Life is Rare

FY2025 Third Quarter Results Briefing Session

January 28, 2026

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.

Life is Rare

FY2025 Third Quarter Financial Results

Yoh Ito
Senior Executive Officer
Executive Director, Corporate Strategy Division

Overview: Consolidated Financial Results

(Unit：million yen)

Consolidated	FY2024	FY2025			
	Q3 YTD	Q3 YTD	Year-on-year		Progress Rate
			Difference	Ratio	
Net Sales	25,880	**30,353**	+4,472	+17.3%	76.8%
Cost of Sales	7,007	**6,767**	(239)	(3.4)%	72.0%
Gross Profit	18,873	**23,585**	+4,712	+25.0%	78.4%
Selling, General and Administrative Expenses	19,627	**23,158**	+3,530	+18.0%	78.0%
SG&A Expenses	9,702	**9,786**	+83	+0.9%	74.1%
R&D Expenses	9,925	**13,372**	+3,447	+34.7%	81.0%
Operating profit	(754)	**427**	+1,181	-	-
Non-operating Income	200	**895**	+694	+345.6%	-
Non-operating Expenses	827	**611**	(216)	(26.1)%	-
Ordinary profit	(1,380)	**711**	+2,091	-	-
Extraordinary Income	1,065	**2,091**	+1,026	+96.4%	-
Extraordinary Losses	2	**31**	+29	-	-
Profit before Income Taxes	(317)	**2,772**	+3,089	-	-
Income Taxes	258	**1,028**	+769	+297.1%	-
Profit Attributable to Owners of Parent	(576)	**1,744**	+2,320	-	-
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	11,121	**14,117**	+2,996	+26.9%	79.8%

Additional Remarks

- Net Sales increased, driven by 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 increased mainly due to higher co-promotion fees in line with sales growth.
- R&D expenses increased following an upfront payment for license rights.
- Non-operating income rose on foreign exchange gains.
- Extraordinary income reflected subsidy Income for the Kobe Science Park Center (API Plant).

Net Sales	FY2024 Q3 YTD	FY2025 Q3 YTD	Difference
Cost of Sales Ratio	27.1%	**22.3%**	(4.8)%
Cost of Sales Ratio *excluding income from contractual payment	25.9%	**26.4%**	+0.5%
R&D Expenses Ratio	38.3%	**44.1%**	+5.7%
Operating Profit Ratio	(2.9)%	**1.4%**	+4.3%

YTD: year to date

Breakdown of Net Sales (Consolidated)

(Unit: million yen)

Consolidated	FY2024	FY2025			
	Q3 YTD	Q3 YTD	Year-on-year		Progress Rate
			Difference	Ratio	
GROWJECT™	14,177	**13,539**	(638)	(4.5)%	76.1%
IZCARGO™ *	4,456	**5,179**	+723	+16.2%	80.9%
TEMCELL™HS Inj.	2,296	**2,212**	(84)	(3.7)%	81.9%
Treatments for renal anemia	2,595	**2,346**	(249)	(9.6)%	65.2%
Epoetin Alfa BS Inj. [JCR]	1,250	**595**	(654)	(52.4)%	54.1%
Darbepoetin Alfa BS Inj. [JCR]	1,345	**1,750**	+405	+30.1%	70.0%
Agalsidase Beta BS I.V. Infusion [JCR]	1,149	**863**	(285)	(24.8)%	54.0%
Total Core Products	24,675	**24,141**	(533)	(2.2)%	75.2%
Income from contractual payment	517	**5,249**	+4,732	+914.9%	90.5%
Other*	688	**961**	+273	+39.7%	-
Total Net Sales	25,880	**30,353**	+4,472	+17.3%	76.8%

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

Additional Remarks

- GROWJECT™, IZCARGO™, and TEMCELL™ HS Inj. continued steady performance.
- GROWJECT™ revenue declined due to drug price revisions.
- Sales of the treatments for renal anemia treatments were in line with supply plans to Kissei Pharmaceutical Co., Ltd.
- Sales of Agalsidase Beta BS I.V. Infusion [JCR] were in line with supply plans to Sumitomo Pharma Co., Ltd.
- Licensing revenue primarily consisted of upfront and milestone payments.
- Other income increased due to higher sales from the NPS program.

YTD: year to date

Revision of FY2025 Consolidated Financial Forecast

	Net Sales (Unit: million yen)	Operating Profit (Unit: million yen)	Ordinary Profit (Unit: million yen)	Profit Attributable to Owners of Parent (Unit: million yen)	Earnings Per Share (Unit: yen)
Previously announced forecasts	37,800	2,600	2,400	3,000	24.22
Revised forecasts	**39,500**	**400**	**400**	**1,600**	**13.12**
Change	1,700	(2,200)	(2,000)	(1,400)	-
Change（%）	4.5%	(84.6)%	(83.3)%	(46.7)%	-
Actual Results of the previous fiscal year	33,072	(6,650)	(7,477)	(4,759)	(38.43)

Additional Remarks

Net Sales: revised upward by 1,700 million yen, reflecting stronger-than-expected sales of renal anemia and treatment for fabry disease

