Outline of Consolidated Financial Results for the 3rd Quarter Ended December 31, 2024

February 7, 2025
NIPPON SHINYAKU CO., LTD.

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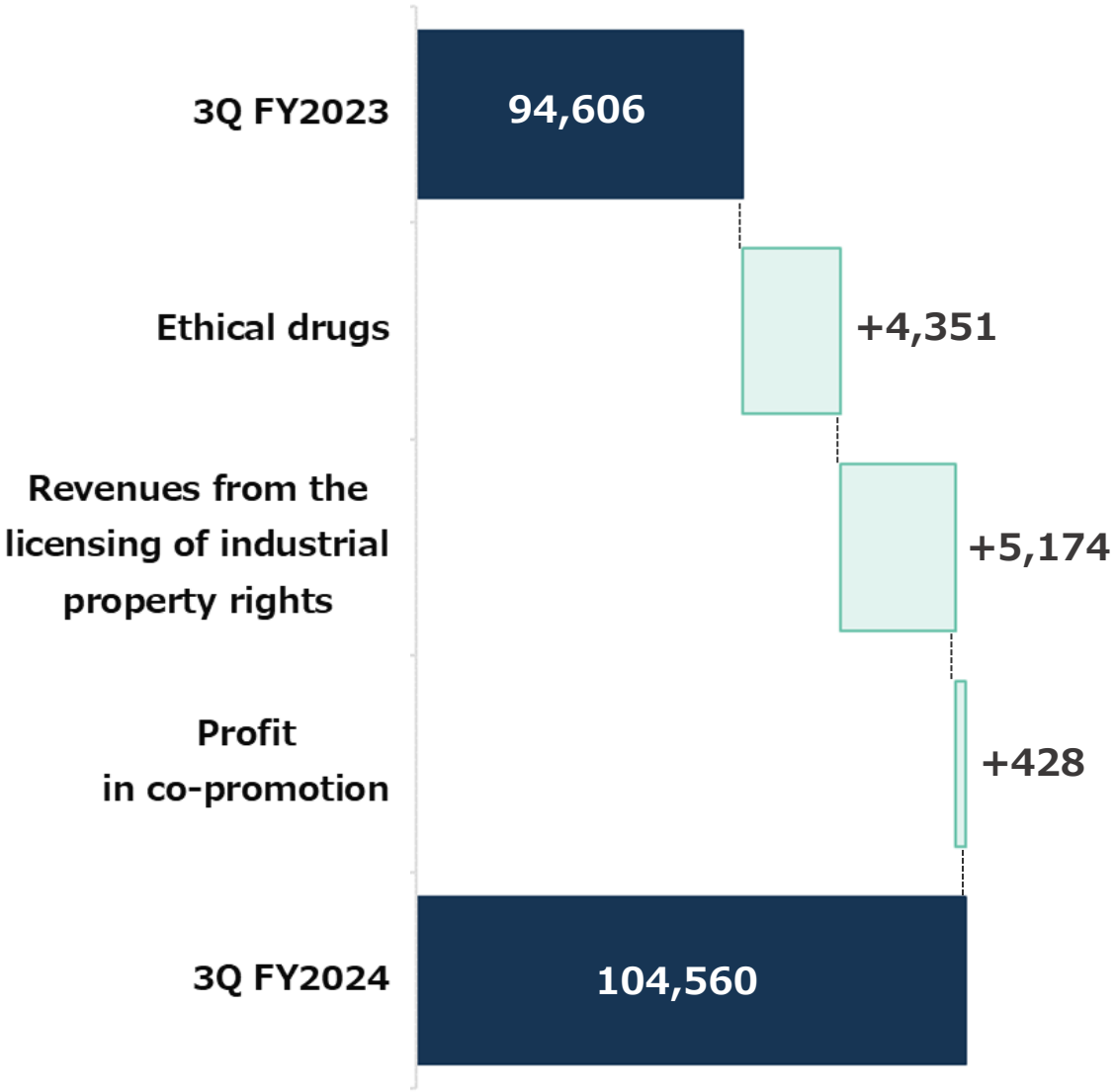
3Q FY2024 Summary (April-December)

