# Outline of Consolidated Financial Results for the 1st Quarter Ended June 30, 2024

August 7, 2024
NIPPON SHINYAKU CO., LTD.

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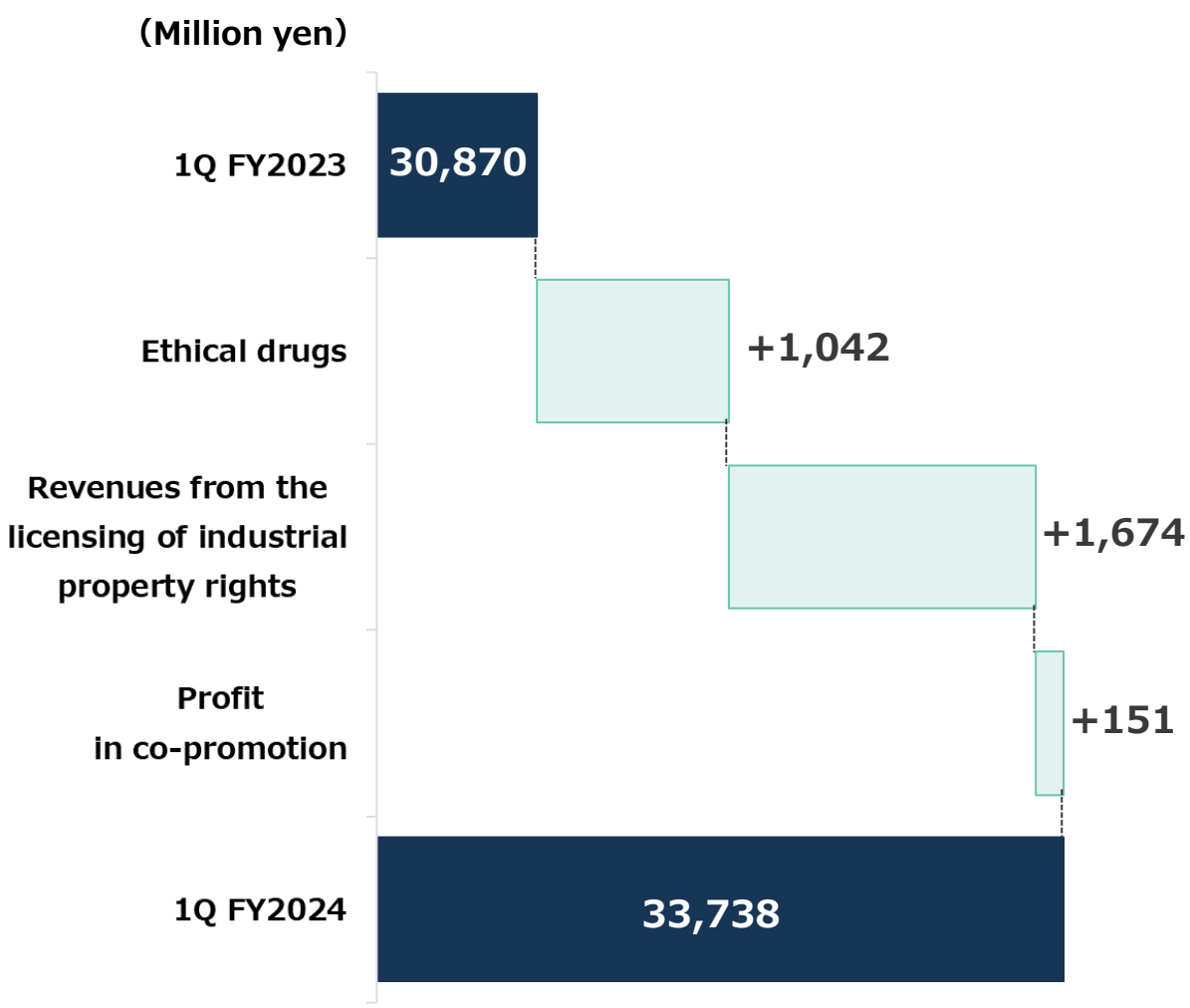
# 1Q FY2024 Summary

| (Million yen) | 1Q FY2023 | | 1Q FY2024 | | YoY Change | |
|---|---|---|---|---|---|---|
| | Results | Ratio | Results | Ratio | Amt | % |
| **Revenue** | **37,012** | **100.0%** | **39,131** | **100.0%** | **+2,118** | **+5.7%** |
| (Pharmaceuticals) | (30,870) | (83.4%) | (33,738) | (86.2%) | (+2,868) | (+9.3%) |
| (Functional Food) | (6,142) | (16.6%) | (5,393) | (13.8%) | (-749) | (-12.2%) |
| **Cost of sales** | **12,962** | **35.0%** | **12,636** | **32.3%** | **-326** | **-2.5%** |
| **SG&A expenses** | **8,418** | **22.7%** | **9,221** | **23.6%** | **+803** | **+9.5%** |
| **R&D expenses** | **5,911** | **16.0%** | **7,497** | **19.2%** | **+1,586** | **+26.8%** |
| **Other income** | **1,572** | **4.2%** | **1,507** | **3.9%** | **-64** | **-4.1%** |
| (Foreign exchange gain) | (1,422) | (3.8%) | (1,211) | (3.1%) | (-210) | (-14.8%) |
| **Other expenses** | **129** | **0.3%** | **204** | **0.5%** | **+74** | **+58.0%** |
| **Operating profit** | **11,163** | **30.2%** | **11,078** | **28.3%** | **-85** | **-0.8%** |
| **Finance income** | **298** | **0.8%** | **363** | **0.9%** | **+65** | **+21.8%** |
| **Finance costs** | **21** | **0.1%** | **31** | **0.1%** | **+9** | **+42.0%** |
| **Profit before tax** | **11,440** | **30.9%** | **11,411** | **29.2%** | **-29** | **-0.3%** |
| **Income tax expense, etc** | **2,690** | **7.3%** | **1,146** | **2.9%** | **-1,544** | **-57.4%** |
| **Profit attributable to owners of parent** | **8,749** | **23.6%** | **10,264** | **26.2%** | **+1,514** | **+17.3%** |

# Segmental Review - Pharmaceuticals -




**Ethical drugs 20,496 million yen**
**(+1,042 million yen, +5.3%, YoY)**

- ✓ Sales growth of Uptravi and Viltepso, etc.
- ✓ Decrease in sales of Vidaza, Tramal/Onetram, etc.

**Revenues from the industrial property rights**
**10,779 million yen**
**(+1,674 million yen, +18.4%, YoY)**

- ✓ Royalty revenue growth due to overseas sales of Uptravi

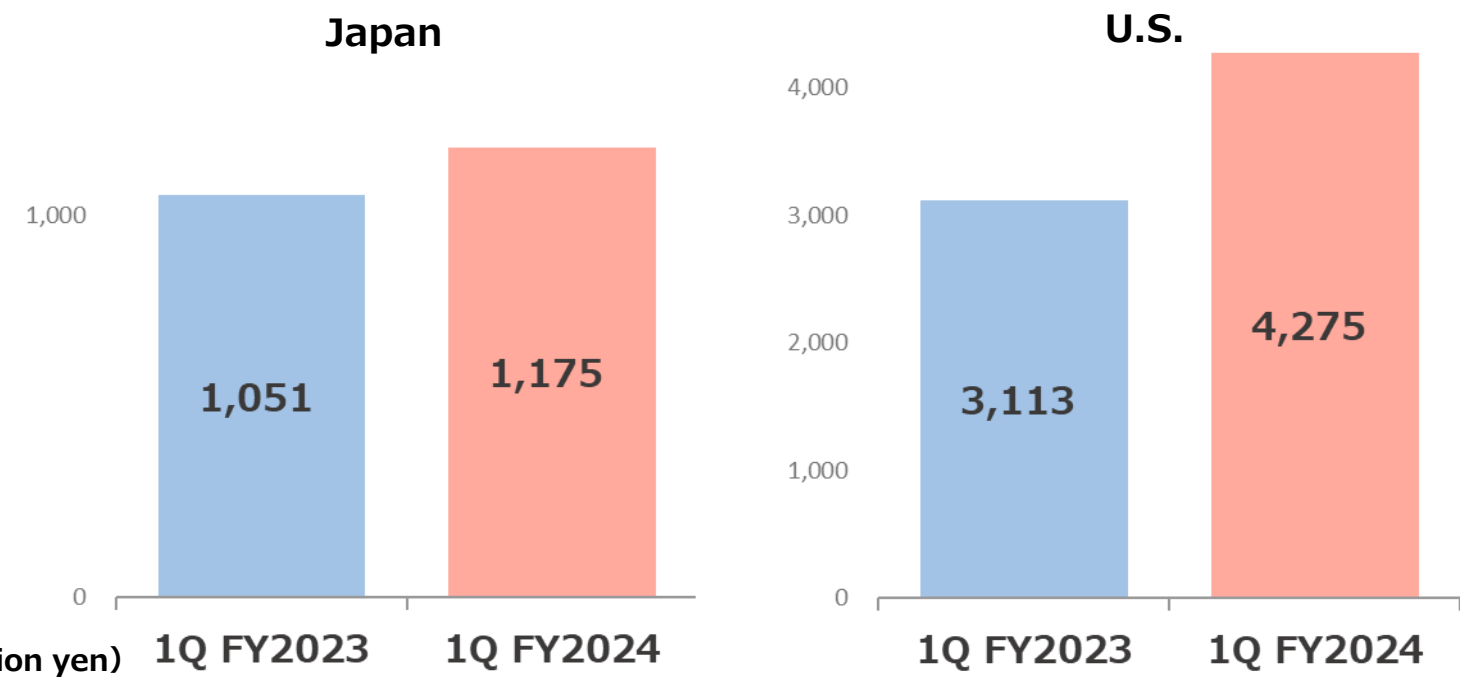
**Profit in co-promotion 2,461illion yen**
**( + 151 million yen, + 6.5%, YoY)**

