# Clinical Policy: Deflazacort (Emflaza)

Reference Number: ERX.SPA.50
Effective Date: 06.01.17
Last Review Date: 02.22
Line of Business: Commercial, 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.*

*Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.*

It is the policy of health plans affiliated with Envolve Pharmacy Solutions™ that Emflaza is **medically necessary** when the following criteria are met:

**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 (e.g., dystrophin deletion or duplication mutation found);
   b. If genetic studies are negative (i.e., no mutation identified), positive muscle biopsy (e.g., absence of dystrophin protein);
2. Prescribed by or in consultation with a neurologist;
3. Age ≥ 2 years;
4. Failure of a ≥ 6 month trial of prednisone, unless contraindicated or clinically significant adverse effects are experienced;
5. Dose does not exceed 0.9 mg/kg per day.

**Approval duration: 6 months**

**B. Other diagnoses/indications**

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

**II. Continued Therapy**

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

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;
2. Member is responding positively to therapy;
3. If request is for a dose increase, new dose does not exceed 0.9 mg/kg per day.

**Approval duration: 12 months**

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

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.
   **Approval duration: Duration of request or 6 months (whichever is less)**; or

2. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

**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 policy – ERX.PA.01 or evidence of coverage documents.

**IV. Appendices/General Information**

*Appendix A: Abbreviation/Acronym Key*
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration

*Appendix B: Therapeutic Alternatives*
*This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent and may require prior authorization.*

| Drug Name | Dosing Regimen | Dose Limit/ Maximum Dose |
|---|---|---|
| prednisone* | 0.75 mg/kg/day PO (preferred)<br><br><u>Alternative dosing regimens</u><br>• 0.3 mg/kg/day PO *(lesser efficacy and fewer adverse events)*<br>• 10 mg/kg/weekend PO | Varies based on weight |

*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 2021; Available at: https://www.emflaza.com. Accessed September 14, 2021.
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.; 2021. 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 September 14, 2021.

| Reviews, Revisions, and Approvals | Date | P&T Approval Date |
|---|---|---|
| 1Q18 annual review:<br>Modified continued approval duration from length of benefit to 12 months. | 11.13.17 | 02.18 |
| 1Q 2019 annual review: no significant changes; references reviewed and updated. | 10.25.18 | 02.19 |
| RT4: no significant changes; updated age down to 2 years old per updated prescribing information. | 06.18.19 | |
| 1Q 2020 annual review: no significant changes; references reviewed and updated. | 10.08.19 | 02.20 |
| 1Q 2021 annual review: no significant changes; references reviewed and updated. | 10.09.20 | 02.21 |
| 1Q 2022 annual review: no significant changes; references reviewed and updated. | 09.14.21 | 02.22 |

**Important Reminder**

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.