FY2026 Second Quarter (Interim Period) Financial Results

November 13, 2026
NIPPON SHINYAKU CO., LTD.

NIPPON SHINYAKU CO., LTD.

Agenda

1

- Q2 FY2026 Financial Results
- Revised Business Forecast for FY2026
- CAP-1002 (deramiocel) Update
- Initiatives to Drive Growth and Improve Profitability

Toru Nakai

Representative Director, President

2

- Progress in the R&D Pipeline
- Viltepso P3 Update

Keiichi Kuwano

Board Director, Research & Development

NIPPON SHINYAKU CO., LTD.

Q2 FY2026 Financial Results and Full-Year Forecast

Toru Nakai

Representative Director, President

Highlight

Q2 FY2026 Financial Results

- ✓ Increased revenue for the fourth consecutive period (strong performance in both the Pharmaceuticals and Functional Food businesses)
- ✓ Decreased operating profit due to an increase in SG&A, R&D expenses, etc.

FY2026 Full-Year Forecast

- ✓ Revenue: Downward revision of 9.0 billion yen due to the exclusion of CAP-1002 (deramiocel)
- ✓ Operating profit: Downward revision of 5.0 billion yen due to a decrease in revenue, and an increase in R&D expenses

NIPPON SHINYAKU CO., LTD.

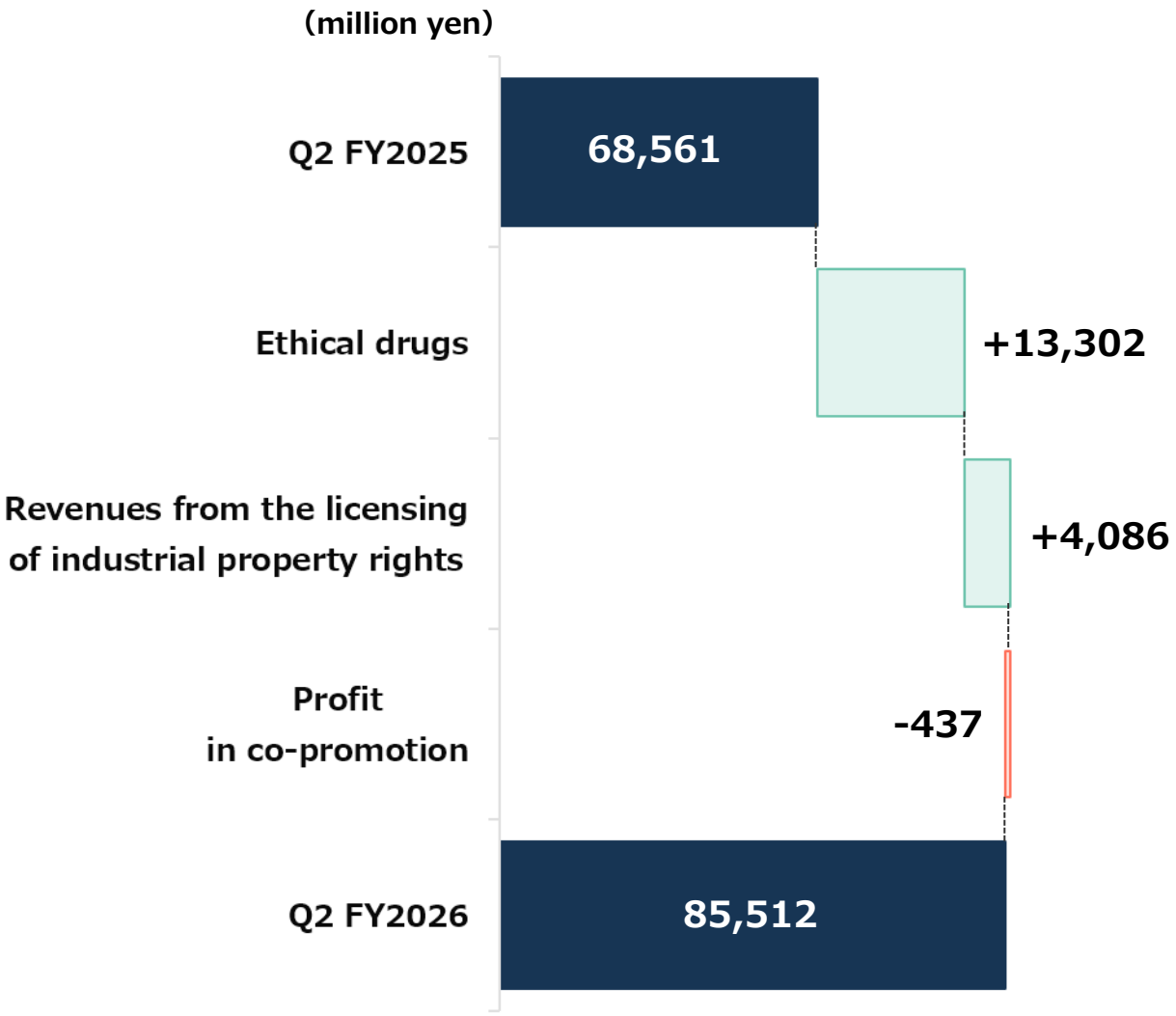
Q2 (Interim Period) FY2026 Summary

- **As a result of strong performance in both the Pharmaceuticals and Functional Food businesses, revenue increased for the fourth consecutive period.**
- **Operating profit decreased due to an increase in cost of goods sold and other expenses**

(million yen)	Q2 FY2025		Q2 FY2026		YoY	
	actual	ratio	actual	ratio	change	%
Revenue	**79,647**	**100.0%**	**97,830**	**100.0%**	**+18,182**	**+22.8%**
(Pharmaceuticals)	(68,561)	(86.1%)	(85,512)	(87.4%)	(+16,950)	(+24.7%)
(Functional Food)	(11,086)	(13.9%)	(12,318)	(12.6%)	(+1,231)	(+11.1%)
Cost of sales	25,336	31.8%	34,511	35.3%	+9,174	+36.2%
SG&A expenses	20,357	25.6%	22,763	23.3%	+2,406	+11.8%
R&D expenses	14,637	18.4%	20,679	21.1%	+6,042	+41.3%
Other income	521	0.7%	835	0.9%	+313	+60.0%
Other expenses	258	0.3%	1,439	1.5%	+1,180	+456.7%
(Foreign exchange loss)	-	-	(1,058)	(1.1%)	(+1,058)	-
Operating profit	**19,580**	**24.6%**	**19,272**	**19.7%**	**-307**	**-1.6%**
Finance income	550	0.7%	837	0.9%	+287	+52.1%
Finance costs	101	0.1%	103	0.1%	+1	+1.7%
Profit before tax	**20,029**	**25.1%**	**20,006**	**20.5%**	**-22**	**-0.1%**
Income tax expense, etc.	4,268	5.4%	4,533	4.6%	+265	+6.2%
Profit attributable to owners of parent	**15,760**	**19.8%**	**15,472**	**15.8%**	**-287**	**-1.8%**

Segmental Review - Pharmaceuticals -

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



Ethical drugs 52,889 million yen (+13,302 million yen, +33.6%, YoY)

✓ Growth in domestic sales of Uptravi, Fintepla, Jaypirca, Erleada, etc.

