FY2026 First Quarter Financial Results

August 6, 2026
NIPPON SHINYAKU CO., LTD.

NIPPON SHINYAKU CO., LTD.

Agenda

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- Q1 FY2026 Financial Results
- CAP-1002 (deramiocel) Update
- Initiatives to Drive Growth and Improve Profitability

Toru Nakai

Representative Director, President

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- R&D Pipeline
- Viltepso P3 Update

Keiichi Kuwano

Director, Research & Development

Q1 FY2026 Financial Results

Toru Nakai
Representative Director, President

Highlight

Q1 FY2026 Financial Results

- ✓ Increased revenue for the fourth consecutive period (strong performance in both the Pharmaceuticals and Functional Food businesses)
- ✓ Increased operating profit
- ✓ No changes from the FY2026 Business Forecast announced on May 15, 2026

Initiatives to Drive Growth and Improve Profitability

- ✓ Expanding the development pipeline to prioritize overcoming the patent cliff
- ✓ Addition of pipelines scheduled for launch during the 7th Five-Year Medium Term Management Plan period

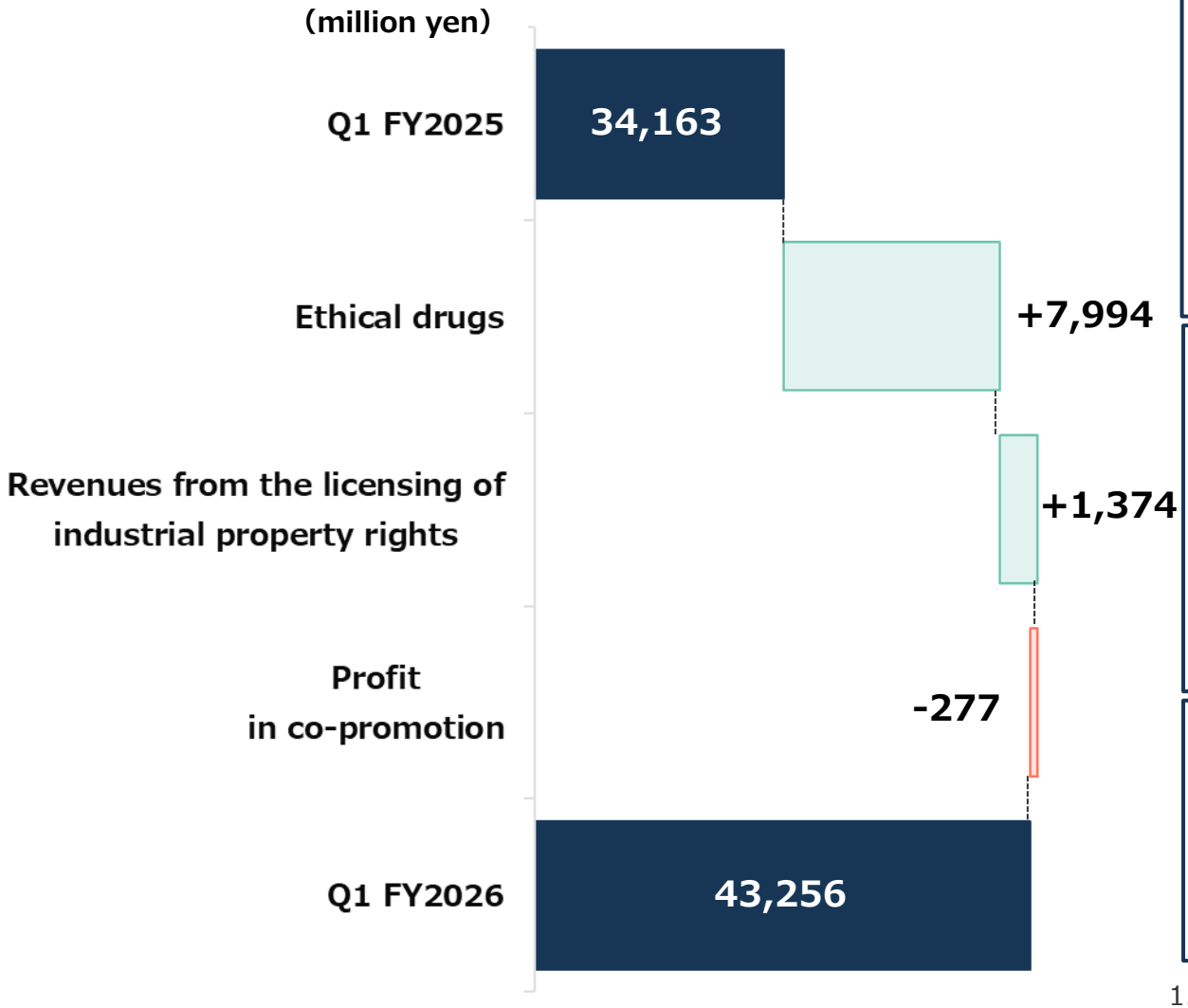
Q1 FY2026 Summary (consolidated)

- **As a result of strong performance in both the Pharmaceuticals and Functional Food businesses, revenue increased for the fourth consecutive period.**
- **Although various expenses increased, profits rose due to factors such as foreign exchange gains.**

(million yen)	Q1 FY2025		Q1 FY2026		YoY	
	actual	ratio	actual	ratio	change	%
Revenue	**39,546**	**100.0%**	**49,066**	**100.0%**	**+9,520**	**+24.1%**
(Pharmaceuticals)	(34,163)	(86.4%)	(43,256)	(88.2%)	(+9,092)	(+26.6%)
(Functional Food)	(5,382)	(13.6%)	(5,810)	(11.8%)	(+428)	(+8.0%)
Cost of sales	12,655	32.0%	17,726	36.1%	+5,070	+40.1%
SG&A expenses	9,995	25.3%	11,075	22.6%	+1,080	+10.8%
R&D expenses	6,189	15.7%	9,197	18.7%	+3,008	+48.6%
Other income	169	0.4%	1,364	2.8%	+1,194	+703.6%
(Foreign exchange gain)	-	-	(727)	(1.5%)	(+727)	-
Other expenses	794	1.9%	119	0.3%	-674	-85.0%
(Foreign exchange loss)	(645)	(1.6%)	-	-	(-645)	-
Operating profit	**10,081**	**25.5%**	**12,311**	**25.1%**	**+2,230**	**+22.1%**
Finance income	468	1.2%	689	1.4%	+220	+47.1%
Finance costs	46	0.1%	41	0.1%	-4	-10.1%
Profit before tax	**10,504**	**26.6%**	**12,959**	**26.4%**	**+2,455**	**+23.4%**
Income tax expense, etc.	2,248	5.7%	2,850	5.8%	+601	+26.8%
Profit attributable to owners of parent	**8,255**	**20.9%**	**10,109**	**20.6%**	**+1,853**	**+22.5%**