(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%**

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

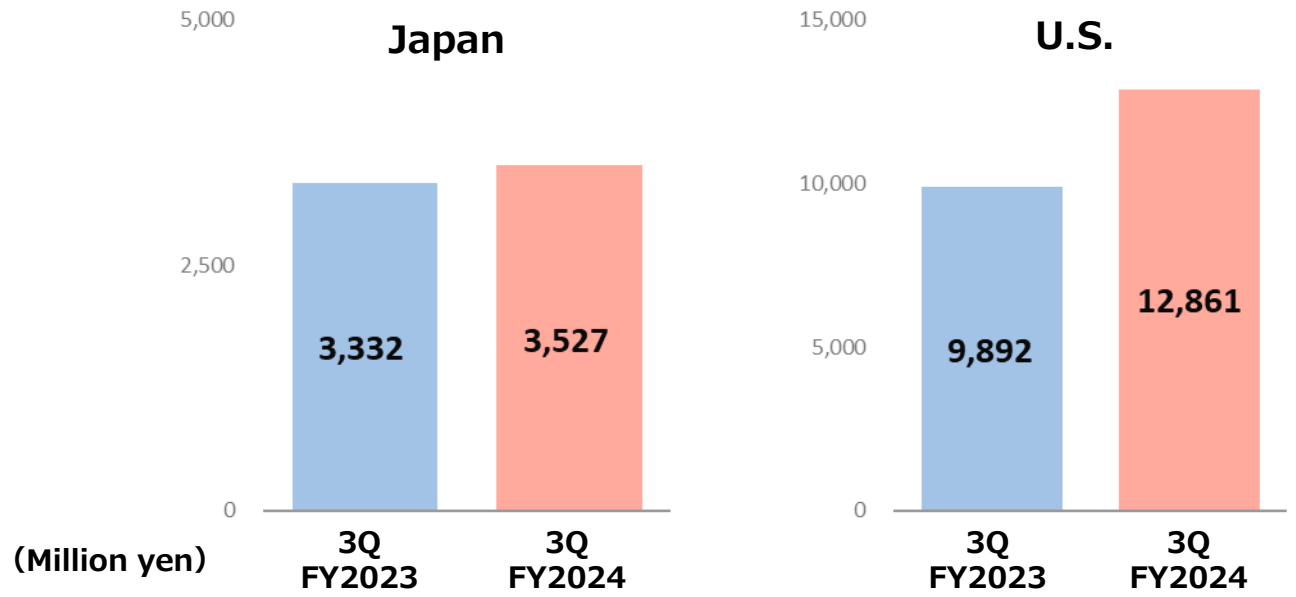
Profit in co-promotion 7,132 million yen
(+428 million yen, +6.4%, YoY)

