

FY2025 Financial Results

May 15, 2026
NIPPON SHINYAKU CO., LTD.

Agenda

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- **FY2025 Financial Results and FY2026 Forecast**
- **CAP-1002 Update**
- **RGX-121 Update**

Toru Nakai
**Representative Director,
President**

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- **R&D Pipeline**

Keiichi Kuwano
**Director,
Research & Development**

FY2025 Financial Results and FY2026 Forecast

Toru Nakai
Representative Director, President

Highlight

FY2025 Financial Results

- ✓ Increased revenue in both the pharmaceuticals and functional food businesses, marking the fourth consecutive period of revenue growth
- ✓ Increased operating profit, marking the third consecutive period of growth

FY2026 Full-Year Forecast

- ✓ Revenue: 200 billion yen (Up 17.1% YoY)
- ✓ Operating profit: 38 billion yen (Up 7.1% YoY)

FY2025 Summary

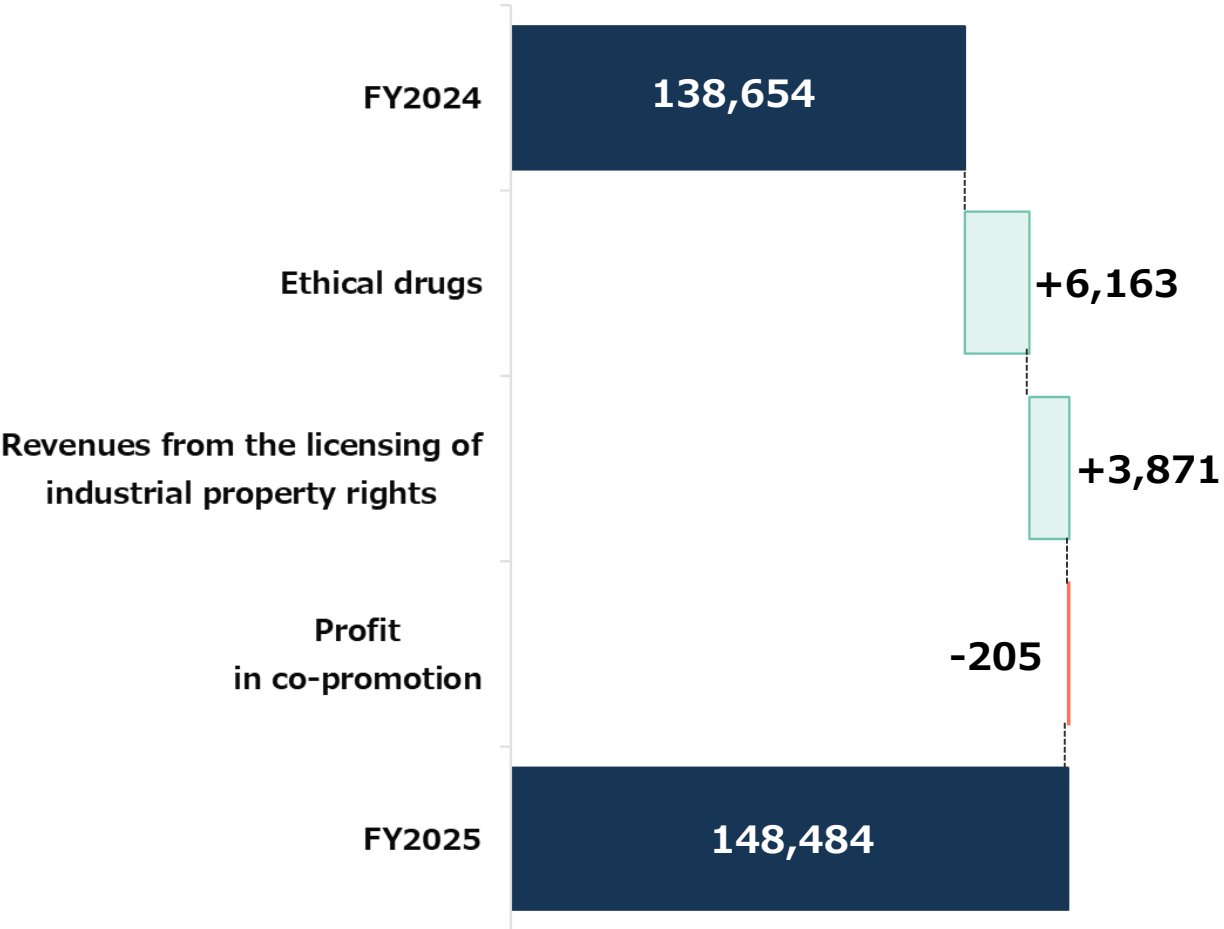
- Increased revenue in both the pharmaceuticals and functional food businesses, marking the fourth consecutive period of revenue growth
- Increased cost of sales and SG&A expenses led to a slight increase in operating profit. Profit attributable to owners of parent decreased due to changes in the tax rate.

(million yen)	FY2024		FY2025		YoY	
	actual	ratio	actual	ratio	change	%
Revenue	160,232	100.0%	170,771	100.0%	+10,539	+6.6%
(Pharmaceuticals)	(138,654)	(86.5%)	(148,484)	(86.9%)	(+9,829)	(+7.1%)
(Functional Food)	(21,577)	(13.5%)	(22,287)	(13.1%)	(+710)	(+3.3%)
Cost of sales	51,116	31.9%	57,460	33.6%	+6,343	+12.4%
SG&A expenses	38,011	23.7%	43,565	25.5%	+5,554	+14.6%
R&D expenses	34,341	21.4%	36,713	21.5%	+2,372	+6.9%
Other income	874	0.5%	3,025	1.8%	+2,151	+246.0%
(Foreign exchange gain)	-	-	(2,265)	(1.3%)	(+2,265)	-
Other expenses	2,186	1.4%	560	0.4%	-1,625	-74.3%
Operating profit	35,450	22.1%	35,496	20.8%	+46	+0.1%
Finance income	830	0.5%	1,132	0.7%	+301	+36.3%
Finance costs	145	0.1%	166	0.1%	+20	+14.1%
Profit before tax	36,135	22.6%	36,462	21.4%	+327	+0.9%
Income tax expense, etc.	3,577	2.2%	6,741	3.9%	+3,164	+88.5%
Profit attributable to owners of parent	32,558	20.3%	29,721	17.4%	-2,837	-8.7%

Segmental Review - Pharmaceuticals -

- Growth in domestic sales of Uptravi, Fintepla, etc., including the contribution of Erleada, and royalty income from overseas sales of Uptravi

(million yen)



Ethical drugs 90,062 million yen
 (+6,163 million yen, +7.3%, YoY)

- ✓ Growth of Uptravi and Fintepla
- ✓ Erleada's revenue has been recognized by the company through a reverse co-promotion arrangement.

