Edamitsu: I am Takanori Edamitsu, Director of Business Management and Sustainability at Nippon Shinyaku Co., Ltd.

Thank you very much for taking time out of your busy schedules to participate in our financial results briefing today. I appreciate it very much.

I will now explain our business results for Q3 FY2024 and the progress of our R&D activities, in accordance with the presentation materials posted on our website.

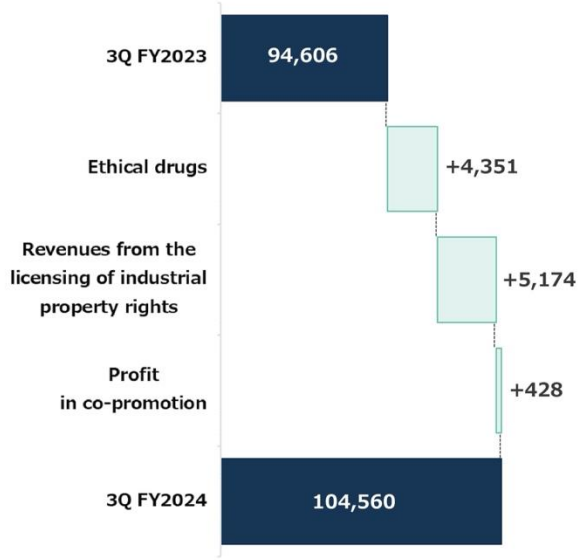
3Q FY2024 Summary (April-December)

(Million yen)	3Q FY2023		3Q FY2024		YoY Change	
	Results	Ratio	Results	Ratio	Amt	%
Revenue	**112,728**	**100.0%**	**121,320**	**100.0%**	**+8,592**	**+7.6%**
(Pharmaceuticals)	(94,606)	(83.9%)	(104,560)	(86.2%)	(+9,954)	(+10.5%)
(Functional Food)	(18,121)	(16.1%)	(16,759)	(13.8%)	(-1,361)	(-7.5%)
Cost of sales	**38,613**	**34.3%**	**38,810**	**32.0%**	**+196**	**+0.5%**
SG&A expenses	**25,741**	**22.8%**	**27,562**	**22.7%**	**+1,821**	**+7.1%**
R&D expenses	**19,500**	**17.3%**	**23,547**	**19.4%**	**+4,047**	**+20.8%**
Other income	**1,887**	**1.7%**	**1,725**	**1.4%**	**-161**	**-8.6%**
(Foreign exchange gain)	(1,361)	(1.2%)	(1,058)	(0.9%)	(-302)	(-22.3%)
Other expenses	**309**	**0.3%**	**371**	**0.3%**	**+61**	**+19.9%**
Operating profit	**30,450**	**27.0%**	**32,752**	**27.0%**	**+2,302**	**+7.6%**
Finance income	**611**	**0.5%**	**774**	**0.6%**	**+162**	**+26.6%**
Finance costs	**89**	**0.1%**	**89**	**0.0%**	**+0**	**+0.1%**
Profit before tax	**30,973**	**27.5%**	**33,438**	**27.6%**	**+2,464**	**+8.0%**
Income tax expense, etc	**6,970**	**6.2%**	**4,885**	**4.0%**	**-2,085**	**-29.9%**
Profit attributable to owners of parent	**24,002**	**21.3%**	**28,552**	**23.5%**	**+4,549**	**+19.0%**

Please see page two of the slide.

As an overview of our performance in Q3 FY2024, we reported consolidated revenue of JPY121,320 million, operating profit of JPY32,752 million, profit before tax of JPY33,438 million, and net profit attributable to owners of the parent of JPY28,552 million.

Segmental Review - Pharmaceuticals -



Ethical drugs 63,357 million yen (+4,351 million yen, +7.4%, YoY)

- ✓ Negative impacts of the NHI drug price revisions and generic drugs
- ✓ Sales growth of Uptravi and Viltepso, etc.
- ✓ New products including Vyxeos, which was launched in Japan in May 2024
- ✓ Foreign exchange contribution (depreciation of yen) to Viltepso's US sales in yen

Revenues from the industrial property rights 34,071 million yen (+5,174 million yen, +17.9%, YoY)

- ✓ Growth in royalty income from Uptravi's overseas sales and depreciation of yen

Profit in co-promotion 7,132 million yen (+428 million yen, +6.4%, YoY)

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

Please see page three of the slide.

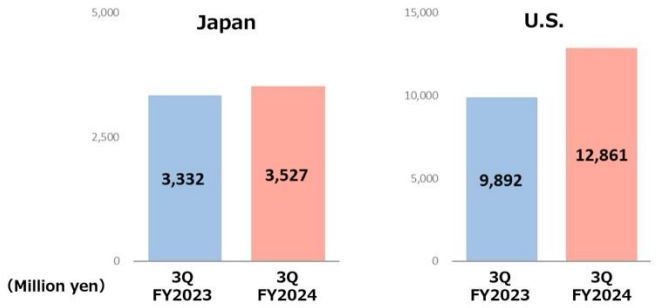
In the pharmaceuticals business, despite the effects of NHI price revisions and generic drugs, sales of Uptravi and Viltepso and royalty income from overseas sales of Uptravi increased.

In addition, Vyxeos, which was launched in last May, contributed to consolidated net sales of JPY104,560 million, up 10.5% from the same period last year.

