Company Announcement

New Data Presented on Givinostat for Treatment of Duchenne Muscular Dystrophy at 2025 Neuromuscular Study Group Annual Scientific Meeting

Additional analyses from the givinostat clinical development program review observations on weight-based flexible dosing, characterization of thrombocytopenia, correlations between pharmacodynamic markers and functional outcomes, and respiratory function

CONCORD, Mass., September 26, 2025 – ITF Therapeutics LLC, the U.S. affiliate of Italfarmaco, today announced that results on givinostat for the treatment of Duchenne muscular dystrophy (DMD) are being presented at the 2025 Neuromuscular Study Group (NMSG) Annual Scientific Meeting held September 26-28 in Stresa, Italy. Poster presentations include analyses of functional outcomes based on weight-based flexible dosing in the pivotal Phase 3 EPIDYS trial, further characterization of thrombocytopenia, the link between vastus lateralis fat fraction (VLFF) measurement and efficacy outcomes, and observations on respiratory function.

"These presentations reflect our focus on generating data that support clinicians in assessing the benefit-risk profile and full potential of givinostat, including the opportunity to consider flexible dosing strategies that may help to optimize tolerability and efficacy for some patients," said Scott Baver, Ph.D., VP, Head of Medical Affairs. "We are pleased that new insights on givinostat are being shared with global leaders in DMD research, treatment, and patient advocacy at the 2025 NMSG Annual Scientific Meeting."

These data from studies sponsored by Italfarmaco presented at the 2025 NMSG Annual Scientific Meeting include:

#1213 Givinostat Weight-based Flexible Dosing: Rationale and Efficacy at the Different Doses

This poster includes an analysis of data on weight-based flexible dosing from the multicenter, randomized, double-blind, placebo-controlled, Phase 3 EPIDYS clinical trial. In the EPIDYS study, patients with DMD received weight-based givinostat dosing plus standard of care (i.e., corticosteroids) at two levels, dose A or B, depending on study protocol. Doses were adjusted (A to B or B to C) based on predefined rules related to tolerability to achieve the highest tolerated dose for each patient. This analysis assesses mean change from baseline in 4-stair climb (4SC) time and total North Star Ambulatory Assessment (NSAA) scores to evaluate efficacy based on the final administered dose at week 72.

#1207 Characterizing Thrombocytopenia in Patients with Duchenne Muscular Dystrophy Treated with Givinostat: Results from the Phase 3 EPIDYS Trial

Thrombocytopenia is a known adverse event of givinostat. This analysis further characterizes the risk and severity of thrombocytopenia or decreased platelet count based on an assessment of data from patients treated with givinostat in the Phase 3 EPIDYS study.

#1211 Vastus Lateralis Fat Fraction is Associated with Functional Efficacy Endpoints in Patients with Duchenne Muscular Dystrophy Treated with Givinostat

This poster reports findings from a post-hoc analysis assessing whether changes from baseline in VLFF correlate with measures of disease progression in patients with DMD. The analysis examines VLFF alongside efficacy endpoints observed at the end of the study (month 18), including 4SC, time to rise, 6-minute walking test, and NSAA.

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#1208 Givinostat Effect on Respiratory Function in Duchenne Muscular Dystrophy Before and After Ambulation Loss: Results from EPIDYS, LTSE, and PRO-DMD-01 Studies

This poster, which includes findings previously presented at the 2025 MDA Clinical and Scientific Conference, reports observations on respiratory function in patients who experienced loss of ambulation during follow-up. The analysis indirectly compares forced vital capacity (FVC) %-predicted trajectories from patients treated with givinostat and corticosteroids in the Phase 3 EPIDYS study and its ongoing open-label extension with FVC %-predicted trajectories from patients in a natural history study of DMD disease progression who received corticosteroids only.

For additional poster details, see abstract #1207, #1208, #1211, and #1213 published here.

"The data presented at NMSG highlight important insights into the patient experience, ranging from the impact of flexible dosing to how thrombocytopenia was observed and characterized in the clinical trial setting," said Aravindhan Veerapandiyan, M.D., Associate Professor of Pediatrics, Division of Pediatric Neurology at the University of Arkansas for Medical Sciences and Arkansas Children's Hospital. "Together, these findings contribute to a deeper understanding of givinostat's use in the treatment of DMD and may help inform clinical decision-making."

ITF Therapeutics is not a sponsor of the 2025 NMSG Annual Scientific Meeting.

About DUVYZAT® (givinostat)

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. 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.

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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.

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. Monitor blood count every 2 weeks for the first 2 months, at month 3, and every 3 months thereafter. Modify the dosage for confirmed thrombocytopenia. Discontinuation may be needed if abnormalities worsen.

Increased Triglycerides: DUVYZAT can cause elevations in triglycerides. Monitor triglycerides at 1 month, 3 months, 6 months, and then every 6 months thereafter. Modify the dosage if fasting triglycerides are verified >300 mg/dL. Treatment with DUVYZAT should be discontinued if triglycerides remain elevated despite adequate dietary intervention and dosage adjustment.

Gastrointestinal Disturbances: Gastrointestinal disturbances, including diarrhea, nausea/vomiting, and abdominal pain were common adverse reactions in DUVYZAT clinical trials. Antiemetics or antidiarrheal medications may be considered during treatment with DUVYZAT. Modify the dosage of DUVYZAT in patients with moderate or severe diarrhea and discontinue treatment if significant symptoms persist.

QTc Prolongation: DUVYZAT can cause prolongation of the QTc interval. Avoid use of DUVYZAT in patients who are at an increased risk for ventricular arrhythmias (including torsades de pointes), such as those with congenital long QT syndrome, coronary artery disease, electrolyte disturbance or in patients taking concomitant medicinal products known to cause QT prolongation. Obtain ECGs prior to initiating treatment with DUVYZAT in patients with underlying cardiac disease or in patients who are taking concomitant medications that cause QT prolongation.

Adverse Reactions

The most common adverse reactions reported in >5% of patients treated with DUVYZAT are diarrhea (37%), abdominal pain (34%), thrombocytopenia (33%), nausea/vomiting (32%), hypertriglyceridemia (23%), pyrexia (13%), myalgia (9%), rash (9%), arthralgia (8%), fatigue (8%), constipation (7%), and decreased appetite (7%).

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Drug Interactions

Closely monitor when DUVYZAT is used in combination with an oral CYP3A4 sensitive substrate or a sensitive substrate of the OCT2 transporter, for which a small change in substrate plasma concentrations may lead to serious toxicities.

Avoid concomitant use with other drugs that prolong the QTc interval; monitor ECG if concomitant use cannot be avoided. If concomitant use cannot be avoided, obtain ECGs when initiating, during concomitant use, and as clinically indicated. Withhold DUVYZAT if the QTc interval is >500 ms or the change from baseline is >60 ms.

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 for additional safety information.

DUVYZAT is a registered trademark of Italfarmaco S.p.A.

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