Drug Coverage Policy

Effective Date……04/01/2026
Coverage Policy Number……IP0131
Policy Title…… Deflazacort

Muscular Dystrophy – Deflazacort

- Emflaza™ (deflazacort tablets and oral suspension - PTC Therapeutics, generic)
- Jaythari (deflazacort tablets and oral suspension – Zydus)
- Kymbee (deflazacort tablets – Upsher-Smith)
- Pyquvi™ (deflazacort oral suspension – Aucta)

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

Deflazacort, a corticosteroid, is indicated for the treatment of **Duchenne muscular dystrophy** (DMD) in patients ≥ 2 years of age.[1] The efficacy and safety of deflazacort have not been established in patients < 2 years of age. Jaythari, Kymbee, and Pyquvi are indicated for DMD in patients ≥ 5 years of age.[9-12] Due to PTC Therapeutics marketing exclusivity rights, these agents are not approved in patients < 5 years of age.

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 males. 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.

Clinical Efficacy
The efficacy and safety of deflazacort was established in two pivotal trials in males with DMD who were ≥ 5 years of age.[7,8] In one study, treatment consisted of deflazacort 0.9 mg/kg/day, deflazacort 1.2 mg/kg/day, or prednisone 0.75 mg/kg/day (n = 196).[7] The primary efficacy analysis, mean change from baseline to Week 12 in average muscle strength (assessed by modified Medical Research Council [MRC]), demonstrated a significant least squares (LS) mean difference in favor of active treatment vs. placebo: deflazacort 0.9 mg/kg/day (0.25 vs. -0.1, P = 0.17), deflazacort 1.2 mg/kg/day (0.36 vs. -0.1, P = 0.0003), and prednisone 0.75 mg/kg/day (0.37 vs. -0.1, P = 0.0002). Adverse events (AEs) differed between prednisone and deflazacort treatment groups. Cushingoid appearance (69.4%), erythema (41.8%), and hirsutism (39.3%) were observed in a numerically greater proportion of patients in the prednisone group compared with either dose of deflazacort. Central obesity was reported in a statistically significantly greater proportion of patients treated with prednisone vs. deflazacort. Psychiatric AEs were generally reported at a higher rate in the prednisone group compared with both deflazacort groups.

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. Previously, guidelines for the use of corticosteroids in DMD were available from the American Academy of Neurology (AAN) [2016, reaffirmed January 2022].[4] These guidelines were retired as of February 2025 and new guidelines are not available.

Coverage Policy

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

Documentation: Documentation is required for use of deflazacort 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. All documentation must include patient-specific identifying information.

Deflazacort 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 ≥ 2 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 meets ONE of the following conditions (a or b):
       - **a)** Patient has tried prednisone or prednisolone for ≥ 6 months **[documentation required]** AND according to the prescriber, the patient has had at least ONE of the following significant intolerable adverse effects [1, 2, 3, or 4]:
         - **1)** Cushingoid appearance **[documentation required]**; OR
         - **2)** Central (truncal) obesity **[documentation required]**; OR
         - **3)** Undesirable weight gain defined as ≥ 10% of body weight gain increase over a 6-month period **[documentation required]**; OR
         - **4)** Diabetes and/or hypertension that is difficult to manage according to the prescriber **[documentation required]**; OR
       - **b)** According to the prescriber, the patient has experienced a severe behavioral adverse event while on prednisone or prednisolone therapy that has or would require a prednisone or prednisolone dose reduction **[documentation required]**; AND
     - **iv.** The medication is prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy and/or neuromuscular disorders.
     - **v.** Preferred product criteria is met for the product(s) as listed in the below table(s)
   - **B)** Patient is Currently Receiving Deflazacort. Approve if the patient meets ALL of the following (i, ii, iii, and iv):
     - **i.** Patient is ≥ 2 years of age; AND
     - **ii.** Patient has tried prednisone or prednisolone **[documentation required]**; AND
     - **iii.** According to the prescriber, the patient has responded to or continues to have improvement or benefit from deflazacort therapy **[documentation required]**; AND
       Note: Examples of improvement or benefit from deflazacort therapy would include improvements in motor function (e.g., time from supine to standing, time to climb four stairs, time to run or walk 10 meters, 6-minute walk test), improvement in muscle strength, improved pulmonary function, etc.
     - **iv.** The medication is prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy and/or neuromuscular disorders.

