Life is Rare

FY2026 First Quarter Results Briefing Session

July 30, 2026

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- 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.

Overview: Consolidated Financial Results (Q1 FY2026)

Life is Rare

(million yen)	FY2025 Q1 YTD	FY2026 Q1 YTD	Year-on-year Difference	Year-on-year Ratio	Progress Rate
Net Sales	8,569	**9,700**	+1,130	+13.2%	21.2%
Cost of Sales	2,357	**2,249**	(107)	(4.6)%	21.2%
Gross Profit	6,212	**7,450**	+1,238	+19.9%	21.2%
Selling, General and Administrative Expenses	6,818	**9,554**	+2,736	+40.1%	28.1%
SG&A Expenses	3,469	**3,665**	+195	+5.6%	25.2%
R&D Expenses	3,348	**5,888**	+2,540	+75.9%	30.4%
Operating profit	(606)	**(2,103)**	(1,497)	-	-
Non-operating Income	74	**261**	+186	+249.4%	-
Non-operating Expenses	218	**242**	+23	+11.0%	-
Ordinary profit	(749)	**(2,084)**	(1,334)	-	-
Extraordinary Income	0	**0**	0	0.0%	-
Extraordinary Losses	1	**0**	(1)	(100.0)%	-
Profit before Income Taxes	(751)	**(2,084)**	(1,333)	-	-
Income Taxes	(205)	**(176)**	+28	-	-
Profit Attributable to Owners of Parent	(546)	**(1,907)**	(1,361)	-	-
Reference: R&D Expenses before Deducting Contribution Amount by Collaborative R&D Destinations	3,518	**6,523**	+3,004	+85.4%	26.8%

Additional Remarks

- Net sales increased mainly due to higher contract revenue.
- Cost of sales ratio (excluding contract revenue) improved due to a favorable product mix.
- R&D expenses increased year on year and exceeded the plan, primarily due to the concentration of certain global clinical trial costs in the current period, and higher CRO-related expenses.
- Non-operating income increased mainly due to the recording of compensation income and foreign exchange gains.
- Non-operating expenses increased mainly due to higher interest expenses.

Net Sales	Q1 FY2025 YTD	Q1 FY2026 YTD	Difference
Cost of Sales Ratio	27.5%	**23.2%**	(4.3)%
Cost of Sales Ratio *Excluding contract revenue	27.7%	**26.1%**	(1.6)%
R&D Expenses Ratio	39.1%	**60.7%**	+21.6%
Operating Profit Ratio	(7.1)%	**(21.7)%**	(14.6)%

Consolidated Net Sales Breakdown (Q1 FY2026)

(million yen)	FY2025	FY2026			
	Q1 YTD	Q1 YTD	Year-on-year		Progress Rate
			Difference	Ratio	
GROWJECT™	4,495	**3,744**	(750)	(16.7)%	21.4%
IZCARGO™	1,562	**1,669**	+106	+6.8%	24.1%
TEMCELL™ HS Inj.	845	**721**	(123)	(14.6)%	26.7%
Treatments for renal anemia	897	**748**	(148)	(16.6)%	19.9%
Epoetin Alfa BS Inj. [JCR]	122	**183**	+61	+50.1%	12.5%
Darbepoetin Alfa BS Inj. [JCR]	775	**565**	(209)	(27.1)%	24.6%
Agalsidase Beta BS I.V. Infusion [JCR]	426	**256**	(169)	(39.9)%	12.3%
Total Core Products	8,226	**7,139**	(1,086)	(13.2)%	21.6%
Contract revenue	106	**1,474**	+1,367	+1278.8%	18.1%
Other	236	**1,086**	+850	+359.9%	-
Total Net Sales	8,569	**9,700**	+1,130	+13.2%	21.2%

Additional Remarks

- GROWJECT™ sales decreased year on year, partly due to the impact of the April 2026 NHI drug price revisions. Although sales fell short of the quarterly plan, JCR continues to aim to achieve the full-year plan.
- The number of patients treated with IZCARGO™ increased ahead of plan.
- TEMCELL™ HS Inj. remained solid against plan.
- Treatments for renal anemia posted sales in line with the supply plan for Kissei Pharmaceutical Co., Ltd.
- Agalsidase Beta BS I.V. Infusion [JCR] sales were in line with the supply plan for Sumitomo Pharma Co., Ltd.
- Contract revenue increased mainly due to receiving an option payment under the existing agreement.

