Life is Rare

Briefing Session on the Agreement with Italfarmaco

January 23, 2026

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- Information concerning pharmaceuticals and medical devices (including those under development) contained herein is not intended as advertising or as medical advice.

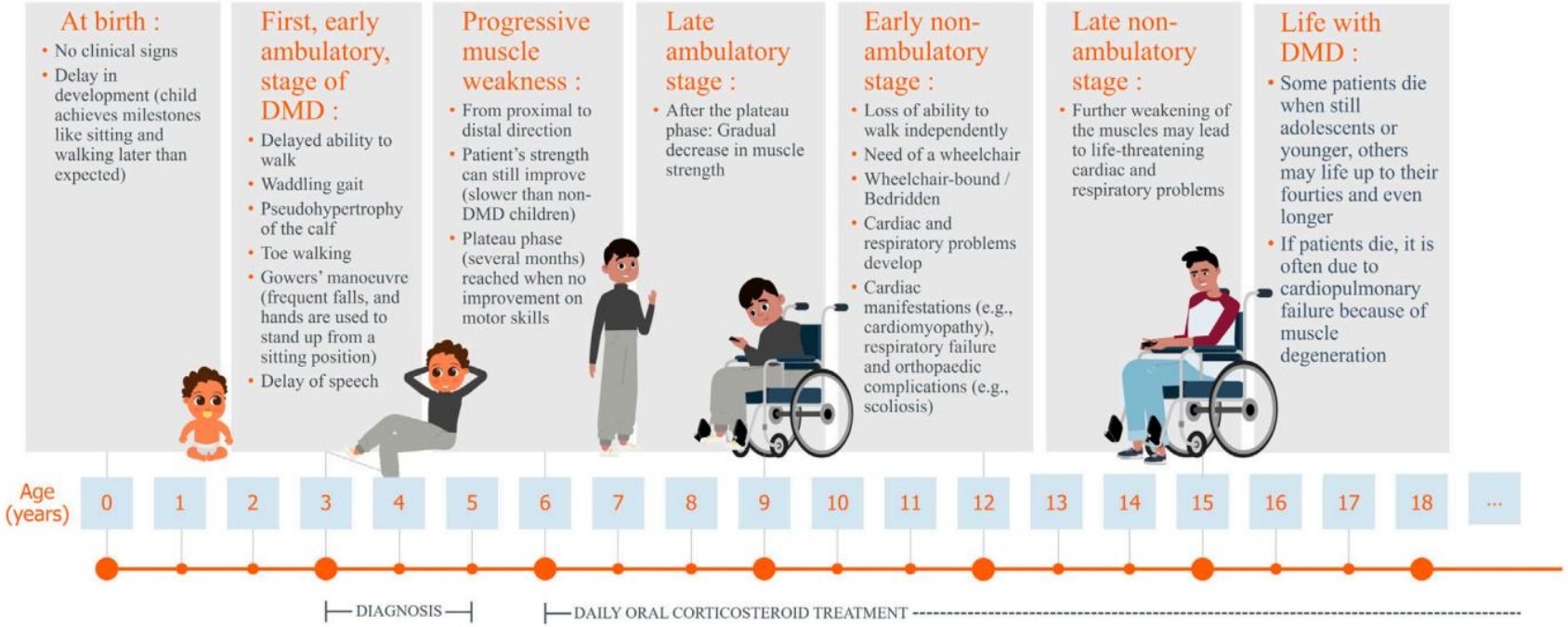
Agreement with Italfarmaco (Press Release on Dec 24, 2025)

- **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

In-licensed asset: Givinostat

Overview: Duchenne Muscular Dystrophy (DMD)



DMD, duchenne muscular dystrophy

Desmet T, et al., *Front Pharmacol.* 2025; 16: 1662586.

