Life is Rare

FY2026 Second Quarter Results Briefing Session

October 29, 2026

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JCR Pharmaceuticals Life is Rare

Toru Ahida

Representative Director, Chairman

FY2026 Q2 Financial Results Highlights

(million yen)

	FY2025	FY2026	
	Q2 YTD	Q2 YTD	Progress Rate
Net sales	21,362	**19,974**	**43.7%**
SG&A	14,659	**17,726**	**52.2%**
of which: R&D expenses	7,835	**10,230**	**52.7%**
Operating profit (loss)	2,429	**(3,504)**	**-** *Full-year forecast: 1,100 million yen

Net sales

Net sales declined YoY on lower contract revenue. Full-year forecast remains the target despite slower progress

- Excluding Contract revenue, sales remain on track, supported by strong IZCARGO™ performance despite slower GROWJECT™ sales
- No contract revenue was recorded in Q2. Targeting the full-year forecast with contract revenue expected from Q3 onward

SG&A

SG&A rose sharply YoY, mainly due to higher development costs, with a higher rate of budget utilization

- R&D expenses may exceed the full-year forecast, but efforts will focus on keeping overall SG&A close to plan

Building the Future Through Three Established Revenue Pillars

Toward ¥100 billion revenue in the 2030s

Enabled by three growth drivers

Domestic Sales

Expansion of domestic product sales and revenue growth from in-licensed products

Platform licensing revenue

Growth in licensing revenue through out-licensing of platform technologies

Overseas revenue (LSD pipeline)

Expansion of overseas revenue through out-licensing of the LSD pipeline

LSD, lysosomal storage disorders

Life is Rare

Hiroyuki Sonoda Ph.D.

Representative Director,
President

Givinostat: Accelerating the Development Timeline Ahead of the Original Plan

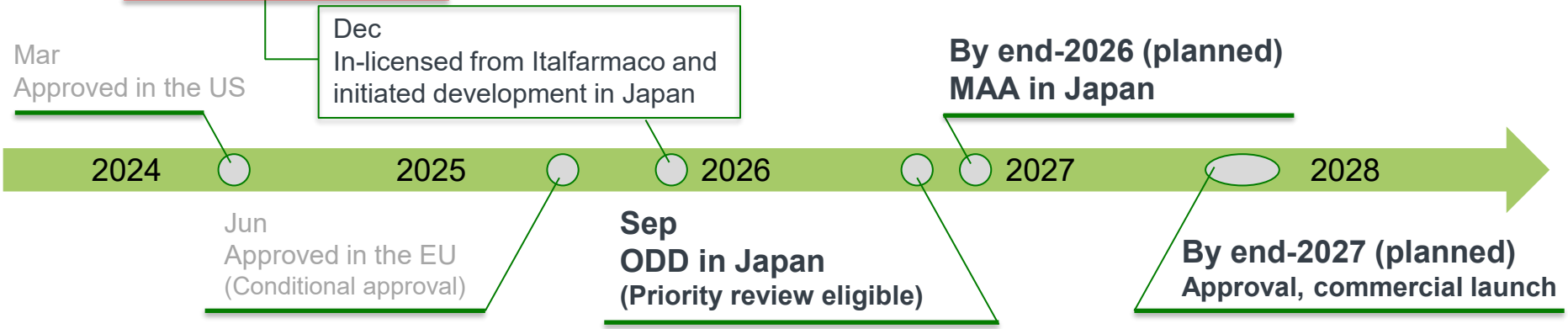
givinostat

Histone deacetylase (HDAC) inhibitor

Potential indication: Duchenne muscular dystrophy (DMD)

Domestic Sales

- Novel mechanism of action distinct from existing therapies and **applicable regardless of genetic mutation status**
- Met the primary endpoint in a placebo-controlled Phase III study and is already approved in multiple countries
- Drug lag persists in Japan



On track for a MAA in 2026 based on overseas clinical data

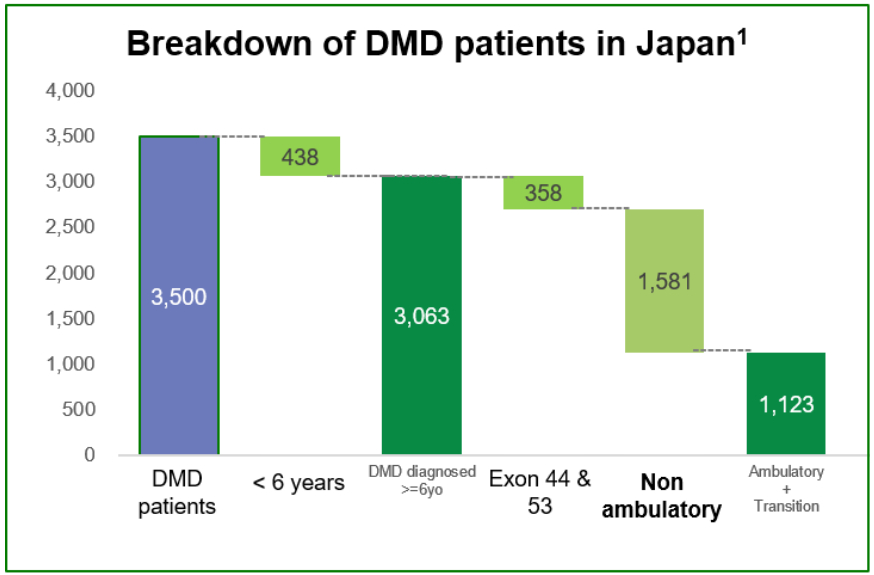
DMD, duchenne muscular dystrophy; HDAC, histone deacetylase; MAA, marketing authorization application; ODD, orphan drug designation.

Givinostat: Potential for Domestic Revenue Growth

givinostat

Overseas Indication
US; ≥6 years
EU; Ambulatory patients ≥6 years on concomitant steroid therapy

Domestic Sales



‘Ambulatory patients ≥6 years’
≥1,000 patients

Treatment cost; **JPY 50 million/year**[2]
Assuming **70%** of eligible patients receive treatment

Estimated Revenue
JPY 35 Billion

Oct 2026 First DMD patient enrolled in a 20-patient study in Japan
(10 ambulatory and 10 non-ambulatory patients planned)

DMD, duchenne muscular dystrophy; JPY, Japanese Yen
1. Company estimated based on ‘Kawai M. *No To Hattatsu*. (Japanese) 2013;45(Supple.):S324’, ‘Remudy (Registry of Muscular Dystrophy)’ and ‘Nakamura H et al. Orphanet J Rare Dis. 2013;8:60’
2. Estimated based on overseas treatment costs of givinostat (Company analysis)

Application of J-Brain Cargo® to Antibody Therapeutics

Platform licensing revenue

Jul 2025

Entered into a license agreement with Acumen that includes option right

- Granted Acumen an exclusive option for worldwide development, manufacturing, and commercialization
- Eligible to receive up to USD 555 million in milestone payments

Development of an Alzheimer's disease therapy applying J-Brain Cargo® to an AβO-selective antibody

Jun 2026: Acumen exercised option right

Mid-2027: Planned IND submission

BBB, blood-brain barrier; IND, investigational new drug

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

IZCARGO™: Marketing Authorization Obtained in the Middle East

IZCARGO™ (Code: JR-141)