- ✓ Sales growth of Opsumit and Erleada

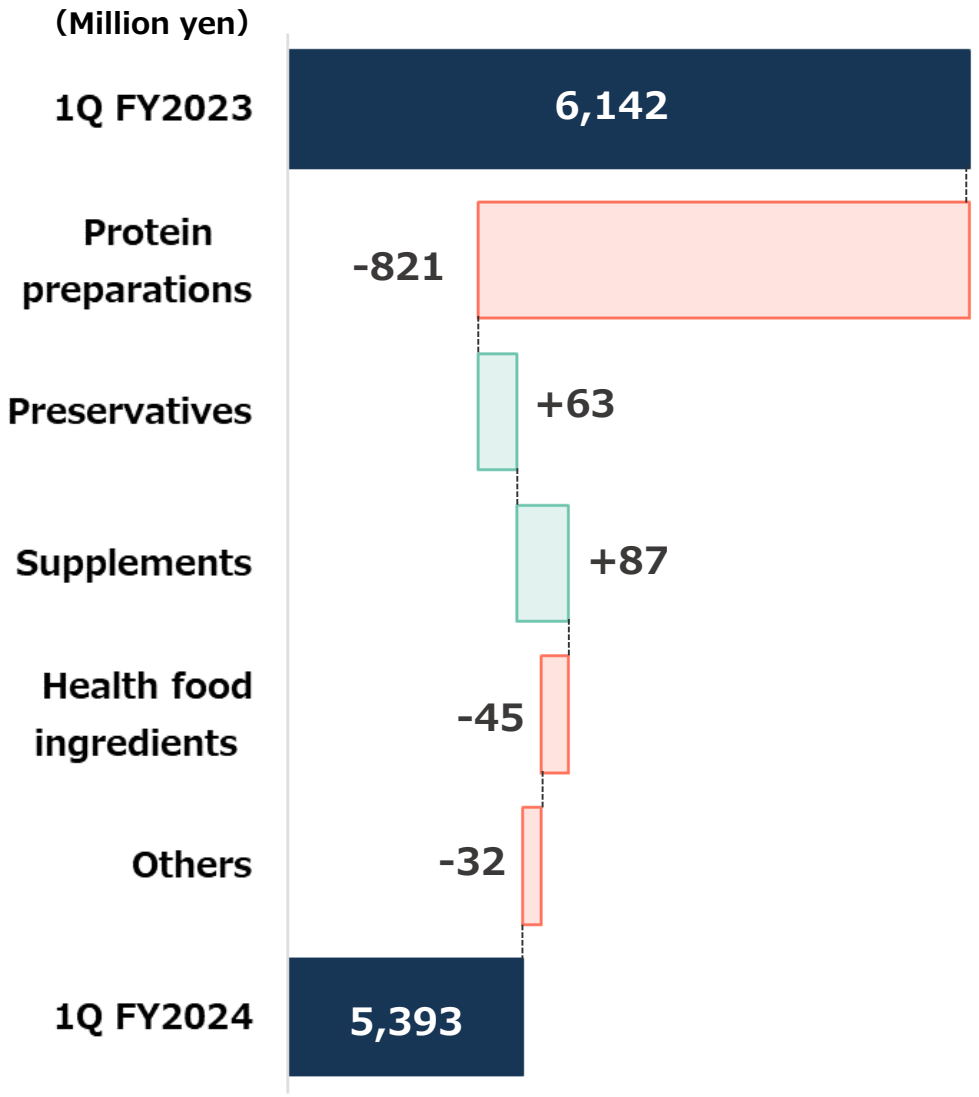
# Sales Trends of Viltepso® (viltolarsen)

| (Million yen) | | 1Q FY2023 Results | 1Q FY2024 Results | YoY Change Amt | YoY Change % | |
|---|---|---|---|---|---|---|
| Viltepso | Japan | 1,051 | 1,175 | +124 | +11.8% | ✓The number of patients currently receiving the drug is more than two-thirds of the peak number of 128 patients in the data by the Central Social Insurance Medical Council (Chuikyo) . |
| | U.S. | 3,113 | 4,275 | +1,162 | +37.3% | ✓The number of patients receiving or wishing to receive the drug is increasing.<br>✓Phase III Trial (RACER53 Study) is under additional analysis. |
| | total | 4,165 | 5,450 | +1,285 | +30.9% | |

| Exchange rate | 1Q FY2023 Actual rate | 1Q FY2024 Actual rate |
|---|---|---|
| 1USD | 137.5yen | 155.9yen |



