Outline of Consolidated Financial Results for the 1st Quarter Ended June 30, 2023

August 10, 2023
NIPPON SHINYAKU CO., LTD.

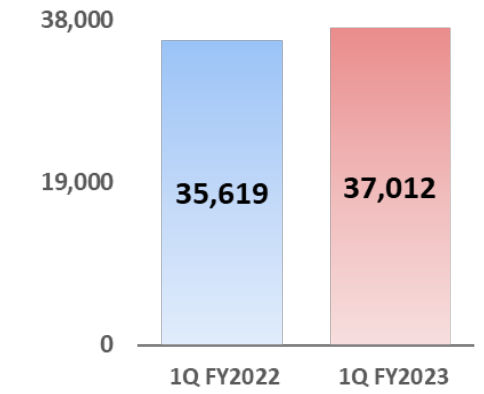
NIPPON SHINYAKU CO., LTD.

1Q FY2023 Summary

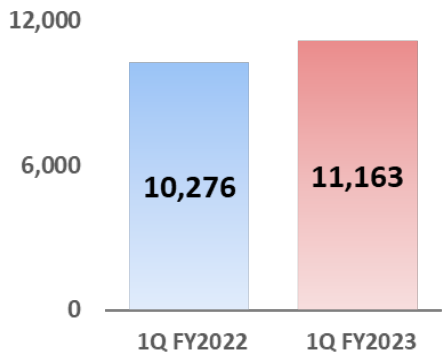
- **Revenue** : 37,012 million yen （+ 3.9%）
- **Operating profit** : 11,163 million yen （+ 8.6%）
- **Profit before tax** : 11,440 million yen （+ 8.8%）
- **Profit attributable to owners of parent** : 8,749 million yen （+ 6.1%）

Revenue

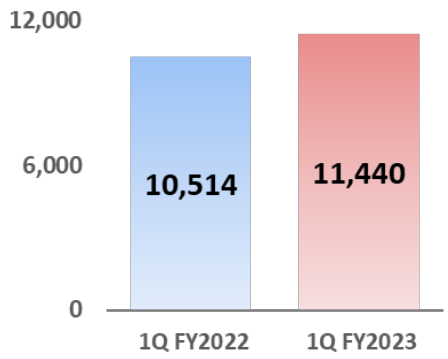
(Million yen)



