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# FY2025 Results Briefing Session

May 13, 2026

**JCR Pharmaceuticals Co., Ltd.**

[Securities code]4552, Prime. TSE
[Contacts] ir-info@jp.jcrpharm.com

# Disclaimer Regarding Forward-Looking Statement

- This material contains forecasts, projections, goals, plans, and other forward-looking statements regarding the Company's financial results and other data. Such forward-looking statements are based on the Company's assumptions, estimates, outlook, and other judgments made in light of information available at the time of disclosure of such statements and involve both known and unknown risks and uncertainties. Accordingly, forecasts, plans, goals, and other statements may not be realized as described, and actual financial results, success/failure or progress of development, and other projections may differ materially from those presented herein.

- Information concerning pharmaceuticals and medical devices (including those under development) contained herein is not intended as advertising or as medical advice.

- The figures in this document are rounded down to the nearest million yen, and percentages are rounded to the nearest whole number. As a result, there may be discrepancies in the total figures.

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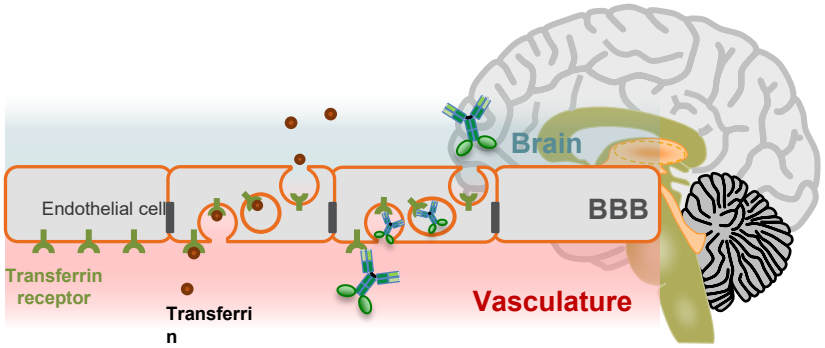
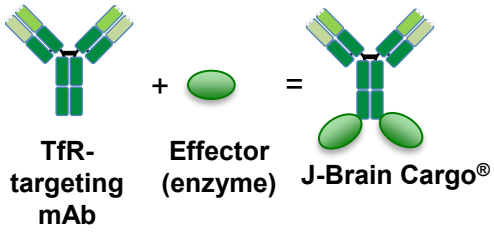
# Achievements to Date and Future Outlook

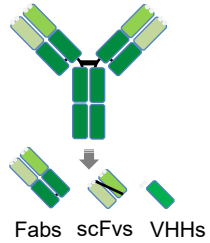
**Hiroyuki Sonoda, Ph. D.**
Representative Director, President
Chief Scientific Officer

**2021**
A pharmaceutical product (IZCARGO™) applying J-Brain Cargo® has received marketing approval in Japan

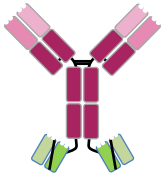
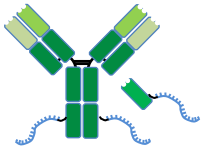
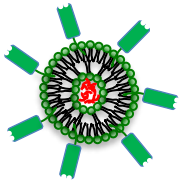



Platform expansion into new indications




| Neuro Degeneration | Neuro inflammation |
|---|---|
| Neuro Oncology | Neuro-muscular Diseases |

| Proteins (Enzymes,Antibodies) | Gene therapy |
|---|---|
|  |  JUST-AAV |
| Oligonucleotides (ASO, siRNA) | Lipid nanoparticles (mRNA, Compounds) |
|  |  |

TfR: Transferrin receptor BBB: Blood Brain Barrier

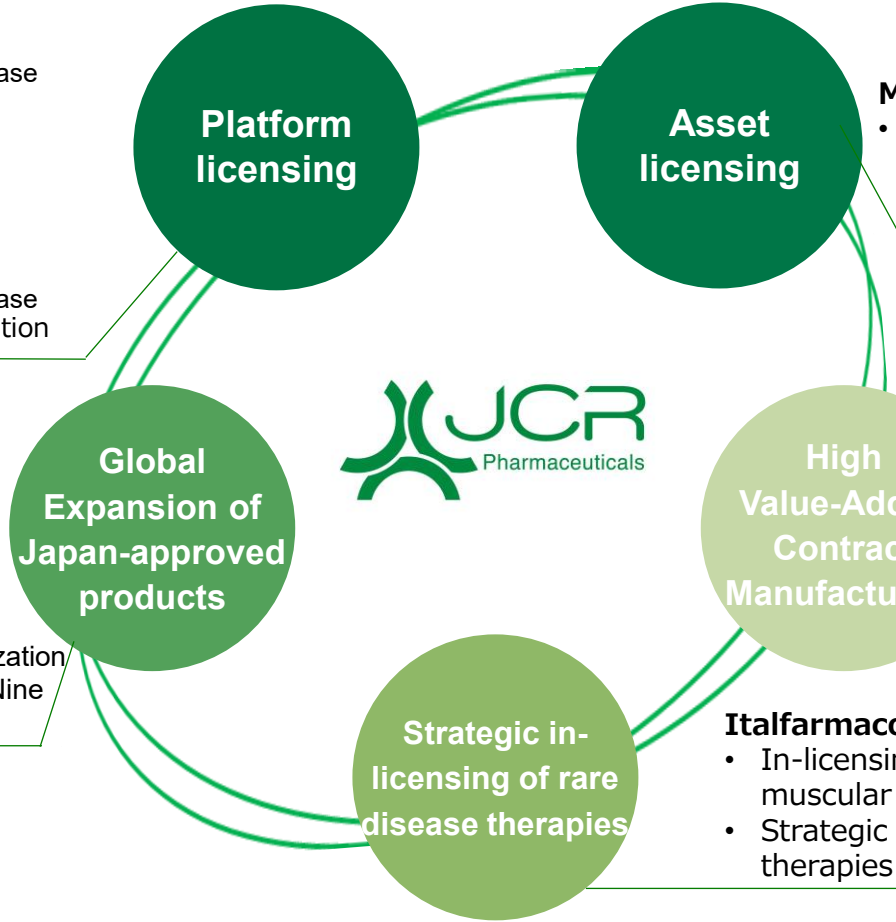
# Overview of Clinical or Late Preclinical Pipeline