# Segmental Review - Functional Food -




**Protein preparations 3,463 million yen**
**(-821 million yen, -19.2%, YoY)**

✓ Decline in unit sales prices

**Preservatives 800 million yen**
**(+63 million yen, +8.6%, YoY)**

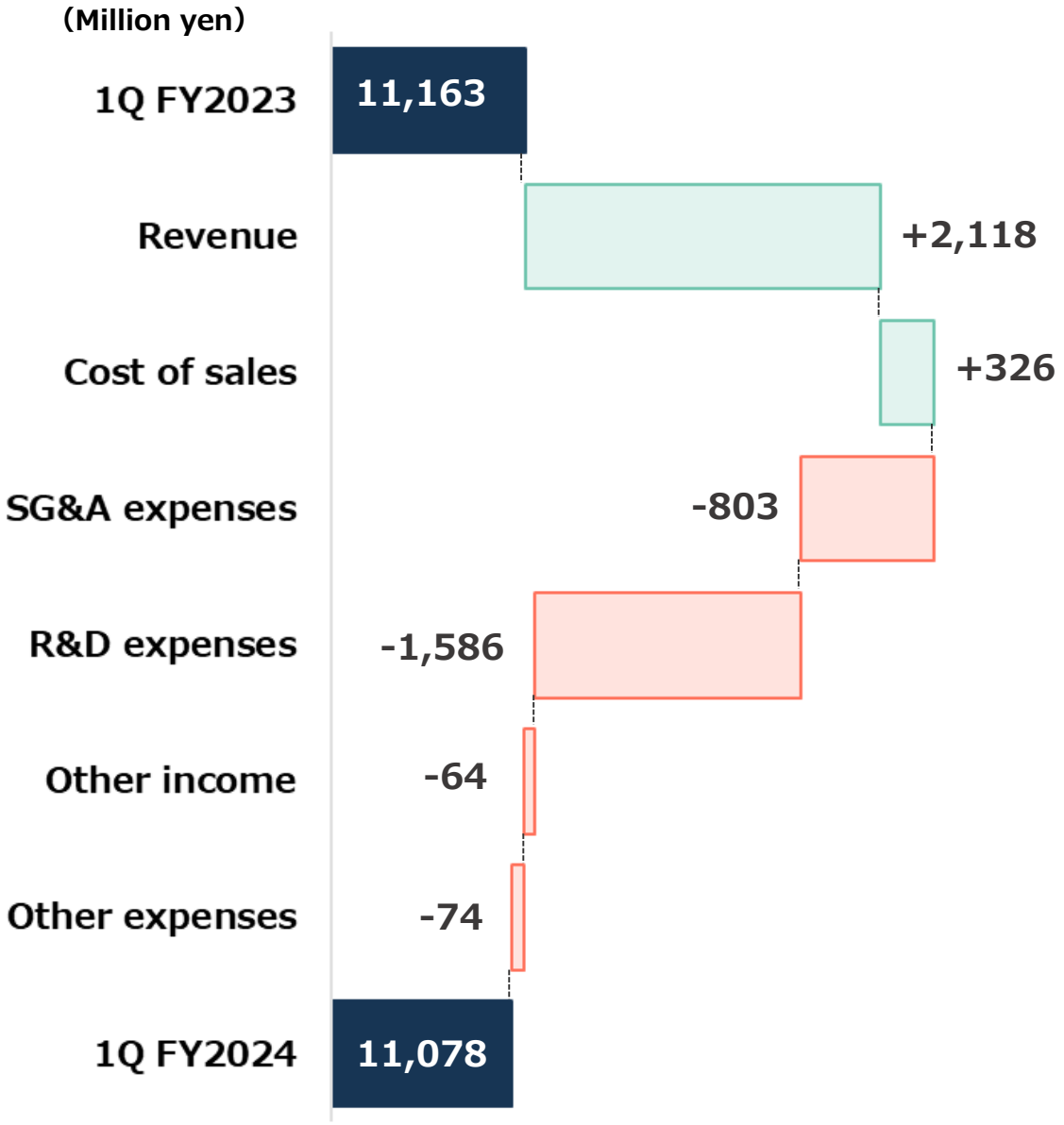
✓ Recovery in food service-related business due to active tourism and excursions

**Supplements 552 million yen**
**(+87 million yen, +18.8%, YoY)**

✓ Growth in both the sports and anti-aging care fields

**Health food ingredients 260 million yen**
**(-45 million yen, +14.8%, YoY)**

# Operating Profit




**Revenue 39,131 million yen**
**(+2,118 million yen, +5.7%, YoY)**

✓ Increase in revenue from industrial property rights and the U.S. sales of Viltepso.

**Cost of sales 12,636 million yen**
**(-326 million yen, -2.5%, YoY)**
**The ratio was improved by 2.7 points YoY.**

✓ Negative impact of NHI price revision
✓ Cost of sales ratio improvement due to factors such as revenues from industrial property rights and the change in sales segment mix (pharma vs. food)

**SG&A expenses 9,221 million yen**
**(+803 million yen, +9.5%, YoY)**

✓ Increase in promotional expenses in NS Pharma
✓ Increase in commission for promotional activities of Uptravi

**R&D expenses 7,497 million yen**
**(+1,586 million yen, +26.8%, YoY)**

✓ Increase in cost of investigational products

# Business Forecast for FY2024 (consolidated)

| (Million yen) | FY2023 | | FY2024 | | | |
|---|---|---|---|---|---|---|
| | 1Q Results | FY Results | 1Q Results | Progress for 1H | 1H Forecasts | FY Forecasts |
| **Revenue** | **37,012** | **148,255** | **39,131** | **51.6%** | **75,800** | **154,000** |
| (Pharmaceuticals) | (30,870) | (125,105) | (33,738) | (51.7%) | (65,300) | (132,500) |
| (Functional Food) | (6,142) | (23,150) | (5,393) | (51.4%) | (10,500) | (21,500) |
| **Cost of sales** | **12,962** | **50,234** | **12,636** | | **25,400** | **51,000** |
| **SG&A expenses** | **8,418** | **34,959** | **9,221** | | **18,600** | **38,700** |
| **R&D expenses** | **5,911** | **31,676** | **7,497** | | **16,800** | **32,400** |
| **Other income** | **1,572** | **3,163** | **1,507** | | **350** | **500** |
| **Other expenses** | **129** | **1,252** | **204** | | **350** | **400** |
| **Operating profit** | **11,163** | **33,295** | **11,078** | **73.9%** | **15,000** | **32,000** |
| **Finance income** | **298** | **650** | **363** | | **250** | **600** |
| **Finance costs** | **21** | **329** | **31** | | **50** | **100** |
| **Profit before tax** | **11,440** | **33,616** | **11,411** | **75.1%** | **15,200** | **32,500** |
| **Income tax expense, etc** | **2,690** | **7,764** | **1,146** | | **1,700** | **3,500** |
| **Profit attributable to owners of parent** | **8,749** | **25,851** | **10,264** | **76.0%** | **13,500** | **29,000** |

| Exchange rate | 1Q FY2023 Actual rate | 1Q FY2024 Actual rate | FY2024 Forecast rate |
|---|---|---|---|
| 1USD | 137.5yen | 155.9yen | 140.0yen |

# Segmental Forecast - Pharmaceuticals -

| (Million yen) | FY2023 | | FY2024 | | YoY Change | |
|---|---|---|---|---|---|---|
| | 1Q Results | FY Results | 1Q Results | FY Forecasts | Amt | % |
| Ethical drugs | 19,456 | 76,143 | 20,496 | 81,300 | +5,157 | +6.8% |
| Revenue from the licensing of industrial property rights | 9,104 | 40,304 | 10,779 | 42,100 | +1,796 | +4.5% |
| Profit in co-promotion | 2,310 | 8,658 | 2,461 | 9,100 | +442 | +5.1% |
| Revenue | 30,870 | 125,105 | 33,738 | 132,500 | +7,395 | +5.9% |

# Segmental Forecast - Functional Food -

| (Million yen) | FY2023 | | FY2024 | | YoY Change | |
|---|---|---|---|---|---|---|
| | 1Q Results | FY Results | 1Q Results | FY Forecasts | Amt | % |
| Protein preparations | 4,284 | 15,600 | 3,463 | 13,000 | -2,600 | -16.7% |
| Preservatives | 736 | 3,105 | 800 | 3,200 | +95 | +3.1% |
| Supplements | 464 | 1,905 | 552 | 3,100 | +1,195 | +62.7% |
| Health food ingredients | 306 | 1,248 | 260 | 1,100 | -148 | -11.9% |
| Others | 349 | 1,290 | 316 | 1,100 | -190 | -14.7% |
| Revenue | 6,142 | 23,150 | 5,393 | 21,500 | -1,650 | -7.1% |

# R&D PIPELINE

# R&D Updates (1/2)

For updates since May 14, 2024, see highlighted text in red.

| Recent status/event | Code No. (Generic name) | Product name | Indications and topics | Schedule |
|---|---|---|---|---|
| Launch | ZX008 (fenfluramine) | Fintepla | Lennox-Gastaut syndrome (additional indication) | March 2024 |
| Launch | NS-87 (daunorubicin / cytarabine) | Vyxeos | high-risk acute myeloid leukemia | May 2024 |
| Approval | LY3527727 (piltobrutinib) | Jaypirca | patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors | June 2024 |
| In application | NS-304 (selexipag) | Uptravi | pediatric pulmonary arterial hypertension | April 2024 |
| Start of P3 | GA101 (obinutuzumab) | Gazyva | systemic lupus erythematosus without nephropathy | October 2023 |
| Start of P2 | NS-089/NCNP-02 (brogidirsen) | – | Duchenne muscular dystrophy | February 2024 |
| | NS-229 | – | eosinophilic granulomatosis with polyangiitis | June 2024 |
| In-license (Vicore Pharma, Sweden) | C21 | – | idiopathic pulmonary fibrosis | Contract signed in February 2024 |
| Alliance Agreement (Eli Lilly Japan) | LY3527727 (piltobrutinib) | Jaypirca | mantle cell lymphoma (MCL)<br>chronic lymphocytic leukemia (CLL) | Contract signed in March 2024 |
| Temporarily suspended | NS-580 | – | endometriosis<br>chronic prostatitis / chronic pelvic pain syndrome | – |

# R&D Updates (2/2)

For updates since May 14, 2024, see highlighted text in red.

