Commercial PA Criteria

Effective: August 16, 2017

Prior Authorization: Emflaza

Products Affected: Emflaza (deflazacort) oral tablets, Emflaza (deflazacort) oral suspension

Medication Description: Deflazacort is a heterocyclic glucocorticoid prodrug belonging to the class of oxazoline steroids, 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 Duchenne muscular dystrophy is unknown.

Covered Uses: Treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

Exclusion Criteria: N/A

Required Medical Information:

1. Diagnosis
2. Previous therapies tried and failed

Age Restrictions: 2 years of age and older.

Prescriber Restrictions: The medication is prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy and/or neuromuscular disorders.

Coverage Duration: 1 year

Other Criteria:

Initial Approval Criteria

1. **Duchenne muscular dystrophy**

   Initial Therapy- Approve if the patient meets the following criteria (A **AND** B)

   A. Patient's diagnosis of Duchenne Muscular Dystrophy is confirmed by one of the following (i **OR** ii)
      i. Genetic testing with a confirmed pathogenic variant in the dystrophin gene; OR
      ii. Muscle biopsy showing the absence of, or marked decrease in, dystrophin protein; **AND**

   B. Patient meets ONE of the following conditions (i **OR** ii):
      i. Patient has tried prednisone or prednisolone for ≥ 6 months **AND** according to the prescriber, the patient has had at least one of the following significant intolerable adverse effects [a, b, c, **OR** d]:
         a. Cushingoid appearance **OR**
         b. Central (truncal) obesity **OR**
         c. Undesirable weight gain defined as ≥ 10% of body weight gain increase over a 6-month period **OR**
         d. Diabetes and/or hypertension that is difficult to manage according to the prescriber **OR**

ii. According to the prescriber, the patient has experienced a severe behavioral adverse event while on prednisone or prednisolone therapy that has or would require a prednisone or prednisolone dose reduction

Continuation

Approve if the patient meets the following (i, ii, iii, **AND** iv):

1. Patient has tried prednisone or prednisolone **AND**
2. According to the prescriber, the patient has responded to or continues to have improvement or benefit from Emflaza therapy **AND**
   Note: Examples of improvement or benefit from Emflaza therapy would include improvements in motor function (time from supine to standing, time to climb four stairs, time to run or walk 10 meters, 6-minute walk test), improvement in muscle strength, improved pulmonary function, etc.

References:

1. Product Information: EMFLAZA™ oral tablets, suspension, deflazacort oral tablets, suspension. Marathon Pharmaceuticals LLC (per FDA), Northbrook, IL, 2017.
2. Griggs RC, Miller JP, Greenberg CR, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. Neurology. 2016 Nov 15;87(20):2123-2131. Doi: 10.1212/WNL.0000000000003217. Accessed March 13, 2017.

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy	New Policy	All	4/14/2017
2	Policy Update	Updated Policy to match FDA Label (change from 5 years to 2 years)	Age Restrictions, Covered Uses	6/18/2019
3	Annual Review	N/A	N/A	3/30/2020
4	Policy Update	Updated age criteria in covered uses	Covered uses	8/11/2021
5	Policy Update	Removal of prescribed by, or in consultation with a neurologist. Addition of "The medication is prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy and/or neuromuscular disorders" Update coverage duration from 6 months to 1 year Removed current initial and renewal criteria for DMD and replaced with select criteria for implementation to label coverage	Prescriber restrictions Coverage Duration Other Criteria	2/1/2024