NEWS RELEASE

PRIX GALIEN ITALIA: GIVINOSTAT IS THE WINNER IN "RARE DISEASES" CATEGORY
"A PRODUCT OF ITALIAN RESEARCH, IT SLOWS THE PROGRESSION OF DUCHENNE MUSCULAR DYSTROPHY"

Collaboration with clinicians in the development of the therapy and the contribution of patient organizations are crucial. Francesco Di Marco, Italfarmaco Group CEO: "This recognition highlights the scientific value of the molecule and the path of innovation undertaken by the company." The medicine offers a new treatment option regardless of the specific genetic mutation, thereby expanding the number of patients who can benefit from the treatment

Milan, November 4, 2026 – Givinostat, a molecule that "in clinical trials has shown to slow the progression of Duchenne muscular dystrophy" thanks to a new mechanism of action has won the Prix Galien Italia in the "Rare Diseases" category. The drug represents a story of Italian scientific excellence and is the result of research and development carried out by Italfarmaco. The award ceremony took place yesterday in Turin.

"Winning the Prix Galien recognizes not only an innovative molecule like givinostat, but also an entrepreneurial and scientific journey built over time through consistent investment in research and development," explained **Francesco Di Marco**, Group CEO. "This prize carries even greater significance because it comes in a field with significant unmet medical needs and a strong demand for innovation—a field where, for a long time, available therapeutic options have been limited. This achievement enhances the innovation path undertaken by Italfarmaco and the capacity of Italian research to generate scientific progress and translate it into concrete therapeutic solutions for patients."

"We are very proud for this prestigious award," said **Julie De Santis**, Board of Directors, Italfarmaco Holding. "Italfarmaco was founded in 1938 in Milan. Building on our family tradition, we are guided by a long-term vision, deeply rooted in our heritage and supported by an international team. Over the past 88 years, the company has consistently invested in research and development, and givinostat is the result of our commitment. We wish to share the Prix Galien with the clinicians and patient organisations that have made a decisive contribution to the development of this molecule. Our aim is to address unmet clinical needs, particularly in rare diseases, and thanks to givinostat, a significant step forward has been taken."

"As a result of Italian research, givinostat—as stated in the award citation—represents a therapeutic innovation for Duchenne muscular dystrophy because it introduces a mechanism of action that is different from and independent of any

specific genetic mutation, with potential applicability to a broad population of patients who meet the authorized criteria. When used in combination with corticosteroids, it has shown to slow disease progression and help maintain motor function and independence for a longer period, addressing a significant unmet therapeutic need in the field of rare diseases with a meaningful impact on the quality of life for patients and their families."

Duchenne muscular dystrophy is a rare genetic neuromuscular disorder affecting almost exclusively male children and is caused by the absence or severe deficiency of dystrophin. When this protein is absent or is not functioning properly, the muscle fibres become more fragile and, over time, an inflammatory process is triggered, leading to the replacement of muscle tissue with fibrous tissue.

"Over time, the progression of the disease leads to a significant loss of muscle strength, mobility, and independence," emphasised **Eugenio Maria Mercuri**, Full Professor at the Università Cattolica del Sacro Cuore. "Patients face also serious complications such as cardiomyopathies, respiratory and neurological problems, with a significant impact on cognitive function. Givinostat, which acts on the cellular mechanisms contributing to muscle damage, represents a major step forward in treatment, as it significantly slows disease progression. The drug belongs to the class of histone deacetylase inhibitors, proteins that regulate the activity of many genes. A key feature of the molecule is its ability to offer a therapeutic option regardless of the specific genetic mutation, thereby broadening the number of patients who can benefit from the treatment and helping to preserve patients' motor function and independence over time."

"The results of the initial preclinical studies, conducted on mouse models with Duchenne muscular dystrophy, were significant," explained **Paolo Bettica**, Chief Medical Officer, Drug Development, Italfarmaco. "This marked the beginning of the molecule's clinical development. In collaboration with patient organisations and Italian clinicians, we designed a Phase 2 trial that enabled us to assess the effect of givinostat directly in the muscle, thanks to muscle biopsies carried out after one year of treatment. This Phase 2 trial was fully conducted in Italy. The results were very positive, as they replicated what had been observed in the mouse model. We therefore initiated an international Phase 3 registration trial, EPIDYS, which demonstrated the benefit of givinostat both functionally and within the muscle, using nuclear magnetic resonance imaging. Duchenne muscular dystrophy patients' and clinicians' experience was crucial in identifying the relevant endpoints for the trials. Histone deacetylase inhibition has been studied across different diseases, but givinostat is the first drug in its class to be developed for Duchenne muscular dystrophy."

"This award," concluded **Francesco Di Marco**, "recognises the scientific value of the molecule and its contribution to the Duchenne community, which provided essential support in carrying out the clinical trials. The Prix Galien marks a particularly significant milestone in Italfarmaco's journey, as the company has successfully combined solid scientific and entrepreneurial roots with a constant commitment to innovation. This

award is not an end point, but a significant milestone on an ongoing journey. Indeed, it reinforces Italfarmaco's mission to transform scientific research into concrete benefits for patients and confirms our commitment, which goes beyond the development of a therapy to embrace the broader aim of offering new prospects to patients living with this disease and their families."

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 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.