| Recent status/event | Code No. (Generic name) | Product name | Indications and topics | Schedule |
|---|---|---|---|---|
| Preliminary analysis results | NS-065/NCNP-01 (viltolarsen) | Viltepso | global Phase 3 trial (RACER53 Study) | May 2024 |
| Conference Presentations | NS-065/NCNP-01 (viltolarsen) | Viltepso | Phase 2 trial (Galactic53 trial): 2024 Muscular Dystrophy Association Clinical & Scientific Conference | March 2024 |
| Manuscript | NS-089/NCNP-02 (brogidirsen) | – | non-clinical data: (Molecular Therapy Nucleic Acids) | October 2023 |
| Orphan Drug Designation | NS-089/NCNP-02 (brogidirsen) | – | Duchenne muscular dystrophy | December 2023 (EU) |
| | NS-401 (tagraxofusp) | – | blastic plasmacytoid dendritic cell neoplasm | August 2023 (Japan) |
| | NS-229 | – | eosinophilic granulomatosis with polyangiitis | January 2024 (EU) |
| Alliance (MiNA Therapeutics) | – | – | a joint research agreement with the aim of creating nucleic acid medicines that are expected to be applied to an intractable and rare disease in the CNS field | April 2024 |

# REFERENCE MATERIALS

# Sales By Product in Pharmaceutical Segment

(Million yen)

| Brand name | Indications | 1Q FY2023 Results | 1Q FY2024 Results | YoY Change | FY2024 Forecasts |
|---|---|---|---|---|---|
| Viltepso | | 4,165 | 5,450 | +30.9% | 20,600 |
| (Japan) | Duchenne muscular dystrophy | (1,051) | (1,175) | (+11.8%) | (4,600) |
| (U.S.) | | (3,113) | (4,275) | (+37.3%) | (16,000) |
| Uptravi | pulmonary arterial hypertension/ chronic thromboembolic pulmonary hypertension | 3,255 | 3,855 | +18.4% | 15,400 |
| Vidaza | myelodysplastic syndrome/ acute myeloid leukemia | 2,871 | 1,462 | - 49.1% | 4,800 |
| Gazyva | CD20-positive follicular lymphoma/ CD20-positive chronic lymphocytic leukemia | 1,220 | 1,254 | +2.8% | 5,100 |
| Tramal/Onetram | cancer pain, chronic pain | 1,104 | 778 | - 29.5% | 2,700 |
| Defitelio | sinusoidal obstruction syndrome | 631 | 709 | +12.4% | 2,300 |
| Cialis | erectile dysfunction | 654 | 647 | - 1.1% | 2,700 |
| Zalutia | urinary disorder caused by benign prostatic hyperplasia | 614 | 493 | - 19.6% | 1,600 |
| Adcirca | pulmonary arterial hypertension | 631 | 480 | - 23.9% | 1,700 |
| Erizas | allergic rhinitis | 334 | 281 | - 15.7% | 2,100 |
| Profit in co-promotion | | 2,310 | 2,461 | +6.5% | 9,100 |
| Revenues from the licensing of industrial property rights | | 9,104 | 10,779 | +18.4% | 42,100 |
| Revenue | | 30,870 | 33,738 | +9.3% | 132,500 |

# Sales by Product Group in Functional Food Segment

(Million yen)

| (Million yen) | 1Q FY2023 | | 1Q FY2024 | | YoY Change | | FY2024 |
|---|---|---|---|---|---|---|---|
| | Results | Ratio | Results | Ratio | Amt | % | Forecasts |
| Protein preparations | 4,284 | 69.7% | 3,463 | 64.2% | -821 | - 19.2% | 13,000 |
| Preservatives | 736 | 12.0% | 800 | 14.8% | +63 | +8.6% | 3,200 |
| Supplements | 464 | 7.6% | 552 | 10.3% | +87 | +18.8% | 3,100 |
| Health food ingredients | 306 | 5.0% | 260 | 4.8% | -45 | - 14.8% | 1,100 |
| Others | 349 | 5.7% | 316 | 5.9% | -32 | - 9.2% | 1,100 |
| Revenue | 6,142 | 100.0% | 5,393 | 100.0% | -749 | - 12.2% | 21,500 |

# Consolidated Balance Sheet

| (Million yen) | End of FY2023 | End of 1Q FY2024 | Change Amt | | End of FY2023 | End of 1Q FY2024 | Change Amt |
|---|---|---|---|---|---|---|---|
| Assets | 263,404 | 265,828 | +2,423 | Liabilities | 42,870 | 38,365 | -4,504 |
| Current assets | 164,285 | 159,973 | -4,311 | Current liabilities | 37,336 | 32,826 | -4,510 |
| Non-current assets | 99,119 | 105,854 | +6,734 | Non-current liabilities | 5,533 | 5,539 | +5 |
| | | | | Equity | 220,534 | 227,462 | +6,928 |
| Total aseets | 263,404 | 265,828 | +2,423 | Total liabilities and equity | 263,404 | 265,828 | +2,423 |

## Assets

| | |
|---|---|
| Cash and cash equivalents | - 3,621 |
| Intangible assets | + 4,316 |
| Other financial assets (non-current) | + 2,016 |

## Liabilities and Shareholders' Equity

| | |
|---|---|
| Trade and other payables | - 3,057 |
| Other current liabilities | + 1,684 |
| Retained earnings | + 6,088 |

# Pipeline（1/2）

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

| Stage | Code No. (Generic name) | Origin | Application type | Indications | Schedule | Country |
|---|---|---|---|---|---|---|
| Launch P3 | NS-065/NCNP-01 | In-house | NME | Duchenne muscular dystrophy | — | Japan/U.S. |
| Preparing for launch | LY3527727 (pirtobrutinib) | Alliance agreement | NME | patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors | Approval : June in 2024 | Japan |
| NDA filing | NS-304 (selexipag) | In-house | New dose New indication | pediatric pulmonary arterial hypertension | Study Completion : FY 2025 Application : April in 2024 | Japan |
| P3 | ZX008 (fenfluramine hydrochloride) | Distribution partnership | New indication | CDKL5 deficiency disorder | Study Completion : FY2026 | Japan |
| | GA101 (obinutuzumab) | In-license | New indication | lupus nephritis | Projected submission : 2026 | Japan |
| | | | | pediatric nephrotic syndrome | Projected submission : 2026 | Japan |
| | | | | extra renal lupus | Projected submission : 2027 and beyond | Japan |
| | CAP-1002 (deramiocel) | Partnership | NME | Duchenne muscular dystrophy | Topline data : end of 2024 | U.S. |
| | LY3527727 (pirtobrutinib) | Alliance agreement | New indication | mantle cell lymphoma | — | Japan |
| | | | | chronic lymphocytic leukemia | — | Japan |

# Pipeline（2/2）

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

| Stage | Code No. (Generic name) | Origin | Application type | Indications | Schedule | Country |
|---|---|---|---|---|---|---|
| P2 | NS-304 (selexipag) | In-house | New indication | arteriosclerosis obliterans | Study Completion : FY2024 | Japan |
| | NS-580 | In-house | NME | endometriosis | Temporarily suspended | Japan |
| | | | | chronic prostatitis/ chronic pelvic pain syndrome | Temporarily suspended | Japan |
| | NS-089/NCNP-02 (brogidirsen) | In-house | NME | Duchenne muscular dystrophy | Study Completion : FY2025<br>FPI : February in 2024 | Japan/U.S. |
| | NS-229 | In-house | NME | eosinophilic granulomatosis with polyangiitis | Study Completion : FY2026 | Japan/U.S. |
| P1/2 | NS-401 (tagraxofusp) | In-license | NME | blastic plasmacytoid dendritic cell neoplasm | Study Completion : FY2026 | Japan |
| Preparing for P1/2 | NS-050/NCNP-03 | In-house | NME | Duchenne muscular dystrophy | Study Completion : FY2027<br>FPI : 1H of FY2024（estimated） | Japan/U.S. |
| P1 | NS-917 (radgocitabine) | In-license | NME | relapsed/refractory acute myeloid leukemia | Study Completion : FY2024 | Japan |
| | NS-025 | In-house | NME | urological diseases | Study Completion : FY2024 | Japan |
| | NS-863 | In-house | NME | cardiovascular diseases | Study Completion : FY2024 | Japan |

