FY2025 Third Quarter Financial Results

February 9, 2026
NIPPON SHINYAKU CO., LTD.

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Agenda

01	- Q3 FY2025 Financial Results and Full-Year Forecast - CAP-1002 (deramiocel) Update - RGX-121 Update	Toru Nakai Representative Director, President
02	- R&D Pipeline - Topics	Keiichi Kuwano Director, Research & Development

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Q3 FY2025 FINANCIAL RESULTS AND FULL-YEAR FORECAST

Toru Nakai

Representative Director, President

Highlight

Q3 FY2025 Financial Results

- ✓ Increased revenue in both the pharmaceuticals and functional food businesses, marking the fourth consecutive period of revenue growth
- ✓ Decreased profits due to increases in SG&A expenses

FY2025 Full-Year Forecast

- ✓ Revenue revised upward by ¥2.0 billion due to changes in assumed foreign exchange rates for Q4, etc.
- ✓ Operating profit remained unchanged due to increased R&D expenses and other factors

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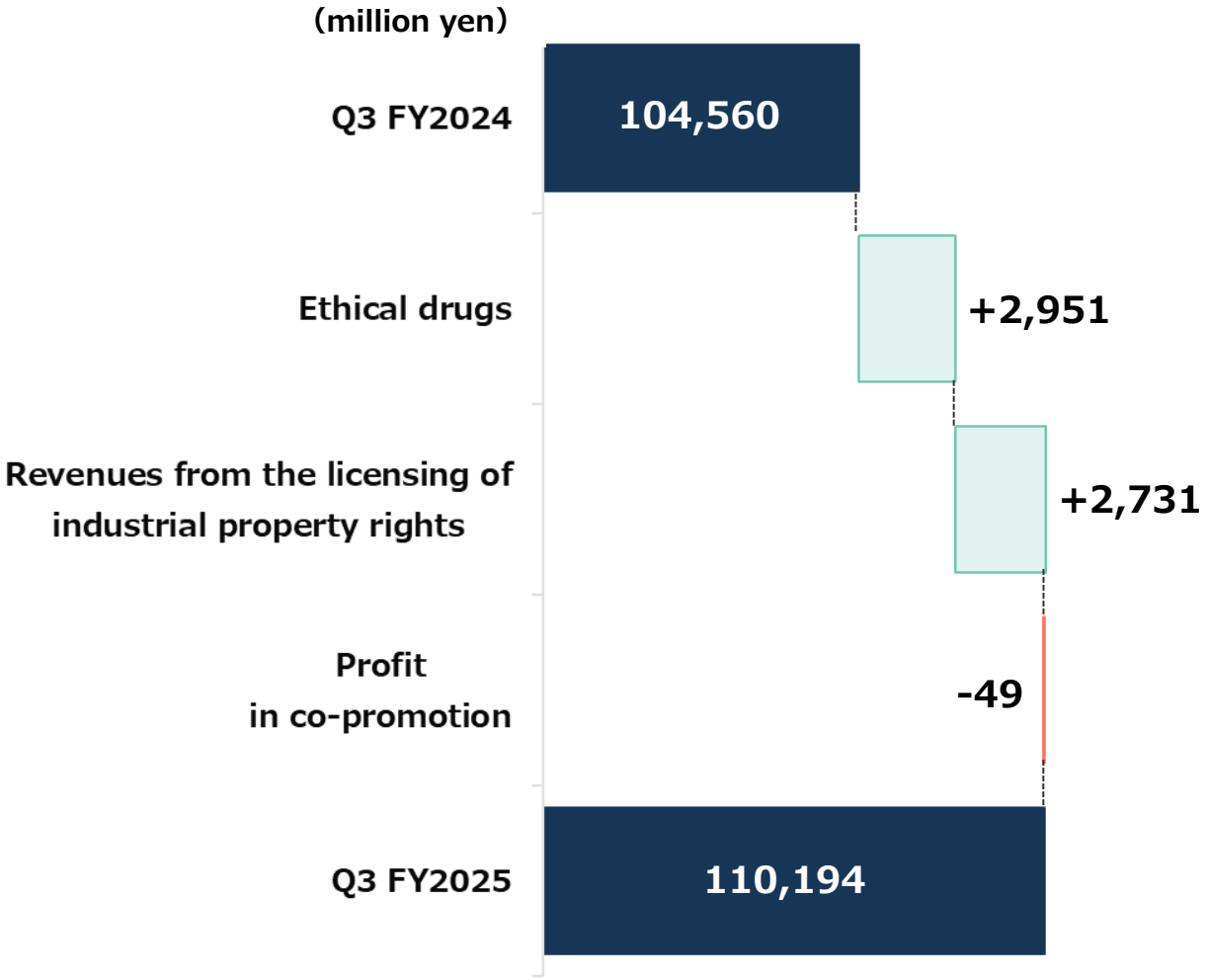
Q3 (Apr - Dec) FY2025 Summary

- **Increased revenue in both the pharmaceuticals and functional food businesses, marking the fourth consecutive period of revenue growth**
- **Increased SG&A expenses led to a decline in both operating profit and profit attributable to owners of parent**

(million yen)	Q3 FY2024		Q3 FY2025		YoY	
	actual	ratio	actual	ratio	change	%
Revenue	**121,320**	**100.0%**	**127,135**	**100.0%**	**+5,815**	**+4.8%**
(Pharmaceuticals)	(104,560)	(86.2%)	(110,194)	(86.7%)	(+5,633)	(+5.4%)
(Functional Food)	(16,759)	(13.8%)	(16,941)	(13.3%)	(+182)	(+1.1%)
Cost of sales	**38,810**	**32.0%**	**42,011**	**33.0%**	**+3,201**	**+8.2%**
SG&A expenses	**27,562**	**22.7%**	**31,704**	**24.9%**	**+4,141**	**+15.0%**
R&D expenses	**23,547**	**19.4%**	**23,033**	**18.1%**	**-514**	**-2.2%**
Other income	**1,725**	**1.4%**	**2,298**	**1.8%**	**+573**	**+33.3%**
(Foreign exchange gain)	(1,058)	(0.9%)	(1,742)	(1.4%)	(+683)	(+64.6%)
Other expenses	**371**	**0.3%**	**351**	**0.4%**	**-20**	**-5.5%**
Operating profit	**32,752**	**27.0%**	**32,333**	**25.4%**	**-419**	**-1.3%**
Finance income	**774**	**0.6%**	**1,014**	**0.8%**	**+239**	**+31.0%**
Finance costs	**89**	**0.1%**	**120**	**0.1%**	**+31**	**+35.3%**
Profit before tax	**33,438**	**27.6%**	**33,227**	**26.1%**	**-210**	**-0.6%**
Income tax expense, etc.	**4,885**	**4.0%**	**7,382**	**5.8%**	**+2,496**	**+51.1%**
Profit attributable to owners of parent	**28,552**	**23.5%**	**25,844**	**20.3%**	**-2,707**	**-9.5%**

Segmental Review - Pharmaceuticals -

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



Ethical drugs 66,308 million yen (+2,951 million yen, +4.7%, YoY)

- ✓ Growth of new product lines including Fintepla, Uptravi, and Vyxeos
- ✓ Through reverse co-promotion, Erleada is now included in revenue for the company[1]

Revenues from the industrial property rights 36,802 million yen (+2,731 million yen, +8.0%, YoY)

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

Profit in co-promotion 7,082 million yen (-49 million yen, -0.7%, YoY)

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

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

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Sales Trends of Viltepso® (viltolarsen)