Segmental Review - Pharmaceuticals -

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



Ethical drugs 28,129 million yen (+7,994 million yen, +39.7%, YoY)

- ✓ Growth of Uptravi, Fintepla and other products
- ✓ Erleada[1], which is recognized in our product sales through a reverse co-promotion arrangement, also contributed to the segment revenue.

Revenues from the licensing of industrial property rights 12,885 million yen (+1,374 million yen, +11.9%, YoY)

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

Profit in co-promotion 2,240 million yen (-277 million yen, -11.0%, YoY)

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

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

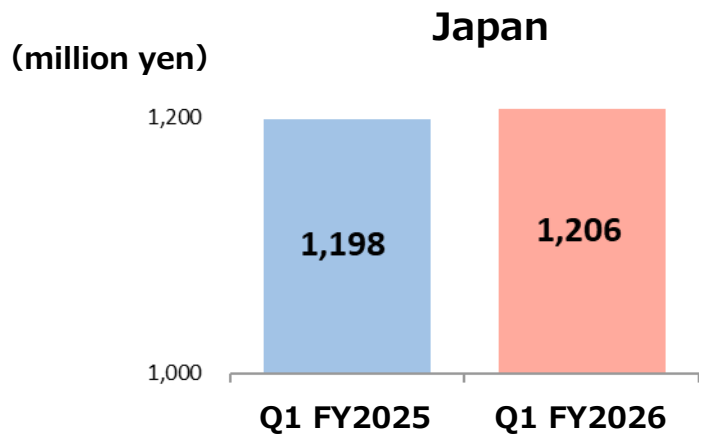
Sales Trends of Viltepso® (viltolarsen)

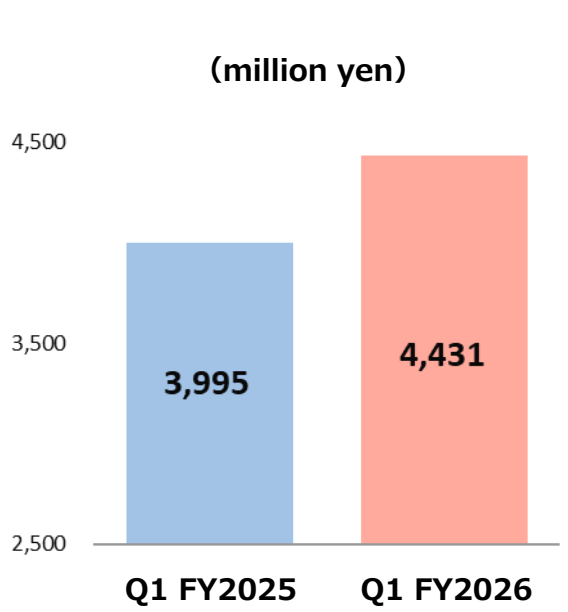
- **Expected U.S. sales growth due to new patient starts, increased dosing as treated patients gain weight, among other factors.**

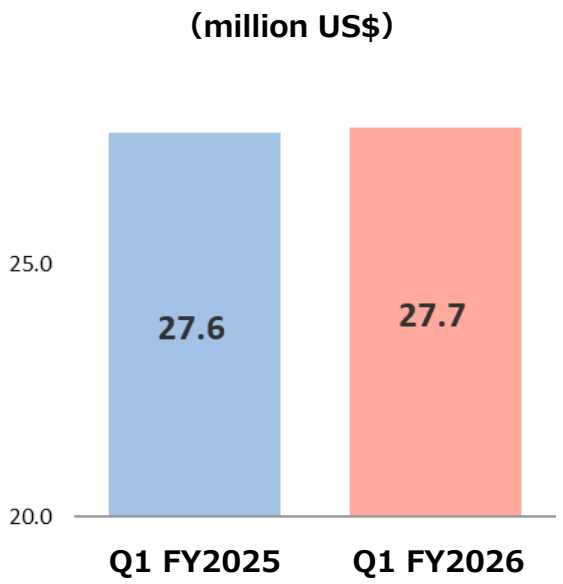
(million yen)	Q1 FY2025 actual	Q1 FY2026 actual	YoY change	YoY %	FY2026 forecast	Notes on Q1 FY2026 results
Japan	**1,198**	**1,206**	**+7**	**+0.6%**	**4,800**	✓ The number of patients currently on therapy with Viltepso is more than three-quarters of the peak number of 128 patients in the data from Chuikyo[1]. ✓ Promoting early diagnosis and intervention for younger patients
US	**3,995**	**4,431**	**+435**	**+10.9%**	**17,500**	✓ Insurance reauthorizations became stricter after launch of multiple DMD treatment options. ✓ Expected sales growth due to new patient starts, increased dosing as treated patients gain weight, among other factors.
(million US$)	(27.6)	(27.7)	(+0.1)	(+0.5%)	(116.6)	
Total	**5,194**	**5,637**	**+443**	**+8.5%**	**22,300**	.

