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
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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	
Three months ended	Million yen	%	Million yen	%	Million yen	%	Million yen	%
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: ¥ 210million [-%]

	Basic earnings per share	Diluted earnings per share
Three months ended	Yen	Yen
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

2. Dividends

	Annual dividends				
	1st quarter-end	2nd quarter-end	3rd quarter-end	Year-end	Total
	Yen	Yen	Yen	Yen	Yen
Fiscal year ended December 31, 2021	-	0.00	-	0.00	0.00
Fiscal year ending December 31, 2022	-				
Fiscal year ending December 31, 2022 (Forecast)		0.00	-	0.00	0.00

(Note) Revision to the forecast for dividends announced most recently: No

3. Consolidated Financial Results Forecast for the Fiscal Year Ending December 31, 2022(January 1, 2022 to December 31, 2022)

The earnings forecasts for fiscal year 2022 are not presented due to the difficulty of formulating reasonably accurate estimates at this time. For further details, please refer to page 3 “Explanation of Consolidated Financial Results Forecast and Other Forward-looking Information”

*** Notes:**

(1) Changes in significant subsidiaries during the three months ended March 31, 2022 (changes in specified subsidiaries resulting in changes in scope of consolidation): No
Notes on changes in significant subsidiaries during the three months ended March 31, 2022

(2) Accounting policies adopted specially for the preparation of quarterly consolidated financial statements: No
Notes on accounting policies adopted specially for the preparation of quarterly consolidated financial statements

(3) Changes in accounting policies, changes in accounting estimates and retrospective restatement
1) Changes in accounting policies due to the revision of accounting standards: Yes
2) Changes in accounting policies other than 1) above: No
3) Changes in accounting estimates: No
4) Retrospective restatement: No
Notes on changes in accounting policies

(4) Total number of issued shares (common shares)

1) Total number of issued shares at the end of the period (including treasury shares):

March 31, 2022: 29,062,500 shares

December 31, 2021: 28,967,500 shares

2) Total number of treasury shares at the end of the period:

March 31, 2022: 63 shares

December 31, 2021: 63 shares

3) Average number of shares during the period:

Three months ended March 31, 2022: 29,015,611 shares

Three months ended March 31, 2021: 28,546,560 shares

Notes on total number of issued shares

* These quarterly financial results are outside the scope of quarterly review by certified public accountants or audit firms.

* Explanation of the proper use of financial results forecast and other notes

The earnings forecast and other forward-looking statements herein are based on information available and certain assumptions judged to be reasonable as of the date of publication of this document. They do not represent a commitment from the Company that they will be achieved. Actual results may differ significantly from these forecast due to a wide range of factors. Please refer to page 3 of the attachment for matters related to the earnings forecast.

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1. Qualitative Information on Quarterly Financial Results for the Period under Review

(1) Explanation of Business Results

In Japan's economy during the first quarter of the current consolidated cumulative period, the outbreak of COVID-19 pandemic is showing signs of peaking out, but the outlook for economic activity is once again uncertain due to Russia's invasion of Ukraine.

Under the corporate philosophy of "Every Life Deserves Attention," the Group is committed to research and development with the aim of providing breakthrough precision therapeutics for genetic diseases that overlap substantially with rare diseases. Based on our proprietary CRISPR-GNDM® platform, which serves as the technological foundation, the Company has continued to lead cutting-edge research as a leading company since its inception in 2016 to the current consolidated period, which is the seventh fiscal year since its establishment as the world's first company to develop gene modulation therapy using CRISPR. We continue to make full-scale efforts toward clinical trials of programs including MDL-101.

In the past, we have been developing pipelines using CRISPR-GNDM® technology both on our own and in collaboration with partners, but we have begun to broaden our capabilities with the aim of more efficient and speedy research and development and horizontal deployment of our development know-how. As a result, we believe that we are now able to provide higher added value not only by promoting the "in-house pipeline" in which we develop therapeutic drugs with our own funds, but also by providing the know-how we have established to the "collaborative pipeline" in which we develop therapeutic drugs in collaboration with partners.

