FY2025 Second Quarter Results Briefing Session

October 30, 2025

JCR Pharmaceuticals Co., Ltd.

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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 Second Quarter Financial Results

Yoh Ito
Senior Executive Officer
Executive Director, Corporate Strategy Division

Overview: Consolidated Financial Results

(Unit：million yen)

Consolidated	FY2024 Q2 YTD	FY2025 Q2 YTD	FY2025 Year-on-year Difference	FY2025 Year-on-year Ratio	FY2025 Progress Rate
Net Sales	16,657	**21,362**	+4,705	+28.2%	56.5%
Cost of Sales	4,330	**4,323**	(6)	(0.1)%	52.7%
Gross Profit	12,326	**17,038**	+4,711	+38.2%	57.6%
Selling, General and Administrative Expenses	13,066	**14,659**	+1,593	+12.2%	54.3%
SG&A Expenses	6,489	**6,824**	+334	+5.2%	56.9%
R&D Expenses	6,576	**7,835**	+1,258	+19.1%	52.2%
Operating profit	(739)	**2,379**	+3,118	-	-
Non-operating Income	134	**353**	+218	+162.5%	-
Non-operating Expenses	1,016	**370**	(646)	(63.6)%	-
Ordinary profit	(1,621)	**2,362**	+3,983	-	-
Extraordinary Income	1,065	**209**	(855)	(80.3)%	-
Extraordinary Losses	0	**31**	+30	+15,975.2%	-
Profit before Income Taxes	(556)	**2,541**	+3,097	-	-
Income Taxes	134	**830**	+695	+515.1%	-
Profit Attributable to Owners of Parent	(691)	**1,710**	+2,401	-	-
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	7,314	**8,226**	+912	+12.5%	48.1%

Additional Remarks

- Net Sales increased 28% year on year, mainly reflecting higher upfront and milestone payments from licensing agreements.
- Cost of Sales Ratio (excluding income from contractual payment) rose slightly due to lower capacity utilization.
- SG&A Expenses increased, mainly due to higher co-promotion fees in line with sales growth.
- R&D Expenses increased, driven by progress in overseas clinical development programs.
- Non-operating Income increased due to foreign exchange gains, while Non-operating Expenses decreased as foreign exchange and equity-method losses narrowed.
- Extraordinary Income mainly reflected gains on the sale of investment securities.

Net Sales	FY2024 Q2 YTD	FY2025 Q2 YTD	Difference
Cost of Sales Ratio	26.0%	**20.2%**	(5.8)%
Cost of Sales Ratio *excluding income from contractual payment	25.3%	**26.3%**	+1.0%
R&D Expenses Ratio	39.5%	**36.7%**	(2.8)%
Operating Profit Ratio	(4.4)%	**11.1%**	+15.5%

YTD: year to date

Breakdown of Net Sales (Consolidated)

Life is Rare

(Unit: million yen)

Consolidated	FY2024	FY2025			
	Q2 YTD	Q2 YTD	Year-on-year		Progress Rate
			Difference	Ratio	
GROWJECT®	9,401	**8,915**	(486)	(5.2)%	50.1%
IZCARGO® *	2,845	**3,354**	+508	+17.9%	52.4%
TEMCELL®HS Inj.	1,521	**1,582**	+61	+4.0%	58.6%
Treatments for renal anemia	1,764	**1,580**	(184)	(10.4)%	51.0%
Epoetin Alfa BS Inj. [JCR]	962	**296**	(666)	(69.2)%	37.0%
Darbepoetin Alfa BS Inj. [JCR]	801	**1,283**	+482	+60.2%	55.8%
Agalsidase Beta BS I.V. Infusion [JCR]	714	**426**	(288)	(40.3)%	38.7%
Total Core Products	16,246	**15,858**	(388)	(2.4)%	51.0%
Income from contractual payment	15	**5,015**	+4,999	+31,895.0%	91.2%
Other*	395	**489**	+94	+23.9%	-
Total Net Sales	16,657	**21,362**	+4,705	+28.2%	56.5%

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

Additional Remarks

- GROWJECT®, IZCARGO®, and TEMCELL® HS Inj. all outperformed internal budgets and maintained strong momentum.
- GROWJECT®, volumes were flat year on year, but revenue declined following price revisions.
- 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.
- Licensing revenue primarily consisted of upfront and milestone payments under existing agreements.
- Other income increased, primarily due to higher sales from the NPS program.

YTD: year to date

Financial Status (Consolidated)

Life is Rare

(Unit: million yen)

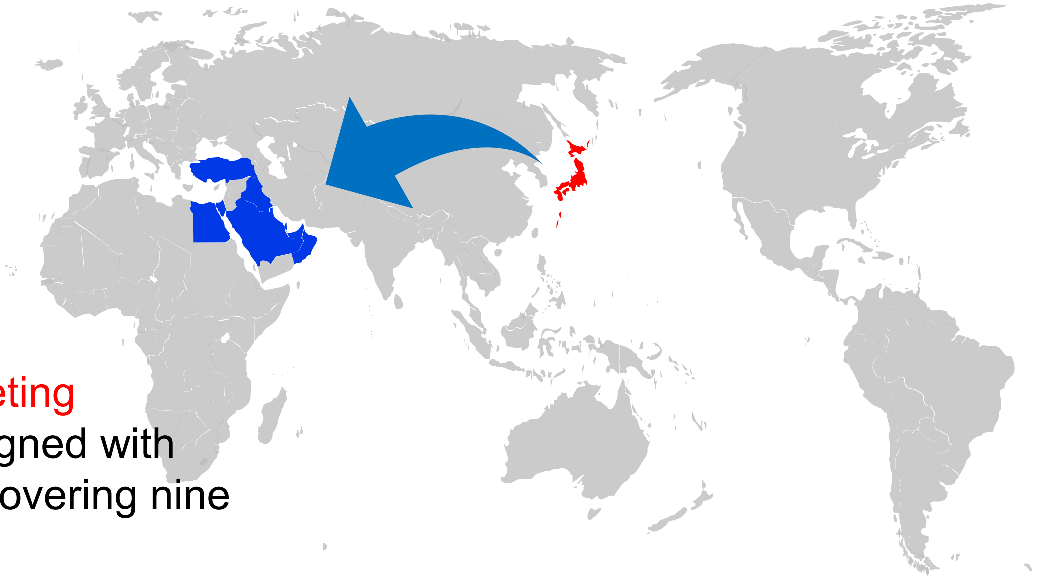
	End-Mar. 2025	End-Sep. 2025	Change • Main Increase/decrease		End-Mar. 2025	End-Sep. 2025	Change • Main Increase/decrease
Current assets	51,056	**55,833**	**+4,776** • Cash and deposits +2,425 • Accounts receivable - trade, and contract assets +1,409 • Inventories +1,655	**Current liabilities**	43,988	**47,508**	**+3,520** • Short-term borrowings +2,400 • Accounts payable - trade +462
				Non-current liabilities	13,431	**13,028**	**(402)**
Non-current assets	53,798	**53,221**	**(576)**	**Total liabilities**	57,420	**60,537**	**+3,117**
				Total net assets	47,435	**48,517**	**+1,082** • Dividends paid (1,220) • Net Profit +1,710
Total	104,855	**109,055**	**4,199**	**Total**	104,855	**109,055**	**4,199**

