Company Announcement

New Data Presented on Givinostat for Treatment of Duchenne Muscular Dystrophy at 2025 World Muscle Society Congress

- Long-term treatment with givinostat in boys with Duchenne muscular dystrophy continues to show a favourable safety and tolerability profile
- Consistent efficacy demonstrated across age groups

MILAN, Italy, October 07, 2025 – Italfarmaco S.p.A. announced today that additional data on givinostat for the treatment of Duchenne muscular dystrophy (DMD) are being presented at the 30th annual International Congress of the World Muscle Society (WMS) held October 7-11 in Vienna, Austria. Poster presentations highlight the long-term safety of givinostat in patients with DMD, explore efficacy by age in a post hoc analysis of the pivotal EPIDYS study, and provide a detailed characterisation of gastrointestinal adverse events observed in this Phase 3 study.

"These presentations provide new insights into the long-term safety, efficacy by age, and tolerability of givinostat in DMD," said Scott Baver, PhD, VP, Head of Global Medical Affairs, Rare Diseases. "We are proud to share this data with global leaders advancing research, treatment, and patient care in DMD."

These data from studies sponsored by Italfarmaco presented at the 2025 WMS Congress include:

#651P Long-Term Safety of Givinostat in Patients With Duchenne Muscular Dystrophy: Results From an Open-Label Extension Study
The ongoing long-term study of givinostat in boys with DMD continues to provide insights into the safety and tolerability of the treatment, with results consistent with previous shorter-duration studies. Some patients have now been taking givinostat for more than 8 years in total, across several studies and extension phases.

#684VP Efficacy of Givinostat by Age (6–7 and >7 Years): A Post Hoc Analysis of EPIDYS
Baseline assessments in the study provided key insights into physical function in boys with DMD across different age groups.

#688VP Characterizing Gastrointestinal Adverse Events of Interest From a Phase 3 Study of Givinostat in Patients With Duchenne Muscular Dystrophy
The study continues to monitor gastrointestinal events in participants, with most reported events being mild to moderate in severity and resolving without requiring discontinuation of treatment. These findings provide important context for understanding the overall safety and tolerability of long-term therapy.

Abstracts have been published in the Sept 2025 supplement of Neuromuscular Disorders here.

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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.1,2

Givinostat is approved as a treatment of DMD in multiple regions, including the US, EU and UK.

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.

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References:

1. Comi G, Bertini E, Vita G, et al. S22.008: Development of the histone deacetylases inhibitor Givinostat in Duchenne Muscular Dystrophy. Poster. Neurology. 2018;90(15 (Supplement)).
2. Licandro SA, Crippa L, Pomarico R, et al. The pan HDAC inhibitor Givinostat improves muscle function and histological parameters in two Duchenne muscular dystrophy murine models expressing different haplotypes of the LTBP4 gene. Skelet Muscle. 2021;11(1):19. https://doi.org/10.1186/s13395-021-00273-6.