Reach Beyond, Together

FY2023 Results Briefing Session

May 13th, 2024
JCR Pharmaceuticals Co., Ltd.

[Securities code]4552, Prime. TSE

Key Messages

FY2023
Revenue and earnings growth from increasing sales of core products

FY2024
Strengthen organizational structure to support global clinical development and increase R&D investment

- Expand clinical development team and establish an integrated structure of experts in Japan, US, Europe and Brazil with JCR Europe playing a lead role
- JR-141：Complete patient enrollment for an interim analysis of the global Phase III
- JR-441：Phase I/II study on track (First patient dosed in Oct 2023)
- JR-446：Initiate Phase I/II study in 1st half of FY2024
- JR-171：Discussions on licensing-out ongoing

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Progress of Developmental Pipelines

Anne Bechet
Development (Acting Officer)
General Manager, JCR Europe B.V.

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

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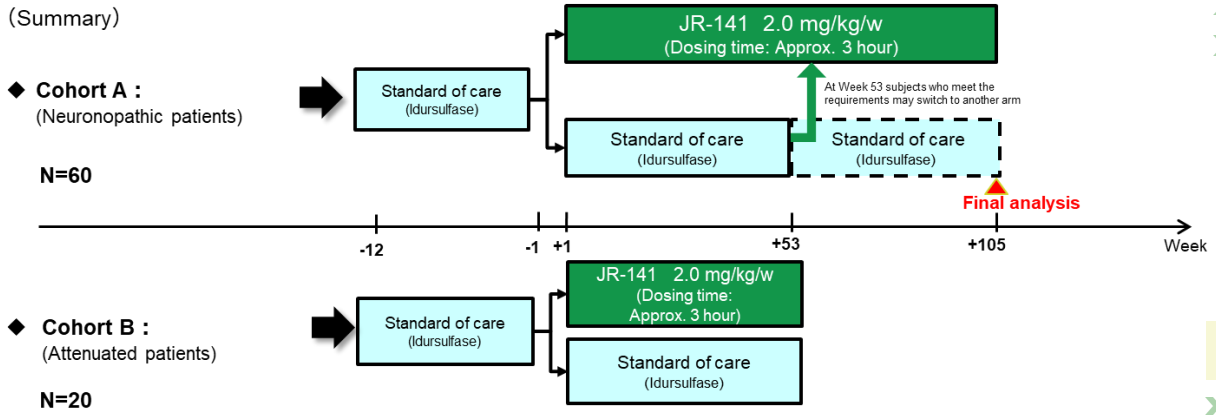
Global Phase III study（JR-141-GS31): STARLIGHT study Overview



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
Clinical Trails.gov	Identifier : NCT04573023

Current Status

- Recruiting
- Number of Clinical trial sites (as of Apr 2024):
  - US: 5
  - Europe: 12
  - Latin America: 4

  ➡Trial is on going in 10 countries
  - Further sites to open in EU, US, Latin America to accelerate recruitment

Achievements

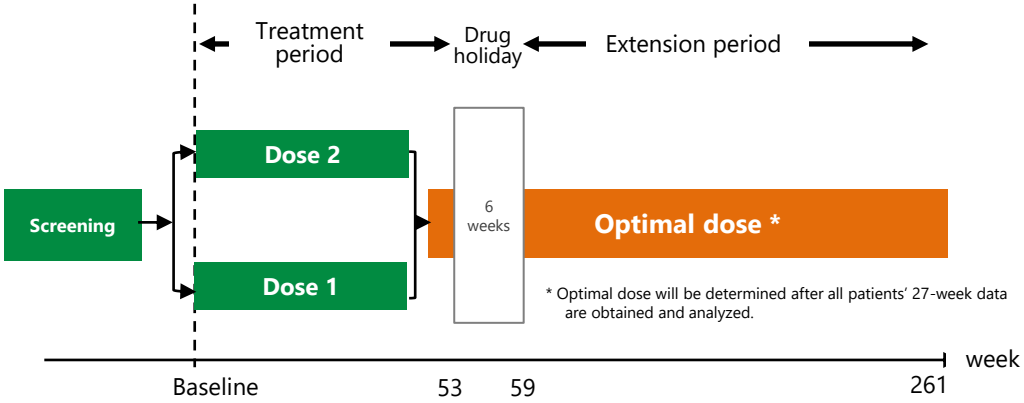
- **Oct -2018** ODD by FDA
- **Feb -2019** ODD by EMA
- **Feb -2021** Fast Track Designation by FDA
- **Oct -2021** PRIME Designation by EMA
- **Feb -2022** First Patient dosed in JR-141-GS31
- **Dec -2022** Rare Pediatric Disease Designation by FDA