Revenues from the licensing of industrial property rights 28,267 million yen (+4,086 million yen, +16.9%, YoY)

✓ Royalty revenue growth due to overseas sales increase of Uptravi

Profit in co-promotion 4,354 million yen (-437 million yen, -9.1%, YoY)

✓ Yuvanci's sales increased, but revenue recognition for Erleada has changed.[1]

1. previously recorded as profit in co-promotion, but now recorded as product sales and cost of goods since the 2H FY2025, based on contract revisions

NIPPON SHINYAKU CO., LTD.

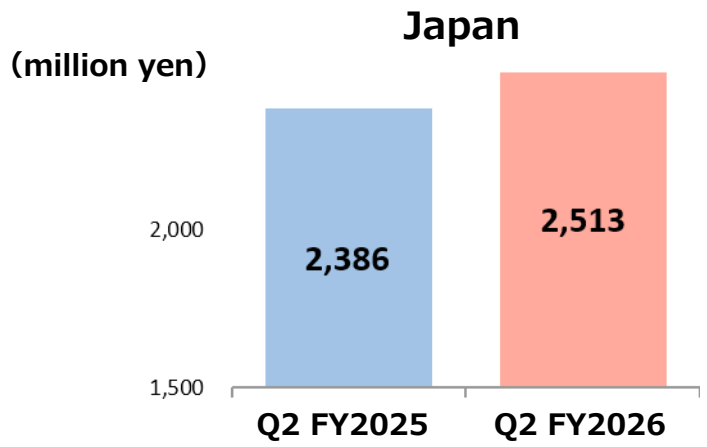
Sales Trends of Viltepso® (viltolarsen)

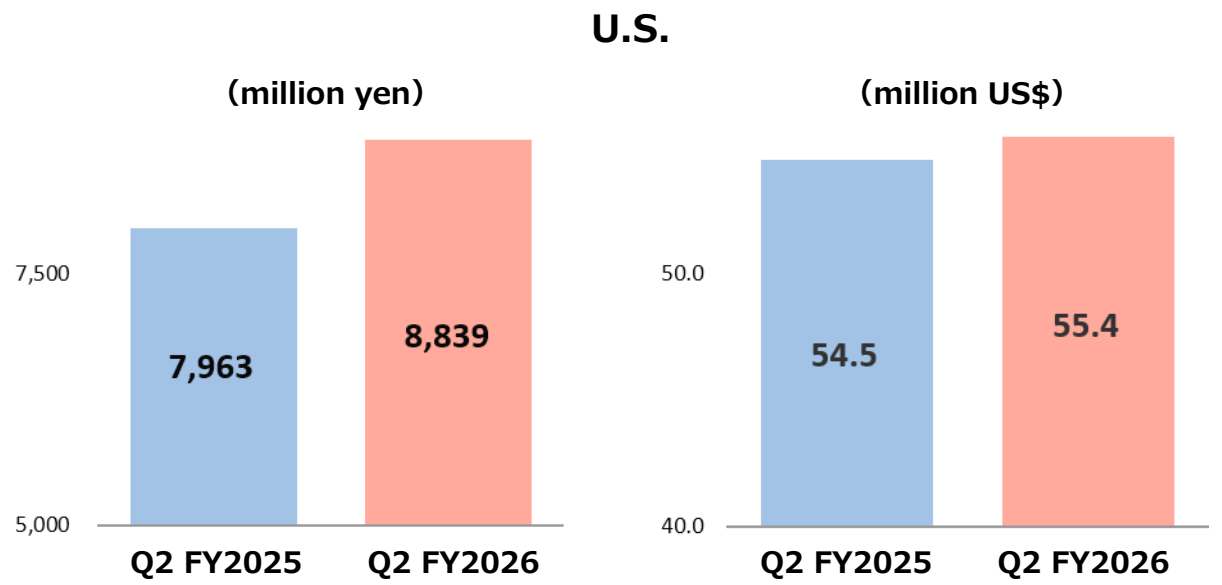
- **Although the number of new patients in the U.S. has continued to rise since April, the full-year forecast for FY2026 has been revised downward from 17.5 billion yen to 17.0 billion yen to reflect the number of patients who discontinued treatment in the 1H.**

(million yen)	Q2 FY2025 actual	Q2 FY2026 actual	YoY change	YoY %	FY2026 forecast	Notes on Q2 FY2026 results
Japan	**2,386**	**2,513**	**+126**	**+5.3%**	**4,900**	✓ The number of patients currently on therapy with Viltepso is about three-quarters of the peak number of 128 patients in the data from Chuikyo[1]. ✓ Promoting early diagnosis and intervention for younger patients
US	**7,963**	**8,839**	**+875**	**+11.0%**	**17,000**	✓ Insurance reauthorizations became stricter after launch of multiple DMD treatment options. ✓ Forecast has been revised downward due to an increase in the number of patients discontinuing treatment, despite a rise in the number of new patients.
(million US$)	(54.5)	(55.4)	(+0.9)	(+1.7%)	(113.3)	
Total	**10,349**	**11,352**	**+1,002**	**+9.7%**	**21,900**	

