Company Announcement

ITF Therapeutics LLC Announces Multiple Data Presentations at 2025 MDA Clinical and Scientific Conference

Presenting seven abstracts demonstrating consistency of effect, long-term safety, and reduced progression of disease with givinostat

Results also demonstrate delayed respiratory decline with givinostat

CONCORD, Mass., February 20, 2025 – ITF Therapeutics LLC, the U.S. affiliate of Italfarmaco, today announced that seven abstracts have been accepted as poster presentations at the Muscular Dystrophy Association (MDA) Clinical and Scientific Conference to be held March 16-19, 2025, in Dallas, Texas.

The presentations include data highlighting the long-term safety and efficacy of treatment with givinostat in patients with Duchenne muscular dystrophy (DMD). Presentations also include analyses of the effect of givinostat on respiratory function in patients with DMD before and after loss of ambulation, disease progression models comparing the standard of care with givinostat, and updates on the design of an observational study to assess real-world outcomes of patients treated with givinostat. Please see the Indication and Important Safety Information for DUVYZAT™ (givinostat) below.

"In 2024, we attended our first MDA Clinical and Scientific Conference to listen to the Duchenne community and learn about their unique needs and goals as we prepared for the U.S. FDA approval of DUVYZAT for the treatment of DMD," said Matt Trudeau, President, ITF Therapeutics. "These insights were instrumental in shaping our commercialization strategy that enabled us to provide access to hundreds of patients within just the first few months. One year later, we are looking forward to returning to this important meeting to provide the community with updates including a range of data on DUVYZAT clinical performance and information about our ongoing research on the role of HDAC inhibition in the management of DMD."

Poster presentations

- **Title**: Givinostat Efficacy in Duchenne Muscular Dystrophy: Natural History Comparison Applying Propensity Score Matching
  Presenter: David Gómez Andrés, Pediatric Neurologist, Hospital Universitari Vall d'Hebron
  Poster number: P87

- **Title**: Givinostat Effect on Respiratory Function in Duchenne Muscular Dystrophy Before and After Ambulation Loss: Results from EPIDYS, OLE, and PRO-DMD-01
  Presenter: Craig M. McDonald, M.D., Chair, Department of Physical Medicine & Rehabilitation, UC Davis Health
  Poster number: P80

- **Title**: Long-term Safety of Givinostat in Patients with Duchenne Muscular Dystrophy: Results From an Open-label Extension Study
  Presenter: John F. Brandsema, M.D., Pediatric Neurologist, Division of Neurology, Children's Hospital of Philadelphia
  Poster number: P275

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- **Title**: Efficacy of Givinostat in the Off-target Population of EPIDYS: A Subgroup Analysis
  Presenter: Richard S. Finkel, M.D., Director, Center for Experimental Neurotherapeutics, St. Jude Children's Research Hospital
  Poster number: P77

- **Title**: Disease Progression Modeling in Duchenne Muscular Dystrophy: Delayed Decay in 4-stair Climb with Givinostat Compared with Standard of Care
  Presenter: Chamindra G. Laverty, M.D., Associate Clinical Professor of Neurosciences, University of California, San Diego
  Poster number: P76

- **Title**: Modeling Disease Progression in Duchenne Muscular Dystrophy: Reduced Decline in Forced Vital Capacity with Givinostat Compared with Standard of Care
  Presenter: Erika L. Finanger, M.D., M.S., Professor of Pediatrics, Division of Neurology, School of Medicine, Oregon Health & Science University
  Poster number: P85

- **Title**: Design of an Observational Study to Assess Real-world Outcomes of Patients with Duchenne Muscular Dystrophy in the US Treated with Givinostat
  Presenter: Erika L. Finanger, M.D., M.S., Professor of Pediatrics, Division of Neurology, School of Medicine, Oregon Health & Science University
  Poster number: P84

As an Impact Sponsor of the MDA Clinical and Scientific Conference, ITF Therapeutics is also supporting two special events during the meeting:

- **Title:** Neuromuscular Advocacy Collaborative Meeting (Invitation Only)
  - **Date and time**: Sunday, March 16, 2025, from 2:00-5:00 p.m. CST
  - Advocacy partners from across the neuromuscular disease field will come together to reflect on 2024 achievements, discuss future priorities, and build collaborative strategies to achieve shared goals in 2025.
- **Title:** Industry Forum Breakfast
  - **Date and time**: Monday, March 17, 2025, from 7:00-8:00 a.m. CST
  - Presenters will discuss key considerations related to the role of histone deacetylase (HDAC) inhibition in supporting the management of DMD.

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.

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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.

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.

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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.

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

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