Additional Remarks

- Inventories increased, reflecting higher stocks of investigational products for ongoing development programs.
- Short-term borrowings increased to fund higher working capital needs.

	End-Mar. 2025	End-Sep. 2025
Equity ratio	44.8%	**44.1%**

Overseas Expansion of an Approved Product in Japan



Agalsidase Beta BS I.V. Infusion [JCR]

- Biosimilar therapeutic for Fabry disease
- Commercially available in Japan since 2018

An exclusive licensing agreement for marketing authorization and commercialization was signed with Menagen Pharmaceutical Industries LLC, covering nine MENAT markets∗

Menagen will file local applications in the licensed territories across the MENAT markets, leveraging the product’s Japanese approval

∗The Kingdom of Saudi Arabia, United Arab Emirates, the Sultanate of Oman, the State of Kuwait, the State of Qatar, the Kingdom of Bahrain, the Republic of Türkiye, the Republic of Iraq, and the Arab Republic of Egypt

Progress of Developmental Pipelines

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

Overview of Clinical or Late Preclinical Pipeline

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Code	Indication	Status				Milestones/Comments
		Preclinical	Phase 1	Phase 2	Phase 3	
JR-141	MPS II (Hunter syndrome)				Global Ph3	• On track for ~FY2027: Approval in US, EU, Brazil
JR-142	Pediatric GHD				Ph3 (Japan)	• Patient recruitment is on track
JR-171	MPS I (Hurler syndrome etc.)			Global Ph1/2 completed		• Partnering activities ongoing
JR-441	MPS IIIA (Sanfilippo syndrome type A)		Ph1/2 (Germany) Ph1 (Japan)			<Ph1/2> • Achieved 1-year clinical data for the initially planned dose groups <Ph1> • Patient enrollment completed
JR-446	MPS IIIB (Sanfilippo syndrome type B)		Ph1/2 (Japan)			• Recruitment of first cohort completed • Partnering with MEDIPAL HOLDINGS
JR-471	Fucosidosis					• Partnering with MEDIPAL HOLDINGS
JR-479	GM2 gangliosidosis (Tay-Sachs disease, Sandhoff disease)					• Partnering with MEDIPAL HOLDINGS

Gene Therapy Platform Technology
~Advancement of JUST-AAV Technology~

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

JCR Life is Rare 50th ANNIVERSARY

Challenges of AAV Gene Therapy

Difficulty in delivery to target tissues

- Central nervous system, muscle, cartilage, etc.
- AAVs do not cross the blood-brain barrier[1]

Safety issues

- Acute liver toxicity, thrombotic microangiopathy, neurotoxicity[2]
- Deaths due to liver injury have occurred in clinical trials[2-4]

Large-scale production of AAV vector

- Complex manufacturing process, requiring advanced technology[5]
- Quality control is extremely important

Neutralizing antibodies

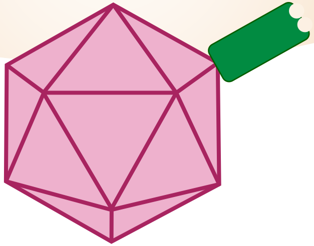
- Risk of pre-existing antibodies making patient ineligible for AAV-mediated gene therapy[6]

Cost of treatment

- Single-treatment solution, but at a high cost[7]

1. Daci R, et al., *Int J Mol Sci*. 2024; 25(2): 1050. 2. Wang JH, et al., *Signal Transduct Target Ther*. 2024; 9(1): 78. 3. Duan D. *Mol Ther*. 2023; 31(11): 3123-3126. 4. *Nat Biotechnol*. 2020; 38(8): 910.
5. Jiang Z, et al., *Trends Biotechnol*. 2023; 41(10): 1268-1281. 6. Weber T. *Front Immunol*. 2021; 12: 658399. 7. Kliegman M, et al., *Nature*. 2024; 634(8033): 307-314.

