDL-101.
- **General & Administrative (G&A) Expenses:** G&A expenses were ¥241 million for full year ended December 31, 2022, compared to ¥231 million for December 31, 2021, an increase of ¥10 million. Increases in G&A expenses were primarily due to increase by personnel costs.
- **Net Loss:** Net Loss was ¥2,702 million for full year ended December 31, 2022, compared to ¥738 million for December 31, 2021.
- 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 is focusing initially 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 Cambridge, Massachusetts. For additional information, visit [www.modalistx.com](http://www.modalistx.com).

## Forward-Looking Statements:

This document has been prepared by Modalis Therapeutics corporation and Modalis Therapeutics Inc. (the "Companies") solely for information purpose 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](mailto:media@modalistx.com)

# Consolidated Financial Results for the Fiscal Year Ended December 31, 2022 [Japanese GAAP]



February 13, 2023

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 Ordinary General Meeting of Shareholders: March 28, 2023

Scheduled date of filing securities report: March 28, 2023

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, 2022 (January 1, 2022 to December 31, 2022)

(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, 2022	40	—	(2,063)	—	(1,995)	—	(2,702)	—
December 31, 2021	1	(99.7)	(1,239)	—	(1,231)	—	(738)	—

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

Fiscal Year ended December 31, 2021: ¥ (731) million [-%]

	Net income per share	Diluted earnings per share	Return of equity	Ordinary income to total assets	Operating income to operating revenue
	Yen	Yen	%	%	%
Fiscal Year ended December 31, 2022	(92.85)	—	(63.8)	(43.4)	—
December 31, 2021	(25.70)	—	(12.6)	(19.9)	—

(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, 2022	3,129	2,941	93.4	99.53
As of December 31, 2021	6,069	5,549	91.4	191.57

(Reference) Equity: As of December 31, 2022: ¥2,922 million

As of December 31, 2021: ¥5,549 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, 2022	(1,895)	(185)	63	2,933
As of December 31, 2021	(747)	171	72	4,936