MEDICAL MUTUAL®

Drug Policy

Policy:	Deflazacort (Emflaza; Pyquvi; Jaythari)	Annual Review Date: 11/20/2025 Last Revised Date: 11/20/2025

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 and Pyquvi are indicated for DMD in patients ≥ 5 years of age.[9,10] Due to PTC Therapeutics marketing exclusivity rights, Jaythari and Pyquvi are not approved in patients < 5 years of age.

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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.

POLICY STATEMENT

This policy involves the use of Emflaza. Prior authorization is recommended for pharmacy benefit coverage of Emflaza. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Emflaza as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Emflaza be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

RECOMMENDED AUTHORIZATION CRITERIA

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

1. <u>Duchenne Muscular Dystrophy (DMD), initial therapy</u>
Criteria. *Patient must meet the following criteria*

A. The patient is 2 years of age or older; AND

B. Patient's diagnosis of Duchenne Muscular Dystrophy is confirmed by one of the following (a or b) **[documentation required]**:
- **a.** Genetic testing with a confirmed pathogenic variant in the dystrophin gene; OR
- **b.** Muscle biopsy showing the absence of, or marked decrease in, dystrophin protein; AND

C. The patient meets ONE of the following conditions:
- **a.** The patient has tried prednisone or prednisolone for 6 months or longer **[documentation required]** AND according to the prescribing physician, the patient has had at least one of the following significant intolerable adverse effects:
  - **i.** Cushingoid appearance **[documentation required]**; OR
  - **ii.** Central (truncal) obesity **[documentation required]**; OR
  - **iii.** Undesirable weight gain defined as ≥ 10% of body weight increase over a 6-month period **[documentation required]**; OR
  - **iv.** Diabetes and/or hypertension that is difficult to manage according to the prescribing physician **[documentation required]**; or
- **b.** According to the prescribing physician, the patient has experienced a severe behavioral adverse effect while on prednisone or prednisolone therapy that has or would require a prednisone or prednisolone dose reduction **[documentation required]**; AND

D. The medication is prescribed by or in consultation with a physician who specializes in the treatment of Duchenne Muscular Dystrophy (DMD) and/or neuromuscular disorders; AND

E. If brand Emflaza tablets or brand Emflaza suspension are prescribed, the patient must meet either (a or b)
- a. If brand Emflaza tablets are prescribed, the patient must meet the following criteria (i and ii)
  - **i.** Patient has tried generic deflazacort or Jaythari tablets **[documentation required];** AND
  - **ii.** Patient cannot take deflazacort or Jaythari 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 allergy or serious adverse reaction **[documentation required]**. OR
- b. If brand Emflaza suspension is prescribed, the patient must meet the following criteria (i, ii, and iii)
  - i. Patient is unable to take deflazacort or Jaythari tablets **[documentation required]**; AND
  - ii. Patient has tried generic deflazacort suspension or Pyquvi suspension **[documentation required];** AND
  - **iii.** Patient cannot take deflazacort suspension or Pyquvi 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]**.

2. <u>Duchenne Muscular Dystrophy (DMD), patient is currently receiving Emflaza</u>
Criteria. *Patient must meet the following criteria*

A. Patient is ≥ 2 years of age; AND
B. Patient has tried prednisone or prednisolone [documentation required]; AND
C. According to the prescriber, the patient has responded to or continues to have improvement or benefit from Emflaza therapy [documentation required]; AND

Note: Examples of improvement or benefit from Emflaza therapy would include improvements in motor function (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, improve pulmonary function, etc.

D. The medication is prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy and/or neuromuscular disorders: AND

E. If brand Emflaza tablets or brand Emflaza suspension are prescribed, the patient must meet either (a or b)

- **a.** If brand Emflaza tablets are prescribed, the patient must meet the following criteria (i and ii)
  - **i.** Patient has tried generic deflazacort or Jaythari tablets **[documentation required];** AND
  - **ii.** Patient cannot take deflazacort or Jaythari 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 allergy or serious adverse reaction **[documentation required]**. OR
- **b.** If brand Emflaza suspension is prescribed, the patient must meet the following criteria (i ii, and iii)
  - **i.** Patient is unable to take deflazacort or Jaythari tablets **[documentation required]**; AND
  - **ii.** Patient has tried generic deflazacort suspension or Pyquvi suspension **[documentation required];** AND
  - **iii.** Patient cannot take deflazacort suspension or Pyquvi 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]**.

Initial Approval/ Extended Approval.
A) *Initial Approval:* 1 year
B) *Extended Approval:* 1 year

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Emflaza has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

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

2. Concomitant use of strong (e.g., efavirenz) or moderate (e.g., carbamazepine, phenytoin) CYP3A4 inducers are contraindicated with EMFLAZA.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational, or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

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. 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.
10. Pyquvi™ oral suspension [prescribing information]. Piscataway, NJ: Aucta Pharmaceuticals; February 2025.