Sales Trends of Viltepso® (viltolarsen)

(Million yen)		3Q FY2023 Results	3Q FY2024 Results	YoY Change Amt	YoY Change %	
Viltepso	Japan	3,332	3,527	+195	+5.9%	✓The number of patients currently being administered is more than two-thirds of the peak number of 128 patients in the data from Chuikyo (Central Social Insurance Medical Council) . ✓Currently working to increase sales by identifying and intervening early with patients who are eligible for the 53-skip treatment at a younger age.
	U.S.	9,892	12,861	+2,969	+30.0%	✓The number of patients receiving and wishing to receive Viltepso has increased. ✓In October 2024, the data from the Phase II (Study 211) was published in the journal Scientific Reports. The improvements in respiratory function and maintenance of motor function in both ambulatory and non-ambulatory patients were observed.
	total	13,225	16,389	+3,164	+23.9%	

Exchange rate	3Q FY2023 Actual rate	3Q FY2024 Actual rate
1US$	¥143.3	¥152.6



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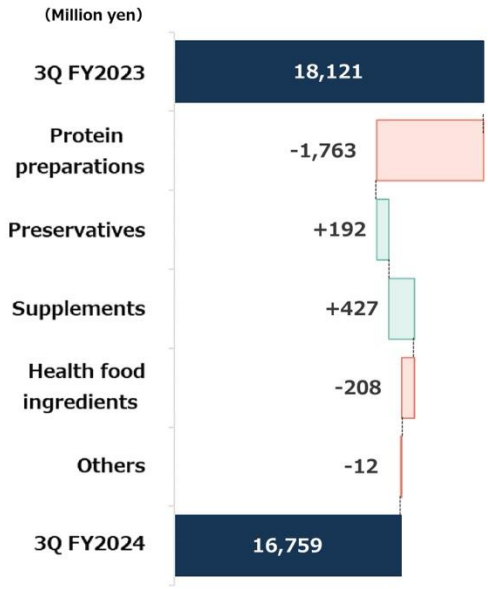
Here we show the sales trends of Viltepso in Japan and the U.S..

Sales in both Japan and the U.S. increased YoY, totaling JPY3,527 million in Japan and JPY12,861 million in the U.S..

Segmental Review - Functional Food -



Protein preparations 10,556 million yen
(-1,763 million yen, -14.3%, 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

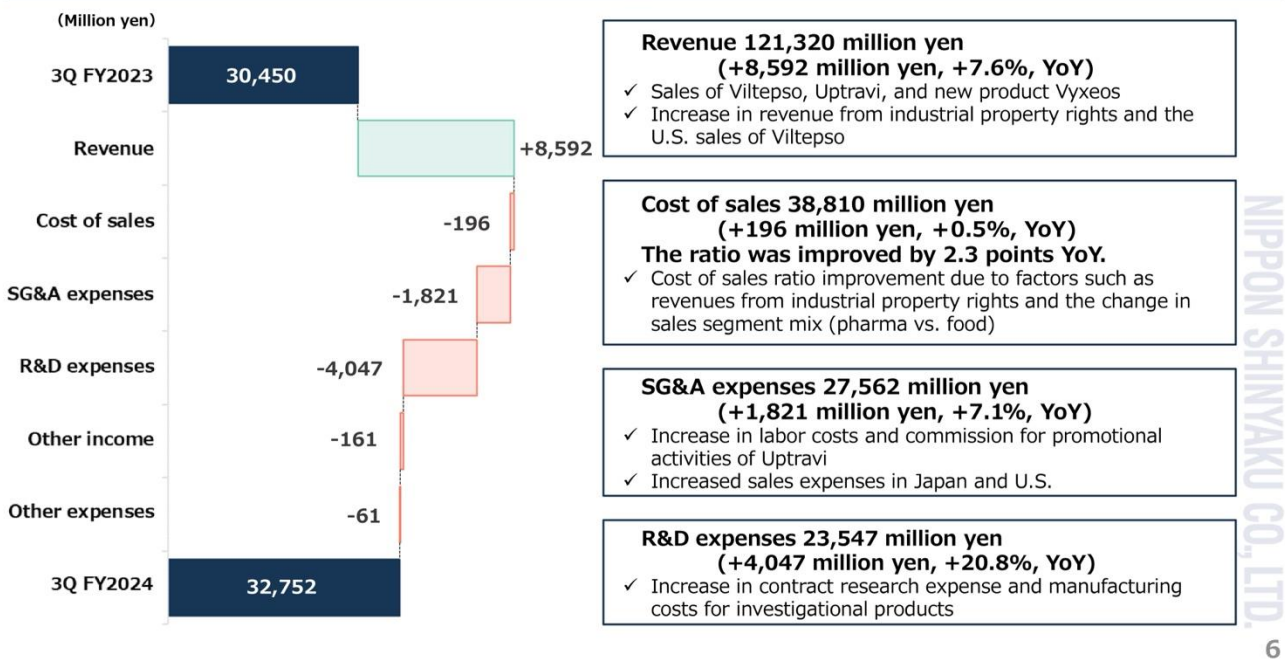
Preservatives 2,576 million yen
(+192 million yen, +8.1%, YoY)
- ✓ Recovery in food service-related business due to active tourism and excursions

Supplements 1,893 million yen
(+427 million yen, +29.2%, 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 748 million yen
(-208 million yen, -21.8%, YoY)

Please see page five of the slide.