# NS-065/NCNP-01 (viltolarsen)

## - Treatment for Duchenne muscular dystrophy -

| | |
|---|---|
| Development Phase | ・Japan : Launch<br>・U.S. : Launch<br>・Global : P3 open-label extension study in progress |
| Origin | Co-development :<br>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<br>・Morpholino based oligonucleotide with possible high safety profile and maximized activity |

# ZX008 (fenfluramine hydrochloride)

## - Treatment for rare intractable epilepsy -

| | |
|---|---|
| Development Phase | Japan : Launch (Dravet syndrome)<br>Japan : Launch（Lennox-Gastaut syndrome)<br>Japan : P3 (CDKL5 deficiency disorder) |
| Origin | [Mar. 2019]　Distribution partnership in Japan : UCB S.A. (former Zogenix, Inc.) |
| Development | UCB S.A.　(former Zogenix, Inc.) |
| Mechanism of action | 5-HT (serotonin) releaser with agonist activity at several 5-HT receptors |
| Indication | Dravet syndrome<br>Lennox-Gastaut syndrome<br>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<br>・ZX008 can be used in combination with other drugs, as standard of care for intractable epilepsy based on combination therapy. |

# LY3527727(pirtobrutinib)

## - Treatment for Mantle cell lymphoma, Chronic lymphocytic leukemia -

| | |
|---|---|
| Development Phase | ・Preparing for launch<br>(for patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors)<br>・P3 (MCL and CLL) |
| Origin | [Mar. 2024] Alliance agreement in Japan : Eli Lilly Japan |
| Development | Eli Lilly Japan |
| Mechanism of action | A reversible non-covalent BTK inhibitor |
| Indication | mantle cell lymphoma (MCL)<br>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. |

# CAP-1002 (deramiocel)

## - Treatment for Duchenne muscular dystrophy -

| | |
|---|---|
| Development Phase | U.S. : P3 |
| Origin | [Jan. 2022] Partnership for commercialization in U.S.<br>[Feb. 2023] Partnership for commercialization in Japan<br>: 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.<br>・Its broad applicability makes it suitable for patients regardless of the type of genetic mutation. |

# GA101 (obinutuzumab)

## - Treatment for lupus nephritis, pediatric nephrotic syndrome, extra renal lupus -

| | |
|---|---|
| Development Phase | Japan : P3（LN）<br>Global : P3（PNS）<br>Japan : P3（ERL） |
| Origin | [Nov. 2012]<br>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)<br>pediatric nephrotic syndrome (PNS)<br>extra renal lupus (ERL) |
| 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 : P2b (ASO)<br>Japan : P2, NDA filing (pediatric PAH) |
| Origin | Nippon Shinyaku |
| Development | Nippon Shinyaku |
| Mechanism of action | Selective IP receptor agonist |
| Indication | arteriosclerosis obliterans (ASO)<br>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 : P2b (endometriosis)<br>Japan : P2a (CP/CPPS) |
| Origin | Nippon Shinyaku |
| Development | Nippon Shinyaku |
| Mechanism of action | Inhibition of membrane-associated prostaglandin E synthase-1 |
| Indication | endometriosis<br>chronic prostatitis/chronic pelvic pain syndrome (CP/CPPS) |
| Dosage form | Oral agent |
| Feature | ・Treatment for endometriosis without hormonal effect and with possible analgesic potency<br>・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 :<br>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<br>・Morpholino based oligonucleotide with possible high safety profile and maximized activity |

# NS-229

## - Treatment for Eosinophilic granulomatosis with polyangiitis -

| | |
|---|---|
| Development Phase | Global：P2 |
| 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<br>・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 : P1/2 |
| Origin | [Mar. 2021]<br>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<br>・Novel targeted therapy directed to CD123 on tumor cells<br>・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 P1/2 |
| Origin | Co-development :<br>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<br>・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 : P1 |
| Origin | [Mar. 2017]<br>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<br>・Tolerable safety profile available to elderly patients with r/r AML |

# NS-025

## - Treatment for urological diseases -

| | |
|---|---|
| Development Phase | Japan : P1 |
| Origin | Nippon Shinyaku |
| Development | Nippon Shinyaku |
| Mechanism of action | – |
| Indication | Urological diseases (to be determined) |
| Dosage form | Oral agent |
| Feature | – |

# NS-863

## - Treatment for cardiovascular diseases -

| | |
|---|---|
| Development Phase | Japan : P1 |
| Origin | Nippon Shinyaku |
| Development | Nippon Shinyaku |
| Mechanism of action | – |
| Indication | Cardiovascular 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 Japanese original and the English translation, the former shall prevail.



NIPPON SHINYAKU CO., LTD.

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# Nippon Shinyaku Co., Ltd.

Financial Results Briefing for the 1st Quarter Ended June 30, 2024

August 7, 2024

# Presentation

**Edamitsu:** I am Takanori Edamitsu, Nippon Shinyaku Co., Ltd. Director, General Manager, Business Management & 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 FY2024 and the progress of our R&D activities, in accordance with the presentation materials posted on our website.

## 1Q FY2024 Summary

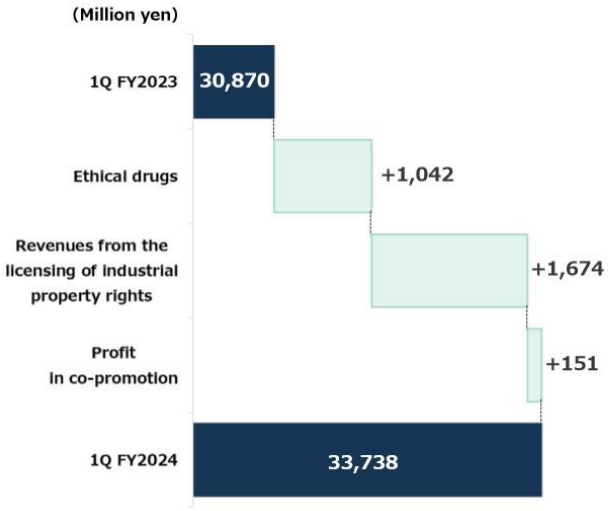
| (Million yen) | 1Q FY2023 | | 1Q FY2024 | | YoY Change | |
|---|---|---|---|---|---|---|
| | Results | Ratio | Results | Ratio | Amt | % |
| **Revenue** | **37,012** | **100.0%** | **39,131** | **100.0%** | **+2,118** | **+5.7%** |
| (Pharmaceuticals) | (30,870) | (83.4%) | (33,738) | (86.2%) | (+2,868) | (+9.3%) |
| (Functional Food) | (6,142) | (16.6%) | (5,393) | (13.8%) | (-749) | (-12.2%) |
| **Cost of sales** | **12,962** | **35.0%** | **12,636** | **32.3%** | **-326** | **-2.5%** |
| **SG&A expenses** | **8,418** | **22.7%** | **9,221** | **23.6%** | **+803** | **+9.5%** |
| **R&D expenses** | **5,911** | **16.0%** | **7,497** | **19.2%** | **+1,586** | **+26.8%** |
| **Other income** | **1,572** | **4.2%** | **1,507** | **3.9%** | **-64** | **-4.1%** |
| (Foreign exchange gain) | (1,422) | (3.8%) | (1,211) | (3.1%) | (-210) | (-14.8%) |
| **Other expenses** | **129** | **0.3%** | **204** | **0.5%** | **+74** | **+58.0%** |
| **Operating profit** | **11,163** | **30.2%** | **11,078** | **28.3%** | **-85** | **-0.8%** |
| **Finance income** | **298** | **0.8%** | **363** | **0.9%** | **+65** | **+21.8%** |
| **Finance costs** | **21** | **0.1%** | **31** | **0.1%** | **+9** | **+42.0%** |
| **Profit before tax** | **11,440** | **30.9%** | **11,411** | **29.2%** | **-29** | **-0.3%** |
| **Income tax expense, etc** | **2,690** | **7.3%** | **1,146** | **2.9%** | **-1,544** | **-57.4%** |
| **Profit attributable to owners of parent** | **8,749** | **23.6%** | **10,264** | **26.2%** | **+1,514** | **+17.3%** |



2

Please see page two of the slide. As an overview of the results for Q1 FY2024, consolidated revenue was JPY39,131 million, operating profit was JPY11,078 million, profit before taxes was JPY11,411 million, and profit attributable to owners of the parent was JPY10,264 million.