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

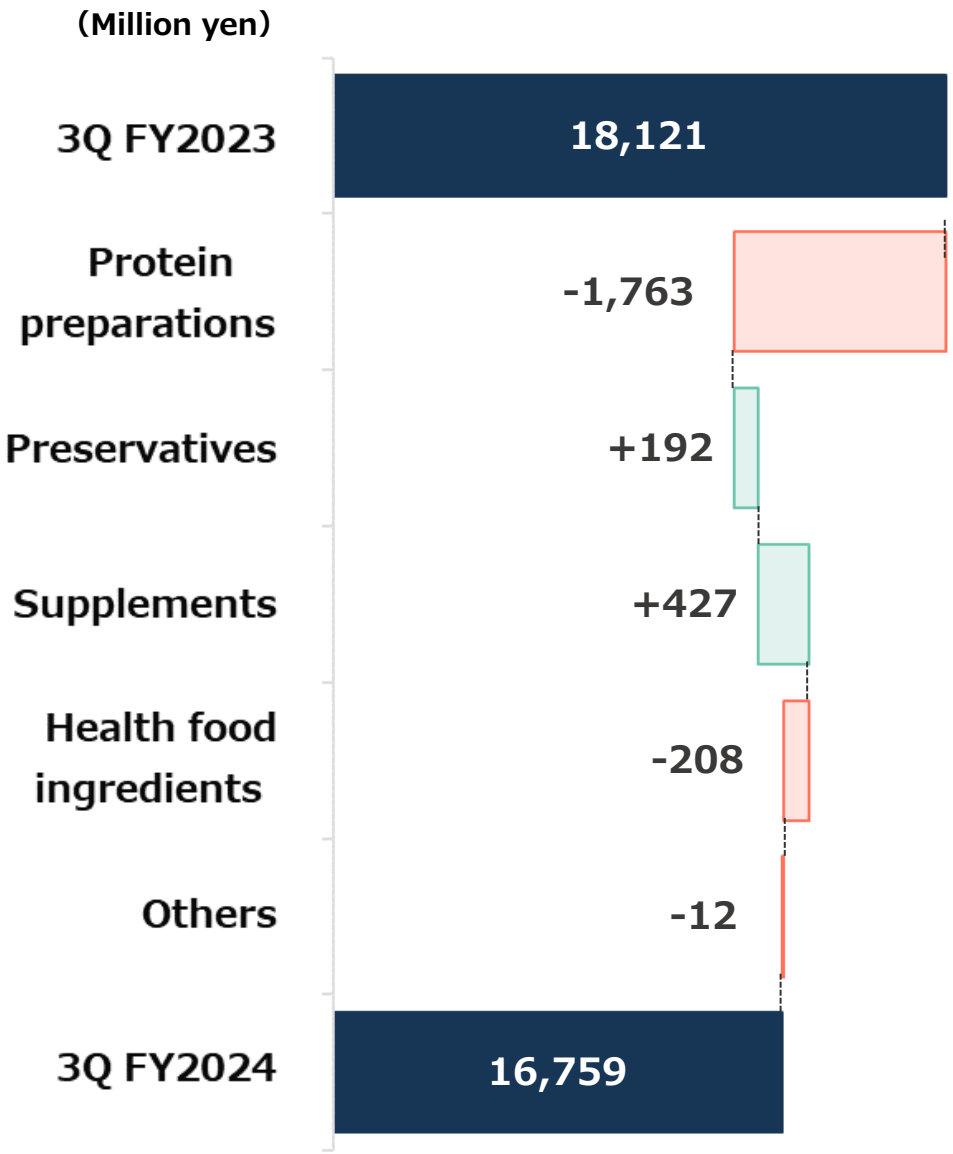
Sales Trends of Viltepso® (viltolarsen)

(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



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)

