Modalis Therapeutics Reports Operational Highlights and Second Quarter 2024 Financial Results

07-Aug-2024 TOKYO & Waltham, Mass.- Modalis Therapeutics Corporation ("Modalis" 4883.T TSE), a pioneer in developing cutting-edge solutions for rare genetic diseases through its proprietary CRISPR-GNDM® epigenetic gene modification technology, today announced its financial results for the second quarter ended June 30, 2024, alongside recent operational milestones.

"The second quarter provided an opportunity to update the world on the progress of our lead program, MDL-101, as we prepare for clinical trials. Preclinical data from mouse and monkey studies were published in a paper in May and reported at conferences in June and July," said Haru Morita, Chief Executive Officer of Modalis. "The response to the results presented in the paper has been overwhelming, and we have received inquiries from patients suffering from LAMA2-CMD, their families, and their physicians from around the world to participate in the clinical trial. In addition, multiple inquiries from pharma/biotech companies about our pipeline and technology platform."

As a result of business restructuring reported in April and June, and of other factors, the Company has announced that it will push back the IND filing target to 2025, while it has filed Orphan Disease Designation and Rare Pediatric Disease Designation applications with the FDA at the end of July and August, respectively, which are expected to be reviewed for the rarity of disease and medical relevance of the target disease. If accepted, the application will provide a number of economic benefits, including tax deductions for clinical trial costs and an exclusive seven-year market exclusivity period after product launch.

In the area of central nervous system (CNS) diseases, as an attempt to expand access to technology that can cross the blood-brain barrier (BBB), we have been collaborating with JCR Pharma since last year to combine their capsid technology, JBC-AAV, with our CRISPR-GNDM® technology based payload, which has obtained excellent preclinical PoC in various target diseases. The company also announced the signing of a memorandum of understanding for research collaboration to develop next-generation therapeutics for Alzheimer's disease with Genixcure, a South Korean emerging company, on modified capsid technology based on AI technology. As BBB-crossing capability is the common bottleneck for gene therapy targeting CNS diseases and is also a rapidly innovating capsid technology, we believe that access to these new technologies will replicate the breakthroughs we have delivered in muscle diseases with increased efficacy and reduced toxicity in CNS diseases.

Recent Preclinical and Business Highlights

- **MDL-101 is advancing to IND**
  - Due to the reallocation of resources from the business restructuring, the forecast for IND filing has been moved back to 2025.
  - Established manufacturing process and analytical methods to achieve stable production and quality control of muscle tropic engineered AAV vectors
  - ODD and RPDD applications filed to FDA
- **Other programs**

- Reassess the value of other programs and the time and cost to reach future milestones, and consider possibilities for collaboration and derivation.
- The Partnership with Ginkgo Bioworks brings a diverse array of partners and opportunities in a wide range of areas, spanning AI, genetic medicines, biologics, and manufacturing.

- **Research collaboration**
  - Continue to work with JCR Pharma in the research collaboration announced last year that aim to validate the approach to address CNS targets by combining JCR's BBB-crossing capsid, JBC-AAV, and our payload base on CRISPR-GNDM technology.
  - Also announced the signing of a memorandum of understanding for research collaboration to develop next-generation therapeutics for Alzheimer's disease with Genixcure, a South Korean emerging company, on a modified capsid technology based on AI technology. (July)
- **IP updates**
  - GNDM-UTRN(MDL-201) patent granted in China (June)
  - GNDM-DM1(MDL202) patent issued in China (June)
- **Conference and presentation**
  - Past presentation
    - Published MDL-101 preclinical data at BioRxiv in May (https://doi.org/10.1101/2024.05.03.592438)
    - 4th Annual Next Generation Gene Therapy Vectors Summit (June 12-14)
    - Cell and Gene Therapy Summit 2024 (July 8-10)
  - Coming presentation
    - Bioprocessing Summit (Aug 20)
    - Gene Therapy Immunogenicity Summit (Aug 22)

Second Quarter 2024 Financial Results:

- **Cash Position**: Cash and deposits as of June 30, 2024, was ¥1,278 million, compared to ¥1,883 million as of December 31, 2023, a decrease of ¥605 million. The decrease in Cash and deposits was primarily due to R&D Expenses and G&A Expenses.
- **Research & Development (R&D) Expenses:** R&D expenses were ¥716 million for the six ended June 30, 2024, compared to ¥906 million for June 30, 2023, a decrease of ¥190 million. Decreases in R&D expenses were primarily due to decreases in development costs associated with the advancement of the Company's proprietary GNDM platform and product candidates.
- **General & Administrative (G&A) Expenses**: G&A expenses were ¥122 million for the six ended June 30, 2024, compared to ¥138 million for June 30, 2023, a Decrease of ¥16 million. Decreases in G&A expenses were primarily due to Decreased personnel costs.
- **Net Loss**: Net Loss was ¥781 million for the six ended June 30, 2024, compared to ¥1,033 million for June 30, 2023.
- 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 epigenome editing. 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 focuses primarily 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 Waltham, Massachusetts. For additional information, visit www.modalistx.com.

Forward-Looking Statements:

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Consolidated Financial Results for the Six Months Ended June 30, 2024 [Japanese GAAP]

August 7, 2024

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Stock exchange listing: Tokyo Stock Exchange
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Scheduled date of filing quarterly securities report: Aug 13, 2024
Scheduled date of commencing dividend payments: -
Preparation of supplementary material on financial results: Yes
Holding of financial results briefing: Yes (for securities analysts and institutional investors)

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

1. Consolidated Financial Results for the Six Months Ended June 30, 2024 (January 1, 2024, to June 30, 2024)

(1) Consolidated Operating Results (% indicates changes from the previous corresponding period.)

	Operating revenue		Operating income		Ordinary income		Profit attributable to owners of parent	
Six months ended	Million yen	%	Million yen	%	Million yen	%	Million yen	%
June 30, 2024	-	-	(838)	-	(780)	-	(780)	-
June 30, 2023	-	(100.0)	(1,044)	-	(995)	-	(1,033)	-

(Note)Comprehensive income: Six months ended June 30, 2024: ¥ (772) million [-%]
Six months ended June 30, 2024: ¥ (1,034) million [-%]

	Basic earnings per share	Diluted earnings per share
Six months ended	Yen	Yen
June 30, 2024	(21.98)	-
June 30, 2023	(34.70)	-

(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 June 30, 2024	1,409	1,112	77.8
As of December 31, 2023	2,025	1,380	66.8

(Reference) Equity: As of June 30, 2024: ¥1,096million
As of December 31, 2023: ¥1,353million