



PRIOR AUTHORIZATION POLICY

- POLICY:** Muscular Dystrophy – Deflazacort Prior Authorization Policy
- Emflaza™ (deflazacort tablets and oral suspension – PTC Therapeutics; generics)
 - Jaythari (deflazacort tablets – Zydus Pharmaceuticals)

REVIEW DATE: 01/22/2025; selected revision 09/03/2025

INSTRUCTIONS FOR USE

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CIGNA NATIONAL FORMULARY COVERAGE:

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 is indicated for DMD in patients ≥ 5 years of age.⁹ Due to PTC Therapeutics marketing exclusivity rights, Jaythari is 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 were 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. 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). Emflaza[™] (deflazacort tablets and oral suspension) and prednisone may be used to improve timed motor function, reduce the need for scoliosis surgery, and delay the onset of cardiomyopathy until the patient is 18 years of age (weak evidence). Emflaza 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). Emflaza 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 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.

- **Emflaza[™] (deflazacort tablets and oral suspension - PTC Therapeutics; generics)**
- **Jaythari (deflazacort tablets – Zydus Pharmaceuticals)**

is(are) covered as medically necessary when the following criteria is(are) met for FDA-approved indication(s) or other uses with supportive evidence (if applicable):

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, and iv):

- 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 $\geq 10\%$ of body weight 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; OR

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.

CONDITIONS NOT COVERED

- **Emflaza™ (deflazacort tablets and oral suspension - PTC Therapeutics; generics)**
- **Jaythari (deflazacort tablets – Zydus Pharmaceuticals)**

is(are) considered not medically necessary for ANY other use(s).

REFERENCES

2 Pages - Cigna National Formulary Coverage - Policy: Muscular Dystrophy – Deflazacort Prior Authorization Policy

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. Reaffirmed January 22, 2022. Available at: <https://www.aan.com/Guidelines/Home/GuidelineDetail/731>. Accessed on January 17, 2025.
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.

HISTORY

Type of Revision	Summary of Changes	Review Date
Early Annual Revision	Duchenne Muscular Dystrophy: In the criteria referring to genetic testing, deleted "...or likely pathogenic" reference to dystrophin gene. Under "Patient is Currently Receiving Emflaza", added age criterion. Under "Note" for improvements with Emflaza therapy, changed "time to run or walk 10 meters" from 30 feet. Also added 6-minute walk test to the list of motor function tests.	01/10/2024
Selected Revision	Emflaza tablets are available as generic deflazacort tablets. Changed policy name to Deflazacort PA. Also, within the policy changed Emflaza to deflazacort wherever applicable.	03/06/2024
Selected Revision	From policy heading deleted "generic for tablets only" since the oral suspension is now available as a generic. Duchenne Muscular Dystrophy: For diagnosis confirmation of Duchenne muscular dystrophy, deleted criteria asking for "Muscle biopsy showing the absence of, or marked decrease in, dystrophin protein."	07/03/2024
Annual Revision	No criteria changes	01/22/2025
Selected Revision	Added branded generic Jaythari to the policy with the same criteria applied as the other deflazacort products.	09/03/2025

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