

Corticosteroids – Deflazacort (Emflaza)

WA.PHAR.135

Effective Date: 2/1/2024

Note: New-to-market drugs included in this class based on the Apple Health Preferred Drug List are non-preferred and subject to this prior authorization (PA) criteria. Non-preferred agents in this class require an inadequate response or documented intolerance due to severe adverse reaction or contraindication to at least TWO preferred agents. If there is only one preferred agent in the class documentation of inadequate response to ONE preferred agent is needed. If a drug within this policy receives a new indication approved by the Food and Drug Administration (FDA), medical necessity for the new indication will be determined on a case-by-case basis following FDA labeling.

To see the list of the current publication of the Coordinated Care of Washington, Inc. Preferred Drug List (PDL), please visit:
https://www.coordinatedcarehealth.com/content/dam/centene/centene-pharmacy/pdl/FORMULARY-CoordinatedCare_Washington.pdf

Medical necessity

Drug	Medical Necessity
Deflazacort (Emflaza)	<p>Corticosteroids – Deflazacort (Emflaza) may be considered medically necessary in patients who meet the criteria described in the clinical policy below.</p> <p>If all criteria are not met, the clinical reviewer may determine there is a medically necessary need and approve on a case-by-case basis. The clinical reviewer may choose to use the reauthorization criteria when a patient has been previously established on therapy and is new to Apple Health.</p> <p>Patients new to Apple Health or new to an MCO who are requesting regimens for continuation of therapy are reviewed following the reauthorization criteria listed below.</p>

Clinical policy:

Clinical Criteria	
Duchenne Muscular Dystrophy Deflazacort (Emflaza)	Deflazacort (Emflaza) may be approved when all of the following criteria are met: <ol style="list-style-type: none"> 1. Patient is 2 years of age or older, AND 2. Prescribed by, or in consultation with, a neurologist; AND 3. Diagnosis of Duchenne muscular dystrophy confirmed with genetic testing; AND 4. History of failure, contraindication, or intolerance to a 6-month trial of prednisone within the past 12 months. <ol style="list-style-type: none"> a. Intolerance is defined as ONE of the following after initiation of prednisone: <ol style="list-style-type: none"> i. Increase of 10 weight-for-age percentiles within the past 12 months; OR

	<p>ii. Weight gain resulting in greater than or equal to the 85th weight-for-age percentile within the past 12 months; OR</p> <p>iii. Severe psychiatric adverse effects</p> <p>If ALL criteria are met, the request will be authorized for 6 months.</p> <p>Criteria (Reauthorization)</p> <p>Deflazacort (Emflaza) may be approved when all of the following criteria are met:</p> <ol style="list-style-type: none"> 1. Documentation is submitted demonstrating disease stability or a positive clinical response [e.g. stabilization of muscle strength or pulmonary function]; <p>If ALL criteria are met, the request will be authorized for 12 months.</p>
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Dosage and quantity limits

Drug	Indication	FDA Approved Dosing	Dosage Form
Emflaza	Duchenne muscular dystrophy	0.9 mg/kg once daily	<ul style="list-style-type: none"> • Oral suspension 22.75 mg/1 mL For tablets: Total dose rounded to nearest tablet size • 6 mg oral tablet • 18 mg oral tablet • 30 mg oral tablet • 36 mg oral tablet

Background:

Duchenne muscular dystrophy is a genetic disorder caused by a defective gene on the X chromosome. The gene defect results in a significant reduction in dystrophin which is needed for muscle function. This causes muscle wasting and weakness. As it is a progressive disease, the muscle damage may worsen and spread over time to affect other muscles. The American Academy of Neurology (AAN) published a practice guidelines update for the use of corticosteroids to treat Duchenne muscular dystrophy. The guidelines recommend prednisone should (Level B evidence – moderate value of benefit relative to risk and moderate confidence in evidence) be offered for improving strength and pulmonary function and that prednisone may (Level C evidence - small value of benefit relative to risk and low confidence of evidence) be offered to improve timed motor function, reduce the need for scoliosis surgery and delay cardiomyopathy onset by 18 years of age. Deflazacort may (Level C) be offered for improving strength and timed motor function, delaying age of at loss of ambulation by 1.4 to 2.5 years, improving pulmonary function, reducing the need for scoliosis surgery, delaying cardiomyopathy onset and increasing survival at 5-15 years of age of follow-up. Both prednisone and deflazacort may (Level C) be equivalent in improving motor function. Prednisone may (Level C) have greater weight gain (5.05 kg vs 8.45 kg for deflazacort and prednisone, respectively) during the first 12 months of treatment and no significant difference with extended use. Deflazacort may (Level C) be associated with a greater risk of cataracts than prednisone.

The safety and efficacy of deflazacort (Emflaza) was established in two randomized, double-blind, placebo-controlled trials. In study 1 (N=196), male pediatric patients from ages 5 to 15 showed a significant change in average muscle strength score, assessed by modified Medical Research Council scale, between baseline and week 12 for deflazacort (0.15; 95% CI [0.01, 0.28]) compared to placebo (-0.10; 95% CI [-0.23, 0.03]), P=0.017. There was no significant difference in average muscle strength score with deflazacort compared to prednisone (0.27; 95% CI [0.13, 0.41]) at week 12. In Study 2 (N=29), male participants aged 6 to 12 showed no significant difference for the primary endpoint of average muscle strength scores at 2 years when compared to placebo. Common adverse effects $\geq 5\%$ include Cushingoid appearance, increased weight, increased appetite, upper respiratory tract infection, cough, pollakiuria, nasopharyngitis, hirsutism, central obesity, erythema, irritability, rhinorrhea, and abdominal comfort.

References

1. Griggs R, Miller J, Greenberg C, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. *Neurology*. 2016 Nov 15;87(20):2123-2131.
2. Emflaza [Prescribing Information]. South Plainfield, NJ: PTC Therapeutics. June 2021.
3. Birnkrant D, Bushby K, Bann C, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. *The Lancet Neurology*. 2018 Jan; 1-17.
4. Gloss D, Moxley R, Ashwal S, et al. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy. *Neurology*. 2016; 86: 465-472.
5. Bonifati MD, Ruzza G, Bonometto P, et al. A multicenter, double-blind, randomized trial of deflazacort versus prednisone in Duchenne muscular dystrophy. *Muscle Nerve*. 2000;23(9):1344-1347.
6. Bonifati M, Ruzza G, Bonometto P, et al. A Multicenter Double-Blind Randomized Trial of Deflazacort Versus Prednisone in Duchenne Muscular Dystrophy: Analysis after 2 Years. *Basic Appl Myol*. 2000. 10(4):171-175.
7. Mendell JR, Moxley RT, Griggs RC, et al. Randomized, double-blind six-month trial of prednisone in Duchenne's muscular dystrophy. *N Engl J Med* 1989;320:1592–1597.
8. Reitter B. Deflazacort vs. prednisone in Duchenne muscular dystrophy: trends of an ongoing study. *Brain Dev*. 1995;17 Suppl:39-43. doi: 10.1016/0387-7604(95)00015-1.
9. Dubowitz V. Workshop Report. 75th European Neuromuscular Centre International Workshop: 2nd workshop on the treatment of muscular dystrophy 10–12 December, 1999, Naarden, the Netherlands. *Neuromuscul Disord*. 2000;10(4):313-320.
10. Karimzadeh P, Ghazavi A. Comparison of deflazacort and prednisone in Duchenne muscular dystrophy. *Iran J Child Neurol*. 2012;6.
11. Clinical Growth Charts. Centers for Disease Control and Prevention. https://www.cdc.gov/growthcharts/clinical_charts.htm. Accessed 6/8/2023.

History

Approved Date	Effective Date	Version	Action and Summary of Changes
08/16/2023	02/01/2024	22.10.00.AA-1	Approved by DUR Board on 8/16/2023 New policy created