| Code | Indication | Status | Milestones/Comments |
|---|---|---|---|
| | | Preclinical / Phase 1 / Phase 2 / Phase 3 | |
| **JR-141** | MPS II (Hunter syndrome) | Global Ph3 | • On track for ~FY2027: Approval in US, EU, Brazil |
| **JR-142** | Pediatric GHD | Ph3 (Japan) | • Mar 2026: Patient enrollment completed |
| **JR-401G** | Pediatric GHD | Ph3 (Japan) | • Apr 2026: Initiation of first dosing in Ph3<br>• Dose comparison study of GROWJECT™ aimed at changing the approved dosage |
| **JR-171** | MPS I (Hurler syndrome etc.) | Global Ph1/2 completed | • Partnering activities ongoing |
| **JR-441** | MPS IIIA (Sanfilippo syndrome type A) | Ph1/2 (Germany)<br>Ph1 (Japan) | • Ph1/2: Achieved 1-year clinical data for the initially planned dose groups<br>• Ph1: Patient enrollment completed<br>• Actively pursuing early approval in Japan |
| **JR-446** | MPS IIIB (Sanfilippo syndrome type B) | Ph1/2 (Japan) | • Recruitment of first cohort completed<br>• Actively pursuing early approval in Japan<br>• Partnered with MEDIPAL HOLDINGS |
| **JR-471** | Fucosidosis | | • Mar 2026: Initiation of natural history study<br>• Partnered with MEDIPAL HOLDINGS |
| **JR-479** | GM2 gangliosidosis (Tay-Sachs disease, Sandhoff disease) | | • Partnered with MEDIPAL HOLDINGS |
| **Givinostat** | Duchenne muscular dystrophy | Approved in the US, the EU and other countries | • Under discussions with PMDA toward domestic approval by 2028 |

# Driving Value through Strategic Alliances






**J-Brain Cargo®**
- Alexion, AstraZeneca Rare Disease (Neurodegenerative disease, oligonucleotide therapeutics)
- Angelini Pharma (Epilepsy)
- Acumen Pharmaceuticals, Inc. (Alzheimer's disease)

**JUST-AAV**
- Alexion, AstraZeneca Rare Disease
- Modalis Therapeutics Corporation

**MEDIPAL HOLDINGS CORPORATION**
- Out-licensing of therapies for ultra-rare diseases (JR-471, JR-446, JR-479)

**Menagen Pharmaceutical Industries LLC**
- Out-licensing of local marketing authorizations and commercialization rights for Agalsidase Beta BS (Nine MENAT markets)

**SanBio Co., Ltd.**
- Contract manufacturing for pilot production toward commercial manufacturing of Akuugo®

**Italfarmaco S.p.A.**
- In-licensing of givinostat for duchenne muscular dystrophy
- Strategic collaboration in rare disease therapies

# Givinostat: Oral Treatment for DMD

## 1 Multifaceted MoA: Increases muscle regeneration and reduces fibrogenesis, adipogenesis, and immune/inflammatory responses

- HDAC inhibition mechanism enabling mutation-independent use

## 2 Regulatory approvals outside Japan

- Regulatory approvals in the US, the EU and other countries[1]
- Achieved the primary endpoint in a placebo-controlled Phase III clinical trial[2]
  (mean change in the 4SC study (shown on the right))

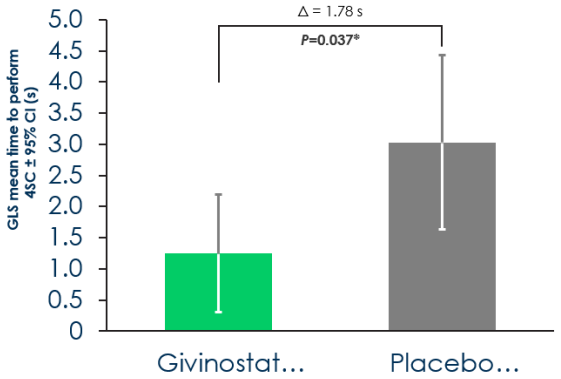
## 3 Ongoing Studies Aimed at Expanding Clinical Evidence

- Phase III study in non-ambulatory DMD patients (NCT05933057)
- Phase II study in DMD patients aged 2-5 years (NCT06769633)

## 4 Strong commercial potential in Japan

- ~3,500 individuals in Japan diagnosed with DMD[3]
  - Over 1,000 individuals: Ambulatory, ≥6 years of age[4]
  - Over 3,000 individuals: ≥6 years of age[4]

**Mean change from baseline in time to perform the 4SC test at 72 weeks (non-log transformed)**




*Data are means and 95% confidence intervals. The confidence intervals have not been adjusted for multiplicity and should not be used for hypothesis testing. Baseline mean values were 3.39 s and 3.48 s for the givinostat and placebo groups, respectively. All patients were also receiving systemic corticosteroids in a dose and regimen that was to remain unchanged over the follow-up period.

DMD, duchenne muscular dystrophy; HDAC, histone deacetylase; 4SC, 4-stair climb.