JUST-AAV



AAV: Adeno-Associated Virus

JCR

Ultimate destination of organ

Safeguarding against off-target delivery

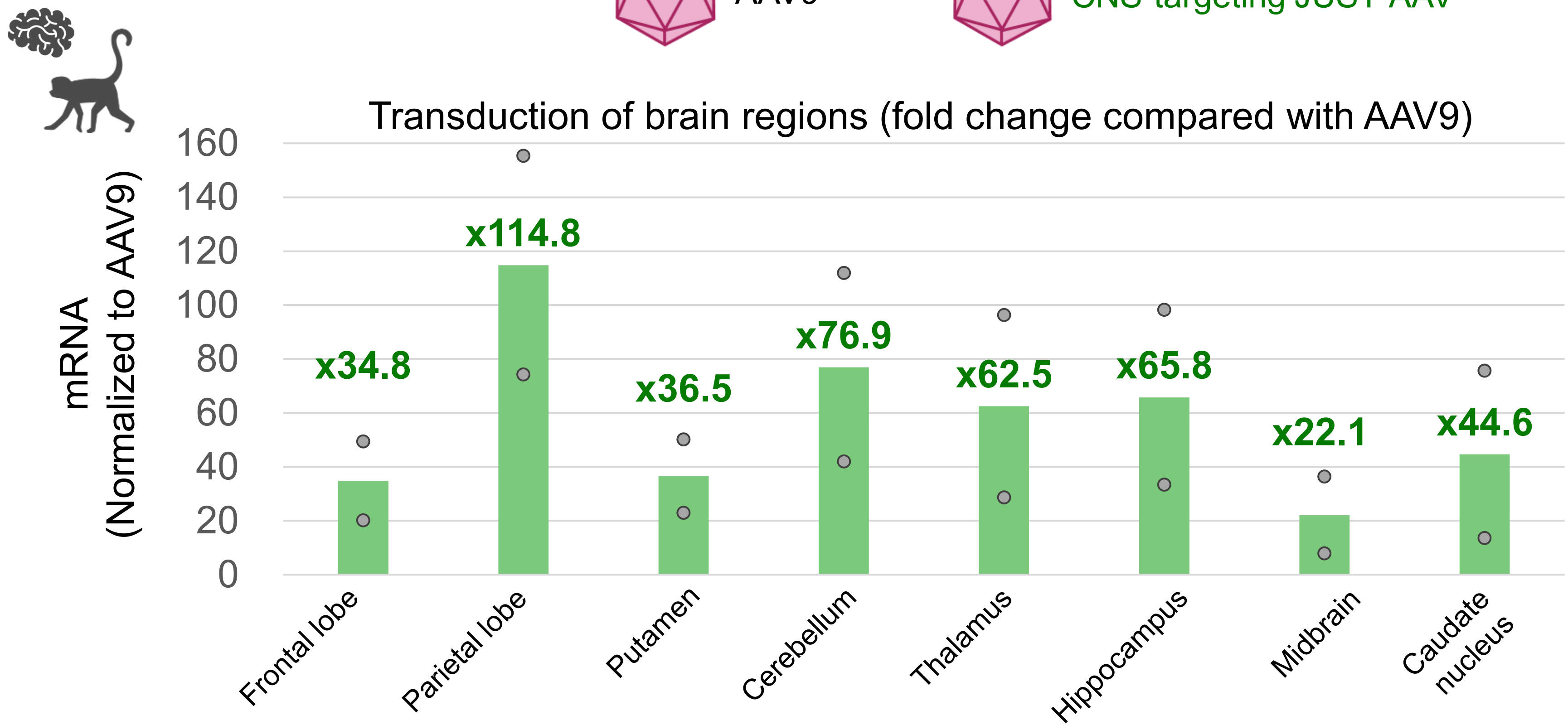
Transformative technology

ex. CNS,
Muscle

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

ex. Liver

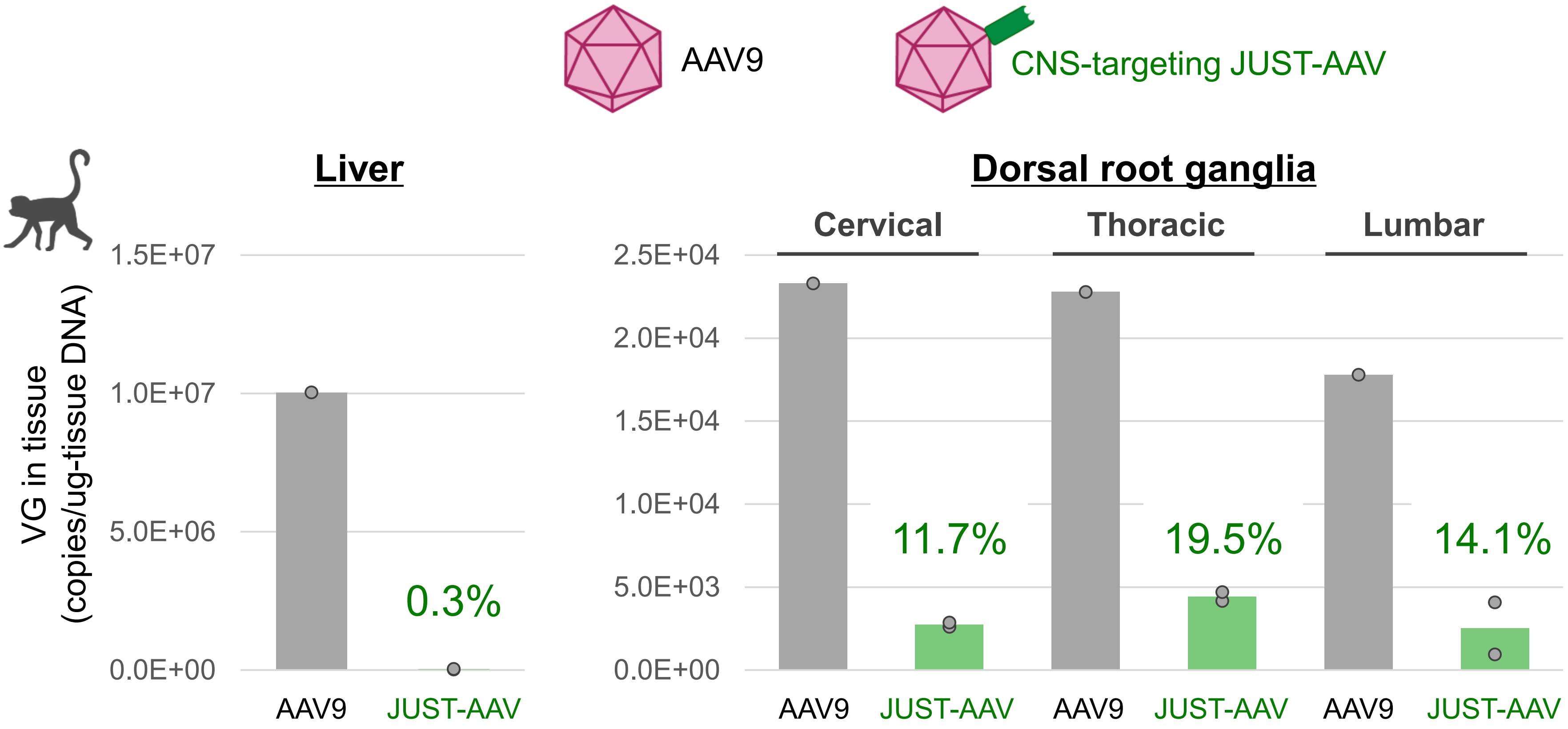
Evaluation of CNS-targeting JUST-AAV Delivery Efficiency