During the quarter, the Company made steady progress in research and development of its co-development pipeline, centered on MDL-101, as well as its in-house development pipeline. Development of MDL-101 is continuing with the aim of filing a IND by the end of 2023. The focus of our work is toxicology studies and process development. On the regulatory front, the U.S. Food and Drug Administration (FDA) issued a draft guidance on genome editing in March. We believe that this is reflection of the fact that genome editing is becoming more widely recognized as a major field in gene therapy as clinical trials of genome editing pipelines are underway at various companies. In February, the company submitted an INTERACT meeting application to the FDA for MDL-101, which was accepted in March, and we have received notification that the meeting will be held in mid-July. We are also preparing to file for a Pre-IND meeting by the end of 2022. We believe that this will clarify the hurdles to the start of the clinical trial and allow us to tune up the trial plan. In other pipelines, the collaboration pipeline with Eisai, MDL-205, has achieved its research milestones and the consideration for the milestones has been recorded. In addition, the collaboration period has been extended based on a mutual agreement.

In addition, the company is to present six abstracts at the annual meeting of the American Society for Gene and Cell Therapy (ASGCT), May 16-19, 2022, in Washington, D.C., USA. 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 dilated cardiomyopathy (DCM) that targets the Titin gene. This program has been newly selected as part of our in-house pipeline, which we have decided to develop.

In February, the second interferences between the Broad Institute and the University of California group regarding the rights of the CRISPR foundational patents in the U.S. were concluded in favor of the Broad Institute. 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 our company and our partner Astellas for " Method for treating muscular dystrophy by targeting utrophin gene" was granted in Japan, and the patent was registered in May. This is a new method of

treating Duchenne muscular dystrophy with a gene therapy drug using the GNDM method, and we believe that this is the result of its novelty and inventive step being recognized by the authorities.

As a result, for the three months ended March 31, 2022, operating revenue amounted to ¥40,500 thousand, operating loss to ¥466,718 thousand, ordinary loss to ¥443,703 thousand, and Loss attributable to owners of parent to ¥438,906 thousand.

The Group consists of a single business segment of developing therapeutic agents for genetic disorders. Accordingly, the Company omits statements of segment information.

(2) Explanation of Financial Position

Status of Assets, Liabilities and Net Assets

(Current Assets)

Total current assets at the end of the first quarter of the fiscal year under review decreased by ¥493,945 thousand from the end of the previous fiscal year to ¥4,573,098 thousand. This is mainly attributable to a decrease in cash and deposits by ¥531,220 thousand.

(Non-current Assets)

Total non-current assets at the end of the first quarter of the fiscal year under review increased by ¥48,869 thousand from the end of the previous fiscal year to ¥1,050,962 thousand. This is mainly attributable to an increase in Property, plant and equipment of ¥71,649 thousand.

(Current Liabilities)

Total current liabilities at the end of the first quarter of the fiscal year under review decreased by ¥9,584 thousand from the end of the previous fiscal year to ¥171,133 thousand. This is mainly attributable to a decrease in others by ¥9,685 thousand.

(Non-current Liabilities)

Total non-current liabilities at the end of the first quarter of the fiscal year under review decreased by ¥12,632 thousand from the end of the previous fiscal year to ¥326,574 thousand. This is attributable to a decrease in deferred tax liabilities by ¥5,052 thousand.

(Net Assets)

Total net assets at the end of the first quarter of the fiscal year under review decreased by ¥422,859 thousand from the end of the previous fiscal year to ¥5,126,352 thousand. This is mainly attributable to a decrease in retained earnings by ¥438,906 thousand thanks to the posting of loss.

(3) Explanation of Consolidated Financial Results Forecast and Other Forward-looking Information

At present, there are many uncertain factors affecting our business performance, including the progress of negotiations with our partners, significant fluctuations in performance due to upfront payments for licensing, the possibility of concluding agreements with new partners, and the acquisition of new pipelines. In regard to the future outlook, the Company believes it is difficult to calculate an appropriate and rational figure, so the Company has decided not to disclose the full-year earnings forecast. When it becomes possible to make a forecast, the Company will promptly make an announcement.

2. Quarterly Consolidated Financial Statements and Primary Notes

(1) Quarterly Consolidated Balance Sheets

(Thousand yen)