1. US: Approved for patients with DMD aged 6 years and older, EU: Conditionally approved for ambulatory DMD patients aged 6 years and older and already being treated with corticosteroids
2. Mercuri E et al. Lancet Neurol. 2024;23(4):393-403. 3. Kawai M. *No To Hattatsu.* (Japanese) 2013;45(Supple.):S324
4. Company estimates based on Remudy (Registry of Muscular Dystrophy) and Nakamura H et al. *Orphanet J Rare Dis.* 2013;8:60

# Toward ¥100 billion revenue in the 2030s

## Enabled by three growth drivers

### Domestic Sales

Expansion of domestic product sales and revenue growth from in-licensed products

### Platform licensing revenue

Growth in licensing revenue through out-licensing of platform technologies

### Overseas revenue (LSD pipeline)

Expansion of overseas revenue through out-licensing of the LSD pipeline

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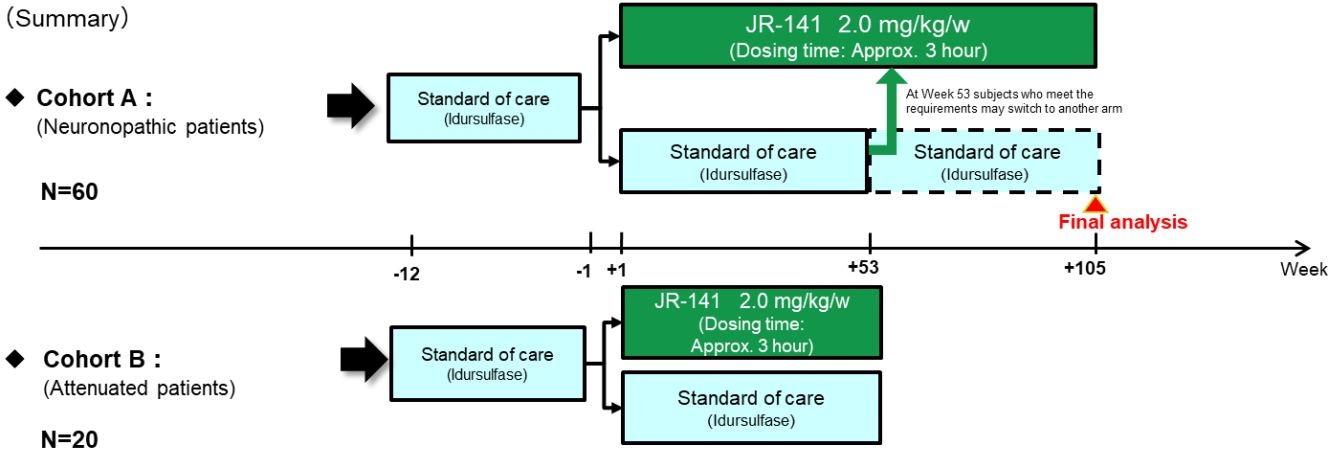
# Progress on Developmental Pipelines

**Anne Bechet**
Managing Executive Officer
Executive Director, Development Division
General Manager, JCR Europe B.V.
CEO, JCR USA Inc.

# JR-141 (pabinafusp alfa: BBB-penetrating ERT for MPS II)

## Global Phase III study（JR-141-GS31): STARLIGHT study Overview




Constructive meeting took place with FDA in June 2025

Enrollment of the target number of 80 participants achieved in July 2025

**On track to obtain approvals in the US, EU, and Brazil in FY2027**

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# FY2025 Consolidated Financial Results
# FY2026 Consolidated Financial Forecasts

**Yoh Ito**
Managing Executive Officer
Executive Director, Corporate Strategy Division

# Revision to FY2024 Actual Results



- The prior-year results shown in this document have been revised to reflect corrections to certain items in the Consolidated Financial Results for the Year ended March 31, 2025 (FY2024) (Japanese standard).

# Overview: Consolidated Financial Results



(Unit：million yen)

| Consolidated | FY2024 | FY2025 | | |
|---|---|---|---|---|
| | Results | Results | Year-on-year | |
| | | | Difference | Ratio |
| **Net Sales** | 33,072 | **40,319** | +7,247 | +21.9% |
| **Cost of Sales** | 10,902 | **10,134** | (767) | (7.0)% |
| **Gross Profit** | 22,169 | **30,185** | +8,015 | +36.2% |
| **Selling, General and Administrative Expenses** | 28,389 | **29,629** | +1,240 | +4.4% |
| **SG&A Expenses** | 12,958 | **12,867** | (90) | (0.7)% |
| **R&D Expenses** | 15,431 | **16,761** | +1,330 | +8.6% |
| **Operating profit** | (6,219) | **555** | +6,775 | - |
| **Non-operating Income** | 260 | **1,449** | +1,188 | +455.5% |
| **Non-operating Expenses** | 1,088 | **839** | (248) | (22.9)% |
| **Ordinary profit** | (7,046) | **1,165** | +8,212 | - |
| **Extraordinary Income** | 1,065 | **2,091** | +1,026 | +96.3% |
| **Extraordinary Losses** | 2 | **32** | +30 | - |
| **Profit before Income Taxes** | (5,983) | **3,224** | +9,208 | - |
| **Income Taxes** | (1,523) | **1,046** | +2,569 | - |
| **Profit Attributable to Owners of Parent** | (4,460) | **2,178** | +6,638 | - |
| **Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations** | 16,994 | **18,398** | +1,403 | +8.3% |

## Additional Remarks

- Net sales increased mainly due to higher product sales, including IZCARGO™, as well as growth in contract-related revenue.
- The cost of sales ratio (excluding contract revenue) declined significantly year-on-year; however, excluding raw material disposal losses recorded in the prior year, it remained broadly unchanged.
- R&D expenses increased due to higher clinical development costs and upfront payment for license rights, while the prior year included inventory disposal losses.
- Non-operating income increased as a result of foreign exchange gains and gains on the sale of investment securities.
- Non-operating expenses decreased, mainly due to a reduction in equity-method investment losses and foreign exchange losses.
- Subsidy income was recorded as extraordinary income following confirmation of the subsidy for the Kobe Science Park Center (API Plant).

| Net Sales | FY2024 | FY2025 | Difference |
|---|---|---|---|
| **Cost of Sales Ratio** | 33.0% | **25.1%** | (7.9)% |
| **Cost of Sales Ratio *Excluding contract revenue** | 32.2% | **27.6%** | (4.6)% |
| **R&D Expenses Ratio** | 46.7% | **41.6%** | (5.1)% |
| **Operating Profit Ratio** | (18.8)% | **1.4%** | +20.2% |

