Nippon Shinyaku Co., Ltd.

IR Meeting (Q4/FY2024)

May 8, 2025

Event Summary

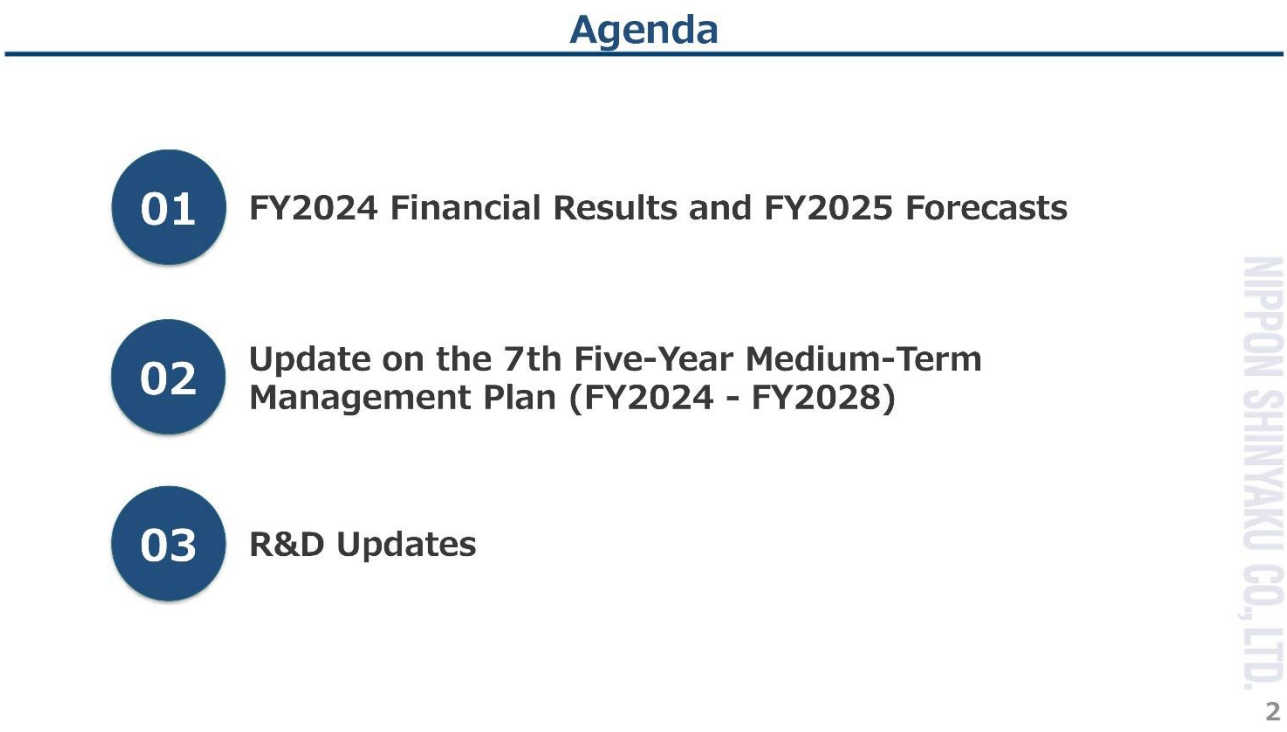
[Company Name]	Nippon Shinyaku Co., Ltd.
[Event Type]	Earnings Announcement
[Event Name]	IR Meeting (Q4/FY2024)
[Fiscal Period]	FY2024 Annual
[Date]	May 8, 2025
[Number of Speakers]	

Toru Nakai	Representative Director, President
Shozou Sano	Managing Director, Sales and Marketing
Takanori Edamitsu	Director, Business Management & Sustainability
Kazuchika Takagaki	Director, Research and Development
Hideyasu Takechi	Corporate Officer, Department Manager, Corporate Planning Dept.

Presentation

Nakai: I am Toru Nakai, Representative Director, President of Nippon Shinyaku Co., Ltd.

Thank you very much for taking time out of your busy schedule today to participate in our FY2024 financial results presentation. Thank you very much.



Today, I would like to share with you our results in FY2024 and our forecasts for FY2025, as well as updates on our Seventh Medium-Term Management Plan.

After that, Mr. Takagaki, in charge of research and development, will explain the progress of R&D items.

FY2024 Summary (consolidated)

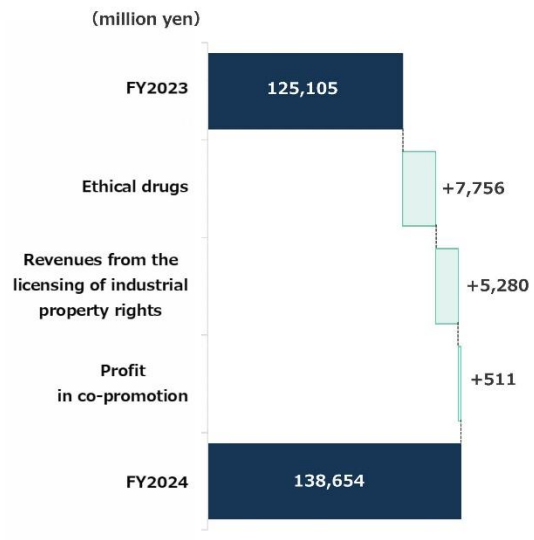
(million yen)	FY2023 actual	FY2023 ratio	FY2024 actual	FY2024 ratio	YoY change	YoY %
Revenue	**148,255**	**100.0%**	**160,232**	**100.0%**	**+11,976**	**+8.1%**
(Pharmaceuticals)	(125,105)	(84.4%)	(138,654)	(86.5%)	(+13,549)	(+10.8%)
(Functional Food)	(23,150)	(15.6%)	(21,577)	(13.5%)	(-1,572)	(-6.8%)
Cost of sales	**50,234**	**33.9%**	**51,116**	**31.9%**	**+882**	**+1.8%**
SG&A expenses	**34,959**	**23.6%**	**38,011**	**23.7%**	**+3,052**	**+8.7%**
R&D expenses	**31,676**	**21.4%**	**34,341**	**21.4%**	**+2,664**	**+8.4%**
Other income	**3,163**	**2.1%**	**874**	**0.5%**	**-2,288**	**-72.4%**
(Foreign exchange gain)	(2,486)	(1.7%)	-	-	(-2,486)	-
Other expenses	**1,252**	**0.7%**	**2,186**	**1.4%**	**+933**	**+74.5%**
(Foreign exchange loss)	-	-	(811)	(0.5%)	(+811)	-
Operating profit	**33,295**	**22.5%**	**35,450**	**22.1%**	**+2,154**	**+6.5%**
Finance income	**650**	**0.4%**	**830**	**0.5%**	**+180**	**+27.7%**
Finance costs	**329**	**0.2%**	**145**	**0.0%**	**-184**	**-55.9%**
Profit before tax	**33,616**	**22.7%**	**36,135**	**22.6%**	**+2,519**	**+7.5%**
Income tax expense, etc.	**7,765**	**5.2%**	**3,577**	**2.3%**	**-4,188**	**-53.9%**
Profit attributable to owners of parent	**25,851**	**17.4%**	**32,558**	**20.3%**	**+6,707**	**+25.9%**

I will now explain our FY2024 results and FY2025 forecast.

Please take a look at page four of the slides.

As a summary of our results in FY2024, consolidated revenue was JPY160,232 million, operating profit was JPY35,450 million, profit before tax was JPY36,135 million, and profit attributable to owners of the parent was JPY32,558 million.

Segmental Review - Pharmaceuticals -



Ethical drugs 83,898 million yen (+7,756 million yen, +10.2%, YoY)

- ✓ Negative impacts of the NHI drug price revisions and generic drugs
- ✓ Sales growth of Uptravi and Viltepso, etc.
- ✓ Contribution from new products including Vyxeos and Fintepla

Revenues from the industrial property rights 45,585 million yen (+5,280 million yen, +13.1%, YoY)

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

Profit in co-promotion 9,170 million yen (+511 million yen, +5.9%, YoY)

- ✓ Sales growth of Opsumit
- ✓ Launch of Yuvanci in November 2024

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In the pharmaceuticals segment, sales of Vidaza and other products declined due to NHI drug price revisions and the impact of generics, but sales of Uptravi, royalty income from overseas sales of the same product, and Viltepso grew.

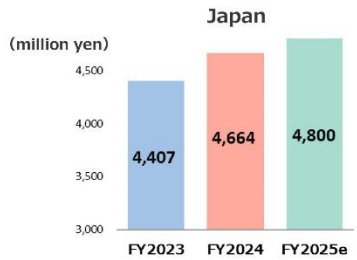
In addition, Vyxeos, which was launched in May 2024 contributed to sales growth, and consolidated revenue was JPY138,654 million, up 10.8% YoY.