Revenues from the licensing of industrial property rights
49,457 million yen
 (+3,871 million yen, +8.5%, YoY)

- ✓ Royalty revenue growth due to overseas sales increase of Uptravi

Profit in co-promotion 8,964 million yen
 (-205 million yen, -2.2%, YoY)

- ✓ Yuvanci's sales increased, but revenue recognition for Erleada has changed.

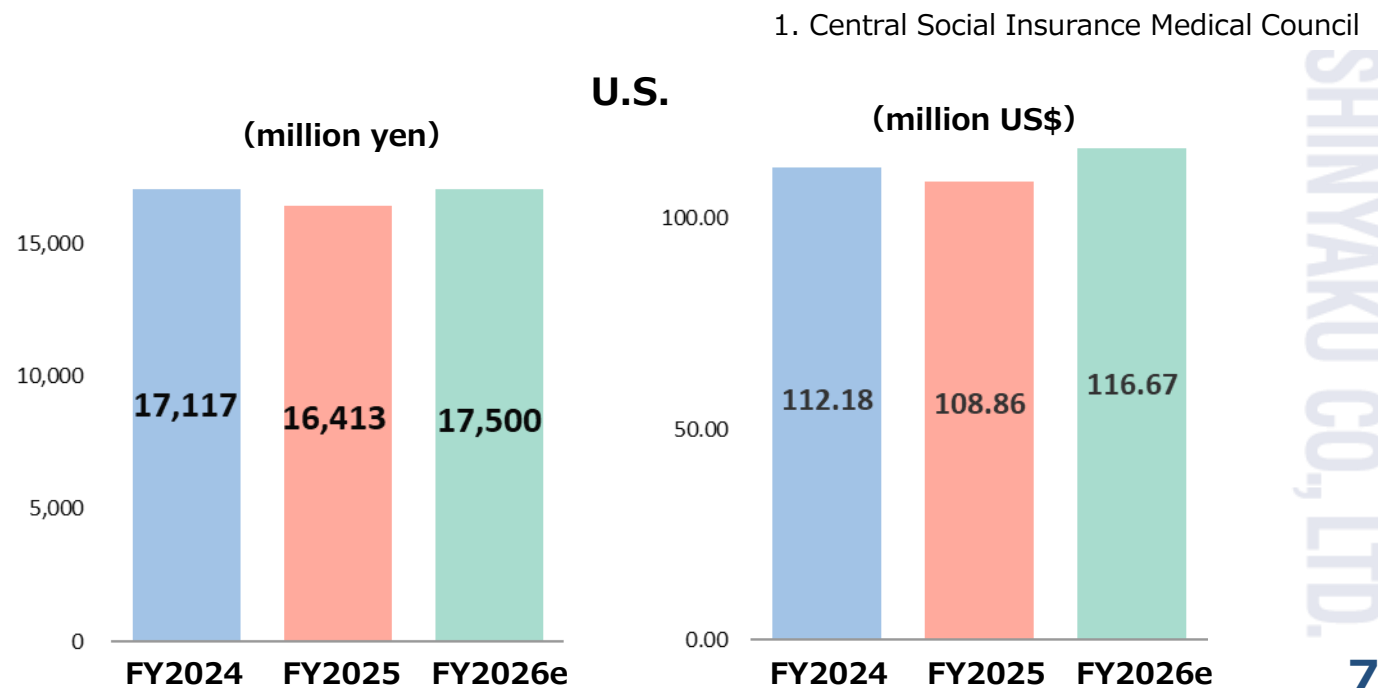
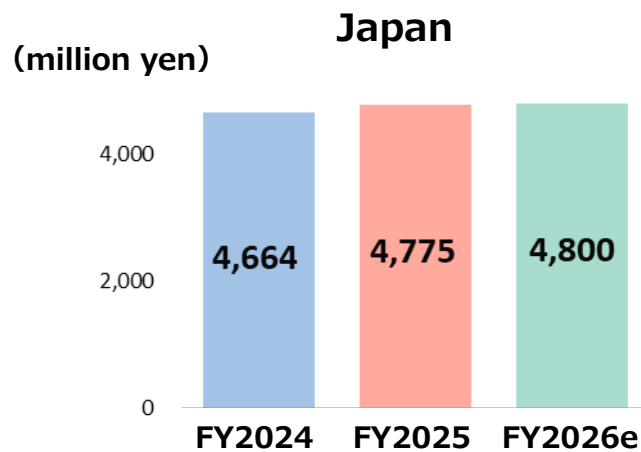
1. previously recorded as profit in co-promotion, but now recorded as product sales and cost of goods sold, based on contract revisions

Sales Trends of Viltepso® (viltolarsen)

- The average dose per patient has decreased due to younger patient population, resulting sales decline of Viltepso in the U.S. However, sales growth is expected due to an increase in the number of new patients and longer-term administration

