BlueCross BlueShield Arizona

An Independent Licensee of the Blue Cross Blue Shield Association

PHARMACY COVERAGE GUIDELINE

EMFLAZA™ (deflazacort) oral

This Pharmacy Coverage Guideline (PCG):

- Provides information about the reasons, basis, and information sources we use for coverage decisions
- Is not an opinion that a drug (collectively "Service") is clinically appropriate or inappropriate for a patient
- Is not a substitute for a provider's judgment (Provider and patient are responsible for all decisions about appropriateness of care)
- Is subject to all provisions e.g. (benefit coverage, limits, and exclusions) in the member's benefit plan; and
- Is subject to change as new information becomes available.

Scope

- This PCG applies to Commercial and Marketplace plans
- This PCG does not apply to the Federal Employee Program, Medicare Advantage, Medicaid or members of out-of-state Blue Cross and/or Blue Shield Plans

Instructions & Guidance

- To determine whether a member is eligible for the Service, read the entire PCG.
- This PCG is used for FDA approved indications including, but not limited to, a diagnosis and/or treatment with dosing, frequency, and duration.
- Use of a drug outside the FDA approved guidelines, refer to the appropriate Off-Label Use policy.
- The "Criteria" section outlines the factors and information we use to decide if the Service is medically necessary as defined in the Member's benefit plan.
- The "Description" section describes the Service.
- The "Definition" section defines certain words, terms or items within the policy and may include tables and charts.
- The "Resources" section lists the information and materials we considered in developing this PCG
- **We do not accept patient use of samples as evidence of an initial course of treatment, justification for continuation of therapy, or evidence of adequate trial and failure.**
- Information about medications that require precertification is available at www.azblue.com/pharmacy. You must fully complete the request form and provide chart notes, lab workup and any other supporting documentation. The prescribing provider must sign the form. Fax the form to BCBSAZ Pharmacy Management at (602) 864-3126 or email it to Pharmacyprecert@azblue.com.

Criteria:

- **Criteria for initial therapy:** Emflaza (deflazacort) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met:

  1. Prescriber is a physician specializing in the patient's diagnosis or is in consultation with a Pediatric Neurologist or Neurologist.

  2. Individual is 2 years of age or older.

  3. Individual has a confirmed diagnosis of Duchenne muscular dystrophy (DMD) with documented mutation of the dystrophin gene or the presence of abnormal dystrophin.

  4. Documented failure, contraindication per FDA label, or intolerance to, or not a candidate for **BOTH** of the following:

a. Prednisone
b. Prednisolone

5. Individual does not have severe hepatic impairment (Child-Pugh Class C).

6. Will not be simultaneously used with live or live attenuated vaccines.

7. Individual is not using moderate or strong CYP3A4 Inducers such as efavirenz, modafinil, nafcillin, rifabutin, rifampin, carbamazepine, phenytoin, phenobarbital, and others.

Initial approval duration: 12 months

- **Criteria for continuation of coverage (renewal request):** Emflaza (deflazacort) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met **(samples are not considered for continuation of therapy**):

1. Individual continues to be seen by a physician specializing in the patient's diagnosis or is in consultation with a Pediatric Neurologist or Neurologist.

2. Individual's condition has responded while on therapy with response defined as **TWO** of the following:
   a. Achieved and maintains an improvement in muscle strength over baseline
   b. Achieved and maintains an improvement in muscle function over baseline as demonstrated by **THREE** of the following:
      i. Reduced falls
      ii. Able to stand
      iii. Able to balance
      iv. Improved time to walk or run 30 feet
      v. Improved time to climb 4 stairs
      vi. Improved time to stand from supine position
   c. Achieved and maintains ability to independently perform activities of daily living
   d. Achieved and maintains ambulation without need for wheelchair
   e. Achieved and maintains an improved 6-minute walking distance
   f. Improvement in forced vital capacity (FVC) or maximum voluntary ventilation (MVV)

3. Individual has been adherent with the medication.

4. Individual not using moderate or strong CYP3A4 Inducers such as efavirenz, modafinil, nafcillin, rifabutin, rifampin, carbamazepine, phenytoin, phenobarbital, and others.

