FY2025 Second Quarter (Interim Period) Financial Results

November 14, 2025
NIPPON SHINYAKU CO., LTD.

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Agenda

01	- Q2 FY2025 Financial Results and Full-Year Forecast - CAP-1002 (deramiocel) Update - Preparations for New Product Launches in the U.S.	Toru Nakai Representative Director, President
02	- R&D Pipeline	Keiichi Kuwano Director, Research & Development

Q2 FY2025 FINANCIAL RESULTS AND FULL-YEAR FORECAST

Toru Nakai

Representative Director, President

Highlight

Q2 FY2025 Financial Results

- ✓ Increased revenue in both the pharmaceuticals and functional food businesses, marking the third consecutive period of revenue growth
- ✓ Higher profits contributed by increased revenue, coupled with reduced R&D expenses and foreign exchange loss

FY2025 Full-Year Forecast

- ✓ Revenue revised upward by ¥2.0 billion
- ✓ Operating profit revised upward by ¥3.0 billion due to increased revenue and reduced SG&A, R&D expenses, and foreign exchange loss

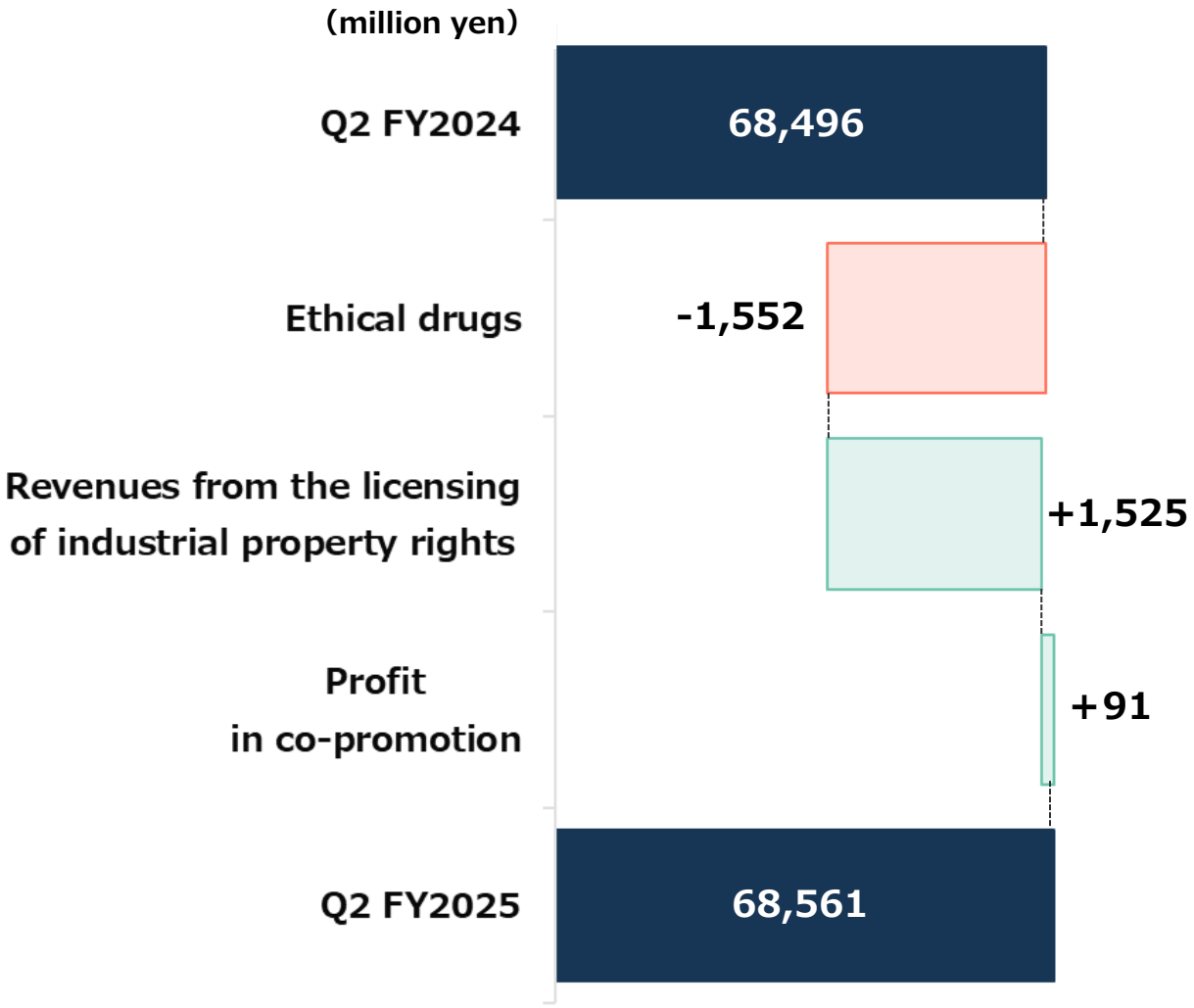
Q2 (Interim Period) FY2025 Summary

- **Increased revenue in both the pharmaceuticals and functional food businesses, marking the third consecutive period of revenue growth**
- **Increased operating profit and decreased profit attributable to owners of parent**

(million yen)	Q2 FY2024		Q2 FY2025		YoY	
	actual	ratio	actual	ratio	change	%
Revenue	**79,332**	**100.0%**	**79,647**	**100.0%**	**+315**	**+0.4%**
(Pharmaceuticals)	(68,496)	(86.3%)	(68,561)	(86.1%)	(+64)	(+0.1%)
(Functional Food)	(10,836)	(13.7%)	(11,086)	(13.9%)	(+250)	(+2.3%)
Cost of sales	**24,935**	**31.4%**	**25,336**	**31.8%**	**+400**	**+1.6%**
SG&A expenses	**18,031**	**22.7%**	**20,357**	**25.6%**	**+2,325**	**+12.9%**
R&D expenses	**16,732**	**21.1%**	**14,637**	**18.4%**	**-2,095**	**-12.5%**
Other income	**455**	**0.6%**	**521**	**0.7%**	**+66**	**+14.7%**
(Foreign exchange gain)	-	-	(153)	(0.2%)	(+153)	-
Other expenses	**2,219**	**2.9%**	**258**	**0.3%**	**-1,960**	**-88.3%**
(Foreign exchange loss)	1,935	(2.4%)	-	-	(-1,935)	-
Operating profit	**17,867**	**22.5%**	**19,580**	**24.6%**	**+1,712**	**+9.6%**
Finance income	**396**	**0.5%**	**550**	**0.7%**	**+153**	**+38.8%**
Finance costs	**65**	**0.1%**	**101**	**0.2%**	**+35**	**+54.4%**
Profit before tax	**18,198**	**22.9%**	**20,029**	**25.1%**	**+1,830**	**+10.1%**
Income tax expense, etc.	**1,825**	**2.3%**	**4,268**	**5.3%**	**+2,442**	**+133.8%**
Profit attributable to owners of parent	**16,373**	**20.6%**	**15,760**	**19.8%**	**-612**	**-3.7%**

