

## **Clinical Policy: Deflazacort (Emflaza)**

Reference Number: IN.CP.PHAR.331

Effective Date: 01.01.2022

Last Review Date: 12.21

Line of Business: Medicaid

[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

### **Description**

Deflazacort (Emflaza™) is a corticosteroid.

### **FDA Approved Indication(s)**

Emflaza is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

### **Policy/Criteria**

*Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.*

#### **I. Initial Approval Criteria**

##### **A. Duchenne Muscular Dystrophy (must meet all):**

1. Diagnosis of DMD confirmed by one of the following (a or b):
  - a. Genetic testing
  - b. If genetic studies are negative (i.e., no mutation identified), positive muscle biopsy (e.g., absence of dystrophin protein);
2. Age  $\geq$  2 years;
3. Prescriber has provided documentation of current clinical status to compare upon re-evaluations of therapy (e.g. Brooke Score, 6 minute walk test, etc.)
4. Requested dose does not exceed 0.9mg/kg/day, rounded up to the nearest possible tablet dose or nearest tenth of a mL of oral suspension once weekly (weight required)

**Approval duration: 6 months**

##### **B. Other diagnoses/indications**

1. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): CP.PMN.53 for Medicaid.

#### **II. Continued Therapy**

##### **A. Duchenne Muscular Dystrophy (must meet all):**

1. History of the requested agent within the past 90 days
2. Documentation from prescriber indicating improvement (including stabilization) in current clinical status (e.g. Brooke Score, 6 minute walk test, etc.)
3. Requested dose does not exceed 0.9mg/kg/day, rounded up to the nearest possible tablet dose or nearest tenth of a mL of oral suspension once weekly (weight required).

**Approval duration:**

**Medicaid** – 6 months

**B. Other diagnoses/indications** (must meet 1 or 2):

1. Currently receiving medication via Centene benefit and documentation supports positive response to therapy.

**Approval duration: Duration of request or 6 months (whichever is less);** or

2. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) CP.PMN.53 for Medicaid.

**III. Diagnoses/Indications for which coverage is NOT authorized:**

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – CP.PMN.53 for Medicaid or evidence of coverage documents.

**IV. Appendices/General Information**

*Appendix A: Abbreviation/Acronym Key*

DMD: Duchenne muscular dystrophy

FDA: Food and Drug Administration

*Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic.*

*\*Off-label*

*Appendix C: Contraindications/Boxed Warnings*

- Contraindication(s): hypersensitivity to deflazacort or any of the inactive ingredients in Emflaza
- Boxed warning(s): none reported

*Appendix D: General Information*

- Examples of positive response to corticosteroid therapy (e.g., Emflaza, prednisone) include improvement in muscle strength tests (e.g., Medical Research Council [MRC] scale for muscle strength with 0 being no movement and 5 being normal strength), pulmonary function tests (e.g., forced vital capacity [FVC] and maximal expiratory pressure), walk tests (e.g., 6 minute walk test (6MWT) distance), and timed functional tests (e.g., standing from lying position; climbing 4 stairs; running/walking 30 feet; propelling a wheelchair 30 feet).
- In clinical trials, Emflaza has demonstrated similar efficacy to prednisone with regard to muscle strength, motor function, pulmonary function, and loss of ambulation. Emflaza may be associated with potentially less weight gain than prednisone; however, it may also be associated with more growth reduction and cataracts. In an evidence report published August 2019, the Institute for Clinical and Economic Review (ICER) concludes: “...we have moderate certainty that deflazacort has comparable or better net health benefits compared to prednisone.”

## V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
DMD	0.9 mg/kg/dose PO QD	0.9 mg/kg/dose

## VI. Product Availability

- Tablets: 6 mg, 18 mg, 30 mg, 36 mg
- Oral suspension: 22.75 mg/mL

## VII. References

1. Emflaza Prescribing Information. South Plainfield, NJ: PTC Therapeutics, Inc.; June 2019; Available at: <https://www.emflaza.com>. Accessed October 9, 2020.
2. Gloss D, Moxley RT, 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. doi:10.1212/WNL.0000000000002337. Reaffirmed on January 26, 2019.
3. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. Lancet Neurol. 2010; 9(1): 77-93.
4. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.; 2017. Available at: <http://www.clinicalpharmacology-ip.com/>.
5. 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: 251-267.
6. Institute for Clinical and Economic Review. Deflazacort, eteplirsen, and golodirsen for Duchenne muscular dystrophy: Effectiveness and value. Published August 15, 2019. Available at: <https://icer-review.org/material/dmd-final-evidence-report>. Accessed October 9, 2020.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created for IN Medicaid Moratorium	12.2021	01.2022