- MPS II enzyme replacement therapy utilizing J-Brain Cargo®, a proprietary BBB-penetrating technology
- Approved in Japan and launched in 2021

Overseas revenue (LSD pipeline)

Entered into a marketing and distribution agreement with Taiba Middle East FZ LLC to expand business utilizing Japanese marketing authorization

Obtained marketing authorization in two countries

Jul 2026 UAE
Oct 2026 Kuwait (Conditional approval)

Seek marketing authorization for IZCARGO™ in countries across the MENA region, with Taiba responsible for sales and distribution in the relevant territories

BBB, blood-brain barrier; MENA, Middle East and North Africa; UAE, United Arab Emirates

JR-141: Global Commercialization Partnership with Italfarmaco

Overseas revenue (LSD pipeline)

Oct 2026

Entered into an exclusive license agreement with Italfarmaco for JR-141

- Granted Italfarmaco an exclusive license to develop and commercialize JR-141 in **the US, Europe, and Latin America**
- Following regulatory approvals, **Italfarmaco will commercialize and distribute** JR-141 in the licensed territories, while **JCR will retain responsibility for manufacturing**
- JCR will receive development and commercial milestones, royalty payments, and revenues associated with product supply

Strengthening the partnership with Italfarmaco toward future approvals of JR-141 in the US and Europe

Overview of Clinical or Late Preclinical Pipeline

Code	Potential 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 • Partnered with Italfarmaco
JR-142	Pediatric GHD				Ph3 (Japan)	• Patient enrollment completed
JR-401G	Pediatric GHD				Ph3 (Japan)	• Apr 2026: Initiation of first dosing in Ph3 • Dose comparison study of GROWJECT™ aimed at changing the approved dosage
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)		• Ph1/2, Ph1: Patient enrollment completed • Actively pursuing early approval in Japan
			Ph1 (Japan)			
JR-446	MPS IIIB (Sanfilippo syndrome type B)			Global Ph1/2		• Sep 2026: Initiation of first dosing in global Ph1/2 study • Discussion on going to assess early approval in Japan • Partnered with MEDIPAL HOLDINGS
				Ph1/2 (Japan)		
JR-471	Fucosidosis					• Global natural history study ongoing • Partnered with MEDIPAL HOLDINGS
JR-479	GM2 gangliosidosis (Tay-Sachs disease, Sandhoff disease)					• Partnered with MEDIPAL HOLDINGS
givinostat	Duchenne muscular dystrophy	Progressing preparations for a marketing authorization application by the end-2026				• Obtain approval and launch in Japan in 2027

JCR Positioned to Benefit from Japan's National Growth Strategy

- Japan's Growth Strategy, approved by the Cabinet in July 2026, identified 17 strategic fields for public and private investment, including "Synthetic Biology & Biotechnology" and "Drug Discovery & Advanced Medical Care"

※Created by Company based on sources 1-3

Key Initiatives under Japan's Growth Strategy Synthetic Biology & Biotechnology / Drug Discovery & Advanced Medical Care※	Areas Aligned with JCR	JCR's Strengths and Initiatives
Support for R&D in rare and intractable diseases	① Rare diseases	• R&D focused on rare diseases
Support for drug discovery platform technologies Enhanced collaboration among academia, pharmaceutical companies, and startups	② Drug discovery platforms technologies	• Proprietary technologies such as J-Brain Cargo® • Innovative initiatives in gene therapy, regenerative medicine, and cell therapy
Reducing delays and gaps in access to innovative medicines in Japan	③ Patient access to innovative medicines	• Introduction of a DMD therapy in Japan • Regulatory filing based solely on overseas clinical data
Strengthening domestic manufacturing capabilities for biopharmaceuticals	④ Domestic biopharmaceutical manufacturing	• Expansion of biopharmaceutical manufacturing capacity supported by government subsidies (new facilities, contract manufacturing, etc.)

JCR's business strategy is strongly aligned with the direction of national policy

1. Japan's Growth Strategy (Cabinet Decision, July 21, 2026) 2. Public-Private Investment Roadmap for Key Products and Technologies across the 17 Strategic Fields (Japan Growth Strategy Headquarters Decision, July 21, 2026) 3. Minutes of the 5th Drug Discovery & Advanced Medical Care Working Group Meeting (April 6, 2026)

Toward ¥100 billion revenue in the 2030s

Enabled by three growth drivers

Domestic Sales

Expansion of domestic product sales and revenue growth from in-licensed products

Platform licensing revenue

Growth in licensing revenue through out-licensing of platform technologies

Overseas revenue (LSD pipeline)

Expansion of overseas revenue through out-licensing of the LSD pipeline

LSD, lysosomal storage disorders

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Appendix

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FY2026

- Q2 Consolidated Financial Results
- Full-Year Consolidated Financial Forecasts

Overview: Consolidated Financial Results (Q2 FY2026)

(million yen)	FY2025 Q2 YTD	FY2026 Q2 YTD	Year-on-year Difference	Year-on-year Ratio	Progress Rate
Net Sales	21,362	**19,974**	(1,388)	(6.5)%	43.7%
Cost of Sales	4,273	**5,752**	+1,479	+34.6%	54.1%
Gross Profit	17,089	**14,221**	(2,867)	(16.8)%	40.5%
Selling, General and Administrative Expenses	14,659	**17,726**	+3,066	+20.9%	52.2%
SG&A Expenses	6,824	**7,496**	+671	+9.8%	51.5%
R&D Expenses	7,835	**10,230**	+2,395	+30.6%	52.7%
Operating profit	2,429	**(3,504)**	(5,934)	-	-
Non-operating Income	353	**232**	(121)	(34.3)%	-
Non-operating Expenses	370	**526**	+155	+42.1%	-
Ordinary profit	2,413	**(3,798)**	(6,211)	-	-
Extraordinary Income	209	**-**	(209)	(100.0)%	-
Extraordinary Losses	31	**10**	(20)	(66.6)%	-
Profit before Income Taxes	2,591	**(3,808)**	(6,400)	-	-
Income Taxes	845	**59**	(786)	(93.0)%	-
Profit Attributable to Owners of Parent	1,746	**(3,867)**	(5,613)	-	-
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	8,226	11,656	+3,430	+41.7%	47.9%

Additional Remarks

- Net sales declined mainly due to lower contract revenue. Targeting the full-year forecast with revenue expected from Q3 onward.
- Cost of sales ratio excluding contract revenue, rose slightly due to product mix.
- R&D expenses rose sharply YoY but remain broadly on plan.
- Non-operating income decreased mainly because FX gains recorded in the previous fiscal year did not recur in the current period.
- Higher non-operating expenses mainly reflect increased interest expenses and FX losses.