Segmental Review - Pharmaceuticals -

- **Negative impacts of the National Health Insurance drug price revisions and generic competition**
- **Growth in domestic sales of Uptravi, Fintepla, etc., and royalty income from overseas sales of Uptravi**



Ethical drugs 39,587 million yen
(-1,552 million yen, -3.8%, YoY)

- ✓ Shipments of Viltepso outside Japan and the U.S. under Managed Access Program delayed to 2H FY2025
- ✓ Decline in sales of off-patent brand-name drugs such as Vidaza due to drug price revisions and generic competition
- ✓ Growth of new product lines including Fintepla, Uptravi, and Vyxeos

Revenues from the industrial property rights
24,181 million yen
(+1,525 million yen, +6.7%, YoY)

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

Profit in co-promotion 4,792 million yen
(+91 million yen, +2.0%, YoY)

- ✓ Sales growth of Yuvanci

Sales Trends of Viltepso® (viltolarsen)

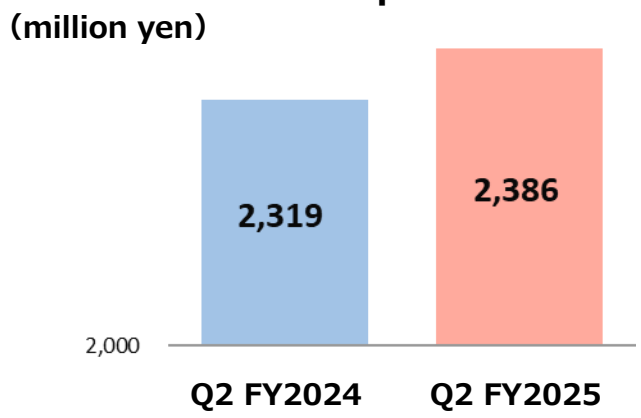
Sales of Viltepso in the U.S. declined YoY in Q2 FY2025, but the full-year forecast (dollar-based) shows a slight increase due to the acquisition of new patients.

(million yen)	Q2 FY2024 actual	Q2 FY2025 actual	YoY change	YoY %	FY2025 forecast	Notes on Q2 FY2025 results
Japan	**2,319**	**2,386**	**+66**	**+2.9%**	**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]. ✓ Currently identifying patients under the age of 20 who are eligible for treatment.
US	**8,682**	**7,963**	**-718**	**-8.3%**	**16,300**	✓ Insurance reauthorizations became stricter after launch of multiple DMD treatment options. ✓ U.S. sales for FY2025 are projected to slightly increase on a dollar basis due to the acquisition of new patients. (FY2024 sales actual: US$112.19 million)
(million US$)	(56.88)	(54.54)	(-2.34)	(-4.1%)	(114.06)	
Total	**11,002**	**10,349**	**-652**	**-5.9%**	**21,100**	

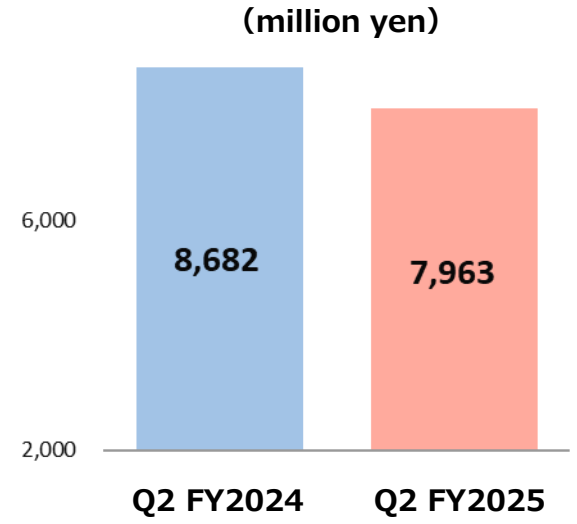
1. Central Social Insurance Medical Council

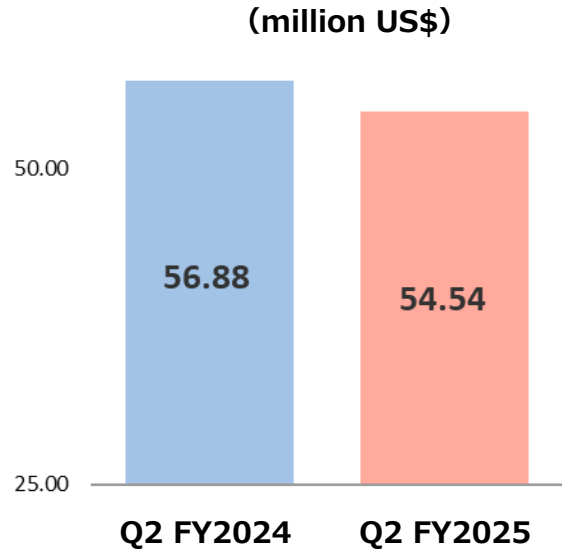
Exchange rates	Q2 FY2024 actual	Q2 FY2025 actual	2H FY2025 forecast
USDJPY	**152.8**	**146.0**	**140.0**