Operating profit: revised downward by 2,200 million yen due to higher COGS and SG&A

- ✓ COGS: +1,200 million yen, from the previous forecast, driven by higher sales and changes in the product mix
- ✓ SG&A: +1,200 million yen, as depreciation prior to subsidy confirmation for the Kobe Science Park Center (API Plant) was recognized and higher Q3 spending was factored in
- ✓ R&D: +1,500 million yen, following an upfront payment for the exclusive license for givinostat in Q3

Revision of FY2025 Consolidated Financial Forecast

(Unit: million yen)

	Previously announced forecasts	Revised forecasts	Change	Change(%)	Reference: FY2024 Results
GROWJECT™	17,800	17,800	—	—	18,098
IZCARGO™*	6,400	6,400	—	—	5,718
TEMCELL™HS Inj.	2,700	2,700	—	—	2,904
Treatments for renal anemia	3,100	3,600	500	16.1%	3,784
Epoetin Alfa BS Inj. [JCR]	800	1,100	300	37.5%	1,690
Darbepoetin Alfa BS Inj. [JCR]	2,300	2,500	200	8.7%	2,093
Agalsidase Beta BS I.V. Infusion [JCR]	1,100	1,600	500	45.5%	1,149
Total Core Products	31,100	32,100	1,000	3.2%	31,655
Income from contractual payment	5,500	5,800	300	5.5%	517
Other*	1,200	1,600	400	33.3%	898
Total Net Sales	37,800	39,500	1,700	4.5%	33,072

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

Agreement with Italfarmaco (Press Release in Dec, 2025)[1]

- **Development and Commercialization of Givinostat for DMD**
  - Exclusive licensing agreement (Japan)

- **Strategic collaboration for the treatment of rare diseases**
  - Enhancing both companies' portfolios
  - Exploring joint opportunities across JCR's R&D pipeline and platform technologies

1. January 23, 2026: Briefing session held regarding the agreement with Italfarmaco (link)

Givinostat: Oral Non-Steroidal Treatment for DMD

1 Distinct mechanism of action from other DMD therapies

- HDAC inhibitor with mutation-agnostic mechanism of action

2 Regulatory approvals outside Japan

- Approved in major markets, including the US and the EU[1]
- Clinical evidence demonstrated in placebo-controlled study

3 Synergy with our core strengths

- Extensive expertise in rare disease drug development
- Robust network with clinicians treating patients with DMD
  - >60% coverage of DMD-treating institutions with existing products (internal data)

4 Strong commercial potential in Japan

- ~3,500 individuals in Japan diagnosed with DMD[2]
  - Over 1,000 individuals: Ambulatory, ≥6 years of age[3]
  - Over 3,000 individuals: ≥6 years of age[3]

DMD, duchenne muscular dystrophy

1. US: Approved for patients with DMD aged 6 years and older, EU: Conditionally approved for ambulatory DMD patients aged 6 years and older and already being treated with corticosteroids
2. Kawai M. *No To Hattatsu*. (Japanese) 2013;45(Supple.):S324 3. Company estimates based on Remudy (Registry of Muscular Dystrophy) and Nakamura H et al. *Orphanet J Rare Dis*. 2013;8:60

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	• On track for ~FY2027: Approval in US, EU, Brazil
JR-142	Pediatric GHD	Ph3 (Japan)	• Patient recruitment 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 • Actively pursuing early approval in Japan
JR-446	MPS IIIB (Sanfilippo syndrome type B)	Ph1/2 (Japan)	• Recruitment of first cohort completed • Actively pursuing early approval in Japan • Partnered with MEDIPAL HOLDINGS
JR-471	Fucosidosis		• Commencement of natural history study • Partnered with MEDIPAL HOLDINGS
JR-479	GM2 gangliosidosis (Tay-Sachs disease, Sandhoff disease)		• Partnered with MEDIPAL HOLDINGS
Givinostat	Duchenne muscular dystrophy	Approved in major markets, including the US and the EU	• Under discussions with PMDA toward domestic approval by 2028