1. Central Social Insurance Medical Council

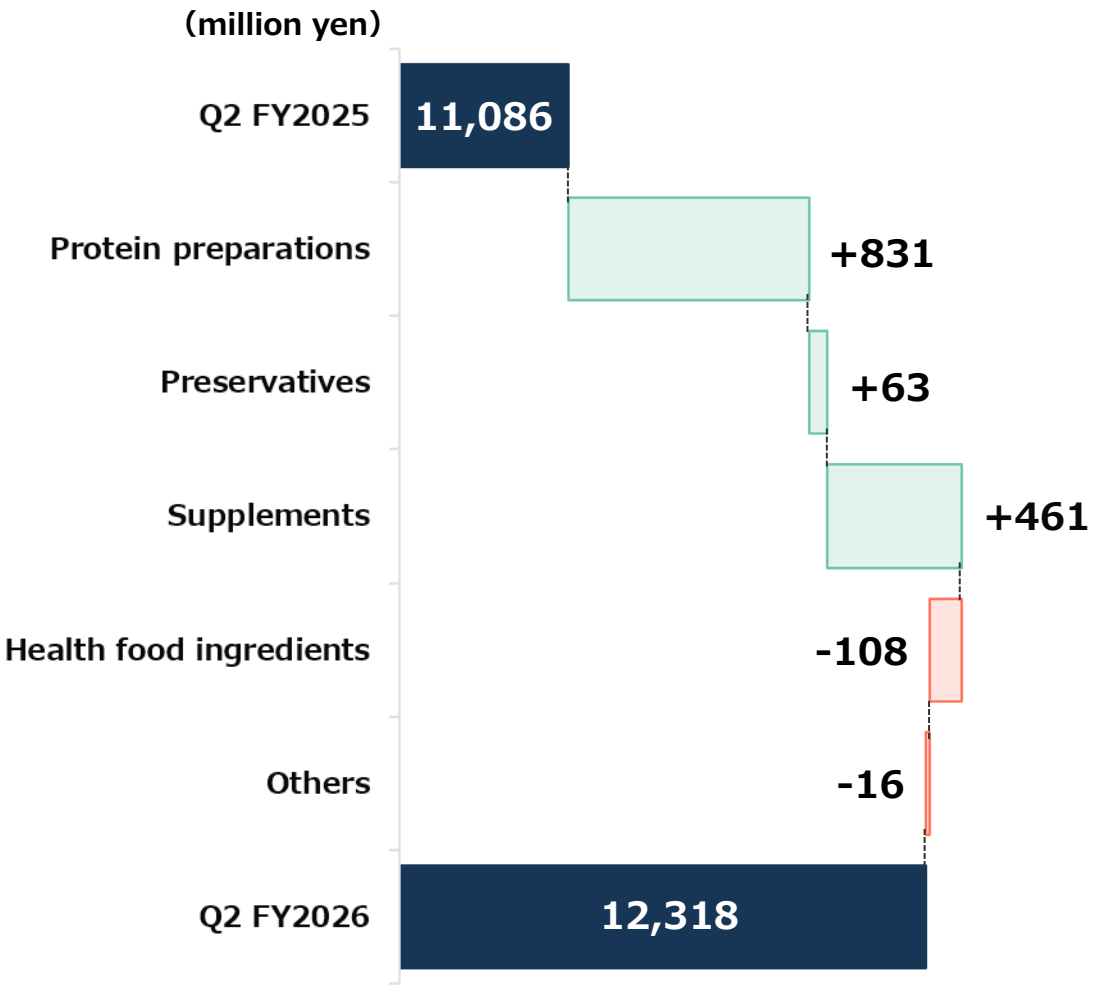
Exchange rates	Q2 FY2025 actual	Q2 FY2026 actual	2H FY2026 forecast
USDJPY	**146.04**	**159.37**	**150.00**





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 7,694 million yen
(+831 million yen, +12.1%, YoY)

✓ Strong sales of milk protein for protein powder

Preservatives 1,687 million yen
(+63 million yen, +3.9%, YoY)

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

Supplements 1,770 million yen
(+461 million yen, +35.2%, YoY)

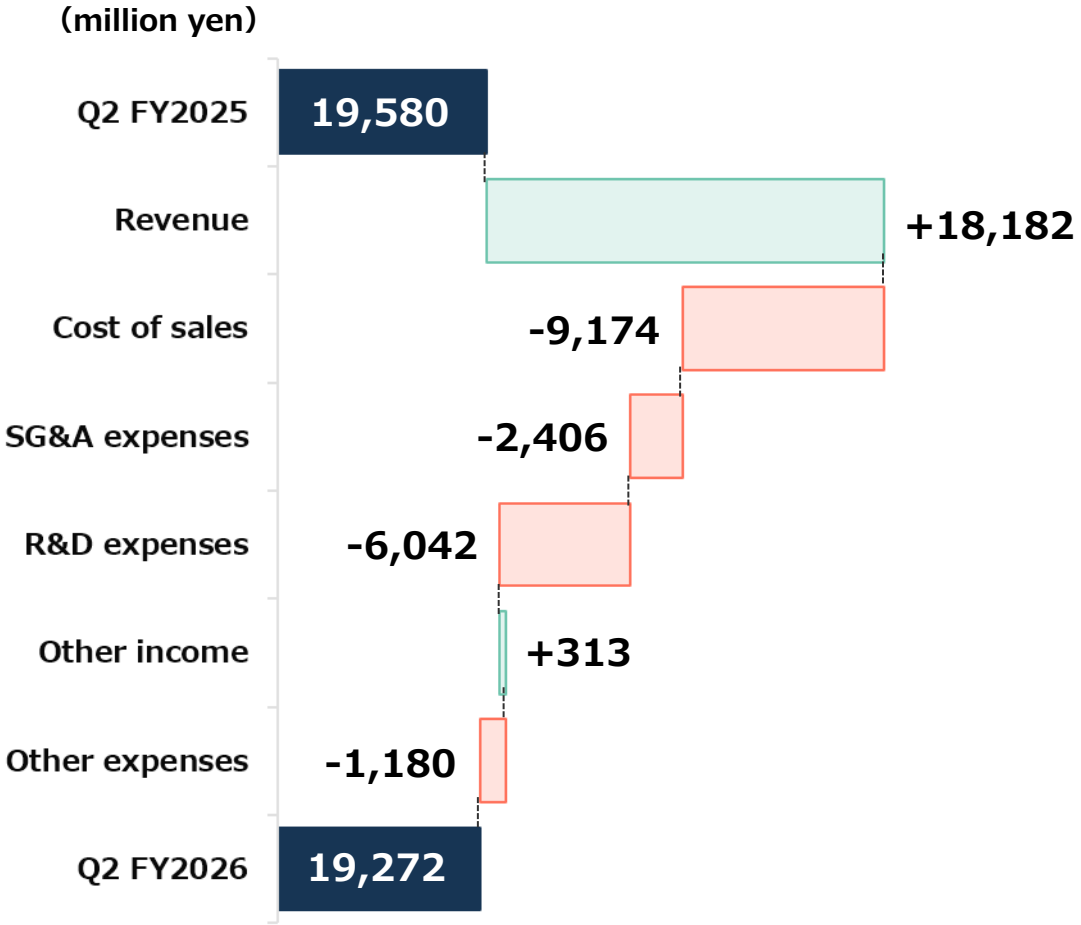
✓ Sales of athletes category are growing due to the expansion of the protein market.
✓ The anti-aging care category is also performing well.
✓ Launching egg protein, the “Third Protein” following whey and soy

Health food ingredients 513 million yen
(-108 million yen, -17.4%, YoY)

✓ Sales to major customers have declined.