In the functional foods business, sales of supplements and other products increased, but sales of protein preparations and other products decreased, resulting in consolidated net sales of JPY16,759 million, down 7.5% YoY.



Please see page six of the slide.

The cost-to-sales ratio improved by 2.3 percentage points YoY to 32%, due to factors such as revenues from industrial property rights and the change in sales segment mix.

SG&A expenses increased to JPY27,562 million, up 7.1% YoY, due to an increase in labor costs, commission for sales promotion activities of Uptravi, and sales expenses of the U.S. subsidiary, NS Pharma.

R&D expenses totaled JPY23,547 million, up 20.8% YoY, mainly due to an increase in contract research expenses.

As a result, operating profit was JPY32,752million, up 7.6% YoY.

Revised Business Forecast for FY2024 (consolidated)

(Million yen)	FY2023 Results	FY2023 Ratio	FY2024 Forecast	FY2024 Ratio	YoY Change Amt	YoY Change %
Revenue	**148,255**	**100.0%**	**160,000**	**100.0%**	**+11,745**	**+7.9%**
(Pharmaceuticals)	(125,105)	(84.4%)	(138,500)	(86.6%)	(+13,395)	(+10.7%)
(Functional Food)	(23,150)	(15.6%)	(21,500)	(13.4%)	(-1,650)	(-7.1%)
Cost of sales	**50,234**	**33.9%**	**51,300**	**32.1%**	**+1,066**	**+2.1%**
SG&A expenses	**34,959**	**23.6%**	**38,700**	**24.2%**	**+3,741**	**+10.7%**
R&D expenses	**31,676**	**21.4%**	**34,300**	**21.4%**	**+2,624**	**+8.3%**
Other income	**3,163**	**2.1%**	**1,000**	**0.6%**	**-2,163**	**-68.4%**
Other expenses	**1,252**	**0.7%**	**700**	**0.4%**	**-552**	**-44.1%**
Operating profit	**33,295**	**22.5%**	**36,000**	**22.5%**	**+2,705**	**+8.1%**
Finance income	**650**	**0.4%**	**700**	**0.4%**	**+50**	**+7.6%**
Finance costs	**329**	**0.2%**	**100**	**0.1%**	**-229**	**-69.7%**
Profit before tax	**33,616**	**22.7%**	**36,600**	**22.9%**	**+2,984**	**+8.9%**
Income tax expense, etc	**7,765**	**5.2%**	**5,100**	**3.2%**	**-2,665**	**-34.3%**
Profit attributable to owners of parent	**25,851**	**17.4%**	**31,500**	**19.7%**	**+5,649**	**+21.9%**

3Q FY2023 Actual	3Q FY2024 Actual	4Q FY2024 Forecast
143.3	**152.6**	**150.0**

Foreign exchange rates (USDJPY)

The exchange rate assumed for 4Q FY2024 in the business forecast is 1 USD=150 yen.
The sensitivity of the exchange rate is assumed to be an increase of approx. 100 million yen in revenue and approx. 200 million yen in operating profit for every 1 yen depreciation of the yen.

Please see page seven of the slide.

I would like to continue by explaining our earnings forecast for FY2024.

This is an upward revision from the earnings forecast announced in last November.

Consolidated sales revenue is expected to be JPY160,000 million.

Regarding operating expenses, the cost-to-sales ratio is expected to be 32.1%, an improvement of 1.8 percentage points YoY.

SG&A expenses are expected to be JPY38,700 million, and R&D expenses are expected to be JPY34,300 million.

As a result, we expect operating profit of JPY36,000 million, profit before tax of JPY36,600 million, and profit attributable to owners of the parent of JPY31,500 million.

Comparison with Previous Forecast

(Million yen)	FY2024 Forecasts		Change	
	Previous*	Revised	Amt	%
Revenue	**157,000**	**160,000**	**+3,000**	**+1.9%**
(Pharmaceuticals)	(135,500)	(138,500)	(+3,000)	(+2.2%)
(Functional Food)	(21,500)	(21,500)	-	-
Cost of sales	**50,500**	**51,300**	**+800**	**+1.6%**
SG&A expenses	**39,000**	**38,700**	**-300**	**-0.8%**
R&D expenses	**33,000**	**34,300**	**+1,300**	**+3.9%**
Other income	**900**	**1,000**	**+100**	**+11.1%**
Other expenses	**2,400**	**700**	**-1,700**	**-70.8%**
Operating profit	**33,000**	**36,000**	**+3,000**	**+9.1%**
Finance income	**700**	**700**	**-**	**-**
Finance costs	**100**	**100**	**-**	**-**
Profit before tax	**33,600**	**36,600**	**+3,000**	**+8.9%**
Income tax expense, etc	**3,600**	**5,100**	**+1,500**	**+41.7%**
Profit attributable to owners of parent	**30,000**	**31,500**	**+1,500**	**+5.0%**

* November 13th, 2024 (2Q FY2024 Financial Results)

Revenue 160,000 million yen
(+3,000 million yen, +1.9% from previous forecast)
- ✓ Foreign exchange contribution (depreciation of yen) to Viltepso's US sales and royalty income from Uptravi's overseas sales
- ✓ Sales increase of new products including Vyxeos

SG&A expenses 38,700 million yen
(-300 million yen, -0.8 % from previous forecast)
- ✓ Reduction of expenses in some departments

R&D expenses 34,300 million yen
(+1,300 million yen, +3.9% from previous forecast)
- ✓ Increase in manufacturing cost of investigational products for Viltepso, R&D cost of licensed products, etc.