Overview: Duchenne Muscular Dystrophy (DMD)

1 Etiology[1]

- Caused by mutations in the dystrophin gene, leading to the absence of dystrophin protein beneath the muscle fiber membrane

2 Patient population[2]

- approx. 3,500 (Japan)

3 Clinical course[1]

- Onset at 3–5 years of age
- Progressive motor decline; loss of ambulation around 10 years
- Respiratory and cardiac complications thereafter
- Inter-individual variability in progression

4 Treatment landscape

- Only two therapies approved in Japan (excluding steroid therapy)
- Drug lag and drug loss remain key challenges for treatments approved by the FDA and EMA

DMD, duchenne muscular dystrophy

1. Clinical practice guidelines for Duchenne Muscular Dystrophy 2014 (Japanese) 2. Kawai M. *No To Hattatsu*. (Japanese) 2013;45(Supple.):S324

Current status of DMD treatment

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

Givinostat

Approaches DMD through a distinct mechanism of action from other therapies approved in Japan

DMD, duchenne muscular dystrophy

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

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

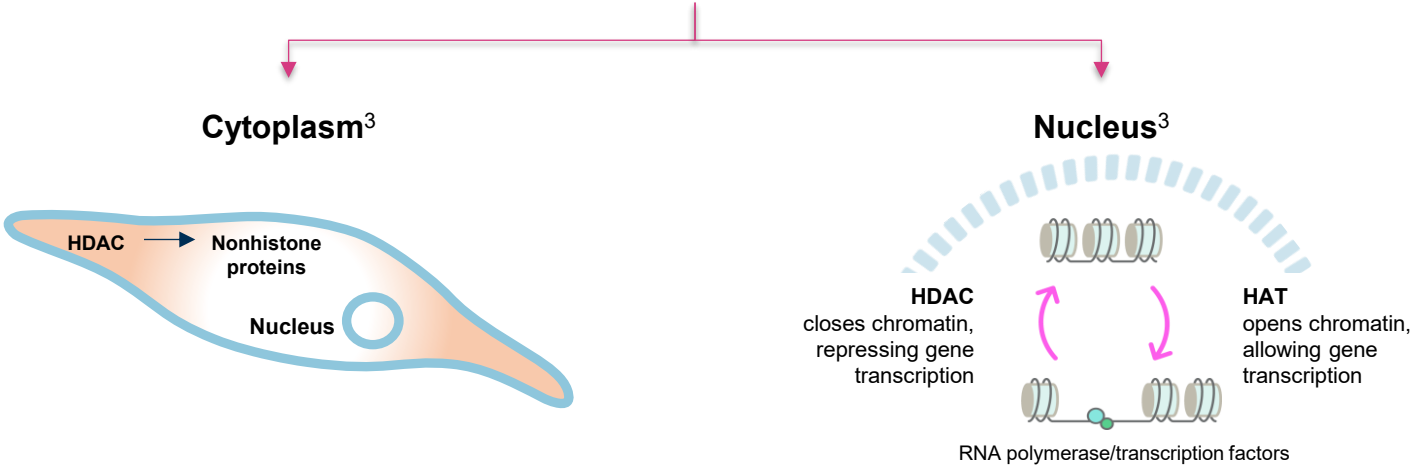
4 Key features

- 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]

