Reach Beyond, Together

FY2024 Second Quarter Results Briefing Session

October 30, 2024

JCR Pharmaceuticals Co., Ltd.

[Securities code]4552, Prime. TSE

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Reach Beyond, Together

FY2024 Second Quarter Financial Results

Yoh Ito
Senior Executive Officer
Executive Director, Corporate Strategy Division

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Overview: Consolidated Financial Results

(Unit：million yen)

Consolidated	FY2023	FY2024			
	Q2 YTD	Q2 YTD	Year-on-year		Progress Rate
			Difference	Ratio	
Net Sales	24,272	**16,657**	(7,615)	(31.4)%	40.3%
Cost of Sales	5,881	**4,330**	(1,550)	(26.4)%	41.6%
Gross Profit	18,391	**12,326**	(6,064)	(33.0)%	39.9%
Selling, General and Administrative Expenses	11,493	**13,066**	+1,573	+13.7%	51.2%
SG&A Expenses	5,957	**6,489**	+532	+8.9%	51.9%
R&D Expenses	5,535	**6,576**	+1,040	+18.8%	50.6%
Operating Profit	6,898	**(739)**	(7,637)	-	-
Non-operating Income	796	**134**	(662)	(83.1)%	-
Non-operating Expenses	568	**1,016**	+447	+78.7%	-
Ordinary Profit	7,126	**(1,621)**	(8,747)	-	-
Extraordinary Income	-	**1,065**	+1,065	-	-
Extraordinary Losses	5	**0**	(5)	(96.5)%	-
Profit before Income Taxes	7,120	**(556)**	(7,676)	-	-
Income Taxes	1,867	**134**	(1,732)	(92.8)%	9.0%
Profit Attributable to Owners of Parent	5,253	**(691)**	(5,944)	-	-
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	6,273	**7,314**	+1,040	+16.6%	48.6%

Additional Remarks

- Revenue and profit decreased year-on-year, primarily due to a reduction in contract revenue, which is expected to be recorded in the second half of the fiscal year.
- The cost of sales ratio (excluding contract revenue) has continued to decline year-on-year since the first quarter.
- The increase in R&D expenses was mainly due to the establishment of overseas development structures and the advancement of clinical trials.
- Non-operating expenses included losses from equity method investments, depreciation, and foreign exchange losses.
- Special gains included income from the cancellation of stock options and Gain on reversal of share acquisition rights.

(Unit: number of people)

	As of September 30, 2023	As ofSeptember 30, 2024	Difference
Number of Employees (Consolidated)	932	**997**	+65

Net Sales	FY2023 Q2 YTD	FY2024 Q2 YTD	Ratio
Cost of Sales Ratio	24.2%	**26.0%**	+1.8%
Cost of Sales Ratio *Excluding income from contractual payment	34.3%	**26.0%**	(8.3)%
R&D Expenses Ratio	22.8%	**39.5%**	+16.7%
Operating Profit Ratio	28.4%	**(4.4%)**	(32.9)%

YTD: year to date

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Breakdown of Net Sales (Consolidated)

(Unit: million yen)

Consolidated	FY2023	FY2024			
	Q2 YTD	Q2 YTD	Year-on-year		Progress Rate
			Difference	Ratio	
GROWJECT®	8,746	**9,401**	+654	+7.5%	51.4%
IZCARGO®	2,556	**2,845**	+288	+11.3%	49.9%
TEMCELL®HS Inj.	1,901	**1,521**	(380)	(20.0)%	54.3%
Treatments for renal anemia	2,674	**1,764**	(910)	(34.0)%	42.0%
Epoetin Alfa BS Inj. [JCR]	1,046	**962**	(83)	(8.0)%	43.8%
Darbepoetin Alfa BS Inj. [JCR]	1,628	**801**	(826)	(50.8)%	40.1%
Agalsidase Beta BS I.V. Infusion [JCR]	590	**714**	+123	+21.0%	64.9%
Total Core Products	16,470	**16,246**	(224)	(1.4)%	50.6%
Income from contractual payment	7,112	**15**	(7,096)	(99.8)%	0.2%
Other	689	**395**	(294)	(42.7)%	35.9%
Total Net Sales	24,272	**16,657**	(7,615)	(31.4)%	40.3%

*The figures for Q2 of FY2023 were revised from the figures announced at the time of publication previous year since the sales generated by the NPS program in IZCARGO® have been reclassified as Other.

Additional Remarks

- GROWJECT® underwent a (4.9%) reduction in reimbursement prices in April, but increased sales volume led to a 7.5% revenue growth year-on-year.
- IZCARGO® performed well, with an 11.3% revenue increase compared to the previous year.
- TEMCELL®HS Inj. revenue fell by 20.0% year-on-year due to competitive market conditions but achieved 54.3% of the full-year revenue target.
- Sales for the renal anemia treatment are in line with the supply plan for Kissei Pharmaceutical Co., Ltd.
- Agalsidase Beta BS I.V. Infusion [JCR] sales are consistent with the supply plan for Sumitomo Pharma Co., Ltd.
- Contract revenue is projected to be higher in the second half of the fiscal year.
- Other revenue declines are attributed to lower outsourced manufacturing sales.