Other operating expenses 700 million yen
(-1,700 million yen, -70.8% from previous forecast)
- ✓ Decrease in foreign exchange losses due to the change in the 4Q foreign exchange rate assumption from 140 yen to 150 yen per dollar

The exchange rate assumed for 4Q FY2024 in the business forecast is 1 USD=150 yen. The sensitivity of the exchange rate is assumed to be an increase of approx. 100 million yen in revenue and approx. 200 million yen in operating profit for every 1 yen depreciation of the yen.

Please see page eight of the slide.

Here we show the comparison with the previous forecast.

Sales revenue is expected to increase by JPY3,000 million compared to the previous forecast due to the impact of the weaker yen on royalty income from overseas sales of Uptravi and U.S. sales of Viltepso.

R&D expenses are expected to increase by JPY1,300 million from the previous forecast due to an increase in manufacturing costs of investigational products for Viltepso.

Other expenses are expected to decrease by JPY1,700 million from the previous forecast due to a decrease in foreign exchange losses by the change in the exchange rate assumption from JPY140 to JPY150 per US dollar. As a result, we expect operating profit to increase by JPY3,000 million.

R&D Updates (1/2)

Recent status/event	Code No. (Generic name)	Product name	Indications and topics	Schedule
P3	NS-065/NCNP-01 (viltolarsen)	Viltepso	Clinical Study Report, including the complete data set of Study 301, has been submitted. Protocol of Study 303 is still under discussion with FDA.	December 2024
Launch	ZX008 (fenfluramine hydrochloride)	Fintepla	Lennox-Gastaut syndrome (additional indication)	March 2024
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
Launch (additional indication) and approval	NS-304 (selexipag)	Uptravi	pediatric pulmonary arterial hypertension	December 2024
In application	CAP-1002 (deramiocel)	–	Duchenne muscular dystrophy cardiomyopathy	December 2024 (U.S.)
Start of P2	NS-089/NCNP-02 (brogidirsen)	–	Duchenne muscular dystrophy	February 2024
Start of P2	NS-229	–	eosinophilic granulomatosis with polyangiitis	June 2024
Start of P1/ P2	NS-050/NCNP-03	–	Duchenne muscular dystrophy	October 2024
Temporarily suspended	NS-580	–	endometriosis chronic prostatitis / chronic pelvic pain syndrome	–

For updates since Q2 FY2024 financial results announcement on November 13, 2024, see highlighted text in red.

Next, I will explain the progress of R&D pipeline.

This section explains the items that have been updated since the release of the previous financial results for Q2 FY2024.

Please see page 10 of the slide.

For NS-065/NCNP-01, a clinical trial summary report including the complete data set from Study 301 has been submitted to the FDA, and the protocol for Study 303 is in ongoing discussions with the FDA.

The sale of Yuvanci, a treatment for pulmonary arterial hypertension, were launched in November 2024.

Regarding Uptravi, in December 2024, we received approval for a partial change to the manufacturing and marketing approval to add usage and dosage for pediatric pulmonary arterial hypertension, as well as manufacturing and marketing approval for Uptravi for pediatric use.

As for CAP-1002, Capricor Therapeutics completed the rolling BLA submission in the U.S. for the expected indication of Duchenne muscular dystrophy cardiomyopathy in December 2024.

R&D Updates (2/2)

Recent status/event	Code No. (Generic name)	Product name	Indications and topics	Schedule
In-license (Vicore Pharma)	C21	–	idiopathic pulmonary fibrosis	Contract signed in February 2024
Alliance agreement (Eli Lilly Japan)	LY3527727 (piltobrutinib)	Jaypirca	mantle cell lymphoma chronic lymphocytic leukemia	Contract signed in March 2024
Letter of Intent (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	LOI signed in September 2024 (Europe)
In-license (Atsena Therapeutics)	ATSN-101	–	GUCY2D-associated Leber congenital amaurosis	Contract signed in November 2024
In-license (REGENXBIO Inc.)	RGX-121 (clemidsogene lanparvovec)	–	Mucopolysaccharidosis Type II	Contract signed in January 2025 (U.S. and Asia including Japan)
	RGX-111	–	Mucopolysaccharidosis Type I	
Option Agreement for Commercialization (AB2 BIO Ltd.)	Tadekinig alfa	–	NLRC4 mutation and XIAP deficiency	Contract signed in January 2025 (U.S.)
Preliminary analysis results	NS-065/NCNP-01 (viltolarsen)	Viltepso	global Phase 3 trial (RACER53 Study)	May 2024
Conference Presentations			Phase 2 trial (Galactic53 trial): 2024 Muscular Dystrophy Association Clinical & Scientific Conference	March 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.)
Alliance (MiNA Therapeutics)	–	–	a joint research agreement with the aim of creating nucleic acid medicines that are expected to be applied to an intractable and rare disease in the CNS field	April 2024

For updates since Q2 FY2024 financial results announcement on November 13, 2024, see highlighted text in red.

Please see page 11 of the slide.