Japan



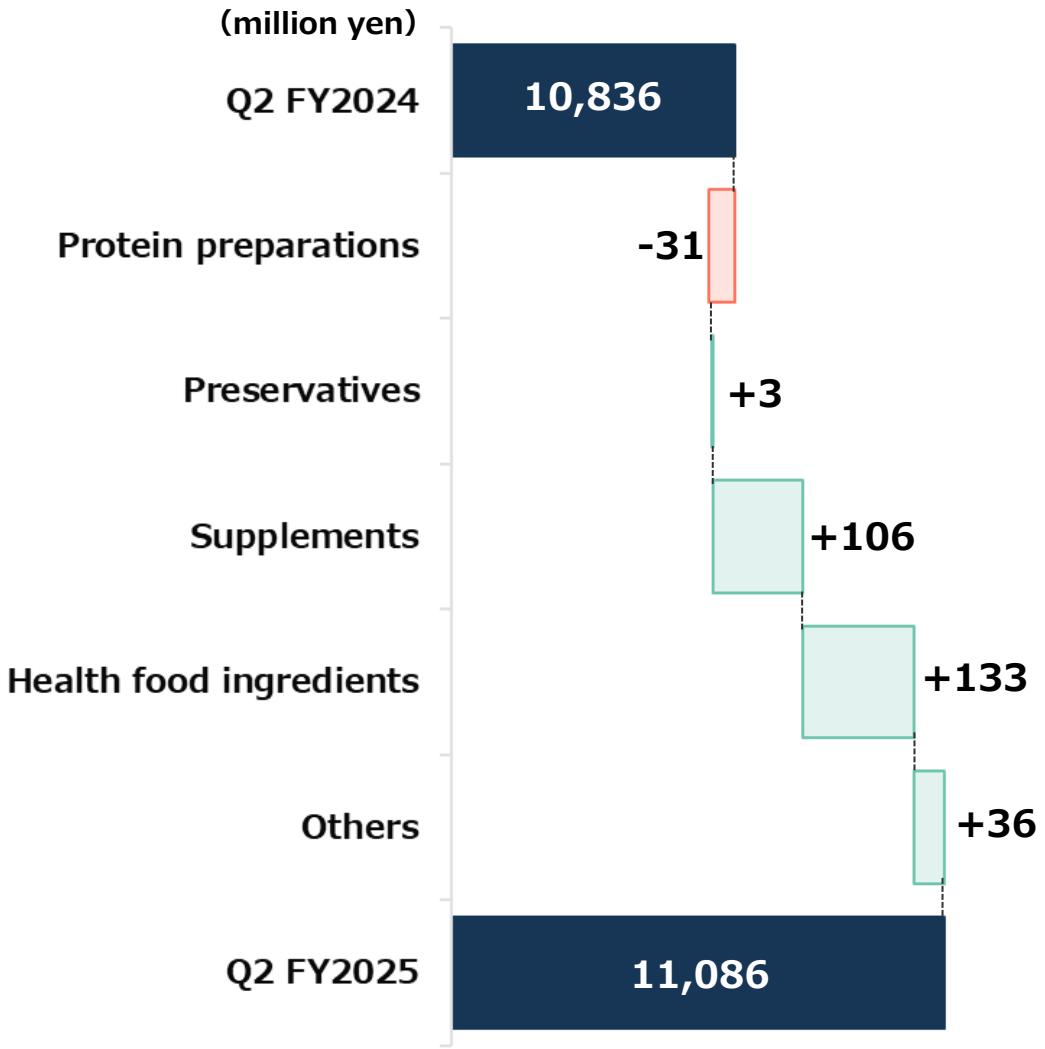
U.S.





Segmental Review - Functional Food -

- **Slight decline in protein preparations sales due to competitive market**
- **Growing Health food ingredients amid rising demand for beauty-related products, and robust supplements helped by the market expansion**



Protein preparations 6,861 million yen
(-31 million yen, -0.5%, YoY)

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

Preservatives 1,624 million yen
(+3 million yen, +0.2%, YoY)

- ✓ Promoting consultative sales to key users

Supplements 1,309 million yen
(+106 million yen, +8.9%, YoY)

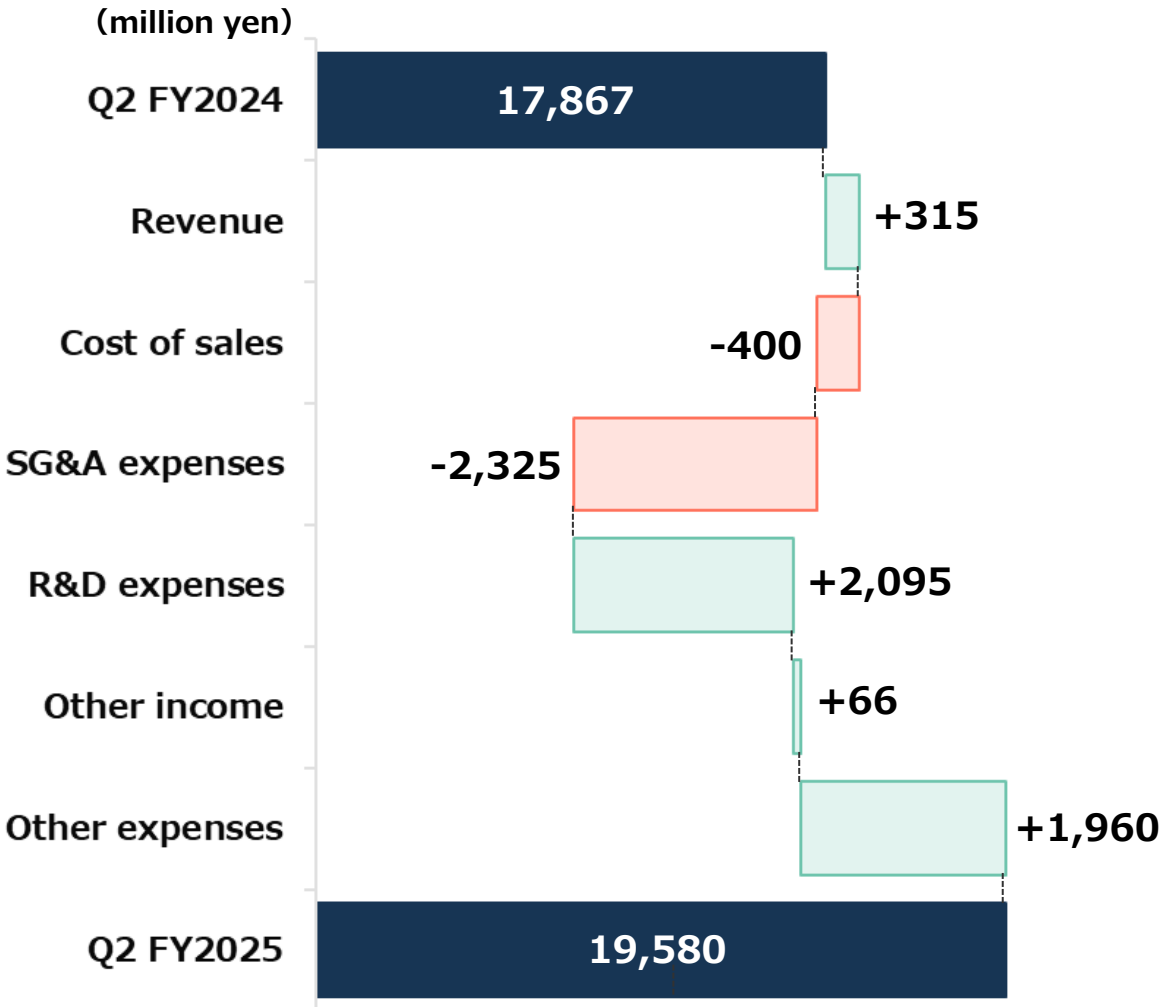
- ✓ Sales increase in both athletes and anti-aging care category