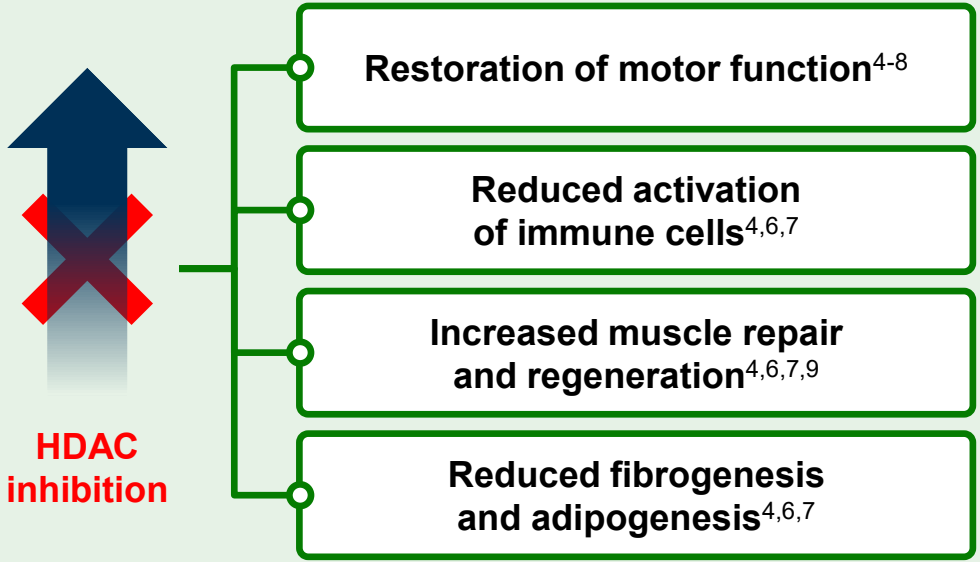
*HDAC and HAT work in balance to regulate the expression of muscle repair factors.

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]

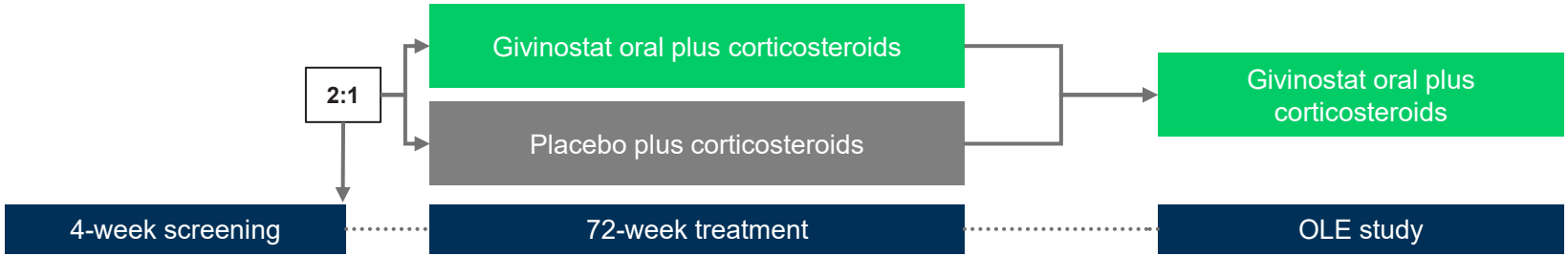


DMD, Duchenne muscular dystrophy; HDAC, histone deacetylase.

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: Phase 3 Study Design[1-3]

- Randomized, double-blind, parallel-group, placebo-controlled study with a total of 179 ambulant boys randomized 2:1 (givinostat:placebo)
- Givinostat or placebo were administered in addition to corticosteroids