Net Sales	Q2 FY2025 YTD	Q2 FY2026 YTD	Difference
Cost of Sales Ratio	20.0%	**28.8%**	+8.8%
Cost of Sales Ratio *Excluding contract revenue	26.0%	**26.7%**	+0.7%
R&D Expenses Ratio	36.7%	**51.2%**	+14.5%
Operating Profit Ratio	11.4%	**(17.5)%**	(28.9)%

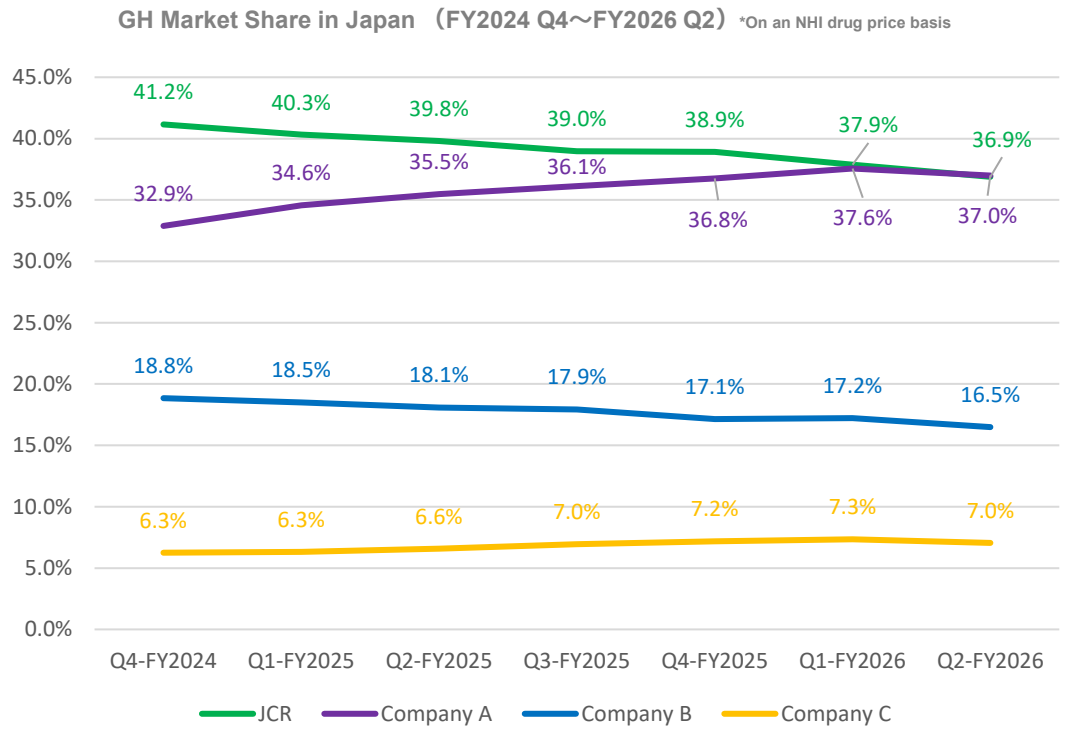
Consolidated Net Sales Breakdown (Q2 FY2026)

(million yen)	FY2025 Q2 YTD	FY2026 Q2 YTD	Year-on-year Difference	Year-on-year Ratio	Progress Rate
GROWJECT™	8,915	**7,888**	(1,026)	(11.5)%	45.0%
IZCARGO™	3,354	**3,634**	+280	+8.4%	52.6%
TEMCELL™ HS Inj.	1,582	**1,323**	(259)	(16.4)%	49.0%
Treatments for renal anemia	1,580	**1,697**	+117	+7.4%	45.1%
Epoetin Alfa BS Inj. [JCR]	296	**460**	+164	+55.6%	31.4%
Darbepoetin Alfa BS Inj. [JCR]	1,283	**1,236**	(46)	(3.7)%	53.8%
Agalsidase Beta BS I.V. Infusion [JCR]	426	**958**	+532	+125.1%	46.1%
Total Core Products	15,858	**15,503**	(354)	(2.2)%	47.0%
Contract revenue	5,015	**1,474**	(3,540)	(70.6)%	18.1%
Other	489	**2,996**	+2,506	+512.4%	-
Total Net Sales	21,362	**19,974**	(1,388)	(6.5)%	43.7%

Additional Remarks

- **GROWJECT™** : Sales declined YoY. The progress rate toward the full-year forecast is low, but efforts remain focused on meeting the forecast.
- **IZCARGO™** : New patient uptake is progressing well, with potential to exceed the full-year plan.
- **TEMCELL™ HS Inj.** : Sales remain on plan.
- **Treatments for renal anemia** : Sales reflect the supply plan for Kissei Pharmaceutical Co., Ltd.
- **Agalsidase Beta BS I.V. Infusion [JCR]** : Sales reflect the supply plan for Sumitomo Pharma Co., Ltd.
- **Contract revenue** : Targeting the full-year forecast with revenue expected from Q3 onward.

GROWJECT™ Market Share Trends in Japan (Quarterly)



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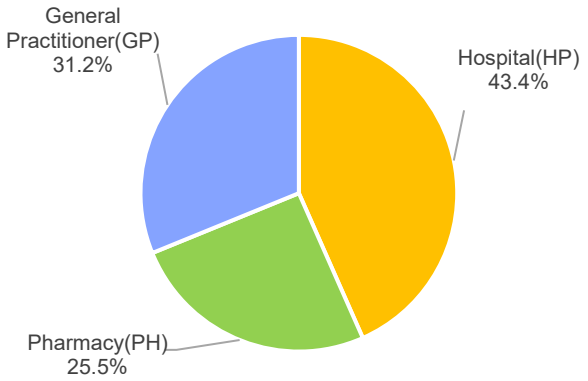
■ Human Growth Hormone Market in Japan



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■ GROWJECT™ Market Share by buyer

	Sep 2026	Sales Change FY2026 Q2 (vs. FY2025 Q2) *On an NHI drug price basis
HP Market	30.4%	-127 million yen
PH Market	26.7%	-66 million yen
GP Market	54.9%	-152 million yen