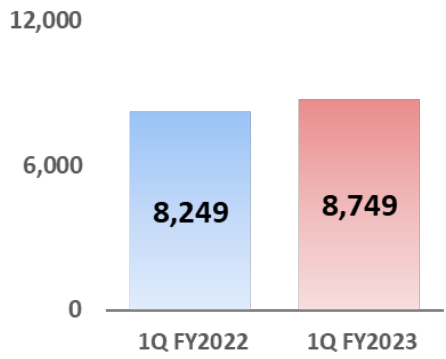
Operating profit



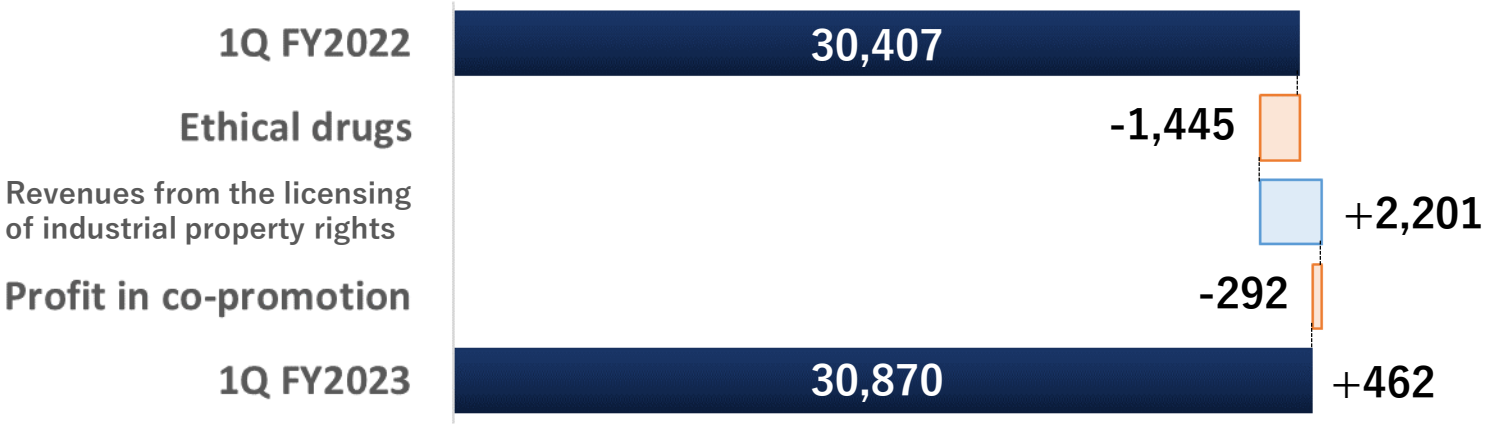
Profit before tax



Profit attributable to owners of parent



Segmental Review - Pharmaceuticals -

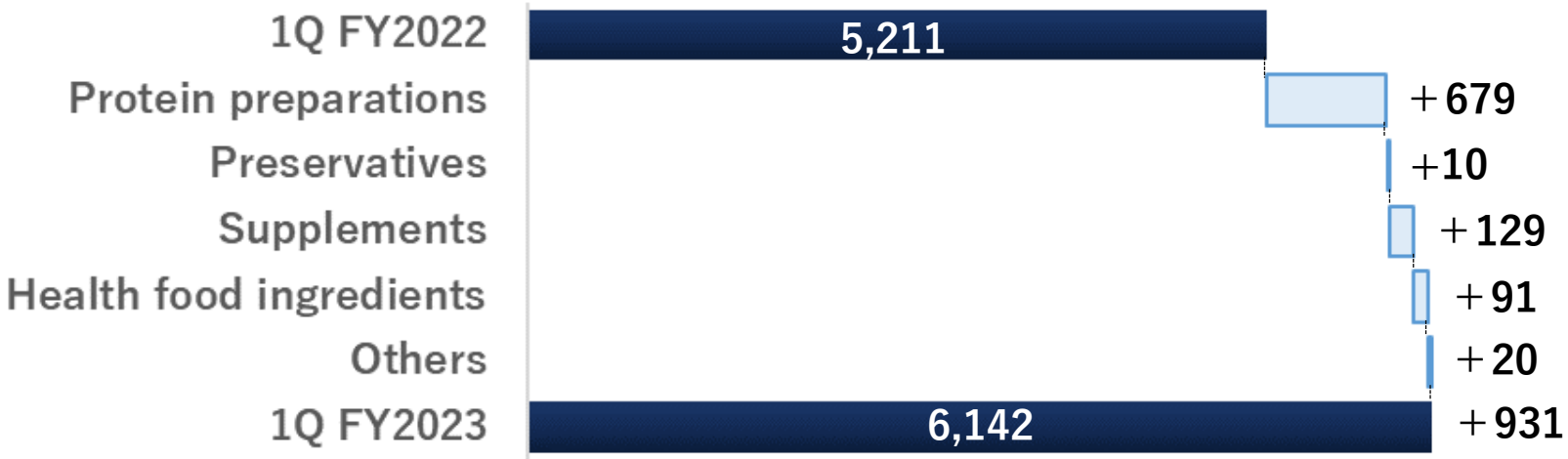


(Million yen)	1Q FY2022		1Q FY2023		YoY Change	
	Results	Ratio	Results	Ratio	Amt	%
Ethical drugs	20,900	68.7%	19,454	63.0%	-1,445	-6.9%
Revenues from the licensing of industrial property rights	6,903	22.7%	9,104	29.5%	+2,201	+31.9%
Profit in co-promotion	2,603	8.6%	2,310	7.5%	-292	-11.2%
Revenue	30,407	100.0%	30,870	100.0%	+462	+1.5%

Despite the effect of price revision by MHLW* and generic products, revenue of consolidated pharmaceuticals segment increased by 1.5% due to increase of sales of Ethical drugs such as "Viltepso" and "Uptravi", and royalty revenue from Uptravi's overseas sales.

*MHLW : Ministry of Health, Labour and Welfare

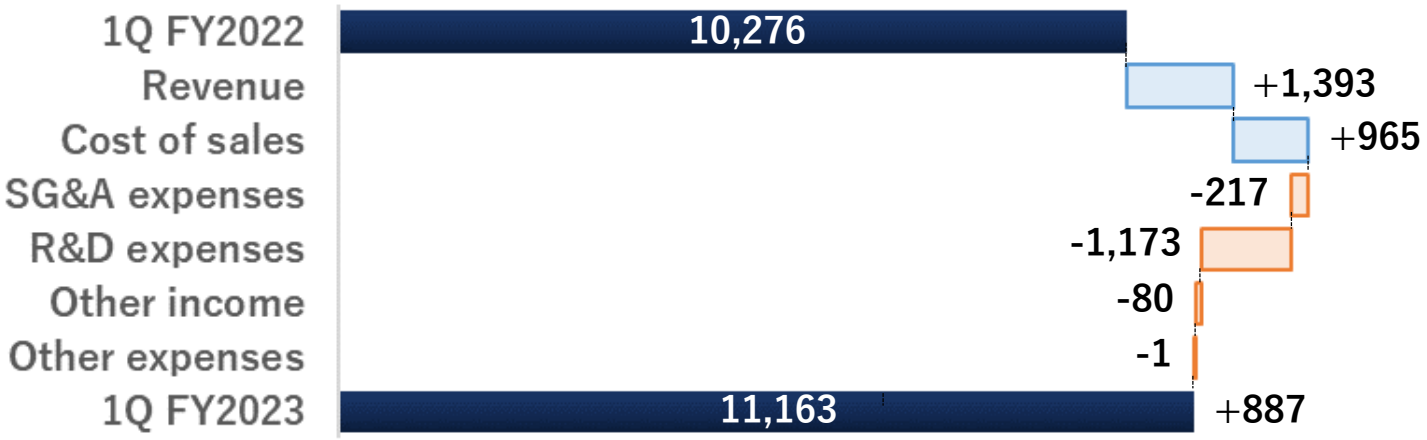
Segmental Review - Functional Food -



(Million yen)	1Q FY2022		1Q FY2023		YoY Change	
	Results	Ratio	Results	Ratio	Amt	%
Protein preparations	3,605	69.2%	4,284	69.8%	+679	+18.9%
Preservatives	726	13.9%	736	12.0%	+10	+1.4%
Supplements	335	6.4%	464	7.6%	+129	+38.7%
Health food ingredients	214	4.1%	306	5.0%	+91	+42.7%
Others	330	6.4%	350	5.6%	+20	+6.2%
Revenue	5,211	100.0%	6,142	100.0%	+931	+17.9%

Revenue of consolidated functional food segment increased by 17.9% through sales increase of Protein preparations and Supplements.

Operating profit



(Million yen)	1Q FY2022		1Q FY2023		YoY Change	
	Results	Ratio	Results	Ratio	Amt	%
Revenue	**35,619**	**100.0%**	**37,012**	**100.0%**	**+1,393**	**+3.9%**
(Pharmaceuticals)	(30,407)	(85.4%)	(30,870)	(83.4%)	(+462)	(+1.5%)
(Functional Food)	(5,211)	(14.6%)	(6,142)	(16.6%)	(+931)	(+17.9%)
Cost of sales	**13,928**	**39.1%**	**12,962**	**35.0%**	**-965**	**-6.9%**
SG&A expenses	**8,200**	**23.0%**	**8,418**	**22.7%**	**+217**	**+2.7%**
R&D expenses	**4,738**	**13.3%**	**5,911**	**16.0%**	**+1,173**	**+24.8%**
Other income	**1,652**	**4.6%**	**1,572**	**4.2%**	**-80**	**-4.8%**
Other expenses	**128**	**0.3%**	**129**	**0.3%**	**+1**	**+1.0%**
Operating profit	**10,276**	**28.9%**	**11,163**	**30.2%**	**+887**	**+8.6%**

Profit attributable to owners of parent

(Million yen)	1Q FY2022 Results	1Q FY2023 Results	YoY Change Amt	YoY Change %
Operating profit	10,276	11,163	+887	+8.6%
Finance income	266	298	+31	+12.0%
Finance costs	28	21	-6	-23.1%
Profit before tax	10,514	11,440	+925	+8.8%
Income tax expense, etc	2,265	2,690	+425	+18.8%
Profit attributable to owners of parent	8,249	8,749	+500	+6.1%

Business Forecast for FY2023

(Million yen)	FY2022		FY2023			
	1Q Results	FY Results	1Q Results	Progress for 1H	1H Forecasts	FY Forecasts
Revenue	**35,619**	**144,175**	**37,012**	**51.8%**	**71,500**	**145,000**
(Pharmaceuticals)	(30,407)	(121,988)	(30,870)	(51.0%)	(60,500)	(123,500)
(Functional Food)	(5,211)	(22,187)	(6,142)	(55.8%)	(11,000)	(21,500)
Operating profit	**10,276**	**30,049**	**11,163**	**69.8%**	**16,000**	**32,000**
Profit before tax	**10,514**	**30,489**	**11,440**	**70.6%**	**16,200**	**32,500**
Profit attributable to owners of parent	**8,249**	**22,812**	**8,749**	**70.6%**	**12,400**	**25,000**

Revenue and each profit have progressed toward achievement of 1H, FY forecasts.