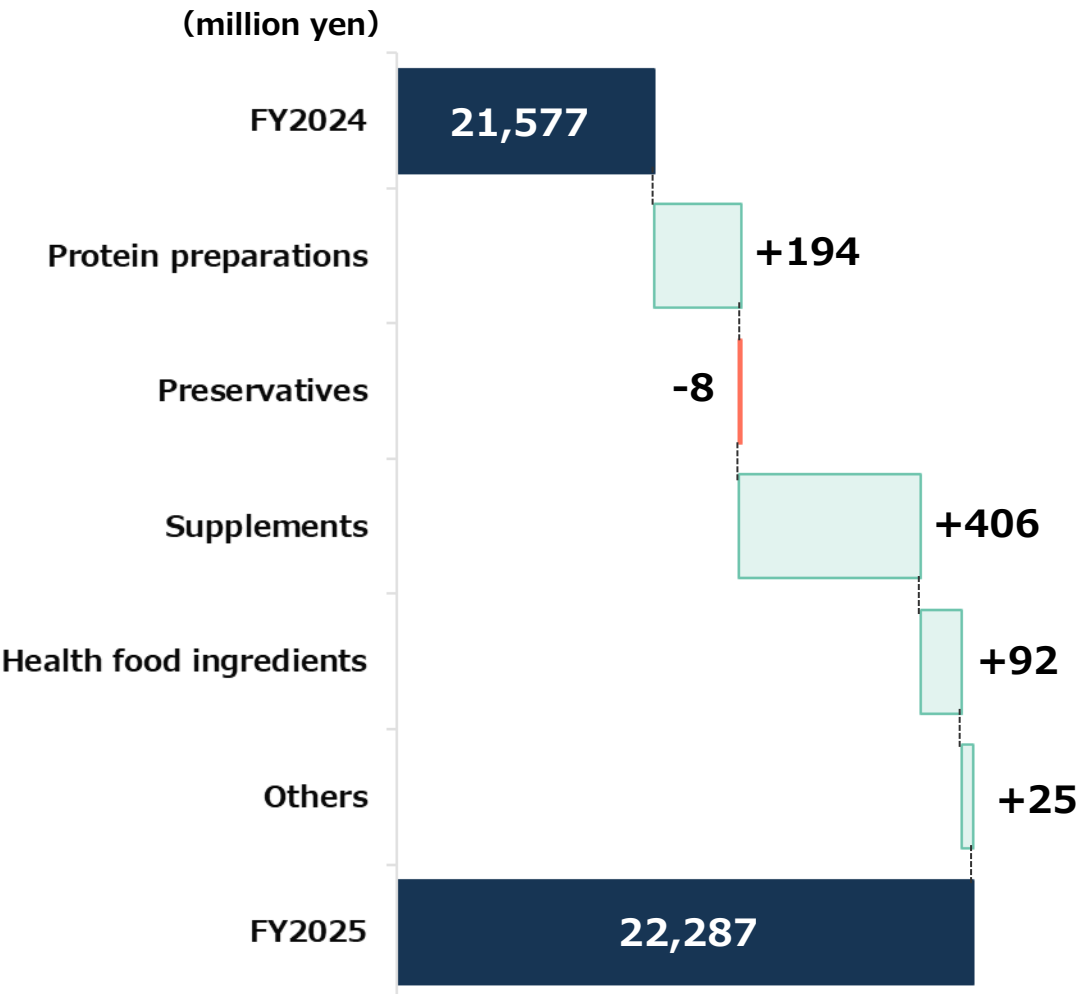
(million yen)	FY2024 actual	FY2025 actual	YoY change	YoY %	FY2026 forecast	Notes on FY2025 results
Japan	4,664	4,775	+110	+2.4%	4,800	<ul style="list-style-type: none"> ✓ The number of patients currently on therapy with Viltepso is more than three-quarters of the peak number of 128 patients in the data from Chuikyo¹. ✓ Promoting early diagnosis and intervention for younger patients
US	17,117	16,413	-704	-4.1%	17,500	<ul style="list-style-type: none"> ✓ Insurance reauthorizations became stricter after launch of multiple DMD treatment options. ✓ The average dose per patient has decreased due to younger patient population, but revenue growth is expected as a result of longer-term administration
(million US\$)	(112.18)	(108.86)	(-3.32)	(-3.0%)	(116.67)	
Total	21,782	21,188	-593	-2.7%	22,300	

Exchange rates	FY2024 actual	FY2025 actual	FY2026 assumption
USDJPY	152.6	150.8	150.0



Segmental Review - Functional Food -

- Increased segment revenue due to the launch of new products and strengthened sales activities
- Sales decline in preservatives due to competitive market



Protein preparations 13,680 million yen
 (+194 million yen, +1.4%, YoY)

- ✓ Growth in the egg protein share and strong sales of protein powder

Preservatives 3,269 million yen
 (-8 million yen, -0.3%, YoY)

- ✓ Rising prices have led to consumers holding back on purchases, resulting in a decrease in user output.

Supplements 2,821 million yen
 (+406 million yen, +16.8%, YoY)

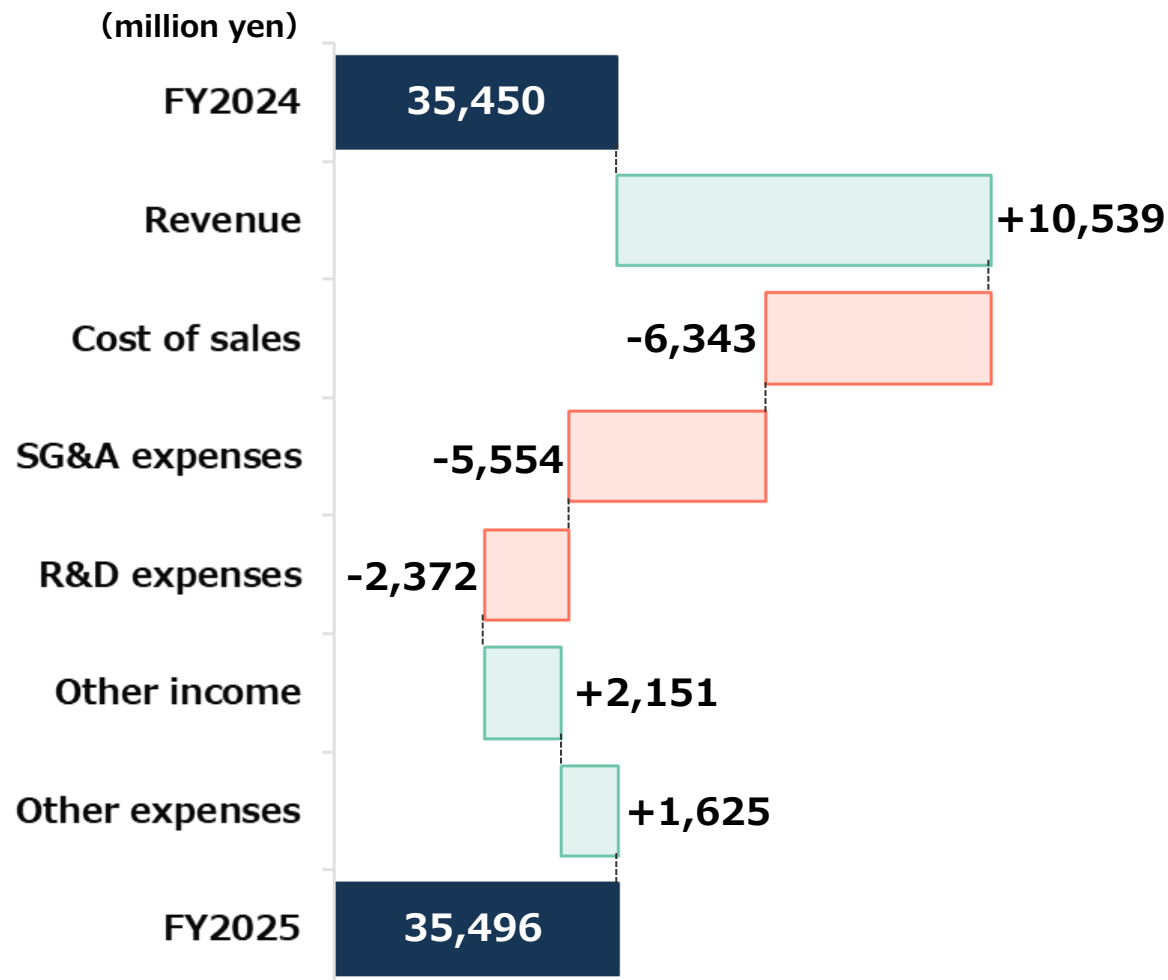
- ✓ Sales increase in both athletes and anti-aging care category
- ✓ New products for children and plant-based protein products launched in February 2026

Health food ingredients 1,214 million yen
 (+92 million yen, +8.2%, YoY)

- ✓ Expanded demand for beauty-related products

Operating Profit

- Slight increases in profits due to increase in cost of sales and SG&A expenses, etc.



