Nippon Shinyaku Co., Ltd.

FY2025/Q1 Earnings Call

August 7, 2025

Event Summary

[Company Name]	Nippon Shinyaku Co., Ltd.	
[Event Name]	FY2025/Q1 Earnings Call	
[Date]	August 7, 2025	
[Number of Speakers]	3	
	Takanori Edamitsu	Director, General Manager, Business Management & Sustainability Division
	Manabu Beppu	Corporate Officer, Head of R&D Planning and Administration Dept.
	Hideyasu Takechi	Corporate Officer, Department Manager, Corporate Planning Dept.

Presentation

Edamitsu: I am Takanori Edamitsu, General Manager, Business Management and Sustainability Division, Nippon Shinyaku Co., Ltd.

Thank you very much for taking time out of your busy schedule to attend our financial results briefing today.

I will now explain our business performance and the progress of R&D for Q1 of FY2025 in line with the presentation materials posted on our website.

Q1 FY2025 Summary (consolidated)

(million yen)	Q1 FY2024		Q1 FY2025		YoY	
	actual	ratio	actual	ratio	change	%
Revenue	**39,131**	**100.0%**	**39,546**	**100.0%**	**+414**	**+1.1%**
(Pharmaceuticals)	(33,738)	(86.2%)	(34,163)	(86.4%)	(+425)	(+1.3%)
(Functional Food)	(5,393)	(13.8%)	(5,382)	(13.6%)	(-11)	(-0.2%)
Cost of sales	**12,636**	**32.3%**	**12,655**	**32.0%**	**+18**	**+0.1%**
SG&A expenses	**9,221**	**23.6%**	**9,995**	**25.3%**	**+773**	**+8.4%**
R&D expenses	**7,497**	**19.2%**	**6,189**	**15.7%**	**-1,308**	**-17.4%**
Other income	**1,507**	**3.9%**	**169**	**0.4%**	**-1,337**	**-88.7%**
(Foreign exchange gain)	(1,211)	(3.1%)	-	-	(-1,211)	-
Other expenses	**204**	**0.5%**	**794**	**1.9%**	**+589**	**+288.8%**
(Foreign exchange loss)	-	-	(645)	(1.6%)	(+645)	-
Operating profit	**11,078**	**28.3%**	**10,081**	**25.5%**	**-996**	**-9.0%**
Finance income	**363**	**0.9%**	**468**	**1.2%**	**+105**	**+29.0%**
Finance costs	**31**	**0.1%**	**46**	**0.1%**	**+15**	**+49.5%**
Profit before tax	**11,411**	**29.2%**	**10,504**	**26.6%**	**-907**	**-7.9%**
Income tax expense, etc.	**1,146**	**2.9%**	**2,248**	**5.7%**	**+1,102**	**+96.1%**
Profit attributable to owners of parent	**10,264**	**26.2%**	**8,255**	**20.9%**	**-2,009**	**-19.6%**

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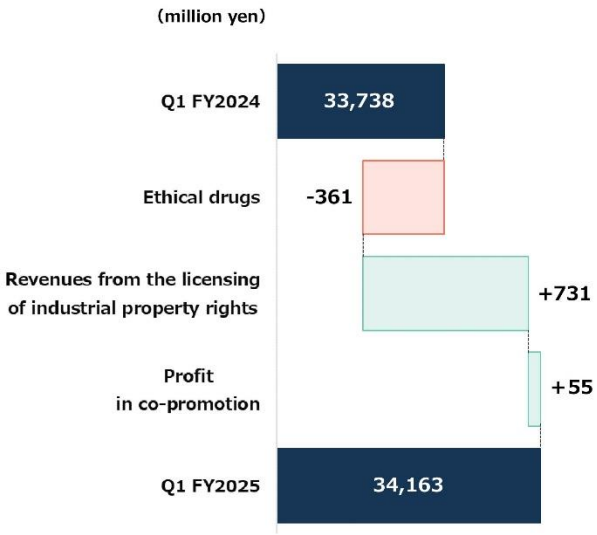
Please take a look at slide two. This is a summary of the results for Q1 of FY2025.

Consolidated revenue was JPY39,546 million, operating profit was JPY10,081 million, profit before tax was JPY10,504 million, and profit attributable to owners of parent was JPY8,255 million.

Segmental Review - Pharmaceuticals -



Ethical drugs 20,134 million yen (-361 million yen, -1.8%, YoY)

- ✓ Negative impacts of the NHI drug price revisions and generic drugs (Vidaza, etc.)
- ✓ Sales increase of Vyxeos, Fintepla, Uptravi, etc.

Revenues from the licensing of industrial property rights 11,511 million yen (+731 million yen, +6.8%, YoY)

- ✓ Increase in royalty income from Uptravi's overseas sales

Profit in co-promotion 2,517 million yen (+55 million yen, +2.3%, YoY)

- ✓ Launch of Yuvanci

Please turn to slide three.

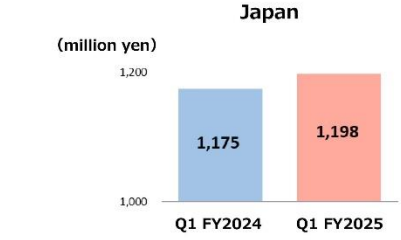
In the pharmaceuticals segment, despite the effects of NHI drug price revisions and generic drugs, growth in sales of Vyxeos, Fintepla, and Uptravi, as well as royalty profit from overseas sales of Uptravi, resulted in consolidated revenue of JPY34,163 million, up 1.3% YoY.

