STATE OF WEST VIRGINIA
DEPARTMENT OF HEALTH AND HUMAN RESOURCES
BUREAU FOR MEDICAL SERVICES

Office of Pharmacy Services
Prior Authorization Criteria
EMFLAZA™ (deflazacort)
Effective 6/01/2021
Prior Authorization Request Form

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

Prior authorization requests may be approved if the following criteria have been satisfied:

1. Diagnosis of Duchenne muscular dystrophy (DMD); **AND**

2. Patient ≥ 2 years old; **AND**

3. Prescribed by, or in consultation with, a neurologist or a specialist in Duchenne Muscular Dystrophy (DMD) or neuromuscular disorders; **AND**

4. Patient must have a documented history of at least 6-months continuous therapy with prednisone; **AND**

5. Documentation must be submitted indicating that the patient has experienced significant adverse effects associated with prednisone therapy. Documentation must include a detailed description of the adverse effect; as the side effect profiles are similar between deflazacort and prednisone, prior authorization shall only be granted for those patients experiencing side effects where deflazacort shows an improved profile. Significant adverse effects are defined as:

    a. Patient has manifested significant psychiatric or behavioral changes negatively impacting function at school, day care, etc; **OR**

    b. Patient has experienced Cushingoid effects or significant weight gain (crossing 2 percentiles and/or reaching 98th percentile for age and sex); **AND**

6. Request must be accompanied with baseline clinical criteria used to assess the patient by at least **one of the following** tests:

    a) Muscle strength tests (such as, Medical Research Council [MRC] scale for muscle strength with 0 being no movement and 5 being normal strength), or
    b) Motor (walk) tests (such as 6-minute walk test [6MWT] distance), or
    c) Pulmonary function tests (such as, forced vital capacity [FVC] and maximal expiratory pressure), or

d) Timed functional tests (such as, standing from lying position; climbing 4 stairs; running/walking 30 feet; propelling a wheelchair 30 feet).

Approval Duration:

Initial approval: will be for 6 months.

Criteria for reauthorization:

1. Patient must continue to meet initial approval criteria; **AND**
2. Demonstrate continued documented compliance; **AND**
3. Documentation that adverse events associated with prednisone therapy were resolved through treatment with Emflaza; **OR**
4. Documented evidence with the most recent results (≤ 6 months prior to request) must be submitted showing clinically significant improvement in DMD associated symptoms, stabilization or lack of progression as compared to the natural history trajectory of the disease demonstrated by at least **one of the following** from pre-treatment baseline status:

   a) Muscle strength tests (such as, Medical Research Council [MRC] scale for muscle strength with 0 being no movement and 5 being normal strength), or
   b) Motor (walk) tests (such as 6-minute walk test [6MWT] distance), or
   c) Pulmonary function tests (such as, forced vital capacity [FVC] and maximal expiratory pressure), or
   d) Timed functional tests (such as, standing from lying position; climbing 4 stairs; running/walking 30 feet; propelling a wheelchair 30 feet).

Continuation of therapy approvals will be granted for 12 months.

References:

1.) Griggs RC, Miller JP, Greenberg CR, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. *Neurology*. 2016;87(20):2123-2131
2.) Lexi-Comp drug monograph for deflazacort (Reviewed 8/22/2017, 1/22/2017, 5/2021)
3.) Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. Neurology. 2016 Nov 15; 87(20): 2123–2131.
4.) UpToDate article: Treatment of Duchenne and Becker muscular dystrophy. Updated July 18, 2017.
5.) Assessing Growth Using the WHO Growth Charts- cdc.gov.
6.) Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. Lancet Neurol. 2010; 9(1): 77-93.
7.) Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. Lancet Neurol. 2018; 17: 251-267.