Revenue 170,771 million yen

(+10,539 million yen, +6.6%, YoY)

- ✓ Growth of new product lines including Uptravi and Fintepla
- ✓ Through reverse co-promotion, Erleada is now included in revenue for the company
- ✓ Royalty revenue growth due to overseas sales of Uptravi

Cost of sales 57,460 million yen

(+6,343 million yen, +12.4%, YoY)

The ratio was 33.6%, worsened by 1.7 points YoY.

- ✓ Negative impact from changes in pharmaceutical product sales mix and NHI drug price revisions

SG&A expenses 43,565 million yen

(+5,554 million yen, + 14.6%, 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 36,713 million yen

(+2,372 million yen, +6.9%, YoY)

- ✓ Increase in contract research expense and raw material costs related to investigational products

Other income 3,025 million yen

(+2,151 million yen, + 246.0%, YoY)

- ✓ Increase in foreign exchange gains

Business Forecast for FY2026 (consolidated)

(million yen)	FY2025		FY2026		YoY		Reasons for the differences
	actual	ratio	forecast	ratio	change	%	
Revenue	170,771	100.0%	200,000	100.0%	+29,228	+17.1%	Revenue ✓ The U.S. launch of CAP-1002 (deramiocel) ✓ Growth in the domestic products ✓ Royalty revenue growth due to overseas sales increase of Uptravi
(Pharmaceuticals)	(148,484)	(86.9%)	(176,500)	(88.3%)	(+28,015)	(+18.9%)	
(Functional Food)	(22,287)	(13.1%)	(23,500)	(11.8%)	(+1,212)	(+5.4%)	
Cost of sales	57,460	33.6%	73,000	36.5%	+15,539	+27.0%	Cost of Sales ✓ Erleada and CAP-1002 (deramiocel)
SG&A expenses	43,565	25.5%	47,500	23.8%	+3,934	+9.0%	
R&D expenses	36,713	21.5%	40,500	20.3%	+3,786	+10.3%	SG&A expenses ✓ Increase in the U.S. sales expenses of NS Pharma ✓ Increase in commission for promotional activities of Uptravi due to domestic sales increase
Other income	3,025	1.8%	1,000	0.5%	-2,025	-67.0%	
Other expenses	560	0.4%	2,000	1.0%	+1,439	+256.5%	
Operating profit	35,496	20.8%	38,000	19.0%	+2,503	+7.1%	R&D expenses ✓ Increase in contract research expense
Finance income	1,132	0.7%	800	0.4%	-332	-29.3%	
Finance costs	166	0.1%	200	0.1%	+33	+20.5%	
Profit before tax	36,462	21.4%	38,600	19.3%	+2,137	+5.9%	
Income tax expense, etc.	6,741	3.9%	8,300	4.2%	+1,558	+23.1%	
Profit attributable to owners of parent	29,721	17.4%	30,300	15.2%	+578	+1.9%	

The foreign exchange rates (USDJPY) are 152.6 for FY2024a, 150.8 for FY2025a and 150.0 for FY2026e. FY2026e's sensitivity indicates that for every ¥1 depreciation of the yen against the USD, revenue is expected to increase by approximately ¥550 million and operating profit is expected to increase by approximately ¥410 million.

Revenue Forecast – Pharmaceuticals Segment -

(million yen)	FY2025		FY2026		YoY	
	actual	ratio	forecast	ratio	change	%
Ethical Drugs	90,062	60.7%	114,900	65.1%	+24,837	+27.6%
Revenues from the licensing of industrial property rights	49,457	33.3%	53,000	30.0%	+3,542	+7.2%
Profit in co-promotion	8,964	6.0%	8,600	4.9%	-364	-4.1%
Revenue	148,484	100.0%	176,500	100.0%	+28,015	+18.9%

Sales increase is expected due to the following factors;

1. New product launch in the U.S. : CAP-1002 (deramiocel)
2. Contribution of new products in Japan: Erleada, Fintepla, Uptravi, etc.
3. Growth in royalty income: overseas sales of Uptravi

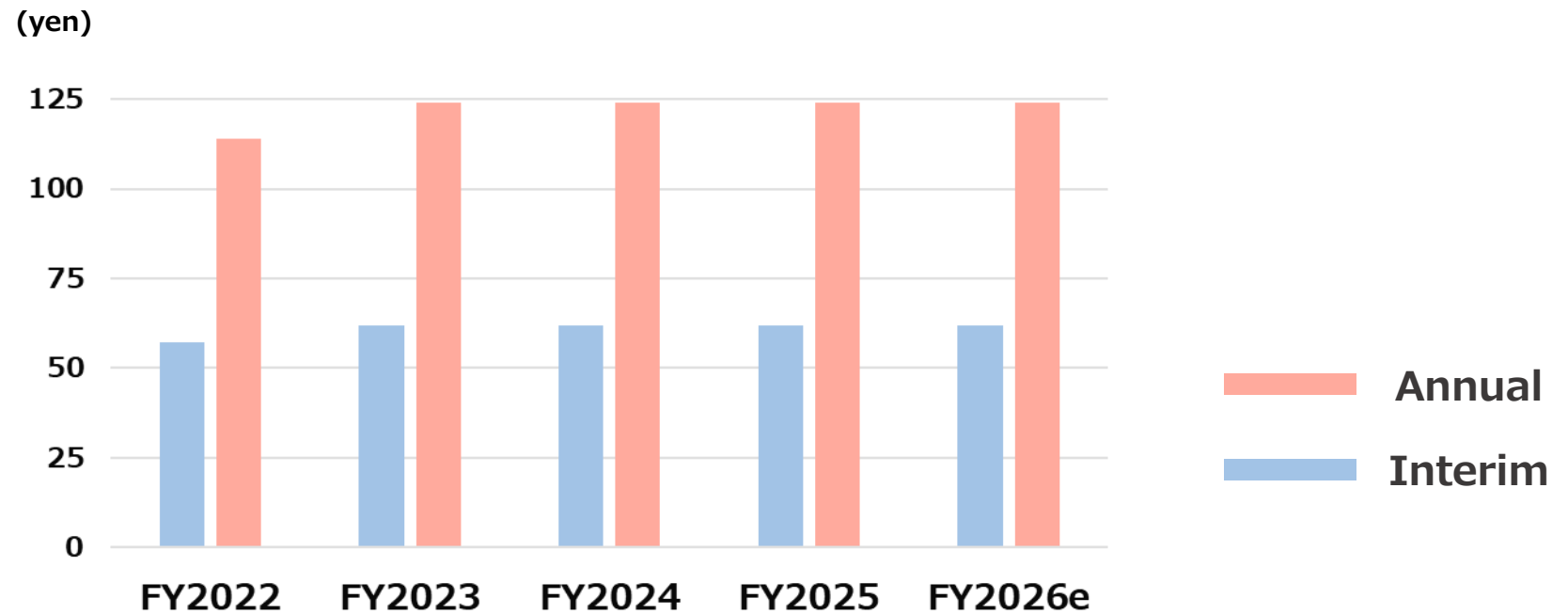
Revenue Forecast - Functional Food Segment-