1. Central Social Insurance Medical Council

Exchange rates	Q1 FY2025 actual	Q1 FY2026 actual	FY2026 assumption
USDJPY	**144.6**	**159.5**	**150.0**

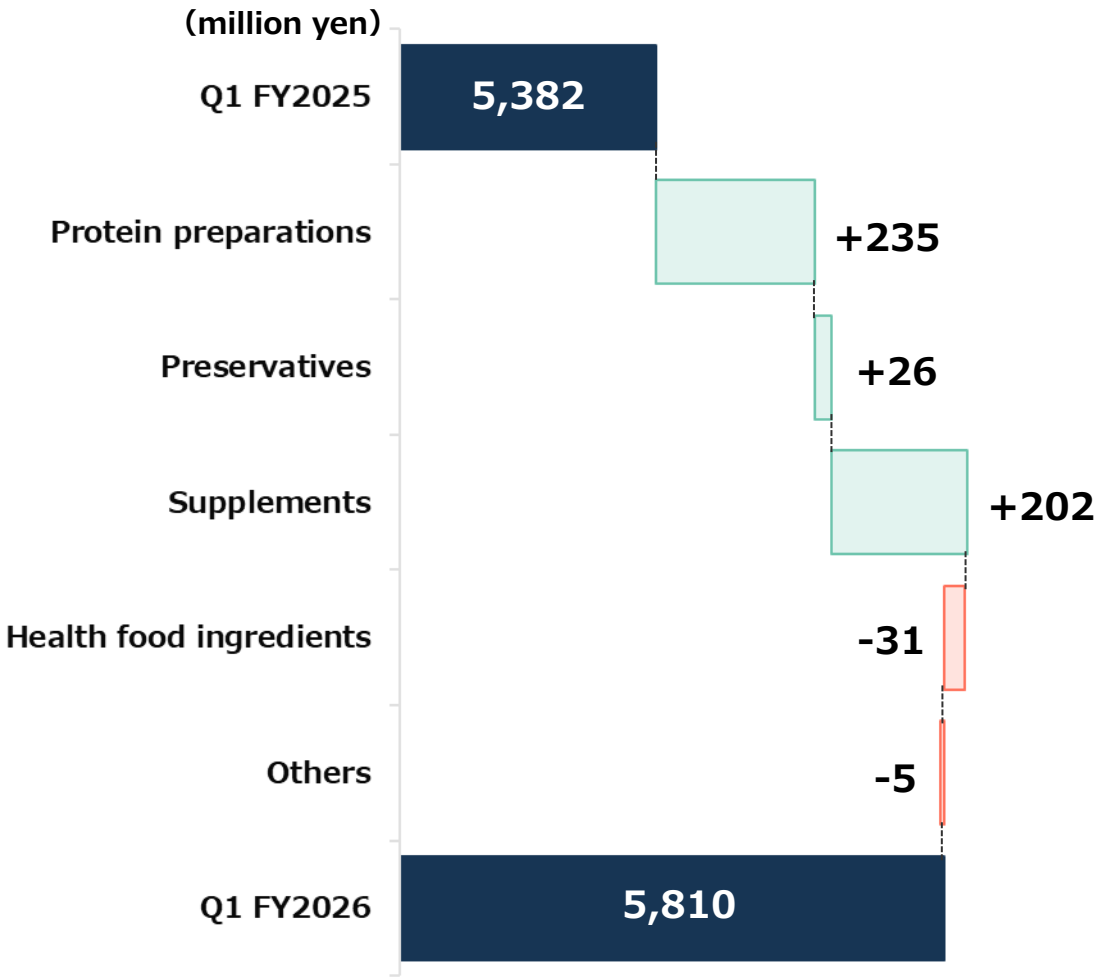






Segmental Review - Functional Food -

- **Increased segment revenue due to the launch of new products and strengthened sales activities**
- **Strong performance in core categories such as milk protein and sports supplements**



Protein preparations 3,554 million yen
(+235 million yen, +7.1%, YoY)

✓ Strong sales of protein powder

Preservatives 823 million yen
(+26 million yen, +3.3%, YoY)

✓ Sales of shelf-life extenders and glycine increased due to promotional efforts aimed at expanding usage among major customers.

Supplements 815 million yen
(+202 million yen, +33.1%, YoY)

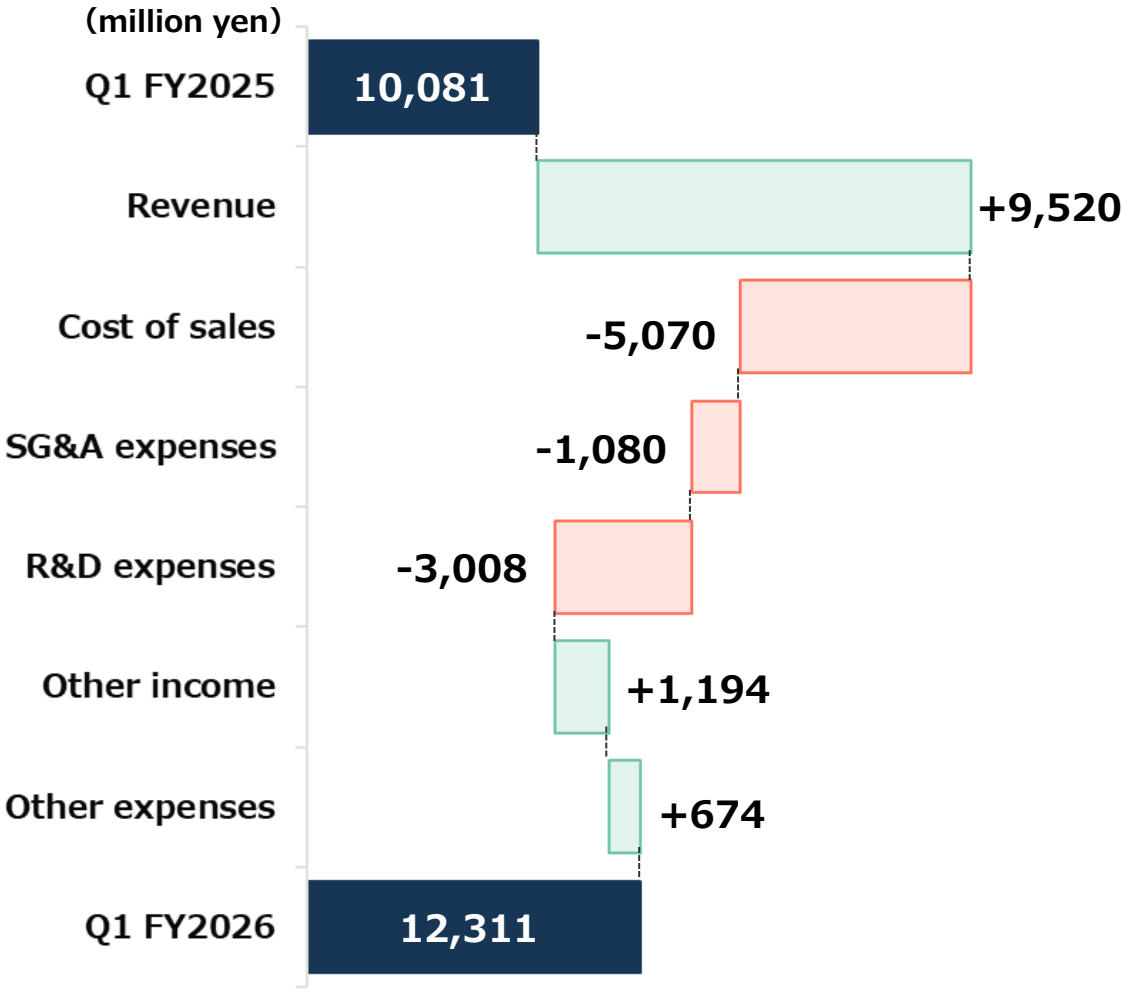
✓ Sales of athletes category are growing due to the expansion of the protein market.
✓ The anti-aging care category is also performing well.

