UnitedHealthcare Pharmacy
Clinical Pharmacy Programs

Program Number	2023 P 3095-7
Program	Step Therapy
Medication	Emflaza® (deflazacort)
P&T Approval Date	5/2017, 10/2018, 10/2019, 10/2020, 10/2021, 10/2022, 10/2023
Effective Date	1/1/2024

1. Background:

Emflaza (deflazacort) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.[1]

In a report from the Guideline Development Subcommittee of the American Academy of Neurology, regarding selection of prednisone versus deflazacort in the treatment of DMD, the following statement is made: "prednisone and deflazacort are possibly equally effective for improving motor function in patients with DMD (2 Class III studies). There is insufficient evidence to directly compare the effectiveness of prednisone vs deflazacort in cardiac function in patients with DMD (1 Class III study of a combined cohort).[2] The UnitedHealthcare Pharmacy and Therapeutics committee has determined that Emflaza is Therapeutically Equivalent to prednisone in the treatment of DMD.

Step Therapy programs are utilized to encourage the use of lower cost alternatives for certain therapeutic classes. This program requires a member to try prednisone or prednisolone prior to receiving coverage for Emflaza.

2. Coverage Criteria[a]:

A. Duchenne Muscular Dystrophy

1. Emflaza will be approved based on **both** of the following criteria:

   a. Diagnosis of Duchenne muscular dystrophy

   -AND-

   b. Patient has a history of failure, contraindication, or intolerance to prednisone or prednisolone

Authorization will be issued for 12 months.

B. Other Diagnoses

1. Emflaza will be approved.

Authorization will be issued for 12 months.

[a] State mandates may apply. Any federal regulatory requirements and the member specific benefit plan coverage may also impact coverage criteria. Other policies and utilization management programs may apply.

3. Additional Clinical Rules:

- Notwithstanding Coverage Criteria, UnitedHealthcare may approve initial and re-authorization based solely on previous claim/medication history, diagnosis codes (ICD-10) and/or claim logic. Use of automated approval and re-approval processes varies by program and/or therapeutic class.
- Supply limits, Medical Necessity and/or Notification may apply

4. References:

1. Emflaza [package insert]. South Plainfield, NJ: PTC Therapeutics Inc. June 2021.
2. Gloss D, Moxley III R, Ashwal S, et. al. 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;465-472.

Program	Step Therapy – Emflaza (deflazacort)
Change Control	
Date	Change
5/2017	New program.
10/2018	Annual review. No changes to criteria. Updated references.
10/2019	Annual review. Updated background updating indication in patients 2 years and older. Updated reference.
10/2020	Annual review with no changes to clinical coverage criteria. Updated references.
10/2021	Annual review with no changes to clinical coverage criteria. Updated references.
10/2022	Annual review. Updated criteria to standard Step Therapy format which includes section for other diagnoses.
10/2023	Annual review with no changes to coverage criteria.