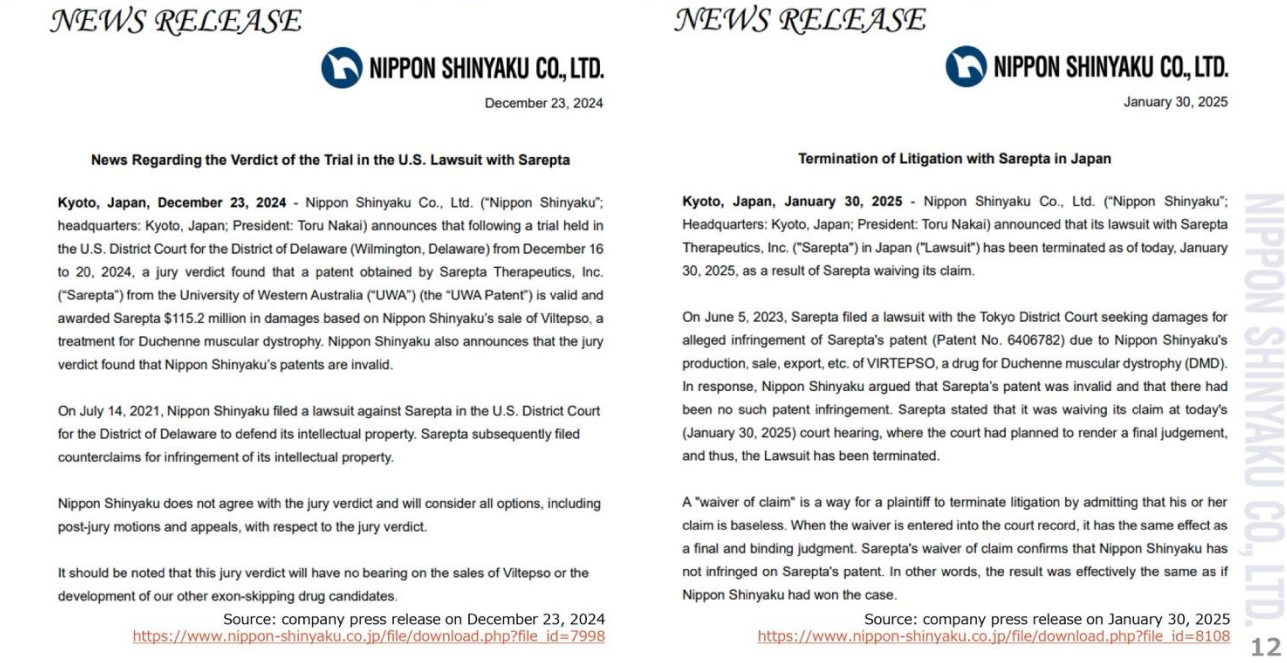
Regarding RGX-121/111, for the treatment of Mucopolysaccharidosis II and I (MPS II and I), we entered into a license agreement with REGENXBIO Inc. in January 2025 for the exclusive commercialization rights in the U.S. and the exclusive development and commercialization rights in Asia including Japan.

Regarding Tadekinig alfa, which is being developed in Europe and the US for the expected indication of NLRC4 mutation and XIAP deficiency, we have signed an option agreement in January 2025 to obtain exclusive commercialization rights in the US.

In December 2024, NS-089/NCNP-02 received Pioneering Drug Designation and Orphan Drug Designation in Japan, and in January 2025, the results of an investigator-initiated clinical trial was published in the academic journal, *Cell Reports Medicine*.

NS-051/NCNP-04 received Rare Pediatric Disease Designation in the U.S. in January 2025.

Litigations with Sarepta Therapeutics, Inc.



NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

December 23, 2024

News Regarding the Verdict of the Trial in the U.S. Lawsuit with Sarepta

Kyoto, Japan, December 23, 2024 - Nippon Shinyaku Co., Ltd. ("Nippon Shinyaku"; headquarters: Kyoto, Japan; President: Toru Nakai) announces that following a trial held in the U.S. District Court for the District of Delaware (Wilmington, Delaware) from December 16 to 20, 2024, a jury verdict found that a patent obtained by Sarepta Therapeutics, Inc. ("Sarepta") from the University of Western Australia ("UWA") (the "UWA Patent") is valid and awarded Sarepta $115.2 million in damages based on Nippon Shinyaku's sale of Viltepso, a treatment for Duchenne muscular dystrophy. Nippon Shinyaku also announces that the jury verdict found that Nippon Shinyaku's patents are invalid.

On July 14, 2021, Nippon Shinyaku filed a lawsuit against Sarepta in the U.S. District Court for the District of Delaware to defend its intellectual property. Sarepta subsequently filed counterclaims for infringement of its intellectual property.

Nippon Shinyaku does not agree with the jury verdict and will consider all options, including post-jury motions and appeals, with respect to the jury verdict.

It should be noted that this jury verdict will have no bearing on the sales of Viltepso or the development of our other exon-skipping drug candidates.

Source: company press release on December 23, 2024
https://www.nippon-shinyaku.co.jp/file/download.php?file_id=7998

NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

January 30, 2025

Termination of Litigation with Sarepta in Japan

Kyoto, Japan, January 30, 2025 - Nippon Shinyaku Co., Ltd. ("Nippon Shinyaku"; Headquarters: Kyoto, Japan; President: Toru Nakai) announced that its lawsuit with Sarepta Therapeutics, Inc. ("Sarepta") in Japan ("Lawsuit") has been terminated as of today, January 30, 2025, as a result of Sarepta waiving its claim.