# Breakdown of Net Sales (Consolidated)

(Unit: million yen)

| Consolidated | FY2024 | FY2025 | | |
|---|---|---|---|---|
| | Results | Results | Year-on-year | |
| | | | Difference | Ratio |
| **GROWJECT™** | 18,098 | **17,933** | (164) | (0.9)% |
| **IZCARGO™** | 5,718 | **6,766** | +1,047 | +18.3% |
| **TEMCELL™ HS Inj.** | 2,904 | **2,831** | (73) | (2.5)% |
| **Treatments for renal anemia** | 3,784 | **3,622** | (162) | (4.3)% |
| **Epoetin Alfa BS Inj. [JCR]** | 1,690 | **1,121** | (569) | (33.7)% |
| **Darbepoetin Alfa BS Inj. [JCR]** | 2,093 | **2,501** | +407 | +19.5% |
| **Agalsidase Beta BS I.V. Infusion [JCR]** | 1,149 | **1,292** | +142 | +12.4% |
| **Total Core Products** | 31,655 | **32,446** | +790 | +2.5% |
| **Contract revenue** | 517 | **5,549** | +5,032 | +972.8% |
| **Other** | 898 | **2,323** | +1,425 | +158.5% |
| **Total Net Sales** | 33,072 | **40,319** | +7,247 | +21.9% |

## Additional Remarks

- Sales of GROWJECT™, IZCARGO™, and TEMCELL™ HS Inj. remained solid and exceeded the announced plan.
- Sales of the Treatments for renal anemia were in line with the supply plan for Kissei Pharmaceutical Co., Ltd.
- Sales of Agalsidase Beta BS I.V. Infusion [JCR] were in line with the supply plan for Sumitomo Pharma Co., Ltd.
- Contract revenue consisted of upfront payments and milestone income under existing agreements.

# Financial Status (Consolidated)

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(Unit: million yen)

| | End-Mar. 2025 | End-Mar. 2026 | Change • Main Increase/decrease | | End-Mar. 2025 | End-Mar. 2026 | Change • Main Increase/decrease |
|---|---|---|---|---|---|---|---|
| **Current assets** | 51,487 | **56,076** | **Total +4,588**<br>• Accounts receivable - trade, and contract assets +1,927<br>• Inventories +1,813 | **Current liabilities** | 43,683 | **48,135** | **Total +16,448**<br>• Short-term borrowings +15,637 |
| | | | | **Non-current liabilities** | 13,431 | **13,741** | **Total (11,686)**<br>• Special suspense account for tax purpose reduction entry (11,750) |
| **Non-current assets** | 53,361 | **53,160** | **Total (201)** | **Total liabilities** | 57,114 | **61,877** | **Total +4,762** |
| | | | | **Total net assets** | 47,734 | **47,359** | **Total (375)**<br>• Dividends paid (2,442)<br>• Profit attributable to owners of parent +2,178 |
| **Total** | 104,849 | **109,236** | **4,386** | **Total** | 104,849 | **109,236** | **4,386** |

## Additional Remarks

- Inventories increased due to higher raw materials and supplies.
- Short-term borrowings increased due to financing for construction of the new drug product manufacturing plant and working capital.
- Following the finalization of subsidies for the Kobe Science Park Center (API plant), both the deferred subsidy account and the related subsidized tangible fixed assets decreased.

| | End-Mar. 2025 | End-Mar. 2026 |
|---|---|---|
| **Equity ratio** | 45.1% | **42.9%** |

# FY2026 Consolidated Financial Forecasts

(Unit : million yen)

| Consolidated | FY2025 | FY2026(Forecast) | | |
|---|---|---|---|---|
| | Results | Forecast | Year-on-year | |
| | | | Difference | Ratio |
| **Net Sales** | 40,319 | **45,700** | +5,381 | +13.3% |
| **Cost of Sales** | 10,134 | **10,600** | +466 | +4.6% |
| **Gross Profit** | 30,185 | **35,100** | +4,915 | +16.3% |
| **Selling, General and Administrative Expenses** | 29,629 | **33,900** | +4,271 | +14.4% |
| **SG&A Expenses** | 12,867 | **14,500** | +1,633 | +12.7% |
| **R&D Expenses** | 16,761 | **19,300** | +2,539 | +15.1% |
| **Operating Profit** | 555 | **1,100** | +545 | +98.2% |
| **Ordinary Profit** | 1,165 | **500** | (665) | (57.1)% |
| **Profit Attributable to Owners of Parent** | 2,178 | **200** | (1,978) | (90.8)% |
| **Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations** | 18,398 | **24,300** | +5,902 | +32.1% |

## Additional Remarks

- Net sales are forecast to exceed the previous fiscal year, mainly due to higher contract revenue.
- SG&A expenses are forecast to increase, mainly due to higher fees and commissions. R&D expenses are also forecast to rise, reflecting progress in global and domestic clinical trials.
- Operating profit is forecast to increase, as higher sales will offset higher expenses.
- Ordinary profit and profit attributable to owners of parent are forecast to decrease, as foreign exchange gains, gains on sales of securities, and subsidy income recorded in the previous fiscal year are not expected this fiscal year.

| Net Sales | FY2025 | FY2026 (Forecast) | Difference |
|---|---|---|---|
| **Cost of Sales Ratio** | 25.1% | **23.2%** | (1.9)% |
| **Cost of Sales Ratio *Excluding contract revenue** | 27.6% | **26.7%** | (0.9)% |
| **R&D Expenses Ratio** | 41.6% | **42.4%** | +0.8% |
| **Operating Profit Ratio** | 1.4% | **2.6%** | +1.2% |

