Company Announcement

European Commission Approves Duvyzat for the Treatment of Duchenne Muscular Dystrophy

- Now approved in the EU, Duvyzat offers an important treatment option for delaying Duchenne muscular dystrophy (DMD) disease progression
- Duvyzat is an orally administered treatment for DMD in patients 6 years and older who are able to walk
- The approval is based on Phase 3 EPIDYS trial data that demonstrated meaningful treatment benefits in ambulant patients

MILAN, Italy, June 06, 2025 – Italfarmaco S.p.A. announced today that the European Commission (EC) has granted conditional marketing authorisation for Duvyzat® (givinostat), a novel histone deacetylase (HDAC) inhibitor. It is approved for the treatment of Duchenne muscular dystrophy (DMD) in ambulant patients 6 years and older, regardless of the underlying genetic mutation, when taken together with corticosteroids. The EC decision follows the positive opinion of the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) on the 25th of April, 2025. The approval applies to all 27 EU member states, as well as Iceland, Liechtenstein, and Norway. Italfarmaco is now working closely with national authorities and distribution partners to facilitate timely access to Duvyzat across the EU.

"People living with DMD in Europe have long awaited new therapeutic options that can alter the course of this devastating disease. Until now, there have been limited approved treatments that address the underlying pathology of DMD across the broad patient population. That changes with the approval of Duvyzat, which slows disease progression and preserves muscle function — regardless of the gene mutation — by targeting disease mechanisms," said **Paolo Bettica, MD, PhD, Chief Medical Officer at Italfarmaco Group**. "We are committed to working closely with health authorities and the DMD community to ensure timely access to this important new treatment across Europe."

"The EC's approval of Duvyzat is a recognition of its therapeutic potential and a testament to Italfarmaco's scientific excellence and commitment to innovation in rare diseases," said **Dr Francesco De Santis, President of Italfarmaco Holding and Chairman of Italfarmaco Group**. "This milestone means that a broad range of patients with DMD have access to a new treatment. At Italfarmaco, this achievement reaffirms our focus on advancing therapies that can make a meaningful difference in people's lives."

The approval is based on the positive results of the EPIDYS Phase 3 multicentre, randomised, double-blind, placebo-controlled trial (NCT02851797). In the EPIDYS study, a total of 179 ambulant boys six years of age or older received either Duvyzat twice daily or placebo, in addition to corticosteroid treatment. The EPIDYS study met its primary endpoint demonstrating a statistically significant and clinically meaningful difference in time to complete the four-stair climb assessment. Duvyzat also showed favourable results on key secondary endpoints including North Star Ambulatory Assessment (NSAA) and fat infiltration evaluation by magnetic resonance imaging. Specifically, Duvyzat treatment was associated with 40% less decline in cumulative loss of NSAA items, indicating Duvyzat's potential to delay disease progression in affected individuals. Most adverse effects observed with Duvyzat were mild to moderate in severity. Results from this study were published in *The Lancet Neurology*

in March 2024.[1] Long-term data from the ongoing EPIDYS extension study was compared to natural history cohorts using propensity score matching showing that the median age at loss of ambulation was 18.1 years in the Duvyzat group versus 15.2 years in controls.[2]

The EC has granted Duvyzat conditional marketing authorisation in the EU. The approval makes Duvyzat available to ambulant DMD patients 6 years and older while Italfarmaco conducts additional clinical studies designed to further confirm and characterise its therapeutic benefit. Outside of the EU, Duvyzat was granted approval by the US Food and Drug Administration (FDA) in March 2024 for the treatment of DMD patients 6 years and older. In the UK, Duvyzat received approval by the Medicines and Healthcare products Regulatory Agency (MHRA) for patients 6 years and older who are ambulatory and granted conditional marketing approval for non-ambulatory patients.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare, progressive neuromuscular disorder caused by mutations in the *DMD* gene. Mutations in the *DMD* gene prevent the production of functional dystrophin, causing the dystrophin-associated protein complex (DAPC) to break down. This makes muscle fibres more vulnerable to damage and increases histone deacetylase (HDAC) levels in the muscle cells, blocking the activation of important genes needed for muscle maintenance and repair. As a result, muscle fibres experience ongoing damage, leading to chronic inflammation and poor regeneration. Over time, muscle cells die and are replaced by scar tissue and fat.[3-6] DMD primarily affects males, with symptoms typically appearing between the ages of two and five. As the condition progresses, muscle weakness worsens, leading to difficulty walking and eventually to loss of ambulation. Over time, the heart and respiratory muscles are also affected, which are the leading causes of premature death.[7] DMD is one of the most severe and common forms of childhood muscular dystrophy, with a global birth incidence of approximately 1 in 5,050 boys.[8]

About Duvyzat®

Duvyzat was discovered through Italfarmaco's research and development efforts in collaboration with Telethon and Duchenne Parent Project (Italy). Duvyzat 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.[9,10]

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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