On June 5, 2023, Sarepta filed a lawsuit with the Tokyo District Court seeking damages for alleged infringement of Sarepta's patent (Patent No. 6406782) due to Nippon Shinyaku's production, sale, export, etc. of VIRTEPSO, a drug for Duchenne muscular dystrophy (DMD). In response, Nippon Shinyaku argued that Sarepta's patent was invalid and that there had been no such patent infringement. Sarepta stated that it was waiving its claim at today's (January 30, 2025) court hearing, where the court had planned to render a final judgement, and thus, the Lawsuit has been terminated.

A "waiver of claim" is a way for a plaintiff to terminate litigation by admitting that his or her claim is baseless. When the waiver is entered into the court record, it has the same effect as a final and binding judgment. Sarepta's waiver of claim confirms that Nippon Shinyaku has not infringed on Sarepta's patent. In other words, the result was effectively the same as if Nippon Shinyaku had won the case.

Source: company press release on January 30, 2025
https://www.nippon-shinyaku.co.jp/file/download.php?file_id=8108

NIPPON SHINYAKU CO., LTD.

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Lastly, I would like to explain the litigations with Sarepta.

Following the trial held in the U.S. from December 16 to 20, 2024, a jury verdict found that the patent obtained by Sarepta Therapeutics, Inc. from the University of Western Australia is valid and awarded Sarepta USD115.2 million in damages based on Nippon Shinyaku's sale of Viltepso.

As for the lawsuit in Japan, Sarepta waved its claim at the court hearing on January 30, 2025, where the court had planned to render a final judgement, and the lawsuit has been terminated as of that date, as a result of Sarepta waiving its claim, which was effectively the same as if Nippon Shinyaku had won the case.
We believe that the outcome of the lawsuit in Japan has increased the likelihood of overturning the jury verdict on the U.S. lawsuit. We will continue to take action with even greater confidence in the U.S. lawsuit.

This concludes my explanation of the results for Q3 FY2024 and the progress of our R&D pipeline.

Q&A for 3Q FY2024 Financial Results (Summary)

February 7, 2025

No.	Questions	Answers
1	In FY2024 forecast, revenue and operating profit have been revised upward by 3 billion yen respectively. Of those, how much is due to changes in foreign exchange assumptions?	Of the 3 billion yen revisions, 450 million yen is for Viltepso's U.S. sales and 1.55 billion yen is for revenues from the industrial property rights. In our estimates, the total of approximately 2 billion yen is due to the impact of changes in foreign exchange assumptions.
2	Is the revised forecast for other expenses almost entirely due to changes in foreign exchange assumptions?	Most of revisions in the other expenses item are due to changes in foreign exchange gains and losses.
3	R&D expenses have been increased by 1.3 billion yen in the revised forecast. Considering that 12 billion yen was spent in the last 4Q alone, will 11-12 billion yen be spent in this 4Q? Will next year's R&D expenses increase by about 3 billion yen from this year's forecast of 34.3 billion yen?	Since a large amount of contract research expenses and manufacturing costs for investigational products will be incurred in the 4Q, the full year forecast has been revised to include such expenses and they will be used as planned. We will disclose FY2025 R&D expenses forecast at the announcement of the full-year financial results for FY2024, but we expect them to increase.
4	FY2024 SG&A expenses forecast is 38.7 billion yen. If CAP-1002 and other products are launched in FY2025, how much will SG&A expenses increase? Should we wait for next year's guidance?	We cannot disclose specific figures at this time, but there is no doubt that SG&A expenses will increase. Guidance for FY2025 will be disclosed at the announcement of the full-year financial results for FY2024.
5	Are the upfront payments for multiple in-licensing agreements this quarter not related to this revision?	Since they are recorded as assets, there is basically no impact.
6	Regarding the domestic sales competition for Viltepso, Chugai Pharmaceutical filed a regulatory application for the gene therapy product, and its approval and launch is expected. Although sales of Viltepso have not increased much in Japan, is the number of new patients still increasing? What will be the domestic sales of Viltepso in FY2025 and beyond?	Since the number of new cases has been canceled out by that of discontinuation cases, the total growth rate has leveled off. Sales growth has also slowed, and we expect domestic sales increase of Viltepso will be flat in FY2025 and beyond.

7	The number of Viltepso patients in Japan has not reached the expected peak number of patients, although it is currently administered to more than two-thirds of the peak number of patients as indicated in the Chuikyo* data at the time of the official drug price calculation. What do you think has caused the divergence in the number of patients administered at a level lower than the initial expectation? * Central Social Insurance Medical Council: an advisory body to the Minister of Health, Labor and Welfare that examines and reports on medical fees, drug prices paid by insurers to medical institutions	The current number of patients is more than two-thirds of the peak number of patients which was Initially assumed to be 128. The major difference from the assumption is that patients with complications cannot be administered. Although the cumulative number of patients treated so far is close to the number assumed at the peak, it is still more than two-thirds due to some cases of discontinuation.
8	Even when assuming the peak number of patients to be 128, you may have expected a certain number of cases to be interrupted. Do you mean that the number of cancellations is higher than your expectation?	Yes, we do.
9	Is the reason that the number of patients in Japan has not reached the initially expected peak number for Viltepso also common to other exon-skipping drugs?	I think this is a common concept for other exon-skipping drugs.
10	It seems that Viltepso's sales in USD decreased slightly in Q3 compared to Q2 and are expected to increase again in Q4. Is this due to the impact of gene therapy drug?	Although we do not have access to the details of the patients' personal information, we estimate that around 2 or 3 cases have switched from Viltepso to gene therapy.
11	In FY2023, U.S. sales of Viltepso in 4Q were not good due to medical insurance renewals. Do you think this will be the case this fiscal year?	Our revised full-year forecast incorporates such seasonal factors.
12	Will sales and patient number for Viltepso in the U.S. remain flat from next year onwards?	While we will not disclose specific figures, we expect so.

