Emflaza

(deflazacort)

New Product Slideshow

MPR

Introduction

- **Brand name:** Emflaza
- **Generic name:** Deflazacort
- **Pharmacological class:** Corticosteroid
- **Strength and Formulation:** 6mg, 18mg, 30mg, 36mg tablets; 22.75mg/mL oral suspension (contains benzyl alcohol)
- **Manufacturer:** Marathon
- **How supplied:** Tabs 6mg—100; 18mg, 30mg, 36mg—30; Oral susp—13mL (w. oral dispensers)
- **Legal Classification:** Rx

EMFLAZA

Indications

- Duchenne muscular dystrophy (DMD)

Dosage & Administration

- **Tablets:**
  - <5yrs: not established
  - Swallow tabs whole or may crush and mix with applesauce
  - ≥5yrs: 0.9mg/kg once daily
  - Round up to the nearest possible dose
  - Concomitant moderate or strong CYP3A4 inhibitors (eg, clarithromycin, fluconazole, diltiazem, verapamil, grapefruit juice): give ⅓ dose

Dosage & Administration

- **Oral suspension:**
  - <5yrs: not recommended
  - Use oral dispenser
  - Slowly add into 3–4oz of juice or milk (avoid grapefruit juice)
  - ≥5yrs: 0.9mg/kg once daily
  - Round to the nearest tenth of a milliliter (mL)
  - Concomitant moderate or strong CYP3A4 inhibitors (eg, clarithromycin, fluconazole, diltiazem, verapamil): give ⅓ dose

Considerations for Special Populations

- **Pregnancy:** Use only if potential benefit justifies potential risk to the fetus
- **Nursing mothers:** Consider health benefits of breastfeeding with potential adverse effects
- **Pediatric:** <5yrs: not established
- **Elderly:** No geriatric experience with Emflaza

Warnings/Precautions

- Increased risk of **infection** (eg, viral, bacterial, fungal, protozoan, helminthic) and may mask signs/symptoms
- If exposed to **chickenpox** or **measles**, consider prophylactic passive immune therapy
- Concomitant systemic fungal infections, active ocular herpes simplex: not recommended
- Hepatitis B virus reactivation

Warnings/Precautions

- Latent or acute amebiasis
- Strongyloides infestation
- Cushing's syndrome
- Hyperglycemia
- Thyroid disorders
- Hypopituitarism
- Adrenal insufficiency
- Congenital adrenal hyperplasia
- Pheochromocytoma
- Supplement with additional steroids during physiologic stress

Warnings/Precautions

- CHF
- Hypertension
- Recent MI
- Renal insufficiency
- Peptic ulcers
- Diverticulitis
- Intestinal anastomoses
- Ulcerative colitis
- Psychotic tendencies
- Myasthenia gravis

Warnings/Precautions

- Thromboembolic disorders
- Risk of **osteoporosis**; monitor bone mineral density with long-term therapy
- **Discontinue** at the first sign of rash
- Avoid abrupt cessation
- Monitor weight, growth, BP, fluid, electrolyte balance, blood glucose, and intraocular pressure (w. therapy >6 weeks)
- **Oral suspension:** neonates/infants (gasping syndrome)

Interactions

- See **Adults and Children**
- Live or live attenuated vaccines: not recommended; may get suboptimal response
- **Avoid** concomitant moderate or strong CYP3A4 inducers (eg, efavirenz, carbamazepine, phenytoin)
- Concomitant **levothyroxine**: give corticosteroid first
- May need to adjust dose of **antidiabetic** agents
- Increased risk of acute myopathy with concomitant **neuromuscular blockers** (eg, pancuronium)

Adverse Reactions

- Cushingoid appearance
- Weight increase
- Increased appetite
- Upper RTI
- Cough
- Pollakiuria
- Hirsutism
- Central obesity
- Nasopharyngitis
- HPA axis suppression
- Steroid withdrawal syndrome
- Avascular necrosis
- GI perforation
- Behavioral/mood changes
- Glaucoma
- Cataracts
- Myopathy
- Kaposi's sarcoma
- Anaphylaxis
- Negative growth/development effects (in children)

Mechanism of Action

- Deflazacort is a corticosteroid prodrug, whose active metabolite acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects

Clinical Trials

- **Study 1** was a multicenter, randomized, double-blind, placebo-controlled, 52-week study (n=196) that included male patients aged 5–15 years
- Study patients were randomized to deflazacort 0.9mg/kg/day or 1.2mg/kg/day, an active comparator, or placebo
- After 12 weeks, placebo-treated patients were re-randomized to either deflazacort or the active comparator

Clinical Trials

- **Efficacy** was assessed as the change between baseline and Week 12 in average strength of 18 muscle groups

Clinical Trials

- Patients in the deflazacort 0.9mg/kg/day group had a **significantly greater change in average muscle strength** score vs. placebo (0.15 [95% CI: 0.01, 0.28] vs. -0.10 [95% CI: -0.23, 0.03]; P=0.017)
- The deflazacort 1.2mg/kg/day group showed a small additional benefit vs. placebo at Week 12 but had a higher incidence of adverse reactions

Clinical Trials

- **Study 2** was a randomized, double-blind, placebo-controlled, 104-week trial that evaluated deflazacort vs. placebo (n=29)
- Analysis data of the primary endpoint of average muscle strength scores at 2 years were not statistically significant
- **Average muscle strength scores** at Months 6 and 12, as well as the average time to loss of ambulation, numerically favored deflazacort vs. placebo although not statistically controlled for multiple comparisons

- For more clinical trial data, see full labeling

New Product Monograph

- For more information view the product monograph available at:

  http://www.empr.com/emflaza/drug/34655/