Clinical Policy: Deflazacort (Emflaza)

Reference Number: PA.CP.PHAR.331
Effective Date: 01/18
Last Review Date: 07/18

Coding Implications
Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for deflazacort (Emflaza™).

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/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. Documentation that member continues to benefit from corticosteroid therapy;
3. Dose does not exceed 0.9 mg/kg/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
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.

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

Appendices
Appendix A: Abbreviation Key
DMD: Duchenne muscular dystrophy

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	

References
1. Emflaza Prescribing Information. Northbrook, IL: Marathon Pharmaceuticals, LLC; February 2017; Available at: https://emflaza.com/. Accessed February 21, 2017.
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.