Drug Coverage Policy

Effective Date.....07/01/2025
Coverage Policy Number.....IP0651
Policy Title.....Duvyzat

Muscular Dystrophy – Duvyzat

- Duvyzat™ (givinostat oral suspension – ITF Therapeutics)

INSTRUCTIONS FOR USE
The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Each coverage request should be reviewed on its own merits. Medical directors are expected to exercise clinical judgment where appropriate and have discretion in making individual coverage determinations. Where coverage for care or services does not depend on specific circumstances, reimbursement will only be provided if a requested service(s) is submitted in accordance with the relevant criteria outlined in the applicable Coverage Policy, including covered diagnosis and/or procedure code(s). Reimbursement is not allowed for services when billed for conditions or diagnoses that are not covered under this Coverage Policy (see "Coding Information" below). When billing, providers must use the most appropriate codes as of the effective date of the submission. Claims submitted for services that are not accompanied by covered code(s) under the applicable Coverage Policy will be denied as not covered. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

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; retired February 2025].[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).

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POLICY STATEMENT
Prior Authorization is required for 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.

Duvyzat is considered medically necessary when the following are met:

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.

When coverage is available and medically necessary, the dosage, frequency, duration of therapy, and site of care should be reasonable, clinically appropriate, and supported by evidence-based literature and adjusted based upon severity, alternative available treatments, and previous response to therapy.

Receipt of sample product does not satisfy any criteria requirements for coverage.

Conditions Not Covered

Duvyzat for any other use is considered not medically necessary. 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 May 12, 2025.

Revision Details

Type of Revision	Summary of Changes	Date
New	New policy	11/1/2024

Annual Revision	No criteria changes.	07/01/2025

The policy effective date is in force until updated or retired.