Health food ingredients 621 million yen
(+133 million yen, +27.5%, YoY)

- ✓ Expanded demand for beauty-related products

Operating Profit

- **Higher profits contributed by increased revenue, coupled with reduced R&D expenses and foreign exchange loss**



Revenue 79,647 million yen
(+ 315 million yen, + 0.4%, YoY)

- ✓ Growth of new product lines including Fintepla, Uptravi, and Vyxeos
- ✓ Royalty revenue growth due to overseas sales of Uptravi

Cost of sales 25,336 million yen
(+400 million yen, +1.6%, YoY)
The ratio was 31.8%, worsened by 0.4 points YoY.

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

SG&A expenses 20,357 million yen
(+2,325 million yen, +12.9%, 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 14,637 million yen
(-2,095 million yen, -12.5%, YoY)

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

Business Forecast : Upward Full-Year Revision from August

- **Operating profit revised upward by ¥3.0 billion due to increased revenue and reductions in SG&A, R&D expenses, and foreign exchange loss**

(Million yen)	FY2025 Forecasts		YoY	
	Previous*	Revised	change	%
Revenue	**166,000**	**168,000**	**+2,000**	**+1.2%**
(Pharmaceuticals)	(143,000)	**(145,000)**	**(+2,000)**	(+1.4%)
(Functional Food)	(23,000)	(23,000)	-	-
Cost of sales	**51,200**	**57,000**	**+5,800**	**+11.3%**
SG&A expenses	**44,000**	**43,000**	**-1,000**	**-2.3%**
R&D expenses	**39,500**	**35,000**	**-4,500**	**-11.4%**
Other income	**600**	**1,000**	**+400**	**+66.7%**
Other expenses	**1,900**	**1,000**	**-900**	**-47.4%**
Operating profit	**30,000**	**33,000**	**+3,000**	**+10.0%**
Finance income	**700**	**900**	**+200**	**+28.6%**
Finance costs	**100**	**200**	**+100**	**+100.0%**
Profit before tax	**30,600**	**33,700**	**+3,100**	**+10.1%**
Income tax expense, etc.	**6,600**	**7,400**	**+800**	**+12.1%**
Profit attributable to owners of parent	**24,000**	**26,300**	**+2,300**	**+9.6%**

Revenue 168,000 million yen
(+2,000 million yen, +1.2% from previous forecast)

✓ Erleada's reverse co-promotion (previously recorded as profit in co-promotion, but now recorded as product sales and cost of goods sold, based on contract revisions)

Cost of sales 57,000 million yen
(+5,800 million yen, +11.3% from previous forecast)

✓ Start of Erleada's reverse co-promotion

SG&A expenses 43,000 million yen
(-1,000 million yen, -2.3% from previous forecast)

✓ Decrease in U.S. sales expenses due to the PDUFA delay for RGX-121

R&D expenses 35,000 million yen
(-4,500 million yen, -11.4% from previous forecast)

✓ Shifting the cost recognition for Viltepso and other items to subsequent fiscal years, etc.

* August 7, 2025 (Q1 FY2025 financial results announcement)

The foreign exchange rate assumed for 2H FY2025 business forecast is ¥140 per USD and its sensitivity indicates that for every ¥1 depreciation of the yen against the USD, revenue is expected to increase by approximately ¥240 million and operating profit is expected to increase by approximately ¥240 million.

Revised Business Forecast for FY2025 (consolidated)

(million yen)	FY2024 actual	FY2024 ratio	FY2025 forecast	FY2025 ratio	YoY change	YoY %
Revenue	**160,232**	**100.0%**	**168,000**	**100.0%**	**+7,767**	**+4.8%**
(Pharmaceuticals)	(138,654)	(86.5%)	(145,000)	(86.3%)	(+6,345)	(+4.6%)
(Functional Food)	(21,577)	(13.5%)	(23,000)	(13.7%)	(+1,422)	(+6.6%)
Cost of sales	**51,116**	**31.9%**	**57,000**	**33.9%**	**+5,883**	**+11.5%**
SG&A expenses	**38,011**	**23.7%**	**43,000**	**25.6%**	**+4,988**	**+13.1%**
R&D expenses	**34,341**	**21.4%**	**35,000**	**20.8%**	**+658**	**+1.9%**
Other income	**874**	**0.5%**	**1,000**	**0.6%**	**+125**	**+14.3%**
Other expenses	**2,186**	**1.4%**	**1,000**	**0.6%**	**-1,186**	**-54.3%**
Operating profit	**35,450**	**22.1%**	**33,000**	**19.6%**	**-2,450**	**-6.9%**
Finance income	**830**	**0.5%**	**900**	**0.5%**	**+69**	**+8.4%**
Finance costs	**145**	**0.0%**	**200**	**0.1%**	**+54**	**+37.5%**
Profit before tax	**36,135**	**22.6%**	**33,700**	**20.1%**	**-2,435**	**-6.7%**
Income tax expense, etc.	**3,577**	**2.3%**	**7,400**	**4.4%**	**+3,822**	**+106.9%**
Profit attributable to owners of parent	**32,558**	**20.3%**	**26,300**	**15.7%**	**-6,258**	**-19.2%**

Foreign exchange rates (USDJPY)