CNS-targeting JUST-AAV: n=2、AAV9: n=1
GOI: H2B-HA, 2.7E13 VG/kg, 4 weeks after admin

The 32nd Annual Congress of the European Society of Gene and Cell Therapy (ESGCT2025)

Evaluation of Tropism Reduction for Specific Tissues



CNS-targeting JUST-AAV: n=2、AAV9: n=1
GOI: H2B-HA, 2.7E13 VG/kg, 4 weeks after admin

Evaluation of Muscle-targeting JUST-AAV Delivery Efficiency

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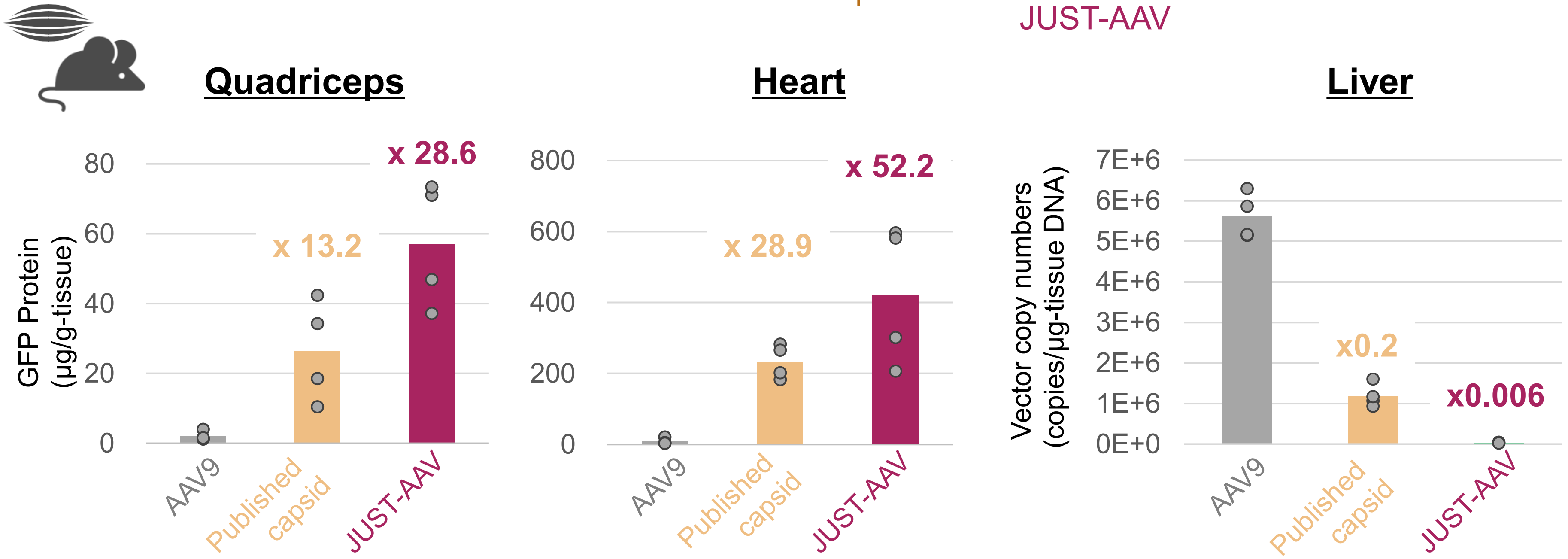