R&D Pipeline

NIPPON SHINYAKU CO., LTD.

R&D Pipeline (Domestic)

Code No. (Generic name) <Origin>	Application type	Indications	PI	Preparation for PI/II	PI/II	Preparation for PII	PII	PIII	NDA filing	Launch
NS-065/NCNP-01 (viltolarsen) <in-house>	NME	Duchenne muscular dystrophy						PIII in progress		
NS-87 (daunorubicin / cytarabine) <in-license>	New combi-nation	High-risk acute myeloid leukemia								
ZX008 (fenfluramine hydrochloride) <Distribution partnership>	New indication	Lennox-Gastaut syndrome								
		CDKL5 deficiency disorder								
GA101 (obinutuzumab) <in-license>	New indication	Lupus nephritis								
		Pediatric nephrotic syndrome								
NS-304 (selexipag) <in-house>	New indication	Arteriosclerosis obliterans								
	New dose	Pediatric pulmonary arterial hypertension								

■ : changes from the Fiscal Year Ended March 31, 2023

R&D Pipeline (Domestic)

Code No. (Generic name) <Origin>	Application type	Indications	PI	Preparation for PI/II	PI/II	Preparation for PII	PII	PIII	NDA filing	Launch
NS-580 <in-house>	NME	Endometriosis								
		Chronic prostatitis / Chronic pelvic pain syndrome								
NS-089/NCNP-02 (brogidirsen) <in-house>	NME	Duchenne muscular dystrophy								
NS-229 <in-house>	NME	Eosinophilic granulomatosis with polyangiitis								
NS-401 (tagraxofusp) <in-license>	NME	Blastic plasmacytoid dendritic cell neoplasm								
NS-050/NCNP-03 <in-house>	NME	Duchenne muscular dystrophy								
NS-917 (radgocitabine) <in-license>	NME	Relapsed/refractory acute myeloid leukemia								
NS-161 <in-house>	NME	Inflammatory diseases								
NS-025 <in-house>	NME	Urological diseases								

■ : changes from the Fiscal Year Ended March 31, 2023

R&D Pipeline (Overseas)

Code No. (Generic name) <Origin>	Application type	Indications	PI	Preparation for PI/II	PI/II	Preparation for PII	PII	PIII	NDA filing	Launch
NS-065/NCNP-01 (viltolarsen) <in-house>	NME	Duchenne muscular dystrophy						PIII in progress		
CAP-1002 <partnership>	NME	Duchenne muscular dystrophy								
NS-018 (ilginatinib) <in-house>	NME	Myelofibrosis								
NS-089/NCNP-02 (brogidirsen) <in-house>	NME	Duchenne muscular dystrophy								
NS-229 <in-house>	NME	Eosinophilic granulomatosis with polyangiitis								
NS-050/NCNP-03 <in-house>	NME	Duchenne muscular dystrophy								

■：changes from the Fiscal Year Ended March 31, 2023

Reference Materials

NIPPON SHINYAKU CO., LTD.

Consolidated Balance Sheet

(Million yen)	End of FY2022	End of 1Q FY2023	Change Amt
Assets	237,451	237,408	-42
Current assets	157,873	154,771	-3,101
Non-current assets	79,578	82,636	+3,058
Total assets	237,451	237,408	-42

	End of FY2022	End of 1Q FY2023	Change Amt
Liabilities	41,518	34,609	-6,908
Current liabilities	35,183	28,655	-6,527
Non-current liabilities	6,334	5,953	-381
Equity	195,933	202,799	+6,866
Total liabilities and equity	237,451	237,408	-42

＝Assets＝

Cash and cash equivalents	-4,795
Inventories	-840
Other financial assets	＋2,810

＝Liabilities and equity＝

Trade and other payables	-4,177
Income taxes payable	-3,848
Retained earnings	+4,910

NS-065/NCNP-01 (viltolarsen)
- Treatment for Duchenne muscular dystrophy -

Development Phase	・Japan : Launch ・USA : Launch ・Global : PIII in progress
Origin	Co-development : National Center of Neurology and Psychiatry
Development	Nippon Shinyaku
Mechanism of action	Exon 53 Skipping
Indication	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

NS-87 (daunorubicin / cytarabine)
- Treatment for high-risk acute myeloid leukemia -

Development Phase	Japan : NDA filing
Origin	[Mar. 2017] Licensed-in from: Jazz Pharmaceuticals plc
Development	Nippon Shinyaku
Mechanism of action	Liposomal combination of daunorubicin and cytarabine
Indication	High-risk acute myeloid leukemia (High-risk AML)
Dosage form	Injection
Feature	・NS-87 is the first therapy for the treatment of high-risk AML in Japan ・The enhancement of antitumor activity and reducing adverse events are expected by NS-87 accumulated in bone marrow

ZX008 (fenfluramine hydrochloride)
- Treatment for rare intractable epilepsy -

Development Phase	Japan : Launch (Dravet syndrome) Japan : NDA filing (Lennox-Gastaut syndrome) Japan : PIII (CDKL5 deficiency disorder)
Origin	[Mar. 2019] Distribution partnership in Japan : UCB S.A. (former Zogenix, Inc.)
Development	UCB S.A. (former Zogenix, Inc.)
Mechanism of action	5-HT (serotonin) releaser with agonist activity at several 5-HT receptors
Indication	Dravet syndrome Lennox-Gastaut syndrome CDKL5 deficiency disorder
Dosage form	Oral liquid agent
Feature	・Effective for Dravet syndrome, 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

CAP-1002
- Treatment for Duchenne muscular dystrophy -

Development Phase	USA : PIII
Origin	[Jan. 2022] Partnership for commercialization in the US [Feb. 2023] Partnership for commercialization in Japan : Capricor Therapeutics, Inc.
Development	Capricor Therapeutics, Inc.
Mechanism of action	Exosomes released from cardiosphere-derived cells
Indication	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

GA101 (Obinutuzumab)

