FY2025 First Quarter Financial Results

August 7, 2025
NIPPON SHINYAKU CO., LTD.

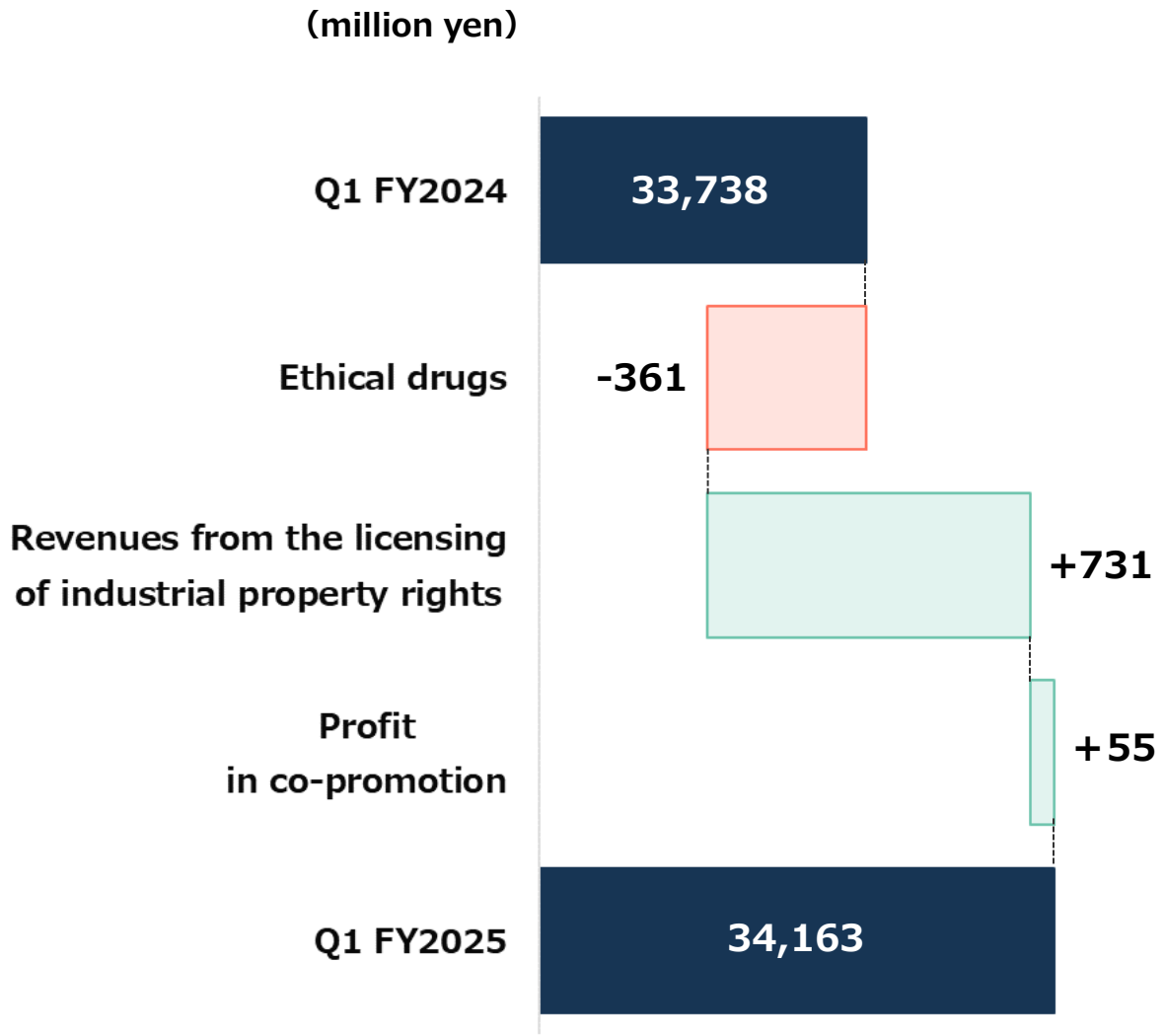
NIPPON SHINYAKU CO., LTD.

NIPPON SHINYAKU CO., LTD.

Q1 FY2025 Summary (consolidated)

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

Segmental Review - Pharmaceuticals -



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

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

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

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

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

- ✓ Launch of Yuvanci

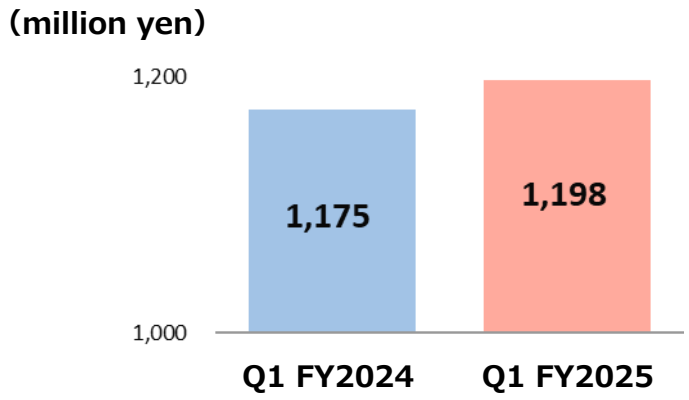
Sales Trends of Viltepso (viltolarsen)

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

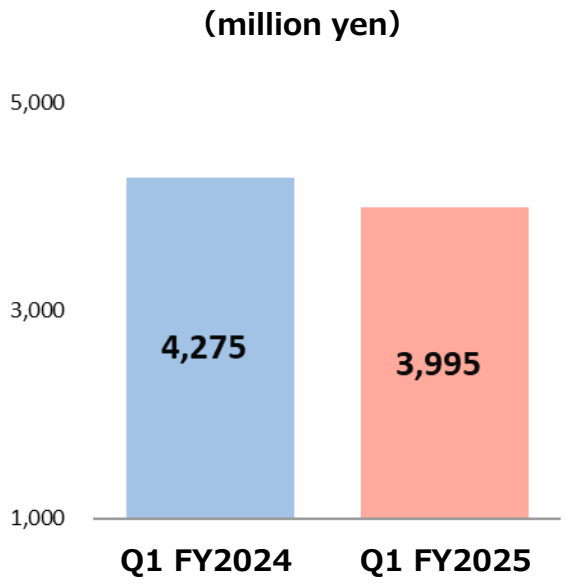
1. Central Social Insurance Medical Council

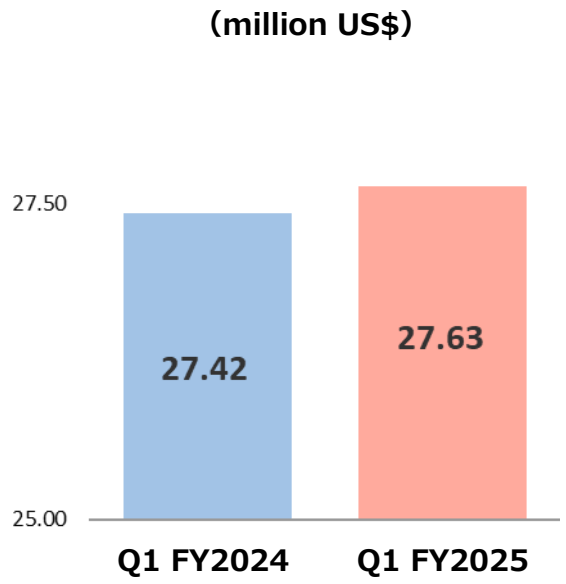
Exchange rates	Q1 FY2024 actual	Q1 FY2025 actual	FY2025 forecast
USDJPY	**155.9**	**144.6**	**140.0**

Japan

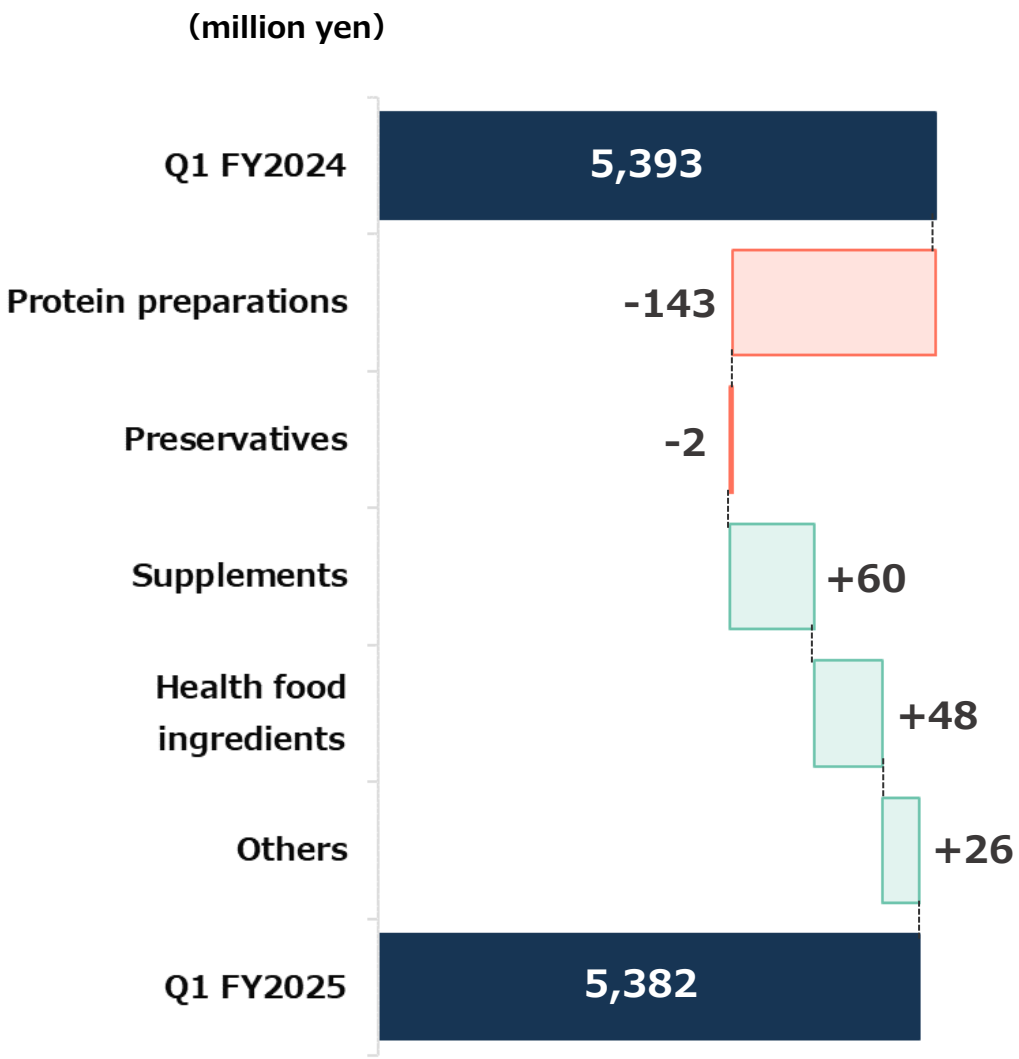


U.S.





Segmental Review - Functional Food -



Protein preparations 3,319 million yen
(-143 million yen, - 4.2%, YoY)

- ✓ Sales prices decline of protein preparations for the processed food industry
- ✓ Decrease in demand for protein due to customers switching to cheaper raw materials

Preservatives 797 million yen
(-2 million yen, -0.4%, YoY)

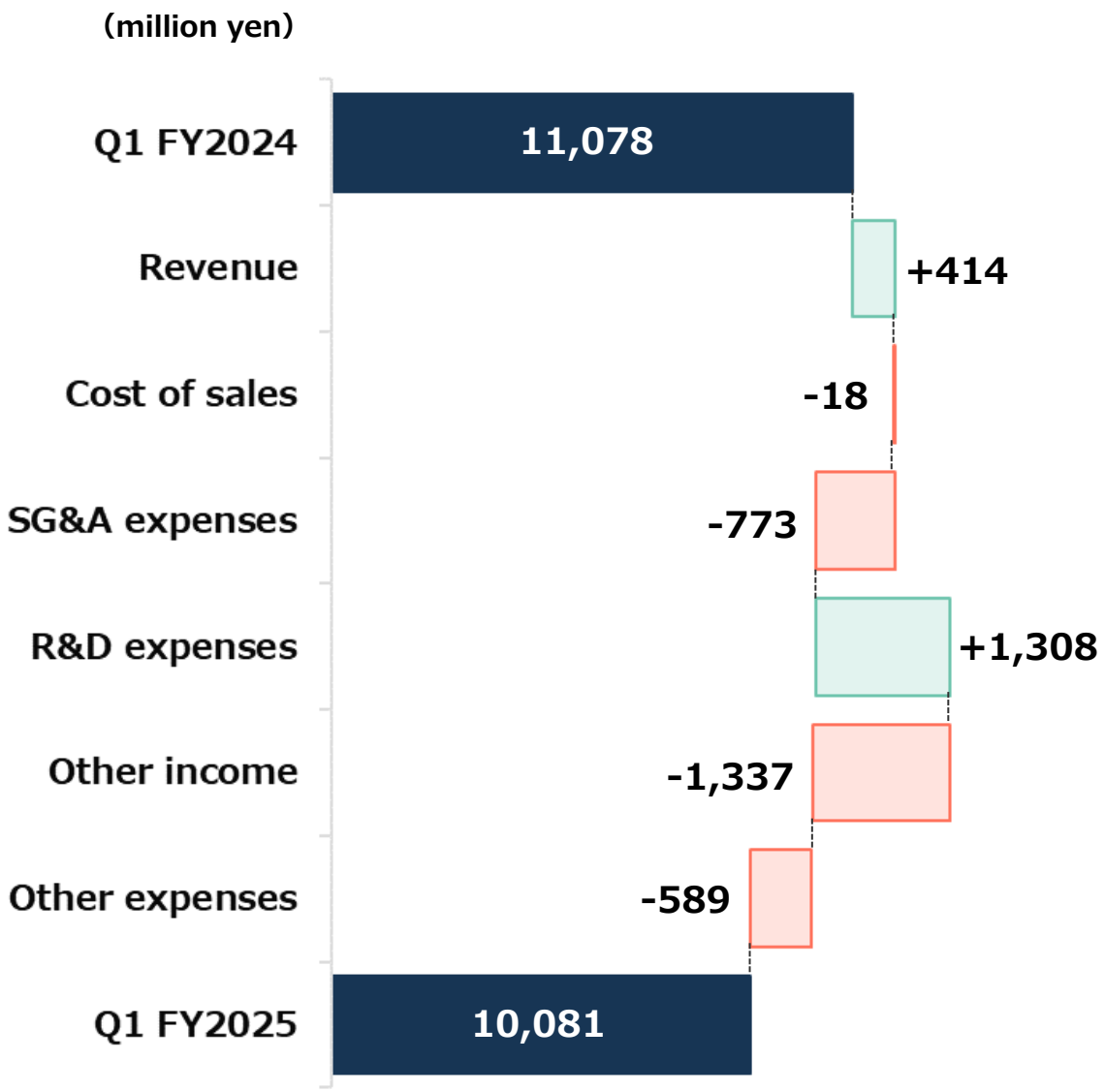
- ✓ Promoting consultative sales to key users

Supplements 612 million yen
(+60 million yen, +10.9%, YoY)

- ✓ Sales increase in both athletes and anti-aging care category

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

Operating Profit



Revenue 39,546 million yen
(+414 million yen, +1.1%, YoY)

- ✓ Sales increase of Vyxeos, Fintepla and Uptravi, etc.
- ✓ Increase in royalty income from Uptravi's overseas sales

Cost of sales 12,655 million yen
(+18 million yen, +0.1%, YoY)
The ratio was 32.0%, improved by 0.3% points YoY.

- ✓ Increase in revenues from industrial property rights
- ✓ Change in product mix of Pharmaceuticals segment

SG&A expenses 9,995 million yen
(+773million yen, + 8.4%, YoY)

- ✓ Increase in the U.S. sales expenses of NS Pharma
- ✓ Increase in commission for promotional activities of Uptravi due to domestic sales increase

R&D expenses 6,189 million yen
(-1,308 million yen, -17.4%, YoY)

- ✓ Decrease in raw material costs related to investigational products

Revised Business Forecast for FY2025 (consolidated)

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

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

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

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

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

Revised Business Forecast for FY2025 (consolidated)

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

Foreign exchange rates (USDJPY)

Q1 FY2024 actual	Q1 FY2025 actual	FY2025 forecast
155.9	144.6	140.0

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

Revenue Forecast – Pharmaceuticals Segment -

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

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

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

Revenue Forecast - Functional Food Segment-

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

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

R&D Updates

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

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

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

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

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

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

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

CAP-1002 (deramiocel) update

July 11, 2025

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

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

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

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

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

- ✓ **FDA issued a Complete Response Letter (CRL)[1] for Capricor's BLA[2] for CAP-1002 (deramiocel).**
- ✓ **Capricor has requested a Type A meeting[3] to determine the path forward toward potential approval.**

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

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

Reference Materials

Sales By Product in Pharmaceutical Segment

(million yen)

Brand name/ code no.	Indications	Q1 FY2024 actual	Q1 FY2025 actual	YoY change	YoY %	FY2025 forecast
Viltepso	Duchenne muscular dystrophy (DMD)	5,450	5,194	-256	-4.7%	21,500
(Japan)		(1,175)	(1,198)	(+23)	(+2.0%)	(4,800)
(U.S.)		(4,275)	(3,995)	(-279)	(-6.5%)	(16,700)
Uptravi	pulmonary arterial hypertension/ chronic thromboembolic pulmonary hypertension	3,855	4,375	+520	+13.5%	16,800
Vyxeos	high-risk AML	847	1,447	+599	+70.7%	7,300
Gazyva	CD20-positive follicular lymphoma/ CD20-positive chronic lymphocytic leukemia	1,254	1,180	-74	-5.9%	5,200
Vidaza	myelodysplastic syndrome/ acute myeloid leukemia	1,462	1,022	-440	-30.1%	3,100
Fintepla	seizures associated with Dravet syndrome/ seizures associated with Lennox-Gastaut syndrome	302	887	+585	+193.6%	4,000
Defitelio	sinusoidal obstruction syndrome	709	650	-58	-8.3%	2,500
Tramal/Onetram	cancer pain, chronic pain	778	571	-206	-26.6%	2,000
Cialis	erectile dysfunction	647	557	-89	-13.9%	2,500
CAP-1002 deramiocel (U.S.)	DMD cardiomyopathy	-	-	-	-	-
Profit in co-promotion		2,461	2,517	+55	+2.3%	9,600
Revenues from the licensing of industrial property rights		10,779	11,511	+731	+6.8%	47,500
Revenue		33,738	34,163	+425	+1.3%	143,000

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

Sales by Product Group in Functional Food Segment

(million yen)	Q1 FY2024		Q1 FY2025		YoY		FY2025
	actual	ratio	actual	ratio	change	%	forecast
Protein preparations	3,463	64.2%	3,319	61.7%	-143	-4.2%	13,900
Preservatives	800	14.8%	797	14.8%	-2	-0.4%	3,400
Supplements	552	10.3%	612	11.4%	+60	+10.9%	3,500
Health food ingredients	260	4.8%	309	5.7%	+48	+18.7%	1,100
Others	316	5.9%	343	6.4%	+26	+8.4%	1,100
Revenue	5,393	100.0%	5,382	100.0%	-11	-0.2%	23,000

Consolidated Balance Sheet

(million yen)	End of Q1 FY2024	End of Q1 FY2025	YoY change		End of Q1 FY2024	End of Q1 FY2025	YoY change
Assets	283,637	289,625	+5,987	Liabilities	36,297	37,791	+1,493
Current assets	149,740	157,667	+7,927	Current liabilities	30,316	31,964	+1,648
Non-current assets	133,897	131,957	-1,939	Non-current liabilities	5,980	5,826	-154
				Equity	247,340	251,834	+4,493
Total aseets	283,637	289,625	+5,987	Total liabilities and equity	283,637	289,625	+5,987

Assets

Cash and cash equivalents	+3,036
Inventories	+3,199
Other financial assets (non-current)	-1,980

Liabilities and Equity

Trade and other payables	+1,335
Retained earnings	+5,897

Pipeline（1/2）

Stage	Code No. (Generic name)	Origin	Application type	Indications	Schedule	Country
Launch P3	NS-065/NCNP-01 (viltolarsen)	Co-development with National Center of Neurology and Psychiatry	NME	Duchenne muscular dystrophy	—	Japan/U.S.
Filed	CAP-1002 (deramiocel)	Partnership Capricor Therapeutics, Inc.	NME	Duchenne muscular dystrophy cardiomyopathy	—	U.S.
Filed	NS-401 (tagraxofusp)	In-license The Menarini Group	NME	blastic plasmacytoid dendritic cell neoplasm	Study Completion : FY2026	Japan
Filed	RGX-121 (clemidsogene lanparvovec)	Partnership REGENXBIO Inc.	NME	Mucopolysaccharidosis Type II	PDUFA date[1] Nov 9, 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	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.

1. PDUFA date : the target action date for completion of the review by the FDA

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 : FY2025	Japan
	NS-580	In-house	NME	endometriosis	Temporarily suspended	Japan
				chronic prostatitis/ chronic pelvic pain syndrome	Temporarily suspended	Japan
	NS-089/NCNP-02 (brogidirsen)	Co-development with National Center of Neurology and Psychiatry	NME	Duchenne muscular dystrophy	Study Completion : FY2026	Japan/U.S.
	NS-229	In-house	NME	eosinophilic granulomatosis with polyangiitis	Study Completion : FY2026	Japan/U.S.
P1/2	NS-050/NCNP-03	Co-development with National Center of Neurology and Psychiatry	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 : FY2026	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 : Launched U.S. : Launched 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
Indications	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

CAP-1002 (deramiocel)

- Treatment for Duchenne muscular dystrophy cardiomyopathy-

Development Phase	**U.S. : P3 (Duchenne muscular dystrophy)** **U.S. : BLA Filed (Duchenne muscular dystrophy cardiomyopathy)**
Origin	**[Jan. 2022] Partnership for commercialization in the 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**
Indications	**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.**

NS-401 (tagraxofusp)

- Treatment for blastic plasmacytoid dendritic cell neoplasm -

Development Phase	**Japan : Filed**
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**
Indications	**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.**

RGX-121 (clemidsogene lanparvovec)

- Treatment for Mucopolysaccharidosis Type Ⅱ -

Development Phase	U.S. : BLA Filed
Origin	[Jan. 2025] Partnership for commercialization in the U.S., Japan and other Asian countries : REGENXBIO Inc.
Development	REGENXBIO Inc.
Mechanism of action	Iduronate-2-sulfatase Gene therapy
Indications	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 • Delivery of the IDS gene within the cells in the central nervous system could provide a permanent source of secreted IDS beyond the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS • One-time administration of RGX-121 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 : Launched (seizures associated with Dravet syndrome) Japan : Launched (seizures associated with 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
Indications	seizures associated with Dravet syndrome seizures associated with Lennox-Gastaut syndrome CDKL5 deficiency disorder
Dosage form	Oral liquid agent
Feature	• Effective for seizures associated with Dravet syndrome, seizures associated with 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
Indications	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	Japan : Launched (for patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors) Japan : 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
Indications	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-304 (selexipag)

- Treatment for arteriosclerosis obliterans -

Development Phase	**Japan : P2b（ASO）**
Origin	**Nippon Shinyaku**
Development	**Nippon Shinyaku**
Mechanism of action	**Selective IP receptor agonist**
Indications	**arteriosclerosis obliterans (ASO)**
Dosage form	**Tablet**
Feature	**Long-acting oral drug**

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
Indications	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
Indications	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
Indications	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-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**
Indications	**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 the U.S. Development and sales license agreement in Japan : Atsena Therapeutics, Inc.**
Development	**Atsena Therapeutics, Inc.**
Mechanism of action	**GUCY2D Gene therapy**
Indications	**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 the U.S., Japan and other Asian countries : REGENXBIO Inc.
Development	REGENXBIO Inc.
Mechanism of action	Alpha-L-iduronidase Gene therapy
Indications	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 • One-time administration of RGX-111 is expected to lead to sustained production of IDUA leading to the attenuation of CNS manifestations in MPS I patients

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**
Indications	**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	–
Indications	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	–
Indications	Cardiovascular diseases (to be determined)
Dosage form	Oral agent
Feature	–

Safe Harbor Statement

- Materials and information provided during this presentation may contain so-called “forward-looking statements.” These statements are based on current expectations, forecasts and assumptions that are subject to risks and uncertainties which could cause actual outcomes and results to differ materially from these statements.

- Risks and uncertainties include general industry and market conditions, and general domestic and international economic conditions such as interest rate and currency exchange fluctuations. Risks and uncertainties particularly apply with respect to product-related forward-looking statements. Product risks and uncertainties include, but are not limited to, technological advances and patents attained by competitors; challenges inherent in new product development, including completion or failure of clinical trials; claims and concerns about product safety and efficacy; regulatory agency’s examination, obtaining regulatory approvals; domestic and foreign social security reforms; trends toward healthcare cost containment; and governmental laws and regulations affecting domestic and foreign operations.

- Also, for products that are approved, there are manufacturing and marketing risks and uncertainties, which include, but are not limited to, inability to build production capacity to meet demand, unavailability of raw materials, and competition with others.

- The Company disclaims any intention or obligation to update or revise any forward-looking statements whether as a result of new information, future events or otherwise.

- This English presentation was translated from the original Japanese version. In the event of any inconsistency between the Japanese original and the English translation, the former shall prevail.

NIPPON SHINYAKU CO., LTD.