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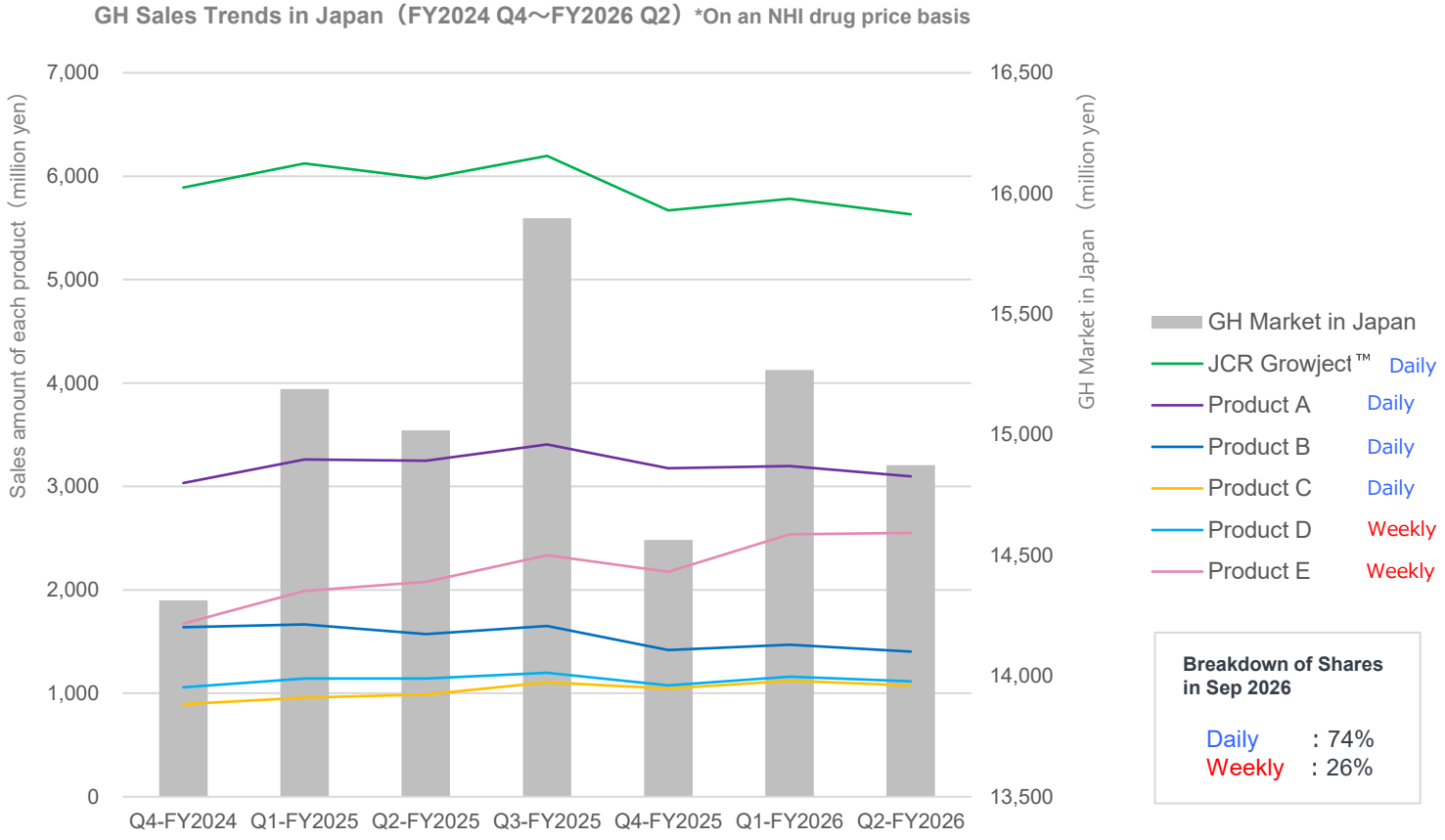
GROWJECT™ Sales Trends in Japan (Quarterly)



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GROWJECT™ Trends of Market Share in Japan

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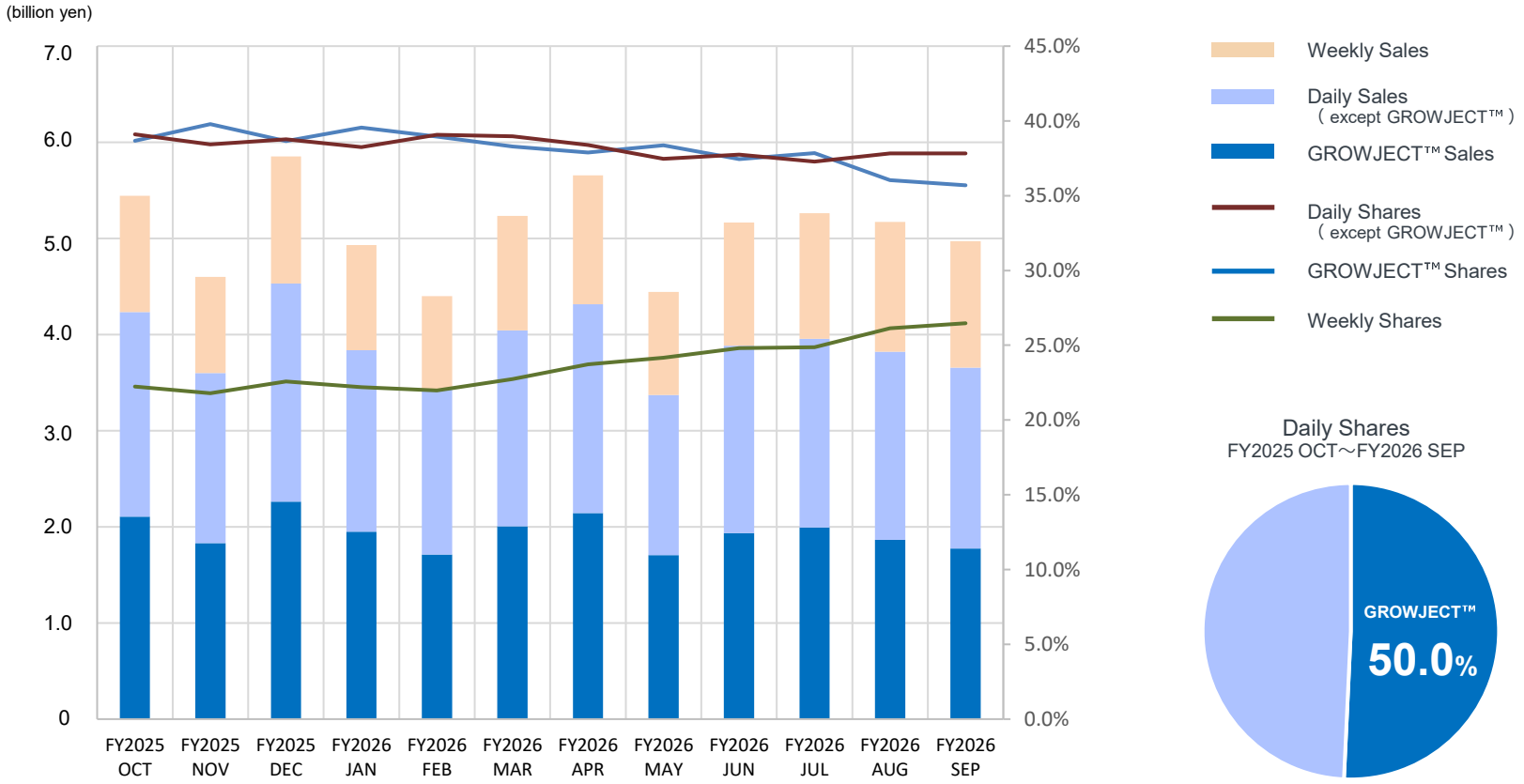