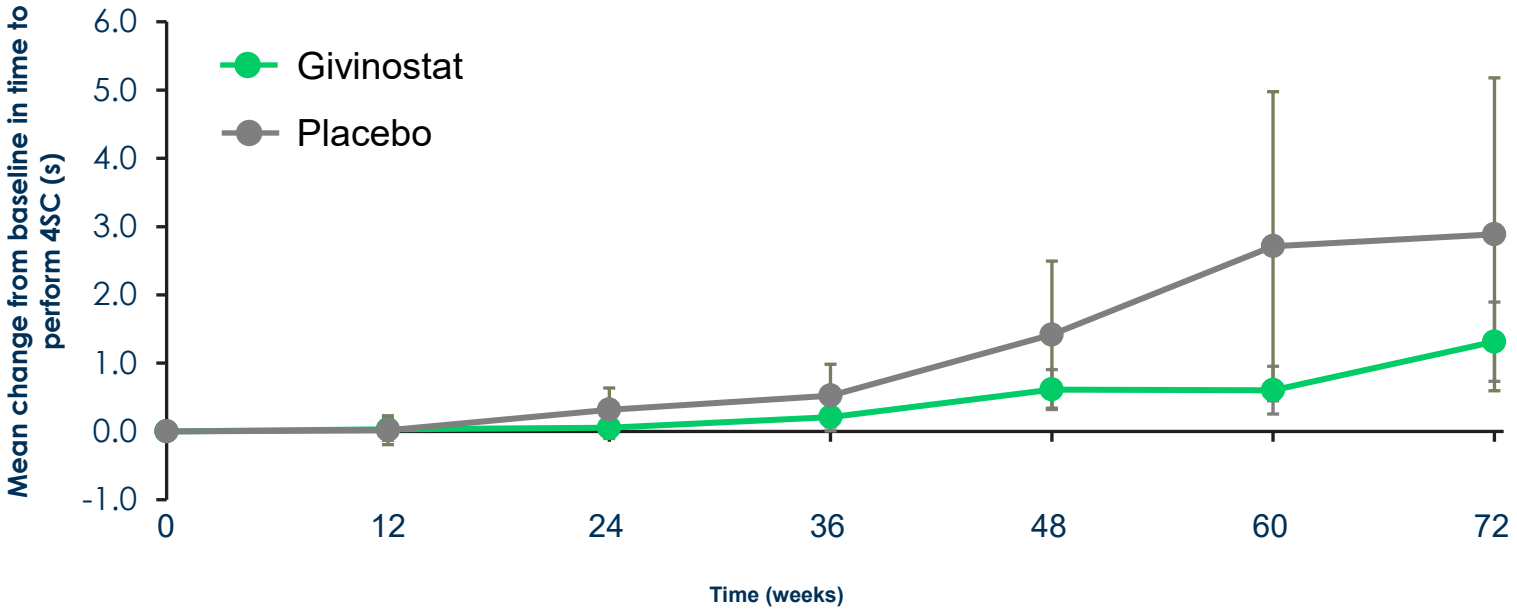


OLE, open-label extension.

1. Mercuri E et al. *Lancet Neurol*. 2024;23(4):393-403. **2**. ClinicalTrials.gov. NCT02851797. Updated February 2, 2023. Accessed May 9, 2024. https://clinicaltrials.gov/study/NCT02851797
3. Vandenborne K. Oral presentation at Muscular Dystrophy Association Clinical & Scientific Conference; March 19-22, 2023; Dallas, TX, USA.

EPIDYS Study: 4SC From Baseline to Week 72[1]

Mean change from baseline over 72 weeks in the 4SC test*



Mean change from baseline (95% CI), s						
Givinostat	0.03 (–0.13, 0.19)	0.05 (–0.10, 0.21)	0.21 (0.01, 0.41)	0.61 (0.32, 0.91)	0.60 (0.26, 0.95)	1.31 (0.73, 1.90)
Placebo	0.18 (–0.19, 0.23)	0.32 (–0.00, 0.64)	0.53 (0.07, 0.98)	1.42 (0.34, 2.49)	2.71 (0.45, 4.98)	2.89 (0.60, 5.18)