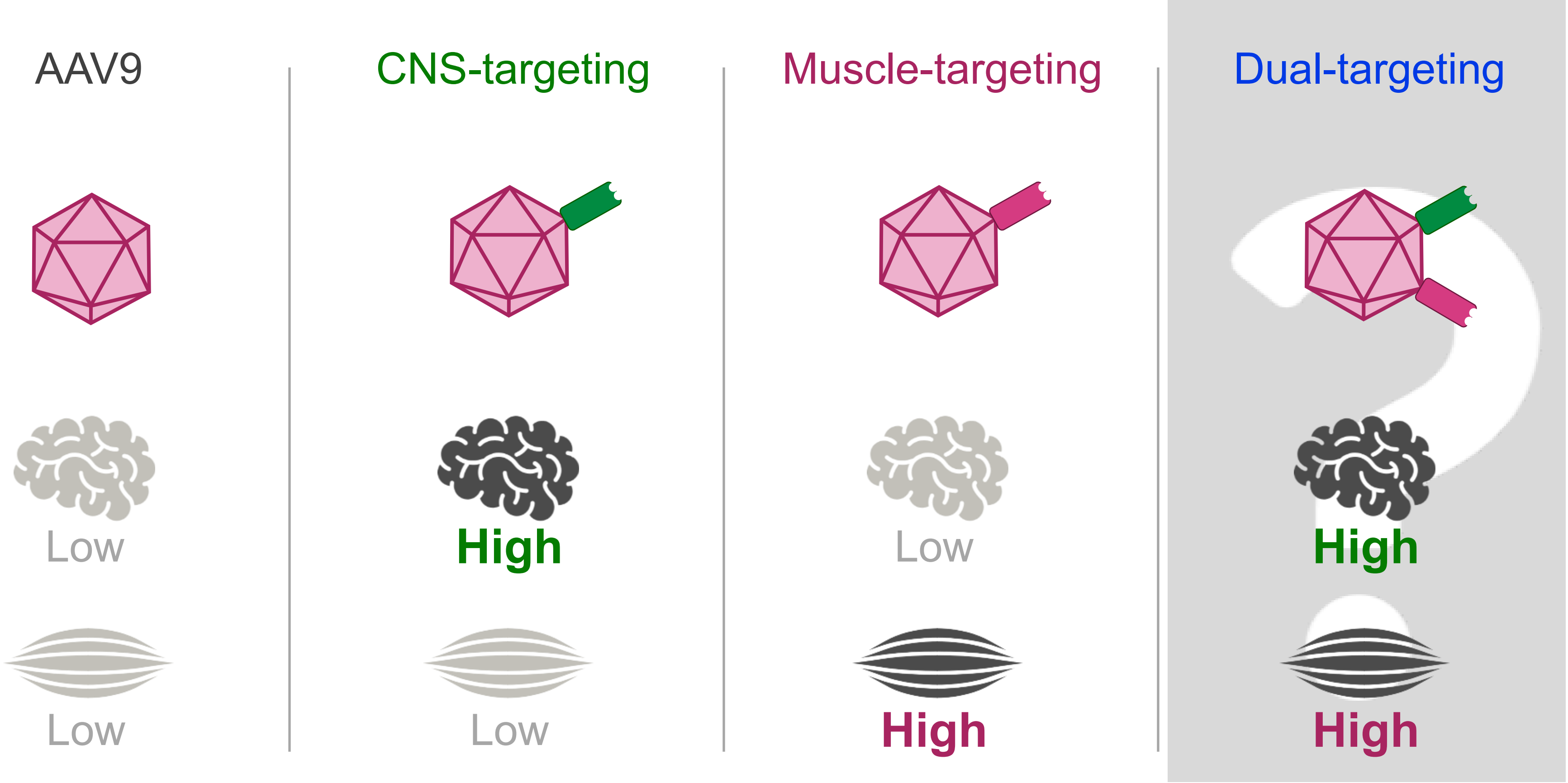
Published capsid: Engineered AAV widely recognized as a muscle-targeting viral vector

GOI: GFP, 1.0E13 VG/kg, n=4, 2 weeks after admin

The 32nd Annual Congress of the European Society of Gene and Cell Therapy (ESGCT2025)

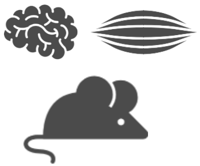
1. Tabebordbar M, et al., *Cell*. 2021; 184(19): 4919-4938. e22.

Combining Tissue-specific Binders for Enhanced Utility

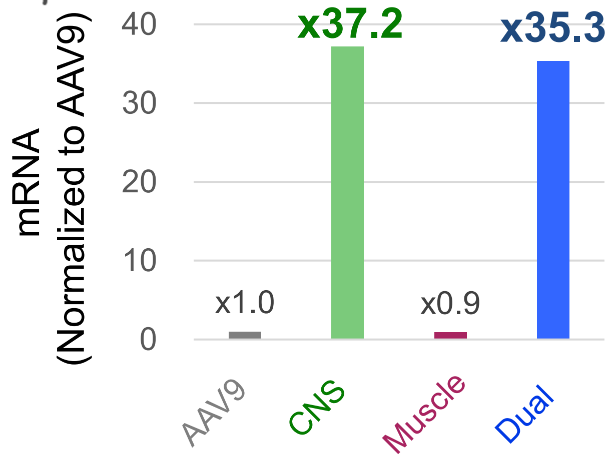


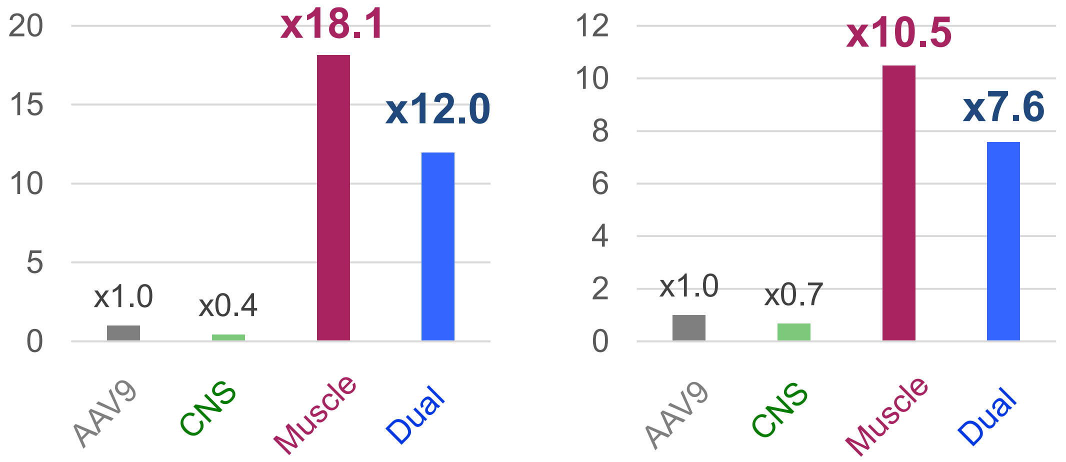
Exploring the Promise of Dual-targeting JUST-AAV

AAV9 CNS-targeting Muscle-targeting Dual-targeting



Brain **Quadriceps** **Heart**





Barcode study (AAV pool was injected)
5.0E12 VG/kg iv per each AAV, n=1, 2 weeks after admin