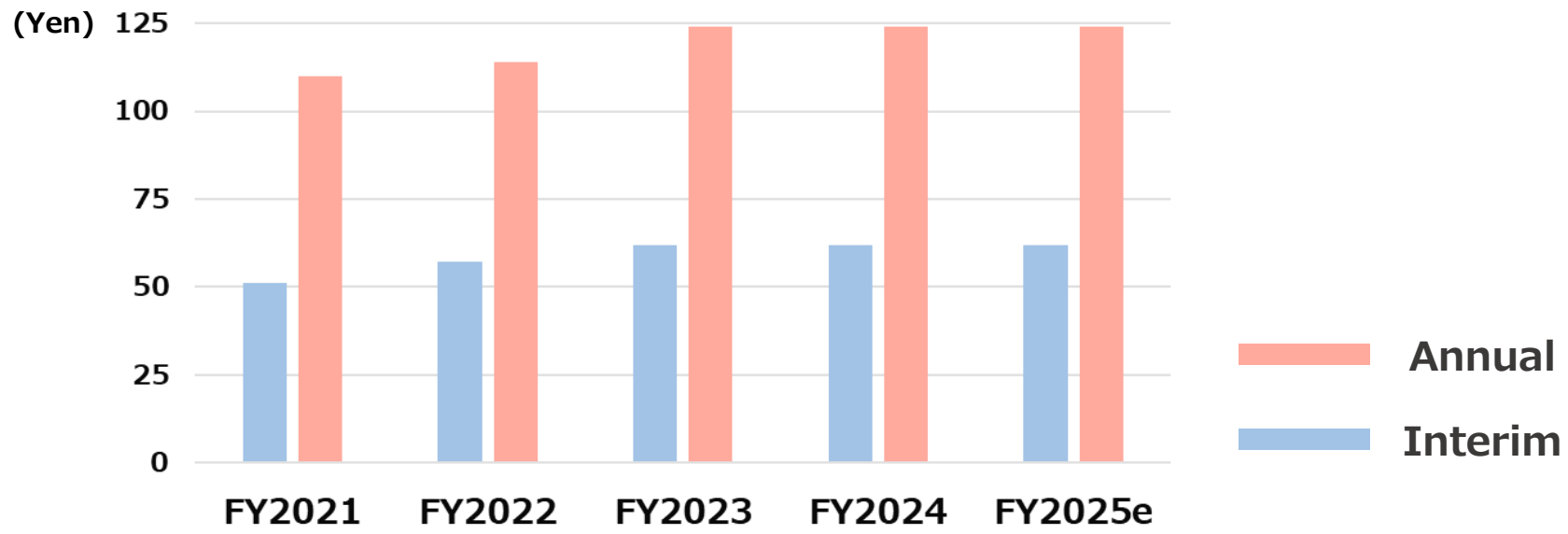
Q2 FY2024 actual	Q2 FY2025 actual	2H FY2025 forecast
152.8	146.0	140.0

The foreign exchange rate assumed for 2H FY2025 business forecast is ¥140 per USD and its sensitivity indicates that for every ¥1 depreciation of the yen against the USD, revenue is expected to increase by approximately ¥240 million and operating profit is expected to increase by approximately ¥240 million.

Dividends Forecast

- The Company's policy is to maintain stable dividends while taking into consideration the dividend on equity ratio（DOE）.

		FY2024	FY2025
Dividends per share	Interim	¥62	¥62
	Annual	¥124	¥124(e)
Basic earnings per share		¥483.40	¥390.20(e)



CAP-1002 (DERAMIOCEL) UPDATE

CAP-1002 (deramiocel) update

September 25, 2025

Capricor Therapeutics

Capricor Therapeutics Provides Regulatory Update on Deramiocel Program for Duchenne Muscular Dystrophy Following Type A Meeting

- *FDA and Capricor aligned on endpoints for HOPE-3 pivotal trial*
- *HOPE-3 pivotal trial completed; topline data expected mid-Q4 2025 to support BLA resubmission*
- *Company preparing to resubmit CRL response under the current BLA*
- *Conference call and webcast scheduled for today at 8:30 a.m. ET*

SAN DIEGO, Sept. 25, 2025 (GLOBE NEWSWIRE) -- Capricor Therapeutics (NASDAQ: CAPR), a biotechnology company developing transformative cell and exosome-based therapeutics for rare diseases, today announced a regulatory update for its Biologics License Application (BLA) for Deramiocel, the Company's investigational cell therapy for the treatment of Duchenne muscular dystrophy (DMD). This update follows a recent Type A meeting with the U.S. Food and Drug Administration (FDA) after the receipt of a Complete Response Letter (CRL) in July 2025.

The goal of the Type A meeting was to establish a path toward potential approval of Deramiocel for the treatment of DMD. Key outcomes included:

- The HOPE-3 clinical trial should serve as the "additional study" requested in the CRL.
- The HOPE-3 data can be submitted within the current BLA, maintaining PUL v2.0 as the primary efficacy endpoint and suggesting left ventricular ejection fraction (LVEF) as a key secondary endpoint, which Capricor intends to request for labeling consideration.
- Capricor plans to submit HOPE-3 data with its complete response to the CRL, with the goal of securing a label encompassing both cardiac and skeletal muscle function in DMD. In its meeting minutes, the FDA further emphasized its commitment, stating: "The FDA remains committed to collaborating with the applicant and will exercise further regulatory flexibility by reviewing data from the HOPE-3 trial."

"We are encouraged by the outcome of our discussions with the FDA, which provided clarity on our regulatory strategy and reinforced the opportunity to deliver HOPE-3 data as the basis for approval, should it meet regulatory requirements," said Linda Marbán, Ph.D., Capricor's Chief Executive Officer. "The results from the HOPE-2 and HOPE-2-OLE studies have already demonstrated clinically meaningful and statistically significant benefits in both cardiac and skeletal muscle function, and HOPE-3 is designed to further validate these findings in an adequate and well-controlled study. With HOPE-3 completed and data expected later this year, we remain confident in our ability to advance Deramiocel toward potential approval. Above all, our mission remains unchanged: to bring this therapy to patients and families living with Duchenne as quickly as possible."

Importantly, prior to issuance of the CRL, the majority of the BLA had undergone rigorous review with no significant deficiencies identified by the FDA during the mid-cycle review or pre-licensing inspections. All CMC items identified in the CRL have been addressed and communicated to the FDA. Capricor believes that the addition of HOPE-3 data will further strengthen the clinical package and support the broad potential of Deramiocel as a treatment for DMD.