Operating Profit

- **Although sales increased, profits declined due to higher cost of goods sold and other expenses.**



Revenue 97,830 million yen
(+18,182 million yen, +22.8%, YoY)
- ✓ Growth of Uptravi, Fintepla, Jaypirca, Erleada, etc.
- ✓ Royalty revenue growth due to overseas sales of Uptravi

Cost of sales 34,511 million yen
(+9,174 million yen, +36.2%, YoY)
The ratio was 35.3%, worsened by 3.5 points YoY.
- ✓ Negative impact from changes in pharmaceutical product sales mix, NHI drug price revisions and other factors

SG&A expenses 22,763 million yen
(+2,406 million yen, +11.8%, YoY)
- ✓ Increase in the U.S. sales expenses of NS Pharma

R&D expenses 20,679 million yen
(+6,042 million yen, +41.3%, YoY)
- ✓ Increase in contract research expense, etc.
- ✓ Upfront Payment for the Option Agreement for EXG-7001

Other income 835 million yen
(+313 million yen, +60.0%, YoY)
- ✓ Gain on sale of real estate, etc.

Other expenses 1,439 million yen
(+1,180 million yen, +456.7%, YoY)
- ✓ Increase in foreign exchange loss, etc.

NIPPON SHINYAKU CO., LTD.

Business Forecast : Full-Year Revision from August

- **CAP-1002 (deramiocel) has been excluded because its launch schedule has not yet been determined.[1]**

(Million yen)	FY2026 Forecasts Previous*	Revised	YoY change	%
Revenue	**200,000**	**191,000**	**-9,000**	**-4.5%**
(Pharmaceuticals)	**(176,500)**	**(166,500)**	**(-10,000)**	**(-5.7%)**
(Functional Food)	(23,500)	(24,500)	(+1,000)	(+4.3%)
Cost of sales	**73,000**	**69,000**	**-4,000**	**-5.5%**
SG&A expenses	**47,500**	**45,500**	**-2,000**	**-4.2%**
R&D expenses	**40,500**	**42,500**	**+2,000**	**+4.9%**
Other income	**1,000**	**1,000**	**-**	**-**
Other expenses	**2,000**	**2,000**	**-**	**-**
Operating profit	**38,000**	**33,000**	**-5,000**	**-13.2%**
Finance income	**800**	**1,200**	**+400**	**+50.0%**
Finance costs	**200**	**200**	**-**	**-**
Profit before tax	**38,600**	**34,000**	**-4,600**	**-11.9%**
Income tax expense, etc.	**8,300**	**7,000**	**-1,300**	**-15.7%**
Profit attributable to owners of parent	**30,300**	**27,000**	**-3,300**	**-10.9%**

Revenue 191,000 million yen
(-9,000 million yen, -4.5% from previous forecast)
Cost of sales 69,000 million yen
(-4,000 million yen, -5.5% from previous forecast)

✓ Exclusion of CAP-1002 (deramiocel), etc.

SG&A expenses 45,500 million yen
(-2,000 million yen, -4.2% from previous forecast)

✓ Decrease in U.S. sales expenses, etc.

R&D expenses 42,500 million yen
(+2,000 million yen, +4.9% from previous forecast)

✓ Progress on the development of in-licensed product candidates, etc.

1. The previous sales forecast for CAP-1002 (deramiocel) as of August 6, 2026 was 11.4 billion yen.

* August 6, 2026 (Q1 FY2026 financial results announcement)

The foreign exchange rate assumed for 2H FY2026 business forecast is 150.00. The sensitivity of the exchange rate in 2H FY2026 is assumed to be an increase of approx. 230 million yen in revenue and approx. 180 million yen in operating profit for every 1 yen depreciation of the yen.

NIPPON SHINYAKU CO., LTD.

Business Forecast for FY2026 (consolidated)

(million yen)	FY2025 actual	FY2025 ratio	FY2026 forecast	FY2026 ratio	YoY change	YoY %
Revenue	**170,771**	**100.0%**	**191,000**	**100.0%**	**+20,228**	**+11.8%**
(Pharmaceuticals)	(148,484)	(86.9%)	(166,500)	(87.2%)	(+18,015)	(+12.1%)
(Functional Food)	(22,287)	(13.1%)	(24,500)	(12.8%)	(+2,212)	(+9.9%)
Cost of sales	57,460	33.6%	69,000	36.1%	+11,539	+20.1%
SG&A expenses	43,565	25.5%	45,500	23.8%	+1,934	+4.4%
R&D expenses	36,713	21.5%	42,500	22.3%	+5,786	+15.8%
Other income	3,025	1.8%	1,000	0.5%	-2,025	-67.0%
Other expenses	560	0.4%	2,000	1.0%	+1,439	+256.5%
Operating profit	**35,496**	**20.8%**	**33,000**	**17.3%**	**-2,496**	**-7.0%**
Finance income	1,132	0.7%	1,200	0.6%	+67	+6.0%
Finance costs	166	0.1%	200	0.1%	+33	+20.5%
Profit before tax	**36,462**	**21.4%**	**34,000**	**17.8%**	**-2,462**	**-6.8%**
Income tax expense, etc.	6,741	3.9%	7,000	3.7%	+258	+3.8%
Profit attributable to owners of parent	**29,721**	**17.4%**	**27,000**	**14.1%**	**-2,721**	**-9.2%**

Foreign exchange rates (USDJPY)