## Segmental Review - Pharmaceuticals -




**Ethical drugs 20,496 million yen**
**(+1,042 million yen, +5.3%, YoY)**

- ✓ Sales growth of Uptravi and Viltepso, etc.
- ✓ Decrease in sales of Vidaza, Tramal/Onetram, etc.

**Revenues from the industrial property rights**
**10,779 million yen**
**(+1,674 million yen, +18.4%, YoY)**

- ✓ Royalty revenue growth due to overseas sales of Uptravi

**Profit in co-promotion 2,461illion yen**
**( + 151 million yen, + 6.5%, YoY)**

- ✓ Sales growth of Opsumit and Erleada

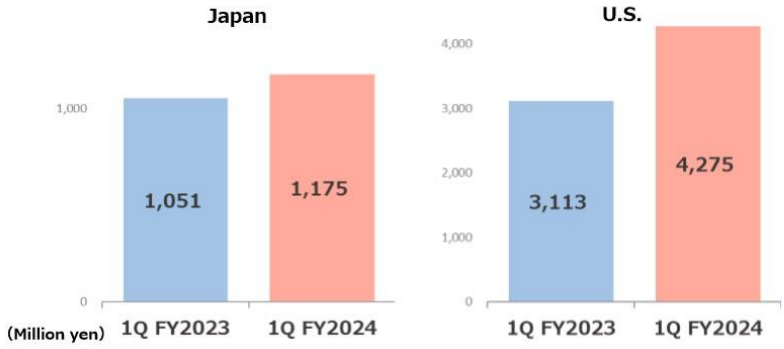



Please see page three of the slide. In the pharmaceuticals business, consolidated net sales increased 9.3% YoY to JPY33,738 million despite the effect of NHI price revision and generic products, due to growth in sales of Viltepso, a treatment for Duchenne muscular dystrophy, and Uptravi, a treatment for pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension, and royalty income from overseas sales of those products.

## Sales Trends of Viltepso® (viltolarsen)

| (Million yen) | | 1Q FY2023 Results | 1Q FY2024 Results | YoY Change Amt | YoY Change % | |
|---|---|---|---|---|---|---|
| Viltepso | Japan | 1,051 | 1,175 | +124 | +11.8% | ✓The number of patients currently receiving the drug is more than two-thirds of the peak number of 128 patients in the data by the Central Social Insurance Medical Council (Chuikyo) . |
| | U.S. | 3,113 | 4,275 | +1,162 | +37.3% | ✓The number of patients receiving or wishing to receive the drug is increasing.<br>✓Phase III Trial (RACER53 Study) is under additional analysis. |
| | total | 4,165 | 5,450 | +1,285 | +30.9% | |

| Exchange rate | 1Q FY2023 Actual rate | 1Q FY2024 Actual rate |
|---|---|---|
| 1USD | 137.5yen | 155.9yen |

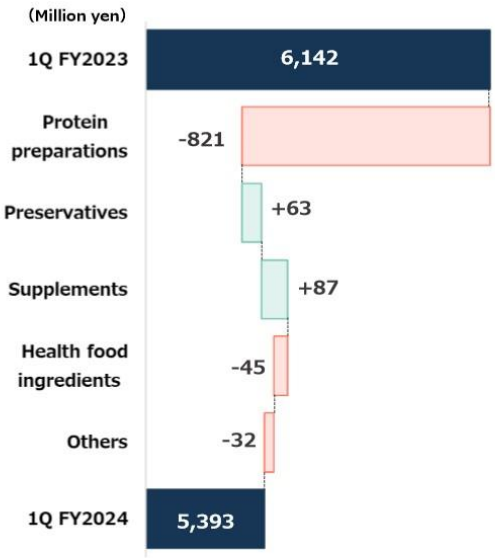






Please see page four of the slide. Here we show sales of Viltepso, a treatment for Duchenne muscular dystrophy, which is marketed in Japan and the United States. Sales in both Japan and the US increased YoY, totaling JPY1,175 million in Japan and JPY4,275 million in the US.

## Segmental Review - Functional Food -




**Protein preparations 3,463 million yen**
**(-821 million yen, -19.2%, YoY)**

✓ Decline in unit sales prices

**Preservatives 800 million yen**
**(+63 million yen, +8.6%, YoY)**

✓ Recovery in food service-related business due to active tourism and excursions

**Supplements 552 million yen**
**(+87 million yen, +18.8%, YoY)**

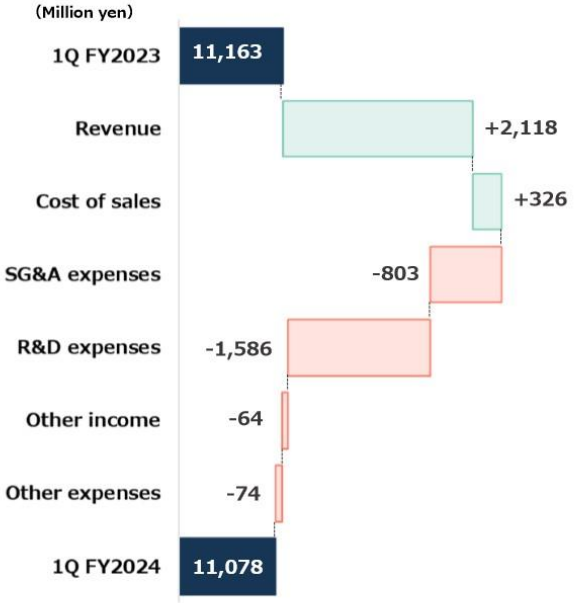
✓ Growth in both the sports and anti-aging care fields

**Health food ingredients 260 million yen**
**(-45 million yen, +14.8%, YoY)**




Please see page five of the slide. In the functional foods business, sales of supplements and other products increased, but sales of protein preparations and other products decreased, resulting in consolidated net sales of JPY5,393 million in the functional foods business, down 12.2% from the same period last year.

## Operating Profit




**Revenue 39,131 million yen (+2,118 million yen, +5.7%, YoY)**

- ✓ Increase in revenue from industrial property rights and the U.S. sales of Viltepso.

**Cost of sales 12,636 million yen (-326 million yen, -2.5%, YoY)**
**The ratio was improved by 2.7 points YoY.**

- ✓ Negative impact of NHI price revision
- ✓ Cost of sales ratio improvement due to factors such as revenues from industrial property rights and the change in sales segment mix (pharma vs. food)

**SG&A expenses 9,221 million yen (+803 million yen, +9.5%, YoY)**

- ✓ Increase in promotional expenses in NS Pharma
- ✓ Increase in commission for promotional activities of Uptravi

**R&D expenses 7,497 million yen (+1,586 million yen, +26.8%, YoY)**

- ✓ Increase in cost of investigational products




Please see page six of the slide. The cost-to-sales ratio improved by 2.7 percentage points YoY to 32.3%, due to factors such as the sales mix, in addition to an increase in industrial property rights and other revenues. Selling, general, and administrative expenses increased to JPY9,221 million, up 9.5% from the same period of the previous year, due to an increase in sales promotion expenses of the US subsidiary NS Pharma and outsourcing fees for sales promotion activities for Uptravi.

R&D expenses totaled JPY7,497 million, up 26.8% YoY, due to an increase in manufacturing costs including investigational drugs, etc. As a result, operating profit was JPY11,078 million, down 0.8% YoY.