- **Completed enrollment of all 20 eligible patients in Cohort B**
- **Enrollment in Cohort A on track**

JR-441 (BBB-penetrating ERT for MPS IIIA)

July 2023 – Started Global Phase I/II study (JR-441-101) in Germany

JR-441-101 study overview



Overview	
Objectives	Safety, dose finding, exploratory efficacy
No. of subjects	12 subjects (≥1 year and ≤18 years)
Clinical Trails.gov	Identifer : NCT06095388

Achievements and next milestones

- **Jan -2022**
  EC grants Orphan Drug Designation
- **Jul -2023**
  Approval of Global Ph I/II Clinical Trial in Germany
- **Oct -2023**
  First Patient First dosed
- **Dec -2023**
  FDA grants Orphan Drug Designation
- **1st Half –FY2024**
  Last Patient In
- **2nd Half –FY2025**
  1-year clinical data is expected

- The Phase I/II study is progressing well
- Started recruiting pediatric patients after approval from the independent data monitoring committee

R&D Pipeline

Code	Indication	Status	Upcoming Milestones
JR-141	MPS II (Hunter syndrome)	Global Ph3	• Q1 FY2024: All patients enrolled necessary for interim analysis • ~FY2027: Approval in US, EU, Brazil
JR-171	MPS I (Hurler syndrome etc.)	Global Ph1/2 completed	• Extension study is ongoing • Licensing out under negotiation
JR-142	Pediatric GHD	Ph2 (Analysis completed)	• FY2024: Phase 3
JR-031HIE	Hypoxic ischemic encephalopathy in neonates	Ph1/2 (Analysis completed)	• TBD
JR-441	MPS IIIA (Sanfilippo syndrome type A)	Global Ph1/2	• 1st Half FY2024: LPI • 2nd Half FY2025: 1-year clinical data is expected
JR-446	MPS IIIB (Sanfilippo syndrome type B)	Pre-clinical	• Under preparation for clinical trial • 1st Half FY2024: FPI in Phase 1/2
JR-471	Fucosidosis	Pre-clinical	• TBD

※Only high-priority projects in the clinical stage or soon to be in the clinical stage are listed in the above table.

Next Wave of Technology Innovation

Hiroyuki Sonoda, Ph. D.
Vice President
Executive Director, Research Division

History of Technological Innovation

Advanced technology and know-how in biopharmaceuticals developed since the company's formation in 1975

Development of proprietary JCR technologies

Stock solutions for COVID-19 Vaccine (AZD1222)