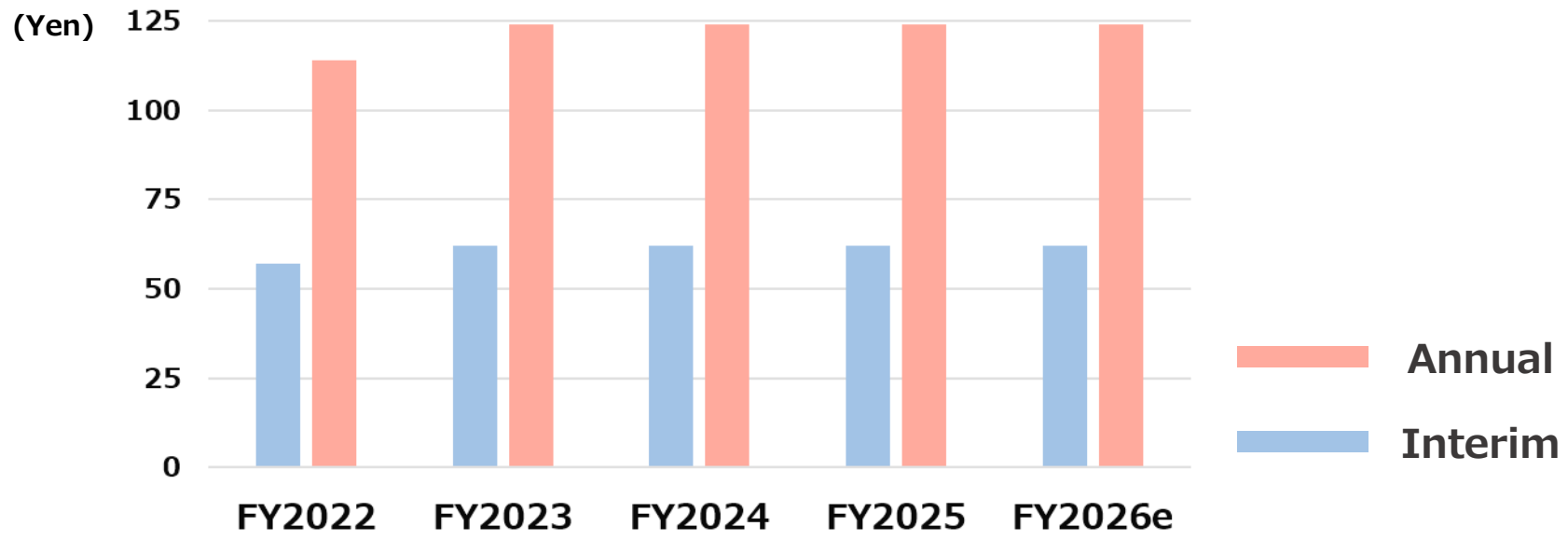
Q2 FY2025 actual	Q2 FY2026 actual	2H FY2026 forecast
146.04	159.37	150.00

The sensitivity of the exchange rate in 2H FY2026 is assumed to be an increase of approx. 230 million yen in revenue and approx. 180 million yen in operating profit for every 1 yen depreciation of the yen.

Dividends Forecast

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

		FY2025	FY2026
Dividends per share	Interim	¥62	¥62
	Annual	¥124	¥124 (e)
Basic earnings per share		¥441.00	¥400.46 (e)



CAP-1002 (deramiocel) Update

CAP-1002 (deramiocel) Update

The U.S. Litigation[1]

The dispute will be resolved through arbitration.

NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

August 10, 2026

Regarding the U.S. Litigation Concerning CAP-1002 (deramiocel)
– U.S. Court Denies Motion for Preliminary Injunction and Refers Dispute to Arbitration –

KYOTO, Japan, August 10, 2026 – Nippon Shinyaku Co., Ltd. ("Nippon Shinyaku"; Headquarters: Kyoto, Japan; President: Toru Nakai) announced that Capricor Therapeutics, Inc. ("Capricor"; Headquarters: San Diego, California, USA; Chief Executive Officer: Linda Marbán) had withdrawn its motion for preliminary injunctive relief and its opposition to Nippon Shinyaku's motion to compel arbitration. The Superior Court of New Jersey subsequently dismissed Capricor's motion for preliminary injunctive relief, granted Nippon Shinyaku's motion to compel arbitration, and ordered that the dispute be resolved through arbitration.

As a result of the court's decision, the dispute will now proceed to arbitration in accordance with the arbitration provisions of the distribution agreement.

As previously announced in Nippon Shinyaku's news release dated May 8, 2026, entitled " Announcement of a Lawsuit Filed against Nippon Shinyaku", Capricor filed a lawsuit in the State of New Jersey seeking, among other things, termination of the U.S. commercialization agreement relating to CAP-1002 (deramiocel).

Should any matters requiring disclosure arise in the future, an update will be promptly provided.

BLA Filing[2]

The PDUFA target action date has been extended to November 22, 2026.

NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

August 25, 2026

CAP-1002 (deramiocel): Notice Regarding Extension of the PDUFA Date

KYOTO, Japan, August 25, 2026 - Nippon Shinyaku Co., Ltd. ("Nippon Shinyaku"; Headquarters: Kyoto, Japan; President: Toru Nakai) hereby announces that the U.S. Food and Drug Administration (FDA) has extended the Prescription Drug User Fee Act (PDUFA) target action date for CAP-1002 (deramiocel) from August 22, 2026, to November 22, 2026. CAP-1002 (deramiocel) is currently under review pursuant to a Biologics License Application (BLA) submitted by Capricor Therapeutics, Inc. ("Capricor"; Headquarters: San Diego, California, USA; Chief Executive Officer: Linda Marbán).

Following discussions with the FDA, Capricor submitted additional data and analyses related to its BLA as a BLA amendment. The FDA accepted the submission as a Major Amendment, indicating that it constituted a significant modification to the application.

1. Company Press Release on August 10, 2026, Regarding the U.S. Litigation Concerning CAP-1002 (deramiocel) – U.S. Court Denies Motion for Preliminary Injunction and Refers Dispute to Arbitration –
2. Company Press Release on August 25, 2026, CAP-1002 (deramiocel): Notice Regarding Extension of the PDUFA Date

NIPPON SHINYAKU CO., LTD.

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



Focus our management resources on promising pipeline projects and aim to maximize value creation while controlling R&D expenses.

Clarifying priorities and focus resources on key items

- Conduct a comprehensive evaluation of each development program and prioritize them

Promoting strategic alliances



Continuously secure new growth drivers through strategic partnerships, including technology introductions and exports, with domestic and international partners

Seizing Growth Opportunities Through External Partnerships

- Introduce new products and expanding the development pipeline
- Continue to in-license products expected to contribute to earnings early on

Reforming revenue structure



Shift to a revenue structure that supports sustainable gowth aimed at enhancing corporate value

Strengthen profitability through both sales growth and cost optimization

- Review the company-wide revenue and expense structure and implement a structural reform plan

Progress in the R&D Pipeline

Keiichi Kuwano
Board Director, Research & Development

Progress in the R&D Pipeline (1/2)

Basic Policy: "Focusing on intractable and rare diseases, we prioritize products expected to contribute to earnings early on."