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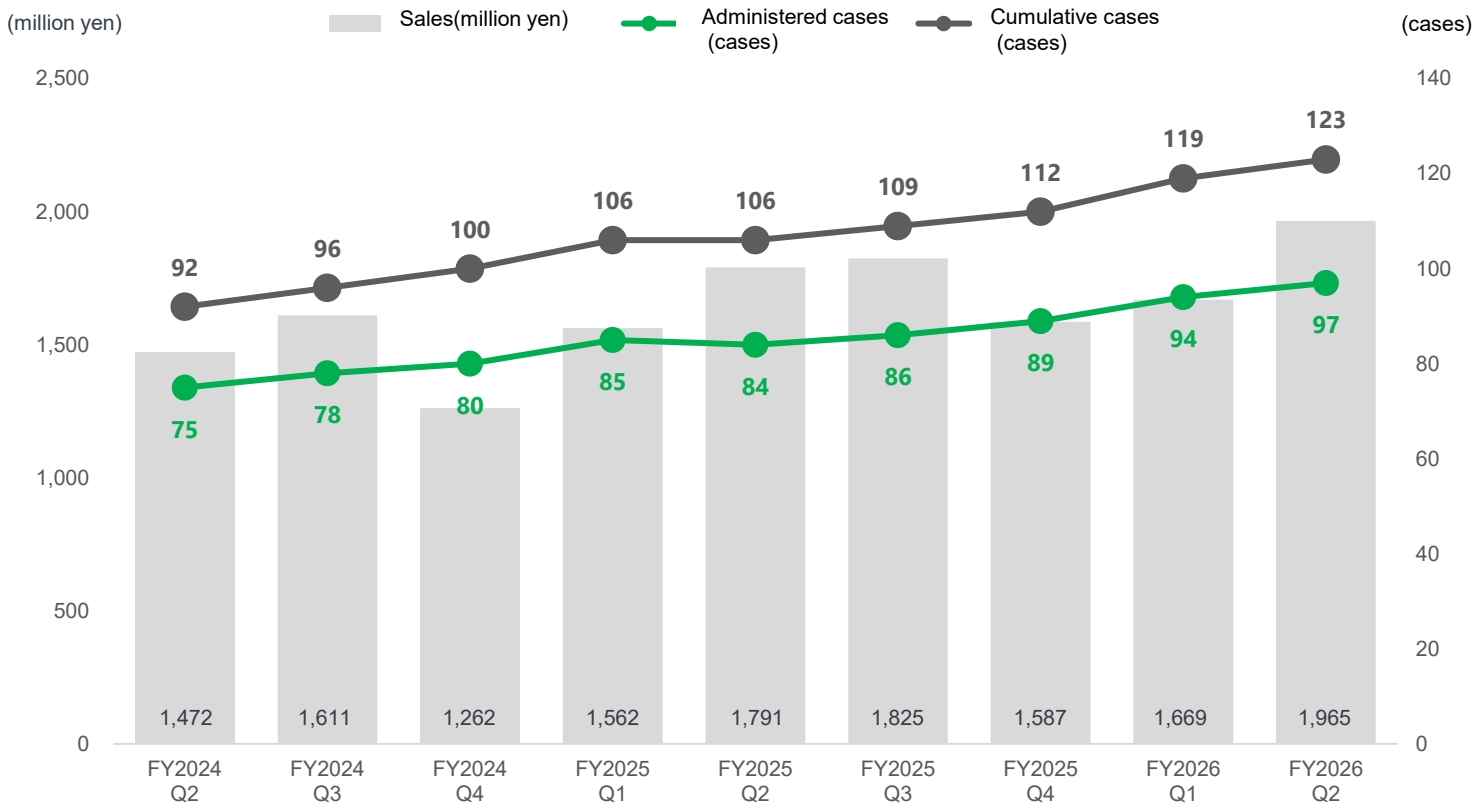
IZCARGO™ Prescription Status in Japan



Financial Status (Consolidated)

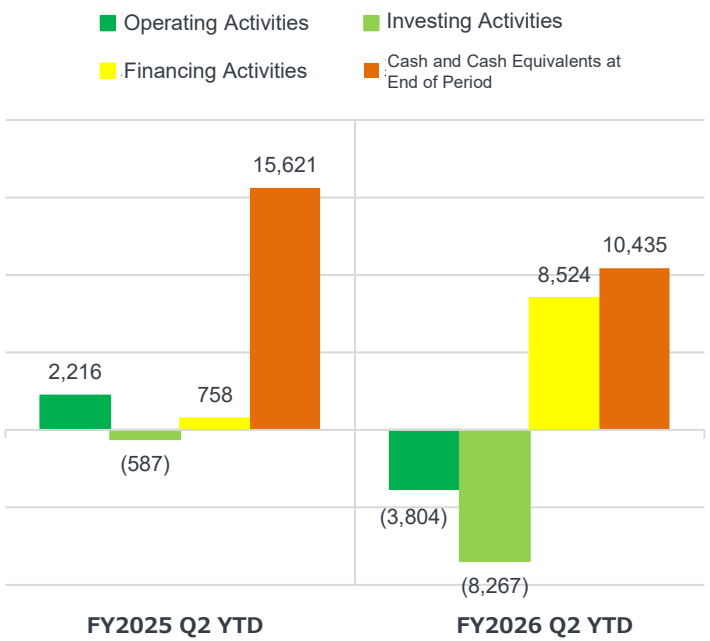
(million yen)	End-Mar. 2026	End-Sep. 2026	Change • Main Increase/decrease		End-Mar. 2026	End-Sep. 2026	Change • Main Increase/decrease
Current assets	56,076	**54,667**	**(1,408)** • Cash and deposits (3,571) • Accounts receivable - trade, and contract assets +878 • Inventories +943	**Current liabilities**	48,135	**59,080**	**+10,945** • Short-term borrowings +10,114
				Non-current liabilities	13,741	**13,406**	**(335)** • Long-term borrowings (350)
Non-current assets	53,160	**59,209**	**+6,049** • Construction in progress +5,341	**Total liabilities**	61,877	**72,487**	**+10,610**
				Total net assets	47,359	**41,389**	**(5,969)** • Dividends paid (1,222) • Net loss (3,867)
Total	109,236	**113,876**	**4,640**	**Total**	109,236	**113,876**	**4,640**

Additional Remarks

- Current assets : Receivables and inventories increased, cash and deposits decreased.
- Non-current assets : Construction in progress increased due to the new formulation plant.
- Current liabilities : Short-term borrowings increased, including bridge financing pending government subsidies for the new formulation plant.

	End-Mar.2026	End-Sep.2026
Equity ratio	42.9%	**35.9%**

Cash Flows (Consolidated)



(million yen)	FY2025	FY2026	
	Q2 YTD	Q2 YTD	Full-year (Forecast)
Depreciation	1,459	1,081	2,000
Capital investment	792	7,799	10,700

Depreciation declined YoY as government subsidies reduced the carrying amount of depreciable assets.