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YTD: year to date

Financial Status (Consolidated)

(Unit: million yen)

	End-Mar. 2024	End-Sep. 2024	Change • Main Increase/decrease		End-Mar. 2024	End-Sep. 2024	Change • Main Increase/decrease
Current assets	57,581	**56,307**	**Total (1,274)** • Cash and deposits (484) • Accounts receivable - trade, and contract assets (2,579) • Inventories +1,830	**Current liabilities**	30,135	**34,133**	**Total +3,998** • Short-term borrowings +7,312 • Income taxes payable (1,534) • Accrued consumption taxes (1,818)
				Non-current liabilities	15,615	**13,438**	**Total (2,176)** • Long-term borrowings (2,200)
Non-current assets	44,644	**48,314**	**Total +3,670** • Property, plant and equipment +1,713 • Investment securities +2,963	**Total liabilities**	45,750	**47,572**	**Total +1,822**
				Total net assets	56,475	**57,049**	**Total +573** • Valuation difference on available-for-sale securities +1,863 • Share acquisition rights (737)
Total	102,226	**104,622**	**2,396**	**Total**	102,226	**104,622**	**2,396**

	End-Mar. 2024	End-Sep. 2024
Equity ratio	54.2%	**54.1%**

Additional Remarks

・The increase in Non-current assets is largely attributed to ongoing construction for the new formulation plant at Kobe Science Park Center and increased investments in securities.

・Current liabilities grew mainly due to an increase in short-term borrowing related to construction and other initiatives at Kobe Science Park Center.

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Progress of Developmental Pipelines

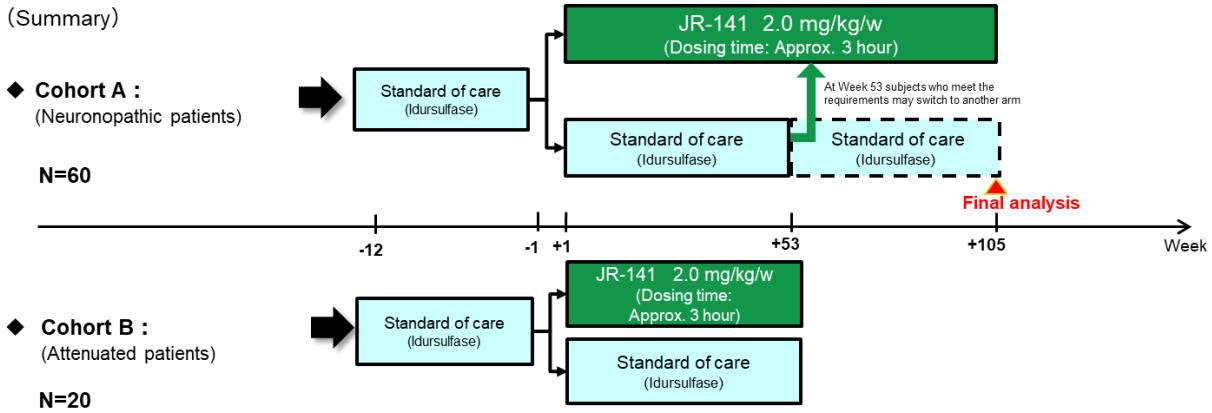
Anne Bechet
Senior Executive Officer
Executive Director, Development Division
General Manager, JCR Europe B.V.
General Manager, JCR USA Inc.

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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

- Recruiting
- Number of Clinical trial sites
  (As of October 30, 2024)
  24 sites
  11 countries
  (USA, Europe, and Latin America)

- **The trial site opened as planned**
- **Enrollment in Cohort A on track**

(Cohort B has been fully enrolled)

- **Close collaboration with agencies to target to file as soon as possible**

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Progress of Developmental Pipelines

JR-441

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

- **Japan**: Phase I study (JR-441-JP11)
  - Completed the clinical trial notification process in Japan
- **Germany**: Phase I/II study (JR-441-101)
  - Progressing as planned
  - Enrollment of the target number of 12 patients completed

JR-446

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

- **Japan**: Phase I/II study (JR-446-101)
  - Completed the clinical trial notification process in Japan
  - First patient in is expected in 2nd half of FY2024

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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				• ~FY2027: Approval in US, EU, Brazil
JR-171	MPS I (Hurler syndrome etc.)	Global Ph1/2 completed				• Extension study ongoing • Partnering intensified
JR-142	Pediatric GHD	Ph3 (Japan)			CTN process completion	• Initiation of Phase 3 in FY2024
JR-441	MPS IIIA (Sanfilippo syndrome type A)	Ph1/2 (Germany) Ph1 (Japan)			CTN process completion	<Ph1/2> • Patient enrollment completed • 2nd Half FY2025: 1-year clinical data
JR-446	MPS IIIB (Sanfilippo syndrome type B)				CTN process completion	• 2nd Half FY2024: FPI in Phase 1/2
JR-471	Fucosidosis					—

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J-Brain Cargo® Applicability to Multiple Modalities ~Application to Gene Therapy~