Optimal allocation of R&D resources

In-house Drug Discovery | In-licensing/Out-licensing | PLCM

Global - Accelerating global expansion to seize growth opportunities -

RD: intractable and rare diseases

Products Under Development	Indications	Development Stage	
In-house NS-089 RD	DMD	PⅠ → PⅡ → Filing	Enrollment of target number of patients completed Phase II Trial scheduled to conclude in February 2027
In-house NS-050 RD	DMD	PⅠ/Ⅱ → Filing	Part 1 (dose-escalation phase) patient enrollment completed
In-house NS-863 RD	PAH PH-ILD	PⅠ → PⅡ → PⅢ → Filing PⅠ → PⅡ → PⅢ → Filing	Patient consents obtained Administration to the first patient scheduled to begin shortly
In-house NS-229 RD	EGPA	PⅠ → PⅡ → PⅢ → Filing	Phase II Trial expected to conclude shortly Top-line data expected to be available during FY2026
In-house NS-245 RD	Inflammatory Diseases	PⅠ → PⅡ → PⅢ → Filing	Phase I Trial scheduled to conclude during FY2026

DMD: Duchenne muscular dystrophy; PAH: pulmonary arterial hypertension; PH-ILD: pulmonary hypertension associated with interstitial lung disease;
EGPA: eosinophilic granulomatosis with polyangiitis

Progress in the R&D Pipeline (2/2)

Optimal allocation of R&D resources

In-house Drug Discovery In-licensing/Out-licensing PLCM
RD intractable and rare diseases

Japan

- Focusing on products in late-stage development with a high probability of success (POS) and expected to contribute to revenue at an early stage -

Products Under Development	Indications	Development Stage	
In-house **NS-035** RD	FCMD	PⅠ → PⅡ → Filing	Phase II Trial scheduled to begin in August Enrollment of more than half of target number of patients completed
In-house PLCM **NS-304**	ASO	PⅠ → PⅡ → PⅢ → Filing	Phase III Trial: Patient Enrollment Progressing Smoothly
In-house **NS-580**	Endometriosis CP/CPPS	PⅠ → PⅡ → PⅢ → Filing PⅠ → PⅡ → PⅢ → Filing	Endometriosis: Phase III Trial Scheduled to Begin CP/CPPS: Phase IIb Trial Scheduled to Begin

Strategic alliances

- Continuously securing profits through strategic partnerships, including in-licensing and out-licensing -

In-licensing **MT-2111** RD	r/r DLBCL	PⅠ/Ⅱ → PⅢ → Filing	Exclusive sales rights in Japan
In-licensing **Tadekinig alfa** RD	NLRC4 deficiency syndrome XIAP deficiency	PⅠ → PⅡ → PⅢ → Preparing for filing	Exclusive commercialization rights in the U.S.
In-licensing PLCM **pirtobrutinib** RD	MCL CLL	PⅠ/Ⅱ → PⅢ → Filing PⅠ/Ⅱ → PⅢ → Filing	Alliance agreements for Japanese market
Out-licensing **NS-025** RD	Sjögren's syndrome-associated xerostomia	PⅠ → Preraring for PⅡ → PⅢ → Filing	Worldwide rights to develop, manufacture, and commercialize

FCMD: Fukuyama-type congenital muscular dystrophy, ASO: arteriosclerosis obliterans, CP/CPPS: chronic prostatitis/chronic pelvic pain syndrome,
r/r DLBCL: relapsed or refractory diffuse large B-cell lymphoma, MCL: mantle cell lymphoma; CLL: chronic lymphocytic leukemia

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).

In preparation for the Type-C meeting, we are currently considering our strategy for discussions with the FDA at that meeting, as well as the optimal timing.

NIPPON NYAKU CO., LTD.

Reference Materials

Sales Forecast in Pharmaceuticals Segment

- **CAP-1002 (deramiocel) has been excluded from the FY2026 forecast.**

* August 6, 2026 (Q1 FY2026 financial results announcement)

(Million yen)

Brand name/ code no.	Indications	Q2 FY2025 actual	Q2 FY2026 actual	YoY change	YoY %	FY2026 Forecasts Previous*	FY2026 Forecasts Revised	YoY change	YoY %
Viltepso		10,349	11,352	+1,002	+9.7%	22,300	21,900	-400	-1.8%
(Japan)	Duchenne muscular dystrophy (DMD)	(2,386)	(2,513)	(+126)	(+5.3%)	(4,800)	(4,900)	100	+2.1%
(U.S.)		(7,963)	(8,839)	(+875)	(+11.0%)	(17,500)	(17,000)	(-500)	(-2.9%)
Uptravi	pulmonary arterial hypertension/ chronic thromboembolic pulmonary hypertension	8,448	9,794	+1,345	+15.9%	19,200	19,700	+500	+2.6%
Erleada	prostate cancer	-	7,059	-	-	14,400	14,300	-100	-0.7%
Vyxeos	high-risk AML	2,941	2,832	-108	-3.7%	6,500	6,200	-300	-4.6%
Gazyva	CD20-positive follicular lymphoma/ CD20-positive chronic lymphocytic leukemia	2,451	2,717	+265	+10.8%	5,100	5,100	-	-
Fintepla	seizures associated with Dravet syndrome/ seizures associated with Lennox-Gastaut syndrome	1,765	2,707	+941	+53.3%	6,100	6,100	-	-
Defitelio	sinusoidal obstruction syndrome	1,252	1,237	-14	-1.2%	2,600	2,500	-100	-3.8%
Cialis	erectile dysfunction	1,119	999	-120	-10.7%	2,000	2,000	-	-
CAP-1002 deramiocel (U.S.)	DMD cardiomyopathy	-	-	-	-	11,400	-	-	-
Profit in co-promotion		4,792	4,354	-437	-9.1%	8,600	8,600	+0	0.0%
Revenues from the licensing of industrial property rights		24,181	28,267	+4,086	+16.9%	53,000	54,000	+1,000	+1.9%
Viltepso (export)		30	3,147	+3,117	+10379.6%	-	4,500	-	-
Revenue		68,561	85,512	+16,950	+24.7%	176,500	166,500	-10,000	-5.7%
Revenue based on the NHI drug price									
Jaypirca	mantle cell lymphoma chronic lymphocytic leukemia	1,086	2,209	+1,122	+103.2%				

Sales forecasts for Jaypirca are not disclosed.