(million yen)	FY2025		FY2026		YoY	
	actual	ratio	forecast	ratio	change	%
Protein preparations	13,680	61.4%	14,200	60.4%	+519	+3.8%
Preservatives	3,269	14.7%	3,400	14.5%	+130	+4.0%
Supplements	2,821	12.7%	3,400	14.5%	+578	+20.5%
Health food ingredients	1,214	5.4%	1,250	5.3%	+35	+2.9%
Others	1,302	5.8%	1,250	5.3%	-52	-4.0%
Revenue	22,287	100.0%	23,500	100.0%	+1,212	+5.4%

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

Dividends Forecast

		FY2025	FY2026e
Dividends per share	Interim	¥62	¥62
	Annual	¥124	¥124
Basic earnings per share		¥441.00	¥449.55
Payout ratio (consolidated)		28.1 %	27.6 %
Dividend on equity ratio		3.1 %	-



CAP-1002 (deramiocel) Update

CAP-1002 (deramiocele) : Regulatory Update

NEWS RELEASE



May 8, 2026

Announcement of a Lawsuit Filed against Nippon Shinyaku

KYOTO, Japan, May 8, 2026 - Nippon Shinyaku Co., Ltd. (Headquarters: Kyoto, Japan; President: Toru Nakai) announces that a lawsuit was filed against Nippon Shinyaku in the Superior Court of New Jersey, Chancery Division, Bergen County, as follows.

1. Date of Complaint

May 7th, 2026

2. Reasons and Background for the Action

Capricor Therapeutics, Inc. (Headquarters: California, USA, CEO: Linda Marbán, NASDAQ: CAPR) announced that it has filed a lawsuit against Nippon Shinyaku and its U.S. subsidiary, NS Pharma, Inc., seeking an injunction against the Commercialization and Distribution Agreement for CAP-1002 (deramiocele) (the Agreement). The lawsuit concerns the Nippon Shinyaku's product distribution rights in the United States in the Agreement.

CAP-1002 (deramiocele) is currently under the FDA review for approval for the treatment of Duchenne muscular dystrophy (DMD) cardiomyopathy.

3. Party Who Filed the Complaint

(1) Name: Capricor Therapeutics, Inc.

(2) Address: 10865 Road to the Cure, Suite 150, San Diego, CA 92121, United States

4. Contents of the Complaint

A judgement regarding the U.S. distribution rights in the United States, etc.

5. Prospects

The complaint has not been served, but our understanding is that Capricor appears to be arguing that the amendments to the Agreement regarding the U.S. launch of CAP-1002 (deramiocele), as well as the launch preparations of Nippon Shinyaku and NS Pharma are insufficient. However, we are confident that both Nippon Shinyaku and NS Pharma have responded appropriately and sincerely to ensure treatment reaches DMD patients after approval. While we recognize that Capricor's claims lack merit, we remain open to discussions with Capricor to maximize the value of CAP-1002 (deramiocele).

- On May 7th, 2026, a lawsuit was filed against Nippon Shinyaku in the Superior Court of New Jersey, Chancery Division, Bergen County.
- In order to ensure treatment reaches DMD patients after approval, both Nippon Shinyaku and NS Pharma have engaged in discussions with Capricor in a proper and sincere manner. In accordance with the Agreement regarding the U.S. launch of CAP-1002 (deramiocele), we have made thorough preparations for the product launch.
- While we recognize that Capricor's claims lack merit, we remain open to constructive discussions with Capricor in the best interests of DMD patients and their families in the U.S.

RGX-121 (clemidsogene lanparvovec)

Update

RGX-121 (clemidsogene lanparvovec) : Regulatory Update

NEWS RELEASE



NIPPON SHINYAKU CO., LTD.

May 15, 2026

Regulatory Update on RGX-121

KYOTO, Japan, May 15, 2026 - Nippon Shinyaku Co., Ltd. (Headquarters: Kyoto, Japan; President: Toru Nakai) announced that the latest regulatory developments regarding RGX-121, a gene therapy product for Mucopolysaccharidosis Type II (MPS II, Hunter syndrome) being developed by REGENXBIO Inc. (Headquarters: Rockville, Maryland, USA; President and Chief Executive Officer: Curran M. Simpson; hereinafter "REGENXBIO").

- The U.S. Food and Drug Administration (FDA) has lifted the clinical hold on RGX-121.
- REGENXBIO recently filed an appeal of the RGX-121 Complete Response Letter (CRL) and is continuing to engage the agency regarding a path forward for the program.

RGX-121 is a gene therapy under development by REGENXBIO for the treatment of Mucopolysaccharidosis Type II (MPS II, Hunter syndrome). In January 2025, Nippon Shinyaku and REGENXBIO entered into a strategic partnership for RGX-121, under which Nippon Shinyaku acquired exclusive commercialization rights in the U.S., as well as exclusive development and commercialization rights in Asia including Japan.

Following regulatory approval in the U.S., NS Pharma, Inc. (Paramus, New Jersey; President: Yukiteru Sugiyama), a U.S. subsidiary of Nippon Shinyaku, plans to commercialize the product.

About REGENXBIO Inc.

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the development of AAV Therapeutics, an innovative class of gene therapy medicines. For more information, please visit www.regenxbio.com.

Contact

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- The clinical hold placed by the U.S. Food and Drug Administration (FDA) on RGX-121 in January 2026 has been lifted.
- The FDA issued a Complete Response Letter¹ in February 2026. REGENXBIO recently filed an appeal of it and is continuing to engage the agency regarding a path forward for the program.
- Nippon Shinyaku will continue to work closely with REGENXBIO to establish a timeline for the product launch.