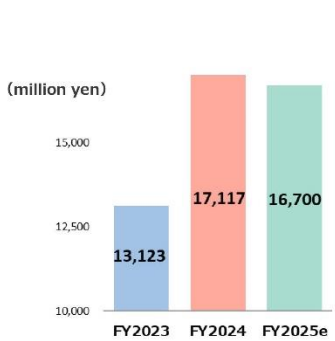
Sales Trends of Viltepso® (viltolarsen)

(million yen)	FY2023 actual	FY2024 actual	YoY change	YoY %	FY2025 forecast	Notes on FY2024 results
Japan	4,407	4,664	+257	+5.8%	4,800	✓ The number of patients currently being administered is more than three-quarters of the peak number of 128 patients in the data from Chuikyo[1]. ✓ No drop-out cases due to P3 study results
US	13,123	17,117	+3,994	+30.4%	16,700	✓ Number of new patients has been increasing after P3 study results. ✓ Insurance reauthorizations have become stricter due to launch of multiple DMD treatment options. The number of patients is expected to grow at a slower pace after FY2024.
(million US$)	(90.74)	(112.19)	(+ 21.44)	(+23.6%)	(119.28)	
Total	17,530	21,782	+4,251	+24.3%	21,500	

1. Central Social Insurance Medical Council

Exchange rates	FY2023 actual	FY2024 actual	FY2025 forecast
USDJPY	144.6	152.6	140.0





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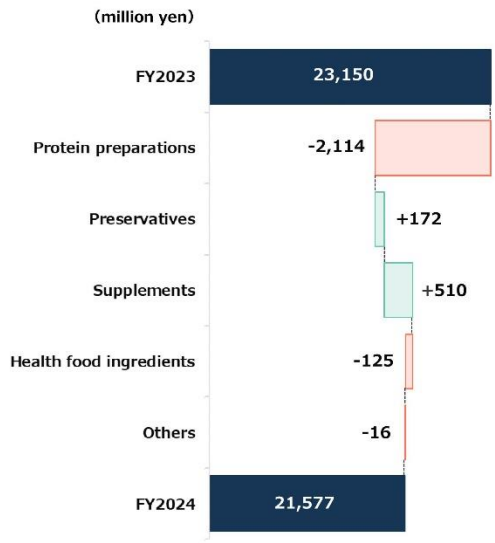
Here we show the sales of Viltepso, which is sold in Japan and the United States.

Sales results for FY2024 were JPY4,664 million in Japan and JPY17,117 million in the US, with YoY growth in both Japan and the US.

For FY2025, we expect sales of JPY4,800 million in Japan and JPY16,700 million in the US.

The projected sales in the US, converted into yen, are expected to decrease due to exchange rate effects, but are expected to increase on a US dollar basis.

Segmental Review - Functional Food -



Protein preparations 13,485 million yen
(-2,114 million yen, -13.6%, YoY)
- ✓ Sales prices decline of protein preparations for the processed food industry
- ✓ Decrease in demand for milk protein due to customers switching to cheaper raw materials

Preservatives 3,278 million yen
(+172 million yen, + 5.6%, YoY)
- ✓ Recovery of food service and tourism industries

Supplements 2,415 million yen
(+ 510 million yen, + 26.8%, YoY)
- ✓ New demand increased from young people and seniors, especially athletes
- ✓ Growth in the anti-aging care category helped by electronic commerce sales measures

Health food ingredients 1,122 million yen
(-125 million yen, - 10.1%, YoY)

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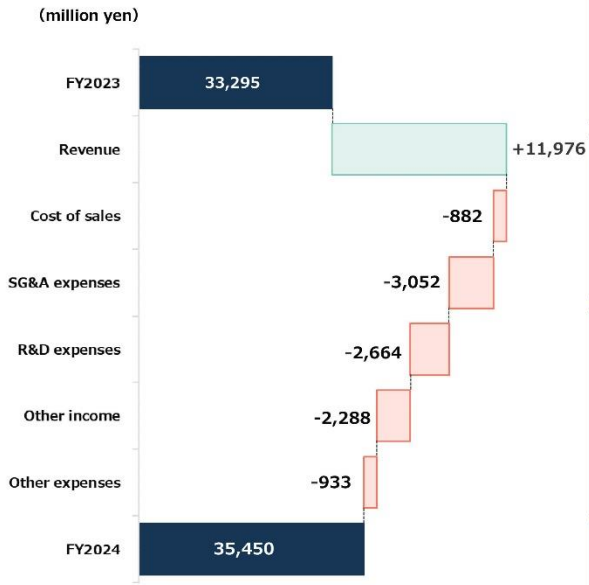
In the functional food segment, sales of supplements increased, but sales of protein preparations decreased, resulting in consolidated revenue of JPY21,577 million, down 6.8% YoY.

Operating Profit



Revenue 160,232 million yen
(+ 11,976 million yen, + 8.1%, YoY)
- ✓ Sales increase of Viltepso, Uptravi, and new product Vyxeos
- ✓ Increase in royalty income from Uptravi's overseas sales

Cost of sales 51,116 million yen
(+882 million yen, + 1.8%, YoY)
The ratio was 31.9%, improved by 2.0% points YoY.
- ✓ Cost of sales ratio improvement due to factors such as revenues from industrial property rights and the change in sales segment mix (pharma vs. food)

SG&A expenses 38,011 million yen
(+ 3,052 million yen, + 8.7%, YoY)
- ✓ Increase in the U.S. sales expenses for launch preparations
- ✓ Increase in domestic sales division expenses due to the launch of Vyxeos and Jaypirca
- ✓ Increase in commission for promotional activities of Uptravi due to domestic sales increase

R&D expenses 34,341 million yen
(+ 2,664 million yen, + 8.4%, YoY)
- ✓ Increase in contract research expenses and manufacturing costs for investigational products

Please turn to page eight.

Next, regarding operating expenses, the cost of sales ratio improved by 2 percentage points YoY to 31.9%, due to factors such as revenues from industrial property rights and the change in sales segment mix, despite the impact of the NHI drug price revisions.

SG&A expenses increased by 8.7% YoY to JPY38,011 million, mainly due to an increase in sales expenses at NS Pharma in preparation for the launch of new products.

R&D expenses totaled JPY34,341 million, up 8.4% YoY, mainly due to an increase in contract research expenses and manufacturing costs for investigational products.

As a result, operating profit was JPY35,450 million, up 6.5% YoY.

Business Forecast for FY2025 (consolidated)

(million yen)	FY2024 actual	FY2024 ratio	FY2025 forecast	FY2025 ratio	YoY change	YoY %
Revenue	160,232	100.0%	173,000	100.0%	+12,768	+8.0%
(Pharmaceuticals)	(138,654)	(86.5%)	(150,000)	(86.7%)	(+11,346)	(+8.2%)
(Functional Food)	(21,577)	(13.5%)	(23,000)	(13.3%)	(+1,423)	(+6.6%)
Cost of sales	51,116	31.9%	55,200	31.9%	+4,084	+8.0%
SG&A expenses	38,011	23.7%	47,000	27.2%	+8,989	+23.6%
R&D expenses	34,341	21.4%	39,500	22.8%	+5,159	+15.0%
Other income	874	0.5%	600	0.3%	-274	-31.4%
Other expenses	2,186	1.4%	1,900	1.1%	-286	-13.1%
Operating profit	35,450	22.1%	30,000	17.3%	-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	17.7%	-5,535	-15.3%
Income tax expense, etc.	3,577	2.3%	6,600	3.8%	+3,023	+84.5%
Profit attributable to owners of parent	32,558	20.3%	24,000	13.9%	-8,558	-26.3%

The sensitivity of the exchange rate is assumed to be an increase of approximately 530 million yen in revenue and approximately 450 million yen in operating profit for every 1 yen depreciation of the yen.

Foreign exchange rates (USDJPY)

FY2024 actual	FY2025 forecast
152.6	140.0

Reasons for increased expenses in FY2025

SG&A expenses
- ✓ Launch preparations of CAP-1002 (deramiocel) and RGX-121 in the U.S.
- ✓ European expansion preparation
- ✓ Commission for promotional activities of Uptravi due to domestic sales increase

R&D expenses
- ✓ Increase in contract research expenses and manufacturing costs associated with research and development of nucleic acid products

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Please turn to page nine.

I will now explain our forecast for FY2025.

Consolidated revenue is expected to be JPY173,000 million.

Regarding operating expenses, the cost-of-sales ratio is expected to be 31.9%, about the same level as the previous fiscal year.

SG&A expenses are expected to be JPY47,000 million, including an increase in sales expenses at NS Pharma in preparation for the launch of new products. Meanwhile, R&D expenses are expected to be JPY39,500 million, including an increase in contract research expenses and manufacturing costs associated with research and development of nucleic acid products.

As a result, we project operating profit of JPY30,000 million, profit before tax of JPY30,600 million, and profit attributable to owners of parent of JPY24,000 million, a decrease YoY.

Revenue Forecast – Pharmaceuticals Segment -

(million yen)	FY2024		FY2025		YoY	
	actual	ratio	estimate	ratio	change	%
Ethical drugs	83,898	60.5%	92,900	61.9%	+9,002	+10.7%
Revenue from the licensing of industrial property rights	45,585	32.9%	47,500	31.7%	+1,915	+4.2%
Profit in co-promotion	9,170	6.6%	9,600	6.4%	+430	+4.7%
Revenue	138,654	100.0%	150,000	100.0%	+11,346	+8.2%

Despite the impact of NHI drug price revisions and generic competition for Vidaza, sales increase is expected due to the following factors;

1. New product launch in the U.S. in FY2025 H2: CAP-1002 (deramiocel) and RGX-121
2. Contribution of new products in Japan: Vyxeos , Fintepla, Uptravi (Pediatric PAH), etc.
3. Growth in royalty income: overseas sales of Uptravi

Next, page 10. In the pharmaceuticals segment, we project revenue of 150,000 million, up 8.2% YoY.

