



Modalis Therapeutics Reports Operational Highlights and Third Quarter 2022 Financial Result

07-Nov-2022 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 third quarter ended September 30, 2022, as well as recent operational highlights.

"We have made a strategic change to introduce a new capsid to our programs," said Haru Morita, Chief Executive Officer of Modalis. "Recent years have seen remarkable innovations in engineered capsid technology while safety concerns were raised to existing capsids used for high-dose systemic injection. We are very pleased that we were able to introduce new technology at this very last minute of the vector lock-in which decides the molecule we work with until the product goes on the market and even after. We are committed to using the best technology available today so that we can deliver the best products to the patients."

Recent Preclinical and Business Highlights

- **MDL-101 vector is subjected to change**
 - The Company has decided to change the vector for MDL-101 aiming to increase potency and decrease the dose and toxicities associated with high-dose systemic injection of AAV.
 - As adjustments have been made to the manufacturing process to accommodate the change in the capsid, the timeline of the GMP campaign was revised with the partner CDMO.
- **MDL-104 got higher priority in the development pipeline**
 - After a thorough review of the current development status and timeline, the company has decided to give MDL-104 a high priority.
 - Currently, the final candidate molecules are being evaluated in several studies and the goal is to file a pre-IND application in mid-2023.
- **Other preclinical programs have shown steady progress**
 - We continue to work on internal and collaboration programs. In the MDL-105 program, we are in the process of validating our product in animal models.
- **Research, Manufacturing, and Corporate Updates**
 - The patent jointly filed by Modalis and our partner, Astellas, titled "Method for treating muscular dystrophy by targeting utrophin gene" has been issued in the US as of October 18th as anticipated.

Third Quarter 2022 Financial Results:

- **Cash Position:** Cash and deposits as of September 30, 2022 was ¥3,681 million, compared to ¥4,936 million as of December 31, 2021, a decrease of ¥1,255 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 the nine ended September 30, 2022 respectively. Increases in Operating Revenue were primarily due to ongoing collaborations.
- **Research & Development (R&D) Expenses:** R&D expenses were ¥1,290 million for the nine ended September 30, 2022, compared to ¥493 million for September 30, 2021, an increase

of ¥797 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 ¥187 million for the nine ended September 30, 2022, compared to ¥184 million for September 30, 2021, an increase of ¥3 million. Increases in G&A expenses were primarily due to increase by personnel costs.
- **Net Loss:** Net Loss was ¥1,309 million for the nine ended September 30, 2022, compared to ¥181 million for September 30, 2021.
- 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 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.

Forward-Looking Statements:

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Contacts

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**Consolidated Financial Results
for the Nine Months Ended September 30, 2022
[Japanese GAAP]**



November 7, 2022

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

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Scheduled date of filing quarterly securities report: November 14, 2022

Scheduled date of commencing dividend payments: -

Availability of supplementary briefing material on quarterly financial results: Available

Schedule of quarterly financial results briefing session: -

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

1. Consolidated Financial Results for the Nine Months Ended September 30, 2022 (January 1, 2022 to September 30, 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	%
Nine months ended September 30, 2022	40	-	(1,438)	-	(1,314)	-	(1,309)	-
September 30, 2021	1	(99.7)	(677)	-	(661)	-	(181)	-

(Note) Comprehensive income: Nine months ended September 30, 2022: ¥ (1,280) million [- %]

Nine months ended September 30, 2021: ¥ (174) million [- %]

	Basic earnings per share	Diluted earnings per share
	Yen	Yen
Nine months ended September 30, 2022	(45.04)	-
September 30, 2021	(6.32)	-

(Notes)

For diluted earnings per share for the fiscal year ended September 30, 2022, the figure is not presented as the Company's shares were not listed and the average share price was not available, although the Company had dilutive shares.

(2) Consolidated Financial Position

	Total assets	Net assets	Capital adequacy ratio
	Million yen	Million yen	%
As of September 30, 2022	4,886	4,283	87.6
As of December 31, 2021	6,069	5,549	91.4

(Reference) Equity: As of September 30, 2022: ¥4,278 million

As of December 31, 2021: ¥5,549 million