J-Brain Cargo® Tech

Biosimilars

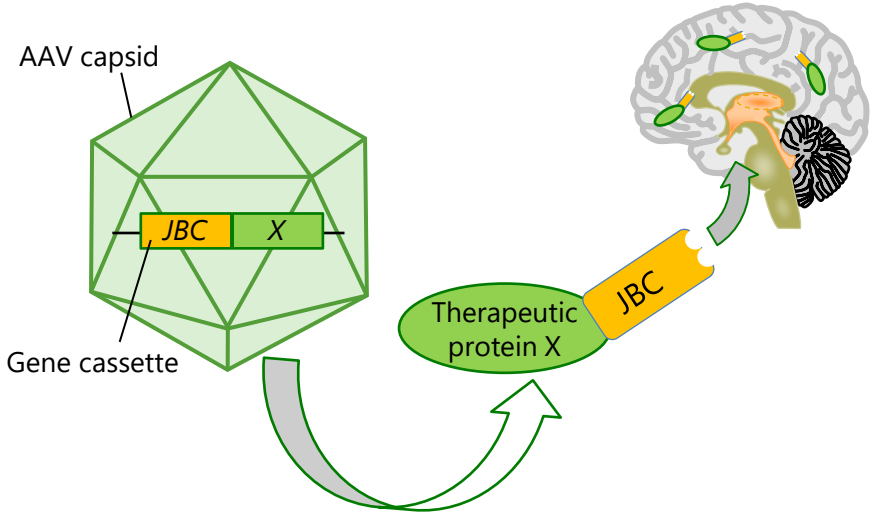
Stem Cell Therapies

Recombinant Proteins

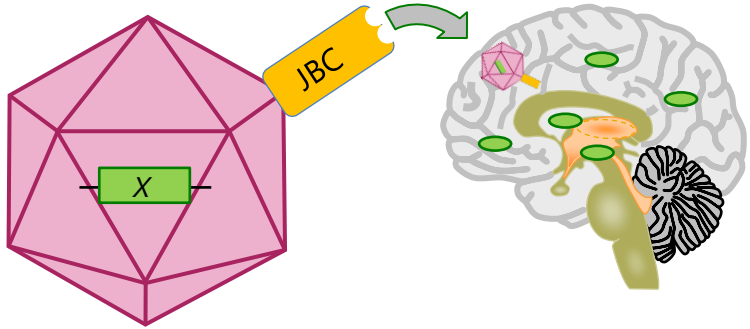
Human-derived therapeutics

Applying J-Brain Cargo® to Gene Therapy

Treatment with AAV expressing JBC-fusion protein



Treatment with modified AAV expressing JBC tag for direct brain penetration

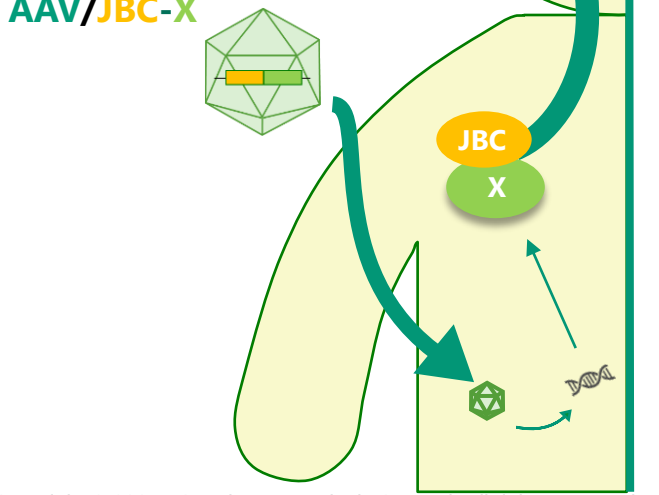


- JCR proprietary gene therapy technology applying J-Brain Cargo®
- Treatment strategies can be tailored depending on the disease characteristics

Characteristics of Gene Therapy used J-Brain Cargo®

Treatment with AAV expressing JBC-fusion protein

- Transgene product, JBC-X, can reach the CNS
- Mechanism of action has been established in enzyme replacement therapy

