



An Independent Licensee of the Blue Cross Blue Shield Association

PHARMACY COVERAGE GUIDELINES  
SECTION: DRUGS

ORIGINAL EFFECTIVE DATE: 7/20/2017  
LAST REVIEW DATE: 8/19/2021  
LAST CRITERIA REVISION DATE: 8/19/2021  
ARCHIVE DATE:

---

## EMFLAZA™ (deflazacort) oral

---

Coverage for services, procedures, medical devices and drugs are dependent upon benefit eligibility as outlined in the member's specific benefit plan. This Pharmacy Coverage Guideline must be read in its entirety to determine coverage eligibility, if any.

This Pharmacy Coverage Guideline provides information related to coverage determinations only and does not imply that a service or treatment is clinically appropriate or inappropriate. The provider and the member are responsible for all decisions regarding the appropriateness of care. Providers should provide BCBSAZ complete medical rationale when requesting any exceptions to these guidelines.

The section identified as "Description" defines or describes a service, procedure, medical device or drug and is in no way intended as a statement of medical necessity and/or coverage.

The section identified as "Criteria" defines criteria to determine whether a service, procedure, medical device or drug is considered medically necessary or experimental or investigational.

State or federal mandates, e.g., FEP program, may dictate that any drug, device or biological product approved by the U.S. Food and Drug Administration (FDA) may not be considered experimental or investigational and thus the drug, device or biological product may be assessed only on the basis of medical necessity.

Pharmacy Coverage Guidelines are subject to change as new information becomes available.

For purposes of this Pharmacy Coverage Guideline, the terms "experimental" and "investigational" are considered to be interchangeable.

BLUE CROSS®, BLUE SHIELD® and the Cross and Shield Symbols are registered service marks of the Blue Cross and Blue Shield Association, an association of independent Blue Cross and Blue Shield Plans. All other trademarks and service marks contained in this guideline are the property of their respective owners, which are not affiliated with BCBSAZ.

---

This Pharmacy Coverage Guideline does not apply to FEP or other states' Blues Plans.

Information about medications that require precertification is available at [www.azblue.com/pharmacy](http://www.azblue.com/pharmacy).

Some large (100+) benefit plan groups may customize certain benefits, including adding or deleting precertification requirements.

All applicable benefit plan provisions apply, e.g., waiting periods, limitations, exclusions, waivers and benefit maximums.

Precertification for medication(s) or product(s) indicated in this guideline requires completion of the [request form](#) in its entirety with the chart notes as documentation. **All requested data must be provided.** Once completed the form must be signed by the prescribing provider and faxed back to BCBSAZ Pharmacy Management at (602) 864-3126 or emailed to [Pharmacyprecert@azblue.com](mailto:Pharmacyprecert@azblue.com). **Incomplete forms or forms without the chart notes will be returned.**



An Independent Licensee of the Blue Cross Blue Shield Association

PHARMACY COVERAGE GUIDELINES  
SECTION: DRUGS

ORIGINAL EFFECTIVE DATE: 7/20/2017  
LAST REVIEW DATE: 8/19/2021  
LAST CRITERIA REVISION DATE: 8/19/2021  
ARCHIVE DATE:

---

## EMFLAZA™ (deflazacort) oral

---

### Criteria:

➤ **Criteria for initial therapy:** Emflaza (deflazacort) is considered *medically necessary* and will be approved when **ALL** of 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. A confirmed diagnosis of Duchenne muscular dystrophy (DMD) with documented mutation of the dystrophin gene or the presence of abnormal dystrophin
4. Individual has failed, or is intolerant to, or has a contraindication to use of **BOTH** of the following:
  - a. Prednisone
  - b. Prednisolone
5. Will not be used in an individual with severe hepatic impairment (Child-Pugh Class C)
6. Will not be simultaneously used with live or live attenuated vaccines
7. There are no significant interacting drugs

**Initial approval duration:** 12 months

➤ **Criteria for continuation of coverage (renewal request):** Emflaza (deflazacort) is considered *medically necessary* and will be approved when **ALL** of the following criteria are met:

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 responded while on therapy
  - a. Response is defined as **TWO** of the following:
    - i. Achieved and maintains an improvement in muscle strength over baseline
    - ii. Achieved and maintains an improvement in muscle function over baseline as demonstrated by **THREE** of the following:
      1. Reduced falls
      2. Able to stand
      3. Able to balance
      4. Improved time to walk or run 30 feet
      5. Improved time to climb 4 stairs
      6. Improved time to stand from supine position
    - iii. Achieved and maintains ability to independently perform activities of daily living
    - iv. Achieved and maintains ambulation without need for wheelchair
    - v. Achieved and maintains an improved 6-minute walking distance



An Independent Licensee of the Blue Cross Blue Shield Association

**PHARMACY COVERAGE GUIDELINES**  
**SECTION: DRUGS**

**ORIGINAL EFFECTIVE DATE:** 7/20/2017  
**LAST REVIEW DATE:** 8/19/2021  
**LAST CRITERIA REVISION DATE:** 8/19/2021  
**ARCHIVE DATE:**

---

## **EMFLAZA™ (deflazacort) oral**

---

- vi. Improvement in forced vital capacity (FVC) or maximum voluntary ventilation (MVV)
- 3. Individual has been adherent with the medication
- 4. There are no significant interacting drugs

**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 a Non-cancer Medications**
  - 2. **Off-Label Use of a Cancer Medication for the Treatment of Cancer without a Specific Coverage Guideline**

---

### **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.



An Independent Licensee of the Blue Cross Blue Shield Association

PHARMACY COVERAGE GUIDELINES  
SECTION: DRUGS

ORIGINAL EFFECTIVE DATE: 7/20/2017  
LAST REVIEW DATE: 8/19/2021  
LAST CRITERIA REVISION DATE: 8/19/2021  
ARCHIVE DATE:

## EMFLAZA™ (deflazacort) oral

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. 02-2021. Available at DailyMed <http://dailymed.nlm.nih.gov>. Accessed on July 09, 2021.

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>. Accessed on July 09, 2021.

Parente L. Deflazacort: Therapeutic index, relative potency and equivalent doses versus other corticosteroids. BMC Pharmacol Toxicol 2017; 18:1-8

Gloss D, Moxley RT, Aswal S, and 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 (Feb 2):465-472.