

FY2025 First Quarter Results Conference Call

July 30, 2025

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.

FY2025 First Quarter Consolidated Financial Results

Yoh Ito

Senior Executive Officer

Executive Director, Corporate Strategy Division

Overview: Consolidated Financial Results

(Unit : million yen)

Consolidated	FY2024	FY2025			
	Q1 YTD	Q1 YTD	Year-on-year		Progress Rate
			Difference	Ratio	
Net Sales	8,145	8,569	+424	+5.2%	22.7%
Cost of Sales	2,073	2,357	+284	+13.7%	28.8%
Gross Profit	6,072	6,212	+139	+2.3%	21.0%
Selling, General and Administrative Expenses	6,368	6,818	+449	+7.1%	25.3%
SG&A Expenses	3,192	3,469	+277	+8.7%	28.9%
R&D Expenses	3,175	3,348	+172	+5.4%	22.3%
Operating Loss	(296)	(606)	(309)	-	-
Non-operating Income	505	74	(430)	(85.2)%	-
Non-operating Expenses	466	218	(247)	(53.2)%	-
Ordinary Loss	(257)	(749)	(492)	-	-
Extraordinary Income	627	-	(627)	(100.0)%	-
Extraordinary Losses	0	1	+1	+946.1%	-
Profit (Loss) before Income Taxes	369	(751)	(1,120)	-	-
Income Taxes	168	(205)	(373)	-	-
Profit (Loss) Attributable to Owners of Parent	201	(546)	(747)	-	-
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	3,462	3,518	+56	+1.6%	20.6%

Additional Remarks

- Net Sales increased 5.2% year over year.
- Cost of sales ratio (Excluding income from contractual payment) rose due to lower plant utilization compared to the same period last year.
- SG&A expenses were higher, mainly reflecting increased commission payments to co-promotion partners.
- R&D expenses increased, primarily due to the progress of clinical development activities overseas.
- Non-operating income declined due to lower foreign exchange gains, while non-operating expenses decreased, mainly due to a reduction in equity-method investment losses.

Net Sales	FY2024 Q1 YTD	FY2025 Q1 YTD	Difference
Cost of Sales Ratio	25.5%	27.5%	+2.0%
Cost of Sales Ratio *Excluding income from contractual payment	25.5%	27.9%	+2.4%
R&D Expenses Ratio	39.0%	39.1%	+0.1%
Operating Profit Ratio	(3.6)%	(7.1)%	(3.5)%

Breakdown of Net Sales (Consolidated)

(Unit: million yen)

Consolidated	FY2024	FY2025			
	Q1 YTD	Q1 YTD	Year-on-year		Progress Rate
			Difference	Ratio	
GROWJECT®	4,649	4,495	(154)	(3.3)%	25.3%
IZCARGO® *	1,372	1,562	+190	+13.9%	24.4%
TEMCELL® HS Inj.	730	845	+115	+15.8%	31.3%
Treatments for renal anemia	907	897	(10)	(1.2)%	28.9%
Epoetin Alfa BS Inj. [JCR]	515	122	(393)	(76.3)%	15.3%
Darbepoetin Alfa BS Inj. [JCR]	392	775	+382	+97.6%	33.7%
Agalsidase Beta BS I.V. Infusion [JCR]	277	426	+149	+53.8%	38.7%
Total Core Products	7,936	8,226	+289	+3.6%	26.5%
Income from contractual payment	15	106	+91	+582.2%	1.9%
Other*	193	236	+43	+22.4%	-
Total Net Sales	8,145	8,569	+424	+5.2%	22.7%

Additional Remarks

- GROWJECT®, IZCARGO®, and TEMCELL® HS Inj. all outperformed internal budgets and maintained strong momentum.
- GROWJECT® revenue declined 3.3% year over year due to the NHI price revision, but unit sales increased, highlighting solid demand.
- Sales of the treatments for renal anemia remained aligned with the supply plans of Kissei Pharmaceutical Co., Ltd.
- Sales of Agalsidase Beta BS I.V. Infusion [JCR] remained aligned with the supply plans of Sumitomo Pharma Co., Ltd.
- Milestone revenue was recorded under existing licensing agreements.
- Other income increased, primarily due to higher sales from the NPS program.