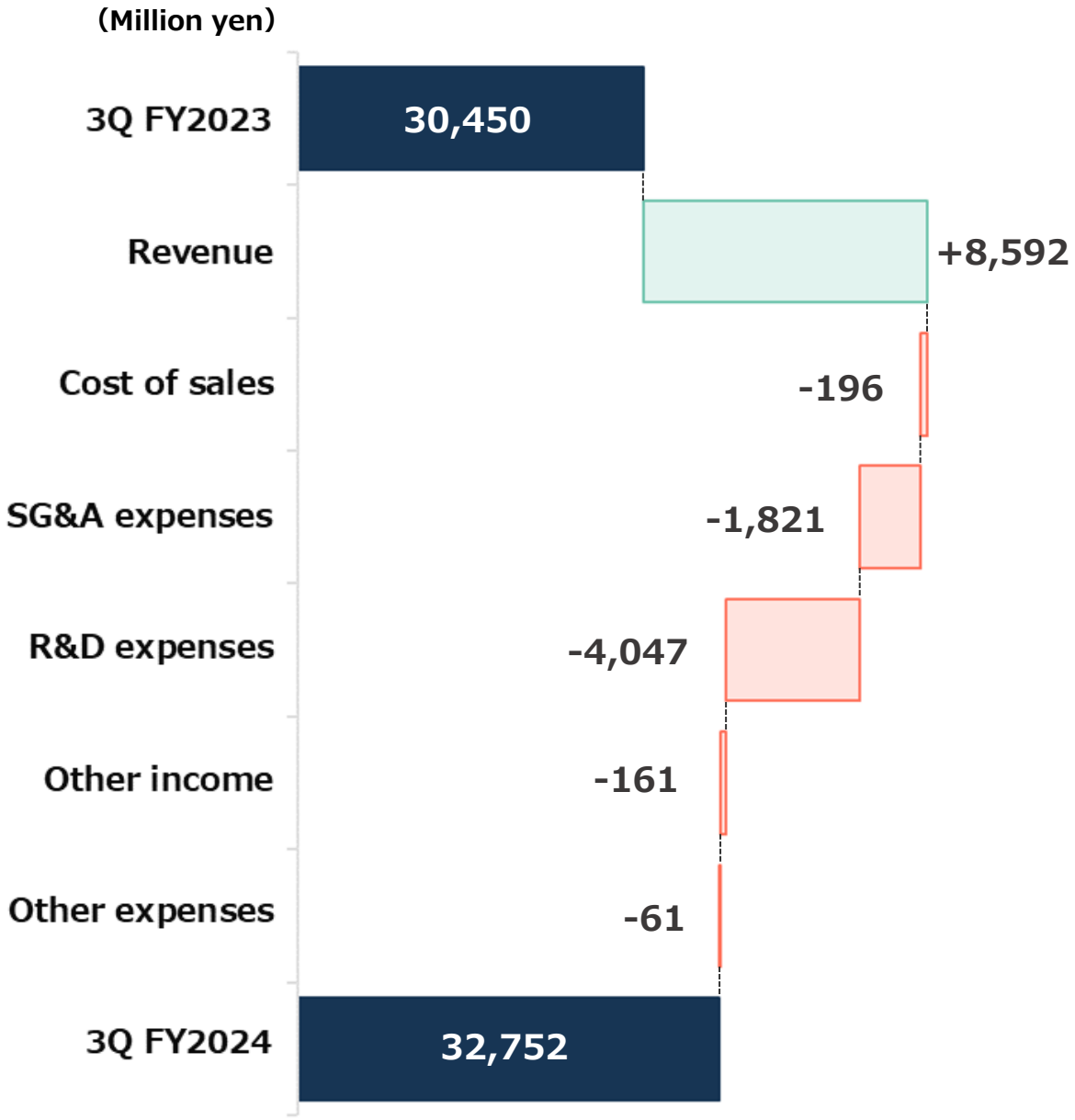
- ✓ Recovery in food service-related business due to active tourism and excursions

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)

Operating Profit



Revenue 121,320 million yen
(+8,592 million yen, +7.6%, YoY)

- ✓ Sales of Viltepso, Uptravi, and new product Vyxeos
- ✓ Increase in revenue from industrial property rights and the U.S. sales of Viltepso

Cost of sales 38,810 million yen
(+196 million yen, +0.5%, YoY)
The ratio was improved by 2.3 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 27,562 million yen
(+1,821 million yen, +7.1%, YoY)

- ✓ Increase in labor costs and commission for promotional activities of Uptravi
- ✓ Increased sales expenses in Japan and U.S.

R&D expenses 23,547 million yen
(+4,047 million yen, +20.8%, YoY)

- ✓ Increase in contract research expense and manufacturing costs for investigational products

Revised Business Forecast for FY2024 (consolidated)

(Million yen)	FY2023		FY2024		YoY Change	
	Results	Ratio	Forecast	Ratio	Amt	%
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.

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.

R&D PIPELINE

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.

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.

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

REFERENCE MATERIALS

Sales Forecast in Pharmaceutical Segment

(Million yen)

Brand name	Indications	3Q FY2023 Results	3Q FY2024 Results	YoY Change Amt	YoY Change %	FY2024 Full-year forecast
Viltepso		13,225	16,389	+3,164	+23.9%	21,850
(Japan)	Duchenne muscular dystrophy	(3,332)	(3,527)	(+195)	(+5.9%)	(4,600)
(U.S.)		(9,892)	(12,861)	(+2,968)	(+30.0%)	(17,250)
Uptravi	pulmonary arterial hypertension/ chronic thromboembolic pulmonary hypertension	10,024	11,623	+1,599	+16.0%	15,200
Vidaza	myelodysplastic syndrome/ acute myeloid leukemia	8,329	4,111	-4,218	-50.6%	5,100
Gazyva	CD20-positive follicular lymphoma/ CD20-positive chronic lymphocytic leukemia	3,692	3,805	+113	+3.1%	4,900
Vyxeos	high-risk AML	-	3,737	+3,737	-	4,900
Tramal/Onetram	cancer pain, chronic pain	3,142	2,179	-963	-30.7%	2,700
Defitelio	sinusoidal obstruction syndrome	1,712	2,090	+377	+22.1%	2,400
Cialis	erectile dysfunction	1,946	1,932	-13	-0.7%	2,400
Zalutia	urinary disorder caused by benign prostatic hyperplasia	1,788	1,235	-552	-30.9%	1,600
Adcirca	pulmonary arterial hypertension	1,791	1,302	-488	-27.3%	1,600
Erizas	allergic rhinitis	1,038	916	-122	-11.7%	2,000
Profit in co-promotion		6,703	7,132	+428	+6.4%	9,100
Revenues from the licensing of industrial property rights		28,897	34,071	+5,174	+17.9%	45,650
Revenue		94,606	104,560	+9,954	+10.5%	138,500

