Reach Beyond, Together

FY2024 Results Briefing Session

May 13, 2025

JCR Pharmaceuticals Co., Ltd.

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FY2024 Consolidated Financial Results
FY2025 Consolidated Financial Forecasts

Yoh Ito
Senior Executive Officer
Executive Director, Corporate Strategy Division

Overview: Consolidated Financial Results

(Unit：million yen)

Consolidated	FY2023	FY2024		
	Results	Results	Year-on-year	
			Difference	Ratio
Net Sales	42,871	**33,072**	(9,799)	(22.9)%
Cost of Sales	11,620	**11,333**	(287)	(2.5)%
Gross Profit	31,251	**21,738**	(9,512)	(30.4)%
Selling, General and Administrative Expenses	23,719	**28,389**	+4,670	+19.7%
SG&A Expenses	12,484	**12,958**	+473	+3.8%
R&D Expenses	11,234	**15,431**	+4,196	+37.4%
Operating Profit (Loss)	7,531	**(6,650)**	(14,182)	-
Non-operating Income	1,056	**260**	(796)	(75.3)%
Non-operating Expenses	1,324	**1,088**	(236)	(17.9)%
Ordinary Profit (Loss)	7,264	**(7,477)**	(14,742)	-
Extraordinary Income	0	**1,065**	+1,064	-
Extraordinary Losses	20	**2**	(18)	(90.0)%
Profit (Loss) before Income Taxes	7,244	**(6,414)**	(13,658)	-
Income Taxes	1,736	**(1,655)**	(3,391)	-
Profit (Loss) Attributable to Owners of Parent	5,507	**(4,759)**	(10,266)	-
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	12,787	**16,994**	+4,207	+32.9%

Additional Remarks

- Both revenue and profit declined year on year.
- The primary factors were: (1) the absence of expected licensing income following the failure to finalize a planned agreement, and (2) inventory-related losses recognized for raw materials and investigational products deemed no longer usable.
- The loss on disposal mentioned above is recognized as 1,950 million yen in Cost of Sales and 1,060 million yen in R&D expenses.
- R&D expenses increased, primarily reflecting investments in strengthening overseas clinical development capabilities, expanding personnel, and advancing development programs.
- Non-operating results were impacted by foreign exchange losses, with no foreign exchange gains recorded this year, while losses from equity-method investments decreased year on year.
- No extraordinary Income that had been anticipated was recognized this period from government subsidies for the API Plant at the Kobe Science Park Center, as the grant confirmation was deferred to the next fiscal year.

Net Sales	FY2023	FY2024	Difference
Cost of Sales Ratio	27.1%	34.3%	+7.2%
Cost of Sales Ratio *Excluding income from contractual payment	32.8%	34.8%	+2.0%
R&D Expenses Ratio	26.2%	46.7%	+20.5%
Operating Profit Ratio	17.6%	(20.1)%	-

*On May 14 2025, Additional Remarks was corrected. Correction is indicated by underlining.

Breakdown of Net Sales (Consolidated)

(Unit: million yen)

Consolidated	FY2023	FY2024		
	Results	Results	Year-on-year	
			Difference	Ratio
GROWJECT®	17,913	**18,098**	+184	+1.0%
IZCARGO® *	5,171	**5,718**	+547	+10.6%
TEMCELL®HS Inj.	3,236	**2,904**	(331)	(10.2)%
Treatments for renal anemia	4,652	**3,784**	(868)	(18.7)%
Epoetin Alfa BS Inj. [JCR]	1,994	**1,690**	(303)	(15.2)%
Darbepoetin Alfa BS Inj. [JCR]	2,658	**2,093**	(564)	(21.2)%
Agalsidase Beta BS I.V. Infusion [JCR]	1,661	**1,149**	(512)	(30.8)%
Total Core Products	32,636	**31,655**	(981)	(3.0)%
Income from contractual payment	7,413	**517**	(6,896)	(93.0)%
Other*	2,820	**898**	(1,922)	(68.1)%
Total Net Sales	42,871	**33,072**	(9,799)	(22.9)%

* Sales of IZCARGO® related to NPS is included in Other.

Additional Remarks

- GROWJECT® revenue increased despite the NHI price revision in April 2024, supported by higher sales volume.
- IZCARGO® continued its strong momentum, posting a 10.6% increase in year on year revenue.
- TEMCELL®HS Inj. revenue declined 10.2% year on year due to intensifying market competition, but remained in line with forecasts.
- Sales of the treatments for renal anemia remained aligned with the supply plans of Kissei Pharmaceutical Co., Ltd.
- Sales of AgalsidaseBeta BS I.V. Infusion [JCR] remained aligned with the supply plans of Sumitomo Pharma Co., Ltd..
- Revenue from licensing-related payments fell short of initial projections because the licensing agreements were not concluded within FY2024.
- Other revenue decreased following the termination of contract manufacturing agreements.

*On May 14 2025, Additional Remarks was corrected. Correction is indicated by underlining.

Financial Status (Consolidated)

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（Unit: million yen）

	End-Mar. 2024	End-Mar. 2025	Change • Main Increase/decrease		End-Mar. 2024	End-Mar. 2025	Change • Main Increase/decrease
Current assets	57,581	**51,056**	**Total (6,524)** • Cash and deposits (5,559) • Accounts receivable - trade, and contract assets (2,698) • Inventories +822	**Current liabilities**	30,135	**43,988**	**Total +13,852** • Short-term borrowings +17,105 • Income taxes payable (1,620)
				Non-current liabilities	15,615	**13,431**	**Total (2,183)** • Long-term borrowings (2,300)
Non-current assets	44,644	**53,798**	**Total +9,154** • Property, plant and equipment +7,369 • Deferred tax assets +1,697	**Total liabilities**	45,750	**57,420**	**Total +11,669**
				Total net assets	56,475	**47,435**	**Total (9,040)** • Net loss (4,759) • Treasury shares (2,103)
Total	102,226	**104,855**	**2,629**	**Total**	102,226	**104,855**	**2,629**

Additional Remarks

- Property, plant, and equipment increased due to the start of construction of the New Drug Product Plant in the Kobe Science Park Center.
- Short-term borrowings increased to fund the construction of the new facility and working capital, reflecting reclassification from Long-term borrowings.