## Business Forecast for FY2024 (consolidated)

| (Million yen) | FY2023 1Q Results | FY2023 FY Results | FY2024 1Q Results | FY2024 Progress for 1H | FY2024 1H Forecasts | FY2024 FY Forecasts |
|---|---|---|---|---|---|---|
| **Revenue** | **37,012** | **148,255** | **39,131** | **51.6%** | **75,800** | **154,000** |
| (Pharmaceuticals) | (30,870) | (125,105) | (33,738) | (51.7%) | (65,300) | (132,500) |
| (Functional Food) | (6,142) | (23,150) | (5,393) | (51.4%) | (10,500) | (21,500) |
| **Cost of sales** | **12,962** | **50,234** | **12,636** | | **25,400** | **51,000** |
| **SG&A expenses** | **8,418** | **34,959** | **9,221** | | **18,600** | **38,700** |
| **R&D expenses** | **5,911** | **31,676** | **7,497** | | **16,800** | **32,400** |
| **Other income** | **1,572** | **3,163** | **1,507** | | **350** | **500** |
| **Other expenses** | **129** | **1,252** | **204** | | **350** | **400** |
| **Operating profit** | **11,163** | **33,295** | **11,078** | **73.9%** | **15,000** | **32,000** |
| **Finance income** | **298** | **650** | **363** | | **250** | **600** |
| **Finance costs** | **21** | **329** | **31** | | **50** | **100** |
| **Profit before tax** | **11,440** | **33,616** | **11,411** | **75.1%** | **15,200** | **32,500** |
| **Income tax expense, etc** | **2,690** | **7,764** | **1,146** | | **1,700** | **3,500** |
| **Profit attributable to owners of parent** | **8,749** | **25,851** | **10,264** | **76.0%** | **13,500** | **29,000** |

| Exchange rate | 1Q FY2023 Actual rate | 1Q FY2024 Actual rate | FY2024 Forecast rate |
|---|---|---|---|
| 1USD | 137.5yen | 155.9yen | 140.0yen |

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Please see page seven of the slide. The Company has revised its consolidated earnings forecast for the fiscal year ending March 31, 2025, from that announced on May 10, as royalty income from overseas sales of Uptravi and sales of new products such as Viltepso exceeded the Company's forecast. Consolidated revenue is expected to be JPY154,000 million, consolidated operating profit JPY32,000 million, and profit before taxes JPY32,500 million for the full year.

In addition, the Company expects net income attributable to owners of the parent of JPY29 billion due to the expected decrease in income tax expense as a result of the consideration of the recoverability of deferred tax assets at consolidated subsidiaries in the US and the reflection in the estimated effective tax rate.

## Segmental Forecast - Pharmaceuticals -

| (Million yen) | FY2023 | | FY2024 | | YoY Change | |
|---|---|---|---|---|---|---|
| | 1Q Results | FY Results | 1Q Results | FY Forecasts | Amt | % |
| Ethical drugs | 19,456 | 76,143 | 20,496 | 81,300 | +5,157 | +6.8% |
| Revenue from the licensing of industrial property rights | 9,104 | 40,304 | 10,779 | 42,100 | +1,796 | +4.5% |
| Profit in co-promotion | 2,310 | 8,658 | 2,461 | 9,100 | +442 | +5.1% |
| Revenue | 30,870 | 125,105 | 33,738 | 132,500 | +7,395 | +5.9% |




Please see page eight of the slide. In the pharmaceutical business, we forecast revenue of JPY132.5 billion, an increase of 5.9% YoY. Despite the effect of NHI price revision and generic products, the Company expects an increase in revenues due to growth in sales of Viltepso, Uptravi, and other new product lines, as well as growth in royalty income associated with overseas sales of Uptravi.

## Segmental Forecast - Functional Food -

| (Million yen) | FY2023 | | FY2024 | | YoY Change | |
|---|---|---|---|---|---|---|
| | 1Q Results | FY Results | 1Q Results | FY Forecasts | Amt | % |
| Protein preparations | 4,284 | 15,600 | 3,463 | 13,000 | -2,600 | -16.7% |
| Preservatives | 736 | 3,105 | 800 | 3,200 | +95 | +3.1% |
| Supplements | 464 | 1,905 | 552 | 3,100 | +1,195 | +62.7% |
| Health food ingredients | 306 | 1,248 | 260 | 1,100 | -148 | -11.9% |
| Others | 349 | 1,290 | 316 | 1,100 | -190 | -14.7% |
| Revenue | 6,142 | 23,150 | 5,393 | 21,500 | -1,650 | -7.1% |




In the functional foods business, sales revenue is expected to be JPY21.5 billion, down 7.1% from the previous year due to a decrease in sales of protein preparations.

Next, I will explain the progress of R&D items. This section describes the items that have been updated since the release of the FY2023 financial results.

## R&D Updates (1/2)

For updates since May 14, 2024, see highlighted text in red.

| Recent status/event | Code No. (Generic name) | Product name | Indications and topics | Schedule |
|---|---|---|---|---|
| Launch | ZX008 (fenfluramine) | Fintepla | Lennox-Gastaut syndrome (additional indication) | March 2024 |
| Launch | NS-87 (daunorubicin / cytarabine) | Vyxeos | high-risk acute myeloid leukemia | May 2024 |
| Approval | LY3527727 (piltobrutinib) | Jaypirca | patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors | June 2024 |
| In application | NS-304 (selexipag) | Uptravi | pediatric pulmonary arterial hypertension | April 2024 |
| Start of P3 | GA101 (obinutuzumab) | Gazyva | systemic lupus erythematosus without nephropathy | October 2023 |
| Start of P2 | NS-089/NCNP-02 (brogidirsen) | – | Duchenne muscular dystrophy | February 2024 |
| | NS-229 | – | eosinophilic granulomatosis with polyangiitis | June 2024 |
| In-license (Vicore Pharma, Sweden) | C21 | – | idiopathic pulmonary fibrosis | Contract signed in February 2024 |
| Alliance Agreement (Eli Lilly Japan) | LY3527727 (piltobrutinib) | Jaypirca | mantle cell lymphoma (MCL)<br>chronic lymphocytic leukemia (CLL) | Contract signed in March 2024 |
| Temporarily suspended | NS-580 | – | endometriosis<br>chronic prostatitis / chronic pelvic pain syndrome | – |

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11

Please see page 11 of the slide. NS-87, Vyxeos, a treatment for high-risk acute myeloid leukemia, was launched in May 2024.

The reversible, non-covalent BTK inhibitor LY3527727 (piltobrutinib), for which we entered into an alliance agreement with Eli Lilly Japan, obtained marketing approval in June 2024 for the treatment of "patients with relapsed or refractory mantle cell lymphoma who are resistant or intolerant to other BTK inhibitors."

A global Phase II study of NS-229 for the treatment of eosinophilic granulomatosis with polyangiitis was initiated in June 2024.

## R&D Updates (2/2)

For updates since May 14, 2024, see highlighted text in red.

| Recent status/event | Code No. (Generic name) | Product name | Indications and topics | Schedule |
|---|---|---|---|---|
| Preliminary analysis results | NS-065/NCNP-01 (viltolarsen) | Viltepso | global Phase 3 trial (RACER53 Study) | May 2024 |
| Conference Presentations | NS-065/NCNP-01 (viltolarsen) | Viltepso | Phase 2 trial (Galactic53 trial): 2024 Muscular Dystrophy Association Clinical & Scientific Conference | March 2024 |
| Manuscript | NS-089/NCNP-02 (brogidirsen) | – | non-clinical data: (Molecular Therapy Nucleic Acids) | October 2023 |
| Orphan Drug Designation | NS-089/NCNP-02 (brogidirsen) | – | Duchenne muscular dystrophy | December 2023 (EU) |
| | NS-401 (tagraxofusp) | – | blastic plasmacytoid dendritic cell neoplasm | August 2023 (Japan) |
| | NS-229 | – | eosinophilic granulomatosis with polyangiitis | January 2024 (EU) |
| Alliance (MiNA Therapeutics) | – | – | a joint research agreement with the aim of creating nucleic acid medicines that are expected to be applied to an intractable and rare disease in the CNS field | April 2024 |





In May 2024, we announced the preliminary results of the analysis of the global Phase III study of NS-065/NCNP-01, Viltepso for the treatment of Duchenne muscular dystrophy.

Additional analysis is currently underway to advance discussions with the regulatory authorities.

This concludes my explanation of the results for Q1 FY2024 and the progress of R&D.