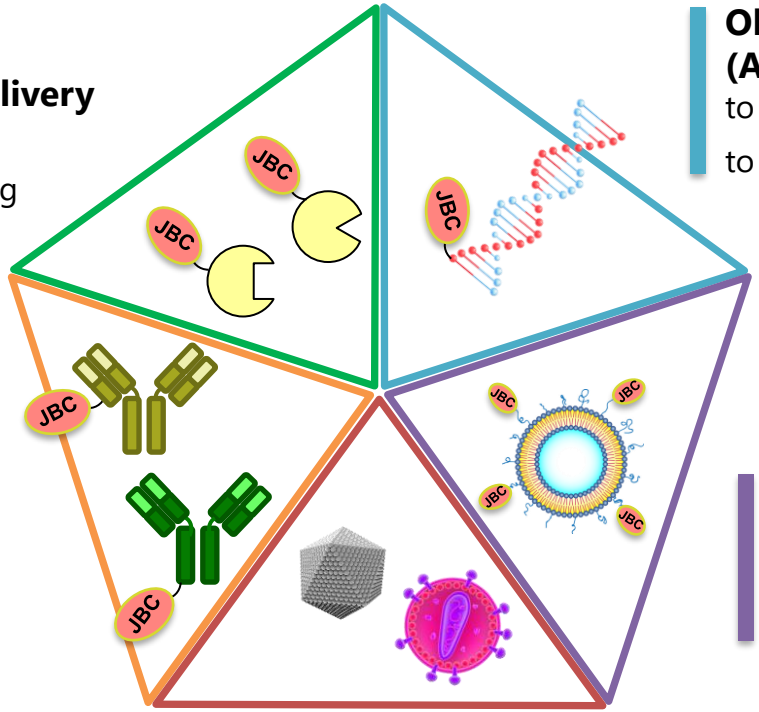
Hiroyuki Sonoda, Ph. D.
Director, Senior Managing Executive Officer
Executive Director, Research Division

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J-Brain Cargo® Applicability

Enzymes and **Protein Delivery**
to the brain and muscle

to replace deficient or missing enzymes and proteins



Oligonucleotide (ASO, siRNA) Delivery
to the brain and muscle

to modify gene expression

Antibody Delivery
to the brain

in various formats (bi-specific, tri-specific)

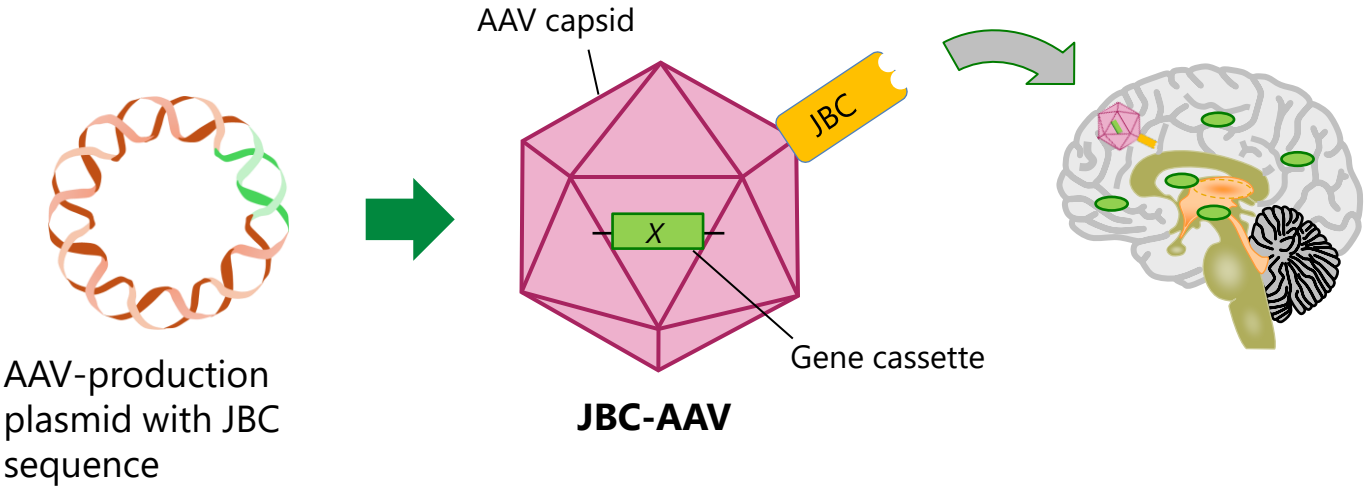
LNP (mRNA, low-molecular compound) Delivery
to the brain and muscle

to replace deficient or missing proteins

Gene/Cell therapy
combined with J-Brain Cargo® technology to pave the way towards functional cure of CNS pathologies

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Applying J-Brain Cargo® to Gene Therapy



- JCR-proprietary gene therapy technology
- J-Brain Cargo® domain embedded in AAV production plasmid for viral delivery into the CNS

