



RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Emflaza (deflazacort) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD). Specifically, deflazacort is a corticosteroid prodrug, whose active metabolite acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The precise mechanism by which deflazacort exerts its therapeutic effects in patients with DMD is unknown (1).

Regulatory Status

FDA-approved indication: Emflaza is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older (1).

Emflaza can suppress the immune system and increase the risk of infection with any pathogen, including viral, bacterial, fungal, protozoan, or helminthic. Corticosteroids reduce resistance to new infections, exacerbate existing infections, increase the risk of disseminated infections, increase the risk of reactivation or exacerbation of latent infections, and mask some signs of infection (1).

All immunizations should be administered according to immunization guidelines prior to starting Emflaza. Live or live-attenuated vaccines should be administered at least 4 to 6 weeks prior to starting Emflaza. Patients on Emflaza may receive concurrent vaccinations, except for live or live-attenuated vaccines (1).

Monitoring motor changes in patients with DMD requires functional evaluation along with measurement of muscle strength. The need for a reliable outcome measure in diseases of rapid deterioration such as DMD has led to the use of motor functional tests. In a large, multicenter, international clinical trial, the six minute walk test (6MWT) proved to be feasible and highly reliable. Also used are the Motor Function Measure (MFM) and North Star Ambulatory Assessment (NSAA) to help predict loss of ambulation 1 year before its occurrence in order to allow time to adapt rehabilitation, change the patient's environment, and consider acquisition of assistive aids or the use of medications (2-4).

Safety and effectiveness in patients 2 years of age and older have been established (1).



**EMFLAZA
(deflazacort)**

Summary

Emflaza (deflazacort) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD). Emflaza acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The most common adverse reactions are Cushingoid appearance, increase weight, increase appetite, upper respiratory tract infection, cough, pollakiuria, hirsutism, central obesity, and nasopharyngitis. Safety and effectiveness in patients 2 years of age and older have been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Emflaza while maintaining optimal therapeutic outcomes.

References

1. Emflaza [package insert]. South Plainfield, NJ: PTC Therapeutics; May 2024.
2. McDonald C, Henricson E, et al. The 6-Minute Walk test and Other Clinical Endpoints in Duchenne Muscular Dystrophy: Reliability, Concurrent Validity, and Minimal Clinically Important Differences from a Multicenter Study. *Muscle Nerve*. 2013 Sep; 48(3): 357–368.
3. McDonald C, Henricson E, et al. The 6-Minute Walk test and Other Endpoints in Duchenne Muscular Dystrophy: Longitudinal Natural History Observations Over 48 weeks from a Multicenter Study. *Muscle Nerve*. 2013 Sep; 48(3): 343–356.
4. Vuillerot C, Girardot F, et al. Monitoring changes and predicting loss of ambulation in Duchenne muscular dystrophy with the Motor Function Measure. *Developmental Medicine & Child Neurology* 2010, 52: 60–65.