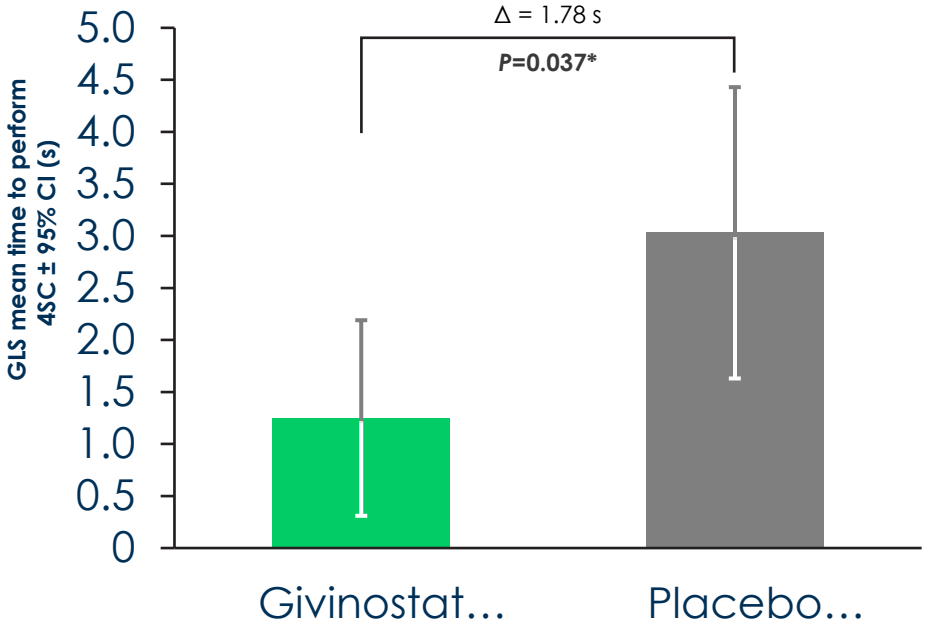
*Data are means and 95% confidence intervals. The confidence intervals have not been adjusted for multiplicity and should not be used for hypothesis testing. Baseline mean values were 3.39 s and 3.48 s for the givinostat and placebo groups, respectively. All patients were also receiving systemic corticosteroids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

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

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)



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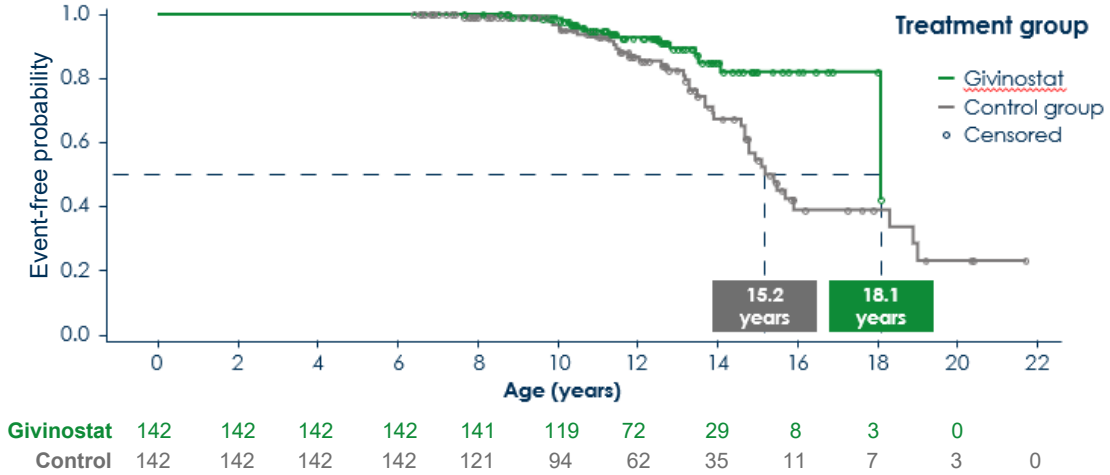
4SC, 4-stair climb; GLS, geometric least squares; s, seconds.

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

Duchenne muscular dystrophy is an indication with high unmet medical need and a significant patient population in Japan

- ~3,500 individuals in Japan diagnosed with DMD[1]
- Over 1,000 subjects meet eligibility criteria per EMA prescription information[2] (≥6 year of age; ambulatory and in transition).
- Over 3,000 individuals with DMD are ≥6 years of age[2]
- Only two non-steroidal DMD treatments are approved in Japan