- Treatment for lupus nephritis, pediatric nephrotic syndrome -

Development Phase	Japan : PIII（LN） Global : PIII（PNS）
Origin	[Nov. 2012] Licensed-in from : Chugai Pharmaceutical Co., Ltd.
Development	Co-development : Chugai Pharmaceutical Co., Ltd.
Mechanism of action	Anti-CD20 monoclonal antibody
Indication	Lupus nephritis (LN) Pediatric nephrotic syndrome (PNS)
Dosage form	Injection
Feature	Anti-CD20 monoclonal antibody, increased antibody-dependent cellular cytotoxicity (ADCC) activity and direct cytotoxicity

NS-304 (selexipag)

- Treatment for pulmonary hypertension, arteriosclerosis obliterans -

Development Phase	Japan : PIIb （ASO） Japan : PII （Pediatric PAH）
Origin	Nippon Shinyaku
Development	・Nippon Shinyaku （ASO） ・Co-development : Janssen Pharmaceutical K.K. （Pediatric PAH）
Mechanism of action	Selective IP receptor agonist
Indication	・Arteriosclerosis obliterans (ASO) ・Pediatric pulmonary arterial hypertension (Pediatric PAH)
Dosage form	Tablet
Feature	Long-acting oral drug

NS-580

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

Development Phase	Japan : PIIb (Endometriosis) Japan : PIIa (CP/CPPS)
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	Inhibition of membrane-associated prostaglandin E synthase-1
Indication	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-018 (ilginatinib)
- Treatment for myelofibrosis -

Development Phase	Global : PII
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	JAK2 inhibitor
Indication	Myelofibrosis
Dosage form	Tablet
Feature	・Potent and highly selective JAK2 inhibitor ・High efficacy and safety are expected for myelofibrosis (MF) patients with low platelet count

NS-089/NCNP-02（brogidirsen）
- Treatment for Duchenne muscular dystrophy -

Development Phase	Global：Preparation for PII
Origin	Co-development : National Center of Neurology and Psychiatry
Development	Nippon Shinyaku
Mechanism of action	Exon 44 Skipping
Indication	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

NS-229

- Treatment for Eosinophilic granulomatosis with polyangiitis -

Development Phase	Global：Preparation for PII
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	JAK1 inhibitor
Indication	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

NS-401 (tagraxofusp)

- Treatment for blastic plasmacytoid dendritic cell neoplasm -

Development Phase	Japan : PI/II
Origin	[Mar. 2021] Licensed-in from: The Menarini Group
Development	Nippon Shinyaku
Mechanism of action	Induction apoptosis of cells by inhibiting protein synthesis by specifically targeting cancer cells expressing CD123
Indication	Blastic plasmacytoid dendritic cell neoplasm (BPDCN)
Dosage form	Injection
Feature	・Composed of diphtheria toxin (DT) fusion protein and recombinant human IL-3 ・Novel targeted therapy directed to CD123 on tumor cells ・IL-3 binds to CD123-expressing tumor cells and delivers the cytotoxic diphtheria toxin to the cells, resulting in the blockage of protein synthesis in the cell and causing cell death in CD123-expressing cells

NS-050/NCNP-03
- Treatment for Duchenne muscular dystrophy -

Development Phase	Global：Preparation for PI/II
Origin	Co-development : National Center of Neurology and Psychiatry
Development	Nippon Shinyaku
Mechanism of action	Exon 50 Skipping
Indication	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

NS-917 (radgocitabine)

- Treatment for relapsed or refractory acute myeloid leukemia -

Development Phase	Japan : PI
Origin	[Mar. 2017] Licensed-in from : Delta-Fly Pharma, Inc.
Development	Nippon Shinyaku
Mechanism of action	DNA strand-break by incorporating itself into DNA
Indication	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

NS-161
- Treatment for inflammatory diseases -

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

NS-025
- Treatment for urological diseases -

Development Phase	Japan : PI
Origin	Nippon Shinyaku
Development	Nippon Shinyaku
Mechanism of action	–
Indication	Urological diseases (to be determined)
Dosage form	Oral agent
Feature	–

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 statements in the two versions, the statements in the Japanese version shall prevail.

NIPPON SHINYAKU CO., LTD.

Nippon Shinyaku Co., Ltd.

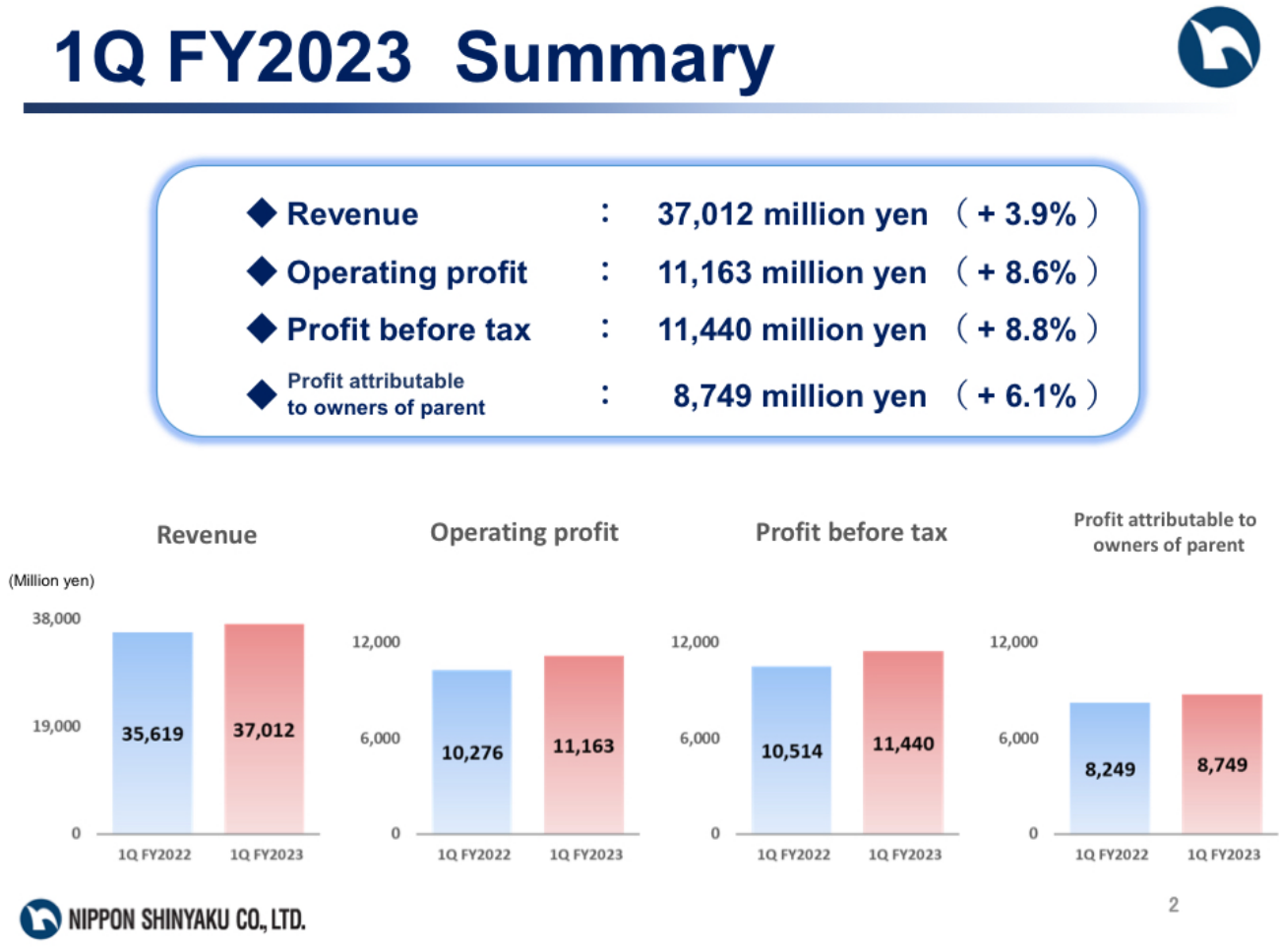
Financial Results Briefing for the 1st Quarter Ended June 30, 2023