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 for every 1 yen depreciation of the yen.

Sales Forecast in Functional Food Segment

(Million yen)	3Q FY2023		3Q FY2024		YoY Change		FY2024
	Results	Ratio	Results	Ratio	Amt	%	Full-year forecast
Protein preparations	12,319	68.0%	10,556	63.0%	-1,763	-14.3%	13,000
Preservatives	2,383	13.1%	2,576	15.4%	+192	+8.1%	3,200
Supplements	1,466	8.1%	1,893	11.3%	+427	+29.2%	3,100
Health food ingredients	957	5.3%	748	4.5%	-208	-21.8%	1,100
Others	993	5.5%	983	5.8%	-12	-2.8%	1,100
Revenue	18,121	100.0%	16,759	100.0%	-1,361	-7.5%	21,500

Consolidated Balance Sheet

(Million yen)	End of FY2023	End of 3Q FY2024	Change Amt		End of FY2023	End of 3Q FY2024	Change Amt
Assets	263,404	284,459	+21,054	Liabilities	42,870	36,676	-6,193
Current assets	164,285	162,359	-1,925	Current liabilities	37,336	29,119	-8,217
Non-current assets	99,119	122,099	+22,980	Non-current liabilities	5,533	7,556	+2,023
				Equity	220,534	247,782	+27,248
Total aseets	263,404	284,459	+21,054	Total liabilities and equity	263,404	284,459	+21,054

Assets

Inventories	+5,389
Intangible assets	+7,570
Other financial assets (non-current)	+11,716

Liabilities and Shareholders' Equity

Trade and other payables	-7,925
Retained earnings	+20,543
Valuation difference on available-for-sale	+6,852

Pipeline（1/2）

Stage	Code No. (Generic name)	Origin	Application type	Indications	Schedule	Country
Launch P3	NS-065/NCNP-01 (viltolarsen)	In-house	NME	Duchenne muscular dystrophy	—	Japan/U.S.
Preparing for launch	NS-304 (selexipag)	In-house	New dose New indication	pediatric pulmonary arterial hypertension	Study Completion : FY 2025 Approval : December 2024	Japan
BLA filing	CAP-1002 (deramiocel)	Partnership Capricor Therapeutics, Inc.	NME	Duchenne muscular dystrophy cardiomyopathy	Approval : July 2025	U.S.
Rolling submission	RGX-121 (clemidsogene lanparvovec)	Partnership REGENXBIO Inc.	NME	Mucopolysaccharidosis Type II	Application completion : March 2025	U.S.
P3	ZX008 (fenfluramine hydrochloride)	Distribution partnership UCB S.A.	New indication	CDKL5 deficiency disorder	Study Completion : FY2026	Japan
	GA101 (obinutuzumab)	In-license Chugai Pharmaceutical Co., Ltd.	New indication	lupus nephritis	Projected submission : CY2026	Japan
				pediatric nephrotic syndrome	Projected submission : CY2026	Japan
				extra renal lupus	Projected submission : CY2027 and beyond	Japan
	CAP-1002 (deramiocel)	Partnership Capricor Therapeutics, Inc.	NME	Duchenne muscular dystrophy	—	U.S.
	LY3527727 (pirtobrutinib)	Alliance agreement Eli Lilly Japan K.K.	New indication	mantle cell lymphoma	—	Japan
				chronic lymphocytic leukemia	—	Japan

*Schedule is based on trial end dates, etc. from jRCT or ClinicalTrials.gov.