	End-Mar. 2024	End-Mar. 2025
Equity ratio	54.2%	**44.8%**

FY2025 Consolidated Financial Forecasts

(Unit：million yen)

Consolidated	FY2024	FY2025(Forecast)		
	Results	Forecast	Year-on-year	
			Difference	Ratio
Net Sales	33,072	**37,800**	+4,727	+14.3%
Cost of Sales	11,333	**8,200**	(3,133)	(27.6)%
Gross Profit	21,738	**29,600**	+7,861	+36.2%
Selling, General and Administrative Expenses	28,389	**27,000**	(1,389)	(4.9)%
SG&A Expenses	12,958	**12,000**	(958)	(7.4)%
R&D Expenses	15,431	**15,000**	(431)	(2.8)%
Operating Profit (Loss)	(6,650)	**2,600**	+9,250	-
Ordinary Profit (Loss)	(7,477)	**2,400**	+9,877	-
Profit(Loss)Attributable to Owners of Parent	(4,759)	**3,000**	+7,759	-
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	16,994	**17,100**	+105	+0.6%

Additional Remarks

- Net sales is expected to increase year on year, as growth in IZCARGO® sales and higher licensing income are likely to outweigh.
- Cost of sales is expected to decline year on year, as the previous year included one-time losses related to the disposal of raw materials.
- SG&A expenses are expected to decline, reflecting greater operational efficiency, while R&D expenses are also projected to decrease, as last year's figures included one-time write-offs of investigational products—costs that are not anticipated this year despite ongoing progress in global clinical trials.
- Operating income is forecast to increase primarily reflecting higher licensing revenue.
- A one-time gain is expected to be recorded as Extraordinary income, stemming from the reversal of depreciation charges previously booked for the API Plant at Kobe Science Park Center, following the final confirmation of the government subsidy amount.

Net Sales	FY2024	FY2025 (Forecast)	Difference
Cost of Sales Ratio	34.3%	**21.7%**	(12.6)%
Cost of Sales Ratio *Excluding income from contractual payment	34.8%	**25.4%**	(9.4)%
R&D Expenses Ratio	46.7%	**39.7%**	(7.0)%
Operating Profit Ratio	(20.1)%	**6.9%**	+27.0%

Breakdown of Net Sales – FY2025 Consolidated Financial Forecasts

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(Unit：million yen)

Consolidated	FY2024	FY2025(Forecast)		
	Results	Forecast	Year-on-year	
			Difference	Ratio
GROWJECT®	18,098	**17,800**	(298)	(1.6)%
IZCARGO®*	5,718	**6,400**	+681	+11.9%
TEMCELL®HS Inj.	2,904	**2,700**	(204)	(7.0)%
Treatments for renal anemia	3,784	**3,100**	(684)	(18.1)%
Epoetin Alfa BS Inj. [JCR]	1,690	**800**	(890)	(52.7)%
Darbepoetin Alfa BS Inj. [JCR]	2,093	**2,300**	+206	+9.9%
AgalsidaseBeta BS I.V. Infusion [JCR]	1,149	**1,100**	(49)	(4.3)%
Total Core products	31,655	**31,100**	(555)	(1.8)%
Income from contractual payment	517	**5,500**	+4,982	+963.2%
Other*	898	**1,200**	+301	+33.5%
Total net sales	33,072	**37,800**	+4,727	+14.3%

* Sales of IZCARGO®related to NPS is included in Other

Additional Remarks

- GROWJECT® is expected to see lower revenue due to the NHI price revision, despite ongoing efforts to grow market share by promoting the value of its auto-injector device and expanding outreach to new and potential patients.
- IZCARGO® is projected to maintain sales growth through continued efforts under the dedicated MR model launched in April 2023 and joint promotional activities with Sumitomo Pharma Co., Ltd.
- TEMCELL®HS Inj. revenue is expected to decline, reflecting a more competitive market landscape.
- Revenue from the treatments for renal anemia and AgalsidaseBeta BS I.V. Infusion [JCR] is forecast to remain in line with the supply schedules of our marketing partners.
- Licensing revenue is expected to exceed that of the prior year, based on the planned completion.

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Progress on Developmental Pipelines

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

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

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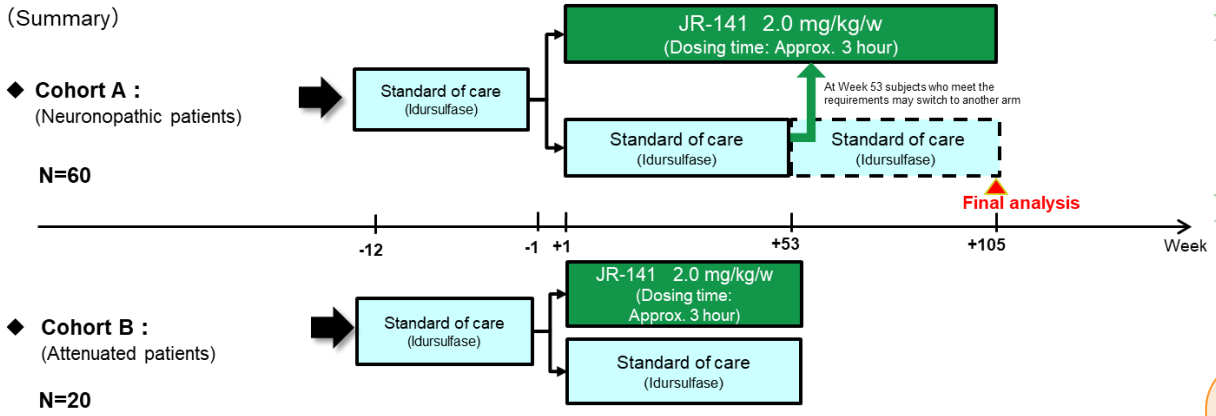
Global Phase III study（JR-141-GS31): STARLIGHT study Overview



Overview	
Objectives	1. To assess the efficacy of JR-141 on CNS signs and symptoms in MPS-II subjects relative to standard ERT 2. To assess control of somatic signs and symptoms by JR-141 relative to standard ERT
Endpoints	• Changes in HS in CSF, CNS symptoms (cognitive, behavior, attention) • Control of systemic sign and symptoms
ClinicalTrials.gov	Identifier : NCT04573023

Current Status

- Number of clinical trial sites
  28 sites
  12 countries
- Achieved **over 95%** of patient recruitment

- **Close collaboration with agencies to target to file as soon as possible**
- **Consultation with FDA to be conducted by June 2025**

Other Pipelines for Lysosomal Storage Disorders

Achieved steady progress in clinical development for rare diseases with no available treatment

JR-441

BBB-penetrating heparan N-sulfatase (rDNA origin)
Indication: MPS type IIIA

- Oct 2023　Initiation of Phase I/II study (Germany)
  - Completed patient enrollment
- Oct 2024　Initiation of Phase I study (Japan)
  - Completed patient enrollment

JR-446

BBB-penetrating α-N-acetylglucosaminidase (rDNA origin)
Indication: MPS type IIIB