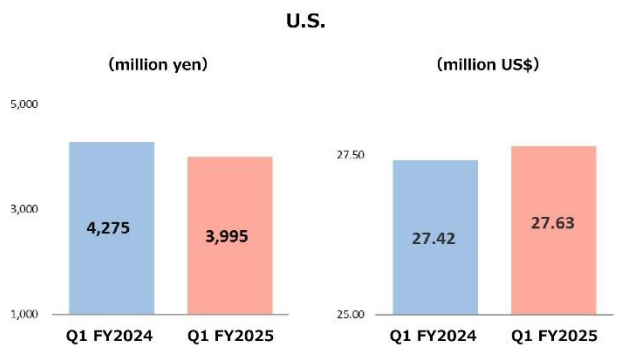
Sales Trends of Viltepso (viltolarsen)

(million yen)	Q1 FY2024 actual	Q1 FY2025 actual	YoY change	YoY %	FY2025 forecast	Notes on Q1 FY2025 results
Japan	1,175	1,198	+23	+2.0%	4,800	✓ The number of patients currently on therapy with Viltepso is more than three-quarters of the peak number of 128 patients in the data from Chuikyo[1].
US	4,275	3,995	-279	-6.5%	16,700	✓ Number of new patients has been increasing after P3 study results. ✓ Due to stricter insurance reauthorizations after launch of multiple DMD treatment options, growth rate of new patient acquisition is getting slower.
(million US$)	(27.42)	(27.63)	(+ 0.21)	(+0.8%)	(119.28)	
Total	5,450	5,194	-256	-4.7%	21,500	

1. Central Social Insurance Medical Council

Exchange rates	Q1 FY2024 actual	Q1 FY2025 actual	FY2025 forecast
USDJPY	155.9	144.6	140.0

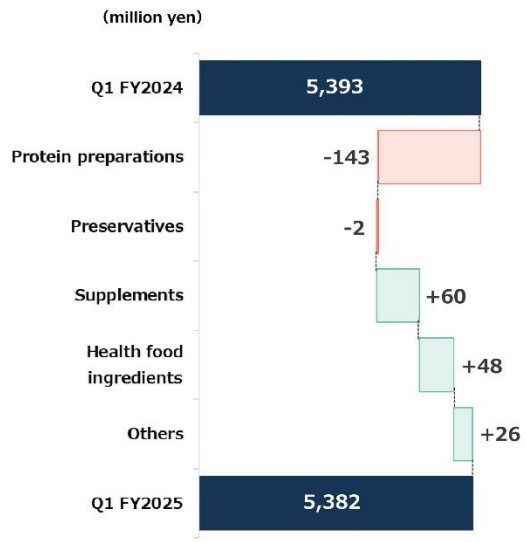




Next, slide four shows the sales of Viltepso, which is sold in Japan and the US. Sales in Japan and the US amounted to JPY1,198 million and JPY3,995 million, respectively.

Sales in the US, converted to yen, decreased due to exchange rate effects, but increased on a US dollar basis.

Segmental Review - Functional Food -



Protein preparations 3,319 million yen
(-143 million yen, - 4.2%, YoY)
- ✓ Sales prices decline of protein preparations for the processed food industry
- ✓ Decrease in demand for protein due to customers switching to cheaper raw materials

Preservatives 797 million yen
(-2 million yen, -0.4%, YoY)
- ✓ Promoting consultative sales to key users

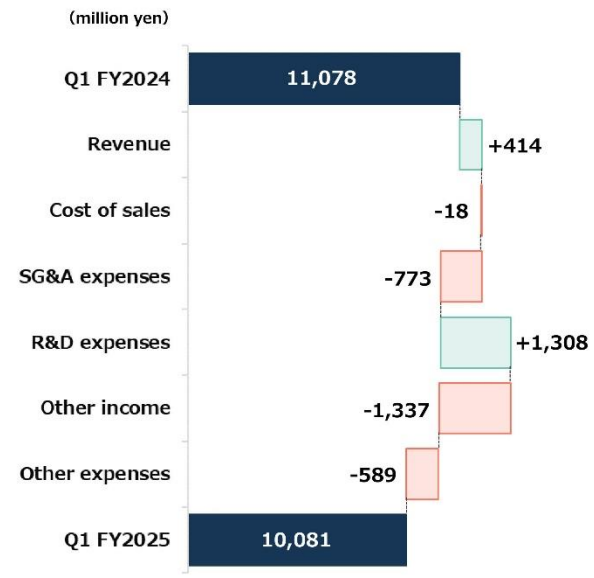
Supplements 612 million yen
(+60 million yen, +10.9%, YoY)
- ✓ Sales increase in both athletes and anti-aging care category

Health food ingredients 309 million yen
(+48 million yen, +18.7%, YoY)

Please move on to slide five.

In the functional food segment, sales of supplements and health food ingredients increased, but sales of protein preparations declined, resulting in consolidated revenue of JPY5,382 million, down 0.2% YoY.

Operating Profit



Revenue 39,546 million yen
(+414 million yen, +1.1%, YoY)
- ✓ Sales increase of Vyxeos, Fintepla and Uptravi, etc.
- ✓ Increase in royalty income from Uptravi's overseas sales

Cost of sales 12,655 million yen
(+18 million yen, +0.1%, YoY)
The ratio was 32.0%, improved by 0.3% points YoY.
- ✓ Increase in revenues from industrial property rights
- ✓ Change in product mix of Pharmaceuticals segment

SG&A expenses 9,995 million yen
(+773million yen, + 8.4%, YoY)
- ✓ Increase in the U.S. sales expenses of NS Pharma
- ✓ Increase in commission for promotional activities of Uptravi due to domestic sales increase

R&D expenses 6,189 million yen
(-1,308 million yen, -17.4%, YoY)
- ✓ Decrease in raw material costs related to investigational products

Please turn to slide six.

The cost of sales ratio improved by 0.3 percentage point YoY to 32%, due to factors such as the sales mix, in addition to an increase in revenue from industrial property rights.

Selling, general, and administrative expenses totaled JPY9,995 million, up 8.4% YoY, due to an increase in sales promotion expenses at a US subsidiary, NS Pharma, and an increase in commission for promotional activities for Uptravi.

R&D expenses decreased by 17.4% YoY to JPY6,189 million due to a decrease in raw material costs related to investigational drugs.

As for the impact of exchange rate, the foreign exchange profit, which was recorded in the same period last fiscal year, was eliminated and the foreign exchange loss was recorded this fiscal year. As a result, operating profit was JPY10,081 million, down 9% YoY.

Revised Business Forecast for FY2025 (consolidated)

We have revised our FY2025 business forecast due to the regulatory schedule change for CAP-1002, but our profit forecast remains unchanged.