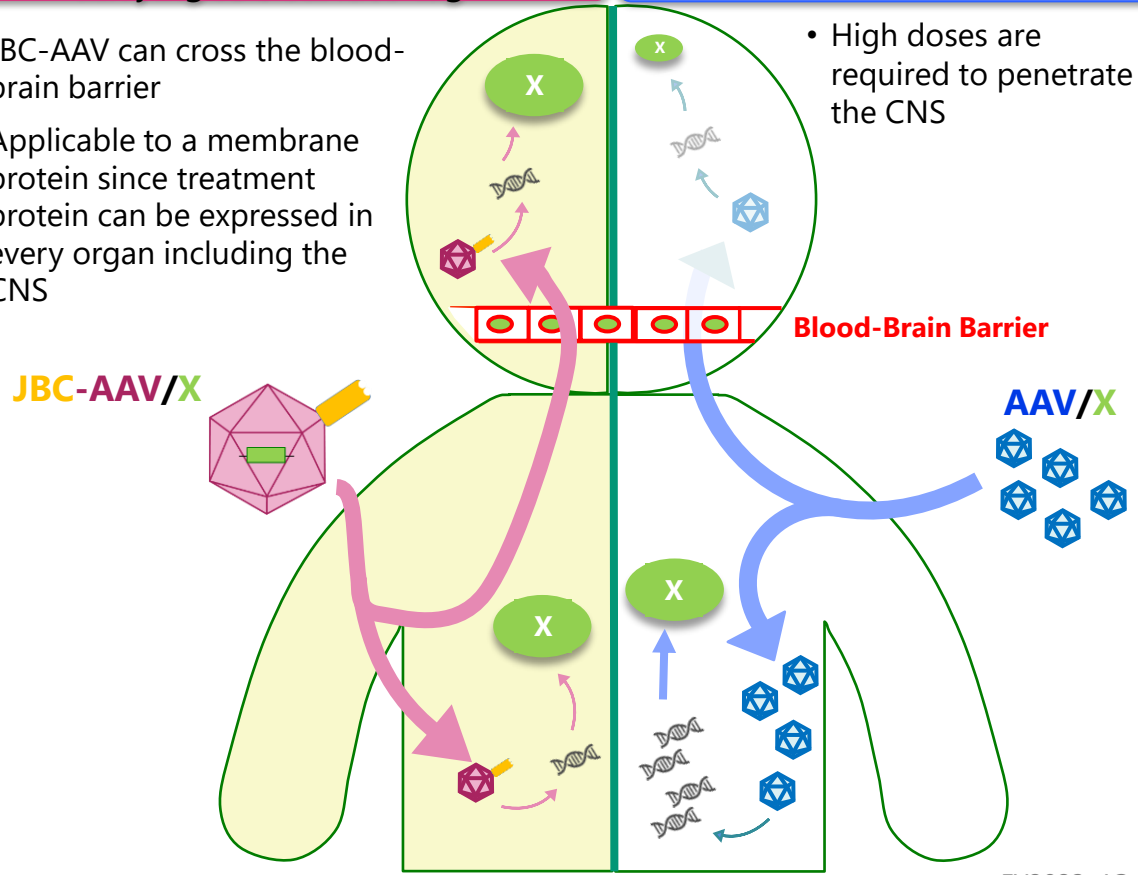


Treatment with AAV carrying JBC surface tag

- JBC-AAV can cross the blood-brain barrier
- Applicable to a membrane protein since treatment protein can be expressed in every organ including the CNS

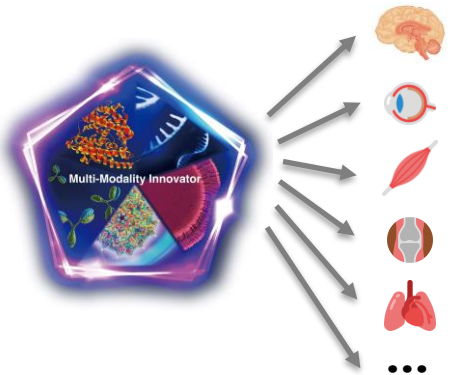
General AAV gene therapy

- High doses are required to penetrate the CNS

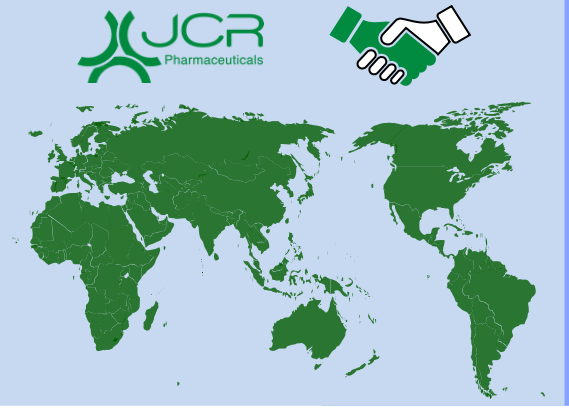


Shaping the Future with our own Technology

Widely applicable technology based on J-Brain Cargo®



- **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*

FY2023 Financial Results

Yoh Ito
Senior Corporate Officer
Executive Director, Corporate Strategy Division

Consolidated Financial Results

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(Unit: million yen)