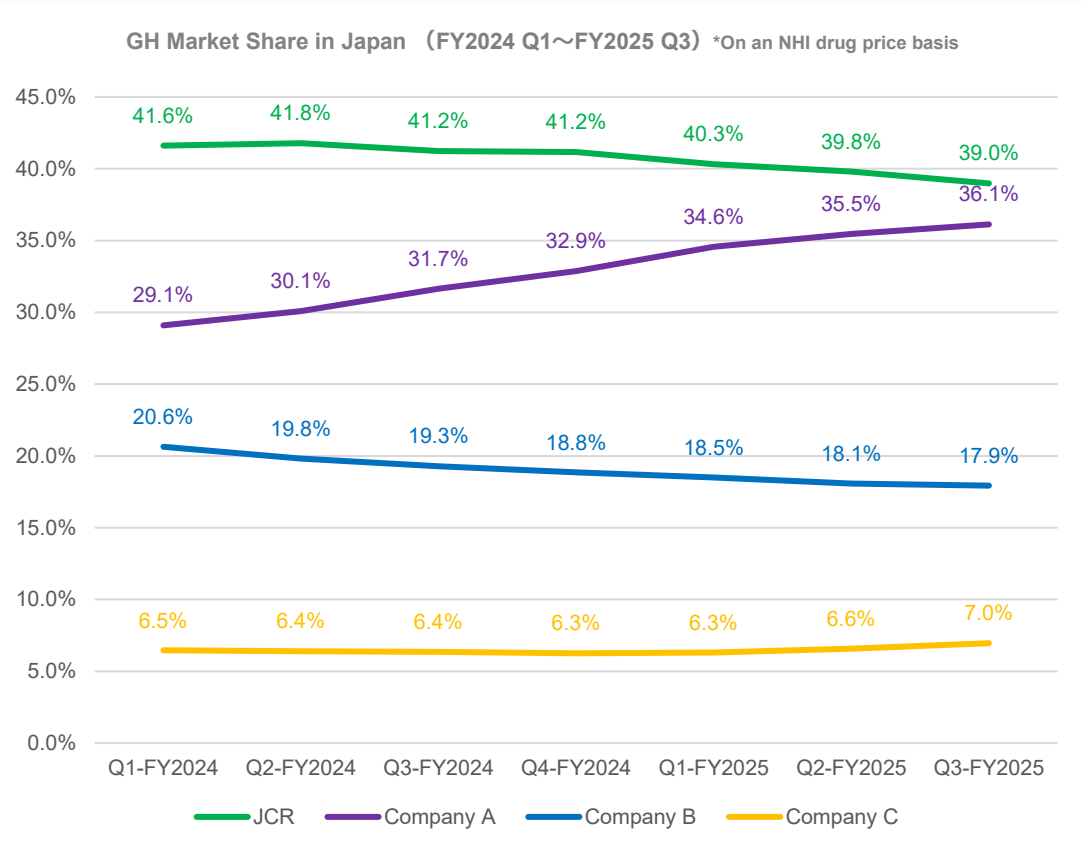
Confidential

JCR Pharmaceuticals

Life is Rare

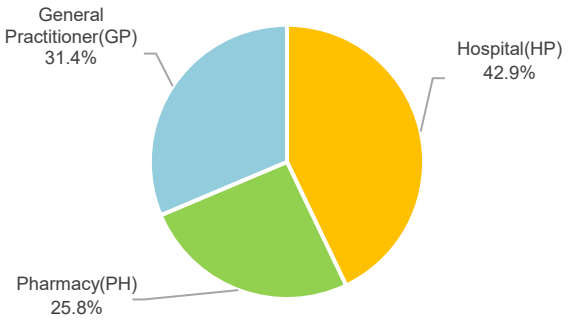
Appendix

GROWJECT™ Market Share Trends in Japan (Quarterly)



Copyright © 2026 IQVIA. Own analysis based on JPM (Apr 2024-Dec 2025). Reprinted with permission

■ Human Growth Hormone Market in Japan



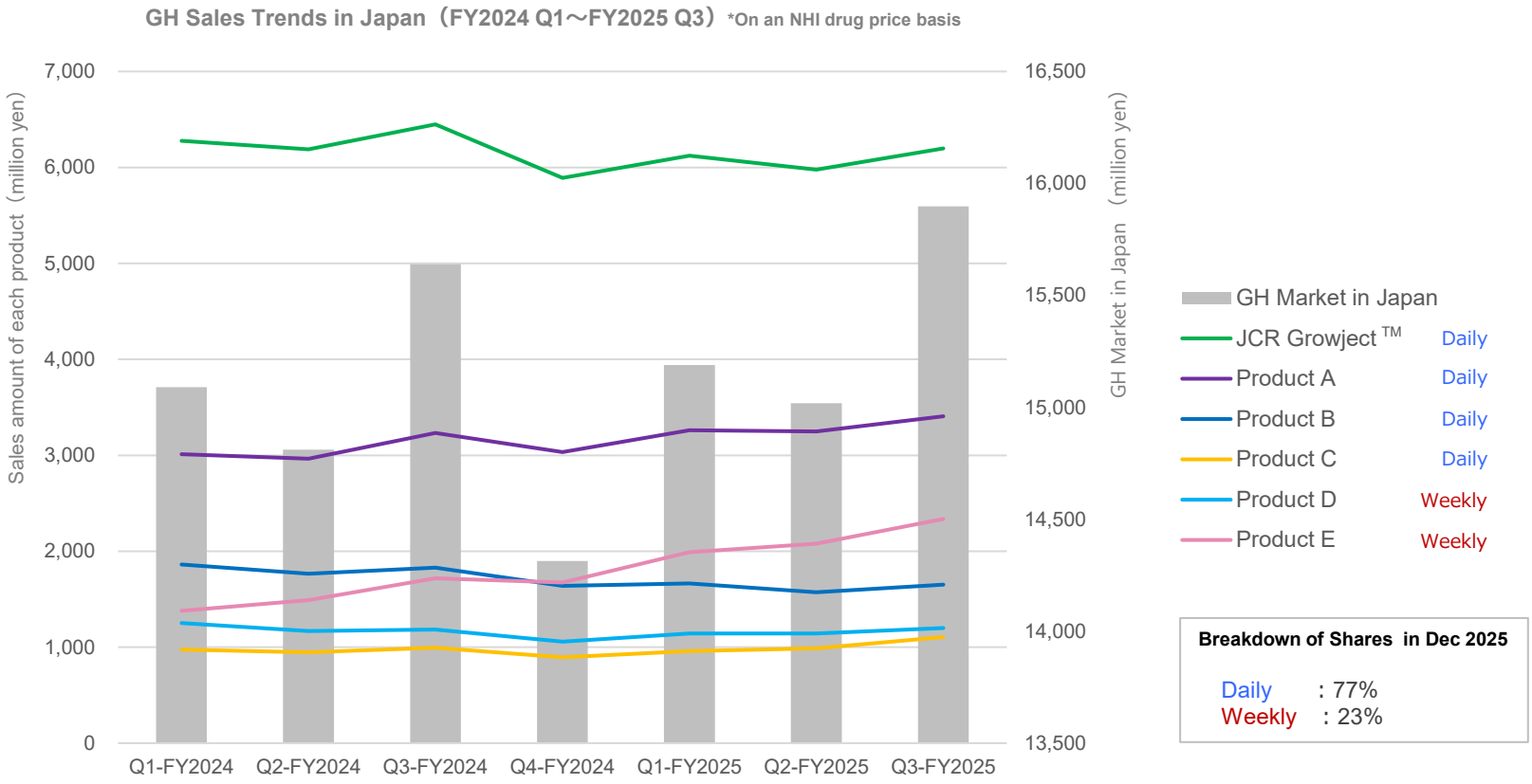
Copyright © 2026 IQVIA.Calculated based on JPM Dec 2025 MAT
*Market definition by JCR Reprinted with permission