Despite the impact of NHI drug price revisions and generic competition, we expect sales of CAP-1002 (generic name: deramiocel) to be launched in the US in H2, will grow. Also, a new domestic product Vyxeos, and Fintepla—for which an additional indication has been approved—will perform well.

Revenue Forecast - Functional Food Segment-

(million yen)	FY2024		FY2025		YoY	
	actual	ratio	forecast	ratio	change	%
Protein preparations	13,485	62.5%	13,900	60.4%	+415	+3.1%
Preservatives	3,278	15.2%	3,400	14.8%	+122	+3.7%
Supplements	2,415	11.2%	3,500	15.2%	+1,085	+44.9%
Health food ingredients	1,122	5.2%	1,100	4.8%	-22	-2.0%
Others	1,276	5.9%	1,100	4.8%	-176	-13.8%
Revenue	21,577	100.0%	23,000	100.0%	+1,423	+6.6%

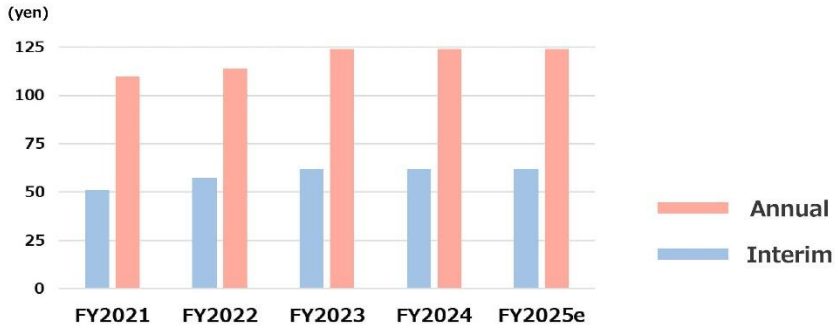
Sales increase is expected through development and launch of new products and strengthen sales efforts in key products.

Please turn to page 11.

In the functional food segment, we expect revenue of JPY23,000 million, an increase of 6.6% YoY, by further focusing on the development and launch of new products and strengthening our efforts in key products.

Dividends Forecast

		FY2024	FY2025e
Dividends per share	Interim	¥62	¥62
	Annual	¥124	¥124
Basic earnings per share		¥483.40	¥356.20
Payout ratio (consolidated)		25.6%	34.8%
Dividend on equity ratio		3.6%	-



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Next, page 12. The Company plans to pay a year-end dividend of JPY62 per share for FY2024, which—together with the interim dividend of JPY62 per share—will result in an annual dividend of JPY124 per share.

For the next fiscal year, we plan to pay an interim dividend of JPY62 per share and a year-end dividend of JPY62 per share, for a total annual dividend of JPY124 per share.

This concludes an explanation of our results in FY2024 and our forecasts for FY2025.

Overview of the 7th Medium-Term Management Plan

From May 27, 2024
The 7th Five-Year Medium-Term Management Plan (FY2024 - FY2028) -For Global Growth Beyond the Cliff-, p.19

During the 7th Medium-Term Management Plan, we will promote "three key themes and strengthening five management foundations" to realize Vision for 2035. In each of the Pharmaceuticals and Functional Food segments, we will thoroughly allocate management resources and reduce costs by prioritizing them based on business strategies, and manage the capital efficiency of each segments by ROIC* to secure earnings that exceed the cost of capital.

*ROIC (%) = Operating profit after tax / Invested capital (Non-current assets + Net working capital)

Targets in FY2028	Revenue 230 billion yen EPS 341 yen		ROE 8% or more	Operating profit 30 billion yen ROIC 9% or more	
Three Key Themes	I Fostering growth drivers to replace Uptravi		II Expanding global development	III Continuous pipeline expansion	
Strengthening five management foundations	① Promoting sustainable management for realizing sustainable society	② Speeding up R&D	③ Promoting human capital management that allows each employee to grow and diverse human resources to play an active role	④ Business process reengineering and productivity improvement by promoting digitalization	⑤ Financial strategy for sustainable growth

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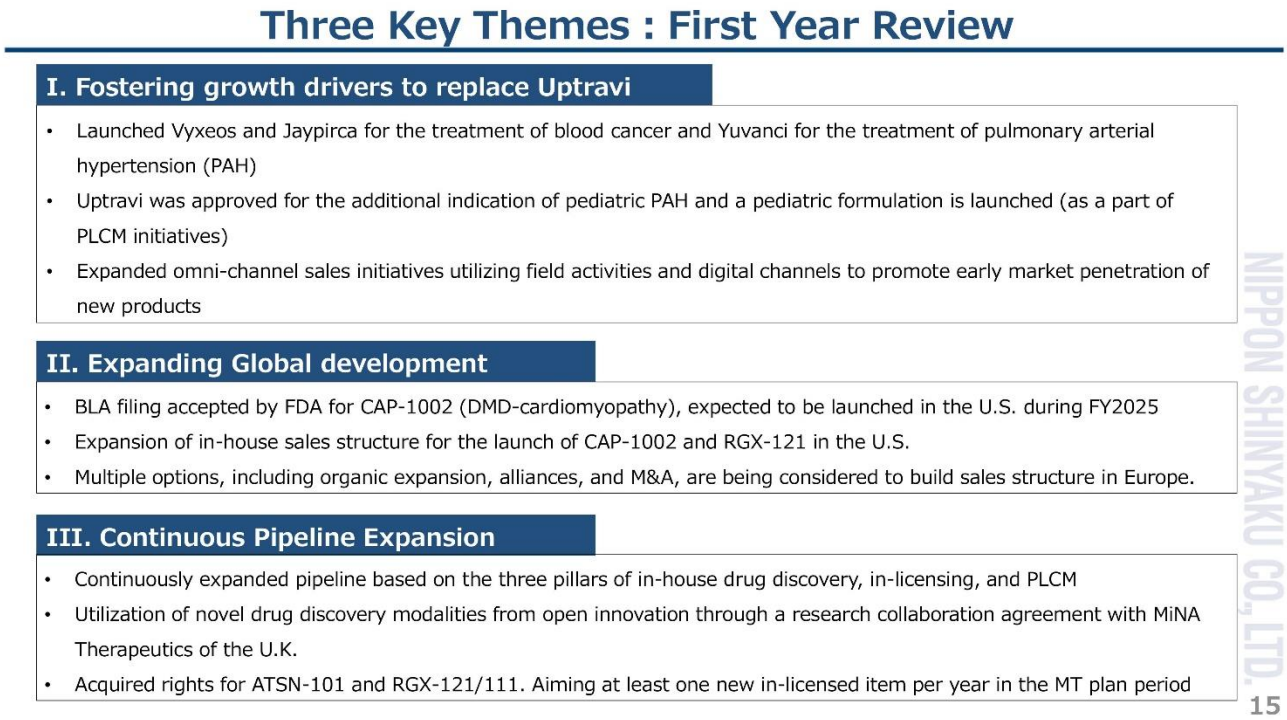
I will now continue with an update of the Seventh Medium-Term Management Plan. Please see page 14.

Under the Seventh Medium-Term Management Plan “For Global Growth Beyond the Cliff,” which started in FY2024, we are working on the three key themes and strengthening five management foundations to support them in order to overcome the patent cliff of Uptravi and achieve sustainable growth.

In FY2024, the Company launched four new products, including Vyxeos, and entered into licensing and other agreements for four new products.

We also actively worked on global development, including strengthening our sales structure in the US and studying the possibility of developing our business in Europe.

Going forward, we will continue to pursue these initiatives and move forward with the plan fully prepared to achieve the quantitative targets of JPY230,000 million in revenue and JPY30,000 million in operating profit for FY2028, the final year of the Seventh Medium-Term Management Plan.



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This slide shows a review of activities in FY2024 for the three key themes of the Seventh Medium-Term Management Plan.

Under the first theme, “Fostering growth drivers to replace Uptravi,” we launched Vyxeos and Jaypirca, drugs for blood cancer, and Yuvanci, a drug for pulmonary arterial hypertension.

In the PLCM, Uptravi received a pediatric indication and a pediatric formulation is now available.

We are expanding omni-channel sales initiatives utilizing field activities and digital channels to promote early market penetration of these new products.

With respect to the second theme, “Expanding global development,” Capricor Therapeutics, our partner, has filed a BLA for deramiocel for the indication of DMD cardiomyopathy and we plan to launch the drug in FY2025. We are expanding our sales structure in the US, for the new products including RGX-121.

We are also conducting a detailed study of all possible means to establish a sales structure in Europe, including in-house sales, alliances, and M&A.

In the third and final theme, we are continuously expanding our pipeline based on the three pillars of in-house drug discovery, in-licensing, and PLCM.

In in-house drug discovery, we believe that the utilization of novel drug discovery modalities, especially through open innovation, will be important, and we have entered into a research collaboration agreement with MiNA Therapeutics of the UK.

As for in-licensing, license agreements for ATSN-101 and RGX-121/111 were concluded.