Pipeline（2/2）

Stage	Code No. (Generic name)	Origin	Application type	Indications	Schedule	Country
P2	NS-304 (selexipag)	In-house	New indication	arteriosclerosis obliterans	Study Completion : FY2024	Japan
	NS-580	In-house	NME	endometriosis	Temporarily suspended	Japan
				chronic prostatitis/ chronic pelvic pain syndrome	Temporarily suspended	Japan
	NS-089/NCNP-02 (brogidirsen)	In-house	NME	Duchenne muscular dystrophy	Study Completion : FY2025	Japan/U.S.
	NS-229	In-house	NME	eosinophilic granulomatosis with polyangiitis	Study Completion : FY2026	Japan/U.S.
P1/2	NS-401 (tagraxofusp)	In-license The Menarini Group	NME	blastic plasmacytoid dendritic cell neoplasm	Study Completion : FY2026	Japan
	NS-050/NCNP-03	In-house	NME	Duchenne muscular dystrophy	Study Completion : FY2027	Japan/U.S.
	ATSN-101	In-license Atsena Therapeutics	NME	GUCY2D-associated Leber congenital amaurosis	Study Completion : FY2027	U.S.
	RGX-111	Partnership REGENXBIO Inc.	NME	Mucopolysaccharidosis Type I	Study Completion : FY2024	U.S.
P1	NS-917 (radgocitabine)	In-license Delta-Fly Pharma, Inc.	NME	relapsed/refractory acute myeloid leukemia	Study Completion : FY2024	Japan
	NS-025	In-house	NME	urological diseases	Study Completion : FY2024	Japan
	NS-863	In-house	NME	cardiovascular diseases	Study Completion : FY2024	Japan

*Schedule is based on trial end dates, etc. from jRCT or ClinicalTrials.gov.

NS-065/NCNP-01 (viltolarsen)

- Treatment for Duchenne muscular dystrophy -

Development Phase	・Japan : Launch ・U.S. : Launch ・Global : P3 open-label extension study in progress
Origin	Co-development : National Center of Neurology and Psychiatry
Development	Nippon Shinyaku
Mechanism of action	Exon 53 Skipping
Indication	Duchenne muscular dystrophy
Dosage form	Injection
Feature	・Improvement in symptoms and prevention of the disease progression by recovery of dystrophin protein expression ・Morpholino based oligonucleotide with possible high safety profile and maximized activity

NS-304 (selexipag)

- Treatment for pulmonary hypertension, arteriosclerosis obliterans -

Development Phase	Japan : P2b (ASO) Japan : Uptravi® tablets 0.2 mg and 0.4 mg for the additional indication of pediatric pulmonary arterial hypertension (PAH) Uptravi® tablets for pediatric 0.05 mg approved and preparing for launch
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	Selective IP receptor agonist
Indication	arteriosclerosis obliterans (ASO) pediatric pulmonary arterial hypertension (pediatric PAH)
Dosage form	Tablet
Feature	Long-acting oral drug

CAP-1002 (deramiocel)

- Treatment for Duchenne muscular dystrophy -

Development Phase	U.S. : P3 (Duchenne muscular dystrophy) U.S. : BLA Filing (Duchenne muscular dystrophy cardiomyopathy)
Origin	[Jan. 2022] Partnership for commercialization in U.S. [Feb. 2023] Partnership for commercialization in Japan : Capricor Therapeutics, Inc.
Development	Capricor Therapeutics, Inc.
Mechanism of action	Exosomes released from cardiosphere-derived cells
Indication	Duchenne muscular dystrophy cardiomyopathy Duchenne muscular dystrophy
Dosage form	Injection
Feature	・Exosomes released from this drug are expected to reduce oxidative stress, inflammation, fibrosis, and increase cell energy and myocyte generation, resulting in improvement of motor and cardiac functions. ・ Its broad applicability makes it suitable for patients regardless of the type of genetic mutation.

