PRIOR AUTHORIZATION POLICY

POLICY: Muscular Dystrophy – Duvyzat Prior Authorization Policy
- Duvyzat™ (givinostat oral suspension – ITF Therapeutics)

REVIEW DATE: 06/19/2024

OVERVIEW

Duvyzat, a histone deacetylase (HDAC) inhibitor, is indicated for the treatment of **Duchenne muscular dystrophy** (DMD) in patients ≥ 6 years of age.[1]

Disease Overview

DMD is a rare, progressive X-linked disease resulting from mutation(s) of the DMD gene, also known as the Dystrophin gene.[2,3] Due to the mutation(s), the dystrophin protein, which is key for maintaining the structural integrity of muscle cells, is not produced or very minimally produced. Since this is an X-linked mutation, DMD almost exclusively impacts young boys. DMD is a progressive muscle-weakening disease that affects skeletal, respiratory, and cardiac muscles. It is usually diagnosed in the second or third year of life. Due to progressive decline, most patients die of cardiac or respiratory complications in the third or fourth decade of life. The incidence of DMD in the US is approximately 1 in 5,000 live male births.

Guidelines

Duvyzat is not addressed in guidelines. Guidelines from the DMD Care Considerations Working Group (2018) state that glucocorticoids and physical therapy are the mainstays of treatment for DMD.[2-6] Both therapies should be continued after the patient loses ambulation. Guidelines for the use of corticosteroids in DMD are available from the American Academy of Neurology (AAN) [2016, reaffirmed January 2022].[4] The AAN notes that in patients with DMD, prednisone should be used to improve strength and pulmonary function (moderate evidence). Deflazacort and prednisone may be used to improve timed motor function, reduce the need for scoliosis surgery, and to delay the onset of cardiomyopathy by 18 years of age (weak evidence). Deflazacort may also be used to improve pulmonary function and to delay the age at loss of ambulation by 1.4 to 2.5 years (weak evidence). There is insufficient evidence to support or refute the benefit of prednisone on survival (insufficient evidence). Deflazacort may be used to increase survival at 5 and 15 years of follow-up (weak evidence).

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of Duvyzat. All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Duvyzat as well as the monitoring required for adverse events and long-term efficacy, approval requires Duvyzat to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Documentation: Documentation is required for use of Duvyzat as noted in the criteria as **[documentation required]**. Documentation may include, but is not limited to, chart notes, prescription claims records, prescription receipts, and/or other information.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Duvyzat is recommended in those who meet the following criteria:

FDA-Approved Indication

1. **Duchenne Muscular Dystrophy.** Approve for 1 year if the patient meets ONE of the following (A or B):
   - **A)** Initial Therapy. Approve if the patient meets ALL of the following (i, ii, iii, iv, and v):
     - **i.** Patient is ≥ 6 years of age; AND
     - **ii.** Patient's diagnosis of Duchenne Muscular Dystrophy is confirmed by genetic testing with a confirmed pathogenic variant in the dystrophin gene **[documentation required]**; AND
     - **iii.** Patient is ambulatory; AND
     - **iv.** Patient is on a stable systemic corticosteroid therapy for at least 6 months; AND
     - **v.** The medication is prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy and/or neuromuscular disorders.
   - **B)** Patient is Currently Receiving Duvyzat. Approve if the patient meets ALL of the following (i, ii, iii, iv, and v):
     - **i.** Patient is ≥ 6 years of age; AND
     - **ii.** Patient is ambulatory; AND
     - **iii.** Patient is continuing to receive stable systemic corticosteroid therapy; AND
     - **iv.** According to the prescriber, the patient continues to benefit from therapy, as demonstrated by a stabilization or slowed decline on timed function tests (e.g., 4-stair climb, 6-minute walk test, time-to-rise) or in the North Star Ambulatory Assessment (NSAA) score; AND
     - **v.** The medication is prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy and/or neuromuscular disorders.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Duvyzat is not recommended in the following situations:

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Duvyzat® oral suspension [prescribing information]. Concord, MA: ITF Therapeutics, LLC; March 2024.
2. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. *Lancet Neurol*. 2018;17(3):251-267.
3. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. *Lancet Neurol*. 2018;17(4):347-361.
4. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 3: primary care, emergency medicine, psychological care, and transitions of care across the lifespan. *Lancet Neurol*. 2018;17(5):445-455.
5. Gloss D, Moxley RT III, Ashwal S, Oskoui M. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy: report of the Guideline Development Subcommittee of the American Academy of Neurology. *Neurology*. 2016;86(5):465-472.
6. Summary of Practice Guidelines for Clinicians. Practice Guideline Update: Corticosteroid Treatment of Duchenne Muscular Dystrophy. Available at: https://www.aan.com/Guidelines/Home/GuidelineDetail/731. Accessed on June 18, 2024.