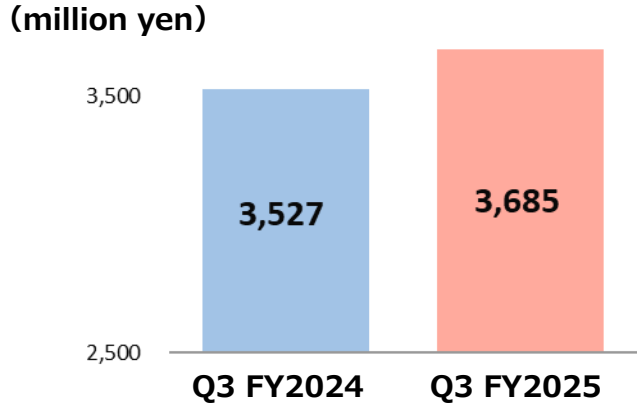
- **Sales of Viltepso in the U.S. declined YoY in Q3 FY2025**
- **The average dose per patient has decreased due to younger patient population**

(million yen)	Q3 FY2024 actual	Q3 FY2025 actual	YoY change	YoY %	FY2025 forecast	Notes on Q3 FY2025 results
Japan	**3,527**	**3,685**	**+157**	**+4.5%**	**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]. ✓ Promoting early diagnosis and intervention for younger patients
US	**12,861**	**12,192**	**-669**	**-5.2%**	**16,400**	✓ Insurance reauthorizations became stricter after launch of multiple DMD treatment options. ✓ The average dose per patient has decreased due to younger patient population .
(million US$)	(84.30)	(81.97)	(-2.33)	(-2.8%)	(110.02)	
Total	**16,389**	**15,877**	**-512**	**-3.1%**	**21,200**	

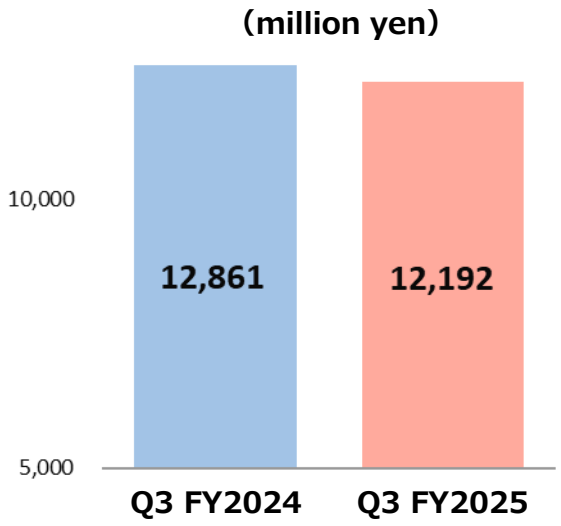
Exchange rates	Q3 FY2024 actual	Q3 FY2025 actual	4Q FY2025 assumption
USDJPY	**152.6**	**148.7**	**150.0**

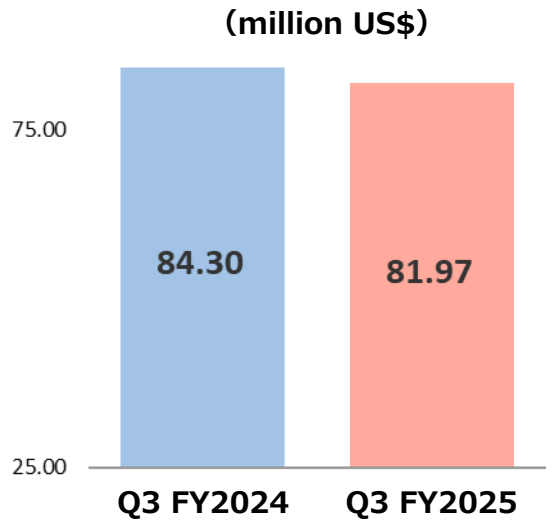
1. Central Social Insurance Medical Council

Japan



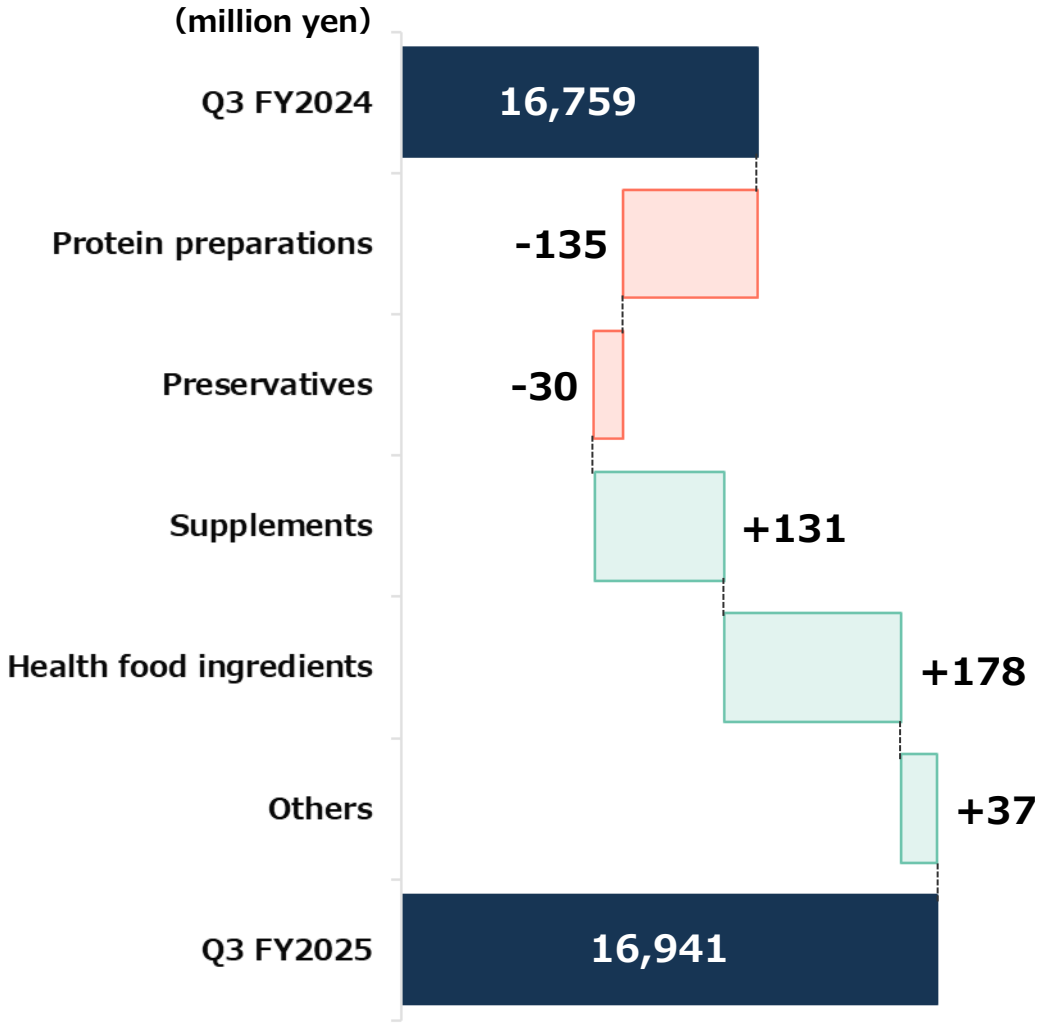
U.S.





