Emflaza® (deflazacort)
Updated July 2020

Emflaza® (deflazacort) is a corticosteroid indicated for the treatment of patients ≥ 2 years of age with Duchenne muscular dystrophy (DMD).

Criteria for Approval:

1. For the treatment of Duchenne muscular dystrophy (DMD) – genetic test to confirm diagnosis must be provided
2. The patient is ≥ 2 years of age
3. Patient has had a 3-month trial of prednisone at the optimal dose of 0.75mg/kg/day unless the patient has experienced an inadequate response, intolerance, or has a contraindication to therapy (intolerance includes, but is not limited to weight gain, behavioral disturbance, growth restriction, pubertal delay, and vertebral fractures)
4. Prescribed by or in consultation with a pediatric/adult neurologist or a physician who is an expert in the treatment of DMD, other neuromuscular disorders
5. Dosing is in accordance with FDA labeling and PI guidelines
6. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Lexi-Drugs, national guidelines, or other peer reviewed evidence

Approval Duration: 6 months

Addendum:

1. Criterion #2 changed to ≥ 2 years of age (was ≥ 5 years of age)
2. Criterion #3 changed to allow more flexibility

References:

1. Emflaza™ [package insert]. PTC Therapeutics, Inc. South Plainfield, NJ. June 2019.
2. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2016. Updated periodically
3. Bushby K, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular,
4. rehabilitation, endocrine, and gastrointestinal and nutritional managementThe Lancet Neurol 2018; 17,3; 251-267.
5. Griggs RC et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy.
6. Neurology. 2016 Nov 15;87(20):2123-2131.