

## **FY2024** First Quarter Results Conference Call

July 30, 2024

JCR Pharmaceuticals Co., Ltd.

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#### **Overview: Consolidated Financial Results**



(Unit: million yen)

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	FY2023		FY2	024		Additional R	
Consolidated			Year-on-year		Progress	Additional	
	Q1 YTD	Q1 YTD Q1 YTD		Rate of change	Rate	The decrease in Sales of Cor	
Net Sales	10,808	8,145	(2,663)	(24.6%)	19.7%	Non-operating	
Cost of Sales	3,363	2,073	(1,289)	(38.4%)	19.9%	Kobe Science	
Gross Profit	7,445	6,072	(1,373)	(18.4%)	19.7%	assets eligible be offset by s	
Selling, General and Administrative Expenses	5,379	6,368	+989	+18.4%	25.0%	purpose reduction financial year.	
SG&A Expenses	3,084	3,192	+108	+3.5%	25.5%	• Gain on cance	
R&D Expenses	2,294	3,175	+881	+38.4%	24.4%	<ul> <li>Gain on cance Extraordinary</li> </ul>	
Operating Profit	2,066	(296)	(2,362)	(114.3%)	(5.5%)	-	
Non-operating Income	520	505	(15)	(3.0%)	-		
Non-operating Expenses	326	466	+139	+42.9%	-		
Ordinary Profit	2,260	(257)	(2,518)	(111.4%)	(5.6%)	Number of Employees (Consolidated)	
Extraordinary Income	-	627	+627	-	-	(consonance)	
Extraordinary Losses	0	0	0	-	-	Net Sales	
Profit before Income Taxes	2,260	369	(1,891)	(83.7%)	-		
Income Taxes	650	168	(482)	(74.2%)	-	Cost of Sales Ratio	
Profit Attributable to Owners of Parent	1,610	201	(1,408)	(87.5%)	5.4%	Cost of Sales Ratio *Excluding income from contractual	
Reference: R&D Expenses before Deducting						payment	
Contribution Amount by Collaborative R&D	2,469	3,462	+992	+40.2%	23.0%	R&D Expenses Ratio	

#### **Additional Remarks**

- The decrease in Cost of Sales due to the decrease in Sales of Core Products.
- Non-operating Expenses include depreciation of Kobe Science Park Centre(KSPC). Non-current assets eligible for grants for KSPC are expected to be offset by special suspense account for tax purpose reduction entry by the end of the current financial year.
- Gain on cancellation of contract is accounted for Extraordinary Income.

(Unit: number of people)

	As of June 30,2023	As of June 30,2024	Difference
Number of Employees (Consolidated)	911	991	+80

Net Sales	FY2023 Q1	FY2024 Q1	Rate of change
Cost of Sales Ratio	31.1%	25.5%	(5.7%)
Cost of Sales Ratio *Excluding income from contractual payment	36.6%	25.5%	(11.1%)

21.2%

+17.8%

39.0%

Destinations

#### **Breakdown of Net Sales (Consolidated)**

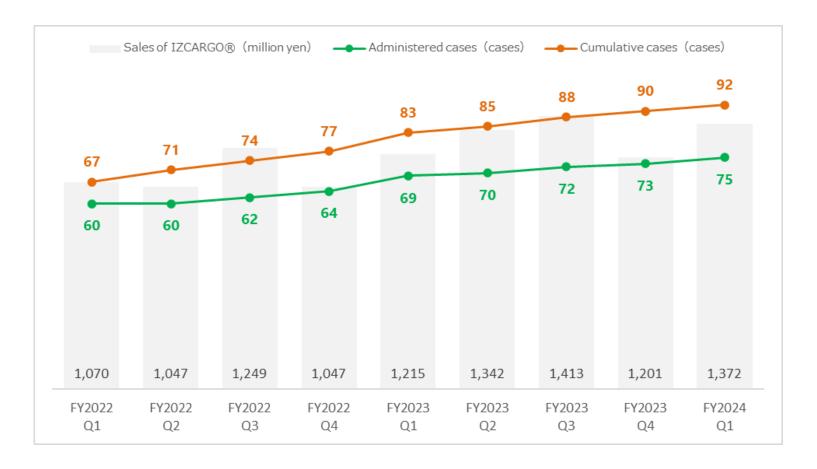


(Unit: million yen)