The foreign exchange rate assumed for 2H FY2026 business forecast is 150.00. The sensitivity of the exchange rate in 2H FY2026 is assumed to be an increase of approx. 230 million yen in revenue for every 1 yen depreciation of the yen.

Revenue Forecast - Functional Food Segment-

- **Sales forecasts for protein preparations have been revised upward because sales in the first half exceeded projections, partly due to factors such as price pass-through.**

(million yen)	Q2 FY2025	Q2 FY2026	YoY		FY2026 Forecasts		YoY	
	actual	actual	change	%	Previous*	Revised	change	%
Protein preparations	6,862	7,694	+831	+12.1%	14,200	15,400	+1,200	+8.5%
Preservatives	1,624	1,687	+63	+3.9%	3,400	3,400	-	-
Supplements	1,309	1,770	+461	+35.2%	3,400	3,400	-	-
Health food ingredients	621	513	-108	-17.4%	1,250	1,100	-150	-12.0%
Others	668	652	-16	-2.5%	1,250	1,200	-50	-4.0%
Revenue	11,086	12,318	+1,231	+11.1%	23,500	24,500	+1,000	+4.3%

* August 6, 2026 (Q1 FY2026 financial results announcement)

Consolidated Balance Sheet

(million yen)	End of FY2025	End of Q2 FY2026	YoY change		End of FY2025	End of Q2 FY2026	YoY change
Assets	346,359	335,890	-10,468	Liabilities	54,488	45,142	-9,345
Current assets	183,086	184,083	+997	Current liabilities	42,105	38,447	-3,658
Non-current assets	163,272	151,806	-11,466	Non-current liabilities	12,382	6,695	-5,687
				Equity	291,871	290,747	-1,123
Total aseets	346,359	335,890	-10,468	Total liabilities and equity	346,359	335,890	-10,468

Assets

Cash and cash equivalents	-3,742
Trade and other receivables	+2,304
Intangible assets	+5,747
Other financial assets (non-current)	-18,643

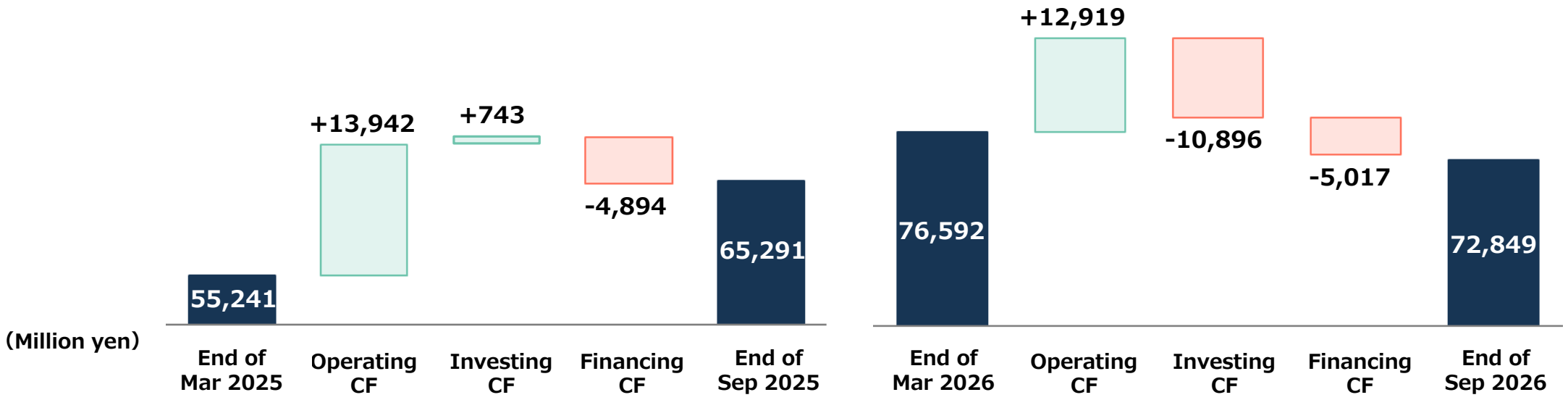
Liabilities and Equity

Income taxes payable	-2,722
Deferred tax liabilities	-5,610
Retained earnings	+11,470
Other components of equity	-12,635

NIPPON SHINYAKU CO., LTD.

Consolidated Statements of Cash Flows

(million yen)	Q2 FY2025 actual	Q2 FY2026 actual	YoY change
Operating activities	13,942	12,919	-1,022
Investing activities	743	-10,896	-11,639
Financing activities	-4,894	-5,017	-123
Cash and cash equivalents at end of period	65,291	72,849	+7,558



NIPPON SHINYAKU CO., LTD.

R&D Updates (1/2)

For updates from FY2025 financial results announcement on August 6, 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
Filed	MT-2111 (loncastuximab tesirine)	–	Relapsed or refractory diffuse large B-cell lymphoma	October 2026
BLA preparation	RGX-121 (clemidsogene lanparvovec)	–	The FDA placed a clinical hold on RGX-121.	August 2026 (U.S.)
			REGENXBIO have aligned with the FDA regarding the next steps needed for an accelerated approval.	June 2026 (U.S.)
Filed	CAP-1002 (deramiocel)	–	Indication has been changed from DMD cardiomyopathy to DMD. The FDA has extended the PDUFA target action date to November 22, 2026.	August 2026 (U.S.)
			The FDA Cellular, Tissue, and Gene Therapies Advisory Committee was held.	July 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
Start of P3	ZX008 (fenfluramine hydrochloride)	Fintepla	Rett syndrome	October 2026
Start of P3	NS-304 (selexipag)	–	arteriosclerosis obliterans	April 2026
Start of P2	NS-035	–	Fukuyama congenital muscular dystrophy	August 2026
P2 preparation	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 August 6, 2026, see highlighted text in red.