Segmental Review - Functional Food -

- **Sales decline in protein preparations and preservatives due to competitive market**
- **Growing health food ingredients amid rising demand for beauty-related products, and solid growth of supplements helped by the market expansion**



Protein preparations 10,420 million yen
(-135 million yen, -1.3%, YoY)

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

Preservatives 2,546 million yen
(-30 million yen, -1.2%, YoY)

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

Supplements 2,025 million yen
(+131 million yen, +7.0%, YoY)

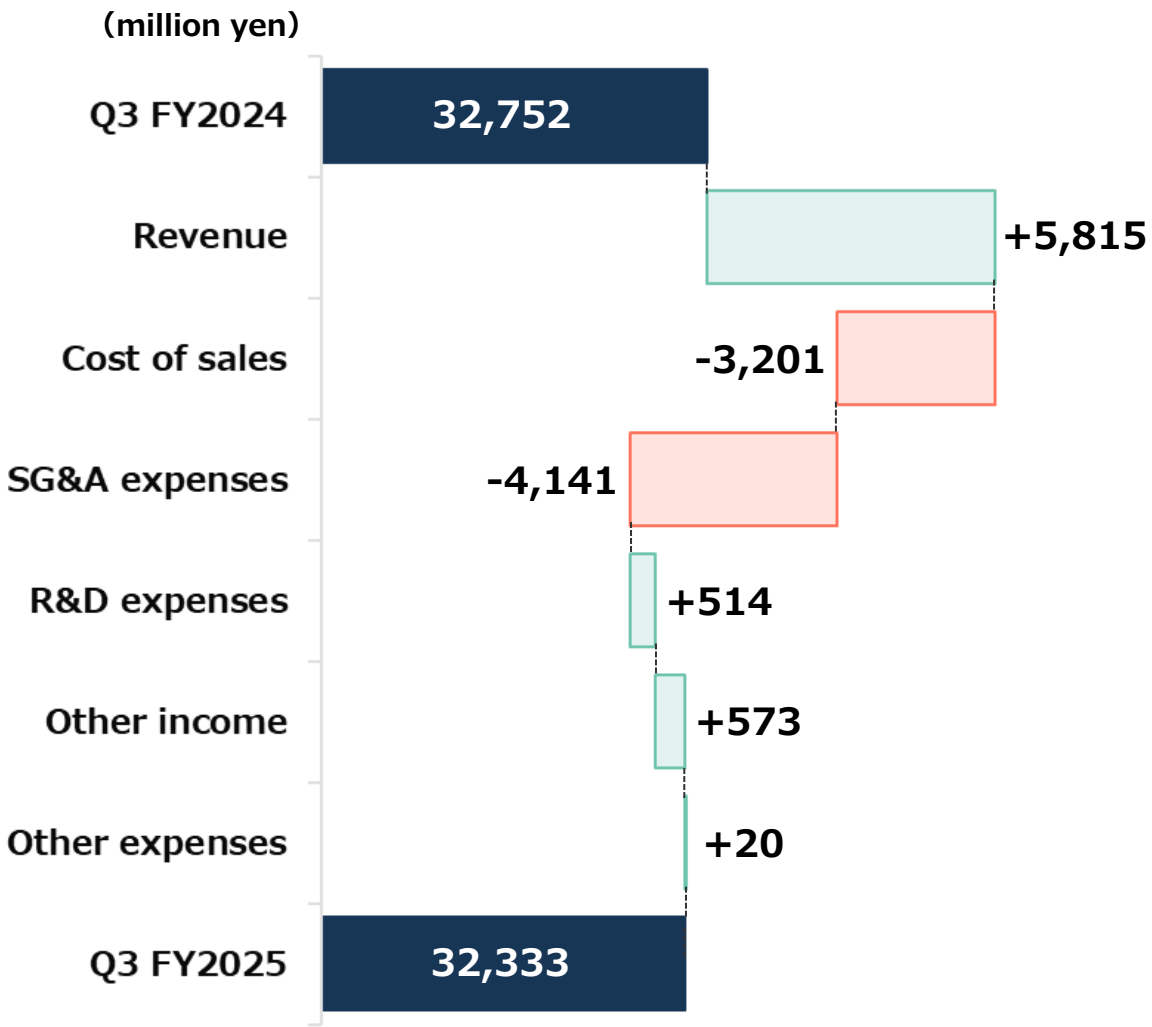
- ✓ Sales increase in both athletes and anti-aging care category

Health food ingredients 927 million yen
(+178 million yen, + 23.8%, YoY)

- ✓ Expanded demand for beauty-related products

Operating Profit

- **Decreased OP due to increases in SG&A expenses**



Revenue 127,135 million yen
(+5,815 million yen, +4.8%, YoY)

- ✓ Growth of new product lines including Fintepla, Uptravi, and Vyxeos
- ✓ 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 42,011 million yen
(+3,201 million yen, +8.2%, YoY)
The ratio was 33.0%, worsened by 1.0 points YoY.

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

SG&A expenses 31,704 million yen
(+4,141 million yen, +15.0%, 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 23,033 million yen
(-514 million yen, -2.2%, YoY)

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

Other income 2,298 million yen
(+573 million yen, +33.3%, YoY)

- ✓ Increase in foreign exchange gains

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Business Forecast : Full-Year Revision from last November

- **Revised revenue upward by ¥2 billion due to factors including changing the assumed foreign exchange rate for Q4 from ¥140 to ¥150 per US dollar**
- **Profit forecast unchanged due to increased R&D expenses and other factors**

(Million yen)	FY2025 Forecasts		YoY	
	Previous*	Revised	change	%
Revenue	**168,000**	**170,000**	**+2,000**	**+1.2%**
(Pharmaceuticals)	(145,000)	**(147,500)**	**(+2,500)**	**(+1.7%)**
(Functional Food)	(23,000)	**(22,500)**	**(-500)**	**(-2.2%)**
Cost of sales	**57,000**	**57,000**	**-**	**-**
SG&A expenses	**43,000**	**43,500**	**+500**	**+1.2%**
R&D expenses	**35,000**	**37,000**	**+2,000**	**+5.7%**
Other income	**1,000**	**1,000**	**-**	**-**
Other expenses	**1,000**	**500**	**-500**	**-50.0%**
Operating profit	**33,000**	**33,000**	**-**	**-**
Finance income	**900**	**1,000**	**+100**	**+11.1%**
Finance costs	**200**	**300**	**+100**	**+50.0%**
Profit before tax	**33,700**	**33,700**	**-**	**-**
Income tax expense, etc.	**7,400**	**7,400**	**-**	**-**
Profit attributable to owners of parent	**26,300**	**26,300**	**-**	**-**

Revenue 170,000 million yen
(+2,000 million yen, +1.2% from previous forecast)

✓ Uptravi's royalty income remained firm, partly due to the weak yen

SG&A expenses 43,500 million yen
(+500 million yen, +1.2% from previous forecast)

✓ Increased costs in preparing for new product launches in the U.S.

R&D expenses 37,000 million yen
(+2,000 million yen, +5.7% from previous forecast)

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

* November 14, 2025 (Q2 FY2025 financial results announcement)

The foreign exchange rate assumed for Q4 FY2025 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 ¥120 million and operating profit is expected to increase by approximately ¥240 million.