August 10, 2023

Presentation

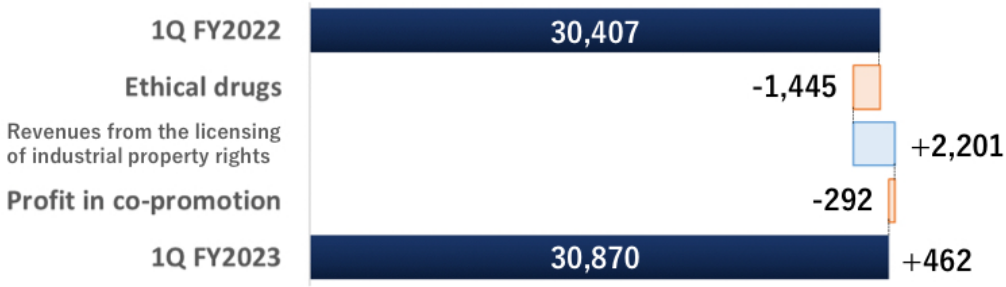
Edamitsu: I am Takanori Edamitsu, Nippon Shinyaku Co., Ltd. Director, General Manager, Business Management and Sustainability Division. Thank you very much for taking time out of your busy schedule to participate in our financial results briefing today. I appreciate it very much.

I will now explain our business results for Q1 of FY2023 and the progress of our R&D activities, in accordance with the presentation materials posted on our website.



As an overview of our performance in Q1 of FY2023, we reported consolidated revenue of JPY37,012 million, operating profit of JPY11,163 million, profit before tax of JPY11,440 million, and profit attributable to owners of the parent of JPY8,749 million.

Segmental Review - Pharmaceuticals -



(Million yen)	1Q FY2022		1Q FY2023		YoY Change	
	Results	Ratio	Results	Ratio	Amt	%
Ethical drugs	20,900	68.7%	19,454	63.0%	-1,445	-6.9%
Revenues from the licensing of industrial property rights	6,903	22.7%	9,104	29.5%	+2,201	+31.9%
Profit in co-promotion	2,603	8.6%	2,310	7.5%	-292	-11.2%
Revenue	30,407	100.0%	30,870	100.0%	+462	+1.5%

Despite the effect of price revision by MHLW* and generic products, revenue of consolidated pharmaceuticals segment increased by 1.5% due to increase of sales of Ethical drugs such as "Viltepso" and "Uptravi", and royalty revenue from Uptravi's overseas sales.

NIPPON SHINYAKU CO., LTD.

*MHLW : Ministry of Health, Labour and Welfare

In the pharmaceuticals business, consolidated net sales increased 1.5% YoY to JPY30,870 million despite the effect of price revision by MHLW and generic products, due to growth in sales of Viltepso, a treatment for Duchenne muscular dystrophy, and Uptravi, a treatment for pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension, and royalty income from overseas sales of Uptravi.

Segmental Review - Functional Food -

(Million yen)	1Q FY2022		1Q FY2023		YoY Change	
	Results	Ratio	Results	Ratio	Amt	%
Protein preparations	3,605	69.2%	4,284	69.8%	+679	+18.9%
Preservatives	726	13.9%	736	12.0%	+10	+1.4%
Supplements	335	6.4%	464	7.6%	+129	+38.7%
Health food ingredients	214	4.1%	306	5.0%	+91	+42.7%
Others	330	6.4%	350	5.6%	+20	+6.2%
Revenue	5,211	100.0%	6,142	100.0%	+931	+17.9%

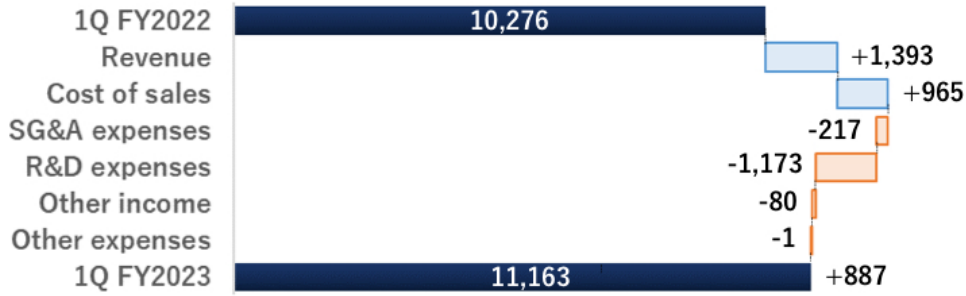
Revenue of consolidated functional food segment increased by 17.9% through sales increase of Protein preparations and Supplements.

NIPPON SHINYAKU CO., LTD.

4

In the functional foods business, sales of protein preparations and supplements increased, and consolidated net sales in the functional foods business increased 17.9% YoY to JPY6,142 million.