Health food ingredients 278 million yen
(-31 million yen, -10.1%, YoY)

✓ Sales to major customers have declined.

Operating Profit

- **Profit increased due to higher revenue and foreign exchange gains.**



Revenue 49,066 million yen
(+9,520 million yen, +24.1%, YoY)

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

Cost of sales 17,726 million yen
(+5,070 million yen, +40.1%, YoY)
The ratio was 36.1%, worsened by 4.1 points YoY.

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

SG&A expenses 11,075 million yen
(+1,080 million yen, +10.8%, YoY)

- ✓ Increase in the U.S. sales expenses of NS Pharma

R&D expenses 9,197 million yen
(+3,008 million yen, +48.6%, YoY)

- ✓ Increase in contract research expense, etc.
- ✓ Upfront Payment for the Option Agreement for EXG-7001

Other income 1,364 million yen
(+1,194 million yen, +703.6%, YoY)

- ✓ Increase in foreign exchange gains, etc.

Business Forecast for FY2026 (consolidated)

(million yen)	FY2025 Q1 actual	FY2025 FY actual	FY2026 Q1 actual	FY2026 progress in 1H	FY2026 1H forecast	FY2026 FY forecast
Revenue	**39,546**	**170,771**	**49,066**	**51.6%**	**95,000**	**200,000**
(Pharmaceuticals)	(34,163)	(148,484)	(43,256)	(51.8%)	(83,500)	(176,500)
(Functional Food)	(5,382)	(22,287)	(5,810)	(50.5%)	(11,500)	(23,500)
Cost of sales	**12,655**	**57,460**	**17,726**		**33,800**	**73,000**
SG&A expenses	**9,995**	**43,565**	**11,075**		**23,500**	**47,500**
R&D expenses	**6,189**	**36,713**	**9,197**		**19,500**	**40,500**
Other income	**169**	**3,025**	**1,364**		**300**	**1,000**
Other expenses	**794**	**560**	**119**		**1,000**	**2,000**
Operating profit	**10,081**	**35,496**	**12,311**	**70.4%**	**17,500**	**38,000**
Finance income	**468**	**1,132**	**689**		**400**	**800**
Finance costs	**46**	**166**	**41**		**100**	**200**
Profit before tax	**10,504**	**36,462**	**12,959**	**72.8%**	**17,800**	**38,600**
Income tax expense, etc.	**2,248**	**6,741**	**2,850**		**3,800**	**8,300**
Profit attributable to owners of parent	**8,255**	**29,721**	**10,109**	**72.2%**	**14,000**	**30,300**

Foreign exchange rates (USDJPY)

Q1 FY2025 actual	Q1 FY2026 actual	FY2026 assumption
144.6	159.5	150.0

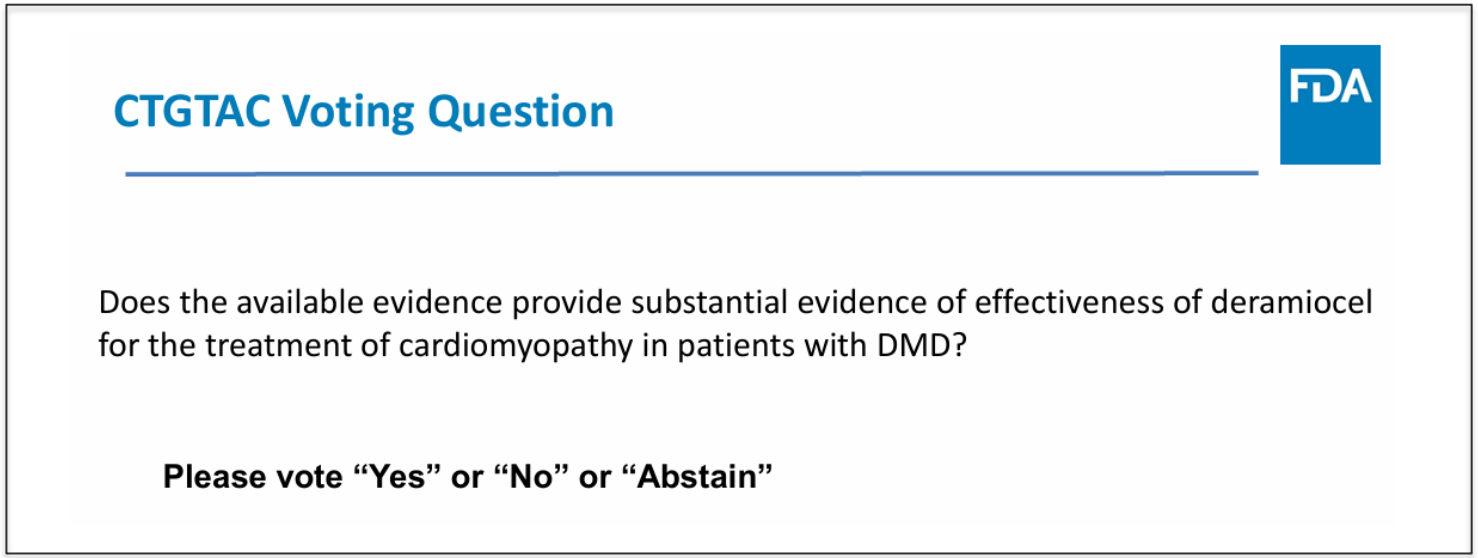
The sensitivity of the exchange rate after Q1 FY2026 is assumed to be an increase of approx. 420 million yen in revenue and approx. 360 million yen in operating profit for every 1 yen depreciation of the yen.