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Revised Business Forecast for FY2025 (consolidated)

(million yen)	FY2024 actual	FY2024 ratio	FY2025 forecast	FY2025 ratio	YoY change	YoY %
Revenue	**160,232**	**100.0%**	**170,000**	**100.0%**	**+9,767**	**+6.1%**
(Pharmaceuticals)	(138,654)	(86.5%)	(147,500)	(86.8%)	(+8,845)	(+6.4%)
(Functional Food)	(21,577)	(13.5%)	(22,500)	(13.2%)	(+922)	(+4.3%)
Cost of sales	**51,116**	**31.9%**	**57,000**	**33.5%**	**+5,883**	**+11.5%**
SG&A expenses	**38,011**	**23.7%**	**43,500**	**25.6%**	**+5,488**	**+14.4%**
R&D expenses	**34,341**	**21.4%**	**37,000**	**21.8%**	**+2,658**	**+7.7%**
Other income	**874**	**0.5%**	**1,000**	**0.6%**	**+125**	**+14.3%**
Other expenses	**2,186**	**1.4%**	**500**	**0.3%**	**-1,686**	**-77.1%**
Operating profit	**35,450**	**22.1%**	**33,000**	**19.4%**	**-2,450**	**-6.9%**
Finance income	**830**	**0.5%**	**1,000**	**0.6%**	**+169**	**+20.4%**
Finance costs	**145**	**0.0%**	**300**	**0.2%**	**+154**	**+106.3%**
Profit before tax	**36,135**	**22.6%**	**33,700**	**19.8%**	**-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**	**15.5%**	**-6,258**	**-19.2%**

Foreign exchange rates (USDJPY)

Q3 FY2024 actual	Q3 FY2025 actual	4Q FY2025 assumption
152.6	148.7	150.0

The foreign exchange rate assumed for Q4 FY2025 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 ¥120 million and operating profit is expected to increase by approximately ¥240 million.

CAP-1002 (deramiocel) UPDATE

CAP-1002 (deramiocel) : Regulatory Update

January 20, 2026

Capricor Therapeutics

Capricor Therapeutics Provides Regulatory Update on Deramiocel BLA Following FDA Review of HOPE-3 Topline Data

- *FDA has requested the HOPE-3 clinical study report (CSR) as part of the BLA review process*
- *Company expects to submit updates to the BLA in February 2026 to support continued FDA review*

SAN DIEGO, Jan. 20, 2026 (GLOBE NEWSWIRE) -- Capricor Therapeutics (NASDAQ: CAPR), a biotechnology company developing transformative cell and exosome-based therapeutics for the treatment of rare diseases, today provided a regulatory update regarding its Biologics License Application (BLA) for Deramiocel, the Company's investigational first-in-class cell therapy for the treatment of Duchenne muscular dystrophy (DMD).

As previously disclosed, the Company provided topline results from its Phase 3 HOPE-3 clinical study to the U.S. Food and Drug Administration (FDA) in late 2025. Following its review of these data, the FDA has formally requested the full HOPE-3 clinical study report (CSR) and supporting data to address the Complete Response Letter (CRL). The FDA did not request any additional clinical studies or new patient data as part of this request.

Preparation of the HOPE-3 CSR is well underway, and the Company plans to submit the requested materials to the FDA in February 2026. The Company expects that this submission will address the items outlined in the CRL and support continued review of the BLA, including the assignment of a new Prescription Drug User Fee Act (PDUFA) target action date.

"We are actively engaging with the FDA in order to facilitate an efficient review of the HOPE-3 data that directly address the issues raised in the CRL we received in July 2025. We were pleased that the FDA requested the HOPE-3 clinical study report, as this is an expected and appropriate next step following their initial review of the topline data," said Linda Marbán, Ph.D., Chief Executive Officer of Capricor. "The HOPE-3 results demonstrated statistically significant and clinically meaningful improvements in both skeletal muscle and cardiac function—key drivers of disease progression and long-term outcomes in Duchenne. These findings build on more than a decade of consistent clinical evidence and reinforce our confidence in Deramiocel's potential. Our near-term priority is to address the FDA's request and continue working collaboratively so that patients with late-stage DMD, who currently have very limited treatment options, may gain access to Deramiocel as soon as possible."

- **Capricor Therapeutics provided update following FDA review of HOPE-3 topline data which was submitted in late 2025.**
- **The company plans to submit the requested full HOPE-3 clinical study report (CSR) in February 2026.**
- **After Capricor's submission of the HOPE-3 CSR, it is expected that the FDA will restart the review clock with a new PDUFA target action date.**

Source : press release by Capricor Therapeutics on January 20, 2026

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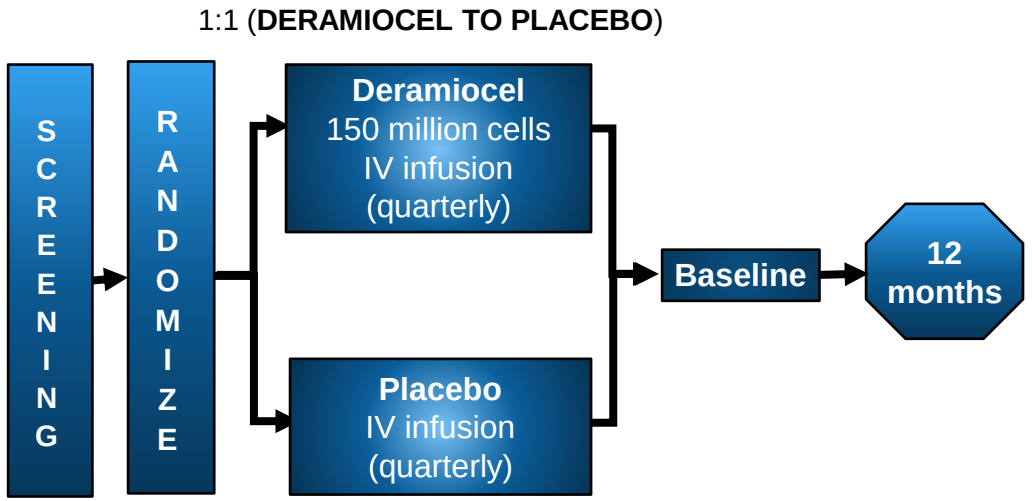
CAP-1002 (deramiocel) HOPE-3 : Trial Design

- **HOPE-3 is a Phase 3, randomized, double-blind, placebo-controlled clinical trial.**
- **The primary efficacy endpoint is the mean change from baseline in upper limb function (PUL v2.0) and the key secondary endpoint is the cardiac MRI assessment at 12 months (LVEF).**

HOPE-3 Pivotal Phase 3 Trial

Overview



HOPE-3
DUCHENNE CLINICAL TRIAL

Design & Endpoints

- Phase 3: randomized (1:1), double-blind, placebo-controlled study
- **N = 106 subjects randomized**
- Conducted in the United States at 20 clinical sites
- **Primary efficacy endpoint**[1]: PUL v2.0
  skeletal muscle assessment
- **Key secondary endpoint**[1]: left ventricular fraction (LVEF)
  cardiac assessment
- **Other secondary endpoints**[1]: mid-level PUL v.2.0, GST[2] and LGE[3]

1. Type-I error controlled
2. GST (Global Statistical Test)
3. LGE (late gadolinium-enhancement)

ClinicalTrials.gov ID: NCT05126758 Source : Capricor Therapeutics, Inc.

