Modalis Therapeutics Reports Operational Highlights and Third Quarter 2024 Financial Results

07-Nov-2024 TOKYO & WALTHAM, Mass.- Modalis Therapeutics Corporation ("Modalis" 4883.T TSE), a pioneer in developing innovative solutions for rare genetic diseases through its proprietary CRISPR-GNDM® epigenetic gene editing technology, today announced its financial results for the third quarter ended September 30, 2014, as well as recent operational milestones.

"We are pleased with our progress in the third quarter. MDL-101, which we are developing for the treatment of LAMA1-CMD, a rare but devastating disease diagnosed in children, has been granted a Rare Pediatric Disease Designation by the U.S. FDA. In addition, in October, it was also designated as an Orphan Drug. We believe these are significant recognitions for us as the world's first gene therapy using epigenome editing technology," said CEO Morita. "We receive calls every week from patients and their families who are eagerly awaiting a cure, and we hope to use these approvals to further advance our development efforts."

Although the financing environment for biotech companies is extremely difficult, we were able to successfully raise funds through the second convertible bond with warrants offering. The conversion of the second convertible bond and the exercise of the 14th warrant were completed by the end of September and October respectively, raising approximately 2.5 billion yen. This financing has enabled us to secure the immediate funds necessary for the development of our pipeline, led by MDL-101, and to accelerate development.

In addition to the lead program MDL-101, we are accelerating the development of MDL-201, a treatment for DMD that may be able to treat a larger number of patients. We believe our proprietary technology for activating an alternative gene and restoring muscular function may offer meaningful benefits over exon-skipping drugs and other gene therapies in development for DMD. Our data demonstrate superior efficacy in comparative studies using animal models of the disease as a benchmark against existing gene therapies for DMD. By advancing this program, we aim to bring a best-in-class product to the market for this serious disease.

Recent Preclinical and Business Highlights

- **MDL-101 is advancing to IND**
  - Preparing for IND enabling studies in 2 species.
  - Working on the technology transfer of the established process to CDMO
  - Received RPDD (September) and ODD (October) assessments from the U.S. regulatory authorities
- **Other programs**
  - MDL-201(DMD): Accelerating development. Established an animal model and converted existing prototype molecules to muscle-specific capsids.
  - MDL-103 (FSHD): Preparation for collaborative research utilizing a disease model
- **Research collaboration**

- JCR Collaboration: Continuing our collaboration on central nervous system diseases by combining our CRISPR-GNDM payload with AAV capsids that can penetrate the blood-brain barrier (BBB).
- Genixcure Collaboration: Continuing our collaboration and AI-based capsid search for GC capsids for Alzheimer's disease.

- **IP updates**
  - Patent for modified Cas9 (US18/058,832) co-filed with the University of Tokyo, was granted in the US (September)
  - MDL-202(GNDM-DMPK) patent (JP 2022-518586) granted in Japan (September)
- **Conference and presentation**
  - Past presentations
    - Cell and Gene Therapy Summit (Jul 8-10 in Boston)
    - Bioprocessing Summit (Aug 19-22 in Boston)
    - Gene Therapy Immunogenicity Summit (Aug 22 in Boston)
    - Nanopore Community Meeting Boston (Sep 16-17, in Boston)
  - Coming presentation
    - 5th Genome Editing Therapeutics Summit (Dec 5 in Boston)

Third Quarter 2024 Financial Results:

- **Cash Position**: Cash and deposits as of September 30, 2024, was ¥2,743 million, compared to ¥1,956 million as of December 31, 2023, an increase of ¥786 million. The increase is due to the fact that the amount raised through corporate bonds and stock acquisition rights exceeded the amount spent on R&D Expenses and G&A Expenses.
- **Research & Development (R&D) Expenses:** R&D expenses were ¥1,062 million for the nine ended September 30, 2024, compared to ¥1609 million for September 30, 2023, a decrease of ¥547 million. The reason for the decrease is primarily the result of our review of R&D expenses, including the layoffs we carried out twice this year in April and July.
- **General & Administrative (G&A) Expenses**: G&A expenses were ¥180 million for the nine ended September 30, 2024, compared to ¥207 million for September 30, 2023, a Decrease of ¥27 million. Decreases in G&A expenses were primarily due to Decreased personnel costs.
- **Net Loss**: Net Loss was ¥1,062 million for the nine months ended September 30, 2024, compared to ¥1,542 million for September 30, 2023.
- Please refer to the 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 the use of epigenome editing. Modalis is developing treatments for rare genetic disorders using its proprietary CRISPR-GNDM technology, which allows for the specific modulation of gene expression or histone modification without the necessity for double-stranded DNA cleavage, gene editing, or base editing. Modalis' primary focus is on genetic disorders caused by loss of gene regulation, resulting in excess or insufficient protein production. This includes multiple neuromuscular disorders. The company is headquartered in Tokyo with laboratories and facilities in Waltham, 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, 2024 [Japanese GAAP]

November 7, 2024

Company name: Modalis Therapeutics Corporation
Stock exchange listing: Tokyo Stock Exchange
Code number: 4883
URL: https://www.modalistx.com/en/
Representative: Haruhiko Morita, CEO and Representative Director
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Scheduled date of filing quarterly securities report: -
Scheduled date of commencing dividend payments: -
Preparation of supplementary material on financial results: Yes
Holding of financial results briefing: -

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

1. Consolidated Financial Results for the Nine Months Ended September 30, 2024 (January 1, 2024, to September 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	
Nine months ended	Million yen	%	Million yen	%	Million yen	%	Million yen	%
September 30, 2024	-	-	(1,062)	-	(1,059)	-	(1,060)	-
September 30, 2023	-	(100.0)	(1,609)	-	(1,542)	-	(1,581)	-

(Note) Comprehensive income: Nine months ended September 30, 2024: ¥ (1,059) million [-%]
Nine months ended September 30, 2023: ¥ (1,586) million [-%]

	Basic earnings per share	Diluted earnings per share
Nine months ended	Yen	Yen
September 30, 2024	(27.21)	-
September 30, 2023	(52.29)	-

(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, 2024	2,811	2,512	88.5
As of December 31, 2023	2,025	1,380	66.8

(Reference) Equity: As of September 30, 2024: ¥2,487million
As of December 31, 2023: ¥1,353million