Deflazacort, (Emflaza®)

EOCCO POLICY

Policy Type: PA/ SP Pharmacy Coverage Policy: EOCCO018

Description

Deflazacort (Emflaza) is an orally administered corticosteroid prodrug whose active metabolite exerts anti-inflammatory and immunosuppressive effects.

Length of Authorization

- Initial: One year
- Renewal: One year

Quantity limits

Product Name	Dosage Form	Indication	Quantity Limit	DDID
deflazacort (Emflaza)	6 mg tablets	Duchenne Muscular Dystrophy	60 tablets/30 days	196769
	18 mg tablets		30 tablets/30 days	196779
	30 mg tablets		60 tablets/30 days	196770
	36 mg tablets		none	196780
	22.75 mg/mL oral suspension		none	196781

Initial Evaluation

I. Deflazacort (Emflaza) may be considered medically necessary when the following criteria below are met:
   A. A diagnosis of **Duchenne Muscular Dystrophy (DMD)**; **AND**
      1. Member is two years of age or older; **AND**
      2. Treatment with oral prednisone has been ineffective, contraindicated, or not tolerated.

II. Deflazacort (Emflaza) is considered investigational when used for all other conditions, including, but not limited to:
   Dysferlinopathies: including Miyoshi Myopathy (MM) and limb girdle muscular dystrophy type 2B (LGMD2B)

Renewal Evaluation

I. Member has a diagnosis of Duchenne Muscular Dystrophy; **AND**
II. Documentation of symptom improvement and/or stability of disease

Supporting Evidence

I. Per the American Academy of Neurology 2016 Guideline on Corticosteroid Use in Duchenne Muscular Dystrophy:
   - Prednisone
     i. Should be offered for improving strength (Level B) and pulmonary function (Level B)
     ii. The preferred dosing regimen of prednisone is 0.75 mg/kg/d (Level B); though this regimen is associated with significant risk of weight gain, hirsutism, and cushingoid appearance (Level B).
     iii. Prednisone 10 mg/kg/weekend is found equally effective at 12 months (Level B).
     iv. Prednisone may be offered for improving timed motor function, reducing the need for scoliosis surgery, and delaying cardiomyopathy onset by 18 years of age (Level C for each).
   - Deflazacort
     i. May be offered for improving strength and timed motor function, and delaying age at loss of ambulation (Level C)
     ii. May be offered for improving pulmonary function, reducing the need for scoliosis surgery, delaying cardiomyopathy onset, and increasing survival (Level C for each.)
     iii. Compared to prednisone, deflazacort is projected to have very high costs relative to its benefits for patients and families. ICER is to release value-based benchmarks July 2019.
   - Prednisone and deflazacort are possibly equally effective for improving motor function in patients with DMD. There is insufficient evidence to directly compare the effectiveness of prednisone vs deflazacort in cardiac function in patients with DMD.
   - Prednisone and deflazacort have both been shown to improve muscle strength compared with placebo.
   - There may be differences in weight gain-related adverse events between prednisone and deflazacort.
     i. Central obesity was seen as an adverse event in 25.0% and 24.6% of deflazacort patients compared to 42.9% of prednisone patients and cushingoid appearance was seen in 60.3% and 69.2% of deflazacort patients compared to 77.8% of prednisone patients.

Investigational or Not Medically Necessary Uses

I. Dysferlinopathies: including Miyoshi Myopathy (MM) and limb girdle muscular dystrophy type 2B (LGMD2B)
   A. Deflazacort as an ineffective therapy in dysferlinopathies was shown in a double-blinded, placebo-controlled trial. Further evaluation is needed to support use of deflazacort (Emflaza) in this setting.

References

1. Emflaza [Prescribing Information]. Northbrook, IL: Marathon Pharmaceuticals. February 2017.
2. Gloss D, et al. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy. Neurology. 2016 Feb;86(5):465-72; DOI: 10.1212/WNL.0000000000002337
3. Griggs RC, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. Neurology. 2016 Nov 15; 87(20): 2123-2131.
4. Matthews E, et al. Corticosteroids for the treatment of Duchenne muscular dystrophy. Cochrane Database Syst Rev. 2016 May 5;(5):CD003725.
5. Walter M, et al. Treatment of dysferlinopathy with deflazacort: a double-blind, placebo-controlled clinical trial. Ophanet Journal of Rare Diseases. 2013 Feb 14; 8(26):1750-1752.
6. Institute for Clinical and Economic Review. Draft Evidence Report – Deflazacort, Eteplirsen, and Golodirsen for DMD. 2019 May; https://icer-review.org/topic/duchenne-muscular-dystrophy/

Policy Implementation/Update:

Date Created	March 2017
Date Effective	May 2017
Last Updated	July 2019
Last Reviewed	05/2017, 01/2017, 07/2019

Action and Summary of Changes	Date
Revised to policy format, include use in pediatric patients down to two years of age.	07/2019