Company Announcement

ITF Therapeutics LLC to Present Patient-Reported Outcome Data for DUVYZAT® (givinostat) in Duchenne Muscular Dystrophy at ISPOR 2025

Analyses evaluate the potential impact of treatment with givinostat on slowing the decline in daily activities in patients with DMD

CONCORD, Mass., May 15, 2025 – ITF Therapeutics LLC, the U.S. affiliate of Italfarmaco, today announced a poster presentation at The Professional Society for Health Economics and Outcomes Research (ISPOR) conference being held May 13-16, 2025, in Montreal, Canada. The presentation includes analyses evaluating patient-reported outcome data from the double-blind, randomized, Phase 3 EPIDYS study, which compared the efficacy and safety of givinostat with placebo in ambulant boys six years of age and older with Duchenne muscular dystrophy (DMD). All patients also received corticosteroids during the study. DUVYZAT® (givinostat) is a histone deacetylase (HDAC) inhibitor indicated for the treatment of patients six years of age and older with DMD. Please see the Indication and Important Safety Information for DUVYZAT below.

"Patient and caregiver-reported outcomes are essential to understanding the realities of life with DMD and the potential impact of treatment with DUVYZAT. These patient-reported outcome data add an important layer to our ongoing clinical studies evaluating the safety and efficacy of DUVYZAT, and provide perspectives on how treatment may affect day-to-day activities," said Matt Trudeau, President, ITF Therapeutics. "We are pleased to share these data at ISPOR 2025 and are grateful for the individuals living with DMD who participated in our clinical trials, their families, and the researchers and healthcare professionals whose valued contributions have helped advance knowledge on the critical role of HDAC inhibition in supporting the management of DMD."

To evaluate patient-reported outcomes, researchers used the Pediatric Outcome Data Collection Instrument (PODCI), a standard tool for measuring daily activities that contribute to quality of life (QoL) in patients with DMD. The PODCI is comprised of a global function score and five subscales, including upper extremity function, transfer and basic mobility, sports/physical function, pain/comfort, and happiness. Scores range from 0 to 100, with higher scores indicating better QoL. PODCI was completed by participants or caregivers in the EPIDYS study at baseline and at months 12 and 18. For details on results from the study, see the abstract here.

Poster presentation details:

Title: Patient-Reported Outcomes From a Phase 3 Study of Givinostat in Patients With Duchenne Muscular Dystrophy
Presenter: Katiana Gruppioni, MPH, Director, Evidence Generation, ITF Therapeutics
Poster Session: 5
Poster Code: PCR230
Poster Session Date and Time: Friday, May 16, 2025, at 9:00-11:30 a.m. ET
Discussion Period: 9:00-10:00 a.m. ET

ITF Therapeutics LLC
575 Virginia Road, Suite 201
Concord, MA 01742
www.itftherapeutics.com

Italfarmaco
www.italfarmaco.com

Company Announcement

Indication and Important Safety Information for DUVYZAT® (givinostat)

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%).

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.

ITF Therapeutics LLC
575 Virginia Road, Suite 201
Concord, MA 01742
www.itftherapeutics.com

Italfarmaco
www.italfarmaco.com

Company Announcement

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.

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.

ITF Therapeutics LLC
575 Virginia Road, Suite 201
Concord, MA 01742
www.itftherapeutics.com

Italfarmaco
www.italfarmaco.com

Company Announcement

U.S.A.

Media inquiries: Brian Connor |+1 212 253 8881| bconnor@cglife.com

Other inquiries: Communications Lead U.S.A.| m.mckennitt@itftherapeutics.com

Global

Media inquiries: Charlotte Spitz or Jacob Verghese |+49 (0)151 7441 6179| italfarmaco@trophic.eu

Other inquiries: Patient Advocacy and Communications Lead| s.parker@italfarmaco.com