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.

R&D Pipeline

Keiichi Kuwano

Director, Research & Development

R&D Updates (1/2)

For updates from Q3 FY2025 financial results announcement on February 9, 2026, see highlighted text in red.

Update	Code No. (Generic name)	Brand name	Indications and topics	Schedule
Launch	NS-401 (tagraxofusp)	Elzonris	blastic plasmacytoid dendritic cell neoplasm (BPDCN)	March 2026
P3	NS-065/NCNP-01 (viltolarsen)	Viltepso	CSR for Study 301 under the FDA's review	January 2026
			Inquiring with FDA regarding the protocol for Study 303	
Additional indication	ACT-064992 (macitentan)	Opsumit	Johnson & Johnson obtained approval for the treatment of pediatric patients with pulmonary arterial hypertension (PAH) in Japan	December 2025
Additional indication	LY3527727 (pirtobrutinib)	Jaypirca	for patients with relapsed or refractory chronic lymphocytic leukemia (including small lymphocytic lymphoma) who are resistant or intolerant to other BTK inhibitors	September 2025
Filed	RGX-121 (clemidsogene lanparvovec)	-	The clinical hold placed by the FDA on RGX-121 in January 2026 has been lifted.	Disclosed in May 2026 (U.S.)
			The FDA issued a Complete Response Letter in February 2026. REGENXBIO recently filed an appeal against it and is continuing to engage the agency regarding a path forward for the program.	
Filed	CAP-1002 (deramiocel)	-	The FDA has set a new PDUFA target action date of August 22, 2026	March 2026 (U.S.)
Filed	GA101 (obinutuzumab)	Gazyva	Idiopathic nephrotic syndrome	May 2026
Revision of electronic package insert			Combination therapy with venetoclax has become available for previously untreated chronic lymphocytic leukemia	November 2025
P3			Roche announced Global Phase III INShore Study topline results for Idiopathic nephrotic syndrome in children and young adults	October 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 P3	NS-304 (selexipag)	-	Arteriosclerosis obliterans	April 2026
Preparation for P2	NS-035	-	Fukuyama congenital muscular dystrophy	January 2026
	NS-863	-	Pulmonary arterial hypertension	March 2026
			Pulmonary hypertension associated with interstitial lung disease	March 2026
Start of P1	NS-245	-	Treatment for inflammatory diseases	December 2025

R&D Updates (2/2)

For updates from Q3 FY2025 financial results announcement on February 9, 2026, see highlighted text in red.

Update	Code No. (Generic name)	Brand name	Indications and topics	Schedule
Collaboration	—	—	Collaboration agreement with xFOREST Therapeutics on Drug Discovery Research Targeting RNA Structures	March 2026
Collaboration	—	—	Launch of co-creation project to evaluate drug discovery seeds with FRONTEO, Inc.	December 2025
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.)
Fast Track Designation	NS-229	—	eosinophilic granulomatosis with polyangiitis (EGPA)	September 2025 (U.S.)
Orphan Drug Designation	NS-051/NCNP-04	—	Duchenne muscular dystrophy	September 2025 (U.S.)
Academic conference presentation	NS-089/NCNP-02 (brogidirsen)		3.5-Year clinical trial data presentation at the World Muscle Society 2025 Congress	October 2025

Reference Materials

Sales Forecast in Pharmaceuticals Segment

- The U.S. launch of the CAP-1002, and growth in domestic sales of Erleada, Fintepla, Uptravi, etc.
- Royalty revenue growth due to overseas sales increase of Uptravi

(Million yen)

Brand name/ code no.	Indications	FY2024	FY2025	YoY		FY2026
		actual	actual	change	%	Forecast
Viltepso		21,782	21,188	-593	-2.7%	22,300
(Japan)	Duchenne muscular dystrophy (DMD)	(4,664)	(4,775)	(+110)	(+2.4%)	(4,800)
(U.S.)		(17,117)	(16,413)	(-704)	(-4.1%)	(17,500)
Uptravi	pulmonary arterial hypertension/ chronic thromboembolic pulmonary hypertension	14,971	17,593	+2,622	+17.5%	19,200
Erleada	prostate cancer	-	5,961	+5,961	0.0%	14,400
Vyxeos	high-risk AML	5,139	5,742	+602	+11.7%	6,500
Gazyva	CD20-positive follicular lymphoma/ CD20-positive chronic lymphocytic leukemia	4,821	5,076	+255	+5.3%	5,100
Fintepla	seizures associated with Dravet syndrome/ seizures associated with Lennox-Gastaut	2,067	3,980	+1,912	+92.5%	6,100
Defitelio	sinusoidal obstruction syndrome	2,364	2,514	+150	+6.3%	2,600
Cialis	erectile dysfunction	2,425	2,221	-203	-8.4%	2,000
CAP-1002 deramiocel (U.S.)	DMD cardiomyopathy	-	-	-	-	11,400
Profit in co-promotion		9,170	8,964	-205	-2.2%	8,600
Revenues from the licensing of industrial property rights		45,585	49,457	+3,871	+8.5%	53,000
Revenue		138,654	148,484	+9,829	+7.1%	176,500
Revenue based on the NHI drug price						
Jaypirca	mantle cell lymphoma chronic lymphocytic leukemia	780	2,626	+1,845	+236.5%	

Sales forecasts for Jaypirca are not disclosed.

The foreign exchange rate assumed for FY2026 business forecast is ¥150 per USD and its sensitivity indicates that for every ¥1 depreciation of the yen against the USD, revenue is expected to increase by approximately ¥550 million.

Consolidated Balance Sheet

(million yen)	End of FY2024	End of FY2025	YoY change		End of FY2024	End of FY2025	YoY change
Assets	283,637	346,359	+62,721	Liabilities	36,297	54,488	+18,191
Current assets	149,740	183,086	+33,346	Current liabilities	30,316	42,105	+11,789
Non-current assets	133,897	163,272	+29,375	Non-current liabilities	5,980	12,382	+6,401
				Equity	247,340	291,871	+44,530
Total assets	283,637	346,359	+62,721	Total liabilities and equity	283,637	346,359	+62,721

Assets

Cash and cash equivalents	+21,351
Trade and other receivables	+3,865
Inventories	+9,094
Other financial assets (non-current)	+27,863