■ GROWJECT™ Market Share by buyer

	Dec 2025	Sales Change FY2025 Q3 (vs. FY2024 Q3) *On an NHI drug price basis
HP Market	31.9%	-162 million yen
PH Market	28.0%	-21 million yen
GP Market	56.9%	-68 million yen

Copyright © 2026 IQVIA. Calculated based on JPM Oct 2025/Dec 2025
Market definition by JCR Reprinted with permission

GROWJECT™ Sales Trends in Japan (Quarterly)

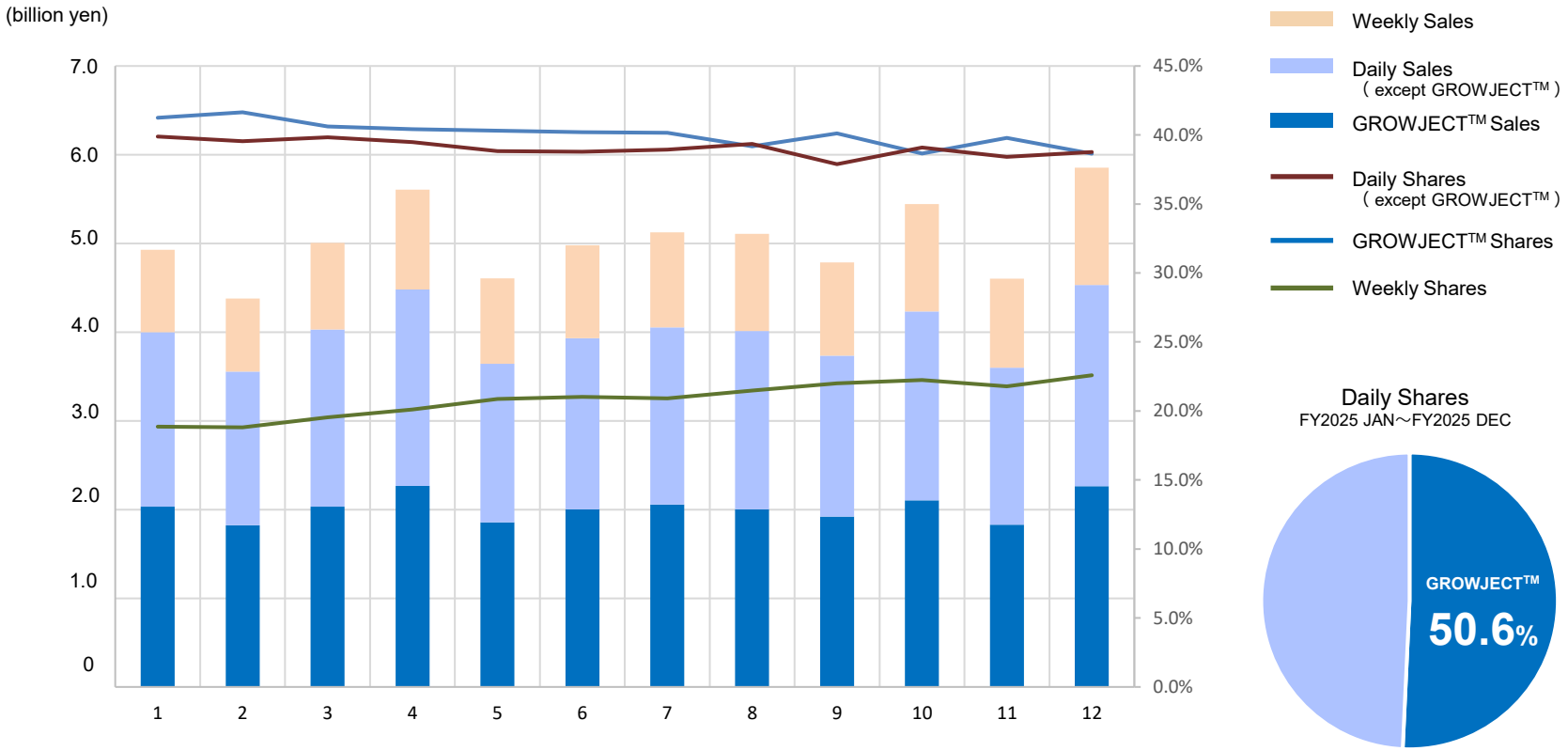


Breakdown of Shares in Dec 2025

Daily : 77%
