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## **DUVYZAT (givinostat)**

### **RATIONALE FOR INCLUSION IN PA PROGRAM**

#### **Background**

Duvyzat (givinostat) is a histone deacetylase inhibitor indicated for the treatment of Duchenne muscular dystrophy (DMD). The precise mechanism by which Duvyzat exerts its effects in patients with DMD is unknown (1).

#### **Regulatory Status**

FDA-approved indication: Duvyzat is a histone deacetylase inhibitor indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 6 years of age and older (1).

Duvyzat has been associated with hematological changes, increased triglycerides, gastrointestinal disturbances, and QTc prolongation. Thrombocytopenia and other signs of myelosuppression, including decreased hemoglobin and neutropenia may occur with Duvyzat use. Complete blood counts should be monitored every 2 weeks for the first 2 months of treatment, then monthly for the first 3 months, and every 3 months thereafter. Triglycerides should also be monitored at 1 month, 3 months, 6 months, and every 6 months thereafter. Gastrointestinal disturbances such as diarrhea, nausea/vomiting, and abdominal pain were common adverse reactions related to Duvyzat use. Treatment dosage should be modified or discontinued depending on severity. Duvyzat may cause QTc prolongation. ECGs should be obtained prior to treatment in patients with underlying cardiac disease or in patients who are taking concomitant QT prolonging medications (1).

In the study, patients had received systemic corticosteroids for at least 6 months and Duvyzat was administered in addition to a stable dose of corticosteroids throughout the study (1).

Monitoring motor changes in patients with DMD requires functional evaluation along with measurement of muscle strength. The need for a reliable outcome measure in diseases of rapid deterioration such as DMD has led to the use of motor functional tests. In a large, multicenter, international clinical trial, the six minute walk test (6MWT) proved to be feasible and highly reliable. Also used are the Motor Function Measure (MFM) and North Star Ambulatory Assessment (NSAA) to help predict loss of ambulation 1 year before its occurrence in order to allow time to adapt rehabilitation, change the patient's environment, and consider acquisition of assistive aids or the use of medications. The timed 4-stair climb test (4SC) is another accepted and widely used tool to



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assess motor function of patients with neuromuscular diseases (2-5).

The safety and effectiveness of Duvyzat in pediatric patients less than 6 years of age have not been established (1).

### **Summary**

Duvyzat (givinostat) is a histone deacetylase inhibitor indicated for the treatment of Duchenne muscular dystrophy (DMD). Duvyzat has been associated with hematological changes, increased triglycerides, gastrointestinal disturbances, and QTc prolongation. Safety and effectiveness in patients less than 6 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Duvyzat while maintaining optimal therapeutic outcomes.

### **References**

1. Duvyzat [package insert]. Concord, MA: ITF Therapeutics, LLC; November 2024.
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4. Vuillerot C, Girardot F, et al. Monitoring changes and predicting loss of ambulation in Duchenne muscular dystrophy with the Motor Function Measure. *Developmental Medicine & Child Neurology* 2010, 52: 60–65.
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