Company Announcement

ITF Therapeutics LLC Presents Long-Term Data of DUVYZAT™ (givinostat) for Treatment of Duchenne Muscular Dystrophy at MDA Clinical and Scientific Conference

Seven abstracts highlight long-term effects, consistent safety profile, and delayed disease progression with givinostat

Results also show that treatment is associated with delayed decline in respiratory function

CONCORD, Mass., March 17, 2025 – ITF Therapeutics LLC, the U.S. affiliate of Italfarmaco, today announced the presentation of seven abstracts at the Muscular Dystrophy Association (MDA) Clinical and Scientific Conference being held March 16-19, 2025, in Dallas, Texas. The poster presentations include data from the Phase 3 EPIDYS study and the company's ongoing open-label extension study in patients with Duchenne muscular dystrophy (DMD) treated with givinostat. Analyses assess the long-term safety and efficacy of givinostat as identified through measures including data on disease progression and respiratory function. Please see the Indication and Important Safety Information for DUVYZAT™ (givinostat) below.

"This year's MDA Clinical and Scientific Conference arrives at a meaningful moment for our team as we reflect on the one-year anniversary of the U.S. FDA approval of DUVYZAT for the treatment of DMD in people 6 years of age and older," said Matt Trudeau, President, ITF Therapeutics. "We are honored to participate in this important meeting and look forward to sharing new insights from our ongoing research, including clinical data from our open-label extension study in DMD. These abstracts assess the long-term efficacy and safety of DUVYZAT, and we are grateful for the opportunity to present this data to the Duchenne community. As a core focus of our work, we strive to provide people living with DMD and their families and clinicians with the resources and information they need to make informed treatment decisions."

One poster presented by Krista Vandenborne, Ph.D., Chair, Department of Physical Therapy, University of Florida, includes data from the Phase 3 EPIDYS study and from an ongoing open-label extension study evaluating the long-term safety, tolerability, and efficacy of givinostat in boys aged ≥ 6 years with DMD. Using propensity score matching based on baseline functional test results and type of steroid, 142 patients from EPIDYS and the extension study treated with givinostat and steroids were matched with 142 patients from two DMD natural history studies treated with steroids only. Results were assessed based on the median age at which DMD progression milestones such as persistent loss to perform 4-stair climb, loss of rise from floor, and loss of ambulation, occurred. The median age at loss of ambulation in patients treated with givinostat was 18.1 years, compared to 15.2 years in the control group.

A second poster presented by Craig M. McDonald, M.D., Chair, Department of Physical Medicine & Rehabilitation, UC Davis Health, evaluates the effect of givinostat on pulmonary function in patients who experienced loss of ambulation during follow-up. Results were assessed based on a comparison of forced vital capacity (FVC) percent trajectories from patients treated with givinostat and steroids in the EPIDYS and extension studies to FVC percent trajectories from patients treated with steroids only in a natural history study of disease progression. A related poster presented by Erika L. Finanger, M.D., M.S., Professor of Pediatrics, Oregon Health & Science University, includes results from an analysis using a validated DMD clinical trial simulation tool to assess differences in the time course of worsening FVC in patients treated with givinostat or standard of care.

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A separate poster presented by John F. Brandsema, M.D., Pediatric Neurologist, Division of Neurology, Children's Hospital of Philadelphia, evaluates safety data from the ongoing open-label extension study of givinostat in patients who completed or who were screened but not randomized into previous studies of givinostat for the treatment of DMD. All treatment-emergent adverse events were consistent with the known safety profile of givinostat, with no new safety signals observed in patients continuing the treatment.

Other presentations include disease progression models comparing the standard of care with givinostat and details related to the design of an observational study to assess real-world outcomes of patients treated with givinostat. A subgroup analysis of an off-target population will also assess givinostat efficacy in a population with relatively more advanced disease compared to target groups.

About DUVYZAT™ (givinostat)

DUVYZAT is a U.S. FDA-approved histone deacetylase (HDAC) inhibitor indicated for the treatment of patients six years of age and older with Duchenne muscular dystrophy (DMD) that was discovered through the research and development efforts of Italfarmaco in collaboration with Telethon and Duchenne Parent Project (Italy). HDACs are enzymes located in the body's cells that play a key role in maintaining and repairing muscles. In DMD, the HDAC enzymes become overactive, leading to chronic muscle inflammation, decreased muscle repair, and replacement of muscle with fat and scar tissue. DUVYZAT inhibits HDAC overactivity and is thought to help reduce inflammation, increase the body's ability to repair muscles, and slow muscle loss. For more information visit www.DUVYZAT.com.

About ITF Therapeutics LLC

ITF Therapeutics was launched in January 2024 as the U.S. affiliate of Italfarmaco focused on the development and commercialization of products to treat rare diseases. Building on a legacy grounded in collaboration and innovation, ITF Therapeutics strives to partner with leaders from the patient advocacy and treatment communities to ensure that our programs reflect and support their unique needs and goals. The establishment of ITF Therapeutics reflects Italfarmaco's goal to build a world-class team of experts who share a passion to make a positive impact for rare disease communities. For more information visit www.itftherapeutics.com.

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 commercialization with proven success in many therapeutic areas including immuno-oncology, gynecology, neurology, cardiovascular disease, and rare diseases. Italfarmaco's rare disease unit includes programs in Duchenne muscular dystrophy, Becker muscular dystrophy, amyotrophic lateral sclerosis, and polycythemia vera. For more information visit www.italfarmaco.com.

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C-DUV-US-0332 03/2025

Company Announcement

Indication

DUVYZAT is a histone deacetylase inhibitor indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 6 years of age and older.

Important Safety Information

Warnings and precautions

- Hematological Changes: DUVYZAT can cause dose-related thrombocytopenia and other signs of myelosuppression, including anemia and neutropenia. Monitor platelets; dosage adjustment or discontinuation may be needed.
- Increased Triglycerides: An increase in triglycerides can occur; dosage modification may be needed. Discontinuation may be needed.
- Gastrointestinal Disturbances: Adjust dosage if moderate or severe diarrhea occurs. Antiemetics or antidiarrheal medications may be considered during treatment with DUVYZAT. Discontinue DUVYZAT if the symptoms persist.
- QTc Prolongation: Avoid use of DUVYZAT in patients who are at an increased risk for ventricular arrhythmias.

Recommended Evaluation and Testing Before Initiation of DUVYZAT:

Obtain and evaluate baseline platelet counts and triglycerides prior to initiation of DUVYZAT. Do not initiate DUVYZAT in patients with a platelet count less than 150 x 10^9/L. Monitor platelet counts and triglycerides as recommended during treatment to determine if dosage modifications are needed.

In addition, in patients with underlying cardiac disease or taking concomitant medications that cause QT prolongation, obtain ECGs when initiating treatment with DUVYZAT, during concomitant use, and as clinically indicated.

Most Common Adverse Reactions:

Most common adverse reactions (≥10% in DUVYZAT-treated patients) are diarrhea, abdominal pain, thrombocytopenia, nausea/vomiting, hypertriglyceridemia, and pyrexia.

To report SUSPECTED ADVERSE REACTIONS, contact ITF Therapeutics LLC at 1-833-582-4312 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Please see full Prescribing Information and Medication Guide.

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C-DUV-US-0332 03/2025