Company Announcement

Italfarmaco Announces U.S. FDA Grants Fast Track Designation to Givinostat in Treatment of Polycythemia Vera

MILAN, Italy, May 6, 2025 – Italfarmaco S.p.A. announced today that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to givinostat for the treatment of patients with polycythemia vera (PV), a rare haematologic cancer, for which treatment options are limited.

"The FDA decision to grant givinostat Fast Track designation underscores the urgent need for innovative treatments for PV and highlights the potential of givinostat to make a meaningful difference," said **Paolo Bettica, MD, PhD, Chief Medical Officer at Italfarmaco Group**. "We look forward to working closely with the FDA as we plan for completion of our Phase III clinical trial."

Givinostat is an orally administered histone deacetylase inhibitor (HDACi) with potential applications in both neuromuscular disorders and oncology. HDACis work by modulating key cellular pathways that regulate gene expression, offering promising therapeutic benefits across a range of diseases. Givinostat is currently being studied for its potential to treat PV, a rare blood cancer characterised by the overproduction of erythroid, myeloid, and megakaryocytic components in the bone marrow. PV commonly causes symptoms like headache, weakness, and itching, with severe complications including stroke, heart attack, and deep vein thrombosis, which are leading causes of mortality. Furthermore, PV carries a variable risk of progression to myelofibrosis or acute myeloid leukaemia. By targeting and modulating abnormal gene expression, givinostat may help control excessive cell proliferation driven by mutations such as JAK2V617F, commonly found in PV patients. This mechanism aims to reduce disease burden, alleviate symptoms, and improve long-term outcomes. The Phase III trial (NCT06093672) is currently enrolling patients with clinical sites open in Europe, the UK, Israel, and North America, with more sites expected soon.

FDA Fast Track designation is a process designed to facilitate the development and expedite the review of new drugs or biologics that are intended to treat serious or life-threatening conditions and address unmet medical needs. This designation aims to accelerate the drug development and review process by encouraging early and frequent communication between the FDA and drug companies.

Givinostat has received orphan drug designation by the FDA and the European Medicines Agency (EMA) for PV.

ITALFARMACO S.p.A.
V.le Fulvio Testi 330, 20126 Milan, Italy
www.italfarmaco.com

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In addition, givinostat (Duvyzat®) has marketing authorisations for Duchenne muscular dystrophy from the FDA and the MHRA[1], with a positive opinion adopted by the CHMP[2] and other regulatory processes currently ongoing.

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 60 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.

Media enquiries:
Anja Heuer / Jacob Verghese | +49 (0) 151 106 199 05 |
italfarmaco@trophic.eu

Other enquiries:
Patient Advocacy and Communications Lead |
s.parker@italfarmacogroup.com

[1] UK's Medicines and Healthcare products Regulatory Agency
[2] European Medicines Agency Committee for Medicinal Products for Human Use