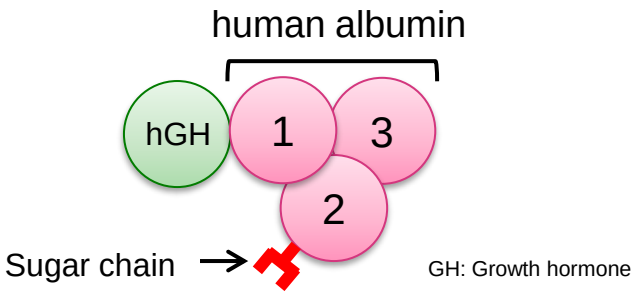
- Dec 2024　Initiation of Phase I/II study (Japan)
- Completed the first safety review by the independent data monitoring committee
  - No safety concerns at this point, decided to continue the study

Growth Hormone Product

JR-142

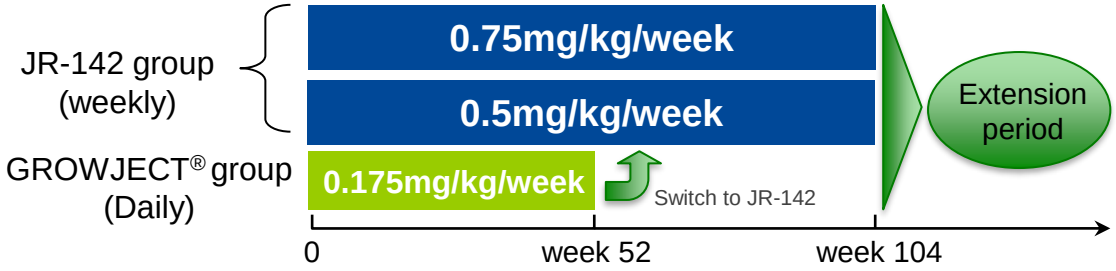
Long-acting growth hormone (rDNA origin)
Indication: Pediatric growth hormone deficiency

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

Dec 2024　First dosing in first subject in Phase III

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	• Q3 FY2025: Enrollment completion • ~FY2027: Approval in US, EU, Brazil
JR-142	Pediatric GHD	Ph3 (Japan)	• Dec 2024: Initiation of first dosing in Ph3
JR-171	MPS I (Hurler syndrome etc.)	Global Ph1/2 completed	• Extension study ongoing • Partnering intensified
JR-441	MPS IIIA (Sanfilippo syndrome type A)	Ph1/2 (Germany) Ph1 (Japan)	<Ph1/2> • Patient enrollment completed • 2H FY2025: 1-year clinical data <Ph1> • Patient enrollment completed
JR-446	MPS IIIB (Sanfilippo syndrome type B)	Ph1/2 (Japan)	• Dec 2024: Initiation of first dosing in Ph1/2 • Partnering with MEDIPAL HOLDINGS
JR-471	Fucosidosis		• Partnering with MEDIPAL HOLDINGS

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Progress on Research

Hiroyuki Sonoda, Ph. D.
Director, Senior Managing Executive Officer
Executive Director, Research Division

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	• Q3 FY2025: Enrollment completion • ~FY2027: Approval in US, EU, Brazil
JR-142	Pediatric GHD				Ph3 (Japan)	• Dec 2024: Initiation of first dosing in Ph3
JR-171	MPS I (Hurler syndrome etc.)				Global Ph1/2 completed	• Extension study ongoing • Partnering intensified
JR-441	MPS IIIA (Sanfilippo syndrome type A)			Ph1/2 (Germany) Ph1 (Japan)		<Ph1/2> • Patient enrollment completed • 2H FY2025: 1-year clinical data <Ph1> • Patient enrollment completed
JR-446	MPS IIIB (Sanfilippo syndrome type B)				Ph1/2 (Japan)	• Dec 2024: Initiation of first dosing in Ph1/2 • Partnering with MEDIPAL HOLDINGS
JR-471	Fucosidosis					• Partnering with MEDIPAL HOLDINGS
JR-479	GM2 gangliosidoses					–

Evaluation in a Mouse Model of GM2 gangliosidosis

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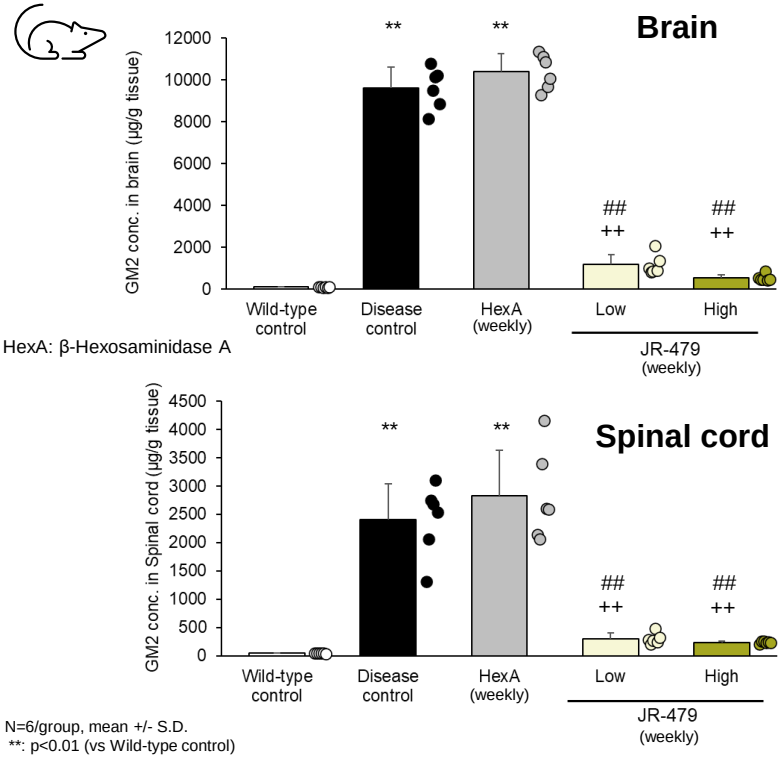
JR-479