Givinostat: Japan Development Plan

Based on discussions with the PMDA, we aim to achieve regulatory filing by the end of 2026 leveraging overseas clinical data

1 Development timeline

By end-2026 ODD, MAA

By end-2027 **Approval**
Commercial Launch

2 Clinical study in Japanese patients

- Evaluation of pharmacokinetics and safety
- **Conduct independently** of the Japanese regulatory filling and review process
- Initial CTN already submitted

Clinical Study Overview (Phase 2)	
Study Design	• Open-label, single-arm
Objective	• Collection of pharmacokinetic and safety data in Japanese patients with DMD
Target Enrollment	• 20 patients (10 ambulatory, 10 non-ambulatory)
Age Range	• ≧6 to <18 years
Treatment Duration	• 6 months

CTN, clinical trial notification; DMD, duchenne muscular dystrophy; MAA, marketing authorization application; ODD, orphan drug designation; PMDA, Pharmaceuticals and Medical Devices Agency

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

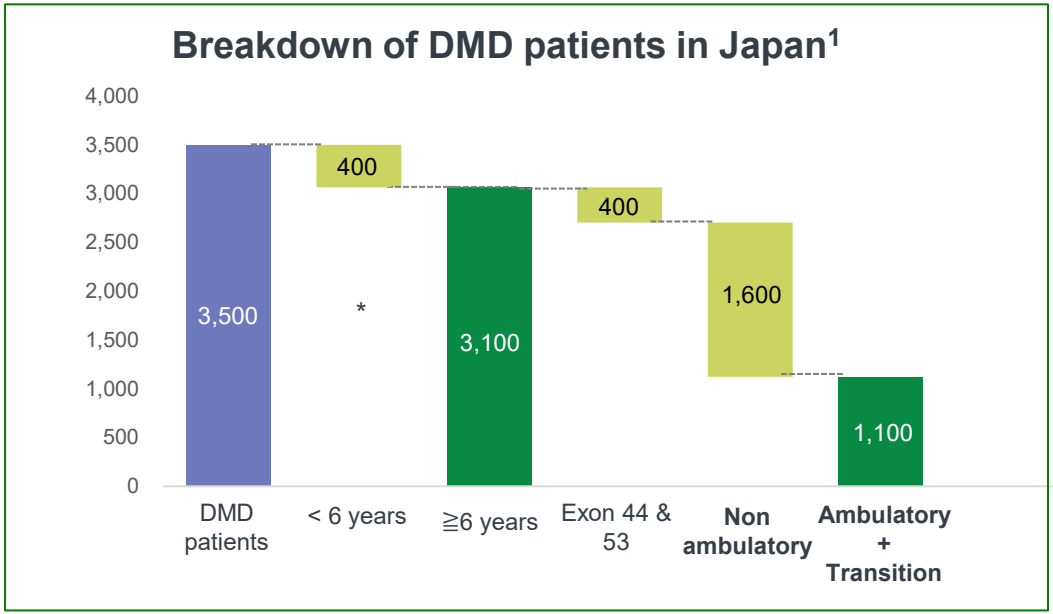
Givinostat	—	✓	✓[2]	**US;** **≥6 years** **EU;** **Ambulatory patients ≥6 years on concomitant steroid therapy**

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

Givinostat as a Key Driver of Revenue Growth

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



‘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

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)

IZCARGO™: First Marketing Authorization Outside of Japan

IZCARGO™

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

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

July 2026

Obtained marketing authorization in the UAE

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

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
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 (Japan)	• Ph1/2, Ph1: Patient enrollment completed • Actively pursuing early approval in Japan
JR-446	MPS IIIB (Sanfilippo syndrome type B)	Ph1/2 (Japan)	• IND cleared for a global Ph1/2 study (separate from the Ph1/2 study in Japan) • Discussion on going to assess early approval in Japan • Partnered with MEDIPAL HOLDINGS
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