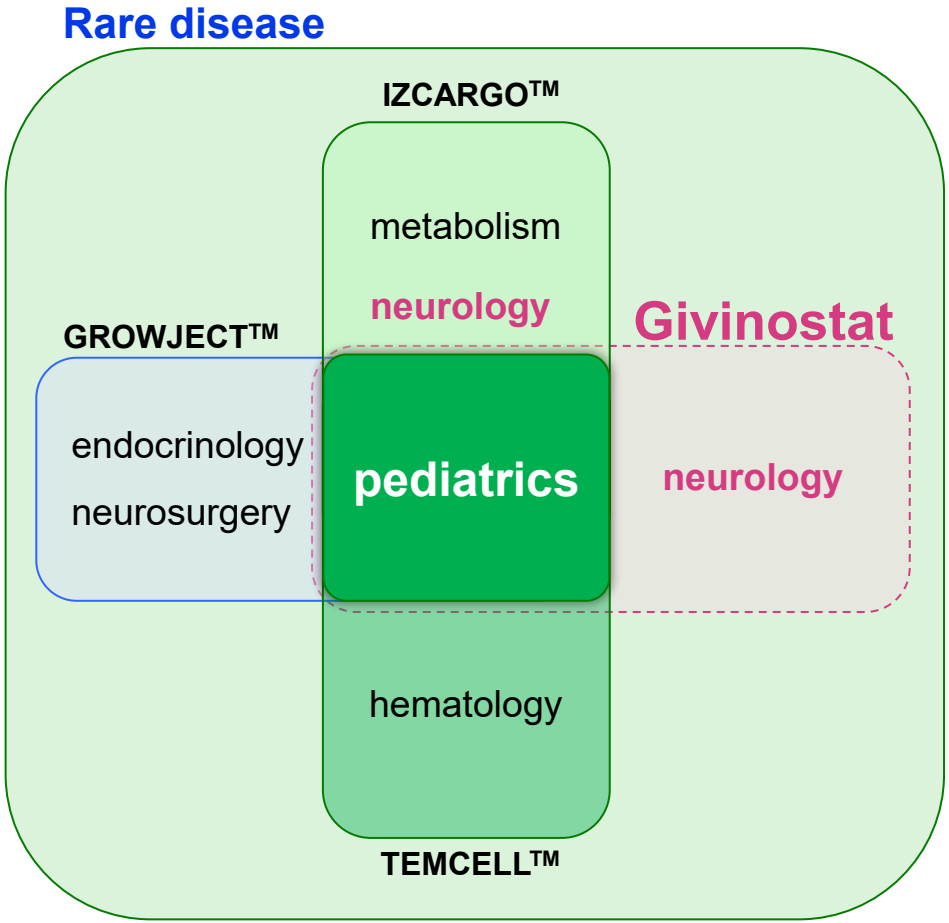
A substantial patient base and significant unmet need underpin strong sales potential in Japan[3]

Aim to obtain manufacturing and marketing approval by 2028

DMD, duchenne muscular dystrophy

1. Kawai M. *No To Hattatsu.* (Japanese) 2013;45(Supple.):S324
2. Company estimates based on Remudy (Registry of Muscular Dystrophy) and Nakamura H et al. *Orphanet J Rare Dis.* 2013;8:60
3. Viltolarsen annual treatment costs (published base price are ~USD250,000 (25 kg bodyweight) – USD450,000 (45 kg bodyweight)

JCR's Competitive Strengths in Pediatric and Rare Disease Areas



- **Pediatric portfolio advantage**
  - Established presence in pediatrics
  - >60% coverage of DMD-treating institutions with existing products (internal data)
  - Clear marketing synergies
- **Extensive expertise in rare disease drug development**

DMD, duchenne muscular dystrophy

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
- Clinical evidence demonstrated in placebo-controlled study

3 Synergy with our core strengths

- Excellent match to JCR's commercialization efforts in the rare pediatric disease space
- Robust network with clinicians treating patients with DMD

4 Strong commercial potential in Japan

50th ANNIVERSARY

JCR Pharmaceuticals

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Appendix

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