(Million yen)	FY2025 Forecasts		Change	
	Previous*	Revised	Amt	%
Revenue	173,000	166,000	-7,000	-4.0%
(Pharmaceuticals)	(150,000)	(143,000)	(-7,000)	(-4.7%)
(Functional Food)	(23,000)	(23,000)	-	-
Cost of sales	55,200	51,200	-4,000	-7.2%
SG&A expenses	47,000	44,000	-3,000	-6.4%
R&D expenses	39,500	39,500	-	-
Other income	600	600	-	-
Other expenses	1,900	1,900	-	-
Operating profit	30,000	30,000	-	-
Finance income	700	700	-	-
Finance costs	100	100	-	-
Profit before tax	30,600	30,600	-	-
Income tax expense, etc.	6,600	6,600	-	-
Profit attributable to owners of parent	24,000	24,000	-	-

* May 8, 2025 (in FY2024 financial results announcement)

The exchange rate assumed in the business forecast is 1 USD=140 yen.
The sensitivity of the exchange rate after Q2 in FY2025 is assumed to be an increase of approx. 360 million yen in revenue and approx. 330 million yen in operating profit for every 1 yen depreciation of the yen.

For our most recent disclosure about CAP-1002, please see the link below (July 14, 2025)
https://www.nippon-shinyaku.co.jp/english/ir/ir_news.php?id=3416

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Please take a look at slide seven for the full-year forecast for FY2025.

The consolidated business forecast for FY2025 has been revised from that announced on May 8, due to a change in the expected approval date of CAP-1002 in the US.

Revenue is expected to decrease by JPY7 billion, while cost of sales and SG&A expenses are expected to decrease by JPY4 billion and JPY3 billion, respectively.

Operating profit and other profits remain unchanged.

Revised Business Forecast for FY2025 (consolidated)

(million yen)	FY2024 actual	FY2024 ratio	FY2025 forecast	FY2025 ratio	YoY change	YoY %
Revenue	160,232	100.0%	166,000	100.0%	+5,767	+3.6%
(Pharmaceuticals)	(138,654)	(86.5%)	(143,000)	(86.1%)	(+4,345)	(+3.1%)
(Functional Food)	(21,577)	(13.5%)	(23,000)	(13.9%)	(+1,422)	(+6.6%)
Cost of sales	51,116	31.9%	51,200	30.8%	+83	+0.2%
SG&A expenses	38,011	23.7%	44,000	26.5%	+5,988	+15.8%
R&D expenses	34,341	21.4%	39,500	23.8%	+5,158	+15.0%
Other income	874	0.5%	600	0.4%	-274	-31.4%
Other expenses	2,186	1.4%	1,900	1.1%	-286	-13.1%
Operating profit	35,450	22.1%	30,000	18.1%	-5,450	-15.4%
Finance income	830	0.5%	700	0.4%	-130	-15.7%
Finance costs	145	0.0%	100	0.1%	-45	-31.2%
Profit before tax	36,135	22.6%	30,600	18.4%	-5,535	-15.3%
Income tax expense, etc.	3,577	2.3%	6,600	4.0%	+3,022	+84.5%
Profit attributable to owners of parent	32,558	20.3%	24,000	14.5%	-8,558	-26.3%

Foreign exchange rates (USDJPY)

Q1 FY2024 actual	Q1 FY2025 actual	FY2025 forecast
155.9	144.6	140.0

The sensitivity of the exchange rate after Q2 in FY2025 is assumed to be an increase of approx. 360 million yen in revenue and approx. 330 million yen in operating profit for every 1 yen depreciation of the yen.

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Please turn to slide eight.

I will now explain our outlook for FY2025 based on the revised forecast.

Consolidated revenue is expected to be JPY166 billion.

As for operating expenses, the cost-of-sales ratio is expected to be 30.8%, an improvement of 1.1 percentage point YoY.

SG&A expenses are expected to be JPY44 billion and R&D expenses JPY39.5 billion.

As a result, we project operating profit of JPY30 billion, profit before tax of JPY30.6 billion, and profit attributable to owners of parent of JPY24 billion.

Revenue Forecast – Pharmaceuticals Segment -

(million yen)	FY2024		FY2025		YoY	
	Q1 actual	FY actual	Q1 actual	FY forecast	change	%
Ethical drugs	20,496	83,898	20,134	85,900	+2,001	+2.4%
Revenues from the licensing of industrial property rights	10,779	45,585	11,511	47,500	+1,914	+4.2%
Profit in co-promotion	2,461	9,170	2,517	9,600	+429	+4.7%
Revenue	33,738	138,654	34,163	143,000	+4,345	+3.1%

Despite the negative impact of NHI drug price revisions and generic competition, Pharmaceuticals Segment is expected to grow due to the following factors:

1. **Sales increase of new products in Japan such as Vyxeos, Fintepla, Uptravi, etc.**
2. **Increase in royalty income from Uptravi's overseas sales**

Please move on to slide nine, the pharmaceuticals segment. Despite the effects of NHI price revisions and generic drugs, the Company forecasts revenue of JPY143 billion, up 3.1% YoY, due to growth in sales of Vyxeos, Fintepla, and Uptravi, as well as an increase in royalty income associated with overseas sales of Uptravi.

Revenue Forecast - Functional Food Segment-

(million yen)	FY2024		FY2025		YoY	
	Q1 actual	FY actual	Q1 actual	FY forecast	change	%
Protein preparations	3,463	13,485	3,319	13,900	+414	+3.1%
Preservatives	800	3,278	797	3,400	+121	+3.7%
Supplements	552	2,415	612	3,500	+1,084	+44.9%
Health food ingredients	260	1,122	309	1,100	-22	-2.0%
Others	316	1,276	343	1,100	-176	-13.8%
Revenue	5,393	21,577	5,382	23,000	+1,422	+6.6%

Sales increase is expected through development and launch of new products and strengthened sales efforts in key products.

Next is the functional food segment, please refer to slide 10.

Full-year revenue is expected to be JPY23 billion, an increase of 6.6% YoY due to growth in sales of supplements and protein products.

Next, I will talk about R&D updates, focusing on items that have been updated since the release of the FY2025 financial statements.

R&D Updates for the Last 12 Months (1/2)

For updates since FY2024 financial results announcement on May 8, 2025, see highlighted text in red.