RGX-121 (clemidsogene lanparvovec)

- Treatment for Mucopolysaccharidosis Type Ⅱ -

Development Phase	U.S. : Rolling submission
Origin	[Jan. 2025] Partnership for commercialization in U.S., Japan and other Asian countries : REGENXBIO Inc.
Development	REGENXBIO Inc.
Mechanism of action	Iduronate-2-sulfatase Gene therapy
Indication	Mucopolysaccharidosis Type Ⅱ
Dosage form	Injection
Feature	・An investigational gene therapy using adeno-associated virus (AAV) 9 to deliver the iduronate-2-sulfatase (IDS) gene to the central nervous system using intracisternal or intraventricular administration ・Transduced cells produce the missing IDS protein ・A single dose is expected to lead to sustained production of IDS leading to the attenuation of CNS manifestations in MPS II patients

ZX008 (fenfluramine hydrochloride)

- Treatment for rare intractable epilepsy -

Development Phase	Japan : Launch (Dravet syndrome) Japan : Launch（Lennox-Gastaut syndrome) Japan : P3 (CDKL5 deficiency disorder)
Origin	[Mar. 2019] Distribution partnership in Japan : UCB S.A. (former Zogenix, Inc.)
Development	UCB S.A. (former Zogenix, Inc.)
Mechanism of action	5-HT (serotonin) releaser with agonist activity at several 5-HT receptors
Indication	Dravet syndrome Lennox-Gastaut syndrome CDKL5 deficiency disorder
Dosage form	Oral liquid agent
Feature	・Effective for Dravet syndrome, Lennox-Gastaut syndrome and CDKL5 deficiency disorder patients refractory to existing treatment options ・ZX008 can be used in combination with other drugs, as standard of care for intractable epilepsy based on combination therapy.

GA101 (obinutuzumab)

- Treatment for lupus nephritis, pediatric nephrotic syndrome, extra renal lupus -

Development Phase	Japan : P3（LN） Global : P3（PNS） Japan : P3（ERL）
Origin	[Nov. 2012] Licensed-in from : Chugai Pharmaceutical Co., Ltd.
Development	Co-development : Chugai Pharmaceutical Co., Ltd.
Mechanism of action	Anti-CD20 monoclonal antibody
Indication	lupus nephritis (LN) pediatric nephrotic syndrome (PNS) extra renal lupus (ERL)
Dosage form	Injection
Feature	Anti-CD20 monoclonal antibody, increased antibody-dependent cellular cytotoxicity (ADCC) activity and direct cytotoxicity

LY3527727(pirtobrutinib)

- Treatment for Mantle cell lymphoma, Chronic lymphocytic leukemia -

Development Phase	・Launch (for patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors) ・P3 (MCL and CLL)
Origin	[Mar. 2024] Alliance agreement in Japan : Eli Lilly Japan K.K.
Development	Eli Lilly Japan K.K.
Mechanism of action	A reversible non-covalent BTK inhibitor
Indication	mantle cell lymphoma (MCL) chronic lymphocytic leukemia (CLL)
Dosage form	Oral agent
Feature	・A highly selective, non-covalent (reversible) inhibitor of the enzyme Bruton's tyrosine kinase (BTK), with having a novel binding mechanism.

NS-580

- Treatment for endometriosis, Chronic prostatitis/Chronic pelvic pain syndrome -

Development Phase	Japan : P2b (endometriosis) Temporarily suspended Japan : P2a (CP/CPPS) Temporarily suspended
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	Inhibition of membrane-associated prostaglandin E synthase-1
Indication	endometriosis chronic prostatitis/chronic pelvic pain syndrome (CP/CPPS)
Dosage form	Oral agent
Feature	・Treatment for endometriosis without hormonal effect and with possible analgesic potency ・Treatment for CP/CPPS with high safety and long-term pain control

NS-089/NCNP-02（brogidirsen）

- Treatment for Duchenne muscular dystrophy -

Development Phase	Global : P2
Origin	Co-development : National Center of Neurology and Psychiatry
Development	Nippon Shinyaku
Mechanism of action	Exon 44 Skipping
Indication	Duchenne muscular dystrophy
Dosage form	Injection
Feature	・Improvement in symptoms and prevention of the disease progression by recovery of dystrophin protein expression ・Morpholino based oligonucleotide with possible high safety profile and maximized activity

