

Italfarmaco Presents New Givinostat Clinical Data Supporting Preservation of Upper Limb Function in Duchenne Muscular Dystrophy Patients at the 2026 World Muscle Society Congress

- New data suggest that givinostat treatment may slow the decline of upper limb function in boys with Duchenne muscular dystrophy compared with modelled external comparator disease trajectories derived from a single centre natural history study
- Findings further expand the growing body of evidence supporting the potential long-term clinical benefits of givinostat across multiple measures of disease progression

MILAN, Italy, September 30, 2026 – [Italfarmaco S.p.A.](https://www.italfarmaco.com) today announced new long-term data from the ongoing Phase 3 EPIDYS open-label extension (OLE) study showing that treatment with givinostat may help preserve upper limb function in boys with Duchenne muscular dystrophy (DMD). The findings, presented at the 31st Annual International Congress of the World Muscle Society (WMS), provide further evidence supporting the long-term clinical benefits of givinostat across multiple measures of disease progression.

John Brandsema, MD, Paediatric Neurologist in the Division of Neurology at Children's Hospital of Philadelphia, presented the poster "*Trajectory of Performance of Upper Limb in Patients With Duchenne Muscular Dystrophy Treated With Givinostat Relative to a Modeled Natural History Cohort from a Single Centre*," which compared upper limb function in givinostat-treated patients with counterfactual trajectories estimated from an external natural history-based model representing predicted disease progression in the absence of treatment, using the Performance of Upper Limb 2.0 (PUL 2.0) assessment. Upper limb function is an increasingly important measure of disease progression as DMD advances and loss of ambulation occurs.

"Preserving upper limb function is critically important for people living with Duchenne because it directly affects independence and the ability to perform everyday activities," said **Scott Baver, PhD, VP, Head of Global Medical Affairs, Rare Diseases**. "These new findings extend our understanding of givinostat beyond ambulatory outcomes and provide exploratory evidence suggesting a potential effect on the preservation of upper limb function, an important aspect of disease progression for people living with DMD."

"Upper limb function becomes increasingly important as Duchenne progresses because it has a direct impact on independence and participation in daily life," said **John Brandsema, MD, Paediatric Neurologist at Children's Hospital of Philadelphia** and presenter of the analysis. "Preserving upper limb function is an outcome that matters greatly to patients and families."

Key findings:

These exploratory analyses compared observed outcomes in patients receiving givinostat with predicted outcomes generated from an external natural history-based model and should be interpreted within the context of this study design.

- Patients were assessed based on PUL 2.0 evaluations: 115 patients were evaluable for the 12-month analysis. In this cohort, the observed mean change in PUL 2.0 score was



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- 0.78, compared with an average predicted decline of –4.57 (a difference of 3.79; nominal $p < 0.001$) based on the model developed using natural history data
- In the cohort evaluable at 24 months ($n=96$), the observed mean change in PUL 2.0 score was –2.26, compared with an average predicted decline of –8.55 (a difference of 6.29; nominal $p < 0.001$)
- These findings suggest that patients receiving givinostat experienced a slower average decline in upper limb function than predicted based on counterfactual trajectories

Abstract details are available under poster session 2, poster ID 2.041eP, in the [2026 WMS Congress programme](#).

About Givinostat

Givinostat was discovered through Italfarmaco's research and development efforts in collaboration with Telethon and Duchenne Parent Project (Italy). Givinostat is an orally administered histone deacetylase (HDAC) inhibitor that regulates the excessive HDAC activity characteristic of DMD muscles. By doing so, it helps restore the expression of key genes and biological processes essential for muscle maintenance and repair. Its mechanism of action is independent of the specific dystrophin gene mutation causing the disease.

The EPIDYS Phase 3 study is a randomized, placebo-controlled clinical trial designed to evaluate the efficacy and safety of givinostat in patients with DMD. MRI-based assessments of muscle composition were included as exploratory endpoints to better characterize disease progression. The ongoing open-label extension (OLE) study is intended to provide additional information on longer-term outcomes in patients previously enrolled in clinical trials.

Givinostat is approved as a treatment of DMD in multiple regions, including the US and EU, for patients aged 6 years and older, with differences across regions in ambulatory status criteria.

About ITALFARMACO

Founded in 1938 in Milan, Italy, Italfarmaco is a private global pharmaceutical company that has led the successful development and approval of many pharmaceutical products around the world. The Italfarmaco group has operations in more than 90 countries through directly controlled or affiliated companies. The company is a leader in pharmaceutical research, product development, production and commercialisation with proven success in many therapeutic areas including immuno-oncology, gynaecology, neurology, cardiovascular disease and rare diseases. Italfarmaco's rare disease unit includes programmes in Duchenne muscular dystrophy, Becker muscular dystrophy, amyotrophic lateral sclerosis and polycythaemia vera. For more information visit www.italfarmaco.com.

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