	As of December 31, 2021	As of March 31, 2022
Assets		
Current assets		
Cash and deposits	4,936,193	4,404,973
Accounts receivable - trade	—	44,550
Other	130,850	123,574
Total current assets	5,067,043	4,573,098
Non-current assets		
Property, plant and equipment	223,784	295,433
Intangible assets		
Right to use patent	704,729	689,051
Other	273	253
Total intangible assets	705,003	689,304
Investments and other assets	73,305	66,224
Total non-current assets	1,002,093	1,050,962
Total assets	6,069,137	5,624,061
Liabilities		
Current liabilities		
Income taxes payable	19,282	10,445
Provision for bonuses	—	8,938
Other	161,434	151,749
Total current liabilities	180,717	171,133
Non-current liabilities		
Provision for share-based compensation for director	988	855
Provision for share-based compensation for employee	4,775	3,946
Deferred tax liabilities	5,052	—
Other	328,391	321,772
Total non-current liabilities	339,207	326,574
Total liabilities	519,925	497,708
Net assets		
Shareholders' equity		
Share capital	2,744,447	2,746,760
Capital surplus	4,066,182	4,068,495
Retained earnings	(1,266,476)	(1,705,382)
Treasury shares	(97)	(97)
Total shareholders' equity	5,544,055	5,109,774
Accumulated other comprehensive income		
Foreign currency translation adjustment	5,156	16,578
Total accumulated other comprehensive income	5,156	16,578
Total net assets	5,549,212	5,126,352
Total liabilities and net assets	6,069,137	5,624,061

(2) Quarterly Consolidated Statements of Income and Comprehensive Income

Quarterly Consolidated Statements of Income

Three Months Ended March 31

(Thousand yen)

	For the three months ended March 31, 2021	For the three months ended March 31, 2022
Operating revenue	600	40,500
Operating expenses		
Research and development expenses	184,182	442,239
Selling, general and administrative expenses	69,053	64,979
Total operating expenses	253,236	507,218
Operating loss	(252,636)	(466,718)
Non-operating income		
Interest income	27	27
Subsidy income	205	—
Foreign exchange income	6,962	24,454
Other	29	—
Total non-operating income	7,223	24,481
Non-operating expenses		
interest expense	—	1,119
Stock issuance cost	397	346
Total non-operating expenses	397	1,466
Ordinary loss	(245,810)	(443,703)
Extraordinary income		
Income of compensation	485,881	—
Total extraordinary income	485,881	—
Profit before income taxes or Loss before income taxes	240,071	(443,703)
Income taxes - current	36,153	306
Income taxes - deferred	2,118	(5,103)
Total income taxes	38,271	(4,797)
Profit or Loss	201,800	(438,906)
Profit attributable to owners of parent or Loss attributable to owners of parent	201,800	(438,906)

Quarterly Consolidated Statements of Comprehensive Income

Three Months Ended March 31

(Thousand yen)

	For the three months ended March 31, 2021	For the three months ended March 31, 2022
Profit or Loss	201,800	(438,906)
Other comprehensive income		
Foreign currency translation adjustment	8,203	11,422
Total other comprehensive income	8,203	11,422
Comprehensive income	210,004	(427,484)
Comprehensive income attributable to		
Comprehensive income attributable to owners of parent	210,004	(427,484)

(3) Notes to Quarterly Consolidated Financial Statements

(Notes on going concern assumption)

There is no relevant information.

(Notes in the case of significant changes in shareholders' equity)

The share capital and the capital surplus both received an addition of ¥2,312 thousand due to the exercise of stock acquisition rights as a stock option. As a result, at the three months ended March 31, 2022, the share capital and capital surplus were ¥2,746,760 thousand, ¥4,068,495 thousand.

(Change in accounting policy)

(Application of the Accounting Standard for Revenue Recognition, etc.)

The Company has applied the "Accounting Standard for Revenue Recognition" (ASBJ Statement No. 29, March 31, 2020) and relevant ASBJ regulations from the beginning of the first quarter of the fiscal year under review, and recognizes revenue at the amounts expected to be received in exchange for promised goods or services, at the point when the control of such goods or services is transferred to customers. The Company has adopted the "Accounting Standard for Revenue Recognition" (ASBJ Statement No.84) in accordance with the transitional treatment. There is no effect on the beginning balance of retained earnings brought forward and profit and loss for the first quarter of the current fiscal year.

(Application of the Accounting Standard for Fair Value Measurement, etc.)

The Company has applied the "Accounting Standard for Fair Value Measurement" (ASBJ Statement No. 30, July 4, 2019) and relevant ASBJ regulations from the beginning of the first quarter of the fiscal year under review, and it has applied the new accounting policy provided for by the Accounting Standard for Fair Value Measurement, etc. prospectively in accordance with the transitional measures provided for in paragraph 19 of the Accounting Standard for Fair Value Measurement, and paragraph 44-2 of the "Accounting Standard for Financial Instruments" (ASBJ Statement No. 10, July 4, 2019). There is no impact on the quarterly consolidated financial statements from the application of this standard.

(Segment information)

The Group consists of a single business segment of developing therapeutic agents for genetic disorders. Accordingly, the Company omits statements of segment information.