(million yen)	FY2025 Q2 YTD	FY2026 Q2 YTD
Profit before income taxes	2,591	**(3,808)**
Depreciation	1,459	**1,081**
Decrease (increase) in trade receivables and accounts receivable – other	(897)	**(972)**
Decrease (increase) in inventories	(1,706)	**(943)**
Increase (decrease) in trade payables and accounts payable – other	144	**1,347**
Income taxes paid	783	**(242)**
Other	(159)	**(266)**
Operating Activities	2,216	**(3,804)**
Capital investment	(792)	**(7,799)**
Other	204	**(467)**
Investing Activities	(587)	**(8,267)**
Bollowings	2,000	**9,764**
Dividends paid/ treasury shares	(1,214)	**(1,218)**
Other	(27)	**(21)**
Financing Activities	758	**8,524**
Net increase (decrease) in cash and cash equivalents	2,425	**(3,571)**
Cash and Cash Equivalents at End of Period	15,621	**10,435**

FY2026 Consolidated Financial Forecasts

No Change from Initial Forecast

(million yen)	FY2025 Results	FY2026 (Forecast) Forecast	Year-on-year Difference	Year-on-year Ratio
Net Sales	40,319	**45,700**	+5,381	+13.3%
Cost of Sales	10,134	**10,600**	+466	+4.6%
Gross Profit	30,185	**35,100**	+4,915	+16.3%
Selling, General and Administrative Expenses	29,629	**33,900**	+4,271	+14.4%
SG&A Expenses	12,867	**14,500**	+1,633	+12.7%
R&D Expenses	16,761	**19,300**	+2,539	+15.1%
Operating Profit	555	**1,100**	+545	+98.2%
Ordinary Profit	1,165	**500**	(665)	(57.1)%
Profit Attributable to Owners of Parent	2,178	**200**	(1,978)	(90.8)%
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	18,398	**24,300**	+5,902	+32.1%

Additional Remarks

- Net sales are forecast to exceed the previous fiscal year, mainly due to higher contract revenue.
- SG&A expenses are forecast to increase, mainly due to higher fees and commissions. R&D expenses are also forecast to rise, reflecting progress in global and domestic clinical trials.
- Operating profit is forecast to increase, as higher sales will offset higher expenses.
- Ordinary profit and profit attributable to owners of parent are forecast to decrease, as foreign exchange gains, gains on sales of securities, and subsidy income recorded in the previous fiscal year are not expected this fiscal year.

Net Sales	FY2025	FY2026 (Forecast)	Difference
Cost of Sales Ratio	25.1%	**23.2%**	(1.9)%
Cost of Sales Ratio *Excluding contract revenue	27.6%	**26.7%**	(0.9)%
R&D Expenses Ratio	41.6%	**42.4%**	+0.8%
Operating Profit Ratio	1.4%	**2.6%**	+1.2%

Breakdown of Net Sales – FY2026 Consolidated Financial Forecasts

No Change from Initial Forecast

(million yen)	FY2025 Results	FY2026 (Forecast) Forecast	Year-on-year Difference	Year-on-year Ratio
GROWJECT™	17,933	**17,500**	(433)	(2.4)%
IZCARGO™	6,766	**6,900**	+134	+2.0%
TEMCELL™HS Inj.	2,831	**2,700**	(131)	(4.6)%
Treatments for renal anemia	3,622	**3,700**	+78	+2.2%
Epoetin Alfa BS Inj. [JCR]	1,121	**1,400**	+279	+24.9%
Darbepoetin Alfa BS Inj. [JCR]	2,501	**2,300**	(201)	(8.0)%
Agalsidase Beta BS I.V. Infusion [JCR]	1,292	**2,000**	+708	+54.8%
Total Core products	32,446	**32,900**	+454	+1.4%
Contract revenue	5,549	**8,100**	+2,551	+46.0%
Other	2,323	**4,500**	+2,177	+93.7%
Total net sales	40,319	**45,700**	+5,381	+13.3%

Additional Remarks

- GROWJECT™ sales are forecast to decrease due to NHI drug price revisions, despite sales volume remaining largely unchanged.
- IZCARGO™ sales are forecast to continue increasing, driven by an increase in treated patients.
- TEMCELL™ sales are forecast to decrease due to changes in the competitive environment.
- Renal anemia products and Agalsidase Beta BS I.V. Infusion [JCR] are forecast to remain solid, with sales in line with supply plans for distribution partners.
- Contract revenue is forecast to exceed the previous fiscal year, driven by new product out-licensing agreements and progress in existing joint research projects.

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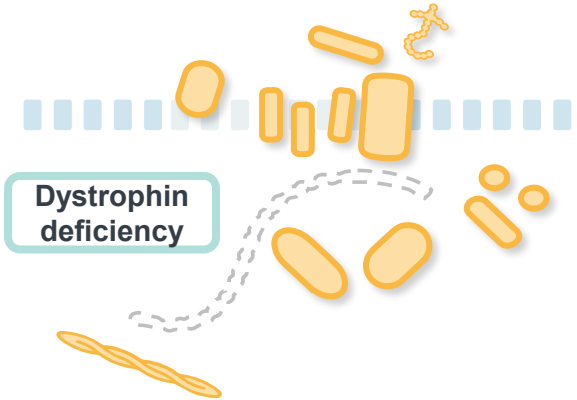
Givinostat

Pathological Hallmarks of DMD

Dystrophin deficiency and disrupted HDAC activity **mediate the regulation of muscle homeostasis**[1-3]



Pathological events associated with HDAC upregulation[3]

HDAC upregulation

- Muscular atrophy[4-8]
- Activation of chronic inflammatory pathways[4,6,7]
- Impairment of muscle repair mechanisms[4,6,7]
- Fibrogenesis and adipogenesis[4,6,7]

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, Alexander MS. *Life.* 2021;11(7):648; **8.** Ervasti JM, et al. *J Cell Biol.* 1993;122(4):809-823.

Givinostat is a potent pan-HDAC inhibitor

Inhibition of the upregulated HDAC in DMD **redresses the imbalance in muscle homeostasis**, leading to[1,2]:

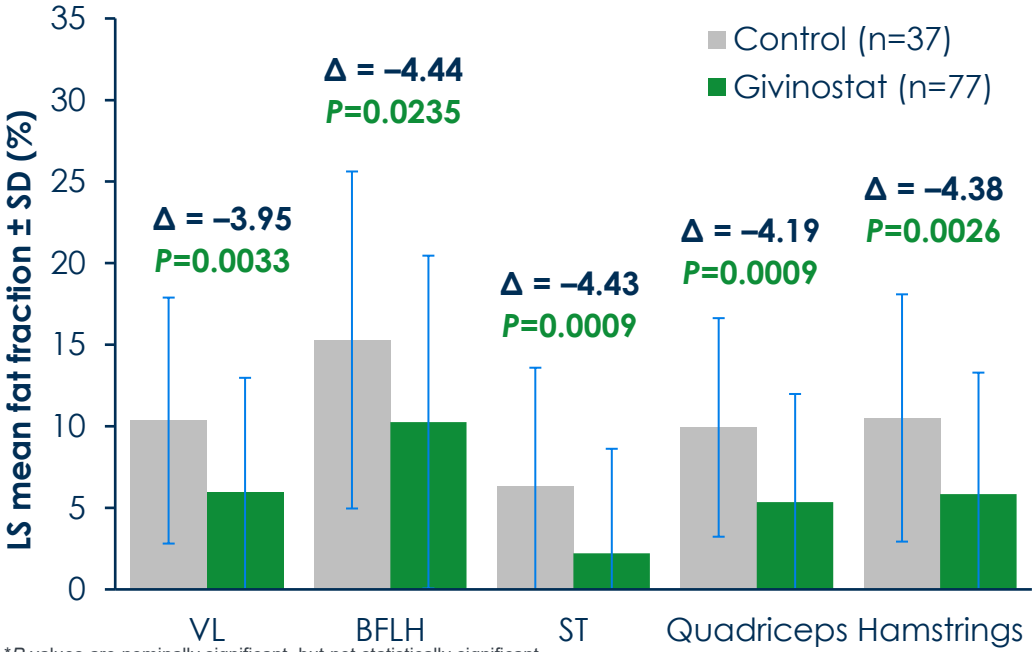
- **Increased** muscle repair and regeneration mechanisms[3,4]
- **Reduced** fibrosis and fat deposition[3]
- **Delayed** disease progression with preservation of functional capabilities[5,6]

