

## Emflaza (deflazacort)

### POLICY

#### I. CRITERIA FOR APPROVAL

An authorization of 6 months may be granted for the treatment of Duchenne Muscular Dystrophy if:

- A. Member is at least 2 years of age
- B. Emflaza is prescribed by or given in consultation with a neurologist
- C. The diagnosis of DMD was confirmed and documented by one of the following criteria:
  - a. Genetic testing demonstrating a mutation in the *DMD* gene.
  - b. Muscle biopsy demonstrating absent dystrophin.
- D. Member has had intolerable side effects to at least a three-month trial of maximal Prednisone dosing
- E. Baseline Documentation of One or More of the following:
  - a. Dystrophin level
  - b. 6-minute walk test (6MWT) or other timed function tests (e.g., time to stand [T\*STAND], time to run/walk 10 meters [T\*TRW], time to climb 4 stairs [T\*CLIMB])
  - c. Upper limb function (ULM) test
  - d. North Star Ambulatory Assessment (NSAA)
  - e. Forced Vital Capacity (FVC) percent predicted

#### II. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for all members (including new members) who meet both of the following:

Patient has responded to therapy compared to pretreatment baseline based on documentation of one or more of the following (not all-inclusive):

- a. Increase in dystrophin level
- b. Stability, improvement, or slowed rate of decline in 6MWT or other timed function tests (e.g., time to stand [T\*STAND], time to run/walk 10 meters [T\*TRW], time to climb 4 stairs [T\*CLIMB])
- c. Stability, improvement, or slowed rate of decline in ULM test
- d. Stability, improvement, or slowed rate of decline in NSAA
- e. Stability, improvement, or slowed rate of decline in FVC% predicted
- f. Improvement in quality of life

#### III. QUANTITY LIMIT

- Emflaza Suspension 22.75mg/ml: 1.8mL per day (or 4 bottles per month)
- Emflaza 18mg, 30mg, and 36mg Tablet: 1 Tablet per day
- Emflaza 6mg Tablet: 2 Tablets per day

Effective Date: 02/01/2021
Reviewed: 11/2020, 5/2021, 4/2022, 4/2023
Scope: Medicaid

**IV. COVERAGE DURATION**

Initial: 6 months  
Renewal: 12 months

**V. REFERENCES**

Emflaza [package insert]. South Plainfield, NJ: PTC Therapeutics, Inc.; February 2023.