Weekly : 23%

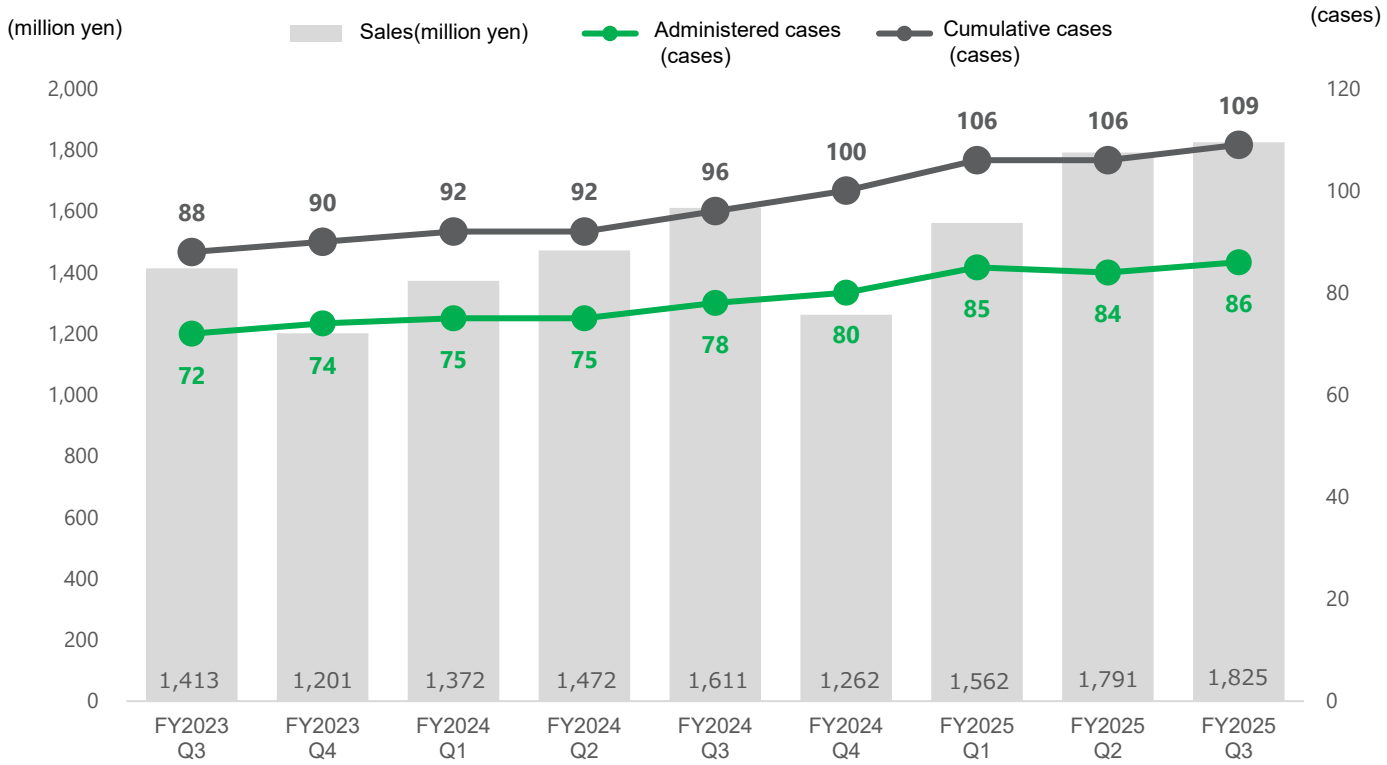
Copyright © 2026 IQVIA. Own analysis based on JPM (Apr 2024-Dec 2025). Reprinted with permission

GROWJECT™ Trends of Market Share in Japan



Copyright © 2026 IQVIA. Own analysis based on JPM (Jan 2025-Dec 2025). Reprinted with permission

IZCARGO™ Prescription Status in Japan



Partnerships are at the Core of JCR's Business Philosophy

Approval of IZCARGO™

JR-141 partnership with Takeda (until 2024)