We aim to acquire at least one new in-licensed product per year in the future.

Our Target for New Product Launch

We have been aiming to launch at least 2 new products each fiscal year

Modified from May 27, 2024
The 7th Five-Year Medium-Term Management Plan (FY2024 - FY2028) -For Global Growth Beyond the Cliff-, p.26

1. Generic name updated to brand name, 2. Schedule moved forward, 3. Schedule delayed, 4. Newly added

	Period of the 7th Five-Year Medium-Term Management Plan					Period of Next MT Plan	
	FY2024a	FY2025	FY2026	FY2027	FY2028	FY2029	FY2030
Domestic	NS-87 (VYXEOS) : high-risk AML	NS-401 (tagraxofusp) : BPDCN[2]	GA101 (Gazyva) : pediatric nephrosis	ZX008 (Fintepla) : CDKL5 gene deficiency[3]	NS-089/NCNP-02 (brogidirsen) : DMD[3]		NS-050/NCNP-03 : DMD[3]
	LY3527727 (Jaypirca) : MCL[1]			GA101 (Gazyva) : lupus nephritis[3]			NS-304 (selexipag) : ASO[4]
	NS-304 (Uptravi) : pediatric PAH			GA101 (Gazyva) : SLE without nephropathy			
Overseas		CAP-1002 (deramiocel) (U.S.) : DMD cardiomyopathy[2]		NS-089/NCNP-02 (brogidirsen) (U.S.) : DMD		NS-050/NCNP-03 (U.S.) : DMD[3]	
		RGX-121 (clemidsogene lanparvovec) (U.S.) : MPS II[4]					

Aiming to launch by the end this MT period
ATSN-101 (U.S.) : LCA1[4], RGX-111 (U.S.) : MPS I[4]

Note:NS-051/NCNP-04 (Japan and U.S.) and NS-065/NCNP-01 (Europe and China) are active programs but are currently under ongoing discussions with regulatory authorities. The year of market launch for these products has not yet been determined.

AML:acute myeloid leukemia; MCL:mantle cell lymphoma; pediatric PAH:pediatric pulmonary arterial hypertension; BPDCN:blastic plasmacytoid dendritic cell tumor; SEL:systemic lupus erythematosus; ASO:arteriosclerosis obliterans; MPS:mucopolysaccharidosis; LCA1:GUCY2D-associated Leber congenital amaurosis

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Please move on to page 16.

This page shows the schedule of new product launches. During the period of this Medium-Term Management Plan, we aim to launch an average of at least two new products per year. Specifically, we plan to launch one product in Japan and two products in the US in FY2025.

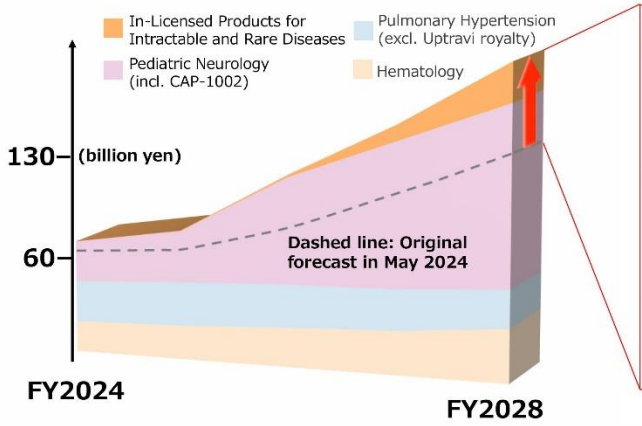
Of these, we plan to be able to launch the NS-401 and deramiocel one year ahead of the assumption made when the Seventh Medium-Term Management Plan was formulated. We also plan to launch RGX-121, for which we concluded an exclusive license agreement in January this year, in the US in FY2025 H2.

Although there are some items, such as NS-050, for which development is expected to be delayed, we aim to complete clinical trials as soon as possible, including in countries other than Japan and the US.

Update of Sales Revenue Forecast in the 7th MT Plan Period

Revenue in the global market, which is a focus area for FY2028, is expected to exceed the forecast of 130 billion yen announced in May 2024 in the 7th MT Plan and continue to grow.

Conceptual image of sales revenue growth in the 7th MT Plan period



Main factors contributing to updated revenue forecast

- **Increased revenue from new in-licensed products in the intractable and rare disease field (ATSN-101 and RGX-121/111)**
- **The Expected launch date for CAP-1002, a cell therapy drug, has been brought forward, and an expansion of patient population due to expected indication for DMD-cardiomyopathy.**
- **Sales growth of Fintepla, rare intractable epilepsy treatment[1]**
- **Market expansion of pulmonary hypertension treatments such as Uptravi and Yuvanci**
- **Sales growth of blood cancer treatments such as Vyxeos, Jaypirca, and NS-401**

1. Sales figures for Fintepla has been disclosed from FY2024.

Modified from May 27, 2024
The 7th Five-Year Medium-Term Management Plan (FY2024 - FY2028) -For Global Growth Beyond the Cliff-, p.28

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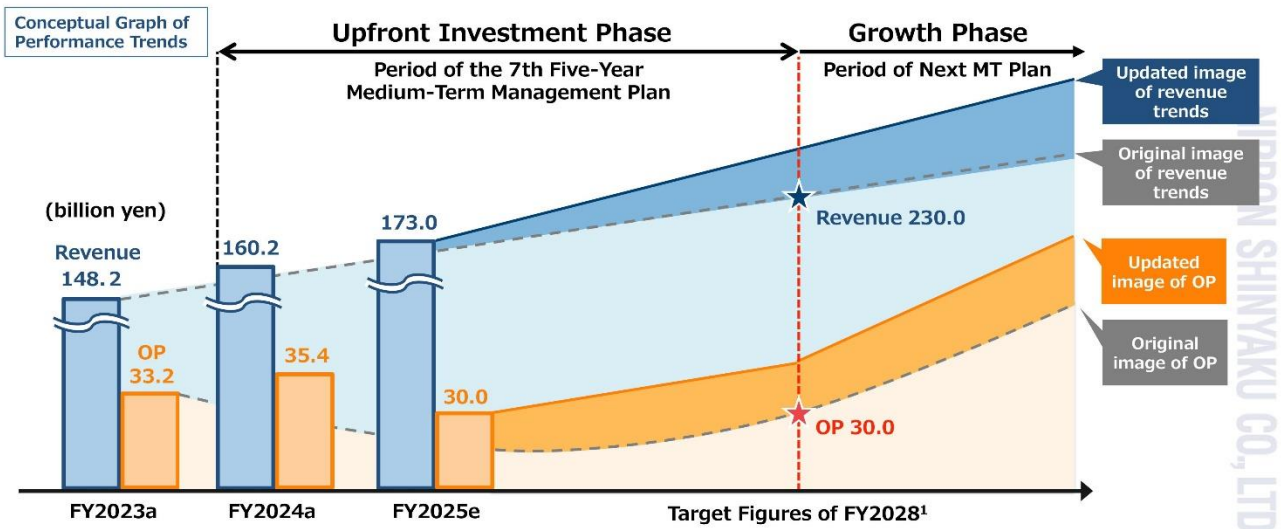
Next, page 17. This slide shows the projected sales revenue for the period of this Medium-Term Management Plan.

In FY2028, the final year of the Medium-Term Management Plan, we expect sales revenue in the global market in our focus areas will exceed our initial plan of JPY130 billion or more. The main reasons for the increase in revenue were the advancement of the launch of deramiocel from the original plan, and newly in-licensed products in the areas of intractable and rare diseases.

Establishing Growth Foundation to Overcome Patent Cliff

Operating profit is expected to increase from FY2025 onwards due to the earlier launch of CAP-1002 and the faster growth of new products.