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

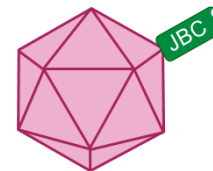
July 2025

License agreement with Alexion for JUST-AAV capsids



- Alexion may use the licensed capsids, which are part of the JUST-AAV platform, in **up to five of Alexion's genomic medicine programs**
- **Milestone payments of up to USD 825 million**
 - Research and development : Up to USD 225 million
 - Commercial : Up to USD 600 million

JUST-AAV



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

AAV: Adeno-Associated Virus
JBC: J-Brain Cargo®

The third partnership with Alexion, following research collaborations involving neurodegenerative disease and oligonucleotide therapeutics

July 2025

Joint collaboration, option and license agreement on J-Brain Cargo® with Acumen



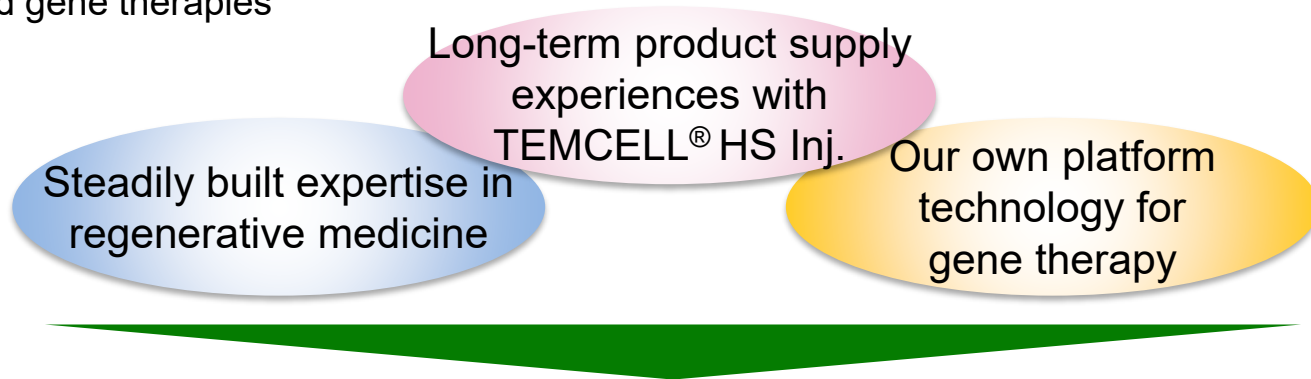
- **To develop blood-brain barrier-penetrating treatment for Alzheimer's disease**
 - Combines JCR's J-Brain Cargo® with Acumen's AβO-selective antibodies
 - Up to two Alzheimer's disease drug candidates eligible for J-Brain Cargo®
 - Regarding one of the candidates, sabirnetug, the Phase II clinical study is ongoing by Acumen
- **Milestone payments of up to USD 555 million**
 - Research and development : Up to USD 40 million
 - Commercial : Up to USD 515 million

AβO: amyloid beta oligomer
Toxic soluble protein, which is a key pathological driver in the onset and progression of Alzheimer's disease

Tackling Alzheimer's disease, one of the most complex healthcare challenges, using our proprietary blood-brain barrier-penetrating technology

Regenerative CDMO Subsidy

- Subsidy program by the Ministry of Economy, Trade and Industry
- Supports the development of domestic CDMO facilities and talent related to regenerative, cell, and gene therapies



**To realize unique value that only JCR can provide,
we are moving forward with investments
in our biomanufacturing facilities**

Progress on Developmental Pipelines

Anne Bechet

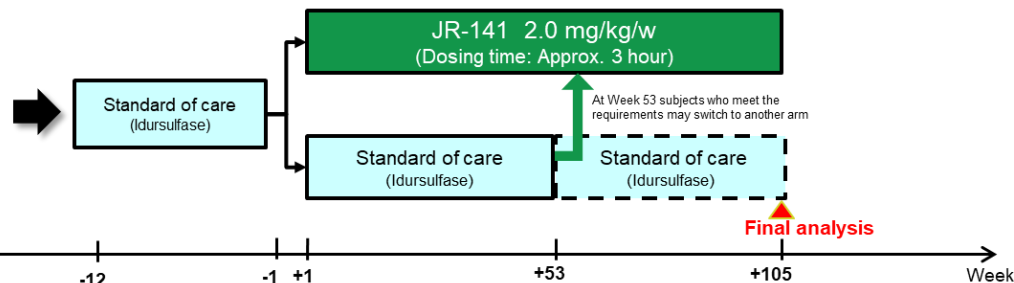
Senior Executive Officer
Executive Director, Development Division
General Manager, JCR Europe B.V.
General Manager, JCR USA Inc.