# Breakdown of Net Sales – FY2026 Consolidated Financial Forecasts



(Unit：million yen)

| Consolidated | FY2025 | FY2026(Forecast) | | |
|---|---|---|---|---|
| | Results | Forecast | Year-on-year | |
| | | | Difference | Ratio |
| GROWJECT™ | 17,933 | **17,500** | (433) | (2.4)% |
| IZCARGO™ | 6,766 | **6,900** | +134 | +2.0% |
| TEMCELL™HS Inj. | 2,831 | **2,700** | (131) | (4.6)% |
| Treatments for renal anemia | 3,622 | **3,700** | +78 | +2.2% |
| Epoetin Alfa BS Inj. [JCR] | 1,121 | **1,400** | +279 | +24.9% |
| Darbepoetin Alfa BS Inj. [JCR] | 2,501 | **2,300** | (201) | (8.0)% |
| Agalsidase Beta BS I.V. Infusion [JCR] | 1,292 | **2,000** | +708 | +54.8% |
| Total Core products | 32,446 | **32,900** | +454 | +1.4% |
| Contract revenue | 5,549 | **8,100** | +2,551 | +46.0% |
| Other | 2,323 | **4,500** | +2,177 | +93.7% |
| Total net sales | 40,319 | **45,700** | +5,381 | +13.3% |

## Additional Remarks

- GROWJECT™ sales are forecast to decrease due to NHI drug price revisions, despite sales volume remaining largely unchanged.
- IZCARGO™ sales are forecast to continue increasing, driven by an increase in treated patients.
- TEMCELL™ sales are forecast to decrease due to changes in the competitive environment.
- Renal anemia products and Agalsidase Beta BS I.V. Infusion [JCR] are forecast to remain solid, with sales in line with supply plans for distribution partners.
- Contract revenue is forecast to exceed the previous fiscal year, driven by new product out-licensing agreements and progress in existing joint research projects.


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# Appendix

# JR-401G : An Adjustable-Dose Growth Hormone Therapy Project



## GROWJECT™

| Indication | Dosage |
|---|---|
| Short stature due to growth hormone deficiency without epiphyseal closure | 0.175 mg/kg/week |
| Short stature associated with Turner syndrome without epiphyseal closure | 0.35 mg/kg/week |
| Adult growth hormone deficiency (limited to severe cases) | 0.021~0.084 mg/kg/week |
| Short stature due to small for gestational age (SGA) without epiphyseal closure | 0.23~0.47 mg/kg/week |
| Short stature associated with SHOX deficiency without epiphyseal closure | 0.35 mg/kg/week |

For daily growth hormone products, dose adjustment according to patient treatment response is permitted outside of Japan
(In the US, dose adjustment is allowed within the range of 0.175–0.3 mg/kg/week)

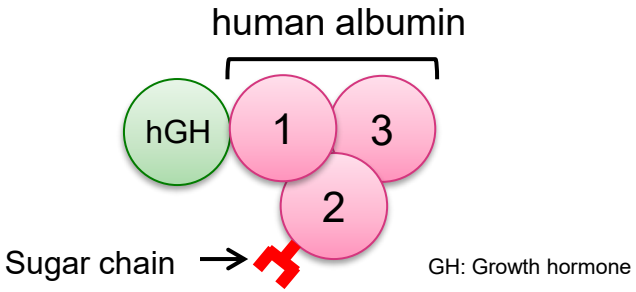
To bridge the gap between Japanese and international dosing practices and to improve final adult height and quality of life (QOL), JCR has initiated development of an adjustable dosing regimen for growth hormone therapy in Japan

# Long-acting Growth Hormone Product

**JR-142**

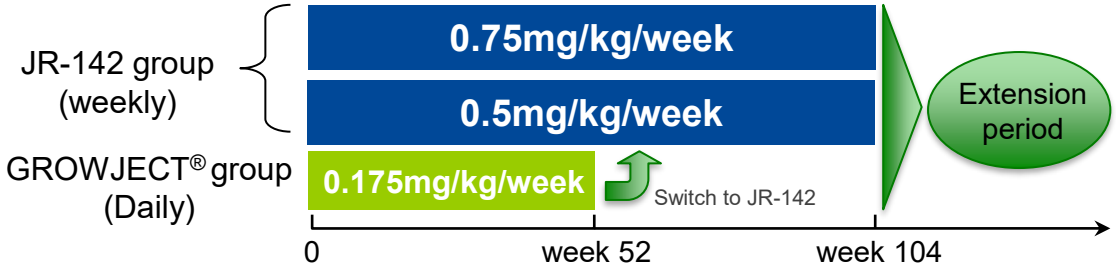
Long-acting growth hormone (rDNA origin)
Indication: Pediatric growth hormone deficiency

## Modified albumin-fused GH




In-house development of fusion protein with modified albumin glycosylation to improve blood retention

