Pharmacy Medical Necessity Guidelines: Emflaza™ (deflazacort)

Effective: October 1, 2020

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	RX	Department to Review	RXUM

These pharmacy medical necessity guidelines apply to the following:

Commercial Products

☒ Tufts Health Plan Commercial products – large group plans
☒ Tufts Health Plan Commercial products – small group and individual plans
☒ Tufts Health Freedom Plan products – large group plans
☒ Tufts Health Freedom Plan products – small group plans
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Tufts Health Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product)
☒ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan

Fax Numbers:

RXUM: 617.673.0988

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMINISTRATION (FDA)-APPROVED INDICATIONS

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

DMD, a form of muscular dystrophy, is a genetic disorder characterized by progressive muscle degeneration and weakness that predominantly affects males. DMD is caused by a deficiency of dystrophin, a protein that helps strengthen muscle fibers and protect them from injury. DMD is the most severe form of muscular dystrophy and without intervention the mean age at death is around 19 years. Currently there is no cure for DMD and therapies are supportive in nature.

Corticosteroids, including Emflaza (deflazacort) slow the decline in muscle strength and function in DMD. Additional benefits of glucocorticoids are reduction in the risk of scoliosis and stabilization of pulmonary function. Emflaza (deflazacort) is the first corticosteroid to gain FDA-approval for the treatment of DMD. Treatment guidelines recommend prednisone or Emflaza (deflazacort) as treatment options to improve strength, pulmonary function, time motor function, reducing the need for scoliosis surgery, and delaying onset of cardiomyopathy. Clinical trials used for the approval of Emflaza (deflazacort) demonstrate less weight gain compared to prednisone; however, the evidence is limited to 12-month data.

COVERAGE GUIDELINES

The plan may authorize coverage of Emflaza (deflazacort) for Members, when the following criteria are met:

Initial Therapy

1. Documented diagnosis of Duchenne muscular dystrophy

AND

2. The Member is at least 2 years of age

AND

3. Documentation of one of the following:
    a. An intolerable adverse event (e.g., Cushingoid appearance, significant weight gain, glucose intolerance, severe behavioral adverse event) has developed as a result of at least a 6 month trial with prednisone
    b. Treatment with prednisone is not clinically appropriate based on existing Member comorbidities

AND

4. The prescribing physician is a neurologist or a provider who specializes in the treatment of Duchenne muscular dystrophy

Reauthorization Criteria

1. Documented diagnosis of Duchenne muscular dystrophy

AND

2. The Member is at least 2 years of age

AND

3. Documentation of improved tolerance to Emflaza (deflazacort) as compared to treatment with prednisone

AND

4. Documentation that based on the prescriber's assessment, the Member continues to benefit from Emflaza (deflazacort), documented by a standardized assessment of motor function or respiratory function

LIMITATIONS

- Initial coverage of Emflaza (deflazacort) will be authorized for 6 months. Reauthorization of Emflaza (deflazacort) will be provided in 12-month intervals.
- Members new to the plan stable on Emflaza (deflazacort) should be reviewed against Reauthorization Criteria.

CODES

None

REFERENCES

1. Angelini C, Pegoraro E, Turella E, et al. Deflazacort in Duchenne dystrophy: study of long-term effect. Muscle Nerve. 1994;17(4):386-91.
2. Bushby K, Finkel R, Birnkrant D, et al. Diagnosis and management of Duchenne muscular dysropthy, part 1: diagnosis, and pharmacological and psychosocial management. Lancet Neurol. 2010a;9(1):77-93.
3. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dysropthy, part 2: implementation of multidisciplinary care. Lancet Neurol. 2010b;9(1):177-89.
4. Emflaza (deflazacort) [prescribing information]. Northbrook, IL: Marathon Pharmaceutical, LLC; 2019 June.
5. Gloss D, Moxley RT, Ashwal S, et al. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy. Neurology. 2016;86(5):465-72.
6. Griggs RC, Miller JP, Greenbert CR, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. Neurology. 2016;86(5):465-72.
7. Matthews E, Brassington R, Kuntzer T, et al. Corticosteroids for the treatment of Duchenne muscular dystrophy. Cochrane Database Syst Rev. 2016 May 5;(5):CD003725. doi: 10.1002/14651858.CD003725,pub4.

APPROVAL HISTORY

October 17, 2017: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

1. June 12, 2018: Effective August 7, 2018 updated criteria to allow documentation of baseline clinical inappropriateness of treatment with prednisone.
2. January 8, 2019: No changes.
3. August 13, 2019: Updated coverage criteria based on an updated indication for use in patients at least 2 years of age.
4. June 9, 2020: Effective October 1, 2020, modified Reauthorization Criteria and updated the Limitations so that members new to the plan stable on Emflaza (deflazacort) should be reviewed against Reauthorization Criteria.

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards

adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.