CAP-1002 (deramiocel) Update

CAP-1002 (deramiocel) : Regulatory Update

- **On July 29, 2026, the U.S. Food and Drug Administration (FDA) Cellular, Tissue, and Gene Therapies Advisory Committee met to discuss CAP-1002 (deramiocel).**

- **In response to the CTGTAC voting question, “Does the available evidence provide substantial evidence of effectiveness of deramiocel for the treatment of cardiomyopathy in patients with DMD?”, the final vote by the 12 advisory committee members was 3 votes for “Yes,” 9 votes for “No,” and 0 abstentions.**



Source : The U.S. Food and Drug Administration
CTGTAC July 29, 2026 Meeting Announcement

Initiatives to Drive Growth and Improve Profitability

Initiatives to Drive Growth and Improve Profitability

Business Strategy for Pharmaceuticals Segment

Launching an average of two or more new products per year through the three pillars of in-house drug discovery, in-licensing, and PLCM, prioritizing response to the patent cliff

Optimal allocation of R&D resources

Clear prioritization of the clinical development pipeline

Promoting strategic alliances

Partnering and commercial alliances

Revenue structure reform

Review of revenue and expense structure

Nippon Shinyaku is advancing initiatives to enhance corporate value and aim to achieve sustainable growth.

Our Target for New Product Launch

Modified from March 2, 2026
FY2025 Investor Meeting, p.5

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

1. Updated from generic name to brand name
2. New indication
3. Newly added
4. Schedule delayed

	Period of the 7th Five-Year Medium-Term Management Plan					Period of Next MT Plan		
	FY2024a	FY2025a	FY2026	FY2027	FY2028	FY2029	FY2030	FY2031 and after
Japan	NS-87 (Vyxeos) : high-risk AML	NS-401 (Elzonris) : BPDCN[1]	GA101 (Gazyva) : pediatric nephrosis	ZX008 (Fintepla) : CDKL5 gene deficiency	NS-089/NCNP-02 (brogidirsen) : DMD	NS-035 : FCMD	NS-050/NCNP-03 : DMD	NS-863 : PAH、PH-ILD[3]
	LY3527727 (Jaypirca) : r/r MCL	LY3527727 (Jaypirca) : r/r CLL		GA101 (Gazyva) : lupus nephritis	GA101 (Gazyva) : SLE without nephropathy		NS-304 (selexipag) : ASO	NS-229 : EGPA[3]
	NS-304 (Uptravi) : pediatric PAH	Opsumit : pediatric PAH[2]						
Overseas			CAP-1002 (deramiocel) (U.S.) : DMD cardiomyopathy	RGX-121 (clemidsogene lanparvovec) (U.S.) : MPS II[3]	NS-089/NCNP-02 (brogidirsen) (U.S.) : DMD[4]	NS-050/NCNP-03 (U.S.) : DMD		NS-863 (U.S.) : PAH、PH-ILD[3]
				Tadekinig alfa (U.S.) : NLRC4 mutation and XIAP deficiency[3]		ATSN-101 (U.S.) : LCA1		NS-229 (U.S.) : EGPA[3]

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

The mucopolysaccharidosis treatments RGX-111 and RGX-121 each received a clinical hold from the FDA in January 2026 followed by a CRL for RGX-121 the following month. The clinical hold on RGX-121 was lifted and REGENXBIO have aligned with the FDA regarding the next steps needed for an accelerated approval.

AML:acute myeloid leukemia; r/r MCL:relapsed or refractory mantle cell lymphoma resistant or intolerant to other BTK inhibitors; PAH:pulmonary arterial hypertension; BPDCN:blastic plasmacytoid dendritic cell tumor; r/r CLL:relapsed or refractory chronic lymphocytic leukemia resistant or intolerant to other BTK inhibitors; DMD:Duchenne muscular dystrophy; SEL:systemic lupus erythematosus; FCMD:Fukuyama congenital muscular dystrophy; LCA1:GUCY2D-associated Leber congenital amaurosis; ASO:arteriosclerosis obliterans; PH-ILD:pulmonary hypertension associated with interstitial lung disease; EGPA:eosinophilic granulomatosis with polyangiitis

R&D Pipeline

Keiichi Kuwano
Director, Research & Development

R&D Updates (1/2)

For updates from FY2025 financial results announcement on May 15, 2026, see highlighted text in red.

Update	Code No. (Generic name)	Brand name	Indications and topics	Schedule
Launch	NS-304 (selexipag)	Uptravi	Uptravi Tablets 0.8 mg	May 2026
Launch	NS-401 (tagraxofusp)	Elzonris	blastic plasmacytoid dendritic cell neoplasm (BPDCN)	March 2026
P3	NS-065/NCNP-01 (viltolarsen)	Viltepso	CSR for Study 302 (P3 extension study) has been submitted to the FDA.	July 2026 (U.S.)
Additional indication	ACT-064992 (macitentan)	Opsumit	Johnson & Johnson obtained approval for the treatment of pediatric patients with pulmonary arterial hypertension (PAH) in Japan	December 2025
Additional indication	LY3527727 (pirtobrutinib)	Jaypirca	for patients with relapsed or refractory chronic lymphocytic leukemia (including small lymphocytic lymphoma) who are resistant or intolerant to other BTK inhibitors	September 2025
Filed	RGX-121 (clemidsogene lanparvovec)	–	REGENXBIO have aligned with the FDA regarding the next steps needed for an accelerated approval.	June 2026 (U.S.)
			The clinical hold placed by the FDA on RGX-121 in January 2026 has been lifted.	Disclosed in May 2026 (U.S.)
Filed	CAP-1002 (deramiocel)	–	The FDA Cellular, Tissue, and Gene Therapies Advisory Committee was held.	July 2026 (U.S.)
			The FDA has set a new PDUFA target action date of August 22, 2026	March 2026 (U.S.)
Filed	GA101 (obinutuzumab)	Gazyva	idiopathic nephrotic syndrome	May 2026
E-labaling revision			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
Start of P3	NS-304 (selexipag)	–	arteriosclerosis obliterans	April 2026
Preparation for P2	NS-035	–	Fukuyama congenital muscular dystrophy	January 2026
	NS-863	–	pulmonary arterial hypertension	March 2026
			pulmonary hypertension associated with interstitial lung disease	March 2026
	NS-025	–	urological disease	July 2026
			xerostomia (dry mouth) associated with Sjögren's disease	July 2026
Start of P1	NS-245	–	treatment for inflammatory diseases	December 2025