Recent status/event	Code No. (Generic name)	Brand name	Indications and topics	Schedule
P3	NS-065/NCNP-01 (viltolarsen)	Viltepso	Currently waitng for the FDA's feedback on 1. Study 301 data 2. Protocol of Study 303	April 2025
Launch	NS-304 (selexipag)	Uptravi	Uptravi Tablets for Pediatric 0.05 mg	March 2025
Additional indication	NS-304 (selexipag)	Uptravi	pediatric pulmonary arterial hypertension	December 2024
Launch	ACT-064992D (macitentan / tadalafil)	Yuvanci	pulmonary arterial hypertension	November 2024
Launch	LY3527727 (piltobrutinib)	Jaypirca	patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors	August 2024
Filed	CAP-1002 (deramiocel)	–	Duchenne muscular dystrophy cardiomyopathy (Capricor received CRL[1] from FDA)	July 2025 (U.S.)
Filed	RGX-121 (clemidsogene lanparvovec)	–	Mucopolysaccharidosis Type II (FDA accepted BLA[2] and assigned PDUFA date[3] of Nov. 9, 2025)	May 2025 (U.S.)
Filed	NS-401 (tagraxofusp)	–	blastic plasmacytoid dendritic cell neoplasm (BPDCN)	March 2025
P3	ZX008 (fenfluramine hydrochloride)	–	UCB announced that P3 for CDKL5 deficiency disorder (CDD) indication met primary and most key secondary clinical endpoints	June 2025
Start of P1/2	NS-050/NCNP-03	–	Duchenne muscular dystrophy	October 2024

1. Complete Response Letters (CRLs) are issued directly to product sponsors when the FDA completes its review cycle and determines that it cannot grant an approval of an application in its current form.
2. BLA : Biologics License Application
3. PDUFA date : the target action date for completion of the review by the FDA

Please take a look at slide 12.

In July, Capricor received a Complete Response Letter from the Food and Drug Administration for CAP-1002.

I will explain this matter in more detail later.

The FDA accepted the Biologics License Application for RGX-121 in May, and the target action date for completion of the FDA's review was set for November 9 of this year.

In June, UCB announced that the primary endpoint was met in the global Phase III study of ZX008 in patients with CDKL5 deficiency disorder.

R&D Updates for the Last 12 Months (2/2)

For updates since FY2024 financial results announcement on May 8, 2025, see highlighted text in red.

Recent status/event	Code No. (Generic name)	Brand name	Indications and topics	Schedule
In-license agreement signed (REGENXBIO Inc.)	RGX-121 (clemidsogene lanparvovec)	–	Mucopolysaccharidosis Type II	January 2025 (U.S. and Asia including Japan)
	RGX-111	–	Mucopolysaccharidosis Type I	
In-license agreement signed (Atsena Therapeutics)	ATSN-101	–	GUCY2D-associated Leber congenital amaurosis	November 2024 (U.S. and Japan)
Letter of Intent signed (Capricor Therapeutics)	CAP-1002 (deramiocel)	–	executed a Letter of Intent stipulating the exclusive right to negotiate over the next few months an exclusive distribution agreement for CAP-1002 in Europe	September 2024 (Europe)
Option Agreement signed for Commercialization (AB2 BIO Ltd.)	Tadekinig alfa	–	NLRC4 mutation and XIAP deficiency	January 2025 (U.S.)
Research Alliance (Boston Children's Hospital)	–	–	a strategic alliance with the aim of developing and delivering innovative therapies for rare diseases	July 2025 (U.S.)
Orphan Drug Designation	NS-229	–	eosinophilic granulomatosis with polyangiitis (EGPA)	April 2025 (U.S.)
Rare Pediatric Disease Designation	NS-051/NCNP-04	–	Duchenne muscular dystrophy	January 2025 (U.S.)
Rare Pediatric Disease Designation	NS-050/NCNP-03	–	Duchenne muscular dystrophy	August 2024 (U.S.)
Senkuteki Iyakuhin (Pioneering Drug) Designation and Orphan Drug Designation	NS-089/NCNP-02 (brogidirsen)	–	Duchenne muscular dystrophy	December 2024 (Japan)
Publication			the results of an investigator-initiated clinical trial (First in human trial) in Cell Reports Medicine	January 2025
Publication	NS-065/NCNP-01 (viltolarsen)	Viltepso	the results of Phase 2 trial (Galactic53 trial) in Scientific Reports	October 2024

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Please turn to slide 13.

As a research alliance, in July we signed a strategic alliance agreement with Boston Children's Hospital for the development of innovative therapies for rare diseases.

CAP-1002 (deramiocel) update

July 11, 2025

Capricor Therapeutics

Capricor Therapeutics Provides Regulatory Update on Deramiocel BLA for Duchenne Muscular Dystrophy

- *FDA issued Complete Response Letter*
- *Capricor plans to resubmit its BLA to include data from the ongoing Phase 3 HOPE-3 trial in Q3 2025 to continue pursuing the indication for the treatment of cardiomyopathy associated with Duchenne muscular dystrophy*
- *FDA advised Capricor to request a meeting to determine next steps toward potential approval*
- *Conference call and webcast scheduled for today at 8:30 a.m. ET*

SAN DIEGO, July 11, 2025 (GLOBE NEWSWIRE) -- Capricor Therapeutics (NASDAQ: CAPR), a biotechnology company developing transformative cell and exosome-based therapeutics for rare diseases, today announced that it has received a Complete Response Letter (CRL) from the U.S. Food and Drug Administration (FDA) regarding its Biologics License Application (BLA) for Deramiocel, the Company's lead cell therapy candidate for the treatment of cardiomyopathy associated with Duchenne muscular dystrophy (DMD).

In the CRL, the FDA stated that it had completed its review of the application but is unable to approve the BLA in its current form, specifically citing that the BLA does not meet the statutory requirement for substantial evidence of effectiveness and the need for additional clinical data. The CRL also referenced certain outstanding items in the Chemistry, Manufacturing, and Controls (CMC) section of the application, most of which Capricor believes it has addressed in prior communications to the FDA. However, these materials were not reviewed by the FDA due to the timing of the CRL issuance. The FDA confirmed that it will restart the review clock upon resubmission. In addition, the agency offered the company the opportunity to request a Type A meeting to discuss the path forward. Capricor plans to engage further with the FDA to determine the appropriate next steps.

Capricor's BLA for Deramiocel was granted Priority Review in March 2025 and was supported by data from the HOPE-2 trial, its open-label extension (OLE), and natural history comparisons from FDA-funded datasets.

✓ **FDA issued a Complete Response Letter (CRL)[1] for Capricor's BLA[2] for CAP-1002 (deramiocel).**

✓ **Capricor has requested a Type A meeting[3] to determine the path forward toward potential approval.**

1. Complete Response Letters (CRLs) are issued directly to product sponsors when the FDA completes its review cycle and determines that it cannot grant an approval of an application in its current form.
2. BLA : Biologics License Application
3. Type A Meetings are reserved for discussions necessary for an otherwise stalled product development program to proceed or to address an important safety issue.