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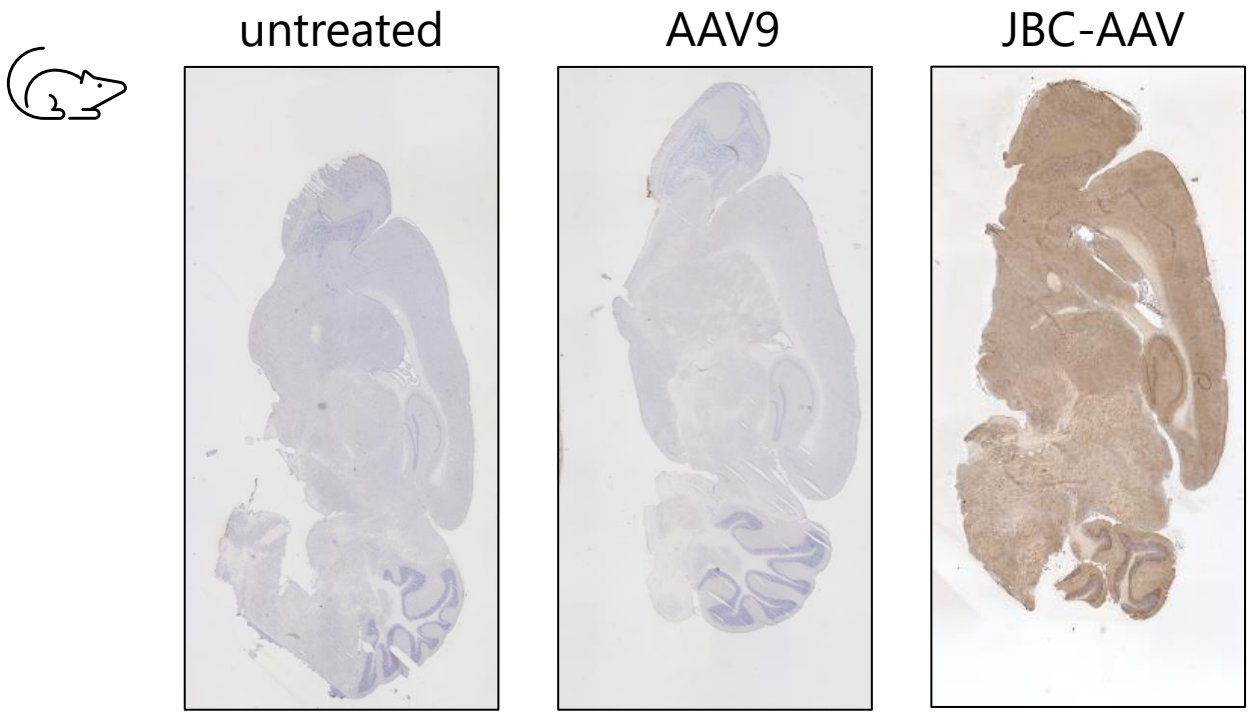
Advantages of J-Brain Cargo® Applied Gene Therapy

- Novel mechanism with superior brain uptake compared with conventional AAV9
- Same J-Brain Cargo® technology led to approval of pabinafusp alfa in Japan
- No added manufacturing complexity, stable production yields
  - JBC tags are included in the AAV capsid (and not conjugated post capsid production)
- Versatility to fine-tune affinity and uptake
  - Designing the optimal JBC-AAV capsid for each therapeutic target

Continuously advancing the J-Brain Cargo® technology sets up JCR for a brilliant future

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Non-clinical Data: Evaluation of Brain in Mice by Immunohistochemical Staining



Inserting J-Brain Cargo® tags into AAVs enables efficient gene delivery to the brain

The 7th International Forum of Lysosomal Disorders, 2024

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Shaping the Future with our own Technology



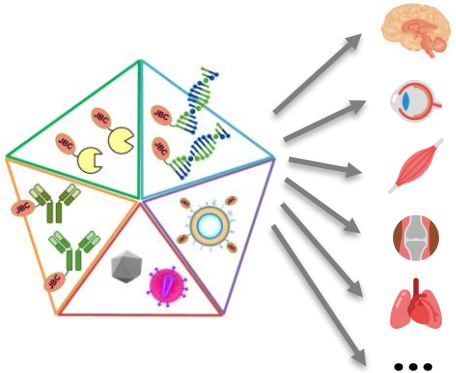
- Expansion of partnering opportunities
- Increasing R&D collaborations

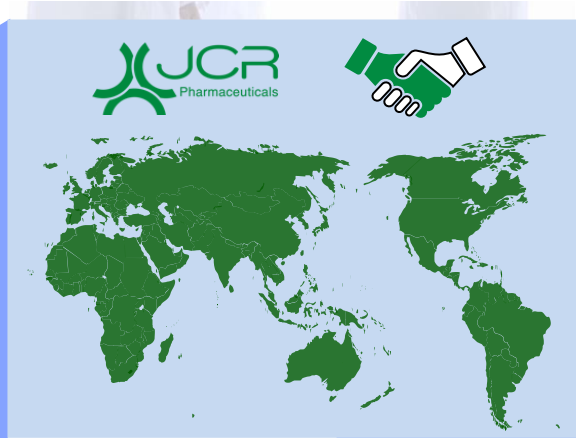


Creation of breakthrough therapeutics in various disease areas

- Lysosomal Storage Diseases
- Neurodegenerative Diseases
- Ocular Diseases
- Orthopedic Diseases
- Muscular Diseases

