



## Modalis Therapeutics Reports Operational Highlights and First Quarter 2022 Financial Result

09-May-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® epigenetic gene modulation technology, today reported financial results for the first quarter ended March 31, 2022, as well as recent operational highlights.

"We have made great strides in our science," said Haru Morita, Chief Executive Officer of Modalis. "As a leader in the field of epigenetic editing, our responsibility is not only to advance the science and our programs, but also to share our knowledge with the community for the advancement of the field. As part of our responsibility, we are pleased to present six papers at this year's ASGCT meeting."

### Recent Preclinical and Business Highlights

- **MDL-101 IND-enabling study initiated**
  - The Company's INTERACT meeting request was accepted by FDA and meeting is scheduled in July.
  - IND enabling studies including NHP study and process development for GMP manufacturing are ongoing.
- **MDL-205 has achieved milestones and collaboration was extended**
  - MDL-205, has achieved research milestones and the consideration for the milestones has been recorded. In addition, the collaboration period has been extended based on a mutual agreement.
- **Modalis develops MDL-105 targeting Titin to treat Dilated Cardiomyopathy**
  - The company has decided to develop a new gene therapy for treatment of Dilated Cardiomyopathy (DCM). The target gene, titin, is the largest human gene for which attempts have been barely made to develop new therapeutics due to its huge size. Taking advantage of CRISPR-GNDM®, which is size agnostic, we have established a unique and differentiated approach to tackle the disorder.
- **To present 6 abstracts at the 25th Annual Meeting of the American Society of Gene and Cellular Therapy (ASGCT)**
  - In addition to research results in the pipeline, including MDL-101, MDL-104, and MDL-205, the abstracts will also discuss the anticipated challenges associated with manufacturing of CRISPR-GNDM® based molecules with AAV vectors and the effects of long-term expression of Cas9 protein in animals on immunity. The research results will be shared to address what may be perceived as technical hurdles and this is an effort to fulfill our responsibility as a leader in the field of epigenome editing and to disseminate this information. We will also report on MDL-105, a new pipeline for the treatment of DCM that targets the Titin gene.
- **Research, Manufacturing, and Corporate Updates**

The interferences between the Broad Institute and the University of California group on the rights of the CRISPR foundational patents in the U.S. were concluded in favor of the Broad Institute in February. This decision ensures the continued validity of the CRISPR foundational patents developed by Broad Institute and licensed to us through our license agreement with Editas.

In April, after the consolidated quarter, a patent application jointly filed by Modalis and our partner, Astellas, titled "Method for treating muscular dystrophy by targeting utrophin gene" was granted in Japan, and the patent was just registered in May. This is a new method of treating Duchenne muscular dystrophy with a gene therapy drug using the GNDM method.

### First Quarter 2022 Financial Results:

- **Cash Position:** Cash and deposits as of March 31, 2022 was ¥4,404 million, compared to ¥4,936 million as of December 31, 2021, a decrease of ¥532 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 three ended March 31, 2022 respectively. Increases in Operating Revenue were primarily due to ongoing collaborations.
- **Research & Development (R&D) Expenses:** R&D expenses were ¥442 million for the three ended March 31, 2022, compared to ¥184 million for March 31, 2021, an increase of ¥258 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 ¥64 million for the three ended March 31, 2022, compared to ¥69 million for March 31, 2021, a decrease of ¥5 million. Decrease by G&A expenses were primarily due to decrease by depreciation costs.
- **Net Profit:** Net Profit was ¥-438 million for the three ended March 31, 2022, compared to ¥201 million for March 31, 2021.
- 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](http://www.modalistx.com).

## Forward-Looking Statements:

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# Consolidated Financial Results for the Three Months Ended March 31, 2022 [Japanese GAAP]



May 9, 2022

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 13, 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 Three Months Ended March 31, 2022 (January 1, 2022 to March 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	%
Three months ended								
March 31, 2022	40	-	(466)	-	(443)	-	(438)	-
March 31, 2021	0	(95.4)	(252)	-	(245)	-	201	-

(Note) Comprehensive income: Three months ended March 31, 2022: ¥ (427) million [-%]  
 Three months ended March 31, 2021: ¥ 210 million [-%]

	Basic earnings per share	Diluted earnings per share
	Yen	Yen
Three months ended		
March 31, 2022	(15.13)	-
March 31, 2021	7.07	6.66

(Notes)

For diluted earnings per share for the fiscal year ended March 31, 2022, 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, 2022	5,624	5,126	91.2
As of December 31, 2021	6,069	5,549	91.4

(Reference) Equity: As of March 31, 2022 : ¥5,126 million  
 As of December 31, 2021: ¥5,549 million