Consolidated	FY2022	FY2023				
	results	results	Year-on-year		Full-year forecast (Revised)	Progress rate
			Difference	Ratio		
Net sales	34,343	**42,871**	+8,528	+24.8%	45,400	**94.4%**
Cost of sales	8,886	**11,620**	+2,733	+30.8%	12,400	**93.7%**
Gross profit	25,456	**31,251**	+5,794	+22.8%	33,000	**94.7%**
Selling, general and administrative expenses	20,480	**23,719**	+3,238	+15.8%	22,500	**105.4%**
SG&A	11,678	**12,484**	+806	+6.9%	12,800	**97.5%**
R&D expenses	8,802	**11,234**	+2,431	+27.6%	9,700	**115.8%**
Operating profit	4,975	**7,531**	+2,556	+51.4%	10,500	**71.7%**
Ordinary profit	5,418	**7,264**	+1,846	+34.1%	10,000	**72.6%**
Profit attributable to owners of parent	3,772	**5,507**	+1,735	+46.0%	7,300	**75.4%**

Breakdown of Net Sales – Consolidated

(Unit: million yen)

	FY2022	FY2023				
	results	**results**	Year-on-year		Full year forecast (Revised)	**Progress rate**
			Difference	Ratio		
GROWJECT®	12,261	**17,913**	+5,652	+46.1%	19,500	**91.9%**
IZCARGO®*	4,414	**5,171**	+757	+17.2%	5,200	**99.4%**
TEMCELL®HS Inj.	3,404	**3,236**	(168)	(4.9)%	3,300	**98.1%**
Treatments for renal anemia	4,696	**4,652**	(44)	(0.9)%	5,000	**93.0%**
Epoetin Alfa BS Inj. [JCR]	2,710	**1,994**	(716)	(26.4)%	2,200	**90.6%**
Darbepoetin Alfa BS Inj. [JCR]	1,986	**2,658**	+672	+33.8%	2,800	**94.9%**
Agalsidase Beta BS I.V. Infusion [JCR]	964	**1,661**	+697	+72.2%	1,400	**118.6%**
Total Core products	25,741	**32,636**	+6,895	+26.8%	34,400	**94.9%**
Income from contractual payment	6,546	**7,413**	+867	+13.3%	8,100	**91.5%**
Other*	123	**2,820**	+2,697	+2192.7%	2,900	**97.2%**
AZD1222 stock solution	1,931	**—**	(1,931)	(100.0)%	—	**—**
Total Net sales	34,343	**42,871**	+8,528	+24.8%	45,400	**94.4%**

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

Consolidated Financial Results FY2024 (Forecast)

(Unit: million yen)

Consolidated	FY2023	FY2024(forecast)		
	results	forecast	Year-on-year	
			Difference	Ratio
Net sales	42,871	**41,300**	(1,571)	(3.7)%
Cost of sales	11,620	**10,400**	(1,220)	(10.5)%
Gross profit	31,251	**30,900**	(351)	(1.1)%
Selling, general and administrative expenses	23,719	**25,500**	+1,781	+7.5%
SG&A	12,484	**12,500**	+16	+ 0.1%
R&D expenses	11,234	**13,000**	+1,766	+ 15.7%
Operating profit	7,531	**5,400**	(2,131)	(28.3)%
Ordinary profit	7,264	**4,600**	(2,664)	(36.7)%
Profit attributable to owners of parent	5,507	**3,700**	(1,807)	(32.8)%

Breakdown of Net Sales – Consolidated FY2024 (Forecast)