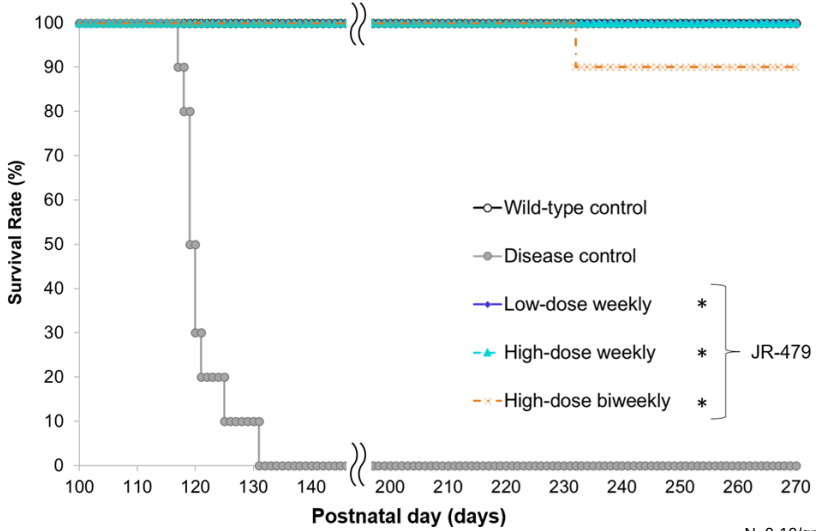
BBB-penetrating β-Hexosaminidase A (rDNA origin)
Indication: GM2 gangliosidoses (Tay-Sachs disease, Sandhoff disease)

Enzyme replacement therapy



HexA: β-Hexosaminidase A

N=6/group, mean +/- S.D.
**: p<0.01 (vs Wild-type control)
##: p<0.01 (vs Disease control)
++: p<0.01 (vs HexA) ,Tukey HSD

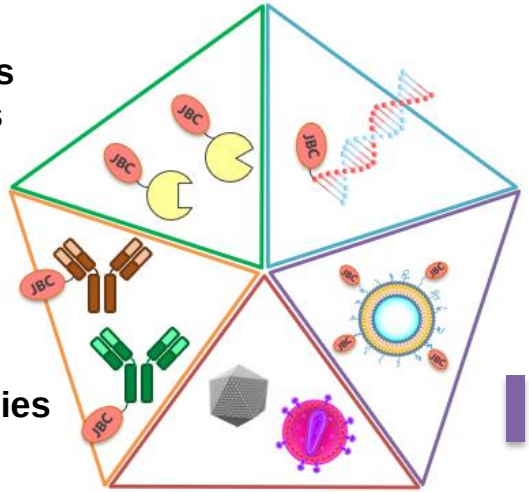


N=9-10/group
*: p<0.01 (vs Disease control)
Log rank test

- Significantly reduced substrates concentrations in brain and spinal cord
- >90% of JR-479-treated mice were still alive at 270 days of age

Collaboration through JCR's Proprietary Technologies

Broad applicability of J-Brain Cargo® to various modalities



- Enzymes Proteins
- Oligonucleotides (ASO, siRNA)
- Antibodies
- LNP (mRNA, low-molecular weight compounds)
- Gene/Cell therapy

2020: Practical use of J-Brain Cargo® technology
Approval of IZCARGO®(Japan)

2022: J-Brain Cargo® partnership with Alexion, Angelini

ALEXION AstraZeneca Rare Disease / Angelini Pharma

2025: Gene therapy partnership with Modalis

MODALIS

JCR

Ultimate Destination of Organ

Safeguarding against off-target delivery

Transformative Technology

AAV: Adeno-Associated Virus

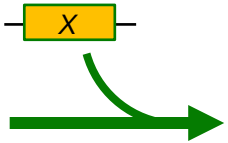
JBC: J-Brain Cargo®

AAV with directionality to target tissues/organs and reduced migration to specific tissues/organs

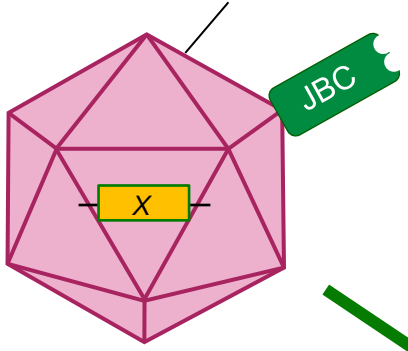
Mechanism of Action of JUST-AAV (in case of brain-targeting type)

AAV-production plasmid with JBC tag sequence

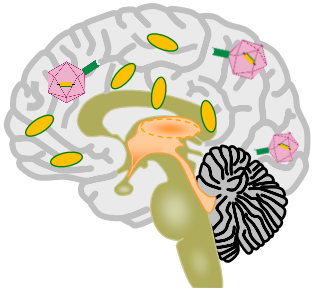
Therapeutic gene X



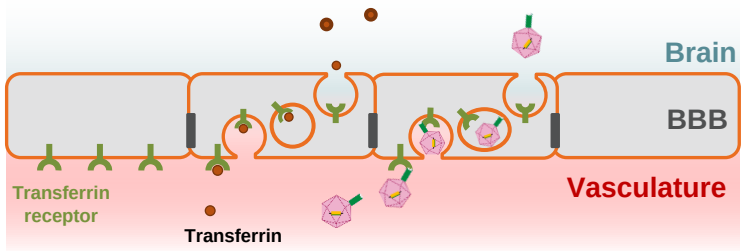
AAV capsid



JUST-AAV

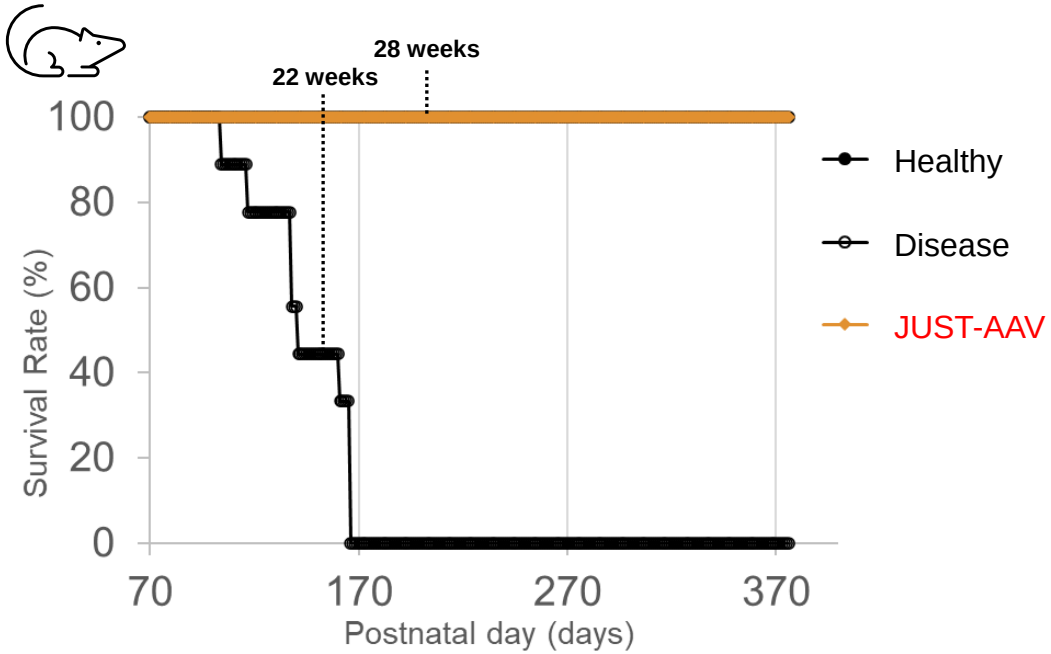


Transgene expression in the brain



Transition of blood-brain barrier via transferrin receptor-mediated transcytosis

Gene Therapy with JUST-AAV: Survival in a Mouse Model of CLN2



Tail coding
Black: Healthy at 28 weeks
Red: JUST-AAV-treated at 28 weeks
None: Disease control at 22 weeks