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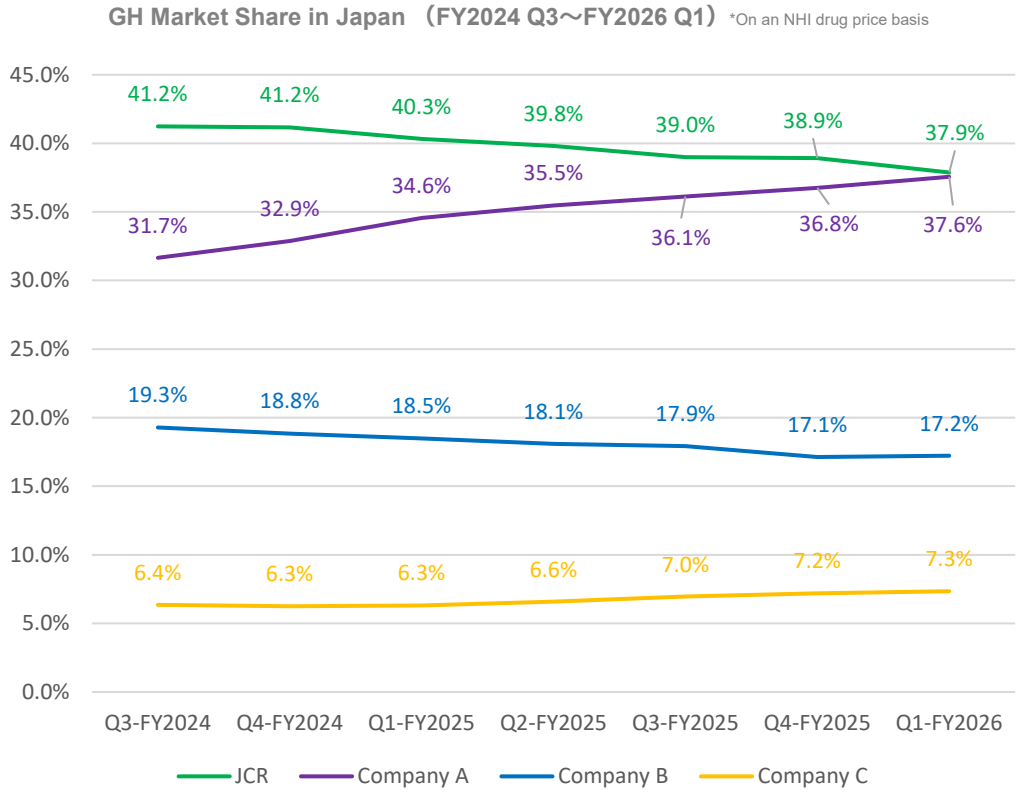
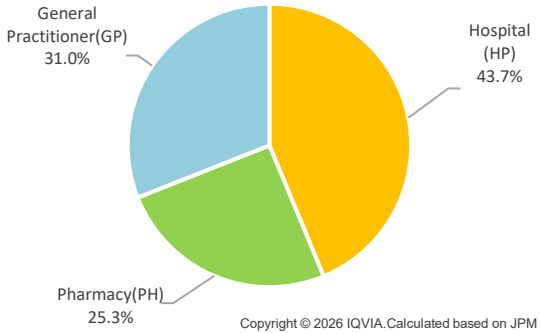
Appendix

GROWJECT™ Market Share Trends in Japan (Quarterly)



