



Modalis Therapeutics Reports Operational Highlights and Third Quarter 2023 Financial Result

07-Nov-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® epigenetic gene modulation technology, today reported financial results of the third quarter ended September 30, 2023, as well as recent operational highlights.

" We have initiated the development of MDL-202 for the treatment of Myotonic Dystrophy Type-1, a serious disease, and unmet medical need, after regaining product rights," said Haru Morita, Chief Executive Officer of Modalis. "We have already converted this molecule to a new delivery platform and are testing candidate molecules equipped with the myotropic capsids in both mice and non-human primates (NHPs). Having already learned and experienced much throughout the prior development of MDL-101, and having prepared for the asset transfer, we were able to resume the development of MDL-202 without wasting precious time. We are also making progress in the development of MDL-101. The target disease, LAMA2-CMD, is a pediatric disease, with symptoms becoming apparent shortly after birth to six months later. Therefore, it is important to confirm that the drug is efficacious and safe at the target age, assuming that the first-in-human clinical trials are conducted in children. We have completed an age-matched NHP study and confirmed the candidate molecule is as potent and safe as in adult NHPs. We believe this demonstrates the prospect of direct benefit (PDB) to support the administration of MDL-101 to a pediatric population with LAMA2-CMD."

Recent Preclinical and Business Highlights

- **Completed asset transfer of MDL-201 and 202 and pipeline reorganization.**
 - Regained rights to develop, manufacture, and commercialize MDL-201 and MDL-202.
 - Refined our corporate strategy to focus on the development of muscle diseases, with a focus on MDL-101 and MDL-202.
- **Resumed development of MDL-202 with a new capsid**
 - Completed transition to the new AAV platform.
 - Completed Pilot production of the candidate molecules.
 - Initiated dosing of mice and NHP for target engagement studies.
 - Readout is expected in 1Q/2024 or later.
- **MDL-101 is advancing to IND**
 - Company completed an age-matched juvenile NHP study and confirmed the candidate molecule is as potent and safe as in adult NHPs.
 - Improved the manufacturing process of MDL-101 with the achievement of reasonable quality and productivity.
 - Revised GLP study design and upcoming clinical trial plan to reflect favorable FDA response to PreIND Meeting

Second Quarter 2023 Financial Results:

- **Cash Position:** Cash and deposits as of September 30, 2023, was ¥1,897 million, compared to ¥2,933 million as of December 31, 2022, a decrease of ¥1,036 million. Decrease in Cash and deposits were primarily due to a decrease in R&D Expense and G&A Expense.
- **Revenues:** No revenue for the nine months ended September 30, 2023.
- **Research & Development (R&D) Expenses:** R&D expenses were ¥1,401 million for the nine ended September 30, 2023, compared to ¥1,290 million for September 30, 2022, an increase of ¥111 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 and product candidates. In addition, increased expenses for conducting clinical trials of MDL-101.
- **General & Administrative (G&A) Expenses:** G&A expenses were ¥207 million for the nine months ended September 30, 2023, compared to ¥187 million for September 30, 2022, an increase of ¥20 million. Increases in G&A expenses were primarily due to increases in personnel expenses.
- **Net Loss:** Net loss was ¥1,581 million for the nine ended September 30, 2023, compared to ¥1,309 million for September 30, 2022.
- 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 Three Months Ended September 30, 2023
[Japanese GAAP]



November 7, 2023

Company name: Modalis Therapeutics Corporation

Stock exchange listing: Tokyo Stock Exchange

Code number: 4883

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

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Scheduled date of filing quarterly securities report: November 13, 2023

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, 2023 (January 1, 2023, to September 30, 2023)

(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	%
Six months ended								
September 30, 2023	-	(100.0)	(1,609)	-	(1,542)	-	(1,581)	-
September 30, 2022	40	-	(1,438)	-	(1,314)	-	(1,309)	-

(Note) Comprehensive income: Nine months ended June 30, 2023: ¥(1,034) million [-%]

Nine months ended June 30, 2022: ¥(1,280) million [-%]

	Basic earnings per share	Diluted earnings per share
Six months ended	Yen	Yen
September 30, 2023	(52.29)	-
September 30, 2022	(45.04)	-

(Notes)

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

(2) Consolidated Financial Position

	Total assets	Net assets	Capital adequacy ratio
	Million yen	Million yen	%
As of September 30, 2023	2,179	1,987	90.0
As of December 31, 2022	3,129	2,941	93.4

(Reference) Equity: As of September 30, 2023: ¥1,961 million

As of December 31, 2022: ¥2,922million