Q1 FY2024 Financial Results Presentation Q&A (Summary)

August 7, 2024

| NO | Questions | Answer. |
|---|---|---|
| 1 | You have increased the sales revenue forecast by 4 billion yen for the full year, although Viltepso sales and royalty income from Uptravi increased only about 1 billion yen in total. I would like to ask about the remaining 3 billion yen increase. What are the sales of products not disclosed at this time other than Fintepla and Vyxeos sales? | The remaining increase will be the sum of Fintepla and Vyxeos sales, Viltepso's increased sales to other regions than Japan and U.S., and other products that are expected to increase in sales gradually. It is certain that Vyxeos sales have significantly exceeded the original plan. |
| 2 | Other than Viltepso sales and Uptravi royalties, are the large majority of the approximately 3 billion yen in either Vyxeos sales in Japan, or Viltepso sales to the rest of the world? | Vyxeos is the major contributor to such sales. |
| 3 | Will Vyxeos sales not be disclosed? | It will be disclosed when it meets our disclosure criteria. |
| 4 | Will revenue from sales of an individual product disclosed when the annual sales forecast for that product reaches 2 billion yen? | Once a product is expected to reach that level, we will disclose its specific revenue. |
| 5 | It seems that costs will be increased in the second half of the year. Do you anticipate that costs will be shifted from the original plan to the second half? | The delayed recognition of some SG&A and R&D expenses in the second half of the year has been taken into account in the revised FY2024 financial forecast. |
| 6 | R&D expenses for the first half of the year were shifted to the second half of the year. Is this largely due to the shift in the timing of the accrual of expenses for nucleic acid API? | In addition to delays in the timing of manufacturing costs recognition, the impact of outsourced research costs is also included in those R&D expenses. |
| 7 | Previously, I heard that CAP-1002-related costs would be included in the U.S. SG&A expenses. Has this cost been incurred at all yet? Or will it be incurred intensively in the second half or 4Q or gradually in the future? | We assume that expenses will be incurred in 4Q. |
| 8 | I think the company's exchange rate assumption was originally 140 yen to the US dollar. What is the rate in the revised FY2024 forecast? | We continue to assume 140 yen to the U.S. dollar. |

| | | |
|---|---|---|
| 9 | Viltepso's U.S. sales have been revised slightly upward this time. Is the sales performance in line with the plan, excluding the impact of foreign exchange rates? | Even on a U.S. dollar basis, Viltepso's U.S. sales have exceeded our internal projections. |
| 10 | I understand that the foreign exchange assumptions have not changed in the revised FY2024 financial forecast. Will you not change your forecast for the second half of the fiscal year? | The full-year forecast for FY2024 is expected to be generally in line with the initial plan. |
| 11 | To what extent did the upward effect of foreign exchange rates affect sales and operating income in the 1Q of this fiscal year? The company has revised its earnings forecast based on the assumption of 140 yen to the dollar. If the actual level of foreign exchange continues from the 2Q onward, will the effect of foreign exchange decrease compared to the 1Q? | The actual foreign exchange in 1Q was about 155 yen to the U.S. dollar, about 15 yen higher than the company's assumption of 140 yen to the U.S. dollar. We believe that the foreign exchange impact can be calculated considering the above difference of 15 yen in the 1Q results of Viltepso sales results in the U.S. and revenues from industrial property rights.<br>The forecast exchange rate remains unchanged at 140 yen from the 2Q onward, so if the actual exchange rate remains close to 140 yen, the results will be close to the plan. On the other hand, if the exchange rate were to fall below 140 yen, our performance would be affected. However, since Viltepso is slightly exceeding the plan even on a volume basis, we do not think this will be a major problem. |
| 12 | The extent to which foreign exchange has had an impact in the upward revision of sales will be clear if we do the calculations you mentioned. How much is the impact of foreign exchange on operating income? I think U.S. costs also have an impact. | We will not disclose specific numbers. |
| 13 | I estimate that the company's effective tax rate is about 10.8% for FY2024. What is your forecast for FY2025 and beyond? | We expect the negative tax expense to be temporary and the tax rate to return to normal next fiscal year. |

| | | |
|---|---|---|
| 14 | Could you please elaborate on the status of your communication with the authorities regarding Viltepso? At the May 28 presentation on the 7th Mid-Term Management Plan, I heard that the P3 (RACER 53 Study) results may have been affected by steroids. What is the current situation? | The situation has not changed significantly since the last presentation. In-house data analysis is largely complete, and we will now move on to discussions with the FDA, which are expected to take place in this fall. We expect the results of the discussions around the end of this year. |
| 15 | Do you assume that the P3 (RACER 53 Study) will be redone? | Yes. |
| 16 | Regarding the P3 (RACER 53 Study) of Viltepso, at the May 27th presentation on the 7th Mid-Term Management Plan, you used a variety of expressions to describe the probability that the results of this trial would result in full approvals, the risk of withdrawal of conditional approvals, and so on. Now that the in-house analysis is almost complete, have there been any new findings that might change those expressions? | At the last presentation, we were not talking about projections based on specific data. Since then, additional analysis has provided data that confirms efficacy in certain populations, and we would like to continue in the direction of maintaining the approvals. |
| 17 | Previously you told that the results of the discussions with the authorities will be disclosed by the end of this year. Do you have a policy or expectation for the disclosure of specific data? | We are considering making a public announcement at about the conclusion of our discussions with the authorities. |
| 18 | How do you perceive the reaction in the medical community to the results of Viltepso's P3 (RACER 53 Study) so far? There were only qualitative comments on top line data, no specific data. How would this affect physician and patient perceptions? How about the willingness of treated patients to continue treatment or the willingness of patients not yet treated? | At this point in the U.S., Viltepso continues to be used. There has not been a single case of a patient discontinuing treatment with Viltepso and switching to a competing product in response to the results of Viltepso's P3 (RACER 53 Study). In Japan, Viltepso is the only available treatment option for DMD. We have explained top-line data to physicians, and Viltepso continues to be used in Japan too. The pace of growth in the number of new patients is slight because the drug has been penetrated well before the P3 (RACER 53 Study) results were available. The exact impact in the U.S. is difficult to determine, but given that Viltepso and Vyondys 53 have been on the market for some time and the number of new patients has been gradually decreasing, we do not believe the impact of P3 (RACER 53 Study) data is significant. |

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| 19 | I would like to know the number of new Viltepso patients in the US in 1Q. Is the number growing QonQ? Has the number been affected since Sarepta's gene therapy received the full FDA approval in June this year? | As originally planned, the number of new patients has been increasing each month by several. |
| 20 | Are there about 90 patients receiving Viltepso in Japan? | It's approximately 90 plus. |
| 21 | Please tell us about the situation surrounding Viltepso in the U.S. market, especially since this past July. | Since July, no patients receiving Viltepso have switched to gene therapy. Sales of Elevidys (Sarepta's gene therapy) are strong, but it appears that patients who cannot use exon-skipping drugs are using Elevidys first. Prior to the full FDA approval, Elevidys was limited to patients aged 4 through 5 years old, so Viltepso patients did not immediately switched to gene therapy. Now that Elevidys has been approved and its age restriction has been removed, we need to watch carefully its impact in the future. We will continue to collect information in the US market. |
| 22 | At the IR Meeting (Q2/FY2023) on November 15, 2023, President Nakai mentioned "There are some insurance policies in the U.S. that can be combined with Viltepso and Elevidys, but we have not yet heard of any actual cases where they have been reimbursed that way". Are there any patients who are using Viltepso after Elevidys? | We have heard that both drugs may be reimbursed in combination, but we have yet to hear of a case where Viltepso was actually administered after a dose of Elevidys. |
| 23 | For now, it is less than a couple of months from the full FDA approval of Elevidys. Do Viltepso and Elevidys coexist in different US markets? | From the sales of Elevidys, it can be inferred that the number of patients is increasing rapidly. However, to date, no patients receiving Viltepso have received Elevidys. |
| 24 | I believe NS-050 has only one clinical trial site and has not yet been administered to patients. Is it likely to be started in the first half of the year? | We have found some candidate patients and expect to begin the clinical trial in the first half of this year. |

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| 25 | Clinical trial information for NS-051 has not yet been disclosed. Do you need to coordinate anything with the FDA regarding animal testing for NS-051? | We are in discussions with the FDA to coordinate the interpretation of non-clinical data to initiate clinical trials. |
| 26 | Has the rolling BLA for CAP-1002 already started? I understand that the meeting with FDA will take place in July-September. | We do not disclose such information as an update. Please check an announcement from Capricor Therapeutics. |
| 27 | At the May 28 presentation on the 7th Mid-Term Management Plan, President Nakai mentioned the possibility of hiring a CMO (Chief Medical Officer) with a medical license. What is the current situation? | Currently, our recruitment efforts are underway in the U.S., but have not yet been completed. |
| 28 | When do you expect to be able to hire a CMO in the U.S.? | We are doing our best but cannot say how long it will take to find a qualified medical doctor to suit the position. |