1. May 28, 2024 The 7th Five-Year Medium-Term Management Plan

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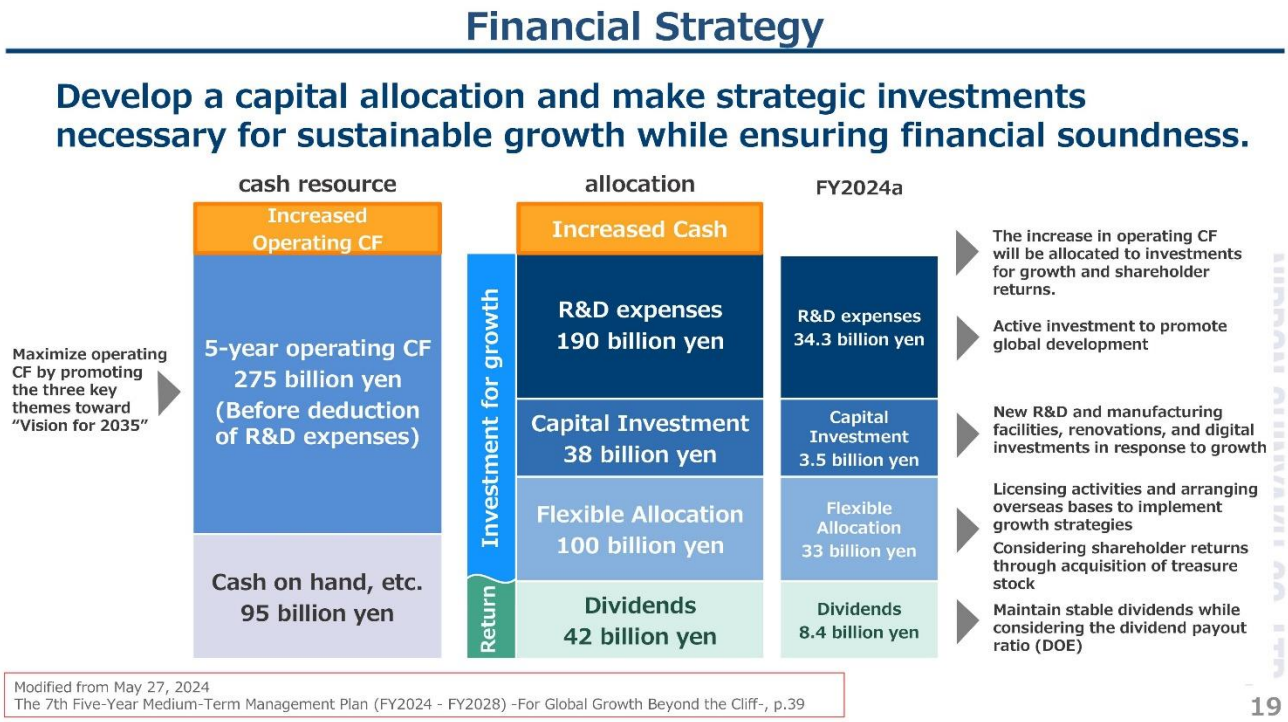
Please see page 18. We have positioned this Medium-Term Management Plan period as a phase to make up-front investments to overcome the patent cliff of Uptravi.

Although we expect a YoY decline in profits for the current fiscal year, from the next fiscal year onward, we expect a quicker recovery due to a smaller decline in operating profit compared to the initial plan. This is because there is an upward trend in projected revenue in the global market, as I have just explained.

This has increased the chance of achieving the quantitative targets of the Seventh Medium-Term Management Plan. We also view the next medium-term management plan period from FY2029 onward as a growth phase and aim to become a company with revenue of JPY300 billion and operating profit of JPY50 billion by FY2030.



Please turn to page 19.

This slide shows our financial strategy.

The basic policy of capital allocation is to actively make the investments necessary for sustainable growth while ensuring financial soundness.

When the Medium-Term Management Plan was first formulated, cash resources were assumed to be JPY370 billion, consisting of operating cash flow before deducting R&D expenses for five years and cash on hand, etc. However, we expect this amount to increase due to the accelerated launch of developed products and the rapid penetration after that.

The increased cash will be allocated to growth investments aimed at mid- to long-term growth and returns.

This will further ensure the profit growth during the next medium-term management plan period.

This concludes the update of the Seventh Medium-Term Management Plan.

CAP-1002 (deramiocel) update

Capricor Therapeutics Announces Completion of Mid-Cycle Review Meeting with FDA on Deramiocel for the Treatment of Duchenne Muscular Dystrophy Cardiomyopathy

-Company remains on track for PDUFA target action date of August 31, 2025-

-Advisory committee meeting to be held in advance of target action date-

SAN DIEGO, May 05, 2025 (GLOBE NEWSWIRE) -- Capricor Therapeutics (NASDAQ: CAPR), a biotechnology company developing transformative cell and exosome-based therapeutics for the treatment of rare diseases, today announced the completion of a mid-cycle review meeting with the U.S. Food and Drug Administration (FDA) for the Company's Biologics License Application (BLA) seeking full approval for deramiocel, an investigational cell therapy, as a treatment for patients diagnosed with Duchenne muscular dystrophy (DMD) cardiomyopathy. During the meeting, FDA stated that no significant deficiencies have been identified by the Review Committee and that the package is on track for a Prescription Drug User Fee Act (PDUFA) action date of August 31, 2025. The FDA has also confirmed its intent to hold an advisory committee meeting, although an official date has not yet been set.

"The successful completion of our mid-cycle review meeting along with the upcoming advisory committee meeting represents major milestones on the path towards approval of deramiocel," said Linda Marbán, Ph.D., Chief Executive Officer of Capricor. "Deramiocel is a first-in-class cellular therapy with the potential to halt or slow the progression of DMD-cardiomyopathy, and we are pleased to have the opportunity to present the efficacy and safety data to the advisory committee. We have been actively preparing for an advisory committee meeting, and we look forward to providing the physician and patient perspectives to highlight the weight of evidence supporting the transformative potential of deramiocel in treating DMD-cardiomyopathy."

The BLA submission is supported by Capricor's cardiac data from its Phase 2 HOPE-2 and HOPE-2 Open Label Extension (OLE) trials compared to patient level data from an FDA-funded and published dataset on the natural history of DMD-cardiomyopathy and potential biomarkers of disease progression. Efficacy from the ongoing HOPE-3 study is not part of this BLA package submission.

- ✓ **The Mid-Cycle Review Meeting[1] with the FDA has been completed.**
- ✓ **PDUFA action date remains August 31, 2025.**
- ✓ **The FDA intends to hold an advisory committee meeting[2].**

1. One of the FDA's review meetings for new drug approval
2. Advisory Committees are open to the public and are held when the FDA reviews a pharmaceutical product. The applicant and the FDA each give a presentation on the risk/benefit of the product under review, and the advisory committee, which is made up of experts in various fields, deliberates, taking into account public opinion, and then votes on whether to recommend the product or not.

Source: May 5, 2025, press release from Capricor Therapeutics
Capricor Therapeutics Announces Completion of Mid-Cycle Review Meeting with FDA on Deramiocel for the Treatment of Duchenne Muscular Dystrophy Cardiomyopathy :: Capricor Therapeutics, Inc. (CAPR)

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Please take a look at page 20.

This slide shows the press release issued by Capricor Therapeutics on May 5.

The FDA mid-cycle review meeting for the full approval of deramiocel has been completed, and at this time no significant deficiencies have been identified in the package. It was confirmed that it is on track to meet the PDUFA date of August 31.

The FDA intends to hold an advisory committee meeting and the date will be determined. We will continue to prepare for the launch of deramiocel in the US.

This concludes my explanation.

Next, Mr. Takagaki, in charge of research and development, will give an update on the progress of R&D.

Takagaki: I am Kazuchika Takagaki, in charge of research and development.

I will continue with an explanation of the progress of R&D items that have been updated since the financial results for Q3 of FY2024.

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

For updates since Q3 FY2024 financial results announcement on February 7, 2025, see highlighted text in red.

Recent status/event	Code No. (Generic name)	Product 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-87 (daunorubicin / cytarabine)	Vyxeos	high-risk acute myeloid leukemia	May 2024
Launch	LY3527727 (piltobrutinib)	Jaypirca	patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors	August 2024
Launch	ACT-064992D (macitentan / tadalafil)	Yuvanci	pulmonary arterial hypertension	November 2024
Additional indication	NS-304 (selexipag)	Uptravi	pediatric pulmonary arterial hypertension	December 2024
Launch	NS-304 (selexipag)	Uptravi	Uptravi® Tablets for Pediatric 0.05 mg	March 2025
Filed (BLA[1] accepted by FDA)	CAP-1002 (deramiocel)	–	Duchenne muscular dystrophy cardiomyopathy	March 2025 (U.S.)
Filed (BLA[1] submitted and waiting for FDA acceptance)	RGX-121 (clemidsogene lanparvovec)	–	Mucopolysaccharidosis Type II	March 2025 (U.S.)
Filed	NS-401 (tagraxofusp)		blastic plasmacytoid dendritic cell neoplasm (BPDCN)	March 2025
Start of P2	NS-229	–	eosinophilic granulomatosis with polyangiitis	June 2024
Start of P1/ P2	NS-050/NCNP-03	–	Duchenne muscular dystrophy	October 2024

1. Biologics License Application

Please take a look at page 22.

The protocol for Study 303 for Viltepso was submitted to the FDA at the end of February, and we are currently inquiring with the FDA regarding the results of the review and future schedule.

In March, a 0.05 mg pediatric formulation of Uptravi was launched.

There were only a limited number of drugs available in Japan for pediatric pulmonary arterial hypertension, and especially as for prostacyclines, only injectable drugs, which require continuous intravenous administration, were available. Therefore, oral drugs with the same mechanism of action were sought.

The drug was developed with the aim of being able to be taken by younger pediatric patients, and the tablet diameter is less than half that of existing Uptravi tablets, improving convenience.

The BLA for deramiocel was accepted in the US in March.

With the priority review designation, the PDUFA date has been set for August 31, 2025, US time. We are currently preparing for the product to be launched by the end of this fiscal year.

Regarding RGX-121, our partner, REGENXBIO Inc., filed a BLA in the US in March.

In March 2021, we in-licensed NS-401, or tagraxofusp, from The Menarini Group in Italy and have been developing the product for the expected indication of blastic plasmacytoid dendritic cell neoplasm. In March, we filed an application for manufacturing and marketing approval in Japan for the expected indication.

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

For updates since Q3 FY2024 financial results announcement on February 7, 2025, see highlighted text in red.