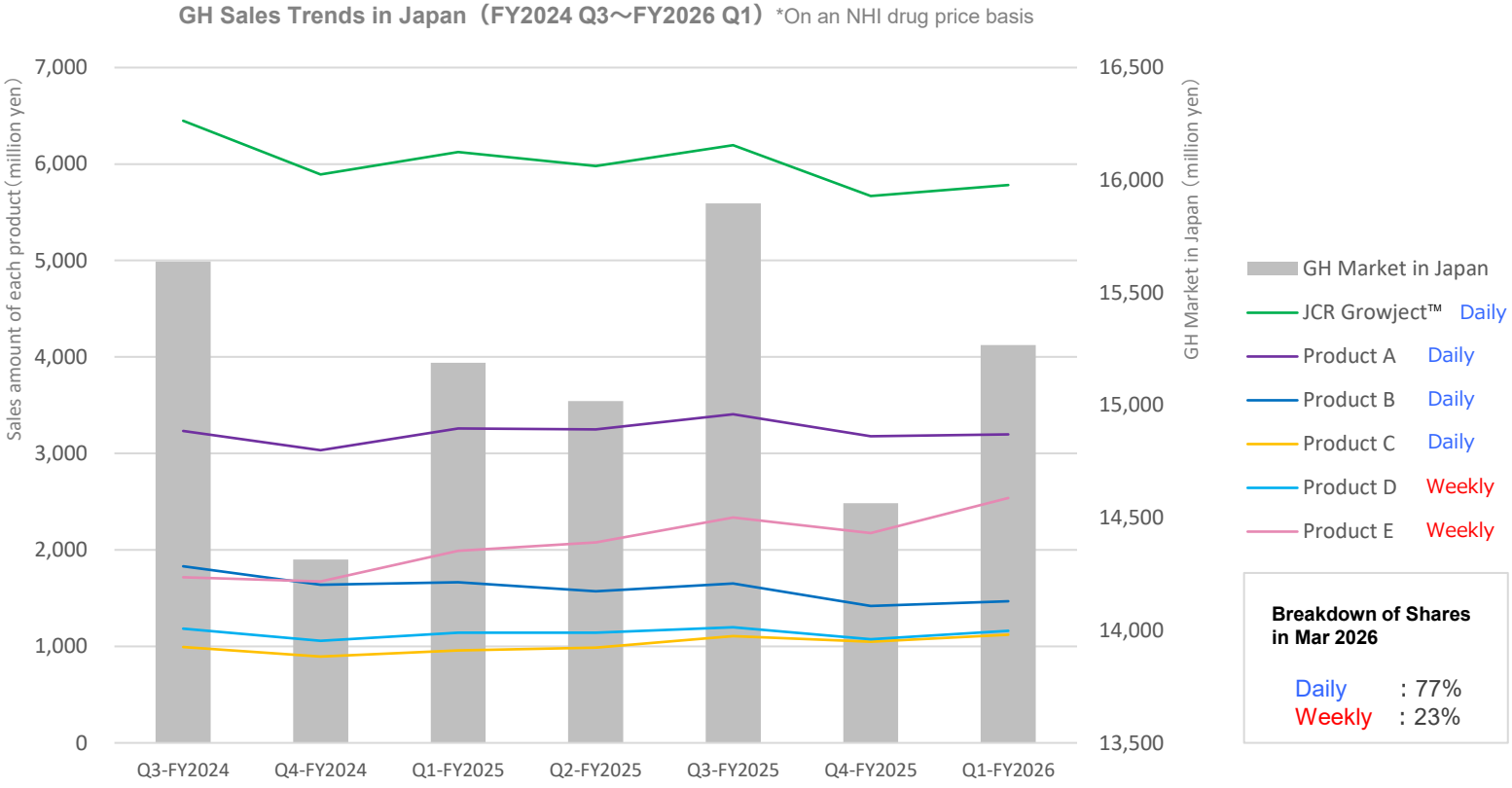
■ Human Growth Hormone Market in Japan



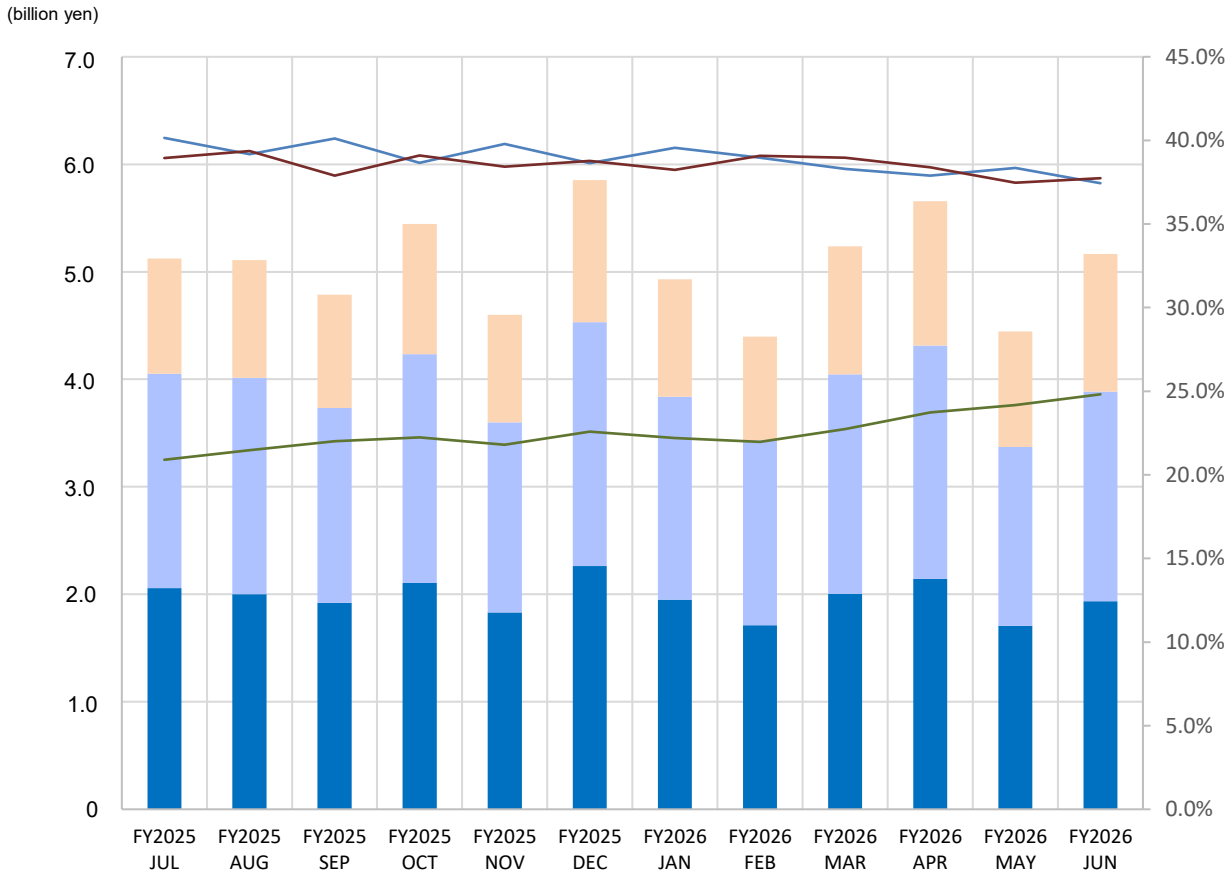
■ GROWJECT™ Market Share by buyer

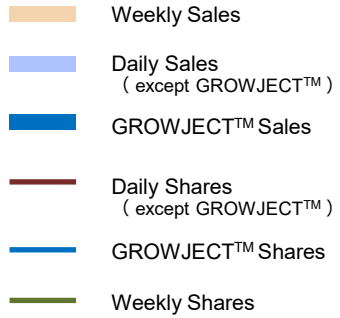
	Jun 2026	Sales Change FY2026 Q1 (vs. FY2025 Q1) *On an NHI drug price basis
HP Market	30.9%	-142 million yen
PH Market	26.8%	-49 million yen
GP Market	55.3%	-149 million yen

GROWJECT™ Sales Trends in Japan (Quarterly)

