

5.75.016

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| <b>Section:</b>    | Prescription Drugs   | <b>Effective Date:</b>       | July 10, 2026  |
| <b>Subsection:</b> | Neuromuscular Agents | <b>Original Policy Date:</b> | March 10, 2017 |
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**Last Review Date:** March 6, 2026

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## Deflazacort

### Description

Emflaza (deflazacort)

Jaythari (deflazacort)

Kymbee (deflazacort)

Pyquvi (deflazacort)

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### Background

Deflazacort is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD). Specifically, deflazacort is a corticosteroid prodrug, whose active metabolite acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The precise mechanism by which deflazacort exerts its therapeutic effects in patients with DMD is unknown (1-4).

### Regulatory Status

FDA-approved indication: Deflazacort is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older (1-4).

Deflazacort can suppress the immune system and increase the risk of infection with any pathogen, including viral, bacterial, fungal, protozoan, or helminthic. Corticosteroids reduce resistance to new infections, exacerbate existing infections, increase the risk of disseminated infections, increase the risk of reactivation or exacerbation of latent infections, and mask some signs of infection (1-4).

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All immunizations should be administered according to immunization guidelines prior to starting Deflazacort. Live or live-attenuated vaccines should be administered at least 4 to 6 weeks prior to starting Deflazacort. Patients on Deflazacort may receive concurrent vaccinations, except for live or live-attenuated vaccines (1-4).

Monitoring motor changes in patients with DMD requires functional evaluation along with measurement of muscle strength. The need for a reliable outcome measure in diseases of rapid deterioration such as DMD has led to the use of motor functional tests. In a large, multicenter, international clinical trial, the six minute walk test (6MWT) proved to be feasible and highly reliable. Also used are the Motor Function Measure (MFM) and North Star Ambulatory Assessment (NSAA) to help predict loss of ambulation 1 year before its occurrence in order to allow time to adapt rehabilitation, change the patient's environment, and consider acquisition of assistive aids or the use of medications (5-7).

Safety and effectiveness in patients 2 years of age and older have been established (1-4).

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#### Related policies

Agamree, Amondys 45, Duvyzat, Elevidys, Exondys 51, Viltepso, Vyondys 53

#### Policy

*This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.*

Deflazacort may be considered **medically necessary** if the conditions indicated below are met.

Deflazacort may be considered **investigational** for all other indications.

### Prior-Approval Requirements

**Age** 2 years of age or older

#### Diagnosis

Patient must have the following:

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Duchenne muscular dystrophy (DMD)

**AND ALL** of the following:

- a. Genetic confirmation of DMD
- b. Serum creatinine kinase activity at least 10 times the upper limit of normal (ULN) prior to initiating treatment
- c. Inadequate treatment response, intolerance, or contraindication to a 3 month trial of prednisone
- d. Obtain a baseline motor milestone score from **ONE** the following assessments:
  - i. 6-minute walk test (6MWT)
  - ii. North Star Ambulatory Assessment (NSAA)
  - iii. Motor Function Measure (MFM)
- e. **NOT** given concurrently with live vaccinations
- f. Absence of active infection [including tuberculosis and hepatitis B virus (HBV)]
- g. If the patient has a history of hepatitis B (HBV) infection
  - i. Prescriber agrees to monitor for HBV reactivation

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## Prior – Approval *Renewal* Requirements

**Age** 2 years of age or older

### Diagnosis

Patient must have the following:

Duchenne muscular dystrophy (DMD)

**AND ALL** of the following:

- a. Stabilization OR improvement in motor milestone score from baseline from **ONE** the following assessments:
  - i. 6-minute walk test (6MWT)
  - ii. North Star ambulatory assessment (NSAA)
  - iii. Motor Function Measure (MFM)
- b. **NOT** given concurrently with live vaccinations

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- c. Absence of active infection [including tuberculosis and hepatitis B virus (HBV)]
- d. If the patient has a history of hepatitis B (HBV) infection
  - i. Prescriber agrees to monitor for HBV reactivation