R&D Updates (2/2)

For updates from FY2025 financial results announcement on May 15, 2026, see highlighted text in red.

Update	Code No. (Generic name)	Brand name	Indications and topics	Schedule
Exercise of option rights (AB2 BIO Ltd.)	Tadekinig alfa	–	U.S. commercialization for the treatment for monogenic IL-18 driven hyperinflammatory syndrome in patients with NLRC4 and XIAP mutations	July 2026 (U.S.)
Agreement of option rights (Elixirgen Therapeutics, Inc.)	EXG-7001	–	Duchenne muscular dystrophy (DMD) exclusive worldwide rights to development and commercialization	June 2026
Sponsored research agreement (Boston Children's Hospital)	–	–	for developing innovative therapeutic drugs in the field of neurology	June 2026 (U.S.)
Collaboration	–	–	Collaboration agreement with xFOREST Therapeutics on Drug Discovery Research Targeting RNA Structures	March 2026
Collaboration	–	–	Launch of co-creation project to evaluate drug discovery seeds with FRONTEO, Inc.	December 2025
Orphan Drug Designation	NS-035	–	Fukuyama congenital muscular dystrophy (FCMD)	June 2026
Fast Track Designation	NS-229	–	eosinophilic granulomatosis with polyangiitis (EGPA)	September 2025 (U.S.)
Orphan Drug Designation	NS-051/NCNP-04	–	Duchenne muscular dystrophy	September 2025 (U.S.)
Academic conference presentation	NS-089/NCNP-02 (brogidirsen)		3.5-Year clinical trial data presentation at the World Muscle Society 2025 Congress	October 2025

Viltepso P3 Update

Viltepso: Toward Full Approval

<Clinical Trials>

・The global Phase III trial (Study 301) is a **confirmatory trial required as a condition of approval**

Results:
For the Primary Endpoint, time to stand (TTSTAND), and the Secondary Endpoints (10-meter walk test, timed 4 stair climb test, six-minute walking distance, NSAA), no statistically significant differences were observed between the viltolarsen group and the placebo group.

・The global Phase III Extension Study (Study 302) **targeted patients who have completed Study 301**

Results:
The change from baseline in time to stand (TTSTAND), one of the efficacy endpoints, showed a statistically significant improvement compared to the natural history group starting at Week 25.

<Discussions with the FDA>

In late June 2026, we received a communication from the FDA recommending that we request a Type-C meeting regarding the protocol for the submitted supplemental Phase III trial (Study 303). We plan to discuss this matter with the FDA at the Type-C meeting.

Reference Materials

Sales Forecast in Pharmaceuticals Segment

- **Domestic sales growth of Erleada, Fintepla, Uptravi, etc.**
- **Royalty revenue growth due to overseas sales increase of Uptravi**

(Million yen)

Brand name/ code no.	Indications	Q1 FY2025 actual	Q1 FY2026 actual	YoY change	YoY %	FY2026 Forecast 1H	FY2026 Forecast full-year
Viltepso	Duchenne muscular dystrophy (DMD)	5,194	5,637	+443	+8.5%	11,100	22,300
(Japan)		(1,198)	(1,206)	(+7)	(+0.6%)	(2,400)	(4,800)
(U.S.)		(3,995)	(4,431)	(+435)	(+10.9%)	(8,700)	(17,500)
Uptravi	pulmonary arterial hypertension/ chronic thromboembolic pulmonary hypertension	4,375	4,750	+375	+8.6%	9,200	19,200
Erleada	prostate cancer	-	3,610	+3,610	-	7,200	14,400
Fintepla	seizures associated with Dravet syndrome/ seizures associated with Lennox-Gastaut	887	1,480	+593	+66.9%	3,000	6,100
Vyxeos	high-risk AML	1,447	1,368	-78	-5.4%	3,100	6,500
Gazyva	CD20-positive follicular lymphoma/ CD20-positive chronic lymphocytic leukemia	1,180	1,360	+179	+15.2%	2,500	5,100
Defitelio	sinusoidal obstruction syndrome	650	583	-67	-10.4%	1,300	2,600
Cialis	erectile dysfunction	557	567	+9	+1.8%	1,000	2,000
CAP-1002 deramiocel (U.S.)	DMD cardiomyopathy	-	-	-	-	-	11,400
Profit in co-promotion		2,517	2,240	-277	-11.0%	4,500	8,600
Revenues from the licensing of industrial property rights		11,511	12,885	+1,374	+11.9%	27,300	53,000
Revenue		34,163	43,256	+9,092	+26.6%	83,500	176,500
Revenue based on the NHI drug price							
Jaypirca	mantle cell lymphoma chronic lymphocytic leukemia	464	1,105	+641	+138.1%	Sales forecasts for Jaypirca are not disclosed.	

The exchange rate assumed in the business forecast is 1 USD=150 yen. The sensitivity of the exchange rate after Q1 in FY2026 is assumed to be an increase of approx. 420 million yen in revenue for every 1 yen depreciation of the yen.