Source: July 11, 2025, press release from Capricor Therapeutics
Capricor Therapeutics Provides Regulatory Update on Deramiocel BLA for Duchenne Muscular Dystrophy :: Capricor Therapeutics, Inc. (CAPR)

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Please move on to slide 14.

Lastly, I would like to explain the current status of CAP-1002 based on Capricor's disclosure.

The target action date for completion of the review, which was scheduled for August 31, became undecided due to Capricor's receipt of a Completion Response Letter from the FDA.

Capricor intends to resubmit the data, including the Phase III HOPE-3 trial data.

Top-line data on the HOPE-3 trial is expected by the end of September, and the Company will continue to prepare for sales.

The future schedule and other details will be discussed by Capricor and the FDA at the Type-A meeting.

This concludes my explanation of the results for Q1 of FY2025 and the progress in the area of research and development.

Question & Answer

Shimokawa [M]: We will now begin the question-and-answer session.

If you have any questions, please press the “raise your hand” button in the shape of a palm at the bottom center of the screen. If you are connecting from a telephone, please raise your hand by pressing the star key and 9, then follow the voice guidance and press the star key and 6 to unmute.

When asking questions, please state your company name and your name before you speak.

Now, we will take your questions. Let’s welcome Mr. Yamaguchi from Citigroup Global Markets.

Yamaguchi [Q]: Thank you. I would like to ask you a few simple questions.

First, in the area of expenses, especially R&D expenses, if we compare with the annual plan, the usage rate seems to be quite low up to Q1, although this may occur sometime. Please tell me if you have any reasons for this low rate. Or please comment on the progress of the costs, including the probability of using it in the future.

Takechi [A]: Regarding R&D expenses, the progress rate in Q1 was 35% of the H1 plan, which may seem a little behind schedule, but we originally planned to incur more contract research expenses in Q2 than in Q1. I think we will make progress as planned. As for the year, we expect to make progress as originally planned.

Yamaguchi [Q]: Thank you. Also, you have disclosed some information about the situation in the US regarding Viltepso. Could you please explain a little more about the contents? Especially for gene therapy, the launch, I think, has now been partially halted. Could you please explain that impact, including its impact on insurance reimbursement?

Takechi [A]: As for the status of Viltepso's sales in the US, as I mentioned in the FY2024 Financial Results presentation, the number of patients who have been using the drug for a number of years has been increasing, and it is taking time for insurance reimbursement procedures to be completed.

While we have seen some patients drop out, we are also working on acquiring new patients. In fact, there are patients who enrolled in Q1, but some of these patients have not yet been administered. Their administration is scheduled to start from July onward, and I believe that progress will generally be made as planned.

Yamaguchi [Q]: Is the fact that there are multiple DMD drugs not a particularly new story?

Takechi [A]: That is something I have said before.

Yamaguchi [Q]: I understand. I think you mentioned that some patients switched to gene therapy in the past, but is there any impact on Viltepso?

Takechi [A]: So far there have been no major changes.

Yamaguchi [Q]: I understand. Also, as for how you removed deramiocel from the earnings forecast, it has been removed from the sales, gross profit, and SG&A expenses. Especially for SG&A expenses, for this fiscal year, since you mentioned that you are still in the process of preparing, are you removing some of them, or are you removing all of them? Can you tell me about how you did?

Takechi [A]: As for sales preparation, we have already used some of it. Excluding that, we have removed a portion of the preparation for future launches and operating expenses after the product is launched.

Yamaguchi [Q]: Is it correct to say that this cost of sales are the figures after such measures?

Takechi [A]: Yes, it is. However, there was a slight increase or decrease in the other non-disclosed items, which is also included. Not all are attributable to CAP-1002.

Yamaguchi [M]: I understand. Thank you very much. That is all.

Shimokawa [M]: Thank you very much. Now, Mr. Tanaka from Mizuho Securities, please ask your question.

Tanaka [Q]: I am Tanaka from Mizuho Securities. Thank you very much.

First, I would like to ask about CAP-1002. I understand that the result of the HOPE-3 trial will be released by the end of September, so I would like to know how it is reviewed after the result is resubmitted. Maybe your company has nothing to say until after the Type-A meeting is over.

In the meantime, Capricor is saying that the review will resume upon resubmission and the current BLA will be maintained. Can I assume that the time clock starts again from there? Are you sure that this will not turn out to be a complete reapplication?

Beppu [A]: I think this will not. Capricor recently held a meeting for investors, and the CEO said so. However, I believe Capricor said that it would negotiate such a policy with the FDA at the Type-A meeting, so I understand that this depends on the outcome of the Type-A meeting with the FDA.

Tanaka [Q]: Originally, in the HOPE-2 trial, I think it was mentioned in the *Lancet* that 84 patients were planned to be included, but it was finished early with 20 patients, and it was agreed with the FDA that Phase III should start earlier. Your company may not be able to say anything different from Capricor, but rather than a statistical analysis, was the fact that there were only these 20 patients a problem?

Beppu [A]: According to what was mentioned by Capricor at the conference, the Complete Response Letter said that the BLA could not be approved with HOPE-2 data. As Mr. Tanaka just said, I believe that they made such a decision because the number of patients was insufficient as a result.

Therefore, since the data from the HOPE-3 trial will be available soon, Capricor will make up the shortfall in the number of patients and resubmit the data to move the time clock again. With such idea, Capricor will participate in the Type-A meeting

Tanaka [Q]: I understand. Thank you very much. Also, have there been any changes since April regarding the additional Phase III study of Viltepso? I think the other day you mentioned that you had made an inquiry but had not yet received a response.

Beppu [A]: There have been no updates since then. We have been inquiring regularly but have yet to receive a response from the FDA.

Tanaka [Q]: Okay. And I have one more question. I think NS-089 had been pushed back at the end of the fiscal period, and the study seems to have been delayed. I think Avidity is about to overtake it. Are there any changes here now? Are there any further delays?

Beppu [A]: Last time, we set the domestic launch target a year later in FY2028 and the US launch in FY2027. Since then, things have been going well, and so far, the study is underway to achieve the previously reported launch date.