Gene therapy partnership with Takeda (until 2024)

Ultra rare disease partnership with MEDIPAL

JBC partnership with Alexion (Oligonucleotides)

JBC partnership with Alexion (Neuro)

JBC partnership with Angelini (epilepsy)

Givinostat partnership (Japan) Strategic partnership

Gene therapy partnership with Alexion

Neurodegeneration partnership with Acumen

2021 2022 2023 2025

ITALFARMACO

Italfarmaco S.p.A

- Private global pharmaceutical company founded in 1938 (Milan, Italy)
- Development, manufacturing, marketing and sales of branded prescription & non-prescription products
- Proven success in many therapeutic areas including immuno-oncology, neurology, and cardiovascular disease
- Rare disease unit includes programs muscular dystrophy, ALS and polycythaemia vera

Employees
>4000

Business
>90 contries

Manufacturing
6 sites

R&D dept.
>300

Licensing-out of JUST-AAV

July 2025

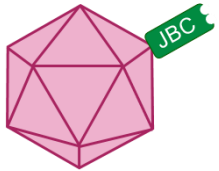
License agreement with Alexion for JUST-AAV capsids

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

- **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

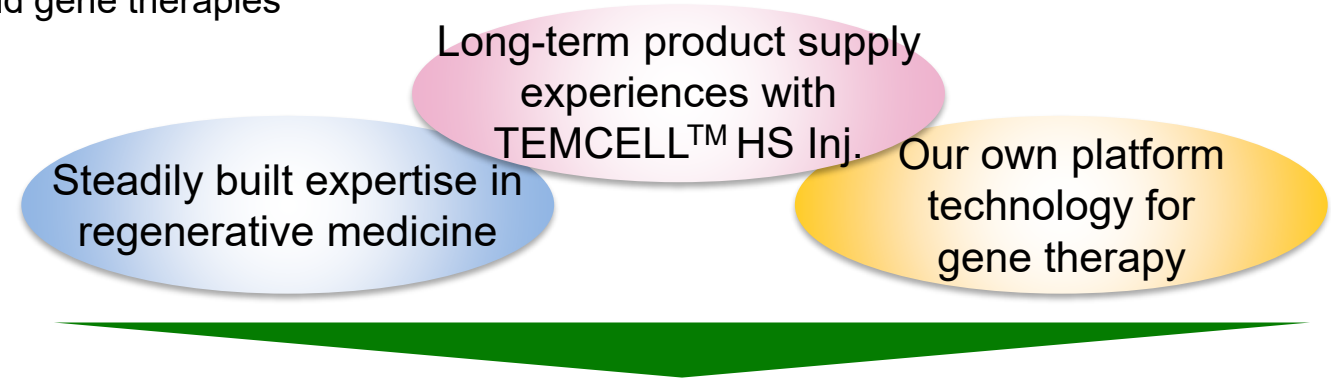
- **Milestone payments of up to USD 555 million**
  - Research and development : Up to USD 40 million
  - Commercial : Up to USD 515 million

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

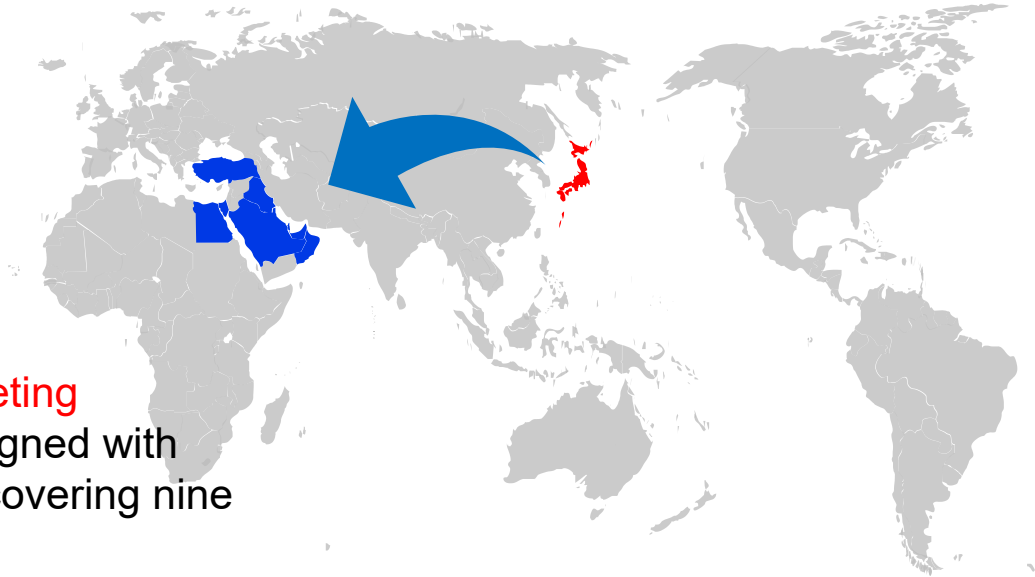
Regenerative CDMO Subsidy

- 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

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

Givinostat: Non-Steroidal Treatment for DMD

1 INN: Givinostat (Brand name: Duvyzat)

- Histone deacetylase (HDAC) inhibitor
- Oral, non-steroidal therapy (twice daily dosing)

