

Drug Therapy Guidelines

Emflaza™ (deflazacort)

Applicable

Medical Benefit		Effective: 1/1/18
Pharmacy- Formulary 1	x	Next Review: 12/18
Pharmacy- Formulary 2	x	Date of Origin: 4/17
Pharmacy- Formulary 3/Exclusive	x	Review Dates: 3/17, 12/17
Pharmacy- Formulary 4/AON	x	

I. Medication Description

Emflaza (deflazacort) is a corticosteroid prodrug that is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients ≥ 5 years of age. Deflazacort has concentration-dependent, anti-inflammatory and immunomodulatory effects from inhibition of leucocyte function; deflazacort has been shown to inhibit IL-1-beta-stimulated IL-6 production from human osteoblast-like cells and chondrocytes. The precise mechanism by which deflazacort exerts its therapeutic effects in patients with Duchenne muscular dystrophy (DMD) is unknown. The aim of therapy is slowing the loss of muscle strength to maximize the quality of life.

II. Position Statement

Coverage is determined through a prior authorization process with supporting clinical documentation for all requests.

III. Policy

Coverage of Emflaza is provided when the following criteria are met:

- Medication is prescribed by a provider who specializes in the treatment of Duchenne Muscular Dystrophy (DMD) and/or neuromuscular disorders **AND**
- Patient must be diagnosed with DMD **AND**
- Patient must be at least 5 years of age **AND**
- At least one of the following are true:
 - The patient has experienced a severe behavioral adverse effect (AE) while on prednisone therapy that has or would require a prednisone dose reduction **OR**
 - The patient has tried prednisone for ≥ 6 months and has had at least one of the following significant intolerable adverse effects (AEs) that is unable to be managed:
 - Cushingoid appearance
 - Central (truncal) obesity
 - Weight gain of at least 10% of body weight over 6-month period
 - Diabetes and/or hypertension that is difficult to manage

IV. Quantity Limitations

- Suspension: allow up to 2 bottles per month (2 x 13ml bottles)
- 6mg, 18mg, 30mg, 36mg tablets: 30 per month overall

- Additional quantities can be reviewed for coverage as needed due to patient's weight. Factors such as ability to use suspension or lowest cost tablet combinations to obtain desired dose will be considered.

V. Coverage Duration

Coverage may be provided for up to 6 months and may be renewed.

VI. Coverage Renewal Criteria

Coverage can be renewed when a clear benefit to the use of Emflaza has been shown compared to other available corticosteroids (i.e. reduction in Cushingoid appearance, reduction in the rate of weight gain). Chart notes must be provided to show an objective benefit.

VII. Billing/Coding Information

Available as:

- 6mg, 18mg, 30mg, and 36mg oral tablets
- 22.75mg/ml oral suspension
 - Available as 13ml bottles
 - Must be dispensed as full, unbroken bottles

VIII. Summary of Policy Changes

- 4/10/17: new policy
- 1/1/18: no policy changes

IX. References

1. Emflaza™ tablets and oral suspension [prescribing information]. Northbrook, IL: Marathon Pharmaceuticals, LLC; February 9 2017.
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13. Houde S, Filiatrault M, Fournier A, et al. Emflaza use in Duchenne muscular dystrophy: an 8-year follow-up. *Pediatr Neurol.* 2008;38(3):200-206.
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17. Markham LW, Spicer RL, Khoury PR, et al. Steroid therapy and cardiac function in Duchenne muscular dystrophy. *Pediatr Cardiol.* 2005;26:768-771.
18. Balban B, Matthews DJ, Clayton GH, and Carry T. Corticosteroid treatment and functional improvement in Duchenne muscular dystrophy. *Am J Phys Med Rehabil.* 2005;84:843-850.
19. Matthews E, Brassington R, Kuntzer T, Jichi F, and Manzur AY. Corticosteroids for the treatment of Duchenne muscular dystrophy. *Cochrane Database Syst Rev.* 2016;(5):CD003725. doi: 10.1002/14651858.CD003725.
20. University of Rochester; Newcastle University, University Medical Center Freiburg, National Institute of Neurological Disorders and Stroke (NINDS). Finding the optimum regimen for Duchenne muscular dystrophy (FOR-DMD). In: *ClinicalTrials.gov* [Internet]. Bethesda (MD): National Library of Medicine (US). 2017- [cited 2017 Feb 16]. Available at: <https://clinicaltrials.gov/ct2/show/record/NCT01603407?term=FOR+DMD&rank=1>. NLM Identifier: NCT01603407.

The Plan fully expects that only appropriate and medically necessary services will be rendered. The Plan reserves the right to conduct pre-payment and post-payment reviews to assess the medical appropriateness of the above-referenced therapies.

The preceding policy is a guideline to allow for coverage of the pertinent medication/product, and is not meant to serve as a clinical practice guideline.