	FY2023		FY2024		
Consolidated			Year-o	n-year	D
	Q1 YTD	Q1 YTD	Difference	Rate of change	Progress Rate
GROWJECT®	4,222	4,649	+426	+10.1%	25.4%
IZCARGO®	1,214	1,372	+158	+13.0%	24.1%
TEMCELL®HS Inj.	1,063	730	(333)	(31.4%)	26.1%
Treatments for renal anemia	1,615	907	(707)	(43.8%)	21.6%
Epoetin Alfa BS Inj. [JCR]	602	515	(87)	(14.5%)	23.4%
Darbepoetin Alfa BS Inj. [JCR]	1,012	392	(620)	(61.3%)	19.6%
Agalsidase Beta BS I.V. Infusion [JCR]	471	277	(194)	(41.2%)	25.2%
<b>Total Core Products</b>	8,587	7,936	(650)	(7.6%)	24.7%
Income from contractual payment	1,612	15	(1,596)	(99.0%)	0.2%
Other	609	193	(416)	(68.3%)	17.5%
Total Net Sales	10,808	8,145	(2,663)	(24.6%)	19.7%

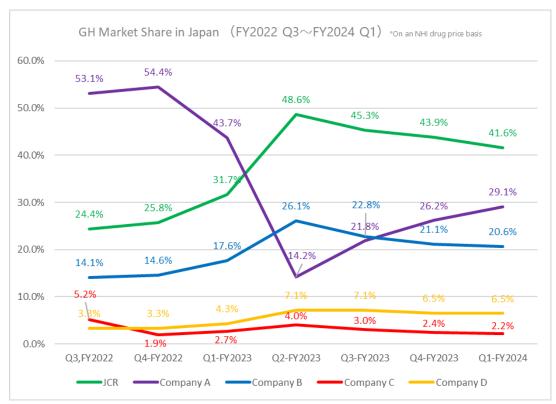
#### **Additional Remarks**

- Sales of GROWJECT® and IZCARGO® increased by more than 10% year-on-year, progressing well.
- Sales of TEMCELL® were down 31.4% year-on-year, but sales progressed as planned against the fullyear forecast. The full-year forecast is expected to be 13.5% lower than FY2023's results due to changes in the competitive environment.
- Sales of treatments for renal anemia were in line with the supply plan to our sales partner, Kissei Pharmaceutical Co., Ltd.
- Sales of Agalsidase Beta BS I.V. Infusion [JCR] were in line with the supply plan to our sales partner, Sumitomo Pharma Co., Ltd.
- Income from contractual payment is expected to be biased toward the second half of the year.

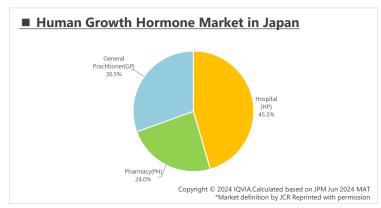


#### **GROWJECT®** Market Share Trends in Japan (Quarterly)





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	Jun 2024	Sales Change FY2024 Q1 (vs. FY2023 Q1) 'On an NHI drug price basis
HP Market	33.6%	+520 million yen
PH Market	31.3%	+273 million yen
GP Market	62.1%	+279 million yen

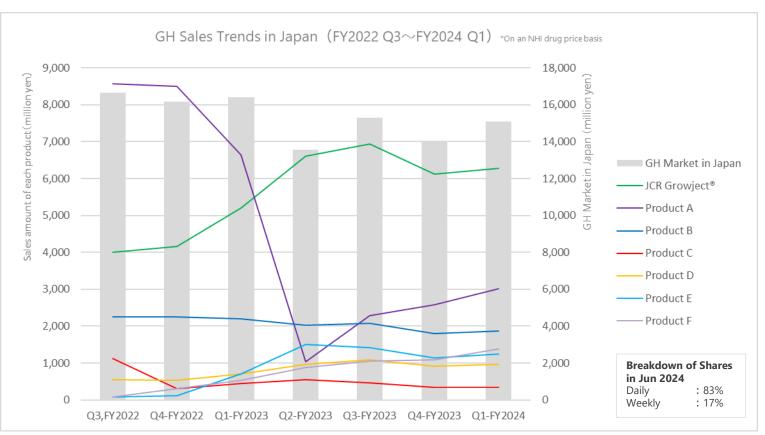
HP: Hospital GP: General Practitioner PH: Pharmacy

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Market definition by JCR Reprinted with permission

#### **GROWJECT® Sales Trends in Japan (Quarterly)**





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#### **FY2024 Progress of Developmental Pipelines**