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CAP-1002 (deramiocel) HOPE-3 Topline Data : Baseline Demograhpics

- **In HOPE-3, all patients were 10 years of age or older, and approximately 85% of patients in both the placebo group and the Deramiocel group were non-ambulatory.**
- **Approximately 75% of patients had a diagnosis of cardiomyopathy.**

HOPE-3: Study Demographics

Baseline Demographics	Placebo (n=52)	Deramiocel (n=54)	Overall (n=106)[1]
Age (years)			
N	52	54	106
Mean (SD)	14.6 (2.95)	15.4 (3.10)	15.0 (3.04)
Median	14	15	15
Min, Max	10, 22	10, 22	10, 22
PUL v2.0 entry item score			
2,3	23 (44.2)	25 (46.3)	48 (45.3)
4,5,6	29 (55.8)	29 (53.7)	58 (54.7)
Diagnosed cardiomyopathy[2]			
No	14 (26.9)	13 (24.1)	27 (25.5)
Yes	38 (73.1)	41 (75.9)	79 (74.5)
Baseline LVEF%			
n	46	45	91
Mean (SD)	59.303 (6.108)	55.345 (7.743)	57.346 (7.206)
Median	59.309	55.892	57.532
Min, Max	47.395, 73.981	36.537, 71.112	36.537, 73.981
Ambulatory status			
Non-ambulatory	44 (84.6)	46 (85.2)	90 (84.9)
Ambulatory	8 (15.4)	8 (14.8)	16 (15.1)

[1]One subject enrolled but dropped out prior to baseline assessment (n=105)

[2]Updated as of Feb. 2026; subgroup: 64 of 79 patients with centrally reviewed and evaluable cardiac MRI LVEF assessments at baseline and 12 months

Source : Capricor Therapeutics, Inc.

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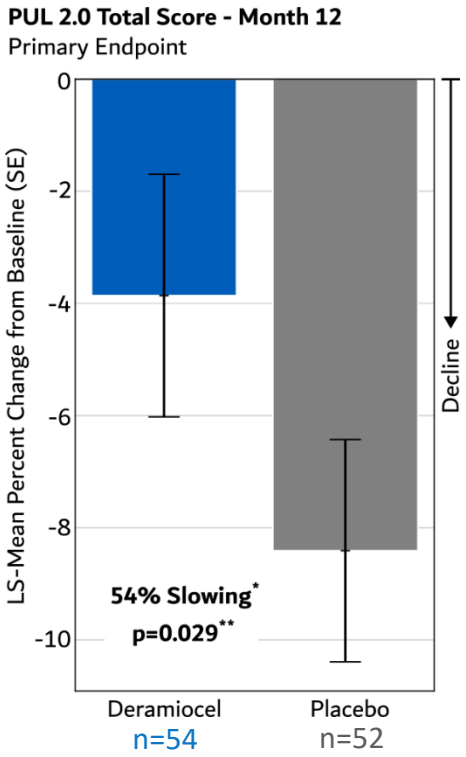
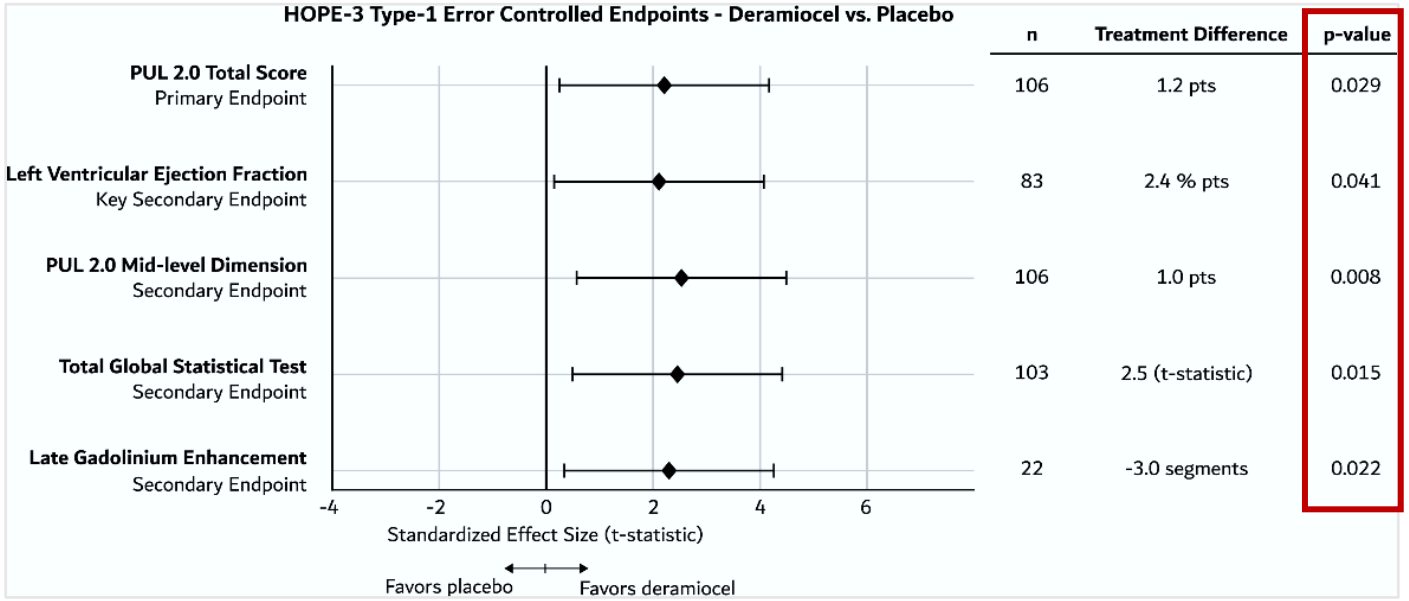
CAP-1002 (deramiocel) HOPE-3 Topline Data : Efficacy

- **HOPE-3 study met the primary endpoint, performance of upper limb (PUL v2.0) and the key secondary cardiac endpoint, left ventricular ejection fraction (LVEF), both achieving statistical significance（p＝0.029 and p＝0.041, respectively)**

HOPE-3: Topline Efficacy Results

Primary Endpoint Met with Statistical Significance Achieved in **All** Type-1 Error Controlled Secondary Endpoints





	n	Treatment Difference	p-value
PUL 2.0 Total Score, Primary Endpoint	106	1.2 pts	0.029
Left Ventricular Ejection Fraction, Key Secondary Endpoint	83	2.4 % pts	0.041
PUL 2.0 Mid-level Dimension, Secondary Endpoint	106	1.0 pts	0.008
Total Global Statistical Test, Secondary Endpoint	103	2.5 (t-statistic)	0.015
Late Gadolinium Enhancement, Secondary Endpoint	22	-3.0 segments	0.022

*LS-Mean difference = 4.55 percentage point (1.2 -point difference on the PUL scale)
** Based on prespecified repeated measures model using percent change from baseline

Source : Capricor Therapeutics, Inc.

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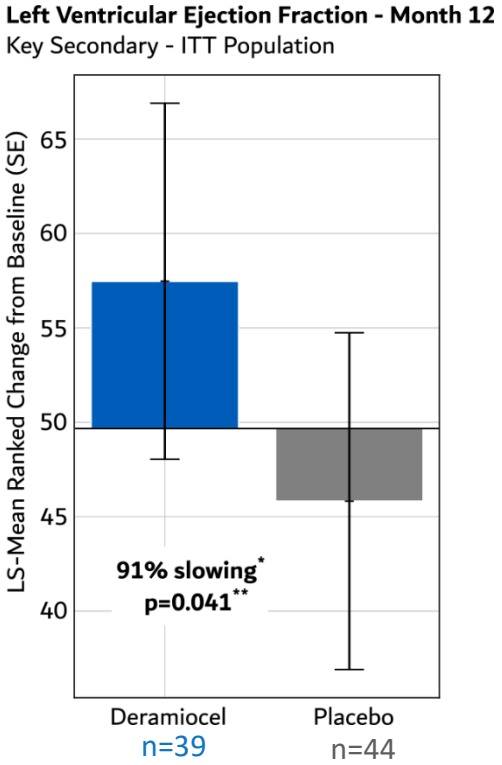
HOPE-3: Topline Cardiac Efficacy Results

Left Ventricular Ejection Fraction



* LS-mean difference = 11.65 ranks (2.4 percentage point difference in LVEF)
** Based on prespecified rank ANCOVA model
LVEF: n reflects the number of patients in the ITT population with centrally reviewed and evaluable cardiac MRI LVEF assessments at baseline and 12 months (n=83)

Source : Capricor Therapeutics, Inc.