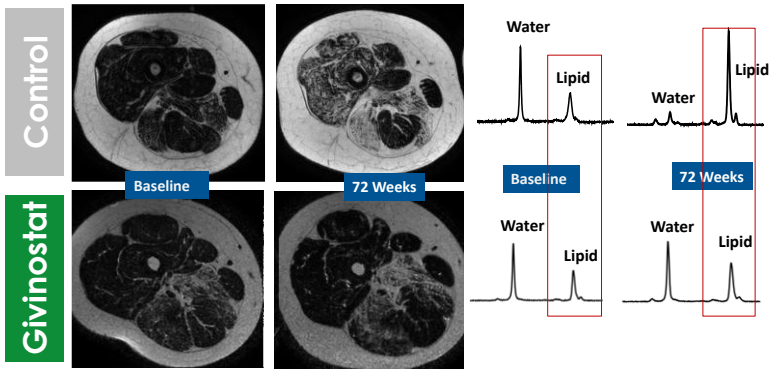
DMD, Duchenne muscular dystrophy; HDAC, histone deacetylase.

1. Consalvi S et al. *Mol Med.* 2011;17(5-6):457-465. **2.** Kodippili K, Rudnicki MA. *Front Physiol.* 2023;14:1180980. **3.** Bettica P et al. *Neuromuscul Disord.* 2016;26(10):643-649. **4.** Sandonà M et al. *EMBO Rep.* 2020;21(9):e50863.
5. Mercuri E et al. *Lancet Neurol*. 2024;23(4):393-403. **6.** ClinicalTrials.gov. NCT02851797. Updated 2 February 2023. Available at https://clinicaltrials.gov/study/NCT02851797. Accessed August 1, 2024.

Givinostat reduced fat fraction in 5 key muscles important for ambulation comprising the quadriceps and hamstrings groups compared with control at 72 weeks

Fat fraction in quadriceps and hamstrings muscles*





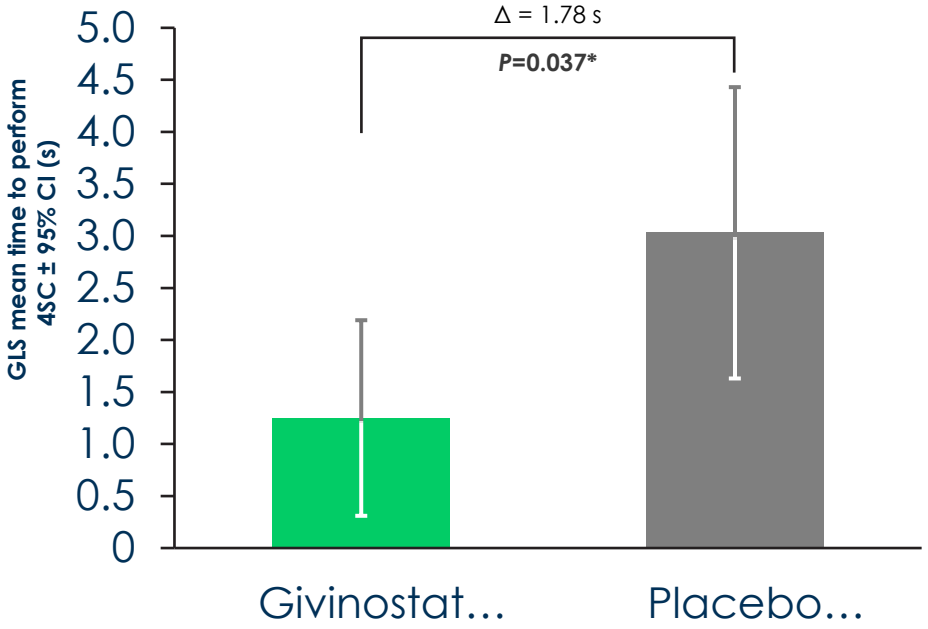
The images on the slide are from a single patient and may not be representative of all patients in EPIDYS.

*P-values are nominally significant, but not statistically significant.
BFLH, biceps femoris long head; LS, least squares; SD, standard deviation; ST, semitendinosus; VL, vastus lateralis.
Vandenborne K. Oral presentation at Muscular Dystrophy Association Clinical & Scientific Conference; March 19-22, 2023; Dallas, TX, USA.

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)

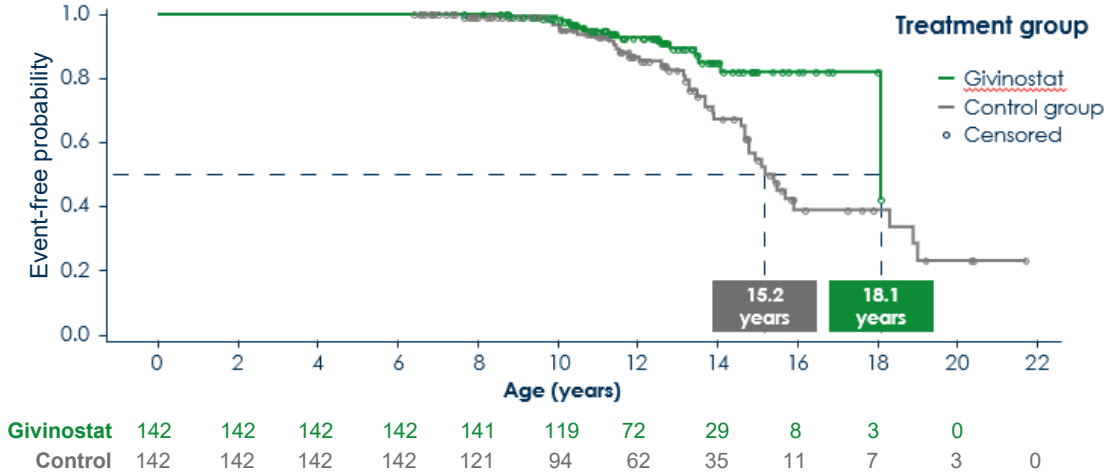


*Data are means and 95% confidence intervals. The confidence intervals have not been adjusted for multiplicity and should not be used for hypothesis testing. Baseline mean values were 3.39 s and 3.48 s for the givinostat and placebo groups, respectively. All patients were also receiving systemic corticosteroids 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

Unmet Medical Needs in DMD Treatment

Only two therapies approved in Japan (excluding steroid therapy)

	Approval status			Target DMD Patients[1]
	Japan	US	EU	
Gene therapy	✓[2]	✓	—	Japan; Negative for anti-AAVrh74 antibodies Ambulatory patients 3 years to less than 8 years
Exon skipping	✓[2]	✓	—	Japan; Patients with specific genetic mutations[3]

Eligible patients remain limited by age and genetic mutation criteria, leaving substantial unmet medical needs in DMD