Widely applicable technology based on J-Brain Cargo®





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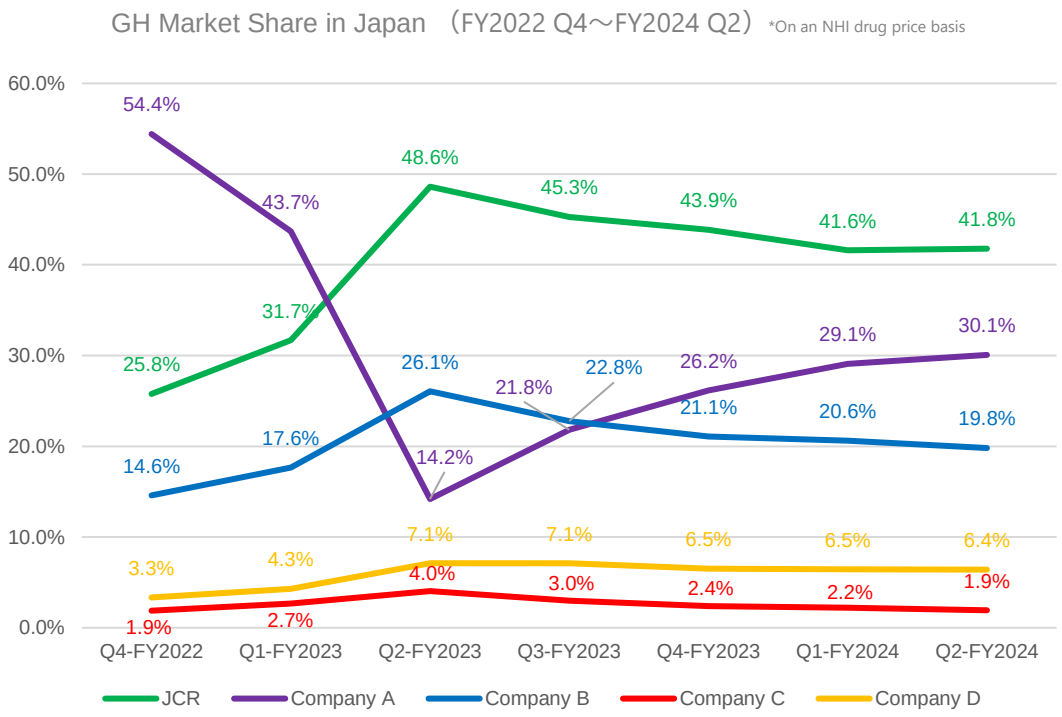


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Appendix

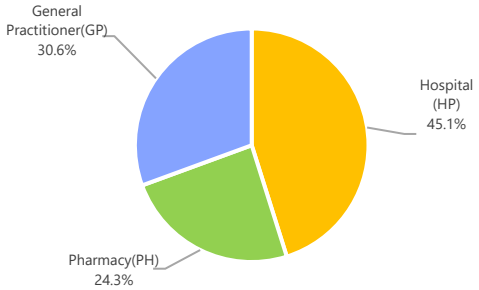
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GROWJECT® Market Share Trends in Japan (Quarterly)



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■ Human Growth Hormone Market in Japan



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*Market definition by JCR Reprinted with permission

■ GROWJECT® Market Share by buyer

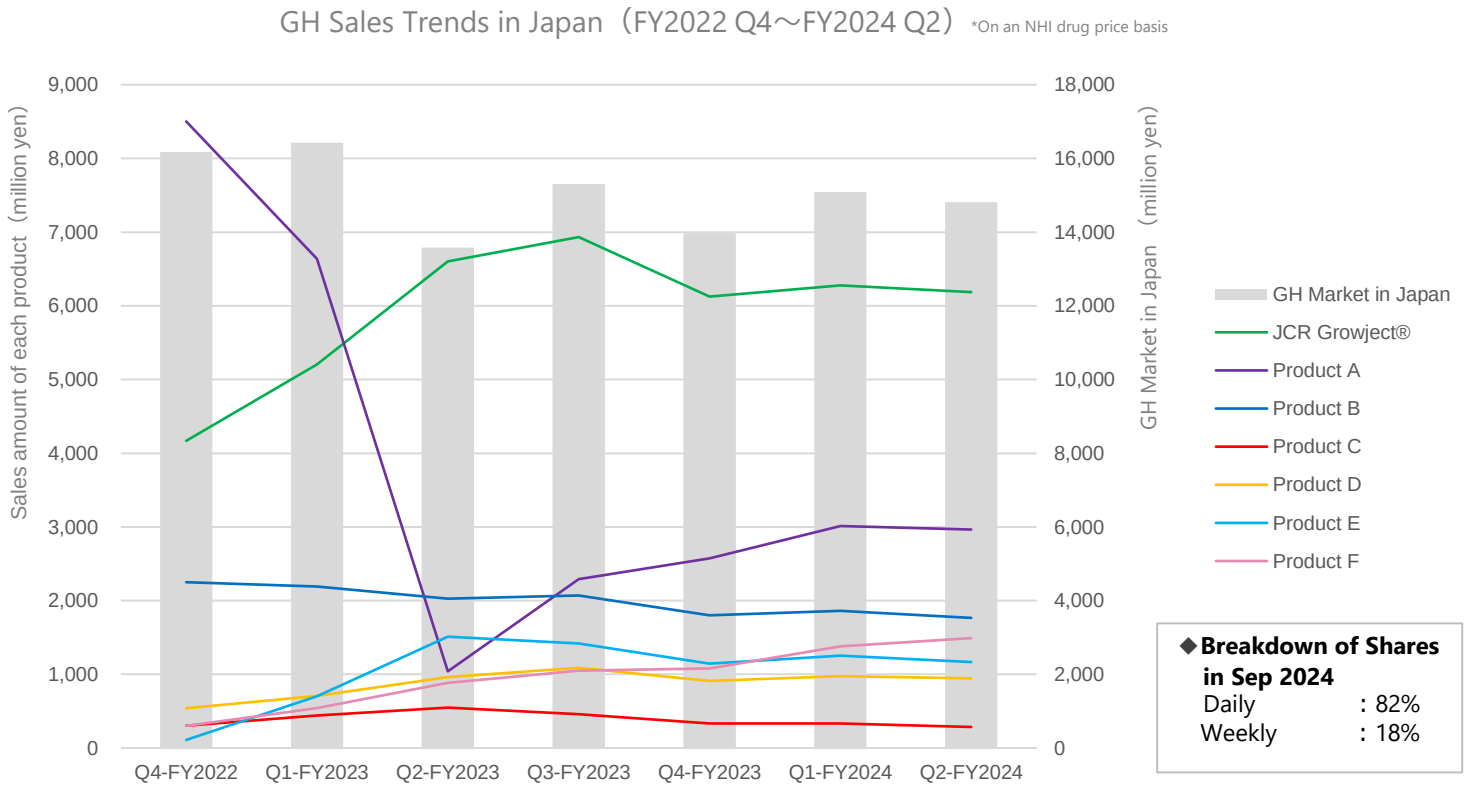
	Sep 2024	Sales Change FY2024 Q2 (vs. FY2023 Q2) *On an NHI drug price basis
HP Market	33.5%	-329 million yen
PH Market	30.7%	-128 million yen
GP Market	62.5%	+44 million yen

