UnitedHealthcare Pharmacy
Clinical Pharmacy Programs

Program Number	2024 P 2127-8
Program	Prior Authorization/Medical Necessity
Medication	Emflaza® (deflazacort)*
P&T Approval Date	5/2017, 10/2018, 10/2019, 10/2020, 10/2021, 10/2022, 10/2023, 10/2024
Effective Date	1/1/2025

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.[3] Three Class III studies directly compared prednisone and deflazacort. In one study, deflazacort and prednisone were shown to have equally beneficial effects on functional motor outcomes, pulmonary function, and development of scoliosis over 5.49 years (SD 1.98).[4] In another study, prednisone and deflazacort were equally effective in improving motor function and functional performance over a 12- month treatment period.[5] A final study reported equivalent cardiac outcome in deflazacort- and prednisone-treated groups over a mean follow-up period of 3.0 years (SD 2.5).[6]

The UnitedHealthcare Pharmacy and Therapeutics Committee has determined that Emflaza is Therapeutically Equivalent to prednisone in the treatment of DMD.

2. Coverage Criteria[a]:

A. <u>Duchenne Muscular Dystrophy</u>

1. Published clinical evidence shows Emflaza* is likely to produce equivalent therapeutic results as other available corticosteroids (e.g., prednisone); therefore, Emflaza* is **not medically necessary** for treatment of Duchenne muscular dystrophy.

[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.

*Emflaza is typically excluded from coverage. Tried/Failed criteria may be in place. Please refer to plan specifics to determine exclusion status.

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.

4. References:

1. Emflaza [package insert]. Warren, NJ: PTC Therapeutics Inc.; May 2024.
2. 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.
3. 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.
4. Balaban B, Matthews DJ, Clayton GH, Carry T. Corticosteroid treatment and functional improvement in Duchenne muscular dystrophy: long-term effect. Am J Phys Med Rehabil. 2005 Nov;84(11):843-50.
5. Bonifati MD, Ruzza G, Bonometto P, Berardinelli A, Gorni K, Orcesi S, Lanzi G, Angelini C. A multicenter, double-blind, randomized trial of deflazacort versus prednisone in Duchenne muscular dystrophy. Muscle Nerve. 2000 Sep;23(9):1344-7.
6. Markham LW, Spicer RL, Khoury PR, Wong BL, Mathews KD, Cripe LH. Steroid therapy and cardiac function in Duchenne muscular dystrophy.

Program	Prior Authorization/Medical Necessity - Emflaza (deflazacort)
Change Control	
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 with no changes to clinical coverage criteria.
10/2023	Annual review with no changes to coverage criteria.
10/2024	Annual review with no changes to coverage criteria. Updated background, added exclusion footnote and updated references.