Nov 20, 2024_R&D meeting

JUST-AAV resulted in survival of more than 370 days, similar to healthy mice

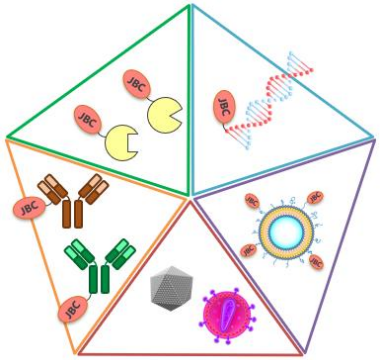
CLN2: Neuronal ceroid lipofuscinosis type 2

Shaping the Future with JCR's Proprietary Technologies

Partnering our groundbreaking technologies and creating breakthrough therapies in various disease areas beyond rare

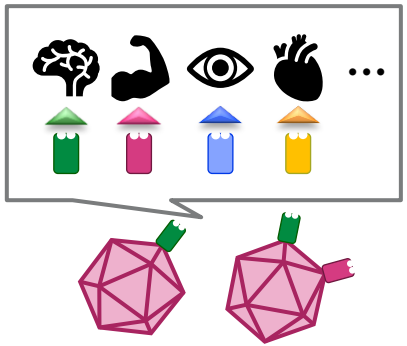
- Lysosomal Storage Disorders
- Neurodegeneration
- Muscular Diseases
- Neuroinflammation
- Neuro-oncology
- . . .

J-Brain Cargo®



Blood-Brain Barrier transport applicable to various modalities

JUST-AAV



AAV with enhanced delivery to target tissues and reduced liver tropism

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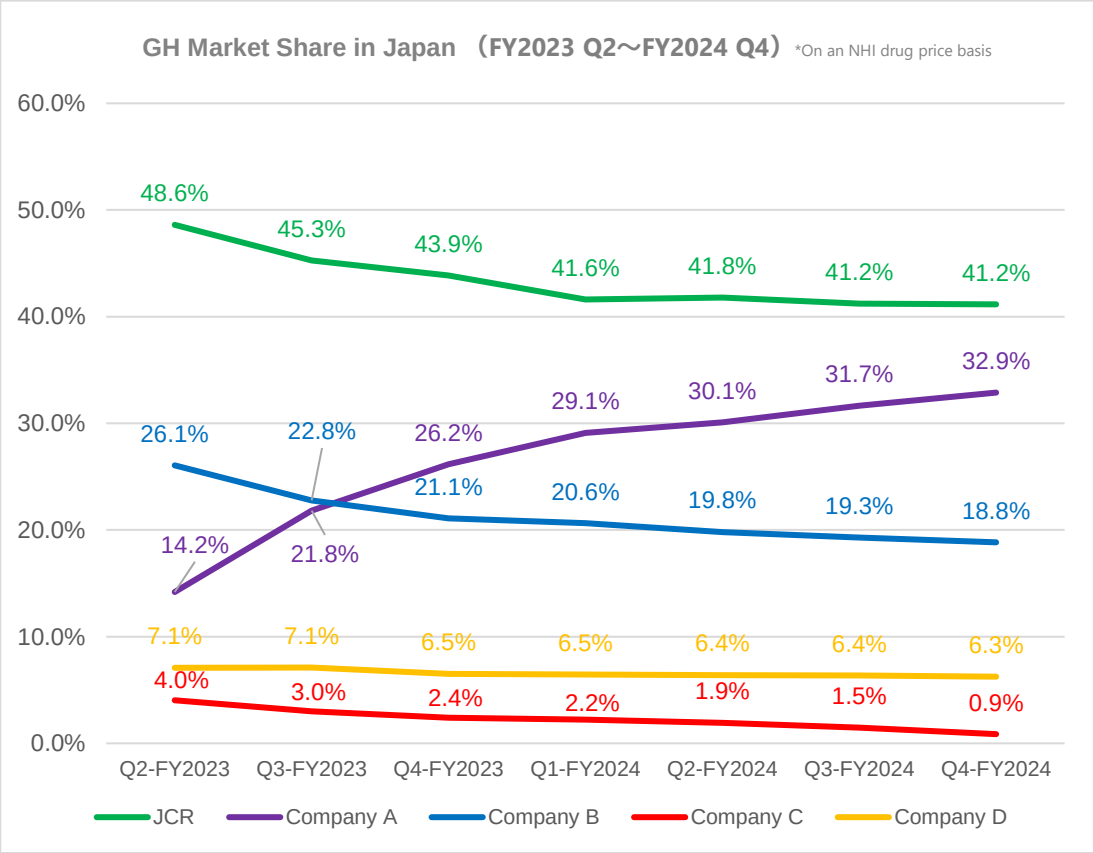
JCR Pharmaceuticals

Appendix

GROWJECT® Market Share Trends in Japan (Quarterly)



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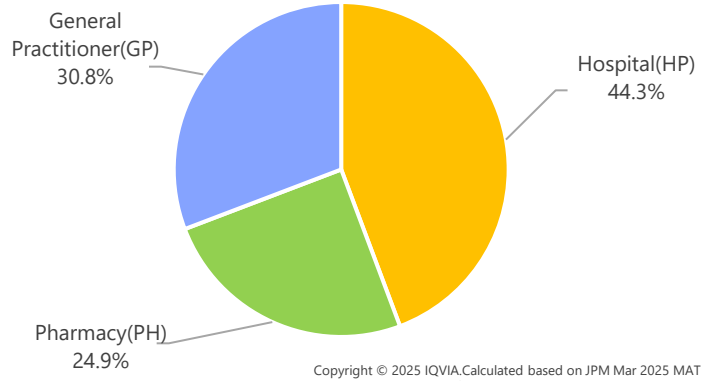
■ Human Growth Hormone Market in Japan



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■ GROWJECT® Market Share by buyer

	Mar 2025	Sales Change FY2024 Q4 (vs. FY2023 Q4) *On an NHI drug price basis
HP Market	33.0%	-166 million yen
PH Market	29.0%	-66 million yen
GP Market	61.6%	-1 million yen