Recent status/event	Code No. (Generic name)	Product name	Indications and topics	Schedule
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)
In-license agreement signed (Atsena Therapeutics)	ATSN-101	–	GUCY2D-associated Leber congenital amaurosis	November 2024 (U.S. and Japan)
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	
Option Agreement signed for Commercialization (AB2 BIO Ltd.)	Tadekinig alfa	–	NLRC4 mutation and XIAP deficiency	January 2025 (U.S.)
Preliminary analysis results	NS-065/NCNP-01 (viltolarsen)	Viltepso	global Phase 3 trial (RACER53 Study)	May 2024
Publication			the results of Phase 2 trial (Galactic53 trial) in Scientific Reports	October 2024
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
Rare Pediatric Disease Designation	NS-051/NCNP-04	–	Duchenne muscular dystrophy	January 2025 (U.S.)
Orphan Drug Designation	NS-229	–	eosinophilic granulomatosis with polyangiitis (EGPA)	April 2025 (U.S.)

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Please turn to page 23.

In April, we received orphan drug designation in the US for NS-229, a selective JAK1 inhibitor discovered and developed in-house for the treatment of eosinophilic granulomatosis with polyangiitis.

This concludes my explanation on the progress of research and development.

Question & Answer

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

We will take questions first from analysts and institutional investors, and then take questions from the press.

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

Now, we will take your questions. Mr. Yamaguchi from Citigroup Global Markets, please ask your questions.

Yamaguchi [Q]: I am Yamaguchi from Citi. Thank you very much. The first question is about the insurance that is mentioned in Viltepso's forecast for the current fiscal year, where [inaudible] many other things are described.

There has been a report of death in gene therapy and there was not much growth QoQ. You mentioned in FY2024 Q3 that you thought that Viltepso would not grow much, due to insurance circumstances. I would like to know how much of this impact is likely to occur and whether it will have a structural impact all the way through or not.

Can you tell me what you mean by this comment in the Viltepso forecast?

Nakai [A]: Thank you for your question. Looking back at the status of Viltepso in the US in FY2024, the number of patients receiving the drug increased during the year, but the number of patients who unfortunately did not qualify for reimbursement due to worsening of their condition, and who dropped out of the treatment because their insurance would not cover the drug, also increased a bit. This means that the growth in the number of patients administered the drug slowed down in FY2024.

I think it is possible to explain that the impact of the insurance and the stricter reauthorization for renewal of insurance coverage has an influence along with the deterioration of the patients' condition, rather than the situation or impact of the gene therapy.

As for the forecast for FY2025, even if we are actually in that situation, the number of patients who want to start treatment with Viltepso have continued to increase, and we expect it to grow in US dollar terms by ensuring new patients receive treatment.

Yamaguchi [Q]: So, in the gene therapy, around Q3 or so, you said that it had an impact. Has anything changed since then?

Nakai [A]: We have confirmed that a few cases have switched to gene therapy, but we have not seen a dramatic increase in the number of such cases. We can confirm that the number of patients who are switching to gene therapy is not increasing much.

Yamaguchi [Q]: Was the switch temporary, or has the number of switches gradually increased? I'm afraid this is a bit detailed.

Nakai [A]: The recent situation is that we have not received such reports of a switch to gene therapy.

Yamaguchi [Q]: So has the switching trend stopped? There may be a temporary move away from gene therapy now.

Nakai [A]: I guess so.

Yamaguchi [Q]: Okay, thank you. Secondly, in your forecast for this fiscal year, there is a considerable increase in SG&A and R&D expenses due to the launch of new drugs, including deramiocel and another drug.

I am wondering if there are any payments for Capricor in the case of deramiocel, and if so, how much of it is actually related to deramiocel and how much to RGX in the contract.

Nakai [A]: Thank you very much. Milestone payments upon approval of deramiocel and RGX-121 will not specifically influence the P&L, but will go into the B/S lump-sum payment, so to speak, and what will go into the SG&A in the P&L are the launch preparation costs for two new products in the US. The increase in SG&A expenses is JPY8,989 million, of which nearly JPY5,000 million is planned to be spent on preparation for the launches in the US.

Yamaguchi [Q]: Does the R&D depends on projects, or rather on the number of projects, although this is increasing quite a bit?

Nakai [A]: We are planning to use for the progress of clinical development of our nucleic acid products in particular, and also to conduct another P3 study of Viltepso.

Yamaguchi [Q]: Lastly, I think that regarding whether deramiocel will be approved or not, if things go normally, I think it will be approved, but I think you are well aware of the news reports of various FDA staff being replaced and their stance changing in various ways. So, there are many people who know that situation in the market and are worried if it's really okay.

You have been talking with the FDA via Capricor, and is there nothing unusual about what is going on? I think I should wait for their review and approval, since the PDUFA date is still August 31, but the environment around us is a bit turbulent. So, please tell me your thought including the meaning behind an advisory committee meeting will be held.

Nakai [A]: Thank you very much. Neither our company nor Capricor believes that the change in leadership at the FDA had any impact on the decision to hold the advisory committee meeting.

The new cell therapy modality would be used for the first time to treat DMD, and Capricor stated that it was necessary to prepare for an advisory committee meeting even before the holding of the meeting was decided. Therefore, we do not think that the recent organizational change at the FDA has had any particular impact.

Yamaguchi [M]: Thank you.

Takechi [M]: Does anyone have any other questions? Mr. Sakai from UBS Securities, please.

Sakai [Q]: I am Sakai from UBS. I think JPY7.3 billion revenue for deramiocel is included in the FY2025 forecast this time, but I wonder how this is accounted for. Is this revenue after paying Capricor or is the gross recorded?

I think you mentioned that about half of sales would be paid to Capricor, and is this treated as SG&A expenses? I would like to know the details in that area.

Also, I am not sure where this JPY7.3 billion is coming from. I believe you mentioned before that there are already about 100 patients on the waiting list, and I think this figure is calculated based on a certain drug price. If we count backwards, we can roughly come up with the price. Is that the way you calculate?

Nakai [A]: I will answer the first question. The JPY7.3 billion for deramiocel is based on NS Pharma's sales. In our partnership scheme with Capricor, NS Pharma purchases products from Capricor, and NS Pharma sells them to medical institutions and wholesalers.

In terms of economic conditions, Mr. Sakai mentioned earlier about half of the net sales, and that is based on the fact that NS Pharma pays Capricor for the products it purchases. Therefore, we would like you to understand that JPY7.3 billion is our product sales.

Sakai [Q]: This is the external sales itself that your company records, isn't it?

Nakai [A]: Yes.

Sakai [Q]: Are you saying that the portion paid to Capricor is recorded under promotional expenses?

Nakai [A]: No, it is cost.

Sakai [Q]: Cost?

Nakai [A]: Yes.

Sakai [Q]: Should I understand that the cost of the product includes, let's say, about 50% of sales you mentioned?

Nakai [A]: Yes, that'll be the cost of sales.

Sakai [Q]: I understand. How about calculation of waiting patients?

Nakai [A]: Regarding the waiting patients, the JPY7.3 billion sales figure assumes that we have 100 patients on the waiting list, who are clinical trial participants, and we will obtain about 100 new patients.

Since the launch is scheduled for the end of this year, and the PDUFA date will be at the end of August, we will start preparing everything from there, and the actual sales will start at the end of the year.

Also, deramiocel is administered once every three months. Therefore, it is administered four times a year. Considering this, I would like you to understand this year's forecast only considers the last quarter.

Sakai [Q]: I understand. Does that mean the last quarter of your company, or Q4 from January to March?

Nakai [A]: That is correct.

Sakai [Q]: Thank you very much. Then I have just one more question.

As for Study 301 and Study 303 for Viltepso, are you still discussing this with the FDA? Mr. Takagaki skipped over the detail, so could you give me some more updates?

Takagaki [A]: As an update, the detailed final results of Study 301 were submitted to the FDA at the end of last year, and the detailed protocol for the additional Study 303 was submitted at the end of February. This was in response to a request from the FDA to see a detailed protocol, and it was submitted at the end of February.

The normal review period is said to be about 60 days, so we are hoping that it is about time. However, we have not yet received any response from the FDA, so we are currently making inquiries. There has been no response yet.

Sakai [Q]: I understand. Is it your understanding that the response from the FDA would then be that you can start the trial with that Study 303 protocol, or that you need to revise a little more?

Takagaki [A]: I think we will have a meeting to discuss the review of Study 301, plus answers for Study 303, including whether to change the protocol or keep it the same.

Sakai [Q]: When it comes to the review of Study 301, does that mean that the data will be questioned again there?

Takagaki [A]: We submitted summary data before last October, and the detailed data was submitted at the end of last year. This should be a review of that.

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

Takechi [M]: Now, Mr. Tanaka from Mizuho Securities, joining us online, please ask your questions.

Tanaka [Q]: I am Tanaka from Mizuho Securities. As a follow-up on the question that was just asked to Mr. Takagaki, basically, is this accelerated approval for Viltepso maintained, and is there no problem with that? Am I correct in assuming that it is okay if you do an additional study? This is my first question.

Takagaki [A]: We are now in the process of consulting with the FDA about maintaining the accelerated approval and conducting an additional P3 study.