Tanaka [Q]: One more thing. This is about a strategic alliance with Boston Children's Hospital. I don't know what kind of disease this is about. Is this related to nucleic acids?

Beppu [A]: We do not disclose specific indications. Specific modalities are also not disclosed at this time. We are sorry.

Tanaka [Q]: Not disclosed. I understand. Has Boston Children's Hospital ever done this kind of partnership? I haven't heard much about it.

Beppu [A]: They have concluded similar alliances with other companies, and I have heard that they have already formed similar alliances with about 10 companies.

Tanaka [M]: I understand. Thank you very much.

Shimokawa [M]: Thank you very much. Next, Mr. Hashiguchi from Daiwa Securities, please ask your question.

Hashiguchi [Q]: I am Hashiguchi. Thank you very much.

I would like you to comment on your company's speculation or interpretation of why discussions with the FDA for additional studies of Viltepso have not progressed.

I believe that when you originally submitted the protocol, you said that the review would be completed in about 60 days. Even though the planned period has already passed, you have not been able to hear the FDA's opinion. Is this because the person in charge has changed, or because there is no one in charge now, or because the FDA has intentionally delayed making a decision? What are your thoughts on this? I would appreciate it if you could give me an outlook as to when this is likely to settle in the future.

Beppu [A]: Originally, the 301 Study data was to be submitted as an additional document requested for the Type-C meeting last October. The expectation was that once we submit the materials for the Type-C meeting, the FDA usually takes about two or two and a half months to complete its review, and then the meeting will be held. We said that it would take about two or three months, based on that time frame.

We have since checked the status of the review after submitting it, but have not received a clear response. We do not know the exact reason. In the meantime, there have been changes in the upper management of the FDA and a change in the director of CDER at the end of July. I think there might be some kind of stagnation in decision-making within the FDA as a result of such change in upper management.

We have not heard of any specific changes in the review team members, so we will continue to make inquiries to the FDA and prepare for the meeting.

Hashiguchi [Q]: So are you saying that the timing of such a move has not yet been determined?

Beppu [A]: Yes. There is no timetable at this time.

Hashiguchi [Q]: In light of this situation, what do you think the status of conditional approval in Japan will be in the future?

Beppu [A]: For Japan, we are continuing to communicate with the Japanese authorities, sharing with them the status of the FDA's review, the status of our dialogue with the FDA, and the materials that we have submitted.

Hashiguchi [Q]: Thank you very much. I think that the start of Phase III will be progressively delayed. Is there any possibility that this will be a downside factor for R&D expenses in the current fiscal year?

Takechi [A]: Well, we have not factored in a lot in the current fiscal year. I think it will be somewhat like that.

Hashiguchi [M]: I understand. That is all. Thank you very much.

Shimokawa [M]: Thank you very much. Next, Mr. Sakai from UBS Securities, please ask your question.

Sakai [Q]: I am Sakai from UBS. This may not be a quality question, though.

First, I think that the sales forecast for CAP-1002 that was originally included at the beginning of the fiscal year was JPY7.3 billion, and now the Company's sales forecast has been reduced by JPY7 billion in total. You mentioned earlier that there were some revisions in the other non-disclosed items, but almost all of the downward revision was from CAP-1002.

Simply calculating the cost of this, the cost has been reduced by JPY4 billion, so I guess the cost ratio is 57%. I wonder if this cost ratio assumption will change when CAP-1002 comes out to the market in the future. Or is it correct for us to calculate our model with this cost ratio?

Takechi [A]: Regarding the cost ratio, I believe Capricor has disclosed in the contract with Capricor that the supply price is 30% to 50% of sales, with royalties included.

As for the JPY4 billion against JPY7 billion, in addition to the supply price, the upfront payment, milestone payments up to that point, and milestone payments that were originally scheduled to be paid when the project was approved on August 31 were included as depreciation after approval.

Sakai [Q]: I am not sure if you mentioned this before, but we can assume that the cost ratio is approximately 30%. Is that correct?

Takechi [A]: Yes. I can only answer that it is between 30% and 50%, but it is somewhere in between.

Sakai [Q]: I understand. Thank you very much. I have one more question. With regard to the PAP of Viltepso, or Patient Assistance Program, or you might call it a support program, what is the current status after the results of the Phase III 301? Has that been temporarily halted, or continued in one way or another? I saw in the data for Q1 that the number of patients has been increasing. What is the current practical situation?

Takechi [A]: Regarding the status of patient support services, there has been no change in that area as in the past. We have not changed our service and support system for patient entry.

Sakai [Q]: So the patients are coming, too.

Takechi [A]: That's right. In fact, as I mentioned earlier, new patients have come to register. We are preparing for home care, support for insurance reimbursement procedures, and other such support, and we are working on those areas.

Sakai [Q]: I understand. Sorry. Now I just need to check one point. You mention that the screening process for this insurance reimbursement will be more stringent. What exactly does this mean? Are you talking about segregating patients in some way or reducing coverage?

Takechi [A]: There are cases in which patients who were originally reimbursed by insurance are no longer reimbursed due to the progression of their disease, so there are some cases that cannot be reimbursed depending on the progression of the disease.

Sakai [Q]: That's not due to a stricter rule, is it? I think that according to the original protocol, patients should be naturally removed from insurance as their medical conditions progressed. Are you saying that this process will become even more stringent?

Takechi [A]: There are patients who are not reimbursed that way.

Sakai [Q]: So there's not so much change in the policy of Medicare or Medicaid, but what you're saying is a stricter application of the rule.

Takechi [A]: The rule has not changed, but the situation is such that the rules have no longer been applied because of progression of the disease from when they originally began to receive reimbursement for them.

Sakai [M]: I don't think what you said is about a stricter rule. Well, okay. Maybe it's something about wording. I understand. Thank you very much.

Shimokawa [M]: Thank you very much. Next, Mr. Wakao from JPMorgan Securities, please ask your question.

Wakao [Q]: I am Wakao from JPMorgan Securities. Thank you for taking my question.

The first question is about the gross margin in the revised plan. I understand that the gross profit margin appears to have improved by excluding CAP-1002 or deramiocel, because the product with high cost ratio was removed. The gross margin on this revised plan appears somewhat high, and you mentioned earlier that other areas have been revised, so is this related here? I would appreciate your comments on the gross margin for this revised plan.

In terms of revenue structure, I don't think it is particularly different from Q1. The Q1 result was 68%, so the gross margin in this revised plan appears slightly higher. Please let me know if there are any factors.