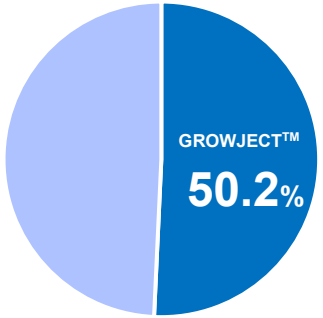


GROWJECT™ Trends of Market Share in Japan

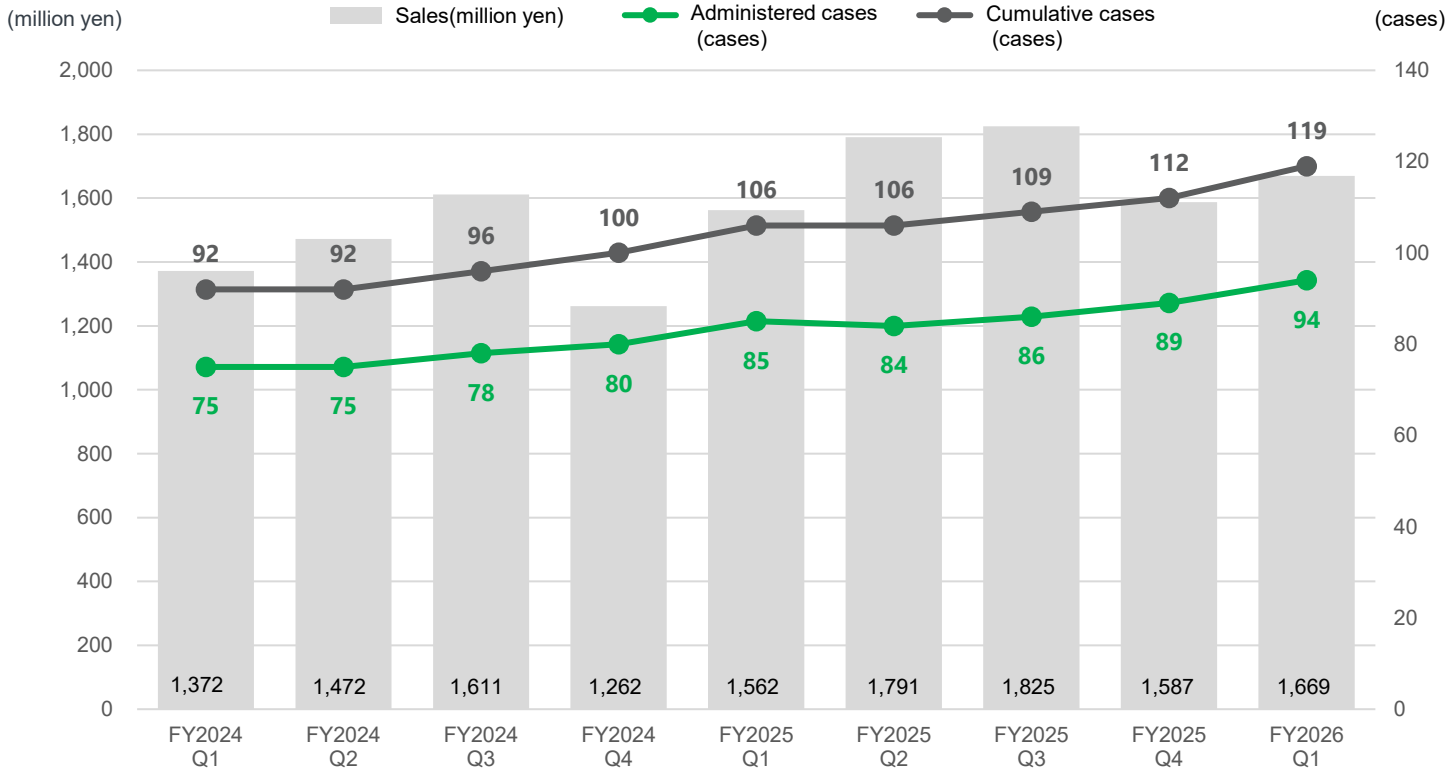




Daily Shares
FY2025 APR～FY2026 MAR



IZCARGO™ Sales Performance in Japan



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**

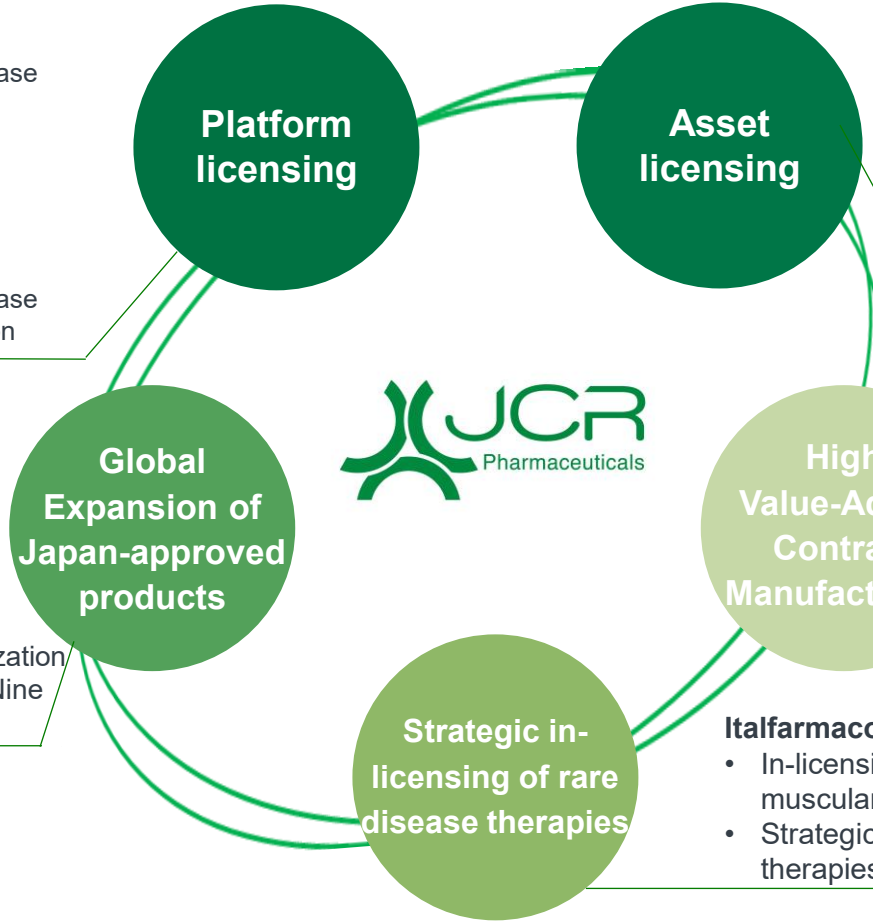
Given the extremely limited existing clinical data, a global natural history study has been initiated to accumulate data

