Italfarmaco Receives FDA Rare Pediatric Disease Designation for Givinostat in Duchenne Muscular Dystrophy, Announces Completed Enrollment in EPIDYS Phase 3 Trial

MILAN, Italy, October 9, 2020 – The Italfarmaco Group provided today an update on the development of Givinostat, its proprietary histone deacetylase (HDAC) inhibitor, in boys with Duchenne Muscular Dystrophy (DMD). The U.S. Food and Drug Administration (FDA) granted a Rare Pediatric Disease designation to Givinostat for the treatment of DMD, which allows an expedited review process for new treatment modalities. The company also announced the completion of patient enrollment in the EPIDYS Phase 3 trial on September 25th, 2020 and remains on track with its proposed timelines for reporting topline data in June 2022.

"This is the third regulatory designation we have received from the FDA for Givinostat after the Orphan Drug designation and Fast Track designation and reflects the agency's recognition of Givinostat's potential to treat DMD, a devastating genetic disease," said Paolo Bettica, MD, PhD, Chief Medical Officer at the Italfarmaco Group. "We are also pleased to have completed enrollment in our Phase 3 trial with 179 boys. This is a milestone for the company as we move closer to establishing the therapeutic value of Givinostat and bringing a new treatment to patients in need. We are grateful to the patients and their families and our study investigators and clinical teams for moving the trial forward despite the challenging pandemic crisis."

The Rare Pediatric Disease designation is granted by the FDA to encourage development of new drugs for serious or life-threatening manifestations of a disease or condition that primarily affect children 18 years of age and younger. Subject to FDA approval of Givinostat for the treatment of DMD, Italfarmaco would be eligible to receive a voucher, which may be redeemed to receive priority review for a subsequent marketing application for a different product candidate or which could be sold or transferred.

In April 2020, Italfarmaco announced that interim data from the EPIDYS Phase 3 clinical trial was reviewed by an Independent Data Monitoring Committee and that the trial was recommended to continue based on the lower limb muscle fat infiltration seen in the Magnetic Resonance Spectroscopy (MRS) images of DMD patients with Givinostat treatment compared to the placebo group after 12 months of treatment. These MRS results corroborate the histological results of the company's Phase 2 study, which revealed slower disease progression in boys treated with Givinostat and on a stable regimen of steroids. The Independent Data Monitoring Committee, which regularly monitors and reviews the study, confirmed the safety of the treatment with Givinostat. With trial enrollment completed, the last patient is expected to complete the 72-week treatment period in the first quarter of 2022. Italfarmaco is expected to report topline data from the Phase 3 EPIDYS trial in June 2022.

About Givinostat

Givinostat is an investigational drug discovered through Italfarmaco's internal research and development efforts in collaboration with Lorenzo Puri (Sanford Burnham Prebys Medical Research Institute, San Diego, formerly Santa Lucia Foundation, Rome) and his team, and partnerships with Telethon and Parent Project aps. It is being evaluated for safety and efficacy for the treatment of Duchenne- and Becker- Muscular Dystrophy. Givinostat inhibits histone deacetylases (HDACs). HDACs

are enzymes that prevent gene translation by changing the three-dimensional folding of DNA in the cell. Studies show that Duchenne patients have higher than normal HDACs activity, which may prevent muscle regeneration, and also trigger inflammation. In the company's clinical study in DMD, boys aged 7 to less than 11 years, Givinostat was observed to slow disease progression, significantly increase muscle mass and reduce the amount of fibrotic tissue. Givinostat treatment also significantly reduced muscle tissue necrosis and fatty replacement, two additional parameters related to disease progression (Bettica et al., Neuromuscular Disorder 2016).

About Italfarmaco Group

Italfarmaco is a specialty pharmaceutical company engaged in the discovery, development, manufacturing and marketing of branded prescription and nonprescription products in more than 60 countries on 5 continents. Italfarmaco's research and development expertise is best demonstrated through its HDAC inhibitor development programs, addressing new therapeutic treatments of specialty and rare diseases. Through both marketed drugs and compounds in development, Italfarmaco is dedicated to serving patients whose needs remain largely unmet.

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