2 Overseas indication

- DMD patients ≥6 years of age
  (EU: Ambulatory patients ≥6 years on concomitant steroid therapy)

3 Approval status

- US: Approved (Mar 2024)
- EU: Conditional approval (Jun 2025)
- Approved in several other countries, including the UK
- Not approved in Japan

4 Key features

- Multiple epigenetic disease-modifying effects, enabling mutation-agnostic use in DMD
- Add-on use with steroid therapy

DMD, duchenne muscular dystrophy; INN, international nonproprietary name

Role of HDACs in Regulating Muscle Homeostasis

HDACs help mediate muscle homeostasis via **cytoplasmic** and **nuclear** activity[1,2]

HDACs regulate cellular homeostasis by acting on both histone and non-histone proteins[4]

- Reduces transcriptional accessibility[5]
- Regulates protein stability and localization, transcription factors, hormone receptors, mitochondrial proteins, enzymatic activity, mRNA stability[4]

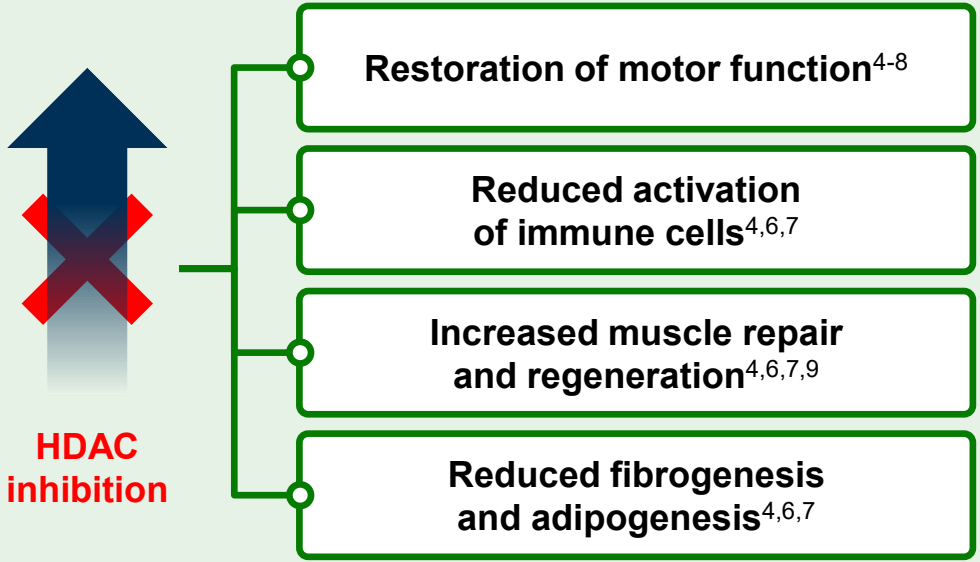
*HDAC and HAT work in balance to regulate the expression of muscle repair factors.

HAT, histone acetyltransferase; HDAC, histone deacetylase; mRNA, messenger ribonucleic acid; RNA, ribonucleic acid.

1. Consalvi S, et al. *Mol Med.* 2011;17(5-6):457-465. **2.** Kodippili K, et al. *Front Physiol.* 2023;14:1180980. **3.** Sandonà M, et al. *Int J Mol Sci.* 2023;24(5):4306. **4.** Milazzo G, et al. *Genes.* 2020;11(5):556. **5.** Ceccacci E, et al. *Br J Cancer.* 2016;114(6):605-11.

HDAC Inhibition Targets the Pathophysiology in DMD[1,2]

HDAC inhibition Counteracts the Pathological Events in DMD[1,3]



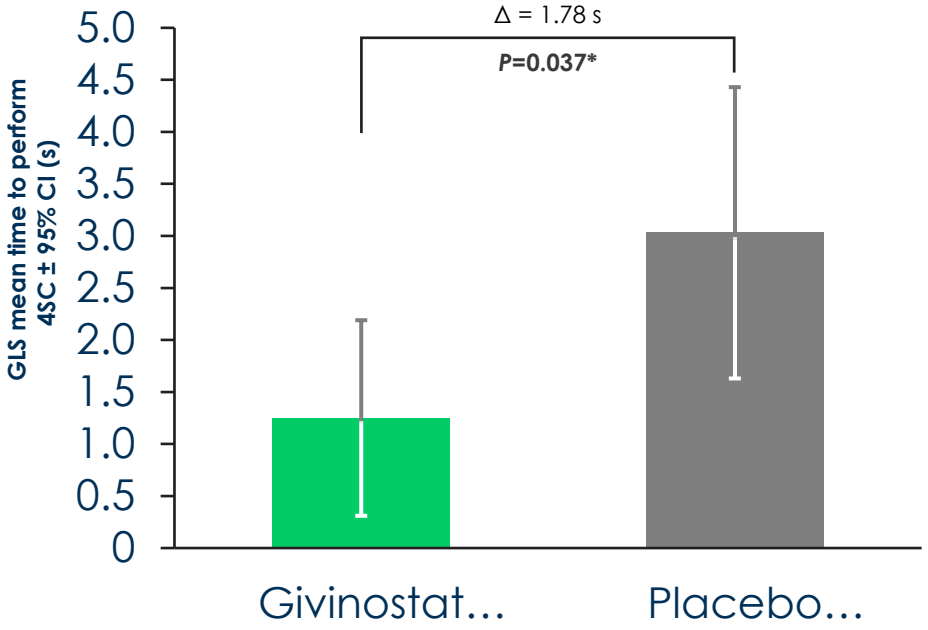
DMD, Duchenne muscular dystrophy; HDAC, histone deacetylase.