Update	Code No. (Generic name)	Brand name	Indications and topics	Schedule
Sublicense agreement signed (Tanabe Pharma)	MT-2111 (loncastuximab tesirine)	–	Relapsed or refractory diffuse large B-cell lymphoma	October 2026
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
Out-license agreement signed (Aquelys Therapeutics)	NS-025	–	Granted exclusive worldwide rights to develop, manufacture, and commercialize NS-025, excluding certain indications in Japan.[1]	July 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
SAKIGAKE Pharmaceutical Product (Pioneering Drug) Designation	NS-035	–	Fukuyama congenital muscular dystrophy (FCMD)	August 2026
Orphan Drug Designation			Fukuyama congenital muscular dystrophy (FCMD)	June 2026
Academic conference presentation	NS-089/NCNP-02 (brogidirsen)		5-Year efficacy and safety data presentation at the World Muscle Society 2026 Congress	October 2026

1. Aquelys Therapeutics plans to conduct a clinical trial for xerostomia associated with Sjögren's syndrome.

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 Considering additional P3	NS-065/NCNP-01 (viltolarsen)	Co-development with National Center of Neurology and Psychiatry	Duchenne muscular dystrophy	—	Japan	—
				—	U.S.	—
Filed P3	CAP-1002 (deramiocel)	Partnership Capricor Therapeutics, Inc.	Duchenne muscular dystrophy	PDUFA date: November 2026	U.S.	NCT03406780[1]
						NCT05126758[2]
Filed	GA101 (obinutuzumab)	In-license Chugai Pharmaceutical Co., Ltd.	idiopathic nephrotic syndrome	Submission : May 2026	Japan	NCT05627557
Filed	MT-2111 (loncastuximab tesirine)	ADC Therapeutics SA (Development in Japan: Tanabe Pharma)	relapsed or refractory diffuse large B-cell lymphoma (DLBCL)	Submission : October 2026	Japan	NCT05658562
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
Preparing for resubmission	RGX-121 (clemidsogene lanparvovec)	Partnership REGENXBIO Inc.	mucopolysaccharidosis type II	Under clinical hold[3]	U.S.	NCT03566043
P3	ZX008 (fenfluramine hydrochloride)	In-license 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
	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
	ZX008 (fenfluramine hydrochloride)	In-license 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 FDA placed a clinical hold on RGX-121 in August 2026.

Pipeline (2/2)

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

Stage	Code No. (Generic name)	Origin	Indications	Schedule	Country	ID#
P2	NS-580 (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
	NS-035	Co-development with Kobe University	Fukuyama congenital muscular dystrophy (FCMD)	Study start : FY2026	Japan	jRCT2031250857
Preparing for P2	NS-863	In-house	pulmonary arterial hypertension	Study start : FY2026	Japan	jRCT2031260222
					U.S.	NCT07441200
			pulmonary hypertension associated with interstitial lung disease		Japan	jRCT2031260223
					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** **Additional global P3 study under consideration**
Origin/Partnership	**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-

Development Phase	U.S. : BLA Filed U.S. : P3 extension study
Origin/Partnership	[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.

NIPPON SHINYAKU CO., LTD.

MT-2111 (loncastuximab tesirine)

- Treatment for relapsed or refractory diffuse large B-cell lymphoma (DLBCL)

Development Phase	Japan : NDA Filed
Origin/Partnership	[Oct. 2026] Partnership for commercialization in Japan with Tanabe Pharma (sublicensing) : ADC Therapeutics SA
Development	Tanabe Pharma
Mechanism of action	CD19-directed antibody-drug conjugate (ADC)
Indications	Relapsed or refractory diffuse large B-cell lymphoma (DLBCL)
Dosage form	Injection
Feature	A first-in-class CD19-targeted antibody-drug conjugate

NIPPON SHINYAKU CO., LTD.

RGX-121 (clemidsogene lanparvovec)

- Treatment for Mucopolysaccharidosis Type Ⅱ -

Development Phase	**U.S. : Preparing for BLA resubmission**
Origin/Partnership	**[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.

GA101 (obinutuzumab)

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

Development Phase	**Japan : Filed（INS） Japan : P3（LN） Japan : P3（ERL）**
Origin/Partnership	**[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.

NIPPON SHINYAKU CO., LTD.

Tadekinig alfa

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

Development Phase	**U.S. : Preparing BLA**
Origin/Partnership	**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) Global : P3 (CDKL5 deficiency disorder) Global : P3 (Rett syndrome)
Origin/Partnership	[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.

NIPPON SHINYAKU CO., LTD.

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/Partnership	**[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/Partnership	**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.

NIPPON SHINYAKU CO., LTD.

NS-580 (friluglanstat)

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

Development Phase	Japan : P2b (endometriosis) Temporarily suspended Japan : P2a (CP/CPPS) Temporarily suspended
Origin/Partnership	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/Partnership	**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/Partnership	**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 FY2025 is 9,899.

NIPPON SHINYAKU CO., LTD.

NS-035

- Treatment for Fukuyama congenital muscular dystrophy-

Development Phase	**Japan : P2**
Origin/Partnership	**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.

NIPPON SHINYAKU CO., LTD.

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/Partnership	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 FY2025 is 5,277.

NS-025

Development Phase	Japan : in preparation for P2
Origin/Partnership	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/Partnership	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

NIPPON SHINYAKU CO., LTD.

NS-050/NCNP-03

- Treatment for Duchenne muscular dystrophy -

Development Phase	**Global : P1/2**
Origin/Partnership	**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.

NIPPON SHINYAKU CO., LTD.

ATSN-101

- Treatment for GUCY2D-associated Leber congenital amaurosis -

Development Phase	**US : P1/2**
Origin/Partnership	**[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/Partnership	**[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/Partnership	**[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%.

NIPPON SHINYAKU CO., LTD.

NS-245

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

NIPPON SHINYAKU CO., LTD.

Safe Harbor Statement

- Materials and information provided during this presentation may contain so-called “forward-looking statements.” These statements are based on current expectations, forecasts and assumptions that are subject to risks and uncertainties which could cause actual outcomes and results to differ materially from these statements.

- Risks and uncertainties include general industry and market conditions, and general domestic and international economic conditions such as interest rate and currency exchange fluctuations. Risks and uncertainties particularly apply with respect to product-related forward-looking statements. Product risks and uncertainties include, but are not limited to, technological advances and patents attained by competitors; challenges inherent in new product development, including completion or failure of clinical trials; claims and concerns about product safety and efficacy; regulatory agency’s examination, obtaining regulatory approvals; domestic and foreign social security reforms; trends toward healthcare cost containment; and governmental laws and regulations affecting domestic and foreign operations.

- Also, for products that are approved, there are manufacturing and marketing risks and uncertainties, which include, but are not limited to, inability to build production capacity to meet demand, unavailability of raw materials, and competition with others.

- The Company disclaims any intention or obligation to update or revise any forward-looking statements whether as a result of new information, future events or otherwise.

- This English presentation was translated from the original Japanese version. In the event of any inconsistency between the Japanese original and the English translation, the former shall prevail.