

DUVYZAT™ (givinostat) FAQ

- **What is DUVYZAT™?**
 - DUVYZAT™ (givinostat) is a histone deacetylase (HDAC) inhibitor. DUVYZAT™ works by targeting pathogenic processes to reduce inflammation and loss of muscle.

- **Is my child eligible for DUVYZAT™?**
 - DUVYZAT™ is approved for patients with Duchenne who are six years of age and older. Currently, it is not indicated for the treatment of individuals younger than six years of age.
 - DUVYZAT™ is not specific to genetic variants nor does it have any contraindications related to specific genetic variants (meaning all genetic variants should be eligible).

- **How does my child get access to DUVYZAT™?**
 - If you want to know if your child is eligible to receive DUVYZAT™ and if your neuromuscular center is able to prescribe it, call your neuromuscular team, who will be able to provide you with the most appropriate information on next steps.
 - If your child is eligible for DUVYZAT™ your neuromuscular provider will start the process of determining insurance coverage of the medication, called a prior authorization, and send a prescription for DUVYZAT™ to a specialty pharmacy, where the prescription will be filled and shipped to your home.
 - ITF Therapeutics anticipates that DUVYZAT™ will become accessible to patients in the third quarter of 2024.
 - *PPMD will update this FAQ when there is more information about how and when DUVYZAT will become accessible to patients.*

- **How soon will my child be able to access DUVYZAT™?**
 - Now that the FDA has approved DUVYZAT™, the drug company responsible for its commercialization, ITF Therapeutics, will work to make the drug available to individuals with a prescription. The process of commercialization can take weeks to months. ITF Therapeutics currently expects that drug will be available to patients in the third quarter of 2024.

- **How is DUVYZAT™ administered?**
 - DUVYZAT™ is an oral suspension (liquid) given by mouth two (2) times per day with food.
 - The recommended dosage of DUVYZAT™ is determined by the individual's body weight and ranges from 22.2mg (2.5mL) twice daily to 53.2mg (6mL) twice daily.

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

- It's important to understand the potential side effects of treatment, including DUVYZAT™. Be sure to talk with your neuromuscular team about risks, benefits, and precautions before starting any medications.
- Patients with platelet counts less than 150 x 10⁹/L should not take DUVYZAT™. Talk to your neuromuscular provider about whether you should take DUVYZAT™.
- **What are possible side effects of DUVYZAT™?**
 - The most common side effects include diarrhea, abdominal pain, a decrease in platelets (which can lead to increased bleeding), nausea/vomiting, an increase in triglycerides (a type of fat in the body) and fever.
- **What precautions are needed while taking DUVYZAT™?**
 - The prescribing information for DUVYZAT™ includes warnings which state that health care providers should evaluate the patient's platelet counts and triglycerides before prescribing DUVYZAT™. Patients with a platelet count less than 150 x 10⁹/L should not take DUVYZAT™. Platelet counts and triglycerides should be monitored as recommended during treatment to determine if changes in dosage are needed.
 - Dosage modifications may also be needed for moderate or severe diarrhea.
 - DUVYZAT™ may also cause QTc prolongation, which can increase the risk for irregular heartbeats. Your doctor will monitor your heart rhythm with an electrocardiogram (ECG) to ensure it remains safe to take DUVYZAT™.
 - Patients taking certain medications that also cause QTc prolongation or have certain types of heart disease should avoid taking DUVYZAT™.
- **How often will I need to follow up with my doctor?**
 - It's important to talk to your neuromuscular provider before starting DUVYZAT™ to understand if more frequent visits or labs will be needed.
- **Will insurance cover DUVYZAT™?**
 - Because it has only recently been approved, insurance companies are still evaluating their coverage policies for DUVYZAT™. If you have questions about whether your insurance covers DUVYZAT™, you can call your insurance company directly to ask about their coverage policy. PPMD is closely tracking insurance plans and will provide updates and support as additional information becomes available.
 - You can learn more about DUVYZAT™ at www.DUVYZAT.com.
 - PPMD has resources to support you as you navigate insurance coverage benefits and reimbursements. Schedule time with the PPMD team to understand your insurance benefits through the PPMD For You "[Navigating Benefits and Resources](#)" appointment type.
- **What if my child is not eligible for DUVYZAT™?**

- DUVYZAT™ is currently approved for individuals with Duchenne six years of age and older. Clinical studies are underway in individuals to support a broader label in the future.
- Although DUVYZAT™ is not variant-specific (meaning all genetic variants should be eligible), PPMD does have genetic counselors and clinical experts on staff who can help you understand your child's genetic variant and which types of treatments may be available. Email the PPMD genetic counselors at coordinator@parentprojectmd.org or the PPMD care team at careteam@parentprojectmd.org.
- **What clinical studies are ongoing or planned for DUVYZAT™?**
 - If you are interested in clinical trials or want to know more about actively recruiting trials, visit clinicaltrials.gov, [PPMD's Clinical Trials site](#), or reach out to one of PPMD's genetic counselors (coordinator@parentprojectmd.org).
- **Where can I get more answers to my questions about DUVYZAT™?**
 - If you have more questions about DUVYZAT™, talk to your neuromuscular provider.
 - For Indication and Important Safety Information, Full Prescribing Information, a Patient Medication Guide, and ITF Therapeutics' list of FAQs, visit <https://www.duvyzat.com/>
 - If you have general questions about approved or investigational therapies in Duchenne and whether your child is eligible, contact the PPMD Genetic Counselors at coordinator@parentprojectmd.org.
 - If you have questions about access, navigating care, or are looking for other types of individualized support, connect with the PPMD Team directly through [PPMD for You](#).