June 2019 Label Expansion

Frequently Asked Questions for Healthcare Professionals

Emflaza®
(deflazacort)
6 mg | 18 mg | 30 mg | 36 mg tablets
22.75 mg/mL oral suspension

1 When did EMFLAZA® (deflazacort) receive approval for use in patients 2 to 5 years of age with Duchenne?

On June 7, 2019, the EMFLAZA label was updated to include the use of EMFLAZA in patients 2 years of age and older. Use of EMFLAZA in pediatric patients is supported by a multicenter, randomized, double-blind, placebo- and active-controlled study in 196 males, 5 to 15 years of age.

2 What is EMFLAZA?

EMFLAZA is the first and only corticosteroid approved in the U.S. for the treatment of Duchenne muscular dystrophy and is now approved for use in patients 2 years of age and older.

3 Is there a specific age range for which EMFLAZA may be used?

EMFLAZA is now approved for use in patients with Duchenne muscular dystrophy who are 2 years of age and older.

4 How is EMFLAZA administered to patients?

EMFLAZA is available as both a tablet and as an oral suspension. Both can be administered with or without food. EMFLAZA tablets can be taken whole or crushed and taken immediately after mixing with applesauce. EMFLAZA oral suspension should be shaken well before administration and used only with the dispenser provided with the product.

5 What do I need to know about EMFLAZA and immunizations?

Prior to treatment with EMFLAZA, administer all immunizations according to immunization guidelines. Live-attenuated or live vaccines must be administered at least 4 to 6 weeks prior to starting EMFLAZA. Your patients may receive concurrent vaccines while taking EMFLAZA except for live-attenuated or live vaccines.

6 Is it safe for my 2- to 5-year-old patients to take EMFLAZA even though the oral suspension contains benzyl alcohol?

EMFLAZA oral suspension can be safely administered to children two years of age and older. The amount of benzyl alcohol that a 2-year-old child would receive with EMFLAZA oral suspension is less than the amount recommended as safe by the World Health Organization as a food additive. Overall combined use of benzyl alcohol from all other sources should be monitored and discussed with a patient's caregiver.

INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA® (deflazacort)

INDICATION

EMFLAZA® is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

IMPORTANT SAFETY INFORMATION

Contraindications: EMFLAZA is contraindicated in patients with a hypersensitivity to deflazacort or any of the inactive ingredients in EMFLAZA.

Warnings & Precautions

- **Alterations in Endocrine Function:** Corticosteroids, such as EMFLAZA, can cause serious and life-threatening alterations in endocrine function, especially with chronic use. Monitor patients receiving EMFLAZA for Cushing's syndrome, hyperglycemia, and adrenal insufficiency after EMFLAZA withdrawal. In addition, patients with hypopituitarism, primary adrenal insufficiency or congenital adrenal hyperplasia, altered thyroid function, or pheochromocytoma may be at increased risk for adverse endocrine events. Acute adrenal insufficiency or "withdrawal syndrome" can occur if corticosteroids are withdrawn abruptly, and can be fatal. The risk is reduced by gradually tapering the corticosteroid dose when withdrawing treatment. During times of medical stress, corticosteroid dosage may need to be increased.
- **Immunosuppression and Increased Risk of Infection:** Increased risk of new, exacerbation, dissemination, or reactivation of latent infections, which can be severe and at times fatal; Signs and symptoms of infection may be masked. Tell patients and/or caregivers to inform their healthcare provider if the patient has had recent or ongoing infections or if they have recently received a vaccine. Warn patients who are on corticosteroids who have not had chickenpox or measles to avoid exposure to chickenpox or measles and to alert their healthcare provider immediately if they are exposed.

7 How do I write a prescription for EMFLAZA?

To write a prescription for EMFLAZA, you will need to take the following steps:

1. Once you determine that EMFLAZA is appropriate for your patient, you will need to fill out the EMFLAZA Prescription Start Form (PSF), available at www.EMFLAZA.com. This form includes insurance and patient information and consent forms.
2. After you and your patient's caregiver have completed the PSF, you will need to submit it to PTC *Cares*™—PTC Therapeutics patient support services program.
3. A PTC *Cares* case manager will then contact your patient's caregiver to review the information on the PSF and work with the pharmacy to support insurance coverage and make arrangements for delivery of EMFLAZA on approval.

If you or your patient's family have any questions about patient support services or want to speak to a case manager, please call PTC *Cares* at 1-844-4PTC-CARES (1-844-478-2227) to learn more.

8 Where can I learn more about EMFLAZA?

To learn more about EMFLAZA, please visit www.EMFLAZA.com or call 1-844-4PTC-CARES (1-844-478-2227).

IMPORTANT SAFETY INFORMATION (CONT'D)

- **Alterations in Cardiovascular/Renal Function:** Monitor for elevated blood pressure. Dietary salt restriction and potassium supplementation may be needed.
- **Gastrointestinal Perforation:** Increased risk of gastrointestinal perforation during corticosteroid use in patients with certain gastrointestinal disorders such as active or latent peptic ulcers, diverticulitis, recent intestinal anastomoses, and inflammatory bowel disease. Signs and symptoms may be masked.
- **Behavioral and Mood Disturbances:** May include euphoria, insomnia, mood swings, personality changes, severe depression, and psychosis. Encourage patients to seek medical attention if symptoms develop or worsen.
- **Effects on Bones:** The risk of osteoporosis increases with prolonged use of EMFLAZA, which can predispose patients to vertebral and long bone fractures. Monitor for decreases in bone density with chronic use of EMFLAZA.
- **Ophthalmic Effects:** May include cataract formation, ocular infections, and glaucoma. If treatment with corticosteroids, including EMFLAZA, are continued for more than 6 weeks, monitor intraocular pressure.
- **Vaccination:** Do not administer live or live attenuated vaccines to patients receiving immunosuppressive doses of corticosteroids. Administer live-attenuated or live vaccines at least 4 to 6 weeks prior to starting EMFLAZA.
- **Serious Skin Rashes:** Toxic epidermal necrolysis has been reported with the use of deflazacort. Discontinue at the first sign of rash, unless the rash is clearly not drug related.
- **Effects on Growth and Development:** Long-term use of corticosteroids, including EMFLAZA, may slow growth and development in children.
- **Thromboembolic Events:** Observational studies have shown an increased risk of thromboembolism. Use EMFLAZA with caution in patients who have or may be predisposed to thromboembolic disorders.

Adverse Reactions: The most common adverse reactions (≥10% for EMFLAZA and greater than placebo) are Cushingoid appearance, weight increased, increased appetite, upper respiratory tract infection, cough, pollakiuria, hirsutism, central obesity, and nasopharyngitis.

Drug Interactions: Give one third of the recommended dose of EMFLAZA when EMFLAZA is administered with strong or moderate CYP3A4 inhibitors. Avoid use of strong or moderate CYP3A4 inducers with EMFLAZA, as they may reduce efficacy.

Please see www.emflaza.com for the full Prescribing Information.

For medical information, product complaints, or to report an adverse event, please call **1-866-562-4620** or email at **usmedinfo@ptcbio.com**.

You may also report adverse events directly to FDA at **1-800-FDA-1088** or **www.fda.gov/medwatch**.

http://hcp.emflaza.com/ 1-844-4PTC-CARES (1-844-478-2227)

PTC *Cares*™