Renewal duration: 12 months

- Criteria for a request for non-FDA use or indication, treatment with dosing, frequency, or duration outside the FDA-approved dosing, frequency, and duration, refer to one of the following Pharmacy Coverage Guideline:

1. **Off-Label Use of Non-Cancer Medications**

2. **Off-Label Use of Cancer Medications**

PHARMACY COVERAGE GUIDELINE

EMFLAZA™ (deflazacort) oral

Description:

Emflaza (deflazacort) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in individuals 2 years of age and older.

Deflazacort is a corticosteroid prodrug whose active metabolite (21-desDFZ) binds to glucocorticoid receptors to exert immunosuppressive and anti-inflammatory effects. Deflazacort is a chemical modification of prednisolone. The precise mechanism by which deflazacort exerts its therapeutic effects in DMD is unknown.

DMD is a rare, genetic, X-linked, recessive neuromuscular disorder that typically afflicts young boys; however, female-manifesting carriers are reported. The disorder is caused by mutations of the dystrophin gene that leads to a disruption in messenger ribonucleic acid resulting in an absence or near absence of dystrophin within muscle cells. Dystrophin is thought to maintain the structural integrity of muscle cell, cushioning it from the stress and strain of repeated contraction and relaxation. Absence of dystrophin leads to muscle damage, with fibrotic and adipose tissue deposition.

In DMD there is significant deterioration of muscle strength and function with individuals experiencing frequent falls; difficulty in walking, standing, and balance; and difficulty in getting up from a lying or sitting position. A child is typically diagnosed with DMD between the ages of 2-5 years of age. There is progressive loss in the ability to perform activities independently, eventually leading to loss of ambulation (LoA) occurring by the teenage years in untreated patients. Other major complications of DMD that occur as the disease progresses include scoliosis, respiratory failure, and cardiomyopathy.

For individuals that are still ambulatory, the goal of treatment is to preserve ambulation and minimize future respiratory, cardiac, and orthopedic complications. For individuals that are not ambulatory, the goal of treatment is to maintain respiratory status, cardiac function, and to improve complications from scoliosis. Glucocorticoids are the only medications available that slow the decline in muscle strength and function in DMD; they also reduce the risk of scoliosis and stabilize pulmonary function.

Prednisone, prednisolone, and deflazacort are believed to work similarly. The choice of which glucocorticoid to use depends on availability, formulation, strengths available, cost, and perceived adverse effect profile. Limited evidence suggests that deflazacort might be preferred to prednisone or prednisolone for some individuals because of a lower risk of weight gain in the first years of treatment, the weight gain was no longer significantly different with longer period of prednisone use. Deflazacort possibly increases the risk of cataracts over prednisone, although they are not vision-impairing.

Prednisone and prednisolone, depending on agent chosen, are available in several different formulations such as tablets, delayed-release tablets, disintegrating tablets, and oral liquid forms. Prednisone strengths include 1 mg, 2 mg, 2.5 mg, 5 mg, 10 mg, 20 mg, and 50 mg. Prednisolone strengths include 5 mg, 10 mg, 15 mg, 20 mg 25 mg and 30 mg. Deflazacort is available as oral tablet and oral suspension; strengths include 6 mg, 18 mg, 22.75 mg, 30 mg, and 35 mg.

Definitions:

	Approximate Equivalent dose	Anti-inflammatory potency
Deflazacort	7.5 mg	N/A
Prednisone	5 mg	4 mg
Prednisolone	5 mg	4 mg
N/A*: not available*		

Resources:

Emflaza (deflazacort) product information, revised by PTC Therapeutics, Inc. 06-2021. Available at DailyMed http://dailymed.nlm.nih.gov. Accessed July 18, 2022.

Darras BT. Duchenne and Becker muscular dystrophy: Glucocorticoid and disease-modifying treatment. In: UpToDate, Patterson MC, Dashe JF (Eds), UpToDate, Waltham MA.: UpToDate Inc. Available at http://uptodate.com. Topic last updated on January 26, 2022. Accessed July 20, 2022.

Darras BT. Duchenne and Becker muscular dystrophy: Management and prognosis. In: UpToDate, Patterson MC, Dashe JF (Eds), UpToDate, Waltham MA.: UpToDate Inc. Available at http://uptodate.com. Topic last updated on July 01, 2022. Accessed July 20, 2022.