Operating profit



(Million yen)	1Q FY2022		1Q FY2023		YoY Change	
	Results	Ratio	Results	Ratio	Amt	%
Revenue	35,619	100.0%	37,012	100.0%	+1,393	+3.9%
(Pharmaceuticals)	(30,407)	(85.4%)	(30,870)	(83.4%)	(+462)	(+1.5%)
(Functional Food)	(5,211)	(14.6%)	(6,142)	(16.6%)	(+931)	(+17.9%)
Cost of sales	13,928	39.1%	12,962	35.0%	-965	-6.9%
SG&A expenses	8,200	23.0%	8,418	22.7%	+217	+2.7%
R&D expenses	4,738	13.3%	5,911	16.0%	+1,173	+24.8%
Other income	1,652	4.6%	1,572	4.2%	-80	-4.8%
Other expenses	128	0.3%	129	0.3%	+1	+1.0%
Operating profit	10,276	28.9%	11,163	30.2%	+887	+8.6%

The cost to sales ratio improved by 4.1 percentage points YoY to 35%, due to factors such as the sales mix, including an increase in revenues from the licensing of industrial property rights. SG&A expenses increased 2.7% YoY to JPY8,418 million, mainly due to an increase in sales promotion fees in line with increased domestic sales of Uptravi.

R&D expenses totaled JPY5,911 million, up 24.8% YoY, mainly due to an increase in contract research expenses in line with progress in clinical trials.

As a result, operating profit was JPY11,163 million, up 8.6% YoY.

Profit attributable to owners of parent

(Million yen)	1Q FY2022 Results	1Q FY2023 Results	YoY Change Amt	YoY Change %
Operating profit	10,276	11,163	+887	+8.6%
Finance income	266	298	+31	+12.0%
Finance costs	28	21	-6	-23.1%
Profit before tax	10,514	11,440	+925	+8.8%
Income tax expense, etc	2,265	2,690	+425	+18.8%
Profit attributable to owners of parent	8,249	8,749	+500	+6.1%

Profit before tax was JPY11,440 million, up 8.8% YoY, and profit attributable to owners of the parent was JPY8,749 million, up 6.1% YoY.

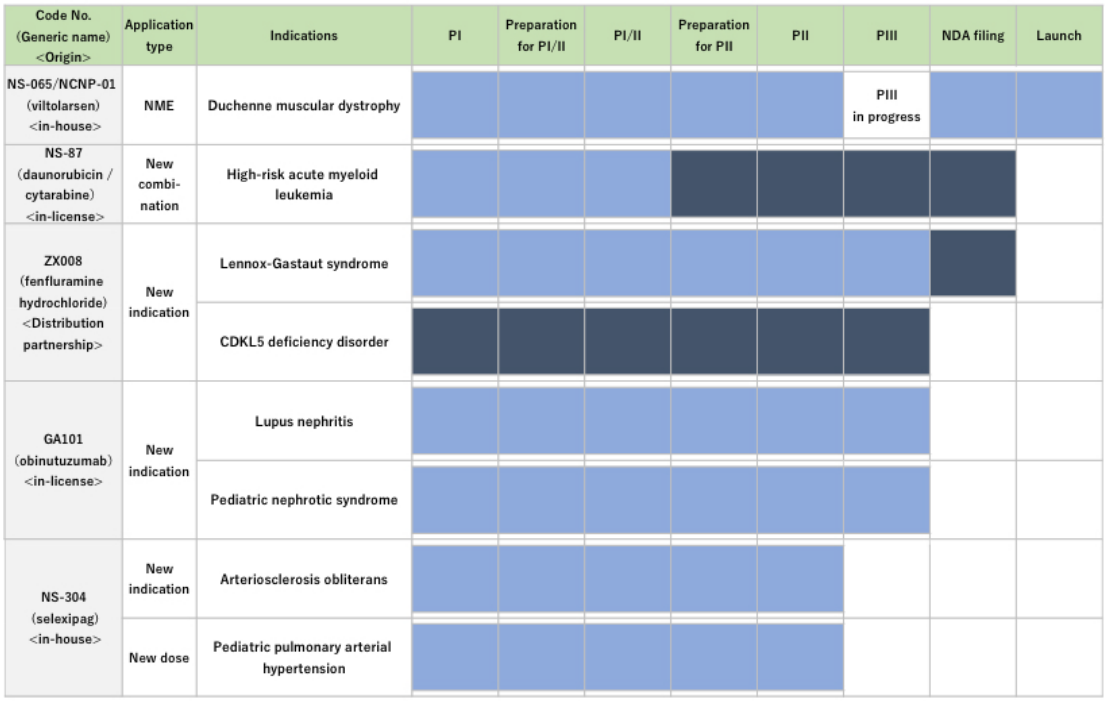
Business Forecast for FY2023

(Million yen)	FY2022		FY2023			
	1Q Results	FY Results	1Q Results	Progress for 1H	1H Forecasts	FY Forecasts
Revenue	**35,619**	**144,175**	**37,012**	**51.8%**	**71,500**	**145,000**
(Pharmaceuticals)	(30,407)	(121,988)	(30,870)	(51.0%)	(60,500)	(123,500)
(Functional Food)	(5,211)	(22,187)	(6,142)	(55.8%)	(11,000)	(21,500)
Operating profit	**10,276**	**30,049**	**11,163**	**69.8%**	**16,000**	**32,000**
Profit before tax	**10,514**	**30,489**	**11,440**	**70.6%**	**16,200**	**32,500**
Profit attributable to owners of parent	**8,249**	**22,812**	**8,749**	**70.6%**	**12,400**	**25,000**

Revenue and each profit have progressed toward achievement of 1H, FY forecasts.

The consolidated earnings forecast for FY2023 remains unchanged from that announced on May 11, with consolidated revenue of JPY145 billion, operating profit of JPY32 billion, profit before tax of JPY32.5 billion, and profit attributable to owners of the parent of JPY25 billion.

R&D Pipeline (Domestic)



NIPPON SHINYAKU CO., LTD.

：changes from the Fiscal Year Ended March 31, 2023

Next, I will explain the progress of R&D items.

First, I would like to explain the development situation in Japan. Exon 53 skipping, NS-065/NCNP-01, Viltepso, for the treatment of Duchenne muscular dystrophy, was launched in May 2020 and is currently in global Phase III trials.

The Phase I/II study for NS-87, a treatment for high-risk acute myeloid leukemia, was completed and an application for approval was filed in June 2023.

In June 2023, UCB submitted a partial change application for ZX008, a drug for the treatment of intractable epilepsy, for an additional indication for Lennox-Gastaut syndrome.

In addition, UCB is conducting a Phase III study for CDKL5 deficiency starting in July 2023.