NS-229

- Treatment for Eosinophilic granulomatosis with polyangiitis -

Development Phase	Global : P2
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	JAK1 inhibitor
Indication	eosinophilic granulomatosis with polyangiitis (EGPA)
Dosage form	Oral agent
Feature	・Potent and highly selective JAK1 inhibitor ・High efficacy and good safety profiles are expected in the treatment for EGPA

NS-401 (tagraxofusp)

- Treatment for blastic plasmacytoid dendritic cell neoplasm -

Development Phase	Japan : P1/2
Origin	[Mar. 2021] Licensed-in from: The Menarini Group
Development	Nippon Shinyaku
Mechanism of action	Induction apoptosis of cells by inhibiting protein synthesis by specifically targeting cancer cells expressing CD123
Indication	blastic plasmacytoid dendritic cell neoplasm (BPDCN)
Dosage form	Injection
Feature	・Composed of diphtheria toxin (DT) fusion protein and recombinant human IL-3 ・Novel targeted therapy directed to CD123 on tumor cells ・IL-3 binds to CD123-expressing tumor cells and delivers the cytotoxic diphtheria toxin to the cells, resulting in the blockage of protein synthesis in the cell and causing cell death in CD123-expressing cells

NS-050/NCNP-03

- Treatment for Duchenne muscular dystrophy -

Development Phase	Global : P1/2
Origin	Co-development : National Center of Neurology and Psychiatry
Development	Nippon Shinyaku
Mechanism of action	Exon 50 Skipping
Indication	Duchenne muscular dystrophy
Dosage form	Injection
Feature	・Improvement in symptoms and prevention of the disease progression by recovery of dystrophin protein expression ・Morpholino based oligonucleotide with possible high safety profile and maximized activity

ATSN-101

- Treatment for GUCY2D-associated Leber congenital amaurosis -

Development Phase	**US : P1/2**
Origin	**[Nov. 2024] Partnership for commercialization in U.S. Development and sales license agreement in Japan : Atsena Therapeutics, Inc.**
Development	**Atsena Therapeutics, Inc.**
Mechanism of action	**GUCY2D Gene therapy**
Indication	**GUCY2D-associated Leber congenital amaurosis (LCA1)**
Dosage form	**Injection**
Feature	**• A first-in-class, investigational gene therapy for the treatment of LCA1 • A gene therapy using adeno-associated virus (AAV) 5, incorporating the human GUCY2D gene into the AAV5 vector. • Subretinal administration to express the normal GUCY2D gene and restore photoreceptor function.**

RGX-111

- Treatment for Mucopolysaccharidosis Type Ⅰ -

Development Phase	Global : P1/2
Origin	[Jan. 2025] Partnership for commercialization in U.S., Japan and other Asian countries : REGENXBIO Inc.
Development	REGENXBIO Inc.
Mechanism of action	Alpha-L-iduronidase Gene therapy
Indication	Mucopolysaccharidosis Type Ⅰ
Dosage form	Injection
Feature	・An investigational gene therapy using adeno-associated virus (AAV) 9 to deliver the alpha-L-iduronidase (IDUA) gene to the central nervous system using intracisternal or intraventricular administration ・Delivery of the IDUA gene within the cells in the central nervous system could provide a permanent source of secreted IDUA beyond the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS ・With a single dose, potential to prevent the progression of cognitive deficits

NS-917 (radgocitabine)

- Treatment for relapsed or refractory acute myeloid leukemia -

Development Phase	Japan : P1
Origin	[Mar. 2017] Licensed-in from : Delta-Fly Pharma, Inc.
Development	Nippon Shinyaku
Mechanism of action	DNA strand-break by incorporating itself into DNA
Indication	relapsed or refractory (r/r) acute myeloid leukemia (AML)
Dosage form	Injection
Feature	・Significant anti-leukemic activity with unique mechanism of action from other nucleoside analogs at low dose continuous infusion ・Tolerable safety profile available to elderly patients with r/r AML

NS-025

- Treatment for urological diseases -

Development Phase	Japan : P1
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	–
Indication	Urological diseases (to be determined)
Dosage form	Oral agent
Feature	–

NS-863

- Treatment for cardiovascular diseases -

Development Phase	Japan : P1
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	–
Indication	Cardiovascular diseases (to be determined)
Dosage form	Oral agent
Feature	–

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