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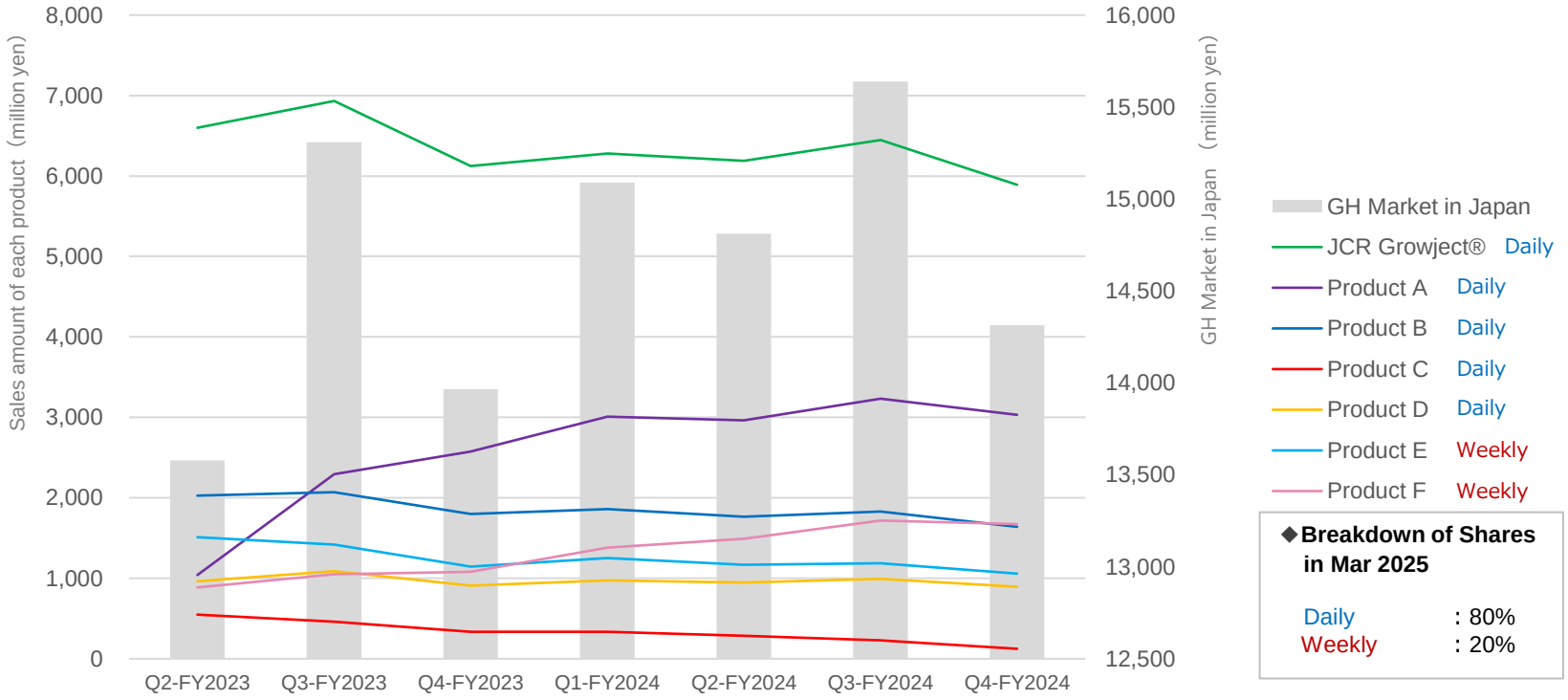
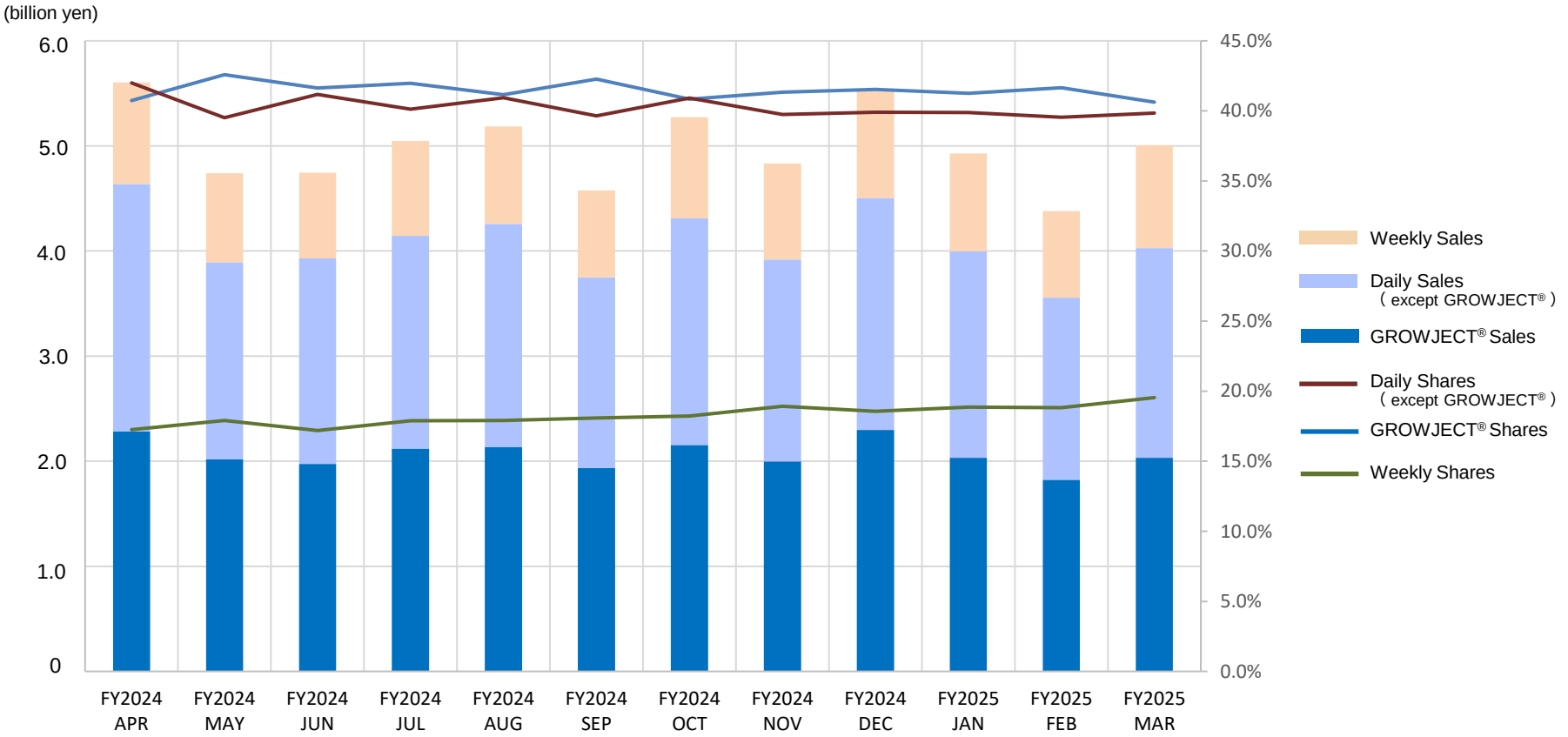
HP: Hospital
PH: Pharmacy
GP: General Practitioner

