
Title: Deflazacort (Emflaza®)

Policy #: Rx.01.200

Application of pharmacy policy is determined by benefits and contracts. Benefits may vary based on product line, group, or contract. Some medications may be subject to precertification, age, quantity, or formulary restrictions (ie limits on non-preferred drugs). Individual member benefits must be verified.

This pharmacy policy document describes the status of pharmaceutical information and/or technology at the time the document was developed. Since that time, new information relating to drug efficacy, interactions, contraindications, dosage, administration routes, safety, or FDA approval may have changed. This Pharmacy Policy will be regularly updated as scientific and medical literature becomes available. This information may include new FDA-approved indications, withdrawals, or other FDA alerts. This type of information is relevant not only when considering whether this policy should be updated, but also when applying it to current requests for coverage.

Members are advised to use participating pharmacies in order to receive the highest level of benefits.

Intent:

The intent of this policy is to communicate the medical necessity criteria for **deflazacort (Emflaza®)** as provided under the member's prescription drug benefit.

Description:

Muscular dystrophies are an inherited, X-linked group of progressive myopathic disorders, resulting from defects in the dystrophin genes, the genes responsible for normal muscle function. Duchenne muscular dystrophy (DMD) is the dystrophinopathy associated with the most severe clinical symptoms. Becker muscular dystrophy (BMD) has a milder clinical course than DMD. An intermediate group may be classified as mild DMD or severe BMD.

Duchenne muscular dystrophy is a type of muscular dystrophy of an inherited group of progressive myopathic disorders resulting from defects in a number of genes required for normal muscle function. Muscle weakness is the primary symptom. Duchenne muscular dystrophy is characterized by weakness, elevated creatine kinase and transaminases, growth delay, cardiomyopathy, orthopedic complications as well as cognitive and behavioral disorders.

Deflazacort (Emflaza®) is a corticosteroid prodrug, 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 DMD is unknown.

Deflazacort (Emflaza®) is indicated for the treatment of DMD in patients 2 years of age and older.

Policy:

INITIAL CRITERIA: Deflazacort (Emflaza®) is approved when ALL of the following are met:

- A. Diagnosis of Duchenne muscular dystrophy (DMD) confirmed by ONE of the following:
 - 1. Mutation of the dystrophin gene; OR
 - 2. Absence of the dystrophin protein confirmed by muscle biopsy; AND
- B. Member is 2 years of age or older; AND
- C. Prescribed by or in consultation with a neurologist who has experience treating children; AND
- D. Member has had an inadequate response or inability to tolerate prednisone or prednisolone given at a dose of 0.75 mg/kg/day or 10 mg/kg/weekend; AND
- E. Dose will not exceed 0.9 milligrams per kilogram of body weight once daily

Initial authorization: 12 months

REAUTHORIZATION CRITERIA: Deflazacort (Emflaza®) is re-approved when BOTH of the following are met:

- A. Member has experienced a benefit from therapy (e.g., improvement or preservation of muscle strength); AND
- B. Dose will not exceed 0.9 milligrams per kilogram of body weight once daily

Reauthorization: 12 months

Black Box Warning as shown in the drug Prescribing Information:

N/A

Guidelines:

Refer to the specific manufacturer's prescribing information for administration and dosage details and any applicable Black Box warnings.

BENEFIT APPLICATION

Subject to the terms and conditions of the applicable benefit contract, the applicable drug(s) identified in this policy is (are) covered under the prescription drug benefits of the Company's products when the medical necessity criteria listed in this pharmacy policy are met. Any services that are experimental/investigational or cosmetic are benefit contract exclusions for all products of the Company.

References:

Darras BT. Treatment of Duchenne and Becker muscular dystrophy. UpToDate June 2018. Available at: <https://www.uptodate.com/contents/duchenne-and-becker-muscular-dystrophy-management-and-prognosis>. Accessed March 30, 2021

Emflaza® [package insert]. Northbrook, IL. Marathon Pharmaceuticals, LLC. June 2019. Available from: https://www.accessdata.fda.gov/drugsatfda_docs/label/2017/208684s000,208685s000lbl.pdf. Accessed March 30, 2021.

Mesa LE, Dubrovsky AL, Corderi J, et al. Steroids in Duchenne muscular dystrophy--deflazacort trial. Neuromuscul Disord 1991; 1:261-6.

Darras BT. Duchenne and becker muscular dystrophy: clinical features and diagnosis. UpToDate website. Last updated September 7, 2018. Available at: <http://www.uptodate.com/>. Accessed March 30, 2021.

Applicable Drugs:

Inclusion of a drug in this table does not imply coverage. Eligibility, benefits, limitations, exclusions, precertification/referral requirements, provider contracts, and Company policies apply.

Brand Name	Generic Name
Emflaza®	Deflazacort

Cross References:

Off-Label Use Rx.01.33

Policy Version Number:	6.00
P&T Approval Date:	March 18, 2021
Policy Effective Date:	July 01, 2021
Next Required Review Date:	March 18, 2022

The Policy Bulletins on this web site were developed to assist the Company in administering the provisions of the respective benefit programs, and do not constitute a contract. If you have coverage through the Company, please refer to your specific benefit program for the terms, conditions, limitations and exclusions of your coverage. Company does not provide health care services, medical advice or treatment, or guarantee the outcome or results of any medical services/treatments. The facility and professional providers are responsible for providing medical advice and treatment.

Facility and professional providers are independent contractors and are not employees or agents of the Company. If you have a specific medical condition, please consult with your doctor. The Company reserves the right at any time to change or update its Policy Bulletins.