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

(Summary)

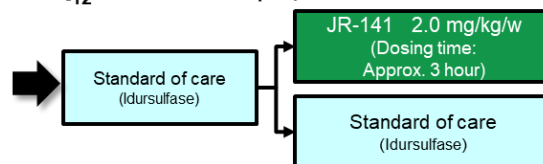
◆ **Cohort A :**
(Neuronopathic patients)

N=60



◆ **Cohort B :**
(Attenuated patients)

N=20



- **Enrollment of the target number of 80 participants achieved**
- **Constructive meeting took place with FDA in June 2025**
 - Ongoing discussion on path forward to BLA

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)	<div>Global Ph3</div>				<ul style="list-style-type: none">Achievement of the patient enrollment~FY2027: Approval in US, EU, Brazil
JR-142	Pediatric GHD	<div>Ph3 (Japan)</div>				<ul style="list-style-type: none">Dec 2024: Initiation of first dosing in Ph3
JR-171	MPS I (Hurler syndrome etc.)	<div>Global Ph1/2 completed</div>				<ul style="list-style-type: none">Extension study ongoingPartnering intensified
JR-441	MPS IIIA (Sanfilippo syndrome type A)	<div>Ph1/2 (Germany)</div>				<Ph1/2> <ul style="list-style-type: none">Patient enrollment completed2H FY2025: 1-year clinical data <Ph1> <ul style="list-style-type: none">Patient enrollment completed
		<div>Ph1 (Japan)</div>				
JR-446	MPS IIIB (Sanfilippo syndrome type B)	<div>Ph1/2 (Japan)</div>				<ul style="list-style-type: none">Dec 2024: Initiation of first dosing in Ph1/2Partnering with MEDIPAL HOLDINGS
JR-471	Fucosidosis	<div></div>				<ul style="list-style-type: none">Partnering with MEDIPAL HOLDINGS