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Market definition by JCR Reprinted with permission

HP: Hospital
GP: General Practitioner
PH: Pharmacy

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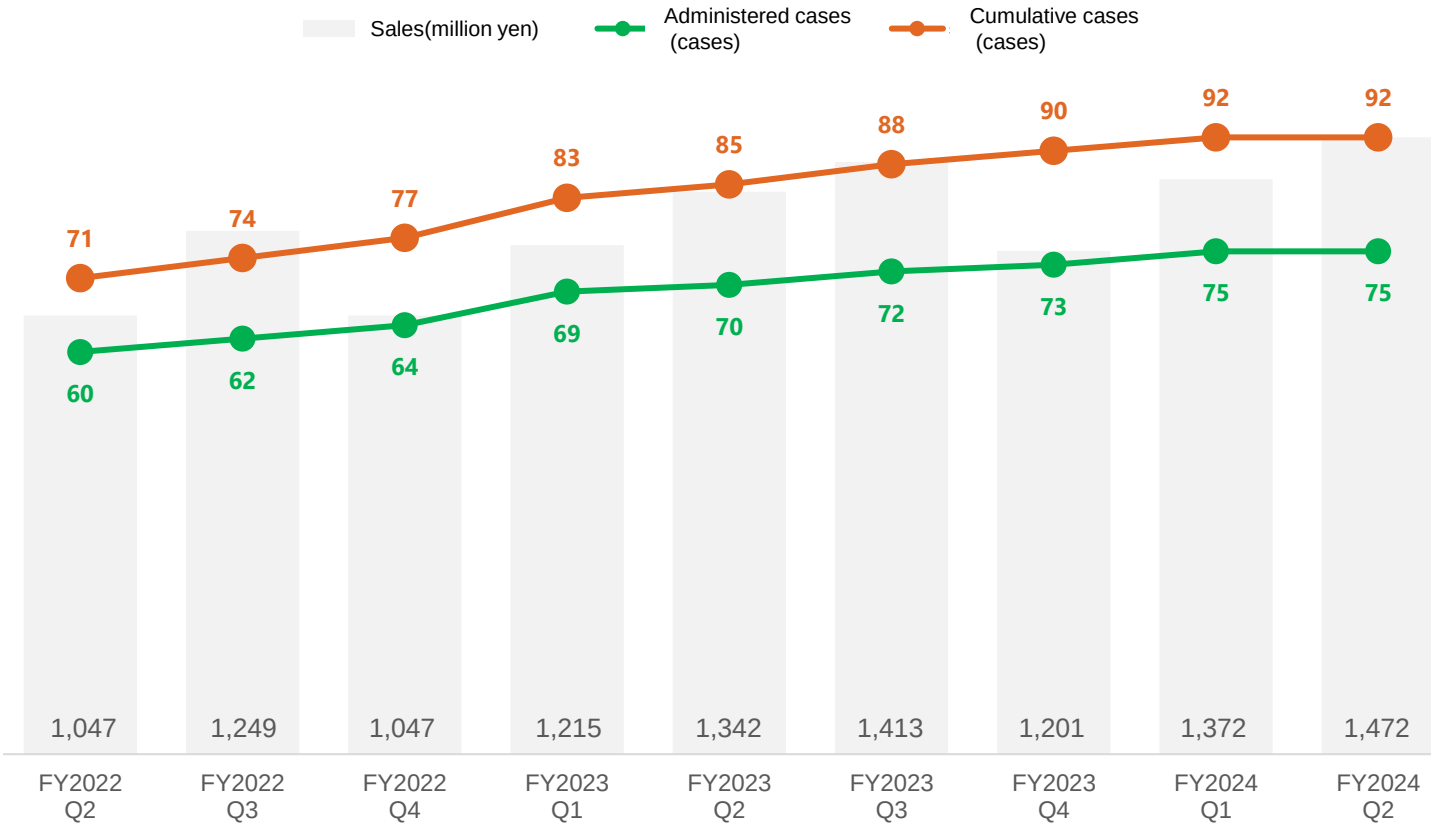
GROWJECT® Sales Trends in Japan (Quarterly)



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IZCARGO® Prescription Status in Japan