The Company also maintains a strong financial position to support the advancement of Deramiocel through regulatory review and toward potential launch.

- **Capricor Therapeutics provided update following a Type-A meeting in August 2025 after the receipt of a Complete Response Letter (CRL) the previous month.**
- **It is expected that the FDA will restart the review clock upon Capricor's submission of the HOPE-3 results with a formal complete response to the CRL.**
- **According to Capricor, key outcomes from the meeting include that the HOPE-3 data can be submitted within the current BLA, maintaining PUL v2.0 as the primary efficacy endpoint and suggesting left ventricular ejection fraction (LVEF) as a key secondary endpoint, while maintaining the existing indication for DMD-associated cardiomyopathy with potential opportunity for label expansion.**

Source : press release by Capricor Therapeutics on September 25,2025
Capricor Therapeutics Provides Regulatory Update on Deramiocel Program for Duchenne Muscular Dystrophy Following Type A Meeting :: Capricor Therapeutics, Inc. (CAPR)

CAP-1002 (deramiocel) : U.S. development timeline update

- **Following outcome of discussions with the FDA at the Type-A meeting, Capricor plans to submit HOPE-3 data under the current BLA.**
- **HOPE-3 topline data is expected to be available in the coming weeks (Q4 2025).**

Source : Capricor Therapeutics

	Before CRL (July 15, 2025 announcement)	Latest (as of Nov 10, 2025 announcement)
BLA submission	BLA submission was completed by the end of 2024, and the FDA accepted it in March 2025.	The HOPE-3 data (Cohort A and B) can be submitted within the current BLA
PDUFA (FDA approval) date	August 31, 2025	TBD[1] (Once the FDA accepts the resubmitted BLA, a new PDUFA date will be provided.)
Review period	n/a	Anticipated to be up to six months following resubmission under a Type 2 classification[1]
Expected launch date	As soon as possible post PDUFA	TBD following resubmission and establishment of new PDUFA date[1]
Designations	1. Regenerative Medicine Advanced Therapy 2. Orphan Drug 3. Rare Pediatric Disease	All designations remain valid (regardless of a CRL).
Expected approval type	Full approval with existing cardiac data from the Phase 2 HOPE-2 and HOPE-2 Open Label Extension (OLE) trials compared to natural history data	Full approval with HOPE-3 data added to current BLA. PUL v2.0 remains the primary efficacy endpoint with left ventricular ejection fraction (LVEF) included as secondary endpoint.
Target indication	Cardiomyopathy Associated with Duchenne Muscular Dystrophy (DMD)	Aim to maintain indication of Cardiomyopathy Associated with Duchenne Muscular Dystrophy (DMD) with possible label expansion
Key event(s)	Late-cycle meeting and Advisory Committee were planned.	The HOPE-3 data (Cohort A and B) expected to be available in the coming weeks (Q4 2025).

1. Classification 1 or 2 applies to NDA, BLA, and efficacy supplement resubmissions under PDUFA after a Complete Response Letter. Under Type 2 classification, a resubmission includes items not specified as Class 1, such as major amendments to the original application, and is subject to an FDA review period of up to six months.

PREPARATIONS FOR NEW PRODUCT LAUNCHES IN THE U.S.

Preparations for New Product Launches in the U.S. (1/2)

- **Focus on recruitment activities and reallocating human resources toward the launches of CAP-1002 and RGX-121 in the U.S.**

Focus on recruitment activities

NS Pharma (NSP), the U.S. subsidiary has hired 30 employees since January 2025, planning to add approximately 10 more employees to Commercial Division.

Newly hired Payer Communications, Marketing, and Sales teams are currently preparing for new product launches.

(As of October 2025, NSP has approximately 160 employees. Of these, about 80 are assigned to either the Commercial or Medical Divisions, including 24 Sales Representatives.)

Field team reorganization

Sales Representatives divided between
KAMs (Key Account Managers) and
ABMs (Area Business Managers) to enhance specialization.

Enhancing collaboration among field team
- Sales Representatives (KAM/ABM)
- Insurance Reimbursement Specialists
  (DPA: Director of Patient Access)
- Patient Support Specialists
  (PEL: Patient Engagement Lead)

The reorganization largely completed as of the end of Q2 FY2025 (Until the new product is launched, they focus on Viltepso promotional activities)

This new organization will also handle ATSN-101 and RGX-111 in the future.

Preparations for New Product Launches in the U.S. (2/2)

CAP-1002

Working with Capricor to prepare for market launch based on the upcoming results of HOPE-3 study and BLA package

Market research and drug price/demand forecast

- Conduct market research targeting healthcare professionals, patients, caregivers, and payers
- Research the expected drug price and demand forecast based on the results of the survey

Preparation of sales and support system

- Prepare reimbursement, HUB services, patient support, and disease information materials[1]
- Develop brand strategy and field staff activity plan

Supply chain development

- Establish the ultra cold-chain
- Select eligible hospitals for administration

Patient support for clinical trial participants

- Inquiry to hospitals regarding the switch from investigational product to commercial product for clinical trial participants upon product launch

RGX-121

Identifying hospitals for rapid market penetration amid a limited number of treatment facilities for Mucopolysaccharidosis Type II (MPS II) patients in the U.S.

Selection of facilities for administration

- Identify several accredited hospitals that can manage MPS II patients and handle commercial gene therapy, and begin building relationships with them

Sales strategy, patient advocacy groups, and academic activities

- Exhibited a booth at MPS-related conferences and co-sponsored MPS II patient advocacy groups
- Work with REGENXBIO to update marketing strategy based on additional long-term pivotal data submitted to the FDA

1. HUB (Comprehensive Patient Assistance) aims to support the patient journey, including insurance reimbursement.

R&D PIPELINE

Keiichi Kuwano
Director, Research & Development

R&D Updates (1/2)

For updates from Q1 FY2025 financial results announcement on August 7, 2024, see highlighted text in red.