13	I would like to ask about the status of discussions with the FDA regarding the maintenance of accelerated approval for Viltepso in the U.S. Originally, we had expected that a Type C Meeting would be held around this coming March, as you had said you would submit the data set and protocol to the FDA by the end of last December, and the FDA generally holds a meeting within 75 days of receiving an application. What was the reason for the delay in your protocol submission?	In submitting the complete protocol, which was originally scheduled to be submitted in December, we asked for expert opinions on statistical analysis methods and received the opinion that a slightly more complex statistical analysis method would be best for this trial, and we are continuing our review, including clinical faculty members. Since the Clinical Study Report was submitted in December, we believe that the FDA is reviewing the detailed information. We are also communicating with the FDA regarding the status of the protocol preparation. Therefore, we do not believe that there will be any delays in the timing of a meeting with the FDA due to the fact that we have not submitted the protocol.
14	When will the protocol submission be complete and when will you apply for a meeting with the FDA?	There is no strict rule as to how many days after we submit materials a meeting can be held. Even after the Type C Meeting scheduled for October last year was canceled, we have continued to communicate with the FDA, and we do not think that the meeting schedule will be delayed significantly.

15	Assuming that there is a wait period of 2 to 3 months after submitting the Clinical Study Report of Study 301, is the protocol for Study 303 being prepared in parallel?	In preparation for the meeting with the FDA scheduled for October last year, we submitted the following two items beforehand. 1. Question as to whether the FDA would agree to conduct an additional P3 study while keeping Viltepso on the market 2. Outline of the Study 303 protocol, which was improved based on our analysis of the reasons why the primary endpoints were not met, referring to Study 301 data In response, the FDA requested the following. A) Additional Clinical Study Report and the data set signed by a principal investigator of Study 301 B) Submission of complete Study 303 protocol We have already submitted the outline of Study 303, so we understand that the FDA is currently conducting a detailed review of the data from Study 301 based on that. We expect to be able to submit the full protocol by the end of this month or the beginning of next month, but we do not believe that the FDA's review is not progressing because we have not yet submitted the full protocol for Study 303.
16	What is the current status of discussions with the FDA regarding maintaining the accelerated approval of Viltepso?	Ongoing discussions are underway with the FDA regarding keeping Viltepso on the market. There were originally two points to discuss at the Type C Meeting, which was supposed to be held last October. 1. Additional P3 Study (303) while keeping Viltepso on the market 2. FDA’s agreement on the design of additional P3 Study (303) Even if we cannot fully agree on the protocol of second point at the next meeting, we may still reach a conclusion on whether to keep Viltepso on the market.

17	Is it correct to understand that there will be some movement around March regarding the protocol for Study 303 and whether or not Viltepso will continue to be sold?	The timing of the meeting with the FDA will be after the FDA has reviewed the data from Study 301 and the protocol for Study 303. At this point, we expect the FDA to complete its review around the beginning of next fiscal year (April 2025).
18	As to whether to keep Viltepso on the market or not, are you feeling any changes from the new government in the U.S.?	Although we have been in communication with the FDA since October last year, there is no indication that the FDA's attitude has changed in response to the new U.S. administration's intentions.
19	Regarding the patent lawsuit with Sarepta Therapeutics, Inc., you told us that the conclusions of the Japanese trial could be applied to the future U.S. decision, but objectively speaking, there are still some concerns since you lost the first trial in the U.S. Your detailed comment is appreciated.	The Japanese court system is different from the U.S. court system, but the issues are largely the same. Because Sarepta's patent is very broad, we argued in Japan that the support and enablement requirements were not met. On January 30 this year, we expected the Tokyo District Court to rule in our favor, but Sarepta waived its claims, and the trial ended. The result was effectively the same as if we had won the case. Although the wording is slightly different in the U.S. court case, the issues are almost the same, and the issues are the description requirement and the enablement requirement. Therefore, just as our arguments were accepted by the patent experts in Japan, we believe that there is a high possibility that our arguments will be accepted in the U.S. as well, as the patent experts at the Federal Circuit will make a judgment.
20	I think the difference from the lawsuit in the U.S. is that Sarepta’s product is not sold in Japan and there is no substantial damage to them. Could this be the reason why Sarepta waived its claims in Japan?	Since the lawsuit was filed in Japan when Sarepta's exon 53 skipping drug was not launched in Japan in June 2023, we do not believe that whether or not their exon 53 skipping drug was launched in Japan is directly relevant to the waiver of their claim. We suppose that Sarepta avoided an obvious ruling because they assumed that our argument - that Sarepta's patent was invalid and that there was no patent infringement by us - would be accepted.