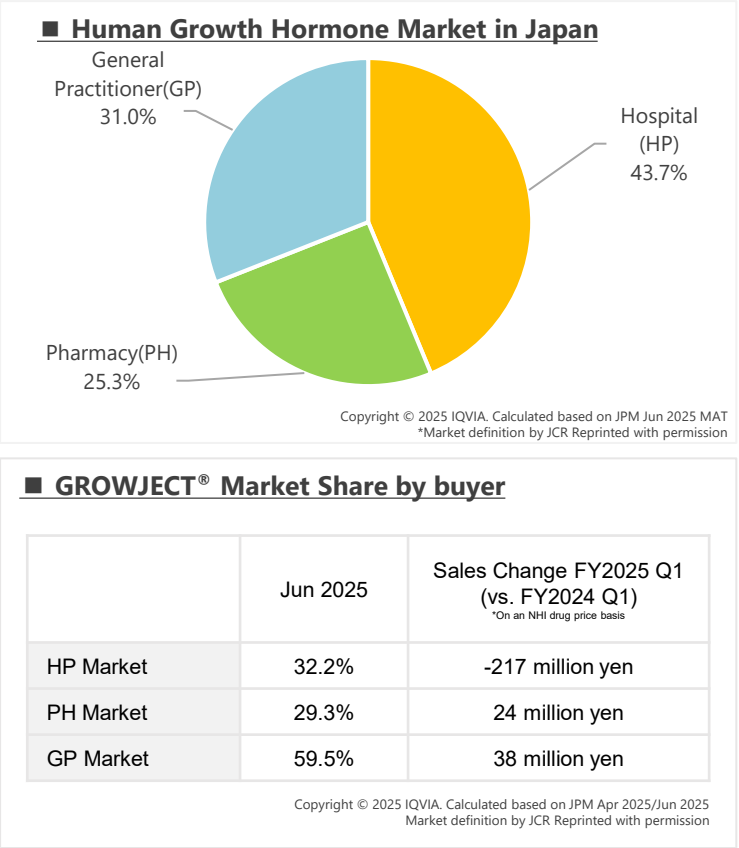
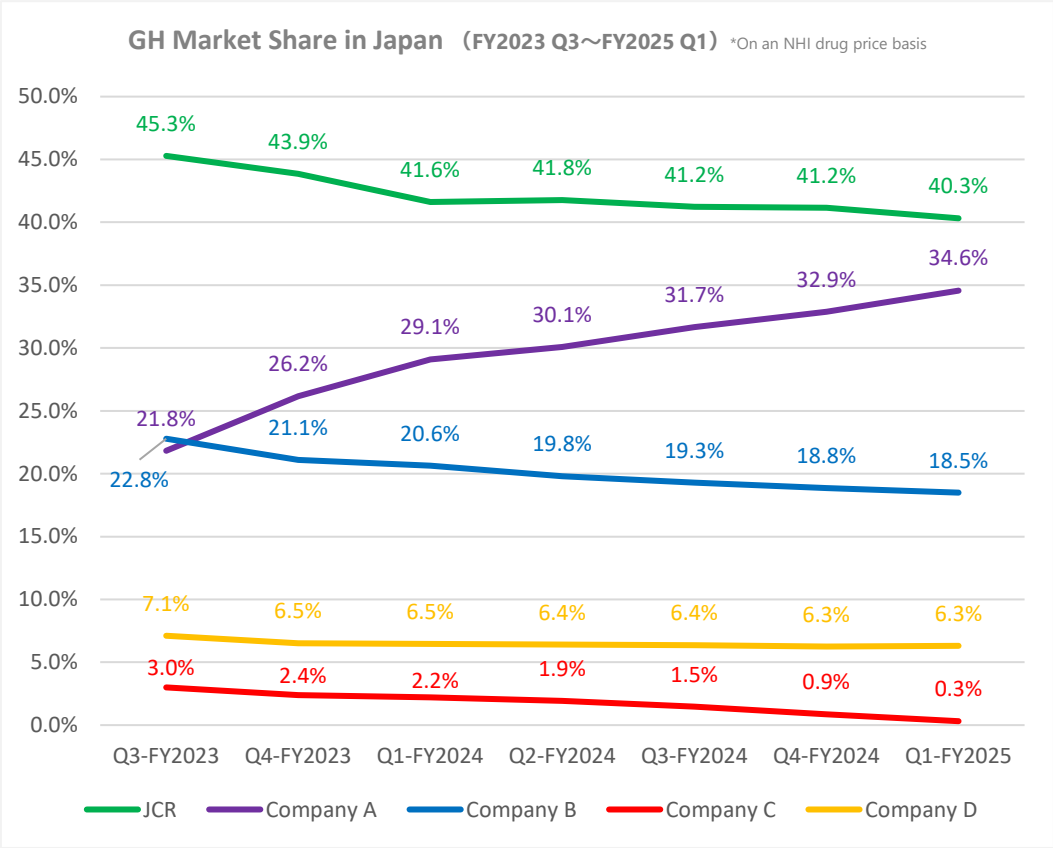
Reach Beyond, Together