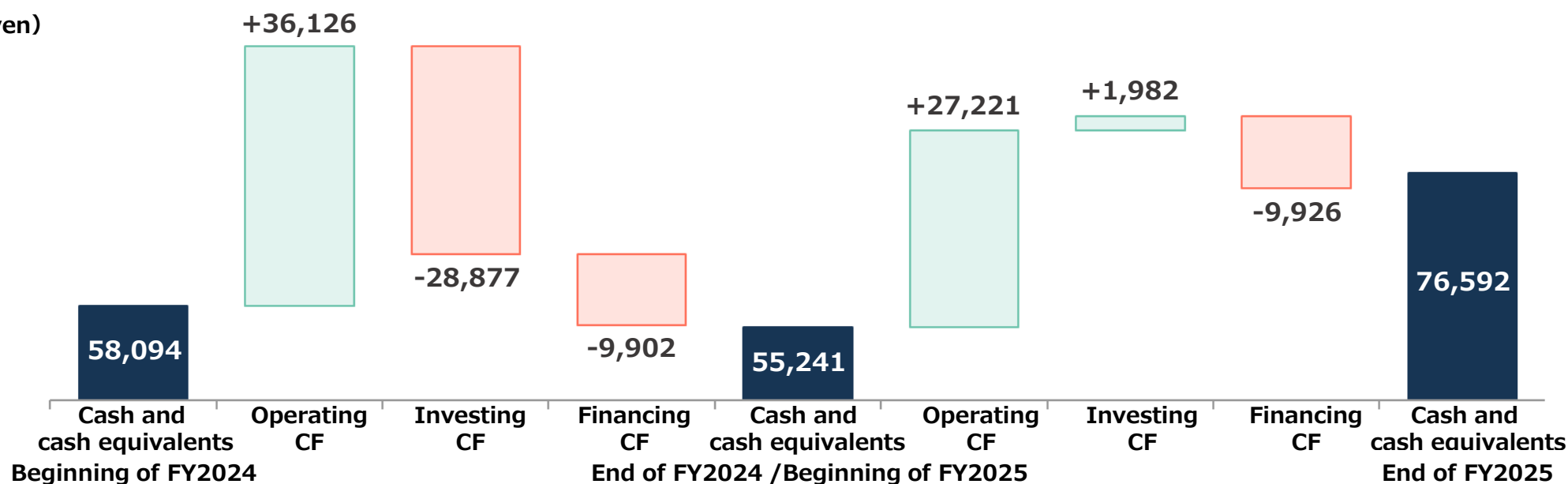
Liabilities and Equity

Trade and other payables	+9,274
Deferred tax liabilities	+5,875
Retained earnings	+24,050

Consolidated Statements of Cash Flows

(million yen)	FY2024 actual	FY2025 actual	YoY change
Operating activities	36,126	27,221	-8,905
Investing activities	-28,877	1,982	+30,859
Financing activities	-9,902	-9,926	-24
Cash and cash equivalents at end of period	55,241	76,592	+21,351

(million yen)



Pipeline (1/2)

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

Stage	Code No. (Generic name)	Origin	Indications	Schedule	Country	ID#
Launch P3	NS-065/NCNP-01 (viltolarsen)	Co-development with National Center of Neurology and Psychiatry	Duchenne muscular dystrophy	—	Japan	jRCT2080224893
				—	U.S.	NCT04060199
Filed	CAP-1002 (deramiocel)	Partnership Capricor Therapeutics, Inc.	Duchenne muscular dystrophy cardiomyopathy	PDUFA date: August 2026	U.S.	NCT03406780 ¹ NCT05126758 ²
	RGX-121 (clemidsogene lanparvovec)	Partnership REGENXBIO Inc.	mucopolysaccharidosis type II	Clinical hold lifted ³ CRL ⁴	U.S.	NCT03566043
	GA101 (obinutuzumab)	In-license Chugai Pharmaceutical Co., Ltd.	idiopathic nephrotic syndrome	Submission : CY2026	Japan	jRCT2011210059
P3	ZX008 (fenfluramine hydrochloride)	In-license ⁵ UCB S.A.	CDKL5 deficiency disorder	Study completion : FY2026	Japan	jRCT2041230015
	GA101 (obinutuzumab)	In-license Chugai Pharmaceutical Co., Ltd.	lupus nephritis	Projected submission : CY2026	Japan	NCT05627557
			extra renal lupus	Projected submission : CY2027	Japan	jRCT2071230031
	LY3527727 (pirtobrutinib)	Alliance agreement Eli Lilly Japan K.K.	mantle cell lymphoma	—	Japan	jRCT2021210026 jRCT2011210061
			chronic lymphocytic leukemia	—	Japan	jRCT2041210150 jRCT2021220024
			CAP-1002 (deramiocel)	Partnership Capricor Therapeutics, Inc.	Duchenne muscular dystrophy	—
	NS-304 (selexipag)	In-house	arteriosclerosis obliterans	Study start : FY2026	Japan	jRCT2071250134

1. The Phase 2 (HOPE-2) study
2. The Phase 3 (HOPE-3) study
3. The clinical hold placed by the FDA on RGX-121 in January 2026 has been lifted.

4. The FDA issued a Complete Response Letter in February 2026. REGENXBIO recently filed an appeal of it and is continuing to engage the agency regarding a path forward for the program.
5. The marketing authorization in Japan has been transferred from UCB Japan Co., Ltd. to Nippon Shinyaku effective April 1, 2026.

Pipeline (2/2)

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

Stage	Code No. (Generic name)	Origin	Indications	Schedule	Country	ID#
P2	NS-580	In-house	endometriosis	Temporarily suspended	Japan	jRCT2031210685
			chronic prostatitis/ chronic pelvic pain syndrome	Temporarily suspended	Japan	jRCT2031230134
	NS-089/NCNP-02 (brogidirsen)	Co-development with National Center of Neurology and Psychiatry	Duchenne muscular dystrophy	Study completion : FY2026	Japan	jRCT2041250028
					U.S.	NCT05996003
	NS-229	In-house	eosinophilic granulomatosis with polyangiitis	Study completion : FY2026	Japan	jRCT2031230526
U.S.					NCT06046222	
Preparation for P2	NS-035	Co-development with Kobe University	Fukuyama congenital muscular dystrophy (FCMD)	Study start : FY2026	Japan	jRCT2031250857
					Japan	In preparation
	NS-863	In-house	pulmonary arterial hypertension	Study start : FY2026	U.S.	NCT07441200
					Japan	In preparation
NS-863	In-house	pulmonary hypertension associated with interstitial lung disease	Study start : FY2026	U.S.	NCT07441278	
				Japan	In preparation	
P1/2	NS-050/NCNP-03	Co-development with National Center of Neurology and Psychiatry	Duchenne muscular dystrophy	Study completion : FY2027	Japan	jRCT2041240060
					U.S.	NCT06053814
	ATSN-101	In-license Atsena Therapeutics	GUCY2D-associated Leber congenital amaurosis	Study completion : FY2027	U.S.	NCT03920007
	RGX-111	Partnership REGENXBIO Inc.	mucopolysaccharidosis type I	Under clinical hold ¹	U.S.	NCT03580083
P1	NS-917 (radgocitabine)	In-license Delta-Fly Pharma, Inc.	relapsed/refractory acute myeloid leukemia	Study completion : FY2026	Japan	jRCT2031210452
	NS-025	In-house	urological diseases	Study completion : FY2024	Japan	jRCT2031220474
	NS-245	In-house	Inflammatory diseases	Study completion : FY2026	Japan	jRCT2071250086

1. The FDA placed a clinical hold on RGX-111 in January 2026.

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	<ul style="list-style-type: none">• 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

Supplementary Information : DMD affects approximately 1 in every 3,500 to 5,000 male births. Estimated patient numbers are 3,500 in Japan, 12,500 to 15,000 in the United States, 7,000 in Europe (UK, France, Germany), and 53,000 in China. Patients eligible for exon 53 skipping treatment represent approximately 8% of all DMD patients.