JR-141

BBB-penetrating iduronate-2-sulfatase (rDNA origin)

Indication: MPS type II

Brand name: IZCARGO® (Launched only in Japan, INN: pabinafusp alfa)

- Global Phase III study (JR-141-GS31: STARLIGHT study)
  - Completed enrollment of more than 60% of the eligible patients in Cohort A
- Revised package insert of IZCARGO® in Japan

JR-441

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

- Phase I/II study (JR-441-101)
  - Completed enrollment of the target number of 12 patients

**JR-446** 

BBB-penetrating  $\alpha$ -N-acetylglucosaminidase (rDNA origin) Indication: MPS type IIIB

- Phase I/II study (JR-446-101)
  - Completed the clinical trial notification process in Japan

#### **FY2024 Progress of Developmental Pipelines**



JR-142

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

- Phase III study (JR-142-301)
  - Completed the clinical trial notification process in Japan

JR-031HIE

human (allogeneic) bone marrow-derived mesenchymal stem cells Indication: Neonatal Hypoxic Ischemic Encephalopathy Brand name: TEMCELL® HS Inj.

Decided to discontinue development based on the results of Phase I/II study

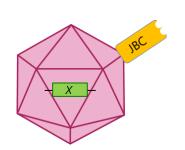
## **R&D Pipeline**



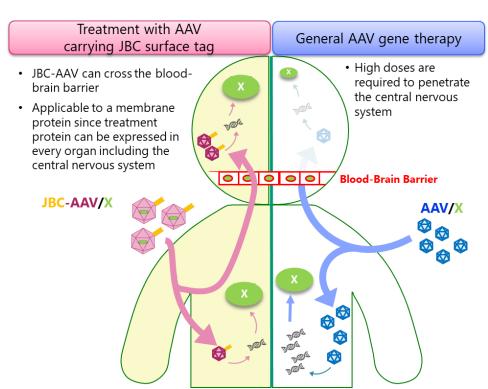
Code	Indication	Status	Upcoming Milestones
JR-141	MPS II (Hunter syndrome)	Global Ph3	• ~FY2027: Approval in US, EU, Brazil
JR-171	MPS I (Hurler syndrome etc.)	Global Ph1/2 completed	<ul><li>Extension study is ongoing</li><li>Licensing out under negotiation</li></ul>
JR-142	Pediatric GHD	Ph2 (Analysis completed)	• FY2024: Phase 3
JR-441	MPS IIIA (Sanfilippo syndrome type A )	Global Ph1/2	<ul> <li>2<sup>nd</sup> Half FY2025: 1-year clinical data is expected</li> </ul>
JR-446	MPS IIIB (Sanfilippo syndrome type B )	CTN process completion	• 1st Half FY2024: FPI in Phase 1/2
JR-471	Fucosidosis	Pre-clinical	_



- Announced platform technology of gene therapy applied J-Brain Cargo®
  - July The 7th International Forum of Lysosomal Disorders
  - October The 31st Annual Congress of the European Society of Gene & Cell Therapy (planned)



J-Brain Cargo®(JBC)-modified adeno-associated virus (JBC-AAV)



## Reach Beyond, Together





Reach Beyond, Together Pharmacoulcals

# **Appendix**

#### **Consolidated Financial Results - FY2024 Forecast**



(Unit: million yen)

	FY2023		FY2024		
Consolidated		Results Forecast	Year-on-year		
	Results		Difference	Rate of change	
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 Expenses	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%)	
Reference :R&D					
expenses before deducting contribution amount by collaborative R&D destinations	12,787	15,062	+2,275	+17.8%	

Net sales	FY2023	FY2024 (Forecast)	YoY Rate of change
Cost of sales ratio	27.1%	25.2%	(1.9%)
Cost of sales ratio *Excluding Income from contractual payment	32.8%	31.3%	(1.4%)
R&D expense ratio	26.2%	31.5%	+5.3%
Operating profit ratio	17.6%	13.1%	(4.5%)

#### **Consolidated Breakdown of Net Sales – FY2024 Forecast**



(Unit: million yen)

	FY2023	FY2024			
	Dogulta	F	Year-oı	Year-on-year	
	Results	Forecast	Difference	Rate of change	
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%)	

### Abbreviations $(A \sim G)$



AAV	Adeno-Associated Virus	アデノ随伴ウイルス
ВВВ	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 $(H\sim Z)$



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