一緒に、その先へ

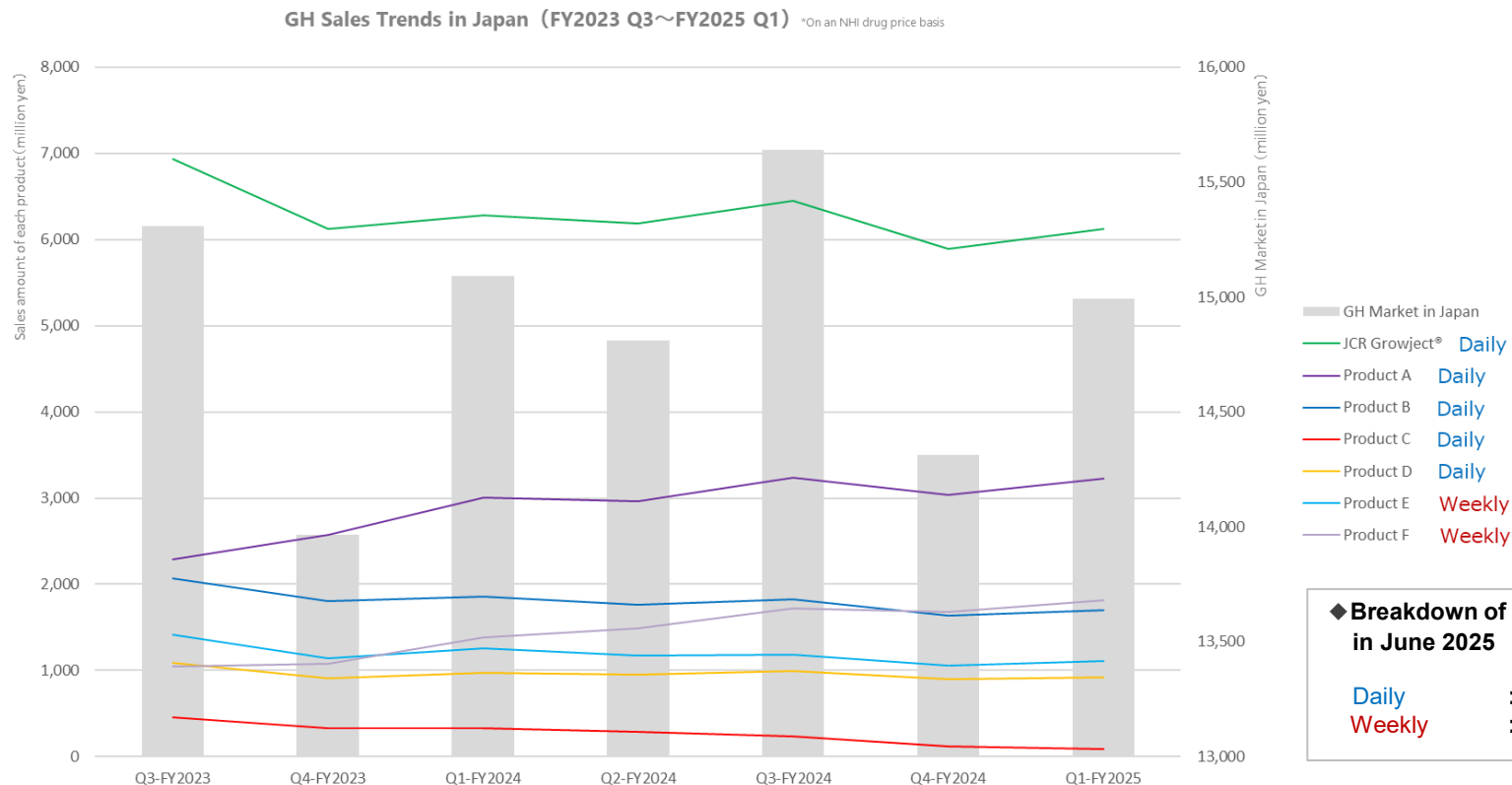


50th
ANNIVERSARY

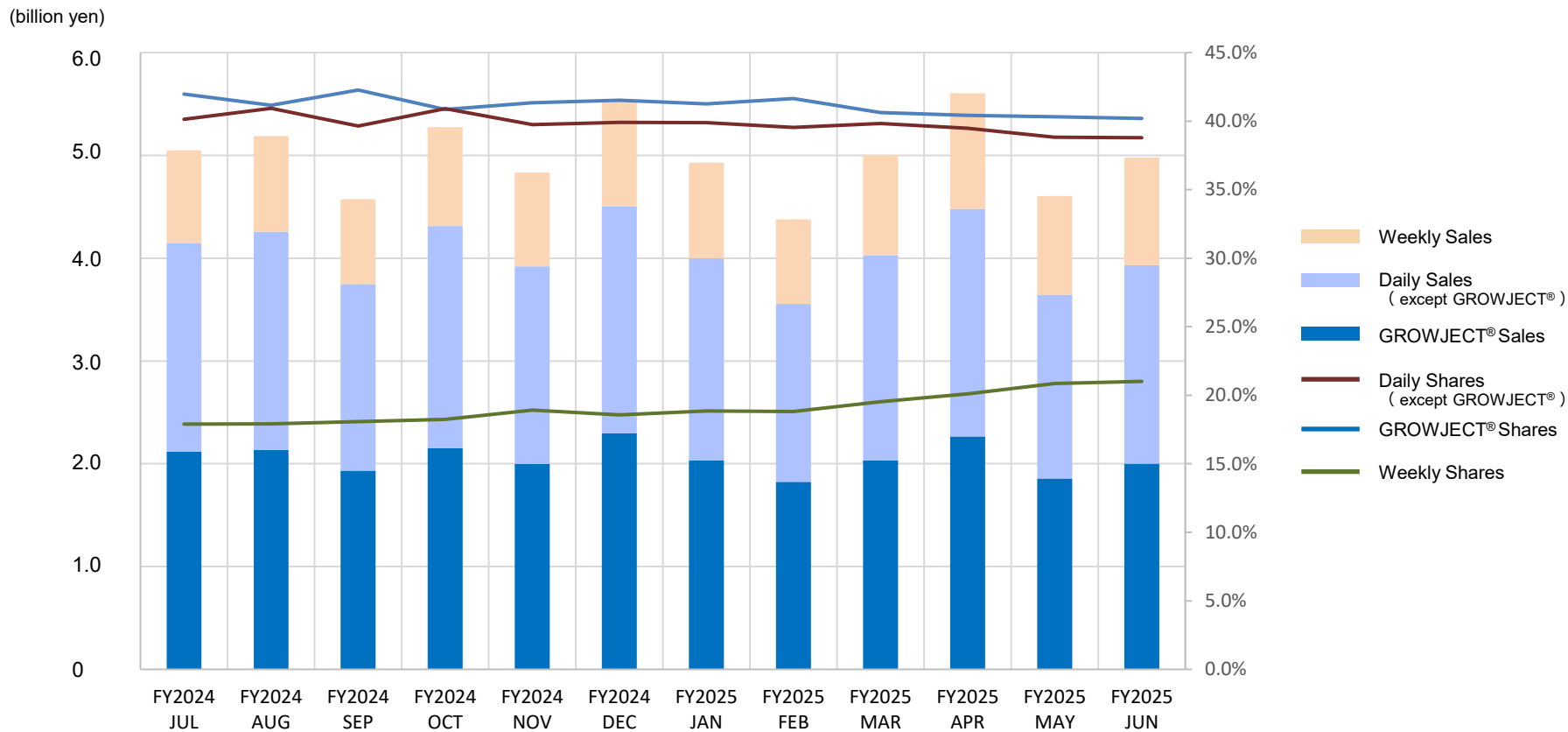
Appendix

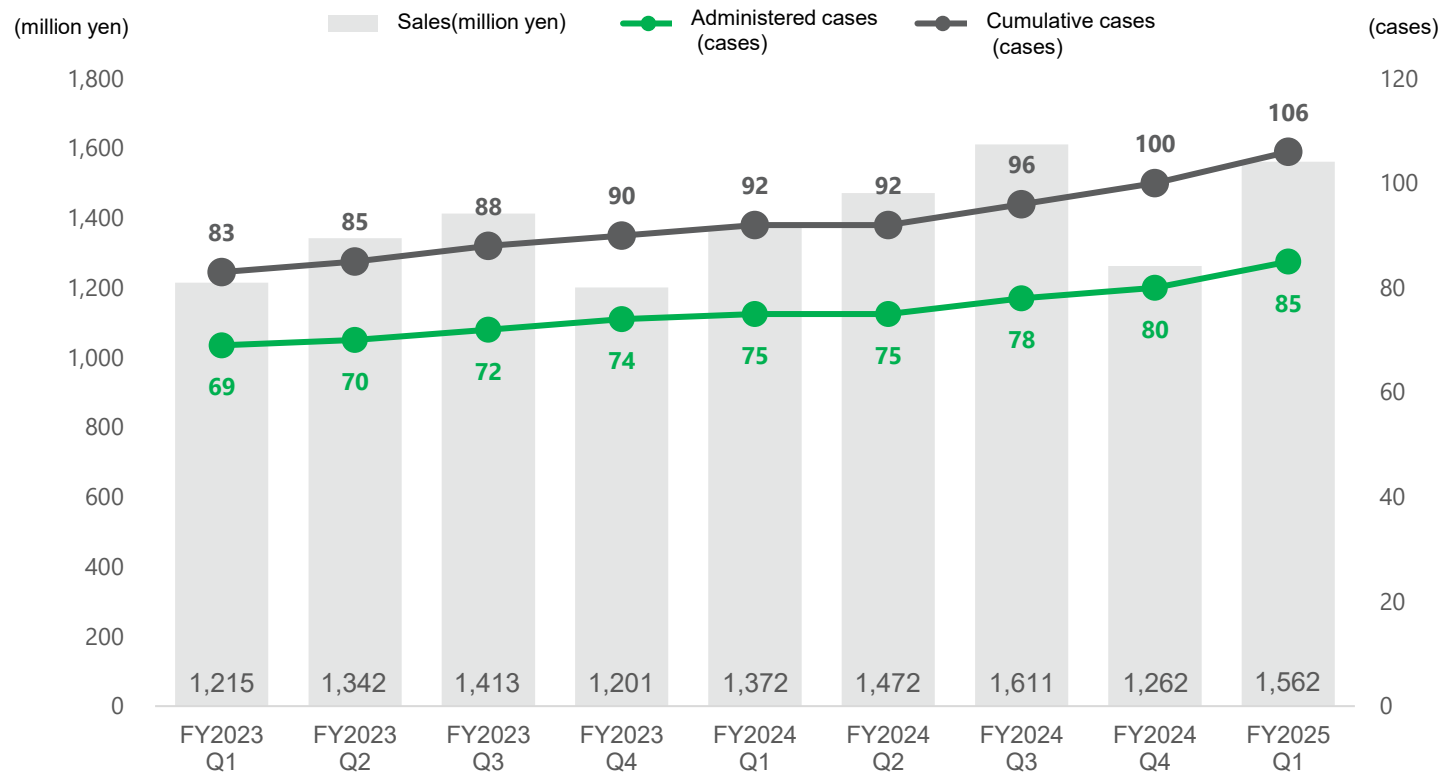


GROWJECT® Sales Trends in Japan (Quarterly)



GROWJECT® Trends of Market Share in Japan





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

(Unit : million yen)

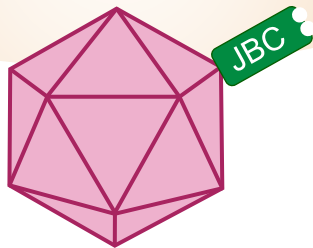
Consolidated	FY2024	FY2025 (Forecast)		
	Results	Forecast	Year-on-year	
			Difference	Ratio
GROWJECT®	18,098	17,800	(298)	(1.6)%
IZCARGO®*	5,718	6,400	+681	+11.9%
TEMCELL®HS Inj.	2,904	2,700	(204)	(7.0)%
Treatments for renal anemia	3,784	3,100	(684)	(18.1)%
Epoetin Alfa BS Inj. [JCR]	1,690	800	(890)	(52.7)%
Darbepoetin Alfa BS Inj. [JCR]	2,093	2,300	+206	+9.9%
Agalsidase Beta 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 Agalsidase Beta 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.