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CAP-1002 (deramiocel) HOPE-3 Topline Data : Safety

- **Deramiocel maintained a favorable safety and tolerability profile consistent with results from previous clinical trials**

HOPE-3: Safety Profile Results

Overview	Placebo (n=52), n (%)	Deramiocel (n=53), n (%)	Overall (n=105[1]), n (%)
Any TEAEs	43 (82.7)	50 (94.3)	93 (88.6)
TEAEs related to IP or administration procedure	19 (36.5)	44 (83.0)	63 (60.0)
TEAEs related to IP	16 (30.8)	44 (83.0)	60 (57.1)
TEAEs related to administration procedure	9 (17.3)	23 (43.4)	32 (30.5)
TEAEs related to IP or administration procedure by maximum severity			
Mild (grade 1)	15 (28.8)	19 (35.8)	34 (32.4)
Moderate (grade 2)	3 (5.8)	25 (47.2)	28 (26.7)
Severe (grade 3)	0	0	0
Life-threatening (grade 4)	1 (1.9)	0	1 (1.0)
Fatal (grade 5)	0	0	0
TEAEs leading to death	0	0	0
Any serious TEAEs	5 (9.6)	1 (1.9)	6 (5.7)
Serious TEAEs related to IP or administration procedure	1 (1.9)	1 (1.9)	2 (1.9)

[1] Safety population (n=105)

Source : Capricor Therapeutics, Inc.

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RGX-121 (clemidsogene lanparvovec) UPDATE

NIPPON SHINYAKU CO., LTD.

RGX-121（clemidsogene lanparvovec）Update

NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

January 29, 2026

Regulatory Update on RGX-111 and RGX-121

KYOTO, Japan, January 29, 2026 - Nippon Shinyaku Co., Ltd. (Headquarters: Kyoto, Japan, President: Toru Nakai) announced that REGENXBIO Inc. (REGENXBIO; Headquarters: Rockville, Maryland, USA; CEO: Curran M. Simpson, NASDAQ: RGNX) has received a notification that the U.S. Food and Drug Administration (FDA) placed a clinical hold on RGX-111 and RGX-121.

For more details, please see the press release from REGENXBIO.
https://ir.regenxbio.com/news-releases/news-release-details/regenxbio-announces-regulatory-update-ultra-rare-mps-programs

RGX-111 and RGX-121 are gene therapies being developed by REGENXBIO for the treatment of mucopolysaccharidosis type I (MPS I, Hurler syndrome) and mucopolysaccharidosis type II (MPS II, Hunter syndrome), respectively. The FDA has accepted the Biologics License Application (BLA) filing for RGX-121 in May 2025.

In January 2025, Nippon Shinyaku and REGENXBIO have entered into a strategic partnership for RGX-111 and RGX-121, under which Nippon Shinyaku acquired exclusive commercialization rights in the U.S. and exclusive development and commercialization rights in Asia including Japan. After REGENXBIO obtains BLA approval for each product in the U.S., NS Pharma, Inc. (New Jersey, USA, President: Yukiteru Sugiyama), a wholly owned subsidiary of Nippon Shinyaku, will market them in the U.S.

Source : January 29, 2026 Company press release

NIPPON SHINYAKU CO., LTD.

R&D PIPELINE

Keiichi Kuwano
Director, Research & Development

R&D Updates (1/2)

For updates from Q2 FY2025 financial results announcement on November 14, 2025, see highlighted text in red.

Update	Code No. (Generic name)	Brand name	Indications and topics	Schedule
P3	NS-065/NCNP-01 (viltolarsen)	Viltepso	・CSR for Study 301 under the FDA's review ・Inquiring with FDA regarding the protocol for Study 303	January 2026
Approved	NS-401 (tagraxofusp)	Elzonris	blastic plasmacytoid dendritic cell neoplasm (BPDCN)	December 2025
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
Launch	NS-304 (selexipag)	Uptravi	Uptravi Tablets for Pediatric 0.05 mg	March 2025
Filed	RGX-121 (clemidsogene lanparvovec)	–	Mucopolysaccharidosis Type II (the FDA placed a clinical hold)	January 2026 (U.S.)
Filed	CAP-1002 (deramiocel)	–	Duchenne muscular dystrophy cardiomyopathy (Capricor Therapeutics announced positive topline results from Pivotal Phase Ⅲ HOPE-3 Study)	December 2025 (U.S.)
Revision of electronic package insert	GA101 (obinutuzumab)	Gazyva	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 P1	NS-245	–	Treatment for inflammatory diseases	December 2025

NIPPON SHINYAKU CO., LTD.

R&D Updates (2/2)

For updates from Q2 FY2025 financial results announcement on November 14, 2025, see highlighted text in red.

Update	Code No. (Generic name)	Brand name	Indications and topics	Schedule
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				April 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

NIPPON SHINYAKU CO., LTD.

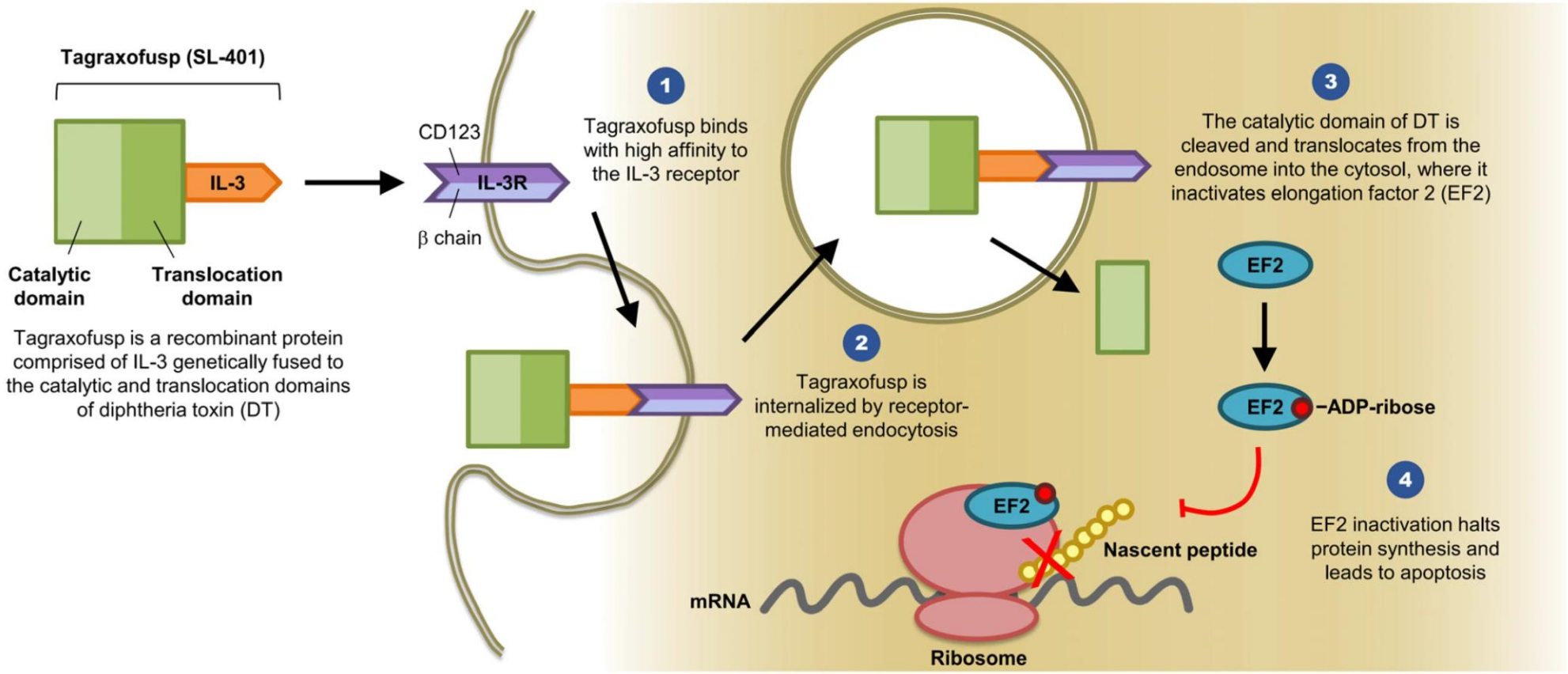
Elzonris® : Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN)

