Clinical Policy: Deflazacort (Emflaza)

Reference Number: PA.CP.PHAR.331
Effective Date: 01/18
Last Review Date: 01/19

Coding Implications
Revision Log

Description
Deflazacort (Emflaza™) is a corticosteroid.

FDA Approved Indication(s)
Emflaza is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 5 years of age and older.

Policy/Criteria
It is the policy of health plans affiliated with Pennsylvania Health and Wellness® 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 Duchenne muscular dystrophy (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. Failure of ≥ 6 month trial of prednisone,unless contraindicated or clinically significant adverse effects are experienced;
4. Member is ≥ 5 years of age;
5. Dose does not exceed 0.9 mg/kg per day.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

II. Continued Approval

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

1. Currently receiving medication via Pennsylvania Health and Wellness benefit, or member has previously met all initial approval criteria or Continuity of Care policy applies;
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 Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or
   Approval duration: Duration of request or 6 months (whichever is less); or

2. Refer to PA.CP.PMN.53

Background
Description/Mechanism of Action:
Deflazacort is a corticosteroid prodrug, whose active metabolite, 21-desDFZ, 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.

III. 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 for all relevant lines of business and may require prior authorization.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
prednisone	0.75 mg/kg/day PO (preferred) <u>Alternative dosing regimens</u> • 0.3 mg/kg/day PO *(lesser efficacy and fewer adverse events)* • 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.

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 testings (e.g., standing from lying position; climbing 4 stairs; running/walking 30 feet; propelling a wheelchair 30 feet).

IV. Dosage and Administration

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

V. Product Availability

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

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
N/A	

Reviews, Revisions, and Approvals	Date	Approval Date
Removed time period in which prednisone trial must have occurred. References reviewed and updated.	02/18	
1Q 2019 annual review: references reviewed and updated.	01/19	

References

1. Emflaza Prescribing Information. South Plainfield, NJ: PTC Therapuetics, Inc.; June 2017; Available at: https://emflaza.com/. Accessed October 25, 2018.
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.
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.