JUST-AAV



AAV: Adeno-Associated Virus

JBC: J-Brain Cargo®

JCR

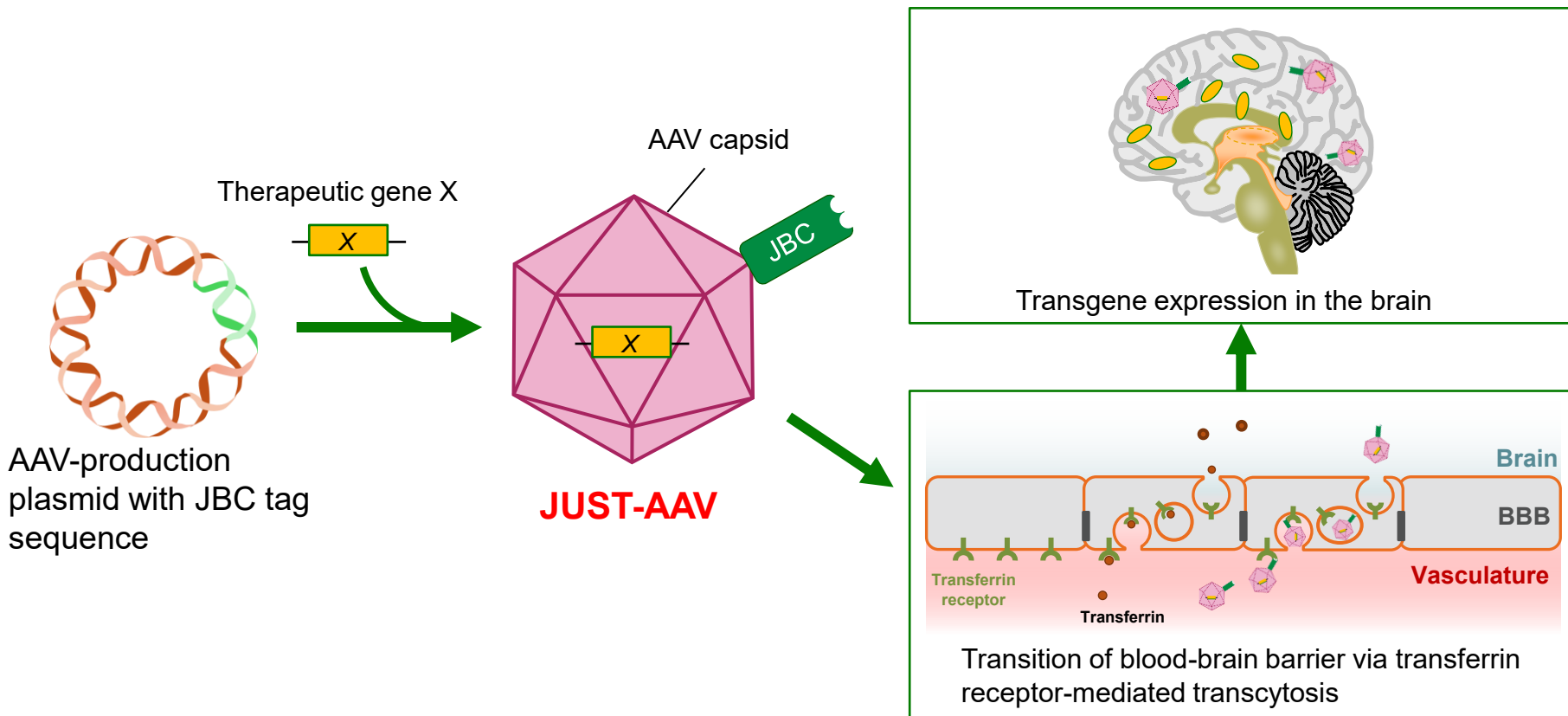
Ultimate Destination of Organ

Safeguarding against off-target delivery

Transformative Technology

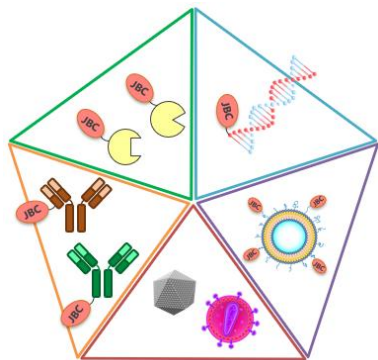
AAV with directionality to target tissues/organs and reduced migration to specific tissues/organs

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



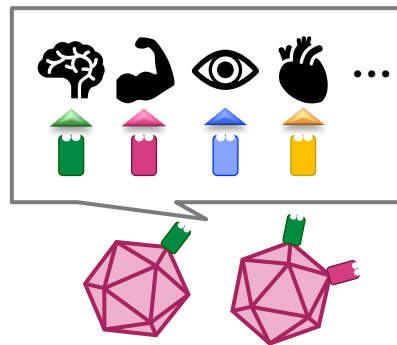
Partnering our groundbreaking technologies and creating breakthrough therapies in various disease areas beyond rare

J-Brain Cargo®



Blood-Brain Barrier transport
applicable to various modalities

JUST-AAV



AAV with enhanced delivery to target
tissues and reduced liver tropism

**Lysosomal Storage
Disorders**

Neurodegeneration

Muscular Diseases

Neuroinflammation

Neuro-oncology

...

AAV	Adeno-Associated Virus	アデノ随伴ウイルス
A β O	Amyloid Beta Oligomer	アミロイドベータオリゴマー
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
BLA	Biologics License Application	生物製剤承認申請
CDMO	Contract Development and Manufacturing Organization	医薬品開発製造受託機関
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 Administration	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

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
YTD	Year to Date	年度累計