- **Blastic plasmacytoid dendritic cell neoplasm (BPDCN) is a rare hematopoietic tumor with a poor prognosis that originates from plasmacytoid dendritic cells (pDC)**
- **The median age at diagnosis is around 70 years, and the number of patients is extremely small.**
- **Currently, there are no drugs indicated for BPDCN in Japan, nor is there a Standard of Care for the disease. The median survival time with existing therapies is 7-13 months, which is a poor prognosis, and the number of patients who can be treated with intensive chemotherapy is limited. Therefore, new treatment options are needed.**

Product Overview	
Development code number	NS-401
Generic name	tagraxofusp (genetical recombination)
Product name	Elzonris® 1000μg for intravenous infusion
Manufacturing and marketing approval	December 22, 2025
Indications	Blastic plasmacytoid dendritic cell neoplasm (BPDCN)
Origin	The Menarini Group (Italy) Elzonris has been approved in over 40 countries. In March 2021, Nippon Shinyaku acquired the rights in Japan.

Pharmacological Action of Elzonris®

- **The first drug targeting CD123**
- **While minimizing toxicity to normal tissues, Elzonris exhibits selective cytotoxic activity against BPDCN cells expressing CD123 (IL-3Rα).**



Clinical Activity and Tolerability of SL-401 (Tagraxofusp): Recombinant Diphtheria Toxin and Interleukin-3 in Hematologic Malignancies - PubMed

NIPPON SHINYAKU CO., LTD.

VILTEPSO UPDATE

NIPPON SHINYAKU CO., LTD.

Background Information of Viltepso®

<Clinical Trial>

- Japan Phase I/II trials in (2016-2017)
- U.S. Phase II (Study 201: 2016-2018) and its extension study (Study 202)

  ↳ Results:
  Expressions of dystrophin protein were found in skeletal muscles of enrolled DMD patients. Significant differences were observed in multiple endpoints compared to the natural history population, suggesting improved motor function.

- Global Phase Ⅲ (Study 301) **Confirmatory trial required as a condition for approval**

  ↳ Results:
  No statistically significant differences were observed between the viltolarsen group and the placebo group for the primary endpoint (time to stand:TTSTAND) or the secondary endpoints (time to run/walk 10 meters, time to climb 4 stairs, six-minute walk test, NSAA).

<Approval and Sales>

Japan: Conditional Early Approval in March 2020, followed by product launch in May of the same year

U.S. : Accelerated Approval and product launch in August 2020

***The CSR (Clinical Study Report) for Study 301 and the protocol for the additional Phase III study (Study 303) have been submitted to the FDA. Currently inquiring with the FDA regarding the conduct of Study 303.**

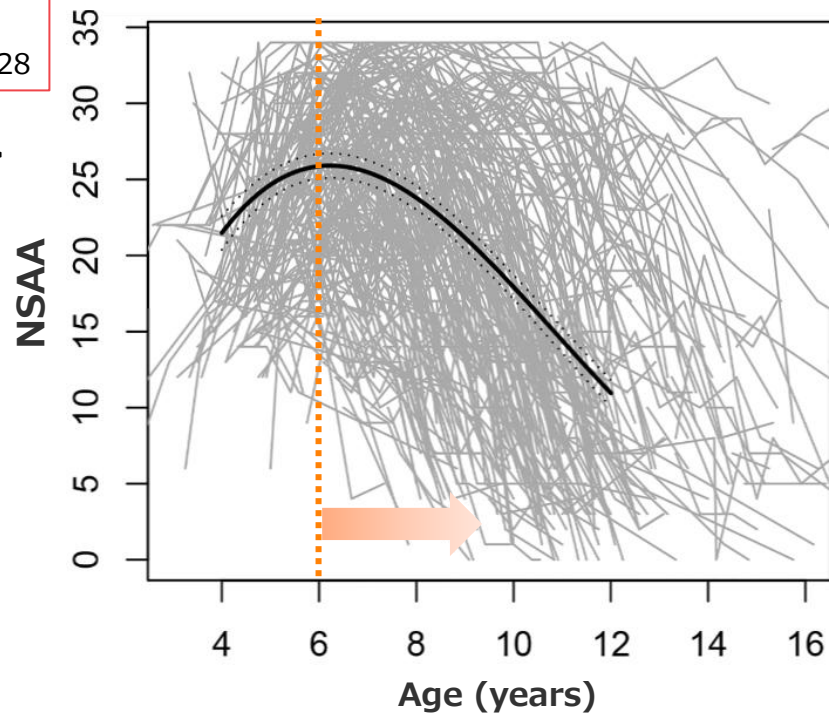
The design of the additional Phase Ⅲ study (Study 303)

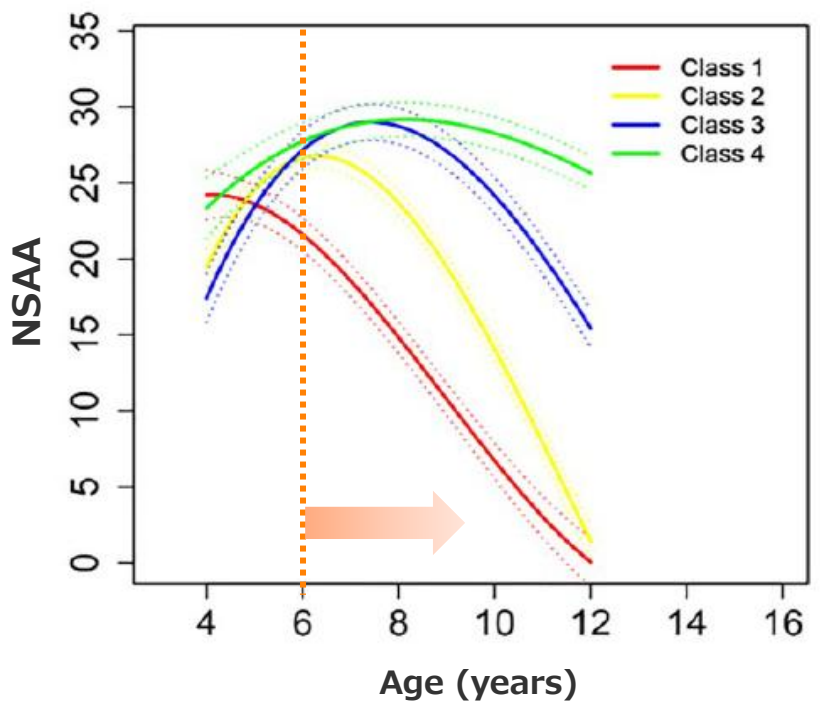
From November 15, 2024
Outline of Consolidated Financial Results for the 2nd Quarter (Interim Period) Ended September 30, 2024, p.28