Revenue Forecast - Functional Food Segment-

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

(million yen)	FY2025 Q1	FY2026 Q1	YoY change	YoY %	FY2026 Forecast 1H	FY2026 Forecast full-year
Protein preparations	3,319	3,554	+235	+7.1%	7,100	14,200
Preservatives	797	823	+26	+3.3%	1,650	3,400
Supplements	612	815	+202	+33.1%	1,600	3,400
Health food ingredients	309	278	-31	-10.1%	600	1,250
Others	343	337	-5	-1.5%	550	1,250
Revenue	5,382	5,810	+428	+8.0%	11,500	23,500

Consolidated Balance Sheet

(million yen)	End of FY2025	End of Q1 FY2026	YoY change		End of FY2025	End of Q1 FY2026	YoY change
Assets	346,359	345,153	-1,205	Liabilities	54,488	49,423	-5,065
Current assets	183,086	183,175	+88	Current liabilities	42,105	39,343	-2,762
Non-current assets	163,272	161,978	-1,294	Non-current liabilities	12,382	10,079	-2,302
				Equity	291,871	295,730	+3,859
Total aseets	346,359	345,153	-1,205	Total liabilities and equity	346,359	345,153	-1,205

Assets

Cash and cash equivalents	-3,892
Trade and other receivables	+4,409
Inventories	+1,727
Other financial assets (non-current)	-3,212

Liabilities and Equity

Trade and other payables	-1,175
Deferred tax liabilities	-1,356
Other components of equity	-2,088
Retained earnings	+5,930

Pipeline（1/2）

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

Stage	Code No. (Generic name)	Origin	Indications	Schedule	Country	ID#
Launch P3	NS-065/NCNP-01 (viltolarsen)	Co-development with National Center of Neurology and Psychiatry	Duchenne muscular dystrophy	—	Japan	jRCT2080224893
				—	U.S.	NCT04060199
Filed	CAP-1002 (deramiocel)	Partnership Capricor Therapeutics, Inc.	Duchenne muscular dystrophy cardiomyopathy	PDUFA date: August 2026	U.S.	NCT03406780[1]
						NCT05126758[2]
	RGX-121 (clemidsogene lanparvovec)	Partnership REGENXBIO Inc.	mucopolysaccharidosis type II	Clinical hold lifted[3] CRL[4]	U.S.	NCT03566043
	GA101 (obinutuzumab)	In-license Chugai Pharmaceutical Co., Ltd.	idiopathic nephrotic syndrome	Submission : May 2026	Japan	NCT05627557
Preparing for filing	Tadekinig alfa	In-license AB2 Bio Ltd.	Monogenic IL-18 driven hyperinflammatory syndrome in patients with NLRC4 and XIAP mutations	—	U.S.	NCT03113760
P3	ZX008 (fenfluramine hydrochloride)	In-license[5] UCB S.A.	CDKL5 deficiency disorder	Study completion : FY2026	Japan	jRCT2041230015
	GA101 (obinutuzumab)	In-license Chugai Pharmaceutical Co., Ltd.	lupus nephritis	Projected submission : CY2026	Japan	jRCT2011210059
			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
	NS-304 (selexipag)	In-house	arteriosclerosis obliterans	Study start : FY2026	Japan	jRCT2071250134
Preparation for P3	ZX008 (fenfluramine hydrochloride)	In-license[5] UCB S.A.	Rett Syndrome	Study start : FY2026	Japan	jRCT2031260136

1. The Phase 2 (HOPE-2) study
2. The Phase 3 (HOPE-3) study
3. The clinical hold placed by the FDA on RGX-121 in January 2026 has been lifted.
4. The FDA issued a Complete Response Letter in February 2026. REGENXBIO have aligned with the FDA regarding the next steps needed for an accelerated approval.
5. The marketing authorization in Japan has been transferred from UCB Japan Co., Ltd. to Nippon Shinyaku effective April 1, 2026. Press Releases | NIPPON SHINYAKU CO., LTD.

Pipeline（2/2）

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

Stage	Code No. (Generic name)	Origin	Indications	Schedule	Country	ID#
P2	NS-580	In-house	endometriosis	Temporarily suspended	Japan	jRCT2031210685
			chronic prostatitis/ chronic pelvic pain syndrome	Temporarily suspended	Japan	jRCT2031230134
	NS-089/NCNP-02 (brogidirsen)	Co-development with National Center of Neurology and Psychiatry	Duchenne muscular dystrophy	Study completion : FY2026	Japan	jRCT2041250028
					U.S.	NCT05996003
	NS-229	In-house	eosinophilic granulomatosis with polyangiitis	Study completion : FY2026	Japan	jRCT2031230526
					U.S.	NCT06046222
Preparation for P2	NS-035	Co-development with Kobe University	Fukuyama congenital muscular dystrophy (FCMD)	Study start : FY2026	Japan	jRCT2031250857
	NS-863	In-house	pulmonary arterial hypertension	Study start : FY2026	Japan	In preparation
					U.S.	NCT07441200
			pulmonary hypertension associated with interstitial lung disease		Japan	In preparation
					U.S.	NCT07441278
	NS-025	In-house	Urological disease	-	Japan	In preparation
			xerostomia (dry mouth) associated with Sjögren's disease	-	Japan/U.S.	In preparation
P1/2	NS-050/NCNP-03	Co-development with National Center of Neurology and Psychiatry	Duchenne muscular dystrophy	Study completion : FY2028	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-245	In-house	Inframmatory diseases	Study completion : FY2026	Japan	jRCT2071250086

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

NS-065/NCNP-01 (viltolarsen)

- Treatment for Duchenne muscular dystrophy -

Development Phase	**Japan : Launched** **U.S. : Launched** **Global P3 open-label extension study in progress**
Origin	**Co-development :** **National Center of Neurology and Psychiatry**
Development	**Nippon Shinyaku**
Mechanism of action	**Exon 53 Skipping**
Indications	**Duchenne muscular dystrophy**
Dosage form	**Injection**
Feature	• **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. : BLA Filed (Duchenne muscular dystrophy cardiomyopathy) U.S. : P3 (Duchenne muscular dystrophy)
Origin	[Jan. 2022] Partnership for commercialization in the U.S. [Feb. 2023] Partnership for commercialization in Japan : Capricor Therapeutics, Inc.
Development	Capricor Therapeutics, Inc.
Mechanism of action	Exosomes released from cardiosphere-derived cells
Indications	Duchenne muscular dystrophy cardiomyopathy Duchenne muscular dystrophy
Dosage form	Injection
Feature	• Exosomes released from this drug are expected to reduce oxidative stress, inflammation, fibrosis, and increase cell energy and myocyte generation, resulting in improvement of motor and cardiac functions. • Its broad applicability makes it suitable for patients regardless of the type of genetic mutation.