Update	Code No. (Generic name)	Brand name	Indications and topics	Schedule
P3	NS-065/NCNP-01 (viltolarsen)	Viltepso	・FDA review of the Study 301 report is scheduled to be completed by December, 2025. ・The protocol of Study 303 is currently under review by the FDA.	October 2025
Launch	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
Launch	NS-304 (selexipag)	Uptravi	Uptravi Tablets for Pediatric 0.05 mg	March 2025
Additional indication			pediatric pulmonary arterial hypertension	December 2024
Launch	ACT-064992D (macitentan / tadalafil)	Yuvanci	pulmonary arterial hypertension	November 2024
Filed	RGX-121 (clemidsogene lanparvovec)	–	Mucopolysaccharidosis Type II (FDA review period extended with new PDUFA date[1] of Feb. 8, 2026)	August 2025 (U.S.)
Filed	CAP-1002 (deramiocel)	–	Duchenne muscular dystrophy cardiomyopathy (Capricor received CRL[2] from FDA)	July 2025 (U.S.)
Filed	NS-401 (tagraxofusp)	–	blastic plasmacytoid dendritic cell neoplasm (BPDCN)	March 2025
P3	GA101 (obinutuzumab)	Gazyva	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

1. PDUFA date : the target action date for completion of the review by the FDA
2. 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 Updates (2/2)

For updates from Q1 FY2025 financial results announcement on August 7, 2024, see highlighted text in red.

Update	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)
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.)
Fast Track Designation	NS-229	–	eosinophilic granulomatosis with polyangiitis (EGPA)	September 2025 (U.S.)
Orphan Drug Designation				April 2025 (U.S.)
Orphan Drug Designation	NS-051/NCNP-04	–	Duchenne muscular dystrophy	September 2025 (U.S.)
Rare Pediatric Disease Designation				January 2025 (U.S.)
Senkuteki Iyakuhin (Pioneering Drug) Designation and Orphan Drug Designation	NS-089/NCNP-02 (brogidirsen)	–	Duchenne muscular dystrophy	December 2024 (Japan)
Academic conference presentation			3.5-Year clinical trial data presentation at the World Muscle Society 2025 Congress	October 2025 (U.S.)
Publication			the results of an investigator-initiated clinical trial (First in human trial) in Cell Reports Medicine	January 2025

REFERENCE MATERIALS

Sales Forecast in Pharmaceuticals Segment

- **FY2025 sales forecasts for Viltepso (U.S.) and Vyxeos have been revised downward due to market conditions.**
- **Revenues from the licensing of industrial property rights reflect the impact of changes such as the Medicare Part D program in the U.S., resulting in a downward revision of FY2025 forecast for Uptravi royalties.**

(Million yen)

Brand name/ code no.	Indications	Q2 FY2024	Q2 FY2025	YoY		FY2025 Forecasts		YoY	
		actual	actual	change	%	Previous*	Revised	change	%
Viltepso		11,002	10,349	-652	-5.9%	21,500	21,100	-400	-1.9%
(Japan)	Duchenne muscular dystrophy (DMD)	(2,319)	(2,386)	(+66)	(+2.9%)	(4,800)	(4,800)	-	-
(U.S.)		(8,682)	(7,963)	(-718)	(-8.3%)	(16,700)	(16,300)	(-400)	(-2.4%)
Uptravi	pulmonary arterial hypertension/ chronic thromboembolic pulmonary hypertension	7,474	8,448	+974	+13.0%	16,800	17,000	+200	+1.2%
Vyxeos	high-risk AML	2,236	2,941	+704	+31.5%	7,300	6,500	-800	-11.0%
Gazyva	CD20-positive follicular lymphoma/ CD20-positive chronic lymphocytic leukemia	2,452	2,451	-0	-0.0%	5,200	5,000	-200	-3.8%
Vidaza	myelodysplastic syndrome/ acute myeloid leukemia	2,757	1,818	-939	-34.1%	3,100	3,200	+100	+3.2%
Fintepla	seizures associated with Dravet syndrome/ seizures associated with Lennox-Gastaut syndrome	680	1,765	+1,085	+159.6%	4,000	4,000	-	-
Defitelio	sinusoidal obstruction syndrome	1,277	1,252	-25	-2.0%	2,500	2,500	-	-
Tramal/Onetram	cancer pain, chronic pain	1,470	1,184	-285	-19.4%	2,000	2,200	+200	+10.0%
Cialis	erectile dysfunction	1,219	1,119	-99	-8.2%	2,500	2,300	-200	-8.0%
Erleada	prostate cancer	-	-	-	-	-	6,300	+6,300	-
CAP-1002 deramiocel (U.S.)	DMD cardiomyopathy	-	-	-	-	-	-	-	-
Profit in co-promotion		4,700	4,792	+91	+2.0%	9,600	9,200	-400	-4.2%
Revenues from the licensing of industrial property rights		22,655	24,181	+1,525	+6.7%	47,500	47,000	-500	-1.1%
Revenue		68,496	68,561	+64	+0.1%	143,000	145,000	+2,000	+1.4%

The foreign exchange rate assumed for 2H FY2025 business forecast is ¥140 per USD and its sensitivity indicates that for every ¥1 depreciation of the yen against the USD, revenue is expected to increase by approximately ¥240 million.

* August 7, 2025 (Q1 FY2025 financial results announcement)

Sales Forecast in Functional Food Segment

- **No changes from the previous forecast**

(million yen)	Q2 FY2024		Q2 FY2025		YoY		FY2025
	actual	ratio	actual	ratio	change	%	forecast
Protein preparations	6,893	63.6%	6,861	61.9%	-31	-0.5%	13,900
Preservatives	1,620	15.0%	1,624	14.7%	+3	+0.2%	3,400
Supplements	1,202	11.1%	1,309	11.8%	+106	+8.9%	3,500
Health food ingredients	487	4.5%	621	5.6%	+133	+27.5%	1,100
Others	632	5.8%	669	6.0%	+36	+5.8%	1,100
Revenue	10,836	100.0%	11,086	100.0%	+250	+2.3%	23,000

Consolidated Balance Sheet

(million yen)	End of FY2024	End of Q2 FY2025	YoY change		End of FY2024	End of Q2 FY2025	YoY change
Assets	283,637	297,037	+13,400	Liabilities	36,297	36,826	+529
Current assets	149,740	165,153	+15,413	Current liabilities	30,316	31,027	+710
Non-current assets	133,897	131,884	-2,013	Non-current liabilities	5,980	5,799	-181
				Equity	247,340	260,211	+12,870
Total aseets	283,637	297,037	+13,400	Total liabilities and equity	283,637	297,037	+13,400