GROWJECT® Sales Trends in Japan (Quarterly)

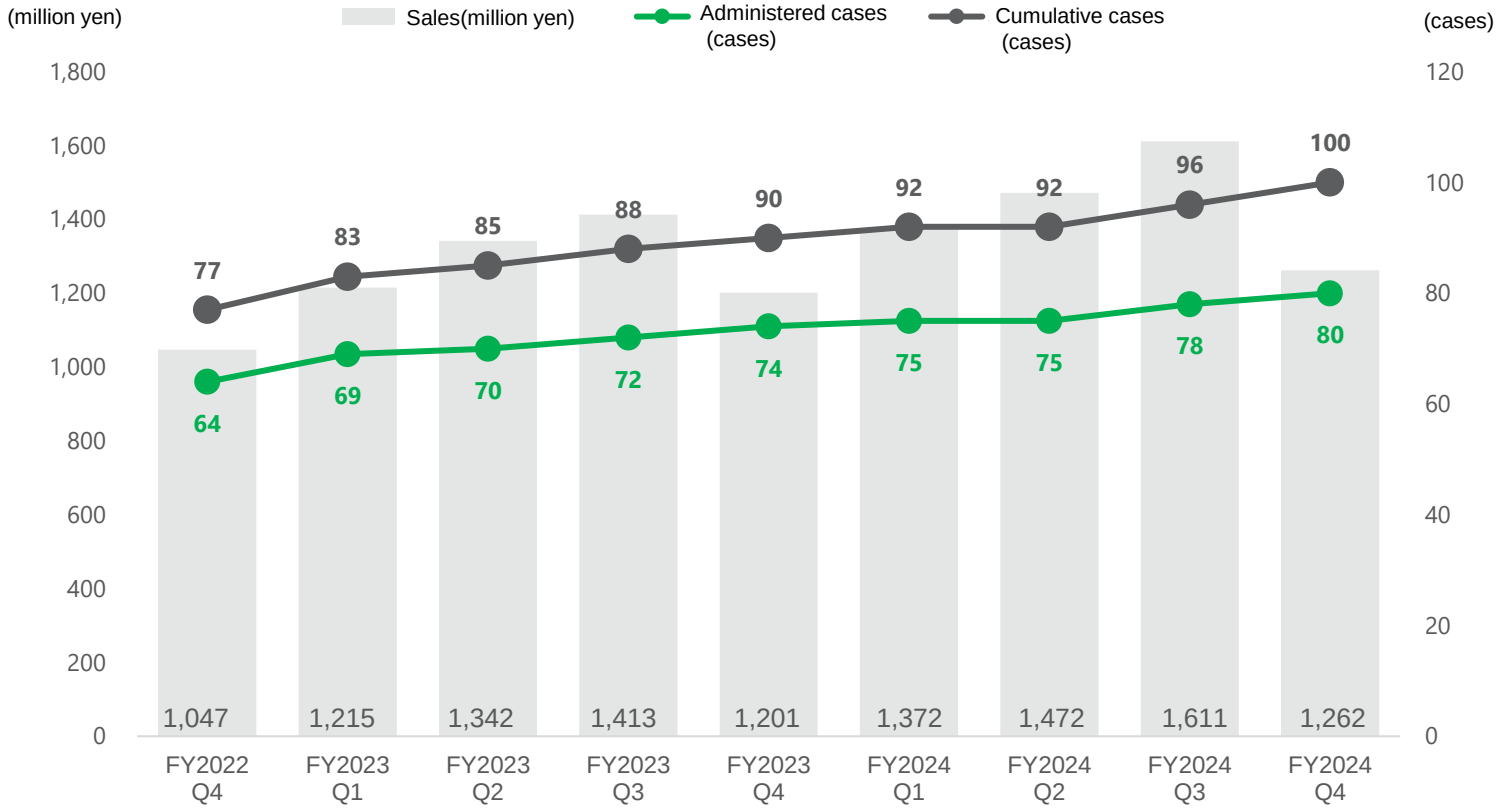
GH Sales Trends in Japan（FY2023 Q2～FY2024 Q4）*On an NHI drug price basis



GROWJECT® Breakdown of Market Share in Japan



IZCARGO® Prescription Status in Japan



Net Sales Trends by Product FY2025 (Forecast)

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(Unit: million yen)

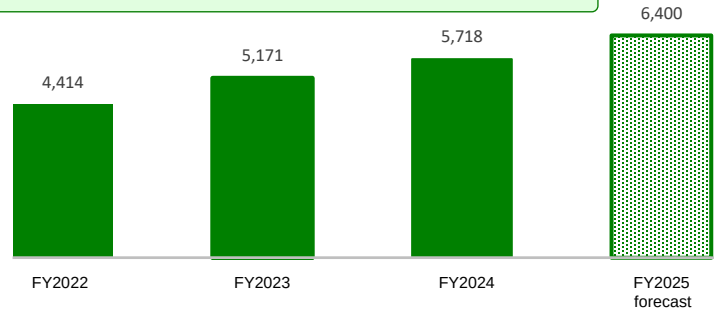
Recombinant human growth hormone product

GROWJECT®

Recombinant treatment for mucopolysaccharidosis II (MPS II)

IZCARGO® I.V. infusion 10mg

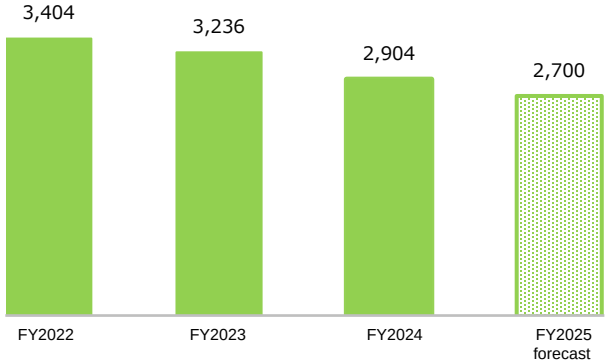
From April 24, 2023: Co-promotion with Sumitomo Pharma Co., Ltd.