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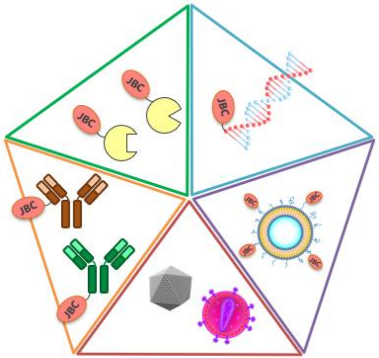
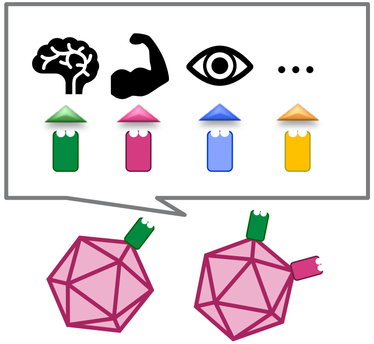
Partnering our groundbreaking technologies and creating breakthrough therapies in various disease areas beyond rare

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
- . . .

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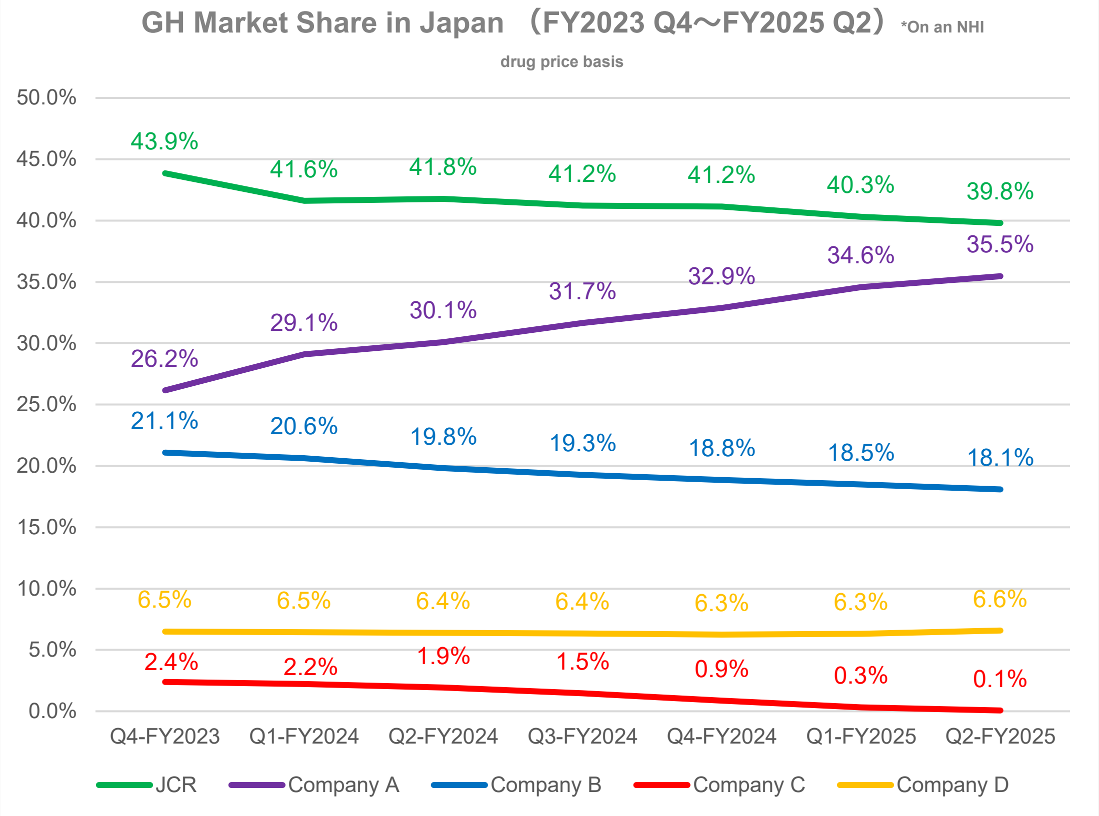
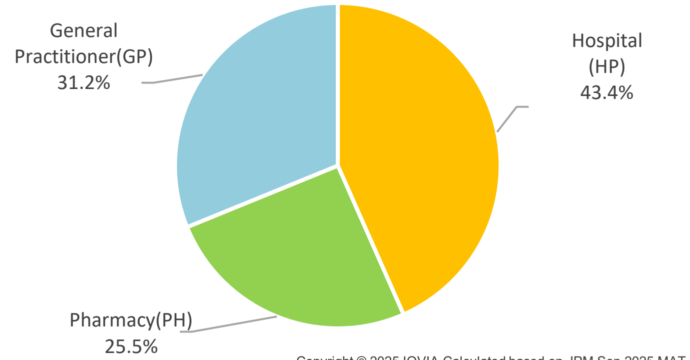
Appendix

GROWJECT® Market Share Trends in Japan (Quarterly)