21	You effectively won the lawsuit against Sarepta in Japan. As for the litigation in the U.S., since Sarepta's patent will expire in June this year, is the jury verdict of $115.2 million the worst-case payment amount for you?	The UWA patent held by Sarepta will expire in June this year, but the $115.2 million in damages is based on sales figures up to just before the trial in December last year. In the worst case scenario, where we lose the case completely, there is a possibility that the amount of damages based on sales of Viltepso for about six months until the patent expires will be added to it.
22	The number of In-licensing has been increasing since last November. Given that RGX-121 and Tadekinig alfa are likely to be approved around the second half of next fiscal year in the best scenario, would it be difficult for the current 125 staff at NS Pharma* to handle multiple products in addition to Viltepso? * also known as NSP, a U.S. subsidiary of Nippon Shinyaku,	Preparations are underway at NSP for the launch of a new product line. Until now, NSP's organization has only dealt with the Viltepso product, but in preparation for the future launch of additional products, the company is planning to hire new staff. Optimal recruitment activities are underway, based on an optimal organizational design that takes into account common tasks for all products.
23	The new product line is similar to Viltepso in terms of pediatric diseases. However, each of them is in a different disease area from DMD. I wonder how many new jobs will be needed.	At present, there is no specific answer that can be disclosed. We will explain as appropriate in the future.
24	Assuming that the overall NSP headcount will increase, I would like to know about SG&A expenses. Next year, there will be additional expenses for launch preparation and actual sales activities for CAP-1002. How will SG&A expenses change as a result of commercialization of CAP-1002 and other new products next year?	The CAP-1002 launch preparation expenses are included in the 4Q budget. Pre and post launch expenses for new products will increase.
25	Since you have acquired the commercialization rights for CAP-1002 in Japan and the U.S., will you also be actively disclosing information? Capricor, being a biotech company, is very energetic in its disclosure and presentations, but are you going to provide more practical information and data explanations for new product sales in the future?	We also want to actively disclose information. We plan to give a detailed explanation of in-licensed products at the R&D Meeting on February 18th.

26	I think that CAP-1002 and RGX-121 will become important in your product lineup in the future, but at the moment, the only data available for RGX-121 is biomarker data. Why do you think this drug will be chosen in a highly competitive market? REGENXBIO has disclosed that the royalty for the license agreement is “meaningful double-digit”. Referring to that Capricor has disclosed that they will receive a 30-50% of product revenue, which includes the product cost, Are the economic terms for the RGX-121 in-licensing agreement similar to those of the CAP-1002’s?	In the therapeutic area of RGX-121, there is one product on the market in the U.S. and three in Japan for enzyme replacement therapy. While enzyme replacement therapy is administered intravenously once a week or intraventricular administration once a month, RGX-121 is a one-time therapy and expected to be effective. In addition, while enzyme replacement therapy has a weak effect on central symptoms, gene therapy is administered directly into the brain and is expected to be effective on central symptoms. In clinical trials, it is being investigated as an add-on to enzyme replacement therapy, and there is data showing that three-quarters of patients in these cases were able to discontinue enzyme replacement therapy. This is a differentiation point from other products, and we believe that it has sufficient sales potential in the mucopolysaccharidosis type II field. Regarding the royalty for RGX-121, we would like to refrain from disclosing specific figures beyond the double-digit range.
27	NS-051 has received Rare Pediatric Disease Designation, will clinical trials begin this quarter or the first half of next fiscal year?	As we are still in discussions with the FDA regarding the findings of toxicity identified in non-clinical trials, it is difficult to start the trials by the end of this fiscal year (March 2025). We are working to resolve the issues one by one through continued discussions with the FDA. However, it is still difficult to say exactly when we will begin clinical trials in FY2025.

28	I would like to know about your future business investment policy. As other companies overseas are also making progress in the development of exon-skipping drugs, I think you will need new platform technologies, including the introduction of early-stage products, in the long term. Your most recent in-licensed activities are late-stage products, and many of them will contribute to sales revenue after the patent for Uptravi expires. Will you continue to in-license products that will contribute to the top line in the near future, or will you also focus on searching for early-stage products?	We have been watching a wide range of product candidates from the early to late stages, and we are not focusing on any particular stage.
29	Is competition in the market for exon-skipping drugs becoming more intense and complex?	Recently, we are aware that several exon-skipping drugs have been developed by small biotech companies in the U.S., etc., that have extended the dosing interval by devising a method of delivering nucleic acids. Our company has also been considering devising a method of delivery, and we would like to continue our research while checking the technological trends of other companies.
30	What will be the theme of the R&D Meeting scheduled for February 18th? There is very little information about Tadekinig alfa, including data, but will it be covered at the R&D Meeting on February 18th? Since your company is working on complex areas, including rare diseases, if you could organize and illustrate the mechanism of action of the disease, etc., it would make it easier for us as analysts to follow your company's pipeline.	Tadekinig alfa is not included in the meeting topics because it is still under option agreement. Instead, we plan to give a detailed explanation of the disease profile and product features of RGX-121/111 and ATSN-101, which were introduced this fiscal year. We will also explain in detail the features of the cell therapy drug “CAP-1002”, including the change in the indication for BLA application to DMD cardiomyopathy.