*Excluding sales of IZCARGO® related to NPS

Human somatic stem cell-processed products
Human (allogenic) bone marrow-derived mesenchymal stem cells

TEMCELL® HS Inj.



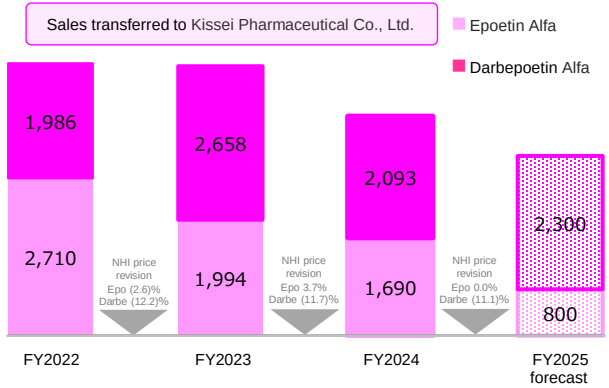
Recombinant erythropoietin product

Epoetin Alfa BS Inj. [JCR]

Long-acting erythropoiesis-stimulating agent

Darbepoetin Alfa BS Inj. [JCR]

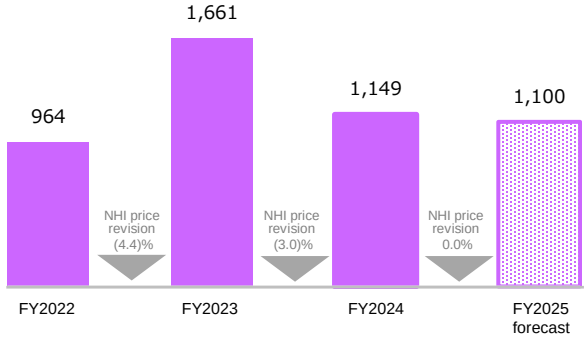
Sales transferred to Kissei Pharmaceutical Co., Ltd.



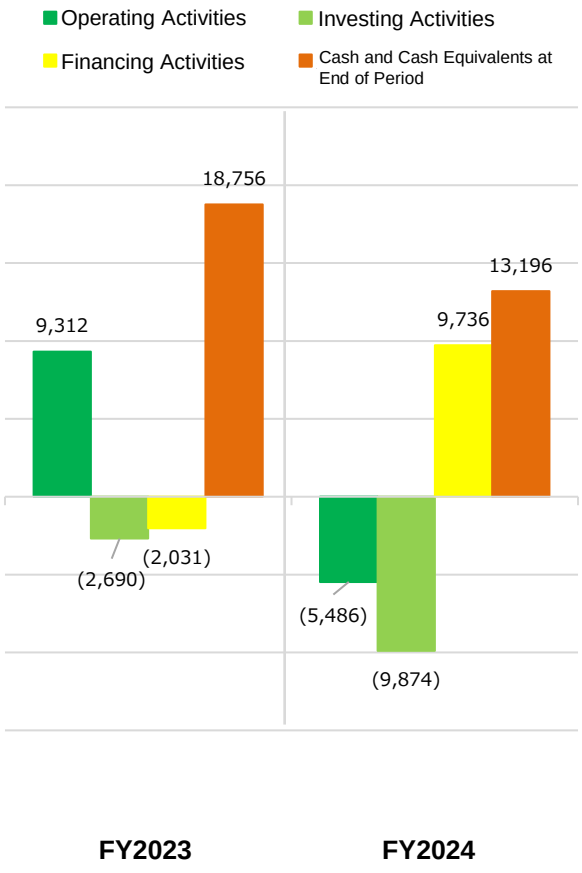
Recombinant treatment for Fabry disease

Agalsidase Beta BS I.V. Infusion [JCR]

Sales transferred to Sumitomo Pharma Co., Ltd.



Cash Flows (Consolidated)



(Unit: million yen)

	FY2023	FY2024	Difference
Profit before income taxes	7,244	**(6,414)**	(13,658)
Depreciation	3,197	**3,374**	+176
Decrease (increase) in trade receivables and accounts receivable – other	(3,390)	**2,453**	+5,844
Decrease (increase) in inventories	(2,437)	**(822)**	+1,615
Increase (decrease) in trade payables and accounts payable – other	(281)	**(36)**	+244
Income taxes paid	1,982	**(2,284)**	(4,266)
Other	2,998	**(1,755)**	(4,754)
Operating Activities	9,312	**(5,486)**	(14,798)
Capital investment(property,plant and equipment)	(2,096)	**(9,888)**	(7,791)
Other	(594)	**13**	+607
Investing Activities	(2,690)	**(9,874)**	(7,184)
Bollowings	500	**14,805**	+14,305
Dividends paid/ treasury shares	(2,485)	**(5,014)**	(2,529)
Other	(46)	**(54)**	(7)
Financing Activities	(2,031)	**9,736**	+11,768
Net increase (decrease) in cash and cash equivalents	5,477	**(5,559)**	(11,036)
Cash and Cash Equivalents at End of Period	18,756	**13,196**	(5,559)

	FY2023	FY2024
Depreciation	3,197	**3,374**
Capital investment	2,096	**9,888***

*This amount includes the amount eligible for subsidy.

Abbreviations (A～G)

AAV	Adeno-Associated Virus	アデノ随伴ウイルス
API	Active Pharmaceutical Ingredient	原薬
ASO	Antisense oligonucleotides	アンチセンス核酸
BBB	Blood-Brain Barrier	血液脳関門
CNS	Central Nervous System	中枢神経系
CSF	Cerebrospinal fluid	脳脊髄液
CTN	Clinical Trial Notification	治験計画届
EC	European Commission	欧州委員会
EMA	European Medicines Agency	欧州医薬品庁
ERT	Enzyme Replacement Therapy	酵素補充療法
EU	European Union	欧州連合
FDA	Food and Drug Administraion	米国食品医薬品局
GHD	Growth Hormone Deficiency	成長ホルモン分泌不全性低身長症

Abbreviations (H～Z)

HS	Heparan Sulfate	ヘパラン硫酸
i.v.	Intravenous Injection	静脈注射
JBC	J-Brain Cargo®	-
LNP	Lipid nanoparticle	脂質ナノ粒子
MPS	Mucopolysaccharidosis	ムコ多糖症
NPS	Named Patient Supply	特定の患者への医薬品提供プログラム
ODD	Orphan Drug Designation	希少疾病用医薬品指定
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
PRIME	Priority Medicines	アンメットメディカルニーズを対象とした医薬品の開発支援を強化するためのスキーム
R&D	Research and Development	研究開発
siRNA	small interfering RNA	短鎖干渉RNA
TBD	To be determined	未定

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.