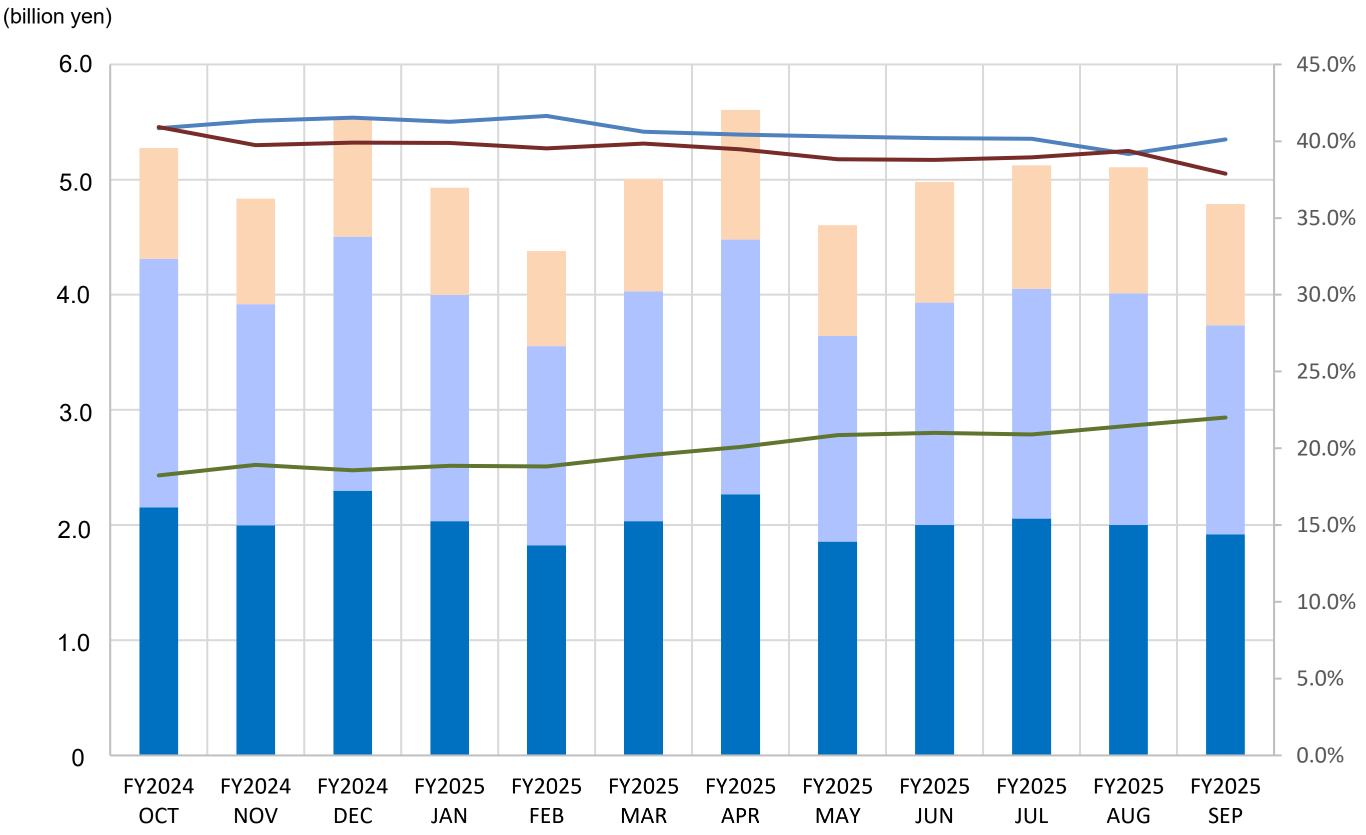
■ Human Growth Hormone Market in Japan



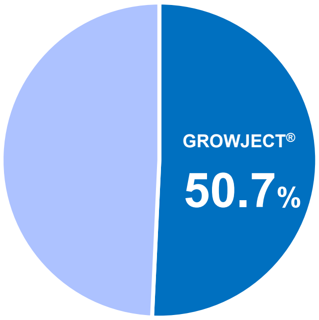
■ GROWJECT® Market Share by buyer

	Sep 2025	Sales Change FY2025 Q2 (vs. FY2024 Q2) *On an NHI drug price basis
HP Market	31.3%	-176 million yen
PH Market	29.1%	-22 million yen
GP Market	59.6%	-11 million yen