Employer Plans:

Product	Criteria
Emflaza (deflazacort tablets)	Patient meets BOTH of the following (A and B): **A)** Patient has tried one of generic deflazacort tablets, Jaythari tablets, or Kymbee tablets **[documentation required]**; AND **B)** Patient cannot take generic deflazacort tablets, Jaythari tablets, or Kymbee tablets due to a formulation difference in the inactive ingredient(s) [e.g., difference in dyes, fillers, preservatives] between the brand and bioequivalent generic product which, per the prescriber, would result in a significant

Product	Criteria
	allergy or serious adverse reaction **[documentation required].**
Emflaza (deflazacort oral suspension)	Patient meets BOTH of the following (A and B): **A)** Patient has tried one of generic deflazacort oral suspension, Jaythari oral suspension, or Pyquvi oral suspension **[documentation required]**; AND **B)** Patient cannot take Pyquvi or generic deflazacort oral suspension due to a formulation difference in the inactive ingredient(s) [e.g., difference in dyes, fillers, preservatives] between the brand and bioequivalent generic product which, per the prescriber, would result in a significant allergy or serious adverse reaction **[documentation required]**.

Conditions Not Covered

Deflazacort for any other use is considered not medically necessary. Criteria will be updated as new published data are available.

References

1. Emflaza™ tablets and oral suspension [prescribing information]. South Plainfield, NJ: PTC Therapeutics; June 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. Retired February 2025. Available at: https://www.aan.com/Guidelines/Home/GuidelineDetail/731. Accessed on January 19, 2026.
7. Griggs RC, Miller JP, Greenberg CR, et al. Efficacy and safety of Emflaza vs prednisone and placebo for Duchenne muscular dystrophy. *Neurology*. 2016;87(20):2123-2131.
8. Angelini C, Pegoraro E, Turella E, et al. Emflaza in Duchenne dystrophy: study of long-term effect. *Muscle Nerve*. 1994;17(4):386-391.
9. Jaythari tablets [prescribing information]. Pennington, NJ: Zydus Pharmaceuticals; May 2025.
10. Pyquvi™ oral suspension [prescribing information]. Piscataway, NJ: Aucta Pharmaceuticals; February 2025.
11. Kymbee™ tablets [prescribing information]. Maple Grove, MN: Upsher-Smith Laboratories; July 2025.
12. Jaythari oral suspension [prescribing information]. Pennington, NJ: Zydus Pharmaceuticals; November 2025.

Revision Details

Type of Revision	Summary of Changes	Date
Annual Revision	• Deleted "…or likely pathogenic variant" for genetic testing criteria in regard to dystrophin gene. • Replaced "time to run or walk 30 feet" with "time to run or walk 10 meters" for Emflaza improvements. • Added 6-minute walk test to motor function tests for Emflaza improvements.	05/01/2024
Selected Revision	Emflaza tablets are available as generic deflazacort tablets. Within the policy changed Emflaza to deflazacort wherever applicable.	06/01/2024
Annual Revision	**Removed** "generic for tablets only" from policy heading since the oral suspension is now available as a generic. **Duchenne Muscular Dystrophy:** **Updated** the diagnosis to require confirmation by genetic testing with a confirmed pathogenic variant in the dystrophin gene. **Removed** the criteria requiring a muscle biopsy showing the absence of, or marked decrease in, dystrophin protein for diagnosis confirmation. **Added** the requirement that the patient has tried prednisone or prednisolone for ≥ 6 months and according to the prescriber, experienced at least one significant intolerable adverse effect: Cushingoid appearance, central (truncal) obesity, undesirable weight gain (≥ 10% body weight increase over 6 months), or difficult-to-manage diabetes and/or hypertension. **Updated** the requirement that the patient has experienced significant adverse effects while on prednisone or prednisolone therapy to now state that "according to the prescriber, the patient has experienced significant a severe behavioral adverse effects event while on prednisone or prednisolone therapy that has or would require a prednisone or prednisolone dose reduction." **Added** "documentation required" for use of deflazacort as noted in the criteria.	10/15/2024
Annual Revision	No criteria changes.	04/15/2025
Selected Revision	**Added** preferred product criteria for Emflaza tablets and oral suspension on Employer Plans, effective 7/1/2025.	05/15/2025

Selected Revision	**Added** branded generic Jaythari to the policy with the same criteria applied as the other deflazacort products. **Added** branded generic Pyquvi to the policy with the same requirements as the other deflazacort products. **Added** Jaythari and Pyquvi to the preferred product table for Employer Plans.	11/15/2025
Selected Revision	**Added** branded generic Kymbee to the policy with the same criteria applied as the other deflazacort products. **Updated** exception criteria to include Kymbee tablets in the Preferred Products Table.	02/15/2026
Annual Revision	Jaythari oral suspension was added to the policy. No criteria changes. Emflaza oral suspension preferred product requirements updated with the addition of Jaythari oral suspension as a preferred product alternative option.	04/01/2026

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