A Phase III study of GA101 for lupus nephritis is in Phase III trials in collaboration with Chugai Pharmaceutical Co.

In addition, a Phase III study for pediatric nephrotic syndrome is underway.

A Phase IIb study of NS-304 for the indication of arteriosclerosis obliterans is being conducted by Nippon Shinyaku on its own.

In addition, a Phase II study for pediatric pulmonary arterial hypertension is underway in collaboration with Janssen Pharmaceutical K.K.

R&D Pipeline (Domestic)

Code No. (Generic name) <Origin>	Application type	Indications	PI	Preparation for PI/II	PI/II	Preparation for PII	PII	PIII	NDA filing	Launch
NS-580 <in-house>	NME	Endometriosis								
		Chronic prostatitis / Chronic pelvic pain syndrome								
NS-089/NCNP-02 (brogidirsen) <in-house>	NME	Duchenne muscular dystrophy								
NS-229 <in-house>	NME	Eosinophilic granulomatosis with polyangiitis								
NS-401 (tagraxofusp) <in-license>	NME	Blastic plasmacytoid dendritic cell neoplasm								
NS-050/NCNP-03 <in-house>	NME	Duchenne muscular dystrophy								
NS-917 (radgocitabine) <in-license>	NME	Relapsed/refractory acute myeloid leukemia								
NS-161 <in-house>	NME	Inflammatory diseases								
NS-025 <in-house>	NME	Urological diseases								

NIPPON SHINYAKU CO., LTD.

■ : changes from the Fiscal Year Ended March 31, 2023

Phase IIb study is underway for NS-580, an endometriosis treatment.

In addition, a Phase IIa study for chronic prostatitis and chronic pelvic pain syndrome was initiated in June 2023.

A global Phase II study for exon 44 skipping, NS-089/NCNP-02, a treatment for Duchenne muscular dystrophy, is in preparation.

A global Phase II study for NS-229, a treatment for eosinophilic granulomatosis with polyangiitis, is in preparation.

A Phase I/II study of NS-401 for the treatment of blastic plasmacytoid dendritic cell neoplasm is underway.

A global Phase I/II study for exon 50 skipping, NS-050/NCNP-03, a treatment for Duchenne muscular dystrophy, is in preparation.

A Phase I trial for NS-917 for the treatment of relapsed/refractory acute myeloid leukemia is underway.

Phase I trials are underway for NS-161, which is being developed for the treatment of inflammatory diseases, and for NS-025, which is being developed for the treatment of urological diseases.

R&D Pipeline (Overseas)

Code No. (Generic name) <Origin>	Application type	Indications	PI	Preparation for PI/II	PI/II	Preparation for PII	PII	PIII	NDA filing	Launch
NS-065/NCNP-01 (viltolarsen) <in-house>	NME	Duchenne muscular dystrophy						PIII in progress		
CAP-1002 <partnership>	NME	Duchenne muscular dystrophy								
NS-018 (ilginatinib) <in-house>	NME	Myelofibrosis								
NS-089/NCNP-02 (brogidirsen) <in-house>	NME	Duchenne muscular dystrophy								
NS-229 <in-house>	NME	Eosinophilic granulomatosis with polyangiitis								
NS-050/NCNP-03 <in-house>	NME	Duchenne muscular dystrophy								

■ : changes from the Fiscal Year Ended March 31, 2023

Next, I will explain the status of overseas development.

NS-065/NCNP-01, Viltepso, for the treatment of Duchenne muscular dystrophy, was launched in the US in August 2020 and is currently in a global Phase III study.

We have signed a partnership agreement for commercialization for CAP-1002 for the treatment of Duchenne muscular dystrophy with Capricor Therapeutics, Inc. in the US in January 2022 and in Japan in February 2023. Capricor Therapeutics, Inc. is currently conducting Phase III trials in the US.

A global Phase II study for NS-018, a drug for myelofibrosis, is underway.

To reiterate, we are currently preparing global trials for NS-089/NCNP-02, NS-050/NCNP-03, and NS-229.

This concludes the overview of our R&D activities.

FY2023 1Q Financial Results Briefing Q&A (Summary)

Held on August 10, 2023

NO	Questions	Answers
1	We felt that the progress of the profit in 1Q was fairly good. Describe the reasons for the good progress of the profit to 1H forecast and the prospects for full year forecast.	Revenue progressed steadily overall. Furthermore, sales of U.S.Viltepso and royalty income from Uptravi grew more than expected due to the yen's depreciation. As a result, overall sales are strengthening. With regard to expenses, the progress of the marketing service expenses is as planned, but progress of R&D expenses has been slightly delayed due to a shift in the schedule for the manufacture of bulk nucleic acid pharmaceuticals to the second half. We think expenses will progress as planned for the full fiscal year.
2	What are the actual and assumed exchange rates?	The actual result is ¥137.5 to USD and the assumption is ¥130 to USD.
3	Other income is about 1.5 billion yen, but does this include a foreign exchange gain? Alternatively, is it another component?	It is the exchange gain. It was about 1.5 billion yen in the previous fiscal year and about 1.4 billion yen in the current fiscal year.
4	Tell us about the situation of domestic Viltepso. In contrast to the full-year sales increase forecast, 1Q sales have declined YoY, and the progress rate does not seem to be very high. What is the factor behind this? And what do you think about the future outlook?	In 1Q, none of the patients were interrupted, and there was acquisition of the new patients and resumption of administration. In the future, we believe that this increase of patients receiving this drug will lead to sales increase.
5	Tell us about the situation of domestic Viltepso. The material of Central Social Insurance shows that the number of patients administered at the peak will be 128.As of the end of the previous year, it has been heard that the drug has been administered to about 90 patients. Although sales of 1Q are almost leveling off, what is the current situation and how many patients are treated? Tell us how many patients increase each year.	In 1Q, several new patients have been started and 90 patients plus-alpha have been treated. There were no dropouts in 1Q. Regarding the progress to 128 patients at the peak in the material of Central Social Insurance Medical Council, many patients who we have grasped at present have not been administered because many of them are older, and so only 90 patients have been administered. On the other hand, there are more new patients than dropouts, and we think that the number of patients receiving them will gradually increase.
6	Tell us about the situation of Viltepso in the United States. In the United States, gene therapy has been received accelerated approval and its promotion has been started. I think the interest of patients and doctors in gene therapy is growing. Tell us how the reputation of gene therapy and its impact on Viltepso are currently being viewed.	Gene therapy has been received accelerated approval in the United States, and Sarepta recently announced that one patient had been treated. In the future, the administration of gene therapy will be advanced in the U.S. We believe that the effects of Viltepso should be adequately appealed as we did, so that it has no major impact. We believe that we will know whether or not it is affected in the future. We need to look at the results of gene-therapy P3 study which will come within the year.
7	Tell us about the situation of Viltepso in the United States. We think Viltepso Sales in the US is strong. We have	Regarding the Quick Start Program, the change in the contractor for HUB servicing was smoothly completed and it has