GROWJECT® Trends of Market Share in Japan



Daily Shares
FY2024 OCT～FY2025 SEP



IZCARGO® Prescription Status in Japan

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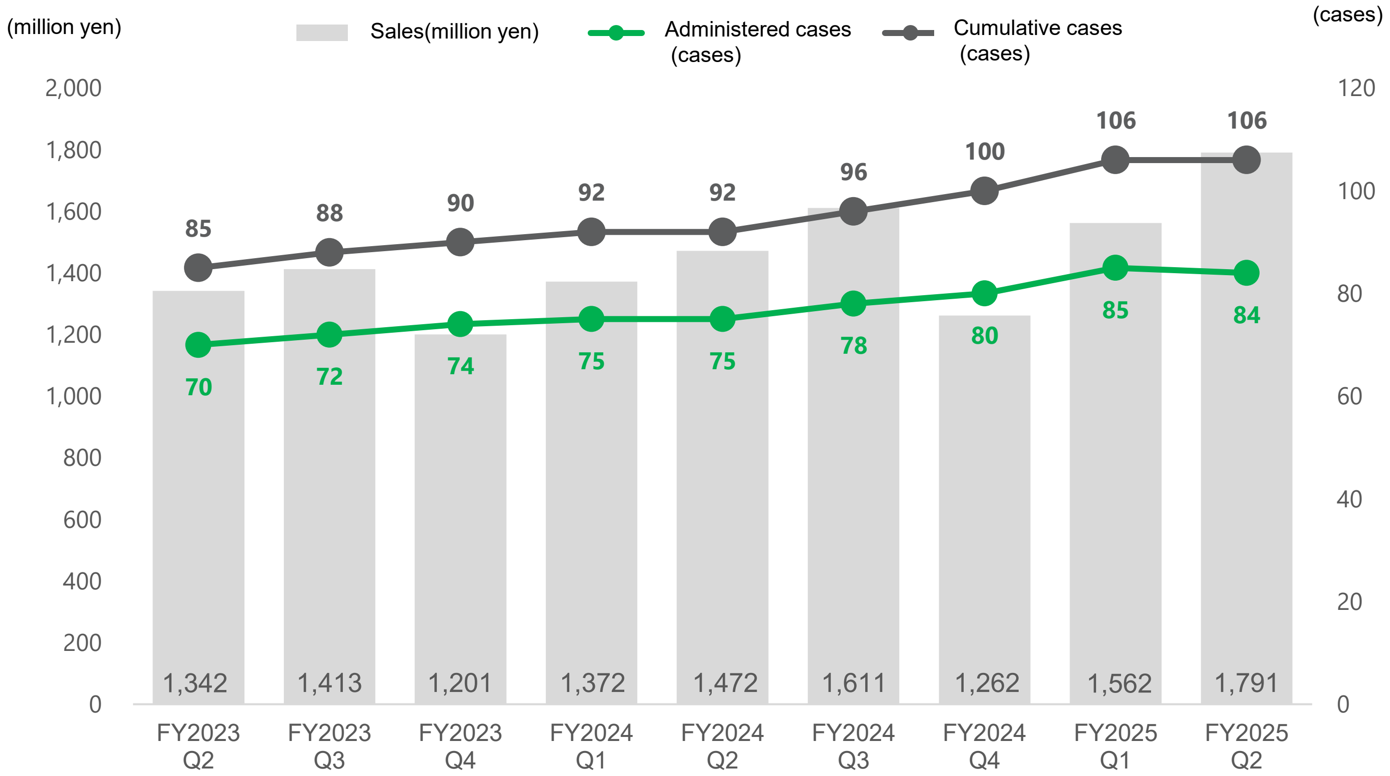
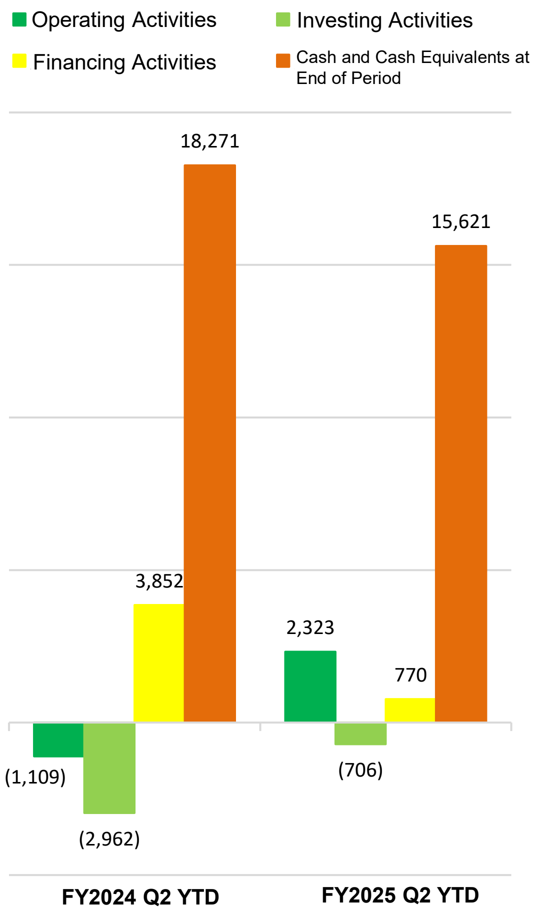


Cash Flows (Consolidated)



(Unit: million yen)

	FY2024 Q2 YTD	**FY2025 Q2 YTD**	Difference
Profit before income taxes	(556)	**2,541**	3,097
Depreciation	1,667	**1,459**	(207)
Decrease (increase) in trade receivables and accounts receivable – other	2,649	**(897)**	(3,546)
Decrease (increase) in inventories	(1,830)	**(1,655)**	174
Increase (decrease) in trade payables and accounts payable – other	(190)	**271**	462
Income taxes paid	(1,451)	**783**	2,235
Other	(1,397)	**(180)**	1,216
Operating Activities	(1,109)	**2,323**	+3,432
Capital investment	(2,992)	**(911)**	2,081
Other	30	**204**	174
Investing Activities	(2,962)	**(706)**	+2,256
Bollowings	5,112	**2,000**	(3,112)
Dividends paid/ treasury shares	(1,233)	**(1,202)**	30
Other	(26)	**(27)**	0
Financing Activities	3,852	**770**	(3,081)
Net increase (decrease) in cash and cash equivalents	(484)	**2,425**	+2,909
Cash and Cash Equivalents at End of Period	18,271	**15,621**	(2,650)

	FY2024		**FY2025**	
	Q2 YTD	Full-year	**Q2 YTD**	**Full-year (Forecast)**
Depreciation	1,667	3,374	**1,459**	**1,900**
Capital investment	2,992	9,888	**911**	**10,300**

YTD: year to date

FY2025 Consolidated Financial Forecasts

Life is Rare

(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

Life is Rare

(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.

Licensing-out of JUST-AAV

July 2025

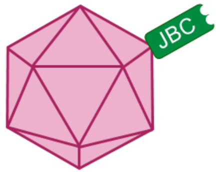
License agreement with Alexion for JUST-AAV capsids

JCR Pharmaceuticals × ALEXION AstraZeneca Rare Disease

- 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

Collaboration to Develop Therapy for Alzheimer's Disease

July 2025

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

JCR Pharmaceuticals × 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

Long-term product supply experiences with TEMCELL® HS Inj.

Steadily built expertise in regenerative medicine

Our own platform technology for gene therapy

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

Abbreviations

AAV	Adeno-associated virus	アデノ随伴ウイルス
AβO	Amyloid beta oligomer	アミロイドベータオリゴマー
BBB	Blood-brain barrier	血液脳関門
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
GFP	Green fluorescent protein	緑色蛍光タンパク質
GHD	Growth hormone deficiency	成長ホルモン分泌不全性低身長症
GOI	Gene of interest	ウイルスベクター内に封入する遺伝子配列
i.v.	Intravenous injection	静脈注射
JBC	J-Brain Cargo®	-
MENAT	Middle East, North Africa and Turkey	中東、北アフリカ、トルコ
MPS	Mucopolysaccharidosis	ムコ多糖症
mRNA	messenger RNA	伝令RNA
NPS	Named patient supply	特定の患者への医薬品提供プログラム
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
R&D	Research and development	研究開発
VG	Viral genome	ウイルスゲノム
YTD	Year to date	年度累計