### Policy Guidelines

#### Pre - PA Allowance

None

#### Prior - Approval Limits

**Duration** 6 months

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#### Prior – Approval *Renewal* Limits

**Duration** 12 months

### Rationale

#### Summary

Deflazacort is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD). Deflazacort acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The most common adverse reactions are Cushingoid appearance, increase weight, increase appetite, upper respiratory tract infection, cough, pollakiuria, hirsutism, central obesity, and nasopharyngitis. Safety and effectiveness in patients 2 years of age and older have been established (1-4).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Deflazacort while maintaining optimal therapeutic outcomes.

#### References

1. Emflaza [package insert]. South Plainfield, NJ: PTC Therapeutics; May 2024.
2. Jaythari [package insert]. Pennington, NJ: Zydus Pharmaceuticals Inc.; May 2025.
3. Kymbee [package insert]. Maple Grove, MN: Upsher-Smith Laboratories, LLC; July 2025.

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4. Pyquvi [package insert]. Piscataway, NJ: Aucta Pharmaceuticals, Inc.; December 2025.
5. Mcdonald C, Henricson E, et al. The 6-Minute Walk test and Other Clinical Endpoints in Duchenne Muscular Dystrophy: Reliability, Concurrent Validity, and Minimal Clinically Important Differences from a Multicenter Study. *Muscle Nerve*. 2013 Sep; 48(3): 357–368.
6. Mcdonald C, Henricson E, et al. The 6-Minute Walk test and Other Endpoints in Duchenne Muscular Dystrophy: Longitudinal Natural History Observations Over 48 weeks from a Multicenter Study. *Muscle Nerve*. 2013 Sep; 48(3): 343–356.
7. Vuillerot C, Girardot F, et al. Monitoring changes and predicting loss of ambulation in Duchenne muscular dystrophy with the Motor Function Measure. *Developmental Medicine & Child Neurology* 2010, 52: 60–65.

### Policy History

| Date           | Action                                                                                                                                                                                                  |
|----------------|---------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| March 2017     | Addition to PA                                                                                                                                                                                          |
| April 2017     | Addition of NSAA, MFM and HFMS assessment tools to obtain baseline scores and improvement requirements                                                                                                  |
| June 2017      | Annual review                                                                                                                                                                                           |
| September 2018 | Annual review and reference update                                                                                                                                                                      |
| June 2019      | Reduced age requirement to 2 and older from 5 and older and revised regulatory status section                                                                                                           |
| September 2019 | Annual review                                                                                                                                                                                           |
| June 2020      | Annual review                                                                                                                                                                                           |
| December 2020  | Annual review                                                                                                                                                                                           |
| March 2021     | Annual review                                                                                                                                                                                           |
| April 2021     | Removed Hammersmith motor function scale score requirement from initiation and continuation. Revised renewal motor function score to stabilization or improvement instead of point requirement per FEP. |
| June 2021      | Annual editorial review and reference update                                                                                                                                                            |
| March 2022     | Annual review and reference update                                                                                                                                                                      |
| March 2023     | Annual review. Changed policy number to 5.75.016                                                                                                                                                        |
| September 2023 | Annual review                                                                                                                                                                                           |
| December 2023  | Annual review                                                                                                                                                                                           |
| March 2024     | Annual review                                                                                                                                                                                           |
| June 2024      | Annual review                                                                                                                                                                                           |
| September 2024 | Annual review and reference update. Per SME, changed initiation requirement wording to “genetic confirmation of DMD”                                                                                    |
| March 2025     | Annual review                                                                                                                                                                                           |

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| March 2026 | Annual review                                                                             |
| July 2026  | Changed policy name to Deflazacort, added products Jaythari, Kymbee, and Pyquvi to policy |

Keywords

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This policy was effective with interim approval on July 10, 2026 and will be reviewed by the FEP® Pharmacy and Medical Policy Committee on September 17, 2026.