Frequently Asked Questions for Patients and Families

1. **What is EMFLAZA® (deflazacort)?**
   EMFLAZA is the first and only corticosteroid approved in the U.S. for the treatment of Duchenne muscular dystrophy and is indicated in patients 2 years of age and older.

2. **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.

3. **How is EMFLAZA administered to my child?**
   EMFLAZA is available both as a tablet and as an oral suspension. Both can be taken 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 taking and used only with the dispenser provided with the prescription.

4. **Is it safe for my 2- to 5-year-old to take EMFLAZA even though the oral suspension contains benzyl alcohol?**
   EMFLAZA oral suspension can be safely prescribed to children two years of age and older. The amount of benzyl alcohol that a 2-year-old would receive with EMFLAZA oral suspension is less than the amount recommended as safe by the World Health Organization as a food additive. Other sources of benzyl alcohol used in combination with EMFLAZA should be monitored and discussed with your child’s healthcare provider.

5. **What do I need to know about EMFLAZA and my child’s immunizations?**
   Before your child begins taking 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 child may receive concurrent vaccines while taking EMFLAZA except for live-attenuated or live vaccines.

6. **How can I get a prescription for EMFLAZA?**
   To receive a prescription for EMFLAZA, you will need to take the following steps:
   1. Talk to your child’s healthcare provider about treatment with EMFLAZA and if it is appropriate for your child.
   2. If EMFLAZA is appropriate, your child’s healthcare provider will then fill out an EMFLAZA Prescription Start Form (PSF), available at EMFLAZA.com, which includes insurance and patient information, and consent forms.
   3. Once you and your child’s healthcare provider have completed the PSF, he or she will submit it to PTC Cares™—PTC Therapeutics patient support services program.
   4. A PTