FY2024 Financial Results

May 8, 2025
NIPPON SHINYAKU CO., LTD.

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Agenda

01 FY2024 Financial Results and FY2025 Forecasts

02 Update on the 7th Five-Year Medium-Term Management Plan (FY2024 - FY2028)

03 R&D Updates

FY2024 Financial Results and FY2025 Forecasts

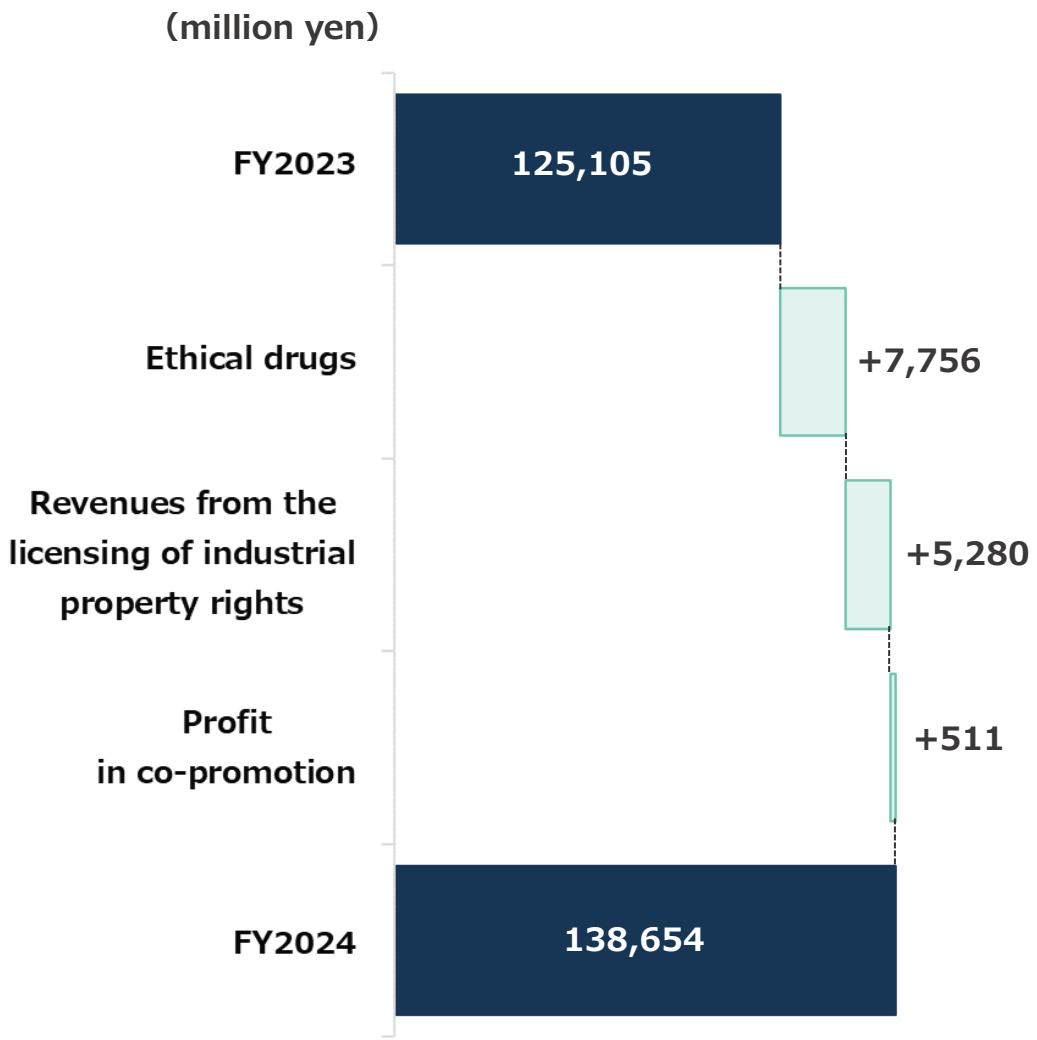
Toru Nakai

Representative Director, President

FY2024 Summary (consolidated)

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

Segmental Review - Pharmaceuticals -



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

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

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

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

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

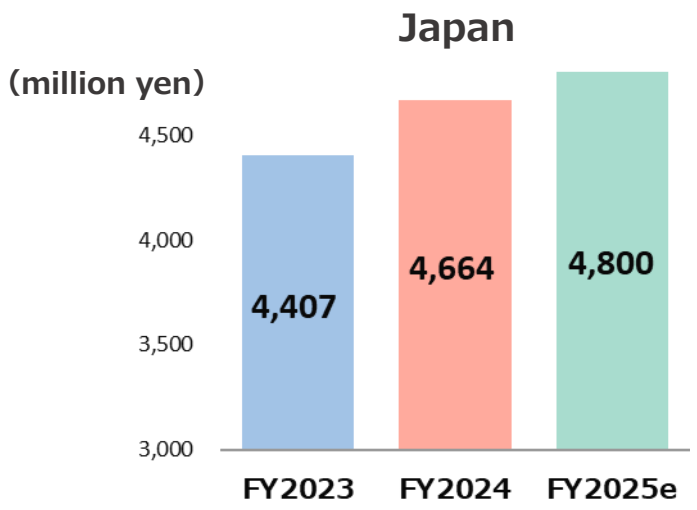
- ✓ Sales growth of Opsumit
- ✓ Launch of Yuvanci in November 2024

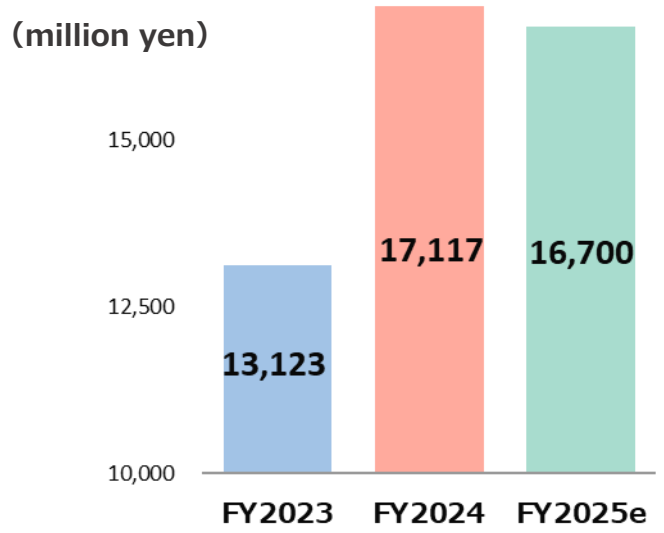
Sales Trends of Viltepso® (viltolarsen)

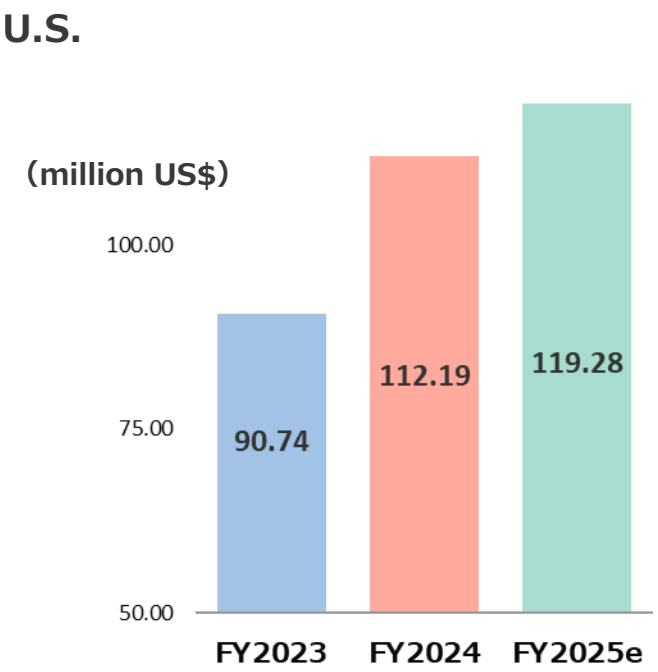
(million yen)	FY2023 actual	FY2024 actual	YoY change	YoY %	FY2025 forecast	Notes on FY2024 results
Japan	**4,407**	**4,664**	**+257**	**+5.8%**	**4,800**	✓ The number of patients currently being administered is more than three-quarters of the peak number of 128 patients in the data from Chuikyo[1]. ✓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

1. Central Social Insurance Medical Council

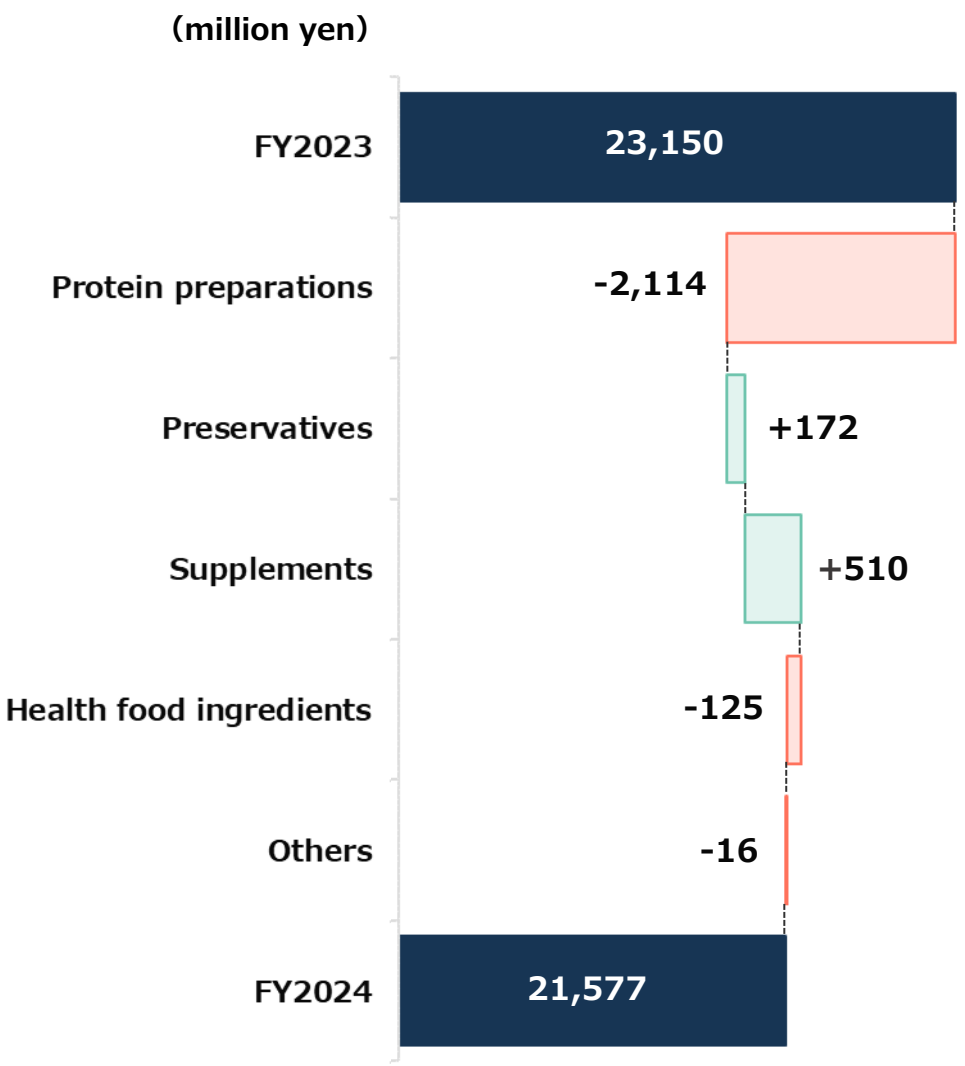
Exchange rates	FY2023 actual	FY2024 actual	FY2025 forecast
USDJPY	**144.6**	**152.6**	**140.0**







Segmental Review - Functional Food -



Protein preparations 13,485 million yen
(-2,114 million yen, -13.6%, YoY)

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

Preservatives 3,278 million yen
(+172 million yen, + 5.6%, YoY)

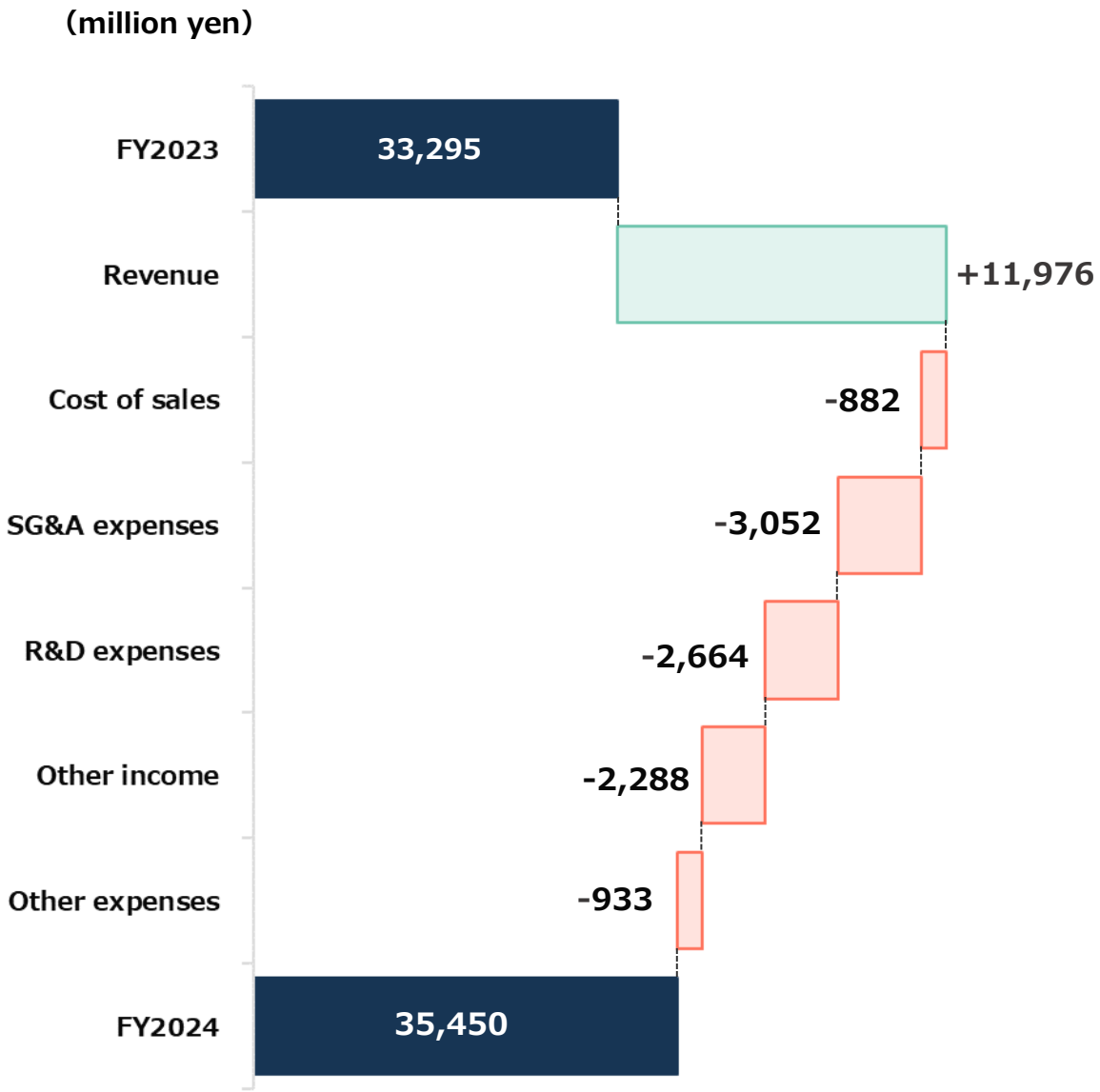
- ✓ Recovery of food service and tourism industries

Supplements 2,415 million yen
(+ 510 million yen, + 26.8%, YoY)

- ✓ New demand increased from young people and seniors, especially athletes
- ✓ Growth in the anti-aging care category helped by electronic commerce sales measures

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

Operating Profit



Revenue 160,232 million yen
(+ 11,976 million yen, + 8.1%, YoY)

- ✓ Sales increase of Viltepso, Uptravi, and new product Vyxeos
- ✓ Increase in royalty income from Uptravi's overseas sales

Cost of sales 51,116 million yen
(+882 million yen, + 1.8%, YoY)
The ratio was 31.9%, improved by 2.0% points YoY.

- ✓ Cost of sales ratio improvement due to factors such as revenues from industrial property rights and the change in sales segment mix (pharma vs. food)

SG&A expenses 38,011 million yen
(+ 3,052 million yen, + 8.7%, YoY)

- ✓ Increase in the U.S. sales expenses for launch preparations
- ✓ Increase in domestic sales division expenses due to the launch of Vyxeos and Jaypirca
- ✓ Increase in commission for promotional activities of Uptravi due to domestic sales increase

R&D expenses 34,341 million yen
(+ 2,664 million yen, + 8.4%, YoY)

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

Business Forecast for FY2025 (consolidated)

(million yen)	FY2024 actual	FY2024 ratio	FY2025 forecast	FY2025 ratio	YoY change	YoY %
Revenue	**160,232**	**100.0%**	**173,000**	**100.0%**	**+12,768**	**+8.0%**
(Pharmaceuticals)	(138,654)	(86.5%)	(150,000)	(86.7%)	(+11,346)	(+8.2%)
(Functional Food)	(21,577)	(13.5%)	(23,000)	(13.3%)	(+1,423)	(+6.6%)
Cost of sales	**51,116**	**31.9%**	**55,200**	**31.9%**	**+4,084**	**+8.0%**
SG&A expenses	**38,011**	**23.7%**	**47,000**	**27.2%**	**+8,989**	**+23.6%**
R&D expenses	**34,341**	**21.4%**	**39,500**	**22.8%**	**+5,159**	**+15.0%**
Other income	**874**	**0.5%**	**600**	**0.3%**	**-274**	**-31.4%**
Other expenses	**2,186**	**1.4%**	**1,900**	**1.1%**	**-286**	**-13.1%**
Operating profit	**35,450**	**22.1%**	**30,000**	**17.3%**	**-5,450**	**-15.4%**
Finance income	**830**	**0.5%**	**700**	**0.4%**	**-130**	**-15.7%**
Finance costs	**145**	**0.0%**	**100**	**0.1%**	**-45**	**-31.2%**
Profit before tax	**36,135**	**22.6%**	**30,600**	**17.7%**	**-5,535**	**-15.3%**
Income tax expense, etc.	**3,577**	**2.3%**	**6,600**	**3.8%**	**+3,023**	**+84.5%**
Profit attributable to owners of parent	**32,558**	**20.3%**	**24,000**	**13.9%**	**-8,558**	**-26.3%**

Foreign exchange rates (USDJPY)

FY2024 actual	FY2025 forecast
152.6	**140.0**

Reasons for increased expenses in FY2025

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

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

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

Revenue Forecast – Pharmaceuticals Segment -

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

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

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

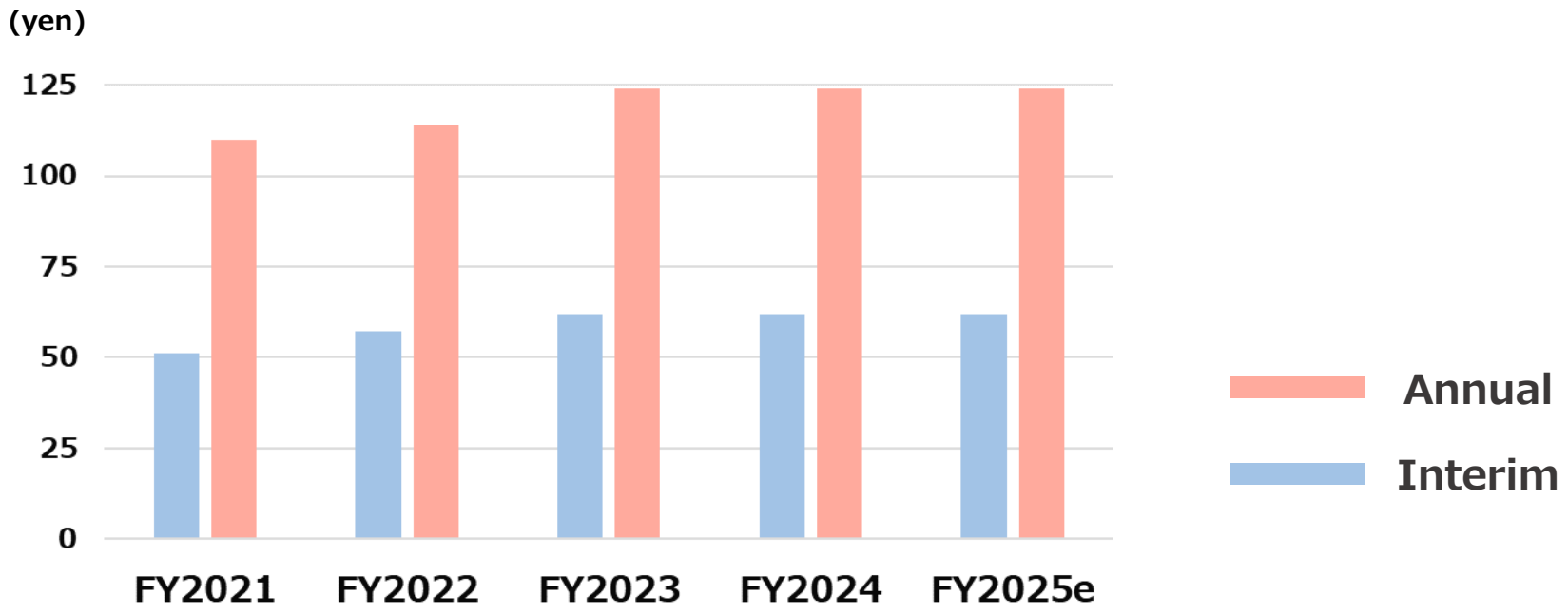
Revenue Forecast - Functional Food Segment-

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

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

Dividends Forecast

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



Update on the 7th Five-Year Medium-Term Management Plan (FY2024 - FY2028)

Toru Nakai

Representative Director, President

Overview of the 7th Medium-Term Management Plan

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

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

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

Targets in FY2028

Revenue 230 billion yen
EPS 341 yen

Operating profit 30 billion yen
ROE 8% or more
ROIC 9% or more

Three Key Themes

I Fostering growth drivers to replace Uptravi

II Expanding global development

III Continuous pipeline expansion

Strengthening five management foundations

① Promoting sustainable management for realizing sustainable society

② Speeding up R&D

③ Promoting human capital management that allows each employee to grow and diverse human resources to play an active role

④ Business process reengineering and productivity improvement by promoting digitalization

⑤ Financial strategy for sustainable growth

Three Key Themes : First Year Review

I. Fostering growth drivers to replace Uptravi

- Launched Vyxeos and Jaypirca for the treatment of blood cancer and Yuvanci for the treatment of pulmonary arterial hypertension (PAH)
- Uptravi was approved for the additional indication of pediatric PAH and a pediatric formulation is launched (as a part of PLCM initiatives)
- Expanded omni-channel sales initiatives utilizing field activities and digital channels to promote early market penetration of new products

II. Expanding Global development

- BLA filing accepted by FDA for CAP-1002 (DMD-cardiomyopathy), expected to be launched in the U.S. during FY2025
- Expansion of in-house sales structure for the launch of CAP-1002 and RGX-121 in the U.S.
- Multiple options, including organic expansion, alliances, and M&A, are being considered to build sales structure in Europe.

III. Continuous Pipeline Expansion

- Continuously expanded pipeline based on the three pillars of in-house drug discovery, in-licensing, and PLCM
- Utilization of novel drug discovery modalities from open innovation through a research collaboration agreement with MiNA Therapeutics of the U.K.
- Acquired rights for ATSN-101 and RGX-121/111. Aiming at least one new in-licensed item per year in the MT plan period

Our Target for New Product Launch

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

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

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

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

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

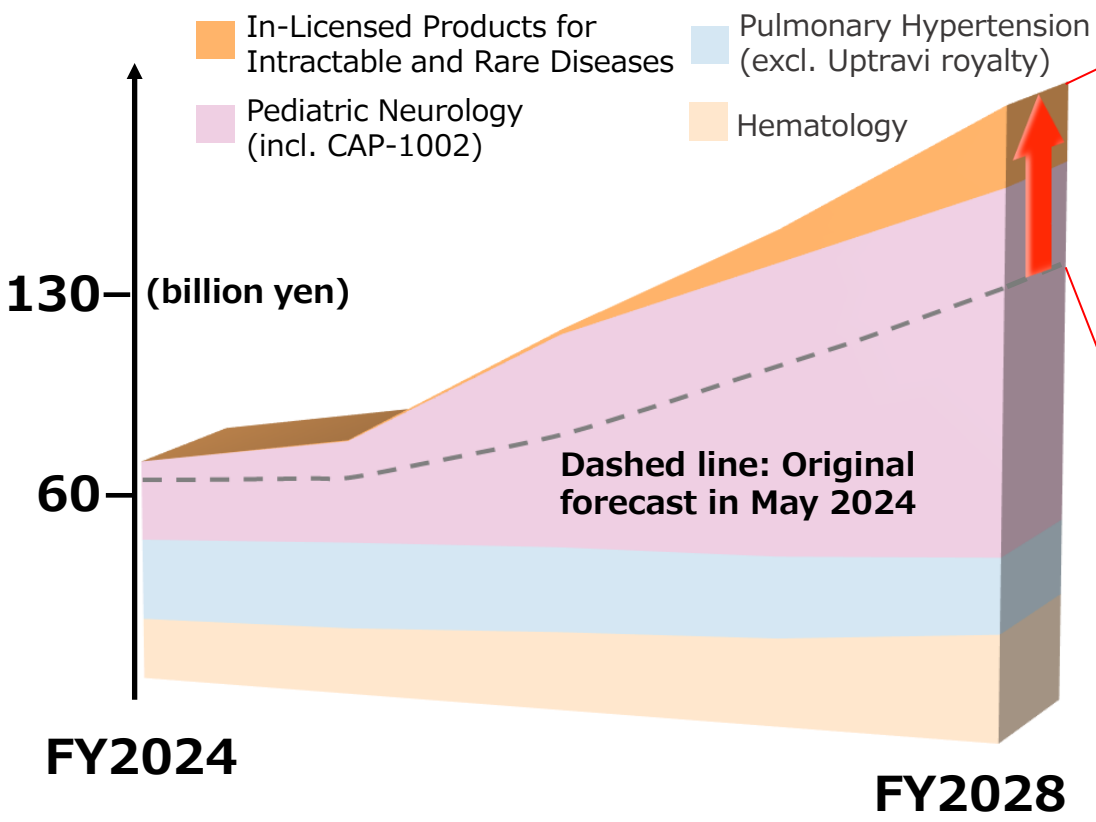
Note:NS-051/NCNP-04 (Japan and U.S.) and NS-065/NCNP-01 (Europe and China) are active programs but are currently under ongoing discussions with regulatory authorities. The year of market launch for these products has not yet been determined.

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

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

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

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



Main factors contributing to updated revenue forecast

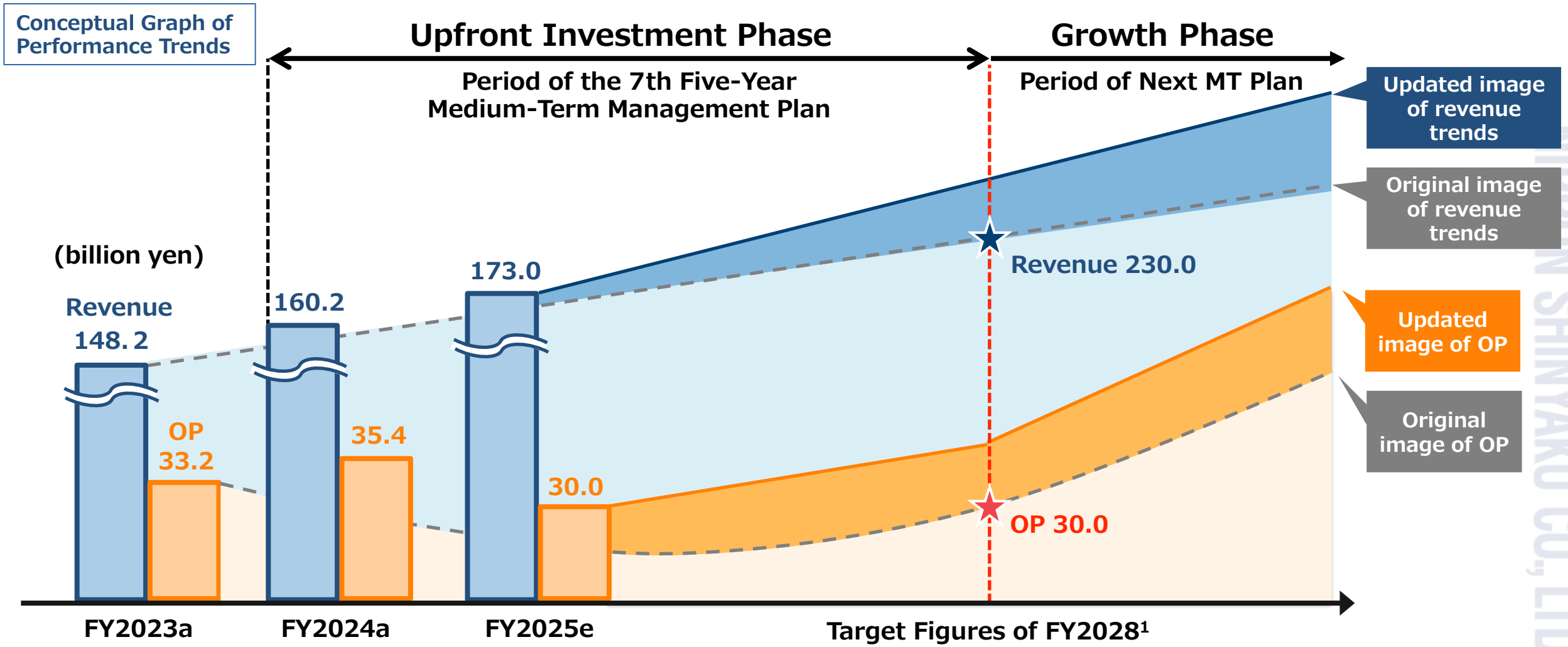
- **Increased revenue from new in-licensed products in the intractable and rare disease field (ATSN-101 and RGX-121/111)**
- **The Expected launch date for CAP-1002, a cell therapy drug, has been brought forward, and an expansion of patient population due to expected indication for DMD-cardiomyopathy.**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

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

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

Establishing Growth Foundation to Overcome Patent Cliff

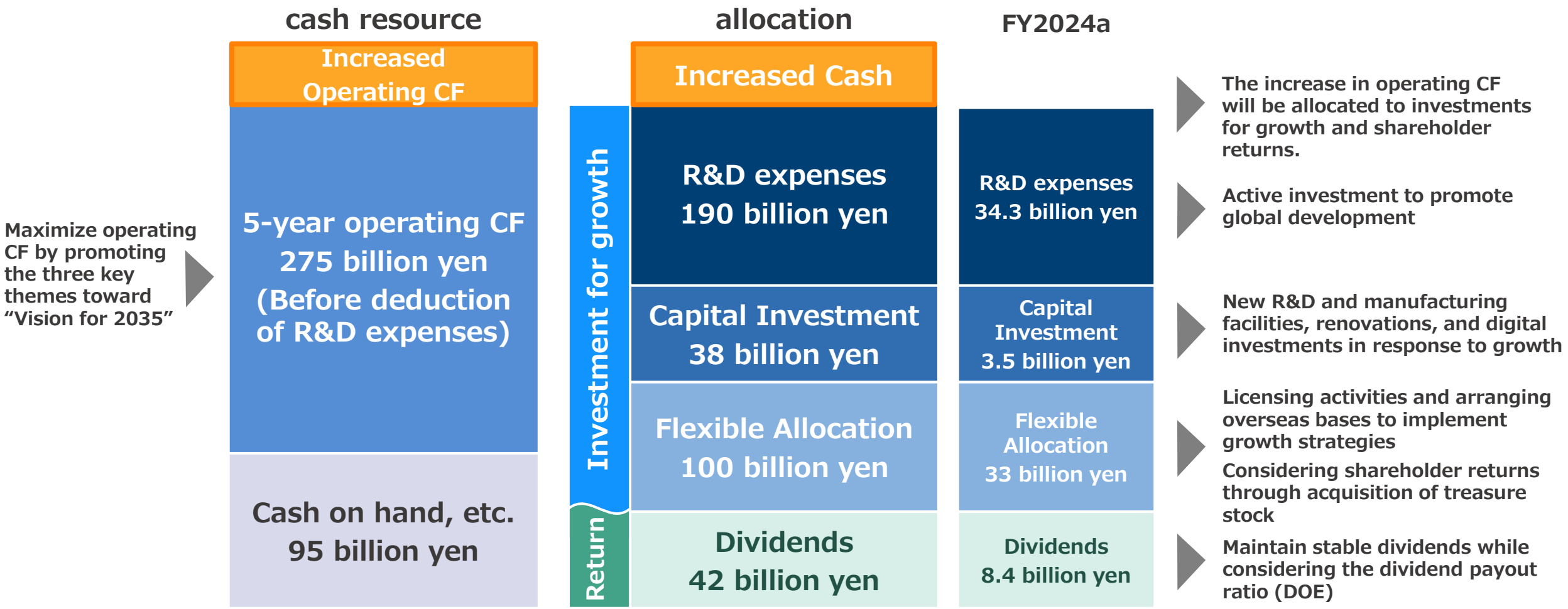
Operating profit is expected to increase from FY2025 onwards due to the earlier launch of CAP-1002 and the faster growth of new products.



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

Financial Strategy

Develop a capital allocation and make strategic investments necessary for sustainable growth while ensuring financial soundness.



Modified from May 27, 2024
The 7th Five-Year Medium-Term Management Plan (FY2024 - FY2028) -For Global Growth Beyond the Cliff-, p.39

CAP-1002 (deramiocel) update

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

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

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

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

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

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

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

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

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

R&D Updates

Kazuchika Takagaki
Director, Research & Development

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

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

Recent status/event	Code No. (Generic name)	Product name	Indications and topics	Schedule
P3	NS-065/NCNP-01 (viltolarsen)	Viltepso	Currently waitng for the FDA's feedback on 1. Study 301 data 2. Protocol of Study 303	April 2025
Launch	NS-87 (daunorubicin / cytarabine)	Vyxeos	high-risk acute myeloid leukemia	May 2024
Launch	LY3527727 (piltobrutinib)	Jaypirca	patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors	August 2024
Launch	ACT-064992D (macitentan / tadalafil)	Yuvanci	pulmonary arterial hypertension	November 2024
Additional indication	NS-304 (selexipag)	Uptravi	pediatric pulmonary arterial hypertension	December 2024
Launch	NS-304 (selexipag)	Uptravi	Uptravi® Tablets for Pediatric 0.05 mg	March 2025
Filed (BLA[1] accepted by FDA)	CAP-1002 (deramiocel)	–	Duchenne muscular dystrophy cardiomyopathy	March 2025 (U.S.)
Filed (BLA[1] submitted and waiting for FDA acceptance)	RGX-121 (clemidsogene lanparvovec)	–	Mucopolysaccharidosis Type II	March 2025 (U.S.)
Filed	NS-401 (tagraxofusp)		blastic plasmacytoid dendritic cell neoplasm (BPDCN)	March 2025
Start of P2	NS-229	–	eosinophilic granulomatosis with polyangiitis	June 2024
Start of P1/ P2	NS-050/NCNP-03	–	Duchenne muscular dystrophy	October 2024

1. Biologics License Application

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

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

Recent status/event	Code No. (Generic name)	Product name	Indications and topics	Schedule
Letter of Intent signed (Capricor Therapeutics)	CAP-1002 (deramiocel)	–	executed a Letter of Intent stipulating the exclusive right to negotiate over the next few months an exclusive distribution agreement for CAP-1002 in Europe	September 2024 (Europe)
In-license agreement signed (Atsena Therapeutics)	ATSN-101	–	GUCY2D-associated Leber congenital amaurosis	November 2024 (U.S. and Japan)
In-license agreement signed (REGENXBIO Inc.)	RGX-121 (clemidsogene lanparvovec)	–	Mucopolysaccharidosis Type II	January 2025 (U.S. and Asia including Japan)
	RGX-111	–	Mucopolysaccharidosis Type I	
Option Agreement signed for Commercialization (AB2 BIO Ltd.)	Tadekinig alfa	–	NLRC4 mutation and XIAP deficiency	January 2025 (U.S.)
Preliminary analysis results	NS-065/NCNP-01 (viltolarsen)	Viltepso	global Phase 3 trial (RACER53 Study)	May 2024
Publication			the results of Phase 2 trial (Galactic53 trial) in Scientific Reports	October 2024
Rare Pediatric Disease Designation	NS-050/NCNP-03	–	Duchenne muscular dystrophy	August 2024 (U.S.)
Senkuteki Iyakuhin (Pioneering Drug) Designation and Orphan Drug Designation	NS-089/NCNP-02 (brogidirsen)	–	Duchenne muscular dystrophy	December 2024 (Japan)
Publication			the results of an investigator-initiated clinical trial (First in human trial) in Cell Reports Medicine	January 2025
Rare Pediatric Disease Designation	NS-051/NCNP-04	–	Duchenne muscular dystrophy	January 2025 (U.S.)
Orphan Drug Designation	NS-229	–	eosinophilic granulomatosis with polyangiitis (EGPA)	April 2025 (U.S.)

Reference Materials

Sales By Product in Pharmaceutical Segment

(million yen)

Brand name/ code no.	Indications	FY2023 actual	FY2024 actual	YoY change	YoY %	FY2025 forecast
Viltepso	Duchenne muscular dystrophy (DMD)	17,530	21,782	+4,251	+24.3%	21,500
(Japan)		(4,407)	(4,664)	(+257)	(+5.8%)	(4,800)
(U.S.)		(13,123)	(17,117)	(+3,994)	(+30.4%)	(16,700)
Uptravi	pulmonary arterial hypertension/ chronic thromboembolic pulmonary hypertension	12,918	14,971	+2,052	+15.9%	16,800
Vyxeos	high-risk AML	-	5,139	+5,139	-	7,300
Vidaza	myelodysplastic syndrome/ acute myeloid leukemia	10,383	5,109	-5,274	-50.8%	3,100
Gazyva	CD20-positive follicular lymphoma/ CD20-positive chronic lymphocytic leukemia	4,695	4,821	+125	+2.7%	5,200
Tramal/Onetram	cancer pain, chronic pain	3,927	2,728	-1,199	-30.5%	2,000
Cialis	erectile dysfunction	2,499	2,425	-73	-3.0%	2,500
Defitelio	sinusoidal obstruction syndrome	2,221	2,364	+142	+6.4%	2,500
Fintepla	Dravet syndrome Lennox-Gastaut syndrome	377	2,067	+1,689	+446.9%	4,000
CAP-1002 deramiocel (U.S.)	DMD cardiomyopathy	-	-	-	-	7,300
Profit in co-promotion		8,658	9,170	+511	+5.9%	9,600
Revenues from the licensing of industrial property rights		40,304	45,585	+5,280	+13.1%	47,500
Revenue		125,105	138,654	+13,549	+10.8%	150,000

The exchange rate assumed for FY2025 forecast is 1 USD=140 yen.
The sensitivity of the exchange rate is assumed to be an increase of approximately 530 million yen in revenue for every 1 yen depreciation of the yen.

Sales by Product Group in Functional Food Segment

(million yen)	FY2023		FY2024		YoY		FY2025
	actual	ratio	actual	ratio	change	%	forecast
Protein preparations	15,600	67.4%	13,485	62.5%	-2,114	-13.6%	13,900
Preservatives	3,105	13.4%	3,278	15.2%	+172	+5.6%	3,400
Supplements	1,905	8.2%	2,415	11.2%	+510	+26.8%	3,500
Health food ingredients	1,248	5.4%	1,122	5.2%	-125	-10.1%	1,100
Others	1,291	5.6%	1,276	5.9%	-14	-1.1%	1,100
Revenue	23,150	100.0%	21,577	100.0%	-1,572	-6.8%	23,000

Consolidated Balance Sheet

(million yen)	End of FY2023	End of FY2024	YoY change		End of FY2023	End of FY2024	YoY change
Assets	263,404	283,637	+20,233	Liabilities	42,870	36,297	-6,572
Current assets	164,285	149,740	-14,544	Current liabilities	37,336	30,316	-7,020
Non-current assets	99,119	133,897	+34,777	Non-current liabilities	5,533	5,980	+447
				Equity	220,534	247,340	+26,806
Total aseets	263,404	283,637	+20,233	Total liabilities and equity	263,404	283,637	+20,233

Assets

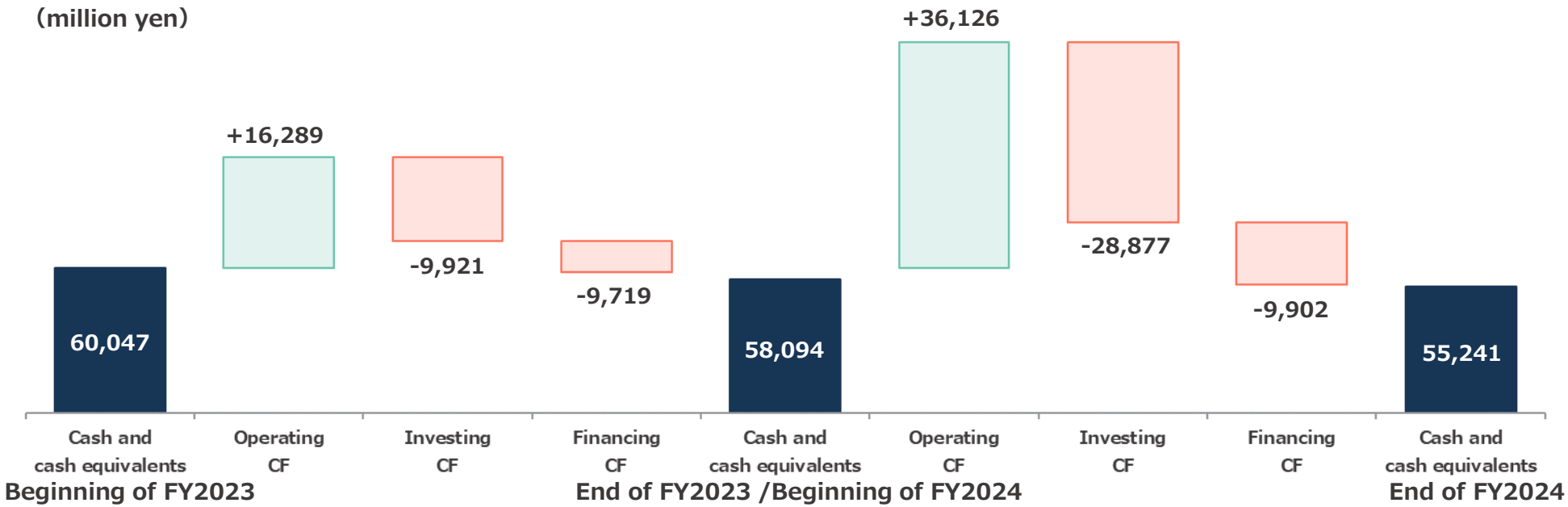
Trade and other receivables	-4,945
Intangible assets	+23,195
Other financial assets (non-current)	+6,630

Liabilities and Shareholders' Equity

Trade and other payables	-7,849
Retained earnings	+24,380

Consolidated Statements of Cash Flows

(million yen)	FY2023 actual	FY2024 actual	YoY change
Operating activities	16,289	36,126	+19,837
Investing activities	-9,921	-28,877	-18,955
Financing activities	-9,719	-9,902	-183
Cash and cash equivalents at end of period	58,094	55,241	-2,852



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.
Filed	CAP-1002 (deramiocel)	Partnership Capricor Therapeutics, Inc.	NME	Duchenne muscular dystrophy cardiomyopathy	Approval : CY2025 H2	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	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	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 : 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)	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-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 : 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
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

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
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.

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
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.

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
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 • 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 (Dravet syndrome) Japan : Launched（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	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
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-304 (selexipag)

- Treatment for pulmonary hypertension, arteriosclerosis obliterans -

Development Phase	Japan : P2b（ASO） Japan : Approved for the additional indication (Uptravi® tablets 0.2 mg and 0.4 mg for the of pediatric pulmonary arterial hypertension (PAH)) Japan : Launched (Uptravi® tablets for pediatric 0.05 mg)
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

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-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 the 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 the 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 • 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
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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