Company Announcement

ITF Therapeutics LLC Announces U.S. Commercial Availability of DUVYZAT™ (givinostat) for Treatment of Patients With Duchenne Muscular Dystrophy

First nonsteroidal treatment approved for patients six years of age and older with DMD regardless of genetic mutation now available in the U.S.

ITF also announces launch of ITF ARC (Access, Resources and Care) patient services program to bring suite of support and educational resources to patients and their families

CONCORD, Mass., July 25, 2024 – ITF Therapeutics LLC, the U.S.-based rare disease commercial arm of Italfarmaco, today announced the U.S. commercial launch of DUVYZAT™ (givinostat), a histone deacetylase inhibitor, for the treatment of patients six years of age and older with Duchenne muscular dystrophy (DMD). DUVYZAT was approved by the U.S. Food and Drug Administration (FDA) on March 21, 2024.

"Following the FDA approval of DUVYZAT, our team has been focused on making this new treatment option available to the DMD community as rapidly as possible. We have also deployed field teams and easy-to-navigate communication platforms and services to provide patients, caregivers, and clinicians with the information and resources they need to make informed decisions about treatment with DUVYZAT for DMD. This includes our recently launched patient services program, ITF ARC, which offers helpful information in areas including navigating insurance coverage. We are very grateful for the guidance that we received from DMD advocacy leaders throughout the development of ITF ARC. Their input has been fundamental in helping us design the suite of services we are offering to the community," said Matt Trudeau, president, ITF Therapeutics. "We also express our gratitude to the individuals living with DMD who participated in our clinical trials, their families, DMD advocacy leaders, and the healthcare professionals who have all played a central role in helping us make DUVYZAT available to the DMD community."

To help enable access to treatment with DUVYZAT for appropriate patients in the U.S., the ITF ARC program from ITF Therapeutics includes services to help patients and families navigate through insurance challenges, personalized pharmacist support, financial and access assistance for eligible patients, educational materials, and other resources. For more information about patient support services offered through ITF ARC, visit www.DUVYZAT.com or call 1-855-448-3272.

After confirming DUVYZAT is an appropriate therapy for a patient, physicians can visit www.DUVYZAT.com to access information about prescribing DUVYZAT and initiate the prescription fulfillment process through PANTHERx® Rare, a leading specialty pharmacy provider that specializes in supporting patients with rare diseases.

The FDA approval of DUVYZAT was supported by the results of the pivotal multicenter, randomized, double-blind, placebo-controlled Phase 3 EPIDYS clinical trial (NCT02851797). In the EPIDYS study, a total of 179 ambulant boys six years of age or older received glucocorticosteroid treatment, and either DUVYZAT twice daily or placebo. The EPIDYS study met its primary endpoint demonstrating that patients treated with DUVYZAT showed a statistically significant and clinically meaningful difference in time to complete the four-stair climb assessment. DUVYZAT also showed favorable results compared to placebo on key secondary endpoints including the North Star Ambulatory Assessment (NSAA) and fat infiltration evaluation by magnetic resonance spectroscopy. The majority of adverse effects

observed with DUVYZAT were mild to moderate in severity. Results from this study were published in *The Lancet Neurology* in March 2024.

DUVYZAT received priority review, orphan drug, and rare pediatric disease designations from the FDA. A Marketing Authorization Application for givinostat as a potential treatment for DMD is currently being reviewed by the European Medicines Agency (EMA). Italfarmaco has an international presence and has initiated or plans to initiate discussions with regulatory agencies in other geographies to support the review of givinostat clinical data.

For more information about DUVYZAT, visit www.DUVYZAT.com.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a severe neuromuscular genetic disease characterized by progressive muscle weakness and degeneration and is the most common type of muscular dystrophy globally. DMD is caused by mutations in the dystrophin gene that result in the absence of a functional dystrophin protein. Without dystrophin, muscle fibers are highly susceptible to injury and this continuous muscle injury typically leads to chronic inflammation, impairment of muscle regeneration, and muscle replacement by fibrotic and fat tissue. The disease primarily affects boys, with symptoms usually first seen between two and five years of age. Symptoms worsen over time and affect the ability to walk. Eventually, heart and respiratory muscles are affected, which are the two main causes of premature death. DMD incidence is approximately one in every 3,500-6,000 male births worldwide.