Assets

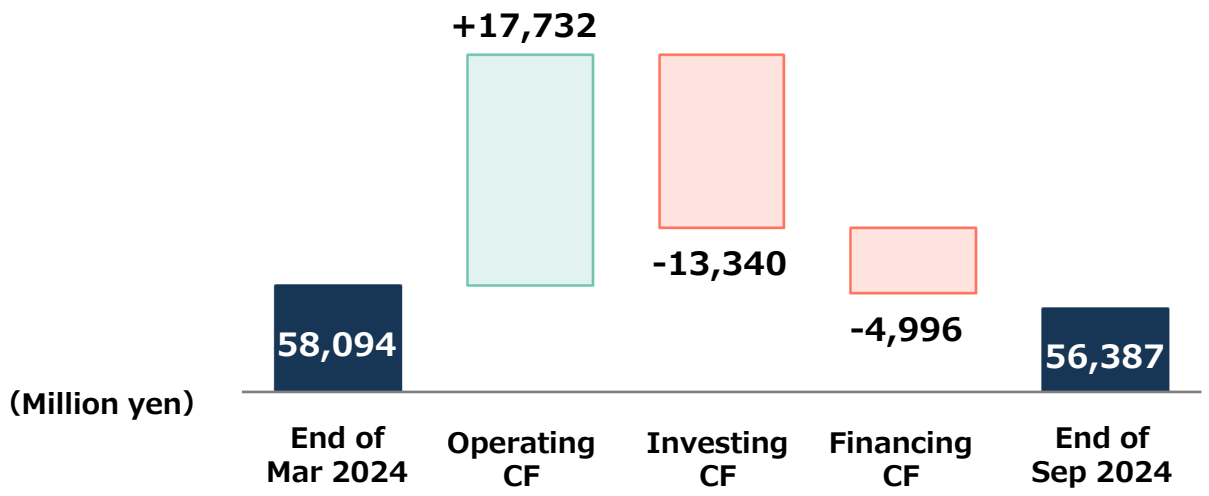
Cash and cash equivalents	+10,050
Inventories	+5,129
Other financial assets (non-current)	-1,255

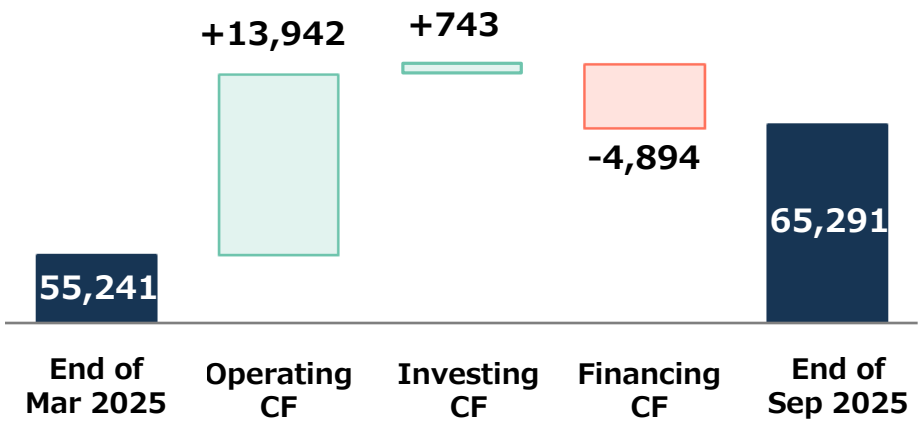
Liabilities and Equity

Trade and other payables	+167
Retained earnings	+13,568

Consolidated Statements of Cash Flows

(million yen)	Q2 FY2024 actual	Q2 FY2025 actual	YoY change
Operating activities	17,732	13,942	-3,790
Investing activities	-13,340	743	+14,083
Financing activities	-4,996	-4,894	+102
Cash and cash equivalents at end of period	56,387	65,291	+8,904





Pipeline（1/2）

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	—	U.S.	NCT03406780[1]
						NCT04428476[2]
Filed	NS-401 (tagraxofusp)	In-license The Menarini Group	blastic plasmacytoid dendritic cell neoplasm	Study Completion : FY2026	Japan	jRCT2031220023
Filed	RGX-121 (clemidsogene lanparvovec)	Partnership REGENXBIO Inc.	Mucopolysaccharidosis Type II	PDUFA date[3] February 8, 2026	U.S.	NCT03566043
P3	ZX008 (fenfluramine hydrochloride)	Distribution partnership 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	jRCT2011210059
			pediatric nephrotic syndrome	Projected submission : CY2026	Japan	NCT05627557
			extra renal lupus	Projected submission : CY2027	Japan	jRCT2071230031
	CAP-1002 (deramiocel)	Partnership Capricor Therapeutics, Inc.	Duchenne muscular dystrophy	—	U.S.	NCT05126758
	LY3527727 (pirtobrutinib)	Alliance agreement Eli Lilly Japan K.K.	mantle cell lymphoma	—	Japan	jRCT2021210026
			chronic lymphocytic leukemia	—	Japan	jRCT2011210061
						jRCT2041210150
						jRCT2021220024

1. The Phase 2 (HOPE-2) study
2. The HOPE-2 Open Label Extension (OLE) study
3. PDUFA date : the FDA's deadline to complete its review of an application

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

Pipeline（2/2）

Stage	Code No. (Generic name)	Origin	Indications	Schedule	Country	ID#
P2	NS-304 (selexipag)	In-house	arteriosclerosis obliterans	Study Completion : FY2025	Japan	jRCT2031210497
	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
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	Study Completion : FY2024	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-863	In-house	cardiovascular diseases	Study Completion : FY2024	Japan	jRCT2071230038

*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**

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 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 (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.**

Supplementary Information : According to the Japanese Society of Hematology, the number of BPDCN cases registered in Japan is approximately 40 per year (Year 2020: 42 cases, 2021: 39 cases, 2022: 45 cases).

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**

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

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**

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

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**

Supplementary Information : The estimated number of patients in Japan is approximately 30,000 for LN, approximately 9,000 for PNS, and approximately 10,000 for ERL.

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 : 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**

Supplementary Information : The number of patients in Japan with intermittent claudication due to ASO is estimated to be approximately 840,000.

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**

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 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.

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**

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

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**

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 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.

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.**

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.

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**

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	• **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

- 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	–

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