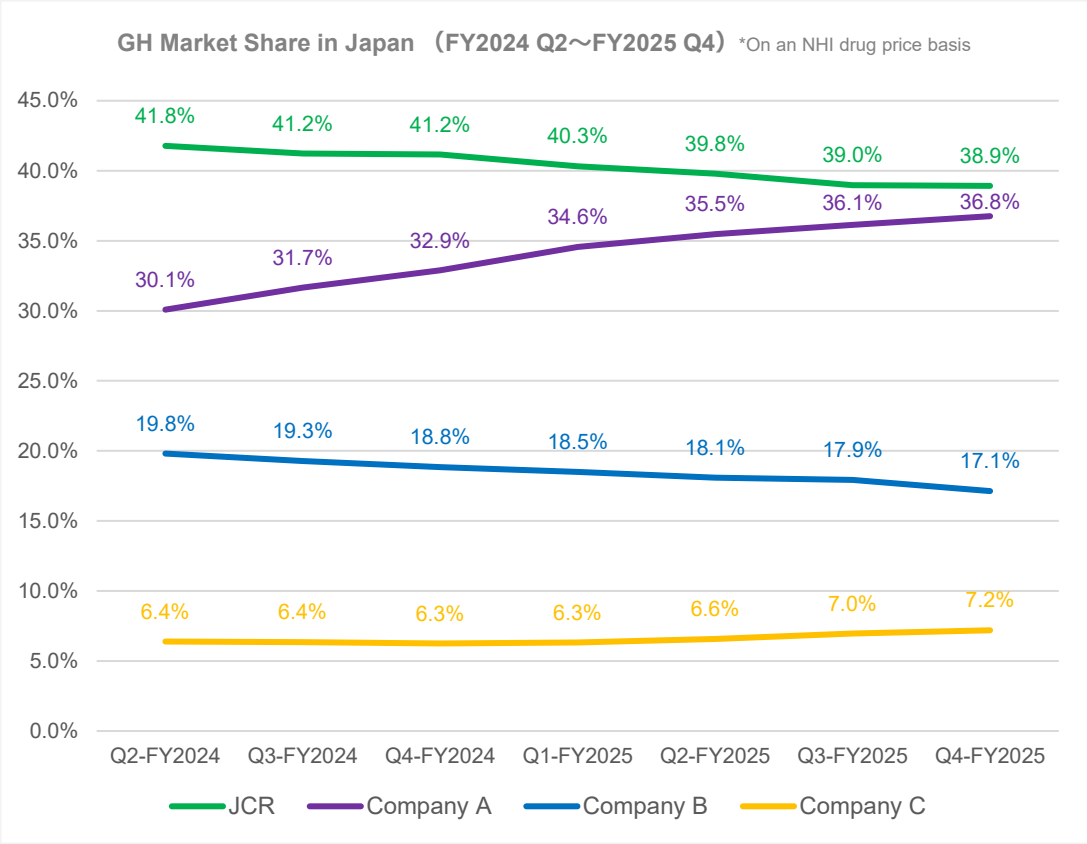
## Phase III study design



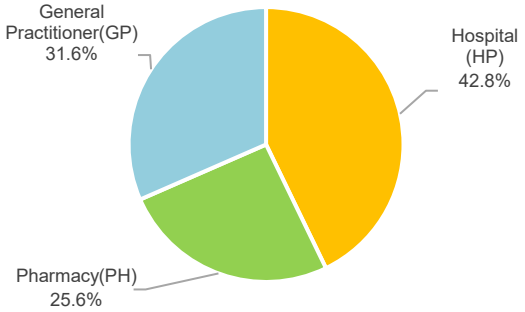

| Overview | |
|---|---|
| **Objective** | • Verify the non-inferiority, and evaluate the efficacy and safety of JR-142 to GROWJECT® |
| **Endpoint** | • Change in height SDS for chronological age from the first administration (Week 52) |
| **Target number of patients** | • 54 |