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

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



MEDIPAL HOLDINGS CORPORATION
- Out-licensing of therapies for ultra-rare diseases
(JR-471, JR-446, JR-479)

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

FY2026 Consolidated Financial Forecasts

(million yen)	FY2025	FY2026(Forecast)		
	Results	Forecast	Year-on-year	
			Difference	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

(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

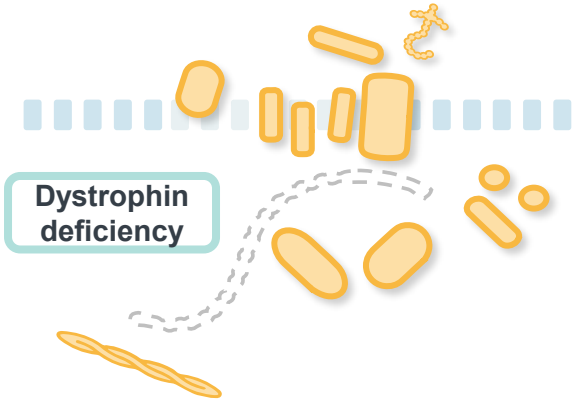
- GROWJECT™ sales are forecast to decrease due to NHI drug price revisions, despite sales volume remaining largely unchanged.
- IZCARGO™ sales are forecast to continue increasing, driven by an increase in treated patients.
- TEMCELL™ sales are forecast to decrease due to changes in the competitive environment.
- 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.

JCR Pharmaceuticals Life is Rare

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]:

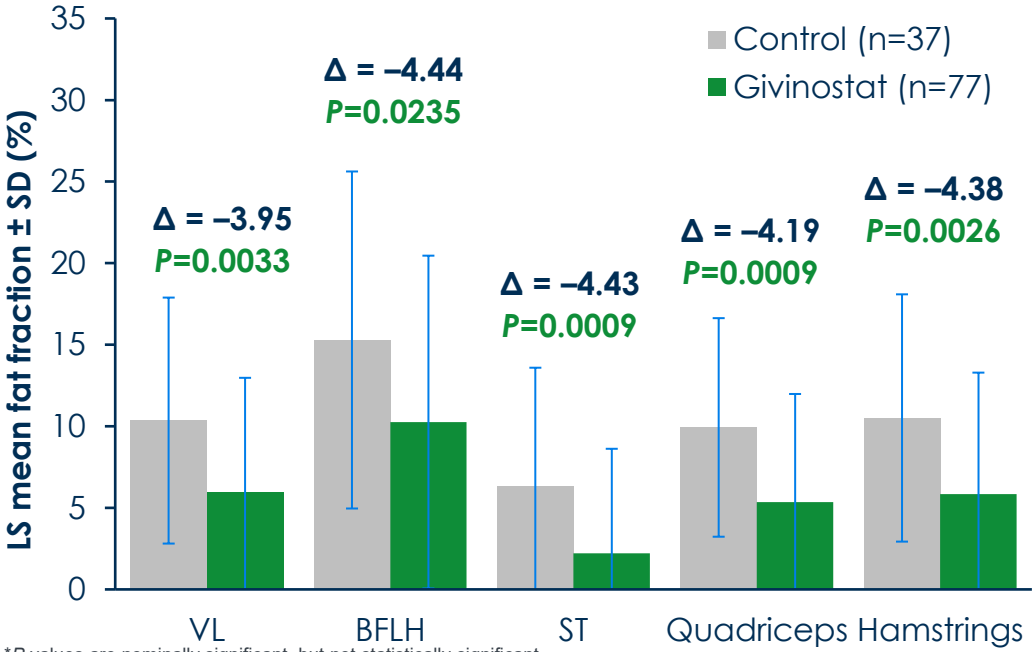
- **Increased** muscle repair and regeneration mechanisms[3,4]
- **Reduced** fibrosis and fat deposition[3]
- **Delayed** disease progression with preservation of functional capabilities[5,6]

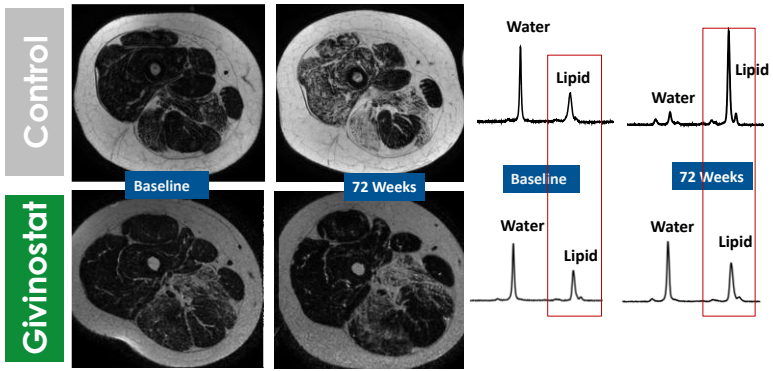
DMD, Duchenne muscular dystrophy; HDAC, histone deacetylase.