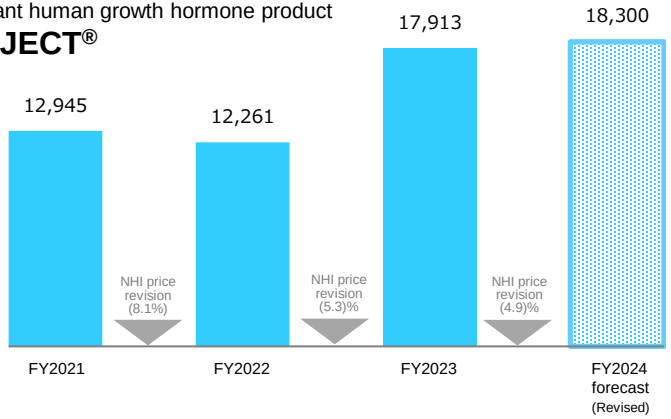


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Net Sales Trends by Product

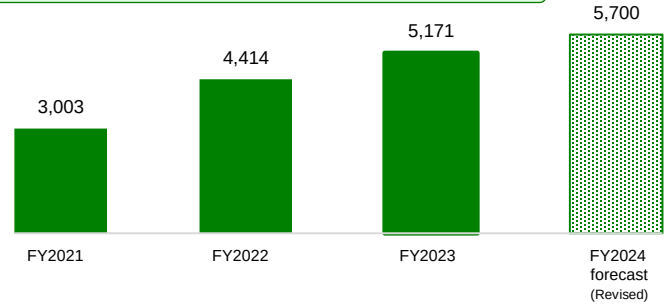
(Unit: million yen)

Recombinant human growth hormone product
GROWJECT®

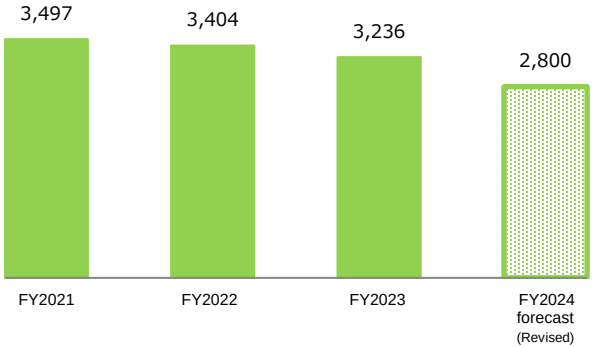


Recombinant treatment for mucopolysaccharidosis II (MPS II)
IZCARGO® I.V. infusion 10mg

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

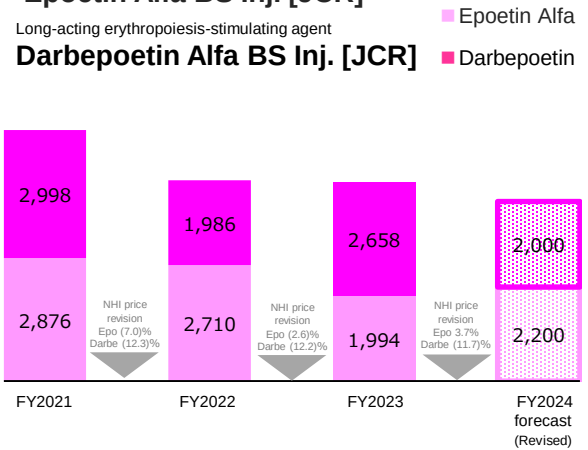


Human somatic stem cell-processed products
Human (allogenic) bone marrow-derived mesenchymal stem cells
TEMCELL® HS Inj.



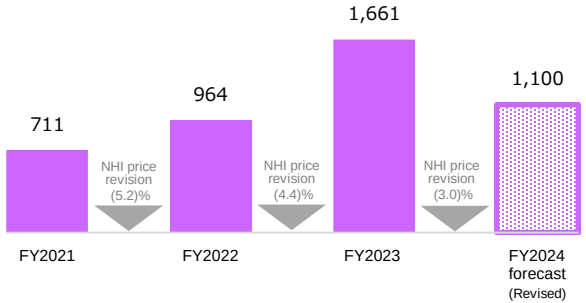
Recombinant erythropoietin product
Epoetin Alfa BS Inj. [JCR]

Long-acting erythropoiesis-stimulating agent
Darbepoetin Alfa BS Inj. [JCR]



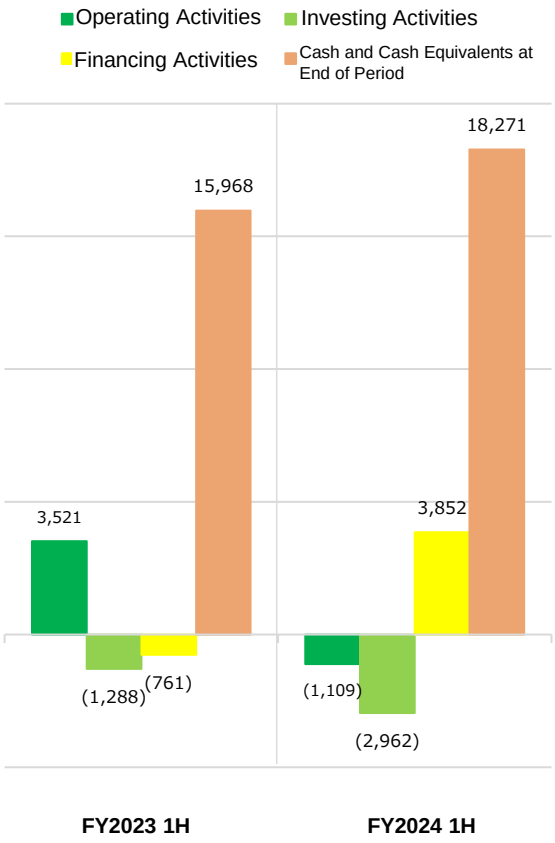
Recombinant treatment for Fabry disease
Agalsidase Beta BS I.V. Infusion [JCR]