RGX-121 (clemidsogene lanparvovec)

- Treatment for Mucopolysaccharidosis Type Ⅱ -

Development Phase	**U.S. : BLA Filed**
Origin	**[Jan. 2025] Partnership for commercialization in the U.S., Japan and other Asian countries : REGENXBIO Inc.**
Development	**REGENXBIO Inc.**
Mechanism of action	**Iduronate-2-sulfatase Gene therapy**
Indications	**Mucopolysaccharidosis Type Ⅱ**
Dosage form	**Injection**
Feature	**• An investigational gene therapy using adeno-associated virus (AAV) 9 to deliver the iduronate-2-sulfatase (IDS) gene to the central nervous system using intracisternal or intraventricular administration • Delivery of the IDS gene within the cells in the central nervous system could provide a permanent source of secreted IDS beyond the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS • One-time administration of RGX-121 is expected to lead to sustained production of IDS leading to the attenuation of CNS manifestations in MPS II patients**

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

GA101 (obinutuzumab)

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

Development Phase	**Japan : Filed（INS） Japan : P3（LN） Japan : P3（ERL）**
Origin	**[Nov. 2012] Licensed-in from : Chugai Pharmaceutical Co., Ltd.**
Development	**Co-development : Chugai Pharmaceutical Co., Ltd.**
Mechanism of action	**Anti-CD20 monoclonal antibody**
Indications	**idiopathic nephrotic syndrome (INS) lupus nephritis (LN) extra renal lupus (ERL)**
Dosage form	**Injection**
Feature	**Anti-CD20 monoclonal antibody, increased antibody-dependent cellular cytotoxicity (ADCC) activity and direct cytotoxicity**

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

Tadekinig alfa

– Treatment for Monogenic IL-18 driven hyperinflammatory syndrome in patients with NLRC4 and XIAP mutations –

Development Phase	**U.S. : Preparing BLA**
Origin	**AB2 Bio Ltd（Switzerland）;** **[Jan. 2025] Exclusive Option Agreement for U.S. Commercialization Rights in lead indication** **[Jul. 2026] Exercised the Option**
Development	**AB2 Bio Ltd**
Mechanism of action	**Selectively targets excess IL-18 (Recombinant Human IL-18 Binding Protein)**
Indications	**Monogenic IL-18 driven hyperinflammatory syndrome in patients with NLRC4 and XIAP mutations**
Dosage form	**Injection (subcutaneous)**
Feature	• **Specifically binds excess IL-18, thereby suppressing IL-18–mediated pathological immune and inflammatory responses.** • **If approved, Tadekinig Alfa would become the first targeted therapy for patients with NLRC4 and XIAP mutations.**

Supplementary Information : The estimated prevalence is approximately 3-5 patients per million for patients with NLRC4 or XIAP mutations.

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) Global : in preparation for P3 (Rett syndrome)
Origin	[Mar. 2019] In-Licensed from :UCB S.A. (former Zogenix, Inc.)
Development	UCB S.A. (former Zogenix, Inc.)
Mechanism of action	5-HT (serotonin) releaser with agonist activity at several 5-HT receptors
Indications	seizures associated with Dravet syndrome seizures associated with Lennox-Gastaut syndrome CDKL5 deficiency disorder Rett syndrome
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.

LY3527727(pirtobrutinib)

- Treatment for Mantle cell lymphoma, Chronic lymphocytic leukemia -

Development Phase	**Japan : Launched (for patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors)** **Launched (for patients with relapsed or refractory chronic lymphocytic leukemia who are resistant or intolerant to other BTK inhibitors)** **Japan : P3 (MCL and CLL)**
Origin	**[Mar. 2024] Alliance agreement in Japan :Eli Lilly Japan K.K.**
Development	**Eli Lilly Japan K.K.**
Mechanism of action	**A reversible non-covalent BTK inhibitor**
Indications	**mantle cell lymphoma (MCL)** **chronic lymphocytic leukemia (CLL)**
Dosage form	**Oral agent**
Feature	**A highly selective, non-covalent (reversible) inhibitor of the enzyme Bruton's tyrosine kinase (BTK), with having a novel binding mechanism**

Supplementary Information : Annually, about one in 200,000 people worldwide develop MCL. (National Organization for Rare Disorders. Mantle cell lymphoma. Accessed 26 October 2022)
CLL is a rare disease in Japan where about 7,000 people are suffering. (Official Statistics of Japan. Patient survey in FY2023.)

NS-304 (selexipag)

- Treatment for arteriosclerosis obliterans -

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

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

NS-580

- Treatment for endometriosis, Chronic prostatitis/Chronic pelvic pain syndrome -

Development Phase	Japan : P2b (endometriosis) Temporarily suspended Japan : P2a (CP/CPPS) Temporarily suspended
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	Inhibition of membrane-associated prostaglandin E synthase-1
Indications	endometriosis chronic prostatitis/chronic pelvic pain syndrome (CP/CPPS)
Dosage form	Oral agent
Feature	• 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 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.

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-035

- Treatment for Fukuyama congenital muscular dystrophy-

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

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

NS-863

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

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

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

NS-025

Development Phase	Japan : in preparation for P2
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	Muscarinic M3 positive allosteric modulator
Indications	Urological disease
Dosage form	Oral agent

Development Phase	Global : in preparation for P2
Origin	Nippon Shinyaku [Jul. 2026] : License agreement with Aquelys Therapeutics, Inc. granting it exclusive worldwide rights to develop, manufacture, and commercialize the product, except for certain indications in Japan.
Development	Aquelys Therapeutics, Inc.　(except for certain indications in Japan)
Mechanism of action	Muscarinic M3 positive allosteric modulator
Indications	Xerostomia (dry mouth) associated with Sjögren's disease
Dosage form	Oral agent

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.

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-245

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

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