	heard that the Quick Start Program has been started in June, but there is still no contribution of the Quick Start Program to 1Q's performance. We think that the contribution will accelerate from 2Q.Is it a correct perception?	been started since June, so we expect it to gradually become effective from June onward.
8	In the United States, is there any information about whether there are new patients who are preparing for Viltepso and are much interested in gene therapy?	We haven't received such information.
9	I think Galactic53 study, a long-term P2 study of Viltepso, is ended in June. Will the results be disclosed in the fall?	Data QC and analysis times are also needed, and we would like to publish the data at the appropriate timing in medical conferences. So the result will be disclosed in the next year
10	Will the results of Viltepso P3 study be disclosed within the year?	Not yet. The study will be completed in December 2024.
11	We have heard FPI of NS-089 and NS-050 is in 1H. Tell us more specifically about the developmental status of NS-089, NS-050 and NS-051.	The preparation of NS-089 P2 study is being promoted to achieve FPI in 1H. NS-050 is also moving toward FPI in 1H as planned, but there is a slight possibility that it will not fit in 1H due to concordance with the institution. NS-051 is under discussion with FDA to begin the study within this fiscal year as originally planned.
12	NS-089 received breakthrough therapy designation. Tell us about the implications and impacts. Also, is it possible to receive breakthrough therapy designation for other nucleic acid products currently under development?	Until now, the discussion with FDA has only been for certain meetings, but in the future we can get support from FDA as appropriate for the development of NS-089. FDA is also committed to the review. We believe that it is of great significance to receive full support from FDA. With regard to breakthrough therapy designation for other items, we would like to challenge if there was an opportunity, but we think that it will depend on the situation in the future whether all items can be ridden on the same rail in general because the situation is different for each item.
13	We think that the interim analysis of CAP-1002 will be within this year. Despite the remainder of this year is more than 4 months, ClinicalTrials.gov says the patient is still being recruited. Tell us about the current situation.	The study has progressed as planned, and the interim analysis will be conducted by December as planned. At present, 48 cases have been entered against the target sample size of 68 cases.
14	Tell us about CAP-1002. I have heard that the approval of drugs with unclear mechanisms, such as cellular therapies, by the U.S. and Japanese regulatory authorities is quite difficult. CAP-1002 is an allogeneic CDC therapy and the mechanism seems to be slightly obscured. I think the mechanism is not clear and FDA's review may not be smooth, even if CAP-1002 test outcomes are good in the future. What do you think about that?	Although it is a cell therapy, we do not believe that it negatively affects the review. Since ongoing P3 study is a confirmatory study, if the efficacy can be clearly confirmed in this study, it will undoubtedly be approved regardless of the cell therapy. Regarding to mechanism, it is not unexplained, because anti-inflammatory and anti-fibrotic effects, etc. are clearly explained.
15	NS-018 has obtained European orphan designations. what is the recruitment status of the patient? I think the number of clinical trial sites is increasing in Europe and Asia, but is there no change in the schedule for study completion date, April of the next year?	Although the start of the study was delayed, the number of study sites in Europe has increased, and patients have begun to enter relatively smoothly. In the future, we will determine how soon we can catch up with our initial schedule.
16	For NS-229, the indication has been disclosed as eosinophilic granulomatosis with polyangiitis (EGPA). Please explain the numbers of patients and why JAK1 inhibitors are effective against this disease.	This disease is an autoimmune disease and designated as an intractable disease. The information on the patient number is about 6000 persons in Japan. Because of autoimmune diseases, steroids have been used as a base treatment, but

		because of repeated relapses, there is a medical need. JAK1 has immunosuppressive effects and is expected to be used to reduce steroidal use.
17	Are JAK1 inhibitors like NS-229 developed by other companies?	JAK1 inhibitor for EGPA is only our product.
18	On NS-229, I'm interested in the indication extension to other diseases, because it is JAK1 inhibitor. Is it correct to think that P2 studies will be conducted for the present diseases and then will the indications be expanded? We believe that JAK1 has safety issues. Tell us about the potential for future expansion of indications.	As a mechanism of NS-229 is immune suppression immunity, we are considering expanding indications to other inflammatory diseases in addition to EGPA.
19	We think that candidates for expanded indications of NS-229 are atopic in inflammatory diseases, and ulcerative colitis and Crohn's disease in autoimmune diseases. From the current data, what disease is suitable for expanded indications?	With regard to areas where other antecedent JAK1 inhibitors have already been launched, we think that the treatment need is already met and will not enter the market. We think that there is a need for our product more in diseases such as EGPA where JAK1 inhibitors has not used and unmet medical needs exist. Expansion of indications to such diseases is considered.
20	Regarding NS-580, P2a study for chronic prostatitis/chronic pelvic pain syndrome has started since June. Because P2a study has ended for the indication of endometriosis, is our understand correct that POC to stop the pain is confirmed?	Your understanding is correct.
21	Tell us why chronic prostatitis/chronic pelvic pain syndromes for NS-580 indications was chosen.	Although there were several candidates for indications, one reason is that we have strengths in the urology field. In terms of the nature of the disease, it has been confirmed that NS-580 is effective for pain. Although there are other medications for short-term pain control, there are no medications that can be used for long periods of time, so we decided to develop chronic prostatitis/chronic pelvic pain syndrome with chronic pain and discomfort.
22	I think other companies have also developed membrane-associated prostaglandin E synthase-1. Is my understanding correct that you are the top batter currently?	Your understanding is correct.
23	Do you think that NS-580 should be licensed out abroad?	The possibility is also considered.
24	Tell us about the status of functional food business. In the full-year forecast, sales will decrease due to factors such as a decline in sales prices, but from 1Q results, there is no sign of decrease. Are Sales and prices declining from 2Q , or are current status changing?	Although raw material prices have declined and stabilized, there is some uncertainty about selling prices because they are affected by the impact of exchange rates. In the future, if the exchange rate is shifted to strong yen, we think that the sales price will decrease. On the other hand, because the effect of coronavirus is being resolved and sports events have been held and the demand for inbound has recovered, the actual increase in quantity is considered to have a larger effect.