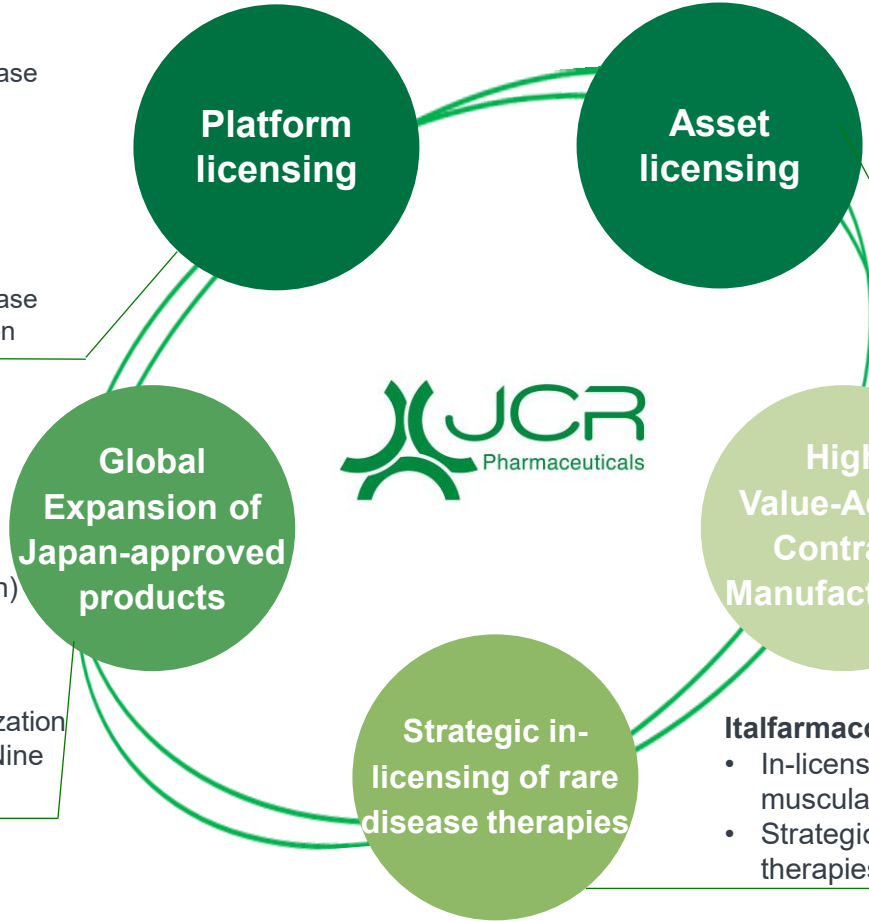
givinostat	—	✓	✓[2]	**US;** **≥6 years** **EU;** **Ambulatory patients ≥6 years on concomitant steroid therapy**

AAV, adeno associated virus; DMD, duchenne muscular dystrophy

1. Based on labels for each product 2.Conditional approval 3. Patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping

Others

Driving Value through Strategic Alliances



J-Brain Cargo®
- Alexion, AstraZeneca Rare Disease (Neurodegenerative disease, oligonucleotide therapeutics)
- Angelini Pharma (Epilepsy)
- Acumen Pharmaceuticals, Inc. (Alzheimer's disease)

JUST-AAV
- Alexion, AstraZeneca Rare Disease
- Modalis Therapeutics Corporation

Italfarmaco S.p.A.
- Out-licensing of JR-141 (the US, Europe, and Latin America)

MEDIPAL HOLDINGS CORPORATION
- Out-licensing of JR-471, JR-446, and JR-479

Taiba Middle East FZ LLC
- Marketing and distribution of IZCARGO™

(Countries across the MENA region)

Menagen Pharmaceutical Industries LLC
- Out-licensing of local marketing authorizations and commercialization rights for Agalsidase Beta BS (Nine MENAT markets)

SanBio Co., Ltd.
- Contract manufacturing for pilot production toward commercial manufacturing of Akuugo®

Italfarmaco S.p.A.
- In-licensing of givinostat for duchenne muscular dystrophy
- Strategic collaboration in rare disease therapies

Italfarmaco S.p.A: Licensor of Givinostat

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 oncology, neurology, and cardiovascular disease
- Rare disease unit includes programs for Duchenne muscular dystrophy, ALS and Polycythemia vera

Employees
>4000

Business
>90 countries

Manufacturing
6 sites
(GMP-compliant)

Gross sales
€ 1.1B
(2024)

ALS, amyotrophic lateral sclerosis

GROWJECT™

Indication	Dosage
Short stature due to growth hormone deficiency without epiphyseal closure	0.175 mg/kg/week
Short stature associated with Turner syndrome without epiphyseal closure	0.35 mg/kg/week
Adult growth hormone deficiency (limited to severe cases)	0.021~0.084 mg/kg/week
Short stature due to small for gestational age (SGA) without epiphyseal closure	0.23~0.47 mg/kg/week
Short stature associated with SHOX deficiency without epiphyseal closure	0.35 mg/kg/week

For daily growth hormone products, dose adjustment according to patient treatment response is permitted outside of Japan
(In the US, dose adjustment is allowed within the range of 0.175–0.3 mg/kg/week)

To bridge the gap between Japanese and international dosing practices and to improve final adult height and quality of life (QOL), JCR has initiated development of an adjustable dosing regimen for growth hormone therapy in Japan

JR-471: Initiation of the Natural History Study

JR-471

BBB-penetrating α-L-fucosidase (rDNA origin)
Potential indication: Fucosidosis

- **Fucosidosis**
  - Etiology: Caused by reduced activity of the metabolic enzyme α-L-fucosidase
  - Clinical features: psychomotor symptoms, muscle hypotonia, visceromegaly, and skeletal abnormalities, etc.
  - An ultra-rare disease with **fewer than 120 reported cases worldwide**
  - **No approved therapies available**

Given the extremely limited existing clinical data, a global natural history study has been initiated to accumulate data

Overview	
Study title	• A Retrospective and Prospective Natural History Study of Patients With Fucosidosis
Objective	• To learn more about fucosidosis, its symptoms, and how it develops over time
Study period	• Mar 2026 ～ Jan 2031（planned）
Target number of patients	• 57
Countries	• Planned to be conducted in the U.S. and multiple other countries
Clinical Trials.gov	• NCT07615400

Abbreviations

AAV	Adeno-associated virus	アデノ随伴ウイルス
BBB	Blood-brain barrier	血液脳関門
CRO	Contract Research Organization	開発業務受託機関
DMD	Duchenne muscular dystrophy	デュシェンヌ型筋ジストロフィー
GHD	Growth hormone deficiency	成長ホルモン分泌不全性低身長症
HDAC	Histone deacetylase	ヒストン脱アセチル化酵素
IND	Investigational New Drug	新薬の臨床試験開始のための規制当局への申請またはその制度
i.v.	Intravenous injection	静脈注射
LSD	Lysosomal storage disorders	ライソゾーム病
MENA	Middle East and North Africa	中東および北アフリカ
MPS	Mucopolysaccharidosis	ムコ多糖症
NHI	National health insurance	-
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
PK	Pharmacokinetics	薬物動態
R&D	Research and development	研究開発
YTD	Year to date	年度累計