



Modalis Therapeutics Reports Operational Highlights and First Quarter 2023 Financial Result

09-May-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 first quarter ended March 31, 2023, as well as recent operational highlights.

"We are glad to start the FY2023 with great progress," said Haru Morita, Chief Executive Officer of Modalis. "The outcome of the modified version of MDL-101 with the new capsid technology tested in a LAMA2-CMD disease model and NHPs was more than we expected, and we were able to demonstrate better potency and safety both in both species. With those results, we have filed a PreIND meeting request to FDA. We are also very excited to share the data at the Annual Meeting of the American Society for Gene and Cell Therapy (ASGCT). This achievement in MDL-101 not only offers a bright future for patients suffering from LAMA2-CMD but can also be extrapolated to our other muscle disease pipelines, which were created based on the same technology. This is truly the strength of platform technology, and we believe we can leverage this advantage to produce more results."

Recent Preclinical and Business Highlights

- **Completed evaluation of MDL-101 and filed preIND meeting request to FDA**
 - The company implemented the change in the vector for MDL-101 and evaluated the molecules in the mice disease model and NHPs.
 - With the favorable results which demonstrated AAV transduction, GNDM expression, and consequent LAMA1 upregulation in both species within a reasonable dose range, the company filed a pre-IND meeting request to FDA.
 - The results will be reported at the Annual Meeting of the American Society for Gene and Cell Therapy (ASGCT), Los Angeles, USA, May 16-20, 2023. The abstract was accepted as a late-breaking abstract and will be presented in an oral presentation on May 19.
- **MDL-104 is also being evaluated in NHP**
 - Concurrently, MDL-104 is also being evaluated in mice and NHPs. This is to see if reasonable biodistribution is achieved with AAV9 and via intra-cisterna magna (ICM) injection. The result will come out in 2Q.
- **MDL-205 is under evaluation**
 - After completion of the research collaboration with Eisai, Modalis is in discussion with Eisai for regaining the right to MDL-202 as well as assessing the new development strategy.
- **MDL-105 for dilated cardiomyopathy (DCM)**
 - CRISPR-based gene modulation technology is evaluated in the cardiovascular space for the first time. Levering its accumulated know-how in muscular disorders, the company has developed candidate molecules targeting Titin truncate variants (TTNtv) and being evaluated in animals to establish animal PoC.
- **The other R&D and Corporate Updates**
 - In April, A PAM-Flex Cas9 patent which we co-filed with University of Tokyo was also granted in Japan.

First Quarter 2023 Financial Results:

- **Cash Position:** Cash and deposits as of March 31, 2023, was ¥2,708 million, compared to ¥2,933 million as of December 31, 2022, a decrease of ¥225 million. Decrease in Cash and deposits were primarily due to a decrease in R&D Expense and G&A Expense.
- **Research & Development (R&D) Expenses:** R&D expenses were ¥443 million for the three ended March 31, 2023, compared to ¥442 million for March 31, 2022, an increase of ¥1 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 ¥71 million for the three ended March 31, 2023, compared to ¥65 million for March 31, 2022, an increase of ¥6 million. Increases in G&A expenses were primarily due to increases in personnel expense.
- **Net Profit:** Net Profit was ¥-532 million for the three ended March 31, 2023, compared to ¥-438 million for March 31, 2022.
- 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 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 March 31, 2023 [Japanese GAAP]



May 9, 2023

Company name: Modalis Therapeutics Corporation

Stock exchange listing: Tokyo Stock Exchange

Code number: 4883

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

Representative: Haruhiko Morita, President and Representative Director

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Scheduled date of filing quarterly securities report: May 12, 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 Three Months Ended March 31, 2023 (January 1, 2023, to March 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	
	Million yen	%	Million yen	%	Million yen	%	Million yen	%
Three months ended								
March 31, 2023	-	(100.0)	(514)	-	(509)	-	(532)	-
March 31, 2022	40	-	(466)	-	(443)	-	(438)	-

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

Three months ended March 31, 2022: ¥ (427) million [-%]

	Basic earnings per share	Diluted earnings per share
Three months ended	Yen	Yen
March 31, 2023	(17.86)	-
March 31, 2022	(15.13)	-

(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 March 31, 2023	2,899	2,653	90.9
As of December 31, 2022	3,129	2,941	93.4

(Reference) Equity: As of March 31, 2023 : ¥2,634million

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