CAP-1002 (deramiocelel)

- Treatment for Duchenne muscular dystrophy cardiomyopathy-

Development Phase	U.S. : BLA Filed (Duchenne muscular dystrophy cardiomyopathy) U.S. : P3 (Duchenne muscular dystrophy)
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	<ul style="list-style-type: none">• 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. : 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	<ul style="list-style-type: none">• 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

Supplementary Information : The prevalence rates per 100,000 people for MPS in Japan and the United States are as follows: MPS overall: 1.53 and 1.2, MPS II: 0.84 and 0.29, MPS I: 0.23 and 0.34 [Epidemiology of Mucopolysaccharidoses Update - PMC](#)

GA101 (obinutuzumab)

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

Development Phase	Japan : Filed (INS) Japan : P3 (LN) 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	idiopathic nephrotic syndrome (INS) lupus nephritis (LN) extra renal lupus (ERL)
Dosage form	Injection
Feature	Anti-CD20 monoclonal antibody, increased antibody-dependent cellular cytotoxicity (ADCC) activity and direct cytotoxicity

Supplementary Information : The estimated number of patients in Japan is approximately 14,000 for INS, approximately 30,000 for LN, and approximately 10,000 for ERL.

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] In-Licensed from :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	<ul style="list-style-type: none">• 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

Supplementary Information : The estimated number of patients in Japan is approximately 3,000 for Dravet syndrome and approximately 4,300 for Lennox-Gastaut syndrome.

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) Launched (for patients with relapsed or refractory chronic lymphocytic leukemia 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

Supplementary Information : Annually, about one in 200,000 people worldwide develop MCL. (National Organization for Rare Disorders. Mantle cell lymphoma. Accessed 26 October 2022)

CLL is a rare disease in Japan where about 7,000 people are suffering. (Official Statistics of Japan. Patient survey in FY2023.)

NS-304 (selexipag)

- Treatment for arteriosclerosis obliterans -

Development Phase	Japan : P3 (ASO)
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	Selective IP receptor agonist
Indications	arteriosclerosis obliterans (ASO)
Dosage form	Oral agent
Feature	Long-acting oral drug

Supplementary Information : The number of patients in Japan with intermittent claudication due to ASO is estimated to be approximately 0.64 to 0.93 million.

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	<ul style="list-style-type: none">• 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	<ul style="list-style-type: none">• 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

Supplementary Information : DMD affects approximately 1 in every 3,500 to 5,000 male births. Estimated patient numbers are 3,500 in Japan, 12,500 to 15,000 in the United States, 7,000 in Europe (UK, France, Germany), and 53,000 in China. Patients eligible for exon 44 skipping treatment represent approximately 6% of all DMD patients.

- 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	<ul style="list-style-type: none"> • Potent and highly selective JAK1 inhibitor • High efficacy and good safety profiles are expected in the treatment for EGPA

Supplementary Information : The number of EGPA cases reported by the Ministry of Health, Labour and Welfare for FY2024 is 8,669.

- Treatment for Fukuyama congenital muscular dystrophy-

Development Phase	Japan : in preparation for P2
Origin	Co-development : Kobe University
Development	Nippon Shinyaku
Mechanism of action	Exon trapping
Indications	Fukuyama congenital muscular dystrophy (FCMD)
Dosage form	Injection
Feature	A nucleic acid therapy designed to restore functional fukutin and improve muscle function. Expected to be the world's first disease-modifying treatment for FCMD, a hereditary disorder prevalent in Japan.

Supplementary Information : Fukuyama congenital muscular dystrophy (FCMD) is a hereditary disorder that is particularly prevalent in the Japanese population. The estimated number of patients in Japan is approximately 1,000 to 2,000.

- Treatment for pulmonary arterial hypertension/ Pulmonary hypertension associated with interstitial lung disease -

Development Phase	Global : in preparation for P2 (PAH) Global : in preparation for P2 (PH-ILD)
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	Selective PDGFR inhibitor
Indications	Pulmonary arterial hypertension (PAH) Pulmonary Hypertension associated with Interstitial Lung Disease (PH-ILD)
Dosage form	Oral agent
Feature	A highly selective PDGFR kinase inhibitor that improves pulmonary vascular occlusion and offers a novel, orally administered treatment option for pulmonary hypertension.

Supplementary Information : The number of PAH cases reported by the Ministry of Health, Labour and Welfare for FY2024 is 4,906.

- 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	<ul style="list-style-type: none">• 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

Supplementary Information : DMD affects approximately 1 in every 3,500 to 5,000 male births. Estimated patient numbers are 3,500 in Japan, 12,500 to 15,000 in the United States, 7,000 in Europe (UK, France, Germany), and 53,000 in China. Patients eligible for exon 50 skipping treatment represent approximately 4% of all DMD patients.

- 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	<ul style="list-style-type: none"> • 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.

Supplementary Information : The estimated number of patients with Leber congenital amaurosis (LCA) is approximately 10,000 (Japan) and nearly 50,000 (U.S.). GUCY2D-associated Leber congenital amaurosis is one of the most common forms of LCA, estimated to account for approximately 10% of all LCA cases.

- 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	<ul style="list-style-type: none"> • 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

Supplementary Information : The prevalence rates per 100,000 people for MPS in Japan and the United States are as follows: MPS overall: 1.53 and 1.2, MPS II: 0.84 and 0.29, MPS I: 0.23 and 0.34 [Epidemiology of Mucopolysaccharidoses Update - PMC](#)

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	<ul style="list-style-type: none">• 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

Supplementary Information : The number of patients receiving AML treatment in Japan is estimated to be approximately 16,000 (ADM2019).
Among them, the number of patients with relapsed or refractory disease accounts for about 50%.

NS-025

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-245

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

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