The situation has not changed because we have not yet received a response from the FDA, but when we sent out the summary data for Study 301 and the outline protocol for Study 303, as the additional validation study, the FDA still wanted to see the detailed results of Study 301 and the detailed protocol for Study 303.

We are hoping that the FDA will be positive about conducting the next validation study, and we are hopeful that FDA will maintain the approval, since the FDA is positive about conducting the validation.

Tanaka [Q]: I understand.

My second question is about NS-050 on page 16. This is another exon-skipping drug. I understand that the launch date is still undecided because the additional P3 for Viltepso has not yet been decided. The expected launch year for NS-050 is delayed into FY2029, right? Wasn't this originally in FY2028? What is the reason for this?

Takagaki [A]: Yes, we are a year behind. We had originally planned to conduct the clinical trials in Japan and the US, but the enrollment of patients has not been progressing well. So, we plan to expand the patient recruitment to other countries. So it is taking a little extra time.

Tanaka [Q]: I understand. So, thank you Mr. Nakai for updating the Medium-Term Management Plan. On page 18, the orange area is an image of the current operating profit trend, and it looks as if the patent cliff of Uptravi has disappeared.

If this current estimate of revenue continues in the pace of exceeding this JPY230 billion, does this mean that your company internally believes that the patent cliff of Uptravi in FY2027 will be overcome?

Nakai [A]: Yes, that's right. To answer your question simply, if the products that we are currently planning to in-license and launch perform as we expect, we should be able to fully cover the loss of profits, especially caused by the patent cliff of Uptravi.

Tanaka [Q]: Is it correct to say that the decrease in royalties of Uptravi can be covered, even if the cost ratio of CAP-1002 in particular is high, about 30% to 50%, I think?

Nakai [A]: Yes. Even with such a cost ratio, we do not expect to increase our SG&A expenses more than the additional JPY5 billion we have assumed this fiscal year in SG&A expenses.

Therefore, I think it will be good if you imagine that we can ensure profits by handling and growing items that do not require much SG&A expenses.

Tanaka [M]: I understand. That's all from me.

Takechi [M]: Mr. Wakao from JPMorgan Securities, please ask your questions.

Wakao [Q]: I am Wakao from JPMorgan Securities. The first question is about Viltepso. I don't mean to be overly negative about it, but I think the premise of the view changes if the accelerated approval is revoked. So, let me confirm although I may be persistent.

The director of CBER is now Vinay Prasad, and I think he is someone who has a rather cautious view of surrogate markers. Then in this DMD, there are nucleic acid drugs that have received accelerated approval based on dystrophin, and I think Viltepso is one of them.

And since the original P3 study did not go well, I wonder if, in light of this, we have to view it as more of a risk than before, or if we have to look at it more cautiously, given the current situation. Please tell me how you feel.

Even though Vinay Prasad becomes the director, there is also a timing issue. I would like to know your thoughts on whether or not it will have much influence and whether or not you will be able to properly maintain accelerated approval.

Nakai [A]: Before I ask Mr. Takagaki to update again, please note that CBER at the FDA is not the authority which reviews Viltepso and nucleic acid drugs, but CDER does. Since nucleic acid drugs are now being reviewed by CDER, we do not believe that there will be any direct impact from the fact the head of CBER has been replaced.

The current review team for nucleic acid drugs also approves them based on the scientific judgment such as scientific verification, published theories, and opinions regarding the correlation or relationship between surrogate markers and expected improvement or maintenance of function. Therefore, I think we do not expect any change in this area.

Mr. Takagaki, do you have any?

Takagaki [A]: I have nothing to add.

Wakao [Q]: Thank you very much. I understand very well.

Second, I would like to know more about the market penetration of CPA-1002. I think the market penetration will be very fast, since you are talking about 100 waiting patients and another 100 patients for this fiscal year. I would like to know more about the background behind this assumption.

I understand the first 100 clinical trial patients, but what is the assumption for another 100 new patients? Furthermore, is it safe to assume that these 100 new patients will penetrate the market at a very rapid pace next fiscal year and beyond, looking at FY2026 and beyond? On the other hand, should we assume that after the 100 patients enter the program, then the growth will be somewhat slower? What can you tell me about that?

Nakai [A]: Thank you very much. As for the 100 new patients we expect to receive the drug, we expect the rapid penetration right after launch of the drug because there are no drugs indicated for DMD cardiomyopathy, and the drug is much awaited.

Especially with Capricor and also NS Pharma's activities in the US, the situation is that DMD patients have been deteriorating or even dying due to the inevitable deterioration of cardiac function even after exon-skipping drugs are administered. We have confirmed in the field that health care providers and patient advocacy groups have high expectations for deramiocel to save such patients, and we expect that it will spread at a reasonable pace.

As for how far the use of the drug will spread after that, we would like to make efforts to spread the use of the drug to as many patients as possible, while having them feel the clinical effects of the drug. It is safe to assume that we have high expectations. That is all.

Wakao [Q]: During the R&D briefing the other day, there was a discussion regarding the number of eligible patients, and it looked as if the patients had not yet been confirmed. However, with your current explanation, you already have some idea of potential patients who are eligible.

Nakai [A]: Yes, that's right. We have obtained data on the number of patients with a left ventricular ejection fraction below a certain percentage, and we have a good idea of how many patients there are.

Wakao [Q]: Do you have more specifics like how many are in which hospitals?

Nakai [A]: Yes. Since the drug is to be administered at medical institutions, we are also working to identify patients who are already in clinical trials at each institution and target those institutions for treatment sites.

We are now making preparations to ensure that untreated patients will be connected to those target institutions, so that they can receive treatment with deramiocel.

Wakao [Q]: I understand. Lastly on page 18, if you consider the market penetration of this CAP-1002, I think the top line will grow well.

And if the SG&A expenses mentioned earlier are also constant and peak at this fiscal year's level, then profits in FY2026 will be above this FY2028 level, decrease a bit in FY2027 with the cliff of Uptravi, and be at the level you have updated us in FY2028. So I have expected an up and down being formed once. Is that understanding correct?

Nakai [A]: We do not expect so. CAP-1002 (deramiocel), RGX, etc. will offset the decline in profits, and it will be a gradual steady rise.

This situation may change depending how quickly such new products penetrate the market after launch, but we believe that if they grow steadily, profits will bottom out in FY2025 and then rise steadily.

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

Takechi [M]: Mr. Muraoka from Morgan Stanley MUFG Securities, please ask your questions.

Muraoka [Q]: Thank you very much. I am Muraoka from Morgan Stanley. I would also like to ask about CAP-1002. I am also hopeful that the product will sell if it is successfully approved and launched, but the recent CBER personnel announcement caused Capricor's stock price to drop by 30% in two days, and the market is still very anxious.

My question is Capricor's market capitalization has gone down to USD300 million and I really think that if your company is so confident in the success of deramiocel, why don't you acquire Capricor? I would like you to comment on that as the first question.

As the second question, if the approval of deramiocel is delayed and the FDA requests additional clinical data, what would be your plan B?

Nakai [A]: Thank you very much. As to the first question, we would prefer not to comment on specific other companies.

We are always discussing internally and with our partners what the best form of partnerships should be. Currently, we are on a straight license deal with Capricor, but we will continue to review the situation internally as it develops and proceed accordingly.

Second, regarding the plan B, as you can see from the financial strategy on page 19, in the capital allocation section, we have already spent JPY33 billion out of the JPY100 billion planned for flexible allocation.

We think that the plan B is to use the remaining cash to acquire different growth drivers in case that the approval of deramiocel gets pushed back or it is not approved.

In the flexible allocation for the implementation of the growth strategy, we have included the payments of sales milestones after the approval. Therefore, if the results are unfavorable, there is no longer any need to use such cash, and we think that the way to proceed is to look for other opportunities using the cash.

Muraoka [Q]: Thank you very much. The other question is about the data book at the back of the summary of financial results (Kessan Tanshin), which shows this year's depreciation expenses will increase by about JPY2 billion from JPY6 billion to JPY7.9 billion YoY. What causes this increase in depreciation?

Is it related to Capricor or REGENXBIO? Or is there something else that you are planning to add? What do you think?

Edamitsu [A]: Depreciation is not from Capricor or contract payments, but from tangible assets. Last year, we built the nucleic acid API purification plant at the Odawara Central Factory, and depreciation expenses for it are expected to increase.

All amortization of contract payments are included in the cost. That is all.

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

Takechi [M]: Mr. Yamakita from Jefferies, please ask your questions.

Yamakita [Q]: I am Yamakita from Jefferies. I have a few questions.

First, regarding the R&D expenses, is my understanding correct that the large increase is included based on the assumption that Study 303 will be started? The point is, if the discussion with the FDA is a bit protracted and the start of Study 303 is delayed, is it quite likely that this will not increase? Can you tell me about it?

Edamitsu [A]: As for R&D expenses for Study 303, we have submitted the protocol to the FDA, but It's not something we can start immediately. We are expecting a certain amount, but it is not very large. That is all.

Yamakita [Q]: Thank you very much. Also are price increases from contractors included here? Or is this only related to pipeline progress?

Edamitsu [A]: There are not big price increases, although there are slight ones.

Yamakita [Q]: Okay, thank you. Also, as for deramiocel, should I consider this as a best case scenario in terms of guidance figures you are giving us? Are you assuming that the label will be clean and the supply chain will be well done?