About DUVYZAT™ (givinostat)

DUVYZAT is a U.S. FDA approved therapy 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). DUVYZAT is a histone deacetylase (HDAC) inhibitor that modulates the deregulated activity of HDACs in the dystrophic muscle, which is a major consequence of the lack of dystrophin associated with DMD. Though the exact mechanism of action of DUVYZAT is unknown, it is believed to inhibit HDAC pathological overactivity to address the cascade of events leading to muscle damage, thereby counteracting the disease pathology and slowing down muscle deterioration.

About ITF Therapeutics LLC

ITF Therapeutics was launched in January 2024 as the U.S. commercial arm of Italfarmaco focused on the development and commercialization of products to treat rare diseases including Duchenne muscular dystrophy. 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 also reflects Italfarmaco's goal to build a world-class team of experts that share a passion to make a positive impact for rare disease communities. For more information visit www.itftherapeutics.com.

ITF Therapeutics LLC
300 Baker Avenue, Suite 280
Concord, MA 01742
www.itftherapeutics.com

Italfarmaco
www.italfarmaco.com

DUV-US-0079 07/2024

Company Announcement

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 and Important Safety Information

What is DUVYZAT?

DUVYZAT is a prescription medicine that is used for the treatment of Duchenne muscular dystrophy (DMD) in people 6 years of age and older.

It is not known if DUVYZAT is safe and effective in children under 6 years of age.

Important Safety Information

What is the most important information I should know about DUVYZAT?

- **Low platelet counts in your blood (thrombocytopenia)**. Platelets are important for blood clotting, and a decrease in their numbers can lead to an increased risk of bleeding or bruising. Your healthcare provider will check your blood count before you start DUVYZAT and regularly during treatment for any signs of thrombocytopenia. Call your healthcare provider right away if you notice any unusual bleeding or small red or purple spots on the skin called petechiae. Your healthcare provider may change your dose of DUVYZAT if your blood platelet counts continue to be low or may stop your treatment with DUVYZAT.
- **Increased levels of fat (triglycerides) in your blood.** You may not have any symptoms, so your healthcare provider will do blood tests before you start DUVYZAT and regularly during treatment to check your triglyceride levels. Your healthcare provider may change your dose of DUVYZAT if your triglyceride levels continue to be high or may stop your treatment with DUVYZAT.
- **Frequent watery loose stools (diarrhea) and vomiting.** DUVYZAT can cause vomiting and moderate to severe diarrhea. If diarrhea occurs, you should keep track of the frequency and severity of your diarrhea symptoms, drink plenty of fluids, and contact your healthcare provider. Your healthcare provider may change your dose of DUVYZAT if the diarrhea cannot be managed or does not go away. Your healthcare provider may also stop your treatment with DUVYZAT.

ITF Therapeutics LLC
300 Baker Avenue, Suite 280
Concord, MA 01742
www.itftherapeutics.com

Italfarmaco
www.italfarmaco.com

DUV-US-0079 07/2024

Company Announcement

Before Taking DUVYZAT, tell your healthcare provider about all of your medical conditions, including if you:

- have any heart problems or if you take any medicines that could increase your chance for irregular heart rhythms.
- have any bleeding problems.

Tell your healthcare provider about all of the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

Taking DUVYZAT with certain other medicines may affect each other. Taking DUVYZAT with other medicines can cause serious side effects. Do not start or stop other medicines without talking to your healthcare provider.

DUVYZAT can cause serious side effects, including:

- **See "What is the most important information I should know about DUVYZAT?"**
- **changes in the electrical activity of your heart called QT Prolongation**. QT Prolongation can increase the risk of developing a type of irregular heart rhythm known as Torsades de Pointes. Call your healthcare provider right away if you feel faint, have an irregular heartbeat, feel dizzy, or lose consciousness.

The most common side effects of DUVYZAT included diarrhea, nausea, vomiting, stomach pain, low platelet counts in the blood, increased fat level in the blood and fever.

These are not all of the possible side effects of DUVYZAT. For more information, ask your healthcare provider or pharmacist.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Please see full Prescribing Information and Medication Guide.

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

USA

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

Other inquiries: Patient Advocacy and Communications Lead USA | c.allen@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@italfarmacogroup.com

ITF Therapeutics LLC
300 Baker Avenue, Suite 280
Concord, MA 01742
www.itftherapeutics.com

Italfarmaco
www.italfarmaco.com

DUV-US-0079 07/2024