<Age of the subject patient >
6 years old and up

<Primary endpoint>
NSAA

<Duration of treatment>
96 weeks





(PLOS ONE 2019, Muntoni)

<Inclusion criteria and conditions>

- ✓ Define the range of baseline NSAA scores
  - ↳ Reduce heterogeneity of motor function changes due to patient disease progression
- ✓ Strictly specified doses/regimens for steroids throughout the pre-dose period and study period

*This slide is based our current assumption. The final study design will be determined after consultation with the FDA in the future.

NIPPON SHINYAKU CO., LTD.

REFERENCE MATERIALS

Sales Forecast in Pharmaceuticals Segment

- **Fintepla is seeing an increase in the number of patients receiving treatment, including the impact of the adjustment surcharge (15% surcharge rate) in the FY2025 NHI drug price revision.**
- **Revenues from the licensing of industrial property rights have been revised upward due to the impact of changes in foreign exchange rate assumptions.**

(Million yen)

Brand name/ code no.	Indications	Q3 FY2024	Q3 FY2025	YoY		FY2025 Forecasts		YoY	
		actual	actual	change	%	Previous*	Revised	change	%
Viltepso		16,389	15,877	-512	-3.1%	21,100	21,200	+100	+0.5%
(Japan)	Duchenne muscular dystrophy (DMD)	(3,527)	(3,685)	(+157)	(+4.5%)	(4,800)	(4,800)	-	-
(U.S.)		(12,861)	(12,192)	(-669)	(-5.2%)	(16,300)	(16,400)	(+100)	(+0.6%)
Uptravi	pulmonary arterial hypertension/ chronic thromboembolic pulmonary hypertension	11,623	13,188	+1,564	+13.5%	17,000	17,300	+300	+1.8%
Vyxeos	high-risk AML	3,737	4,562	+824	+22.1%	6,500	6,000	-500	-7.7%
Gazyva	CD20-positive follicular lymphoma/ CD20-positive chronic lymphocytic leukemia	3,805	3,950	+144	+3.8%	5,000	5,000	-	-
Fintepla	seizures associated with Dravet syndrome/ seizures associated with Lennox-Gastaut syndrome	1,384	3,098	+1,714	+123.8%	4,000	4,200	+200	+5.0%
Erleada	prostate cancer	-	3,039	+3,039	-	6,300	6,300	-	-
Vidaza	myelodysplastic syndrome/ acute myeloid leukemia	4,111	2,624	-1,486	-36.2%	3,200	3,200	-	-
Defitelio	sinusoidal obstruction syndrome	2,090	2,090	+0	+0.0%	2,500	2,500	-	-
Tramal/Onetram	cancer pain, chronic pain	2,179	1,846	-333	-15.3%	2,200	2,200	-	-
Cialis	erectile dysfunction	1,932	1,753	-179	-9.3%	2,300	2,300	-	-
CAP-1002 deramiocel (U.S.)	DMD cardiomyopathy	-	-	-	-	-	-	-	-
Profit in co-promotion		7,132	7,082	-49	-0.7%	9,200	9,000	-200	-2.2%
Revenues from the licensing of industrial property rights		34,071	36,802	+2,731	+8.0%	47,000	49,000	+2,000	+4.3%
Revenue		104,560	110,194	+5,633	+5.4%	145,000	147,500	+2,500	+1.7%

The foreign exchange rate assumed for Q4 FY2025 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 ¥120 million.

* November 14, 2025 (Q2 FY2025 financial results announcement)

Sales Forecast in Functional Food Segment

- **Supplement sales have been revised downward. Rising raw material costs have been passed on to prices, leading to consumer hesitation in purchasing.**

(million yen)	Q3 FY2024		Q3 FY2025		YoY		FY2025 Forecasts		YoY	
	actual	ratio	actual	ratio	change	%	Previous*	Revised	change	%
Protein preparations	10,556	63.0%	10,420	61.5%	-135	-1.3%	13,900	14,000	+100	+0.7%
Preservatives	2,576	15.4%	2,546	15.0%	-30	-1.2%	3,400	3,400	-	-
Supplements	1,893	11.3%	2,025	12.0%	+131	+7.0%	3,500	2,700	-800	-22.9%
Health food ingredients	748	4.5%	927	5.5%	+178	+23.8%	1,100	1,200	+100	+9.1%
Others	983	5.8%	1,021	6.0%	+37	+3.9%	1,100	1,200	+100	+9.1%
Revenue	16,759	100.0%	16,941	100.0%	+182	+1.1%	23,000	22,500	-500	-2.2%

* November 14, 2025 (Q2 FY2025 financial results announcement)

NIPPON SHINYAKU CO., LTD.

Consolidated Balance Sheet

(million yen)	End of FY2024	End of Q3 FY2025	YoY change		End of FY2024	End of Q3 FY2025	YoY change
Assets	283,637	328,108	+44,470	Liabilities	36,297	43,911	+7,614
Current assets	149,740	171,523	+21,783	Current liabilities	30,316	30,408	+92
Non-current assets	133,897	156,584	+22,687	Non-current liabilities	5,980	13,503	+7,522
				Equity	247,340	284,196	+36,856
Total aseets	283,637	328,108	+44,470	Total liabilities and equity	283,637	328,108	+44,470

Assets

Cash and cash equivalents	+5,529
Trade and other receivables	+11,369
Inventories	+6,114
Other financial assets (non-current)	+23,914

Liabilities and Equity

Trade and other payables	+2,050
Deferred tax liabilities	+7,140
Retained earnings	+19,805

NIPPON SHINYAKU CO., LTD.

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]
						NCT05126758[2]
Preparation for launch	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	Under clinical hold[3]	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 Phase 3 (HOPE-3) study
3. The FDA placed a clinical hold on RGX-111 and RGX-121 in January 2026

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

NIPPON SHINYAKU CO.,LTD.

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 (friluglanstat)	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	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-863	In-house	cardiovascular diseases	Study completion : FY2024	Japan	jRCT2071230038
	NS-245	In-house	Inframmatory diseases	Study completion : FY2026	Japan	jRCT2071250086

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

1. The FDA placed a clinical hold on RGX-111 and RGX-121 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	• **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 (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 : Approved and in preparation for launch
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.

NIPPON SHINYAKU CO., LTD.

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

NIPPON SHINYAKU CO., LTD.

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	**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 840,000.

NIPPON SHINYAKU CO., LTD.

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

NIPPON SHINYAKU CO., LTD.

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 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.

NIPPON SHINYAKU CO., LTD.

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.

NIPPON SHINYAKU CO., LTD.

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 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.

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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	–

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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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NS-245

- Treatment for inflammatory diseases

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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