1. Consalvi S, et al. *Mol Med.* 2011;17(5–6):457-465. **2.** Kodippili K, et al. *Front Physiol.* 2023;14:1180980. **3.** Sandonà M, et al. *Int J Mol Sci.* 2023;24(5):4306. **4.** Wilson DGS, et al. *Commun Biol.* 2022;5(1):1022. **5.** Campbell KP, et al. *Nature.* 1989;338(6212):259-262. **6.** Guiraud S, et al. *Exp Physiol.* 2015;100(12):1458-1467. **7.** Reid AL, et al. *Life.* 2021;11(7):648. **8.** Ervasti JM, et al. *J Cell Biol.* 1993;122(4):809-823. **9.** Sandonà M et al. *EMBO Rep.* 2020;21(9):e50863.

EPIDYS Study: Primary Endpoint—4SC at Week 72[1]

- At week 72, givinostat plus corticosteroids reduced the decline in time to perform the 4SC test by 1.78 s when compared with placebo plus corticosteroids

Mean change from baseline in time to perform the 4SC test at 72 weeks (non-log transformed)

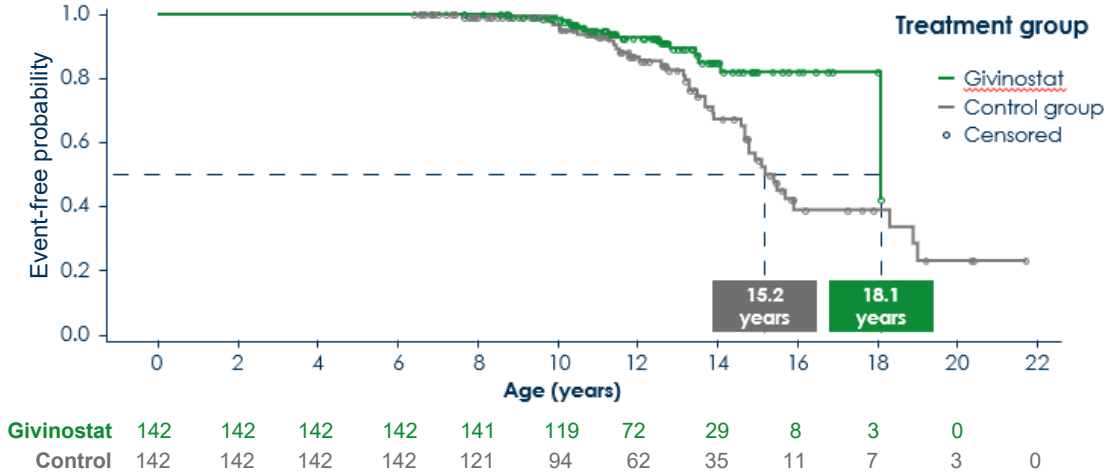


*Data are means and 95% confidence intervals. The confidence intervals have not been adjusted for multiplicity and should not be used for hypothesis testing. Baseline mean values were 3.39 s and 3.48 s for the givinostat and placebo groups, respectively. All patients were also receiving systemic corticosteroids in a dose and regimen that was to remain unchanged over the follow-up period.
4SC, 4-stair climb; GLS, geometric least squares; s, seconds.

1. Mercuri E et al. *Lancet Neurol*. 2024;23(4):393-403.

Givinostat and Permanent Loss of Ambulation[1,2]

- Patients receiving givinostat plus corticosteroids (SoC) preserved their ability to walk for an additional 2.9 years (HR, 0.42; 95% CI, 0.23-0.76; *P*=0.004) compared with patients receiving SoC alone



Parameter	Givinostat (n=142)	Control (n=142)
Patients, n (%)		
Assessed	142 (100)	142 (100)
Who lost ambulation	14 (9.9)	39 (27.5)
Censored	128 (90.1)	103 (72.5)
Age at loss of ambulation, years		
Median (95% CI)	18.1 (18.09, NE)	15.2 (14.70, 18.31)
P-value	0.004	
HR (95% CI)*	0.42 (0.23, 0.76)	

*HR and associated 95% CI and *P* value are obtained from a Cox proportional hazards model, including the treatment group as an independent classification factor.
HR, hazard ratio; NE, not estimable; SoC, standard of care.

1. McDonald CM, et al. *Ann Clin Transl Neurol*. Published online August 19, 2025. 2. Post hoc analysis comparing with natural history disease studies, using data that including the EPIDYS study

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