Nakai [A]: Yes. If deramiocel is approved with the indication for DMD cardiomyopathy, and if it is not accompanied by any age restrictions or ambulation status, and if it is approved for an indication for which it can be used within the percentage of left ventricular ejection fraction that we expect, we can achieve this target amount.

Yamakita [Q]: Thank you very much. I remember that during the previous R&D Meeting, while Capricor reportedly said it could cover about 70% of DMD patients, your company said that the number might be a little less. Is the figure based on a discussion with Capricor?

Nakai [A]: Capricor said 70% of all DMD patients, but we think it is about half of all DMD patients. The target figure has been shared with them.

Yamakita [Q]: Thank you very much. Finally, let me check quickly. You mentioned that you have seen a few cases of switching from gene therapy to Viltepso. Have you seen any cases where Viltepso is administered after gene therapy?

Nakai [A]: Thank you for your question. In fact, we presented data from patients receiving our Viltepso at the annual meeting of the Muscular Dystrophy Association (MDA) in March of this year.

The breakdown of the patients who had used gene therapy prior to receiving Viltepso is about 4% of the total number of patients. Therefore, we have announced that there are, in fact, patients who are receiving treatment with Viltepso after gene therapy.

Since this is data among all patients from the time of product launch until about November 2024, I think this is new information that we can report this time. We have confirmed that there are indeed patients who are on Viltepso after receiving gene therapy last year.

Also, the breakdown shows that, as a supplement, 64% of the patients were untreated, 30% switched from other exon-skipping drugs, 4% received gene therapy and 1% unknown.

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

Takechi [M]: Thank you. Other analysts and institutional investors, does anyone have any questions? Mr. Sakai from UBS Securities, please ask your questions.

Sakai [Q]: This may be a coulda, woulda, shoulda question, but listening to this CAP-1002 discussion, is it possible to remove DMD from this? In other words, considering cardiomyopathy, mode of action, the indication for general cardiomyopathy or heart failure, which is related to the left ventricle, as mentioned earlier, may be quite possible. Is such a thing being considered with Capricor? I wonder if your company has the right to do so.

If that is the case, I think the argument could be that you should buy Capricor.

Nakai [A]: Thank you very much. I will leave the scientific part to Mr. Takagaki, but first, Capricor has announced that in addition to this DMD cardiomyopathy, they are now looking into the possibility of expanding the indication to patients with Becker muscular dystrophy (BMD).

We are not sure if we can expand the indication to cardiomyopathy in general, since the underlying factors that cause cardiomyopathy vary depending on the disease.

Under the agreement with Capricor, our company only has rights for the indication of DMD, and even if the indication is expanded to other cardiomyopathies such as BMD, our company will have no rights.

Takagaki [A]: I think, in addition to BMD, the indication may be for DMD carriers, which is X-chromosome dependent. There are quite a few mothers with DMD or such cardiomyopathy, so I would imagine that such patients would be our potential targets in the future.

Takechi [M]: Thank you very much. We would now like to begin taking questions from the press. When asking a question, please state the name of your company and your name before you speak.

Shimomura [Q]: I am Shimomura from Jiho. I'm sorry to ask a detailed question, but in your financial strategy, you mentioned a capital investment of JPY3.5 billion, and I think you mentioned some digital investment. What is the content?

Edamitsu [A]: Regarding digital investments, as stated in the Seventh Medium-Term Management Plan, we plan to promote investments for promoting business transformation and productivity improvement.

I don't have the specific amount with me at the moment, but do you have, secretariat?

Shimomura [Q]: Specifically, for example, I would like to know whether digitalization will be done in the area of R&D investment, or in the area of sales structure, or in other areas, or whether it will be related to business improvement.

Edamitsu [A]: We will invest in areas related to speeding up R&D, and we will also invest in areas related to the entire company.

Shimomura [Q]: [inaudible].

Edamitsu [A]: I mean capital investment for IT.

Shimomura [M]: Thank you very much.

Takechi [M]: Does anyone have any other questions? Mr. Mizuno from Tokio Marine Asset Management, joining online, please.

Mizuno [Q]: Thank you. I would like to go over the part about deramiocel. The application has been filed ahead the schedule originally planned by Capricor and your company, and I think you mentioned that there was a proposal from the FDA.

I was just thinking about what you said at that time, and I am wondering if I am correct in interpreting that this is not a surrogate marker, but rather the result of an evaluation of clinical efficacy..

Nakai [A]: Yes, that's right. They are looking at deterioration of cardiac function, not biomarkers. They will compare it with the natural history, which Vanderbilt University in the US has, and they will use that natural history data to compare the deterioration between deramiocel and natural history. If the difference can be read as indicating that the drug is effective enough, then they will approve the drug based on that data.

Capricor has explained that they are not seeking for an accelerated approval with surrogate markers, but rather full approval, and that is how we understand it.

Mizuno [Q]: I understand. The number of enrolled patients was never small. There were a number of patients there.

Takagaki [A]: I don't know the exact enrollment number right now, but I understand that the FDA has asked for not only clinical trial data, but also natural history data, so the number of subjects will be increased to build comparative data.

Mizuno [Q]: Thank you. As someone asked earlier, the new CBER director who was appointed a few days ago has been skeptical of surrogate markers so I was thinking that there are no concerns about deramiocel in that point.

On the other hand, what about REGENXBIO? I am wondering if this is a bit of a risk. Since this is an in-licensed product and your company are not an applicant, it may be difficult to comment, but what do you think of it as a risk?

Takagaki [A]: The primary endpoint is surrogate, but they are also looking at the degree of intellectual disability at the same time in the clinical trial, and improvements in that area have been observed. We do not think that approvability will be questioned immediately.

Mizuno [M]: I understand very well. Thank you, that's all.

Takechi [M]: We would like to add one point to our earlier response. Mr. Edamitsu, please explain.

Edamitsu [A]: I would like to reiterate a point that I misunderstood when you asked me about the increase factors in depreciation and amortization earlier.

As I explained earlier, depreciation expenses for nucleic acid API purification plant at the Odawara Central Factory will increase. In addition, as I mentioned in my answer, the amortization of contract payment is also reflected in the cost, but I think I gave the wrong answer when I said that this is also included in depreciation and amortization. That is all.

Takechi [M]: Does anyone have any other questions? How about online participants? Please ask your questions.

Kameda [Q]: I am Kameda from QUICK. Thank you very much. I would like to ask you about Vyxeos. I think it is doing better than initially expected at the time when the drug price was first listed. How do you think about that? Also, please tell me about the forecast for the next year.

Sano [A]: Thank you. The initial peak revenue of Vyxeos was expected to be JPY2 billion, because it is estimated that there are 11,000 AML patients in Japan, of which roughly half, or about 5,000, are high-risk AML, and of those, half of them, or 2,300, are chemotherapy fit patients.

In the overseas P3 and domestic P1/2 studies, Vyxeos significantly improved OS compared to conventional chemotherapy for high-risk AML patients aged 60 to 75. In Japan, the number of the patients aged 60 to 75 is about 300, so we estimated that the peak revenue would be JPY2 billion.

The evaluation of Vyxeos by physicians was very high, even before the product was launched. In practice, this drug has been prescribed to elderly patients over 75 years old and patients under 60 years old.

In addition to new-onset patients we had originally assumed, with such extremely high expectations from physicians, there were many more patients on the waiting list who were relapsed and refractory and required urgent treatments. Many major hospitals and university hospitals adopted the drug on an accelerated basis, for limited patients immediately after launch.

Therefore, I believe that we have contributed a lot here as Vyxeos has really progressed faster than initially expected and new prescriptions expanded rapidly, enabling patients to start treatment with Vyxeos faster than expected. The market penetration was achieved much earlier than originally planned.

From the beginning, our company has a drug called Vidaza, and we have a very strong relationship of trust with myeloid doctors in that aspect. We also believe that our ability to identify the target patients at an early stage is also a major factor.

We intend to further build on our experience with new-onset patients and establish its positioning as the first-line drug for chemotherapy-fit, high-risk AML.

Kameda [M]: Thank you very much.

Takechi [M]: The next question will be the last. Mr. Hashimoto from Nikkei BP, who joins us online, please ask your questions.

Hashimoto [Q]: I am Hashimoto from Nikkei Biotech. I would like to ask about the policies of the Trump administration. I know that there are still many unclear areas, such as tariffs on pharmaceuticals, but as your company is just about to make expansions in the US business and take various steps, please tell me what you are thinking about, including whether you will wait and see or delay your plans a little in relation to the upcoming policies of the Trump administration.

Nakai [A]: Thank you very much. We are not thinking that we will wait and see, and we will speedily carry out what we need to do.

Also, in terms of tariffs, the product subject to tariffs this fiscal year is Viltepso. Viltepso is exported to the US. This has a stable inventory, or rather, items that are already in the US will be sold this fiscal year, so I think the impact on business performance will not be that great.

The situation is still in flux in terms of tariffs and the status of the FDA's review, as I mentioned earlier, so we will keep a close eye on these trends. That is all.

Hashimoto [M]: Thank you very much.

Takechi [M]: That concludes the presentation of the financial results for FY2024 of Nippon Shinyaku.

Thank you for joining us today.

[END]

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