Takechi [A]: As you mentioned, we have reviewed the cost of sales due to a slight increase or decrease in the other undisclosed items.

Wakao [Q]: Is that not a very big factor?

Takechi [A]: No. Not that big. That is almost exclusively due to the impact of CAP.

Wakao [Q]: I understand. I understand that it is simply because CAP was removed. Thank you very much.

The second question is related to deramiocel. Since you are continuing the preparation, I think that your company assumes that the HOPE-3 trial has a very high likelihood of success. I would like to know the background behind your thinking. In case this trial is not successful, or the application for approval is resubmitted, or the approval review period is prolonged or delayed, are you currently considering any measures to deal with the situation? If you are considering it, I would like to know as much as you can tell me about it. I think this is a very important point for deramiocel to eventually grow beyond the cliff of Uptravi.

Beppu [A]: First of all, the reason why we believe that the HOPE-3 trial will have a high probability of success is that the HOPE-3 trial was originally initiated based on the data from the HOPE-2 trial, and the protocol design, including the design of the number of patients, was based on that data. The study was designed with a sufficient number of cases to produce a sufficiently significant difference.

Therefore, we believe that the results of the HOPE-2 trial will be reproduced there with a high probability.

Wakao [Q]: And what about the latter half of my question?

Takechi [A]: At the moment, we are going to resubmit the data with the HOPE-3 result. If we were to reapply, the procedure would be pushed back one year. Originally, we assumed that the CAP-1002 would be released in 2026 in the medium-term management plan, so we are thinking that the schedule will return to the original schedule.

Wakao [Q]: I understand. Then, unless HOPE-3 fails, it is safe to assume that there will be no major changes, especially from our point of view.

Takechi [A]: That is right. There is no impact on the medium-term management plan.

Wakao [M]: I understand. That is all. Thank you very much.

Shimokawa [M]: Thank you very much. Next, Mr. Muraoka from Morgan Stanley MUFG Securities, please ask your question.

Muraoka [Q]: Thank you very much. I am Muraoka from Morgan Stanley.

I'm having a little trouble organizing my thoughts about the HOPE-3 that is coming up. You just mentioned that you think the probability of success is high, but at which endpoints are you saying so?

I am not sure if you are referring to the sub-analysis of DMD cardiomyopathy with the HOPE-3, or if you are referring to the original HOPE-3 study to evaluate motor function, or if you are referring to all of them.

Also, I feel like I'm forced to solve a difficult equation because I'm not sure what to do with the HOPE-3 results, when you talk about indications, how to apply, reapplication, resubmission, and turning the clock around. It would be helpful if you could help me sort that out.

Beppu [A]: The HOPE-3 trial was originally designed with upper limb motor function as the primary endpoint. Therefore, the number of cases was designed based on the upper limb data as the primary endpoint from the HOPE-2 trial.

The HOPE-2 trial was finished halfway, but we have data on upper limb function and DMD cardiomyopathy or LVEF that Capricor is currently applying for. Data on LVEF show clearer efficacy than that on upper limb function.

So, although the design of the original HOPE-3 trial for the number of cases was based on upper limb data, we believe that with that number of cases, the more definitive data will be seen in the effect on DMD cardiomyopathy, for which clearer data are available in P2.

Now, as the CEO mentioned in the Capricor conference call, the primary endpoint of the HOPE-3 is, as I mentioned, upper limb motor function, but the effect on myocardium, which is placed as a secondary endpoint, will be changed to the primary endpoint. After the change, they are going to break the codes and get the results. Capricor has such a plan.

He said that he is thinking of having a Type-A meeting with the FDA to confirm this as well, and after obtaining the FDA's consent, the protocol will be changed, and then the code breaking will be made.

So, since the indication we are applying for now is DMD cardiomyopathy, we will change the primary endpoint of the HOPE-3 trial to LVEF and use that data to get the DMD cardiomyopathy indication. Capricor now has such a plan.

Muraoka [Q]: Thank you very much. I understand. The results of the HOPE-3 will also be analyzed and filed with cardiomyopathy in mind, won't they? It's kind of a perplexing story, but I got it.

Another question. As for RGX-121, PDUFA is scheduled for November 9. I think the vector here was AAV, and I would like to know if this point has had any impact on the current review or anything like that.

Beppu [A]: RGX-121 uses AAV9 as a vector. As for whether this has had any impact on the review process, REGENXBIO is currently communicating with the FDA, and we have not heard of any specific concerns about AAV vectors that have caused any delays in the review process.

Muraoka [M]: I understand. That is all. Thank you very much.

Shimokawa [M]: Thank you very much. Next, Mr. Wada from SMBC Nikko Securities, please ask your question.

Wada [Q]: I am Wada from SMBC Nikko Securities. I would also like to ask about CAP-1002. I would like to confirm the schedule.

I understand what you have said about Phase III disclosures up to September, but I am wondering when the Type-A meeting will be held, and if it goes smoothly and is approved without the need to reapply, what is the estimated time of approval?

On the other hand, if you need to reapply, I would like to know how long it would take to get approval.

Beppu [A]: So far, we have heard that Capricor has applied to the FDA for a Type-A meeting, but we have not heard that the meeting date has been set. As I mentioned earlier, I believe at a Type-A meeting, Capricor will confirm with the FDA on the spot how to proceed with the review in the future, including the changes in the HOPE-3 trial protocol as I mentioned earlier.

Until that meeting is completed, we will not be able to establish a concrete schedule for the rest of the year.

Wada [Q]: I understand. The timing of disclosure for Phase III will probably remain unchanged, so as a movement within the next three months, you may be going to Type-A after the HOPE-3 results are coming out by September, or if Type-A is held before that, you will change the protocol at Type-A and disclose the HOPE-3 results.

Beppu [A]: Yes. We think so.

Wada [Q]: Thank you very much. And I would like to ask about something else.

In the R&D expenses section, on page six, in the breakdown of increase or decrease in operating profit, you have described R&D expenses and a decrease of raw material costs related to new investigational drugs. In the end, the progress rate is now 15-16%, which is low compared to the full-year plan.

Is it correct to understand that there is a plan to introduce bulk pharmaceuticals and investigational drugs centered on nucleic acids in Q2 and Q3, and that this will cause a large increase.

Takechi [A]: Yes, while the timing of the costs is unclear.