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*





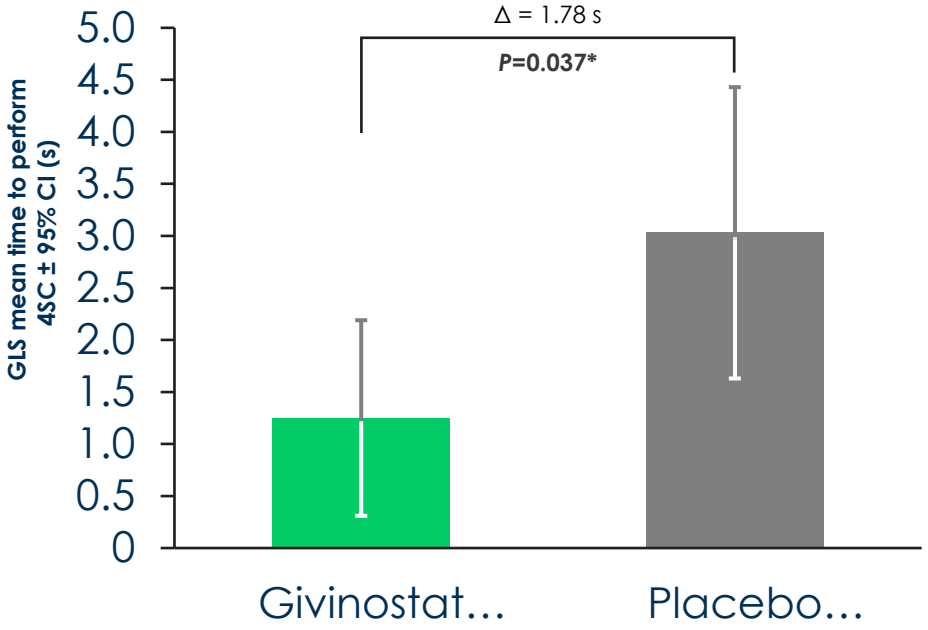
The images on the slide are from a single patient and may not be representative of all patients in EPIDYS.

*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)

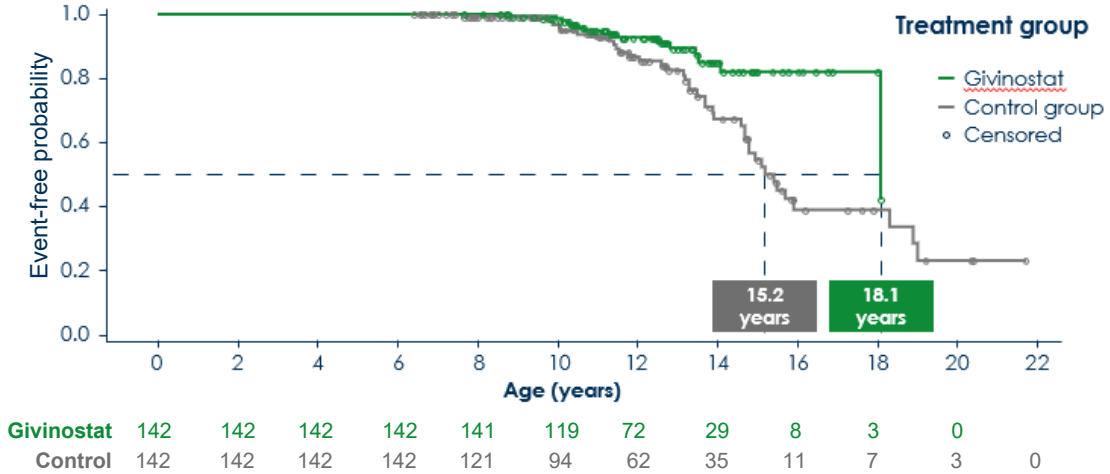


*Data are means and 95% confidence intervals. The confidence intervals have not been adjusted for multiplicity and should not be used for hypothesis testing. Baseline mean values were 3.39 s and 3.48 s for the givinostat and placebo groups, respectively. All patients were also receiving systemic corticosteroids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

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