Apr. 2022: Sales transferred to Sumitomo Pharma



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Cash Flows (Consolidated)



(Unit: million yen)	FY2023 1H	**FY2024 1H**	Difference
Profit before income taxes	7,120	**(556)**	(7,676)
Depreciation	1,335	**1,667**	+331
Decrease (increase) in trade receivables and accounts receivable – other	(8,643)	**2,649**	+11,292
Decrease (increase) in inventories	(757)	**(1,830)**	(1,072)
Increase (decrease) in trade payables and accounts payable – other	373	**(190)**	(564)
Income taxes paid	2,228	**(1,451)**	(3,680)
Other	1,864	**(1,396)**	(3,261)
Operating Activities	3,521	**(1,109)**	(4,630)
Capital investment(property,plant and equipment)	(838)	**(2,992)**	(2,154)
Other	(449)	**30**	+479
Investing Activities	(1,288)	**(2,962)**	(1,674)
Bollowings	500	**5,112**	+4,612
Dividends paid/ treasury shares	(1,238)	**(1,233)**	+5
Other	(22)	**(26)**	(4)
Financing Activities	(761)	**3,852**	+4,613
Net increase (decrease) in cash and cash equivalents	2,689	**(484)**	(3,174)
Cash and Cash Equivalents at End of Period	15,968	**18,271**	+2,302

	FY2023		**FY2024**	
	1H	Full-year	**1H**	**Full-year (forecast)**
Depreciation	1,335	3,197	**1,667**	**3,100**
Capital investment (after subsidy deduction)	838	2,096	**2,992**	**11,100**

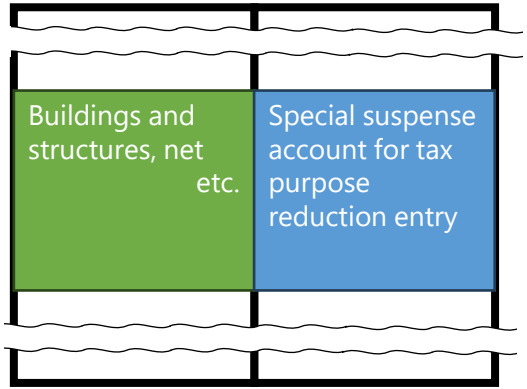
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Handling of subsidies for the construction of the Kobe Science Park Center

- Of the depreciation at Kobe Science Park Center, approximately 300 million yen was recorded as SG&A expenses and approximately 100 million yen was recorded as Non-operating expenses.

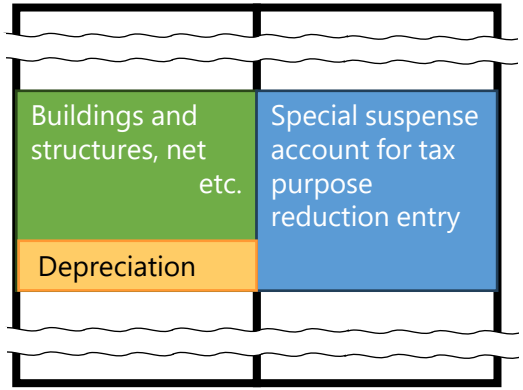
Transfer of items from "construction in progress"

- The portion subject to subsidy was transferred from the "construction in progress" account to the property, plant and equipment account.



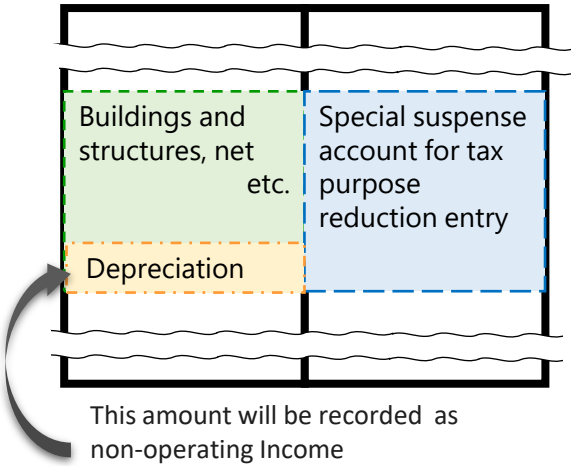
As of March 31, 2024

- Depreciation (approx. 600 million yen) recorded with start of operations in Q2 FY2023



As of March 31, 2025 (scheduled)

- The portion subject to subsidy will be offset against the "Special suspense account for tax purpose reduction entry" (by advanced depreciation)
- The depreciation will be recorded as non-operating income.



*Only the portion of the subsidy that is included in the balance sheet is shown.

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略語集（A～G）

AAV	Adeno-Associated Virus	アデノ随伴ウイルス
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

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略語集（H～Z）

HIE	Hypoxic ischemic encephalopathy in neonates	低酸素性虚血性脳症
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

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