Completed the patient enrollment

# GROWJECT™ Market Share Trends in Japan (Quarterly)




## ■ Human Growth Hormone Market in Japan

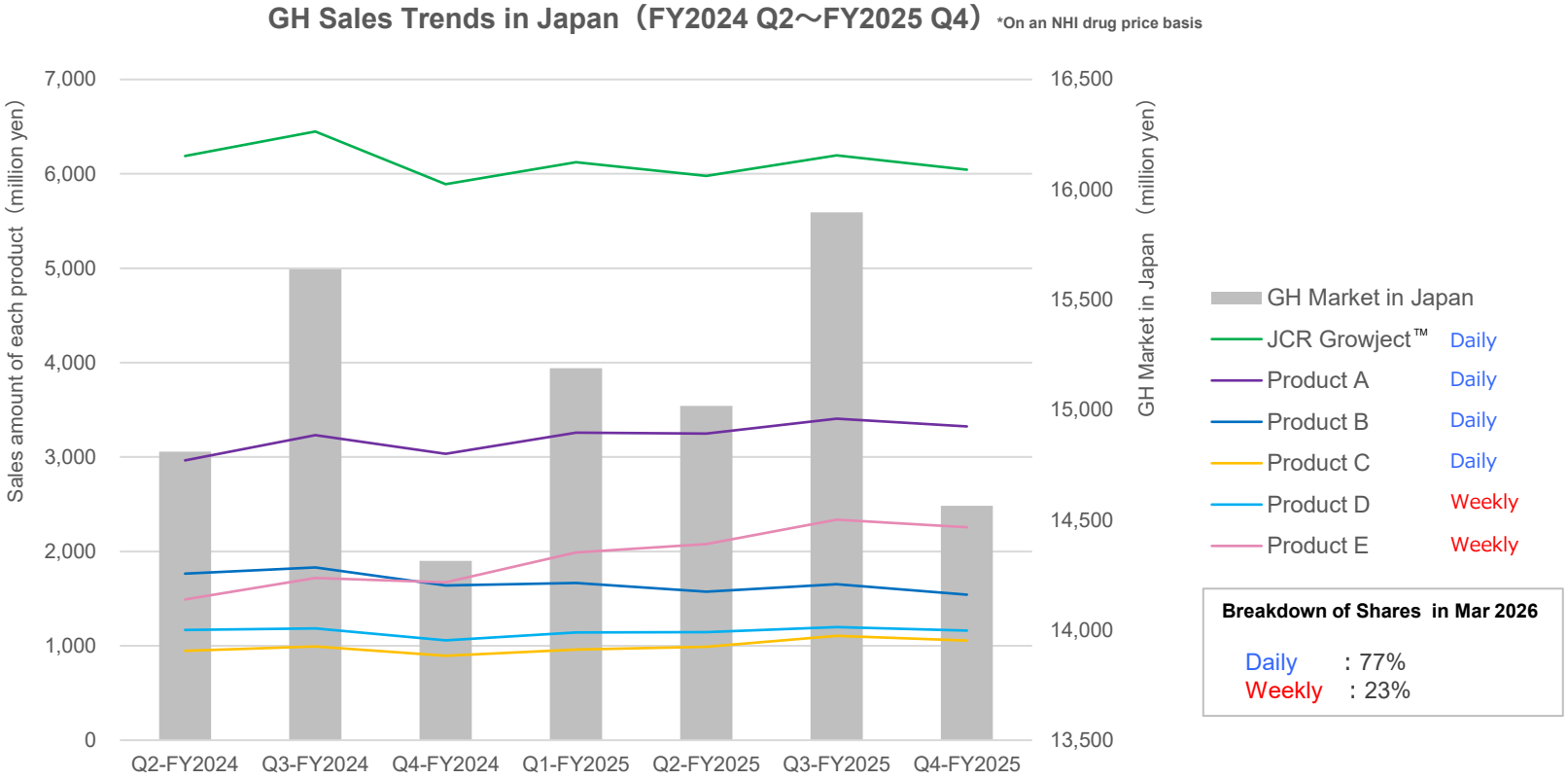




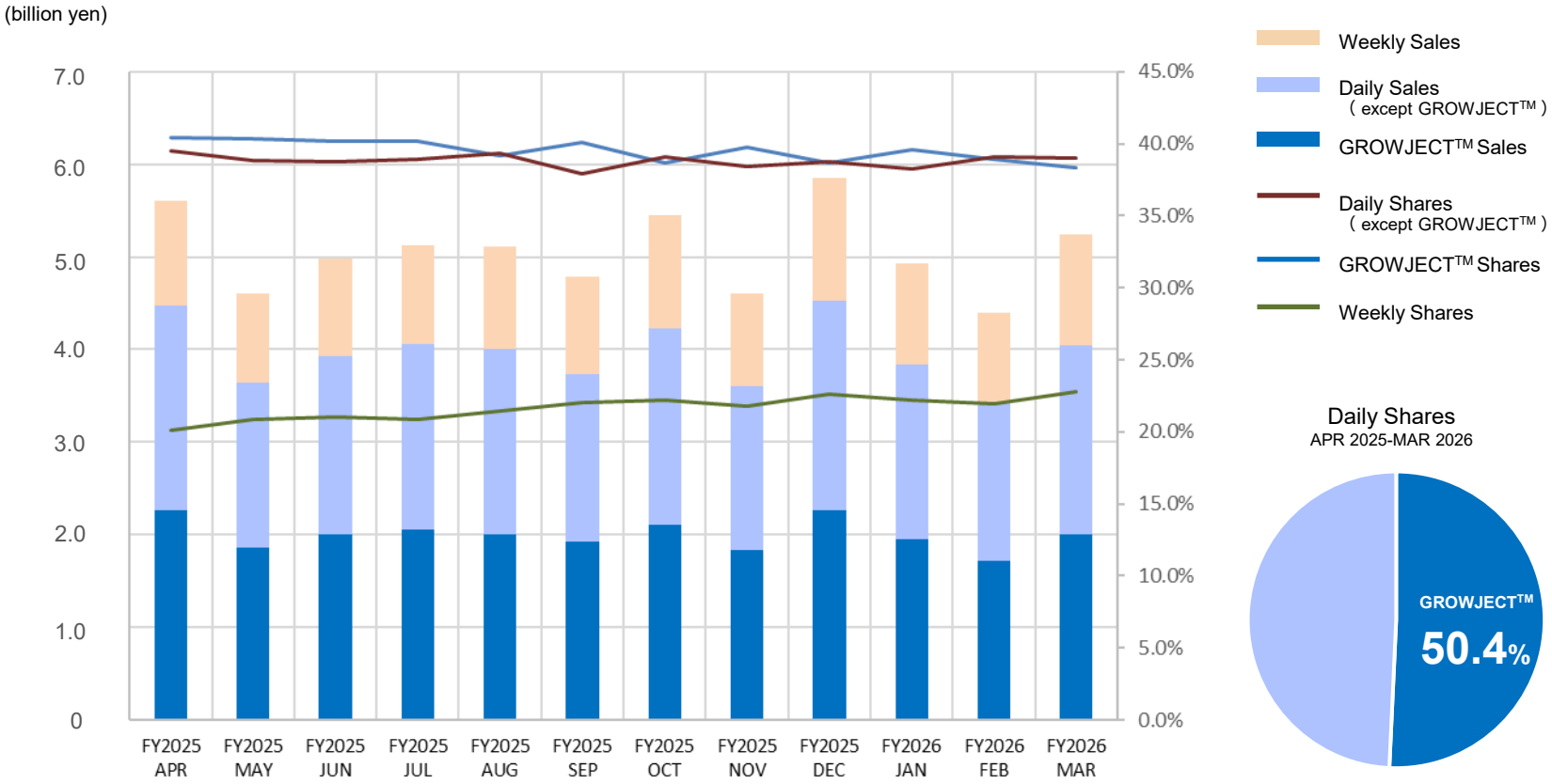

## ■ GROWJECT™ Market Share by buyer

| | Mar 2026 | Sales Change FY2025 Q4 (vs. FY2024 Q4) *On an NHI drug price basis |
|---|---|---|
| HP Market | 31.1% | -88 million yen |
| PH Market | 29.2% | -48 million yen |
| GP Market | 56.0% | -85 million yen |

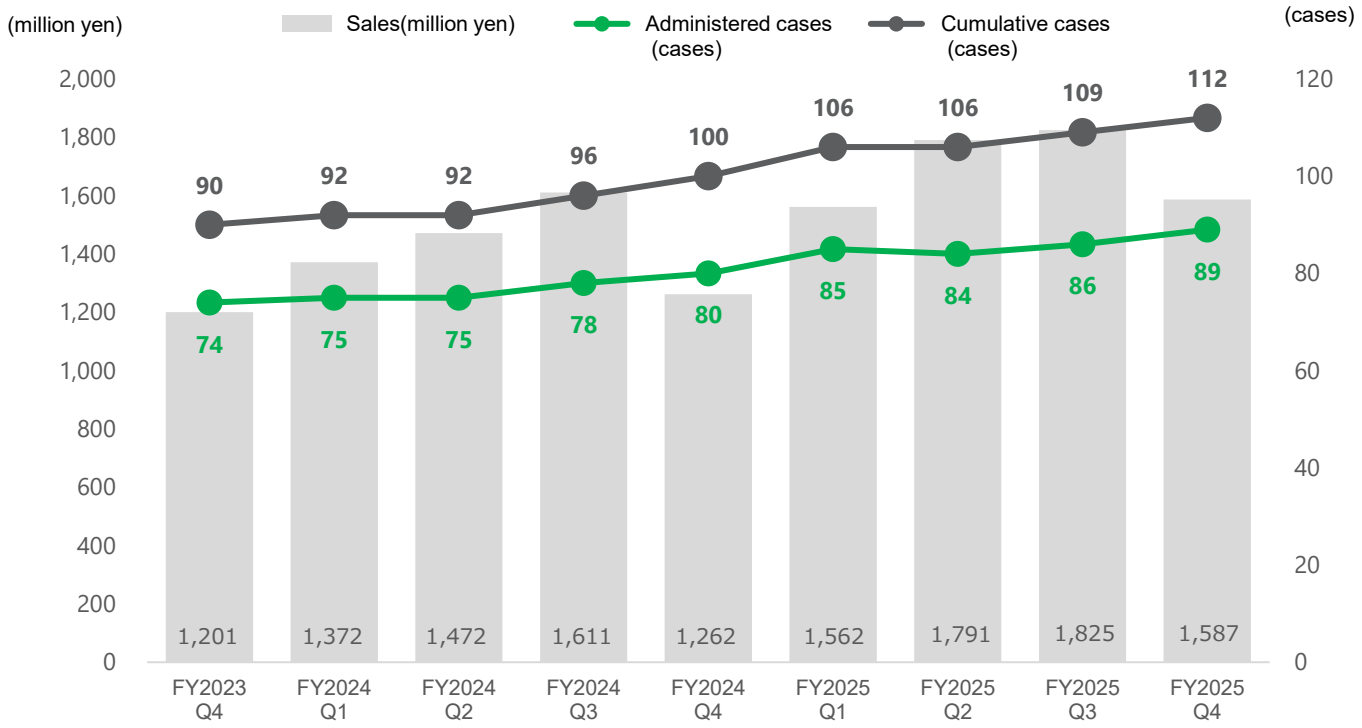
# GROWJECT™ Sales Trends in Japan (Quarterly)