Wada [M]: I understand. Thank you very much. That is all.

Shimokawa [M]: Thank you very much. Next, Mr. Yamakita from Jefferies, please ask your question.

Yamakita [Q]: I am Yamakita from Jefferies. Thank you very much. Two questions, please.

First is about CAP-1002. I would like to ask what the factors of delaying approval will be, especially if the data is good in Phase III. Especially as for the manufacturing matters, Capricor says they have been resolved, but the CRL states that there are outstanding items, or unresolved matters.

From a layman's point of view, I think the FDA will be discussing the equivalence of the cells in San Diego with those in Los Angeles based on the data that will come out of HOPE-3. Should I consider this as a risk here? Also, can you comment on any other risks of delay?

Beppu [A]: In Capricor's conference call, Capricor also mentioned that the CMC-related matters mentioned in the Complete Response Letter were the result of a pre-license inspection, or an inspection of the manufacturing facility. Capricor sent its answers to the FDA in response to the questions raised in the inspection.

However, the Complete Response Letter was issued around the same time as the response was sent, and the Complete Response Letter was issued at a time when the FDA could not confirm the report submitted by Capricor. Capricor had been aware of the issues in the Complete Response Letter in advance and had submitted its response and action.

Capricor had commented that it did not consider the CMC issue to be a particularly big problem. For our part, if the data from the HOPE-3 trial shows that the drug is effective, there should be no major obstacles to its approval.

Yamakita [Q]: I understand. Thank you very much.

Second, sorry, I think this is related to Mr. Wakao's question earlier. You told that a one-year delay in the launch of CAP-1002 would have little impact on the medium-term management plan.

If the speed of start-up is simply the same, I think that sales and profits would be delayed by one year if the launch is delayed by one year. Am I correct in understanding that you currently believe that you can still achieve the medium-term management plan in terms of both sales and operating profit?

Takechi [A]: That is right. I think I told you that the originally announced sales and profits would be brought forward at the end of the last fiscal period, and I think they will be restored to their original timing.

Yamakita [M]: I understand. Thank you very much. That's all from me.

Shimokawa [M]: Thank you very much. Now, Mr. Tanaka from Mizuho Securities, you have raised your hand for your second-round question.

Tanaka [Q]: I am Tanaka from Mizuho. Let me just check one more point. The Type-A meeting for CAP-1002 was requested in July when you received the Complete Response Letter, and a Type-A meeting will normally be held within a month since then. And the primary endpoint for HOPE-3 will also be changed and the CMC matters will also be confirmed, and then you will break the codes. Is that correct?

Beppu [A]: Yes. Our understanding is as Mr. Tanaka said, and we assume that Type-A will be implemented as soon as possible, followed by the disclosure of HOPE-3 data.

Tanaka [Q]: Okay. So it's not likely that Type-A will be very many months later.

Beppu [A]: No. We have assumed so.

Tanaka [Q]: I understand. Also, in the nucleic acid medicine area, Avidity's stock went up a lot yesterday. Your company's nucleic acid medicine business seems to be stagnant. I would like to know if the Company's policy is changing in any way.

Earlier, I think with a partnership with Boston Children's Hospital, you would like to do more nucleic acid medicine. Can you tell me about that?

Beppu [A]: Our policy on the development of nucleic acid drugs has not changed. We have heard that with regard to AOC1044, they will submit BLA at the end of this year according to their report. Their drug is administered less frequently than ours, and the information they have disclosed shows that the amount of dystrophin produced is comparable to that of Viltepso and other drugs.

We have also confirmed a few cases of treatment discontinuation due to adverse events in clinical trials. We believe that our exon-skipping drug can still compete with that drug in terms of the balance of efficacy and safety. We, too, want to hurry up the development of NS-089.

Tanaka [Q]: I understand. I am fine with the DMD area, but if you want to apply your nucleic acid medicine technology to more various diseases, I think a drug delivery system is important. Are you already thinking of using exosomes or some other new method? I think antibodies are already becoming difficult to use.

Beppu [A]: We have continued and will continue to search for DDSs that are compatible with nucleic acids, including those from other companies, either in-house or through open innovation. This includes the modality of exosomes. We will continue to work in this area.

Tanaka [M]: I understand. Thank you very much.

Shimokawa [M]: Thank you very much. Mr. Muraoka from Morgan Stanley MUFG Securities, please ask your second-round question.

Muraoka [Q]: I'm sorry. I forgot to put my hand down, but since I was called on, let me ask one question.

I would like to ask about the question someone asked earlier about the other parts of the sales that had ups and downs. This is something I've asked sometimes before. The growth in sales of Viltepso to non-approved countries has been going on for a long time. Am I correct in understanding that this is also a factor that has contributed to lowering the cost of sales here, since the April-June period was also good?

Takechi [A]: I can't tell you about what these items are, but that's why we reviewed that part as well.

Muraoka [M]: I understand. Thank you very much. That is all.

Shimokawa [M]: Thank you very much. Does anyone else have any questions? Now, Mr. Wakao from JPMorgan Securities, please ask your questions.

Wakao [Q]: I am sorry to ask many times. Let me confirm. After all, regarding deramiocel or CAP-1002, can I assume that the top-line data from HOPE-3, which will be available in Q3, will fully come out after the endpoint change at the Type-A meeting that you have just discussed?

Beppu [A]: Yes. That is what we are assuming from what Capricor said on their conference call.

Wakao [Q]: It looks a little tight as a schedule, but you are okay with it?

Beppu [A]: That is right. Since the administration of the HOPE-3 study has already been completed in June, we believe that they are preparing for the Type-A meeting while concurrently cleaning the data. Therefore, we believe that data disclosure after the Type-A meeting should be possible.

Wakao [Q]: Also, in terms of how that is disclosed, will the release of the results of the Type-A meeting be separated from the release of the top-line data, or will they be combined?

Beppu [A]: We do not know about that. We do not have such information.

Wakao [M]: I understand. Thank you very much. That is all.

Shimokawa [M]: Thank you very much. How about other questions? Since there appear to be no further questions, we will conclude the question-and-answer session.

This concludes the financial results briefing of Nippon Shinyaku for Q1 of FY2025.

Thank you very much for your participation today.

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Document Notes

1. Speaker speech is classified based on whether it [Q] asks a question to the Company, [A] provides an answer from the Company, or [M] neither asks nor answers a question.