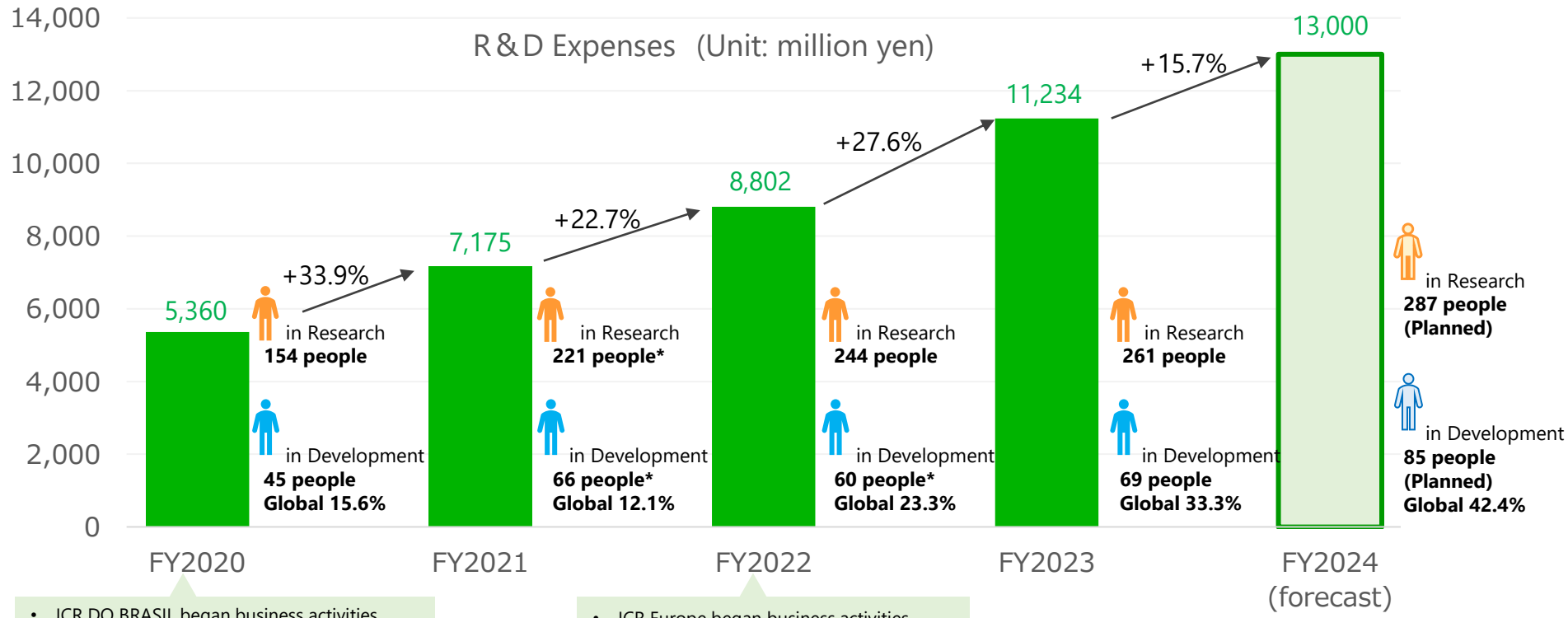
(Unit: million yen)

	FY2023	FY2024(forecast)		
	results	forecast	Year-on-year	
			Difference	Ratio
GROWJECT®	17,913	**18,300**	+387	+2.2%
IZCARGO®*	5,171	**5,700**	+529	+10.2%
TEMCELL®HS Inj.	3,236	**2,800**	(436)	(13.5)%
Treatments for renal anemia	4,652	**4,200**	(452)	(9.7)%
Epoetin Alfa BS Inj. [JCR]	1,994	**2,200**	+206	+10.3%
Darbepoetin Alfa BS Inj. [JCR]	2,658	**2,000**	(658)	(24.8)%
Agalsidase Beta BS I.V. Infusion [JCR]	1,661	**1,100**	(561)	(33.8)%
Total Core products	32,636	**32,100**	(536)	(1.6)%
Income from contractual payment	7,413	**8,100**	+687	+9.3%
Other*	2,820	**1,100**	(1,720)	(61.0)%
Total net sales	42,871	**41,300**	(1,571)	(3.7)%

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

Aggressive R&D Investments

Investments in global clinical development remarkably increased



- JCR DO BRASIL began business activities.
- Bioresearch Center has initiated operation.

- JCR Europe began business activities.

* Including increase/decrease due to organizational changes

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Appendix

What is Named Patient Supply (NPS)?

Named Patient Supply (NPS)

A program whereby a manufacturer makes individual drugs available to physicians who wish to use the drug to patients in countries where it is not approved.

JCR IZCARGO® NPS

A program to make IZCARGO® (Japanese product) available to patients who meet our criteria at the request of a physician in countries where it is not approved, but NPS is allowed under the rules and regulations.

Abbreviations （A～G）

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
CSF	Cerebrospinal fluid	脳脊髄液
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）

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®	-
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
TBD	To be determined	未定