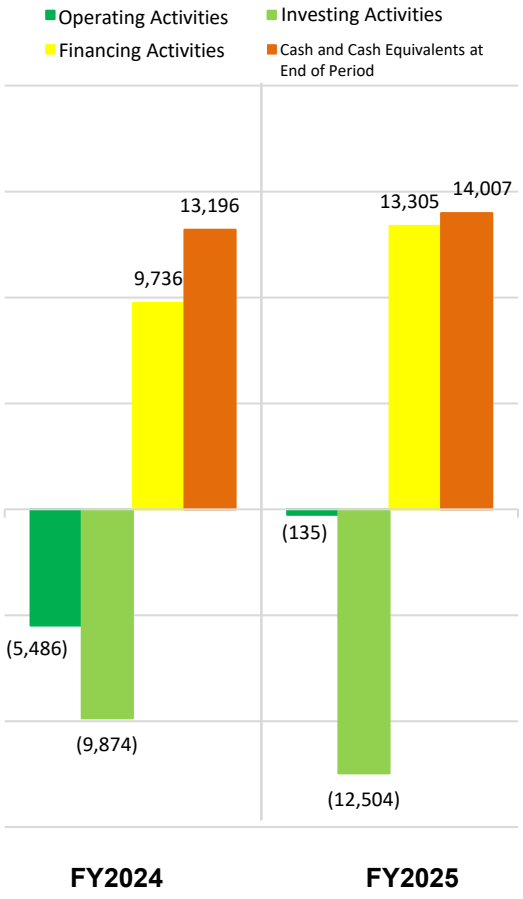
# GROWJECT™ Trends of Market Share in Japan



# IZCARGO™ Sales Performance in Japan



# Cash Flows (Consolidated)




(Unit: million yen)

| | FY2024 | FY2025 | Difference |
|---|---|---|---|
| Profit before income taxes | (5,983) | **3,224** | 9,208 |
| Depreciation | 3,374 | **2,557** | (816) |
| Decrease (increase) in trade receivables and accounts receivable – other | 2,453 | **(1,925)** | (4,379) |
| Decrease (increase) in inventories | (1,253) | **(1,853)** | (599) |
| Increase (decrease) in trade payables and accounts payable – other | (36) | **(106)** | (69) |
| Income taxes paid | (2,284) | **763** | 3,048 |
| Other | (1,755) | **(2,796)** | (1,040) |
| **Operating Activities** | (5,486) | **(135)** | +5,350 |
| Capital investment(property,plant and equipment) | (9,973) | **(11,581)** | (1,607) |
| Other | 99 | **(922)** | (952) |
| **Investing Activities** | (9,874) | **(12,504)** | (2,629) |
| Bollowings | 14,805 | **15,787** | 982 |
| Dividends paid/ treasury shares | (5,014) | **(2,433)** | 2,580 |
| Other | (54) | **(47)** | 6 |
| **Financing Activities** | 9,736 | **13,305** | +3,569 |
| Net increase (decrease) in cash and cash equivalents | (5,559) | **811** | +6,371 |
| **Cash and Cash Equivalents at End of Period** | 13,196 | **14,007** | +811 |

| | FY2024 | FY2025 |
|---|---|---|
| Depreciation | 3,374 | **2,557** |
| Capital investment | 9,973 | **11,581** |

# Abbreviations



| | | |
|---|---|---|
| AAV | Adeno-associated virus | アデノ随伴ウイルス |
| BBB | Blood-brain barrier | 血液脳関門 |
| CNS | Central nervous system | 中枢神経系 |
| ERT | Enzyme replacement therapy | 酵素補充療法 |
| FDA | Food and Drug Administration | 米国食品医薬品局 |
| GH | Growth hormone | 成長ホルモン |
| GHD | Growth hormone deficiency | 成長ホルモン分泌不全性低身長症 |
| i.v. | Intravenous injection | 静脈注射 |
| JBC | J-Brain Cargo® | - |
| LSD | Lysosomal storage disorders | ライソゾーム病 |
| MENAT | Middle East, North Africa and Turkey | 中東、北アフリカ、トルコ |
| MPS | Mucopolysaccharidosis | ムコ多糖症 |
| mRNA | messenger RNA | 伝令RNA |
| Ph I | Phase I | 臨床第１相試験 |
| Ph II | Phase II | 臨床第２相試験 |
| Ph III | Phase III | 臨床第３相試験 |
| R&D | Research and development | 研究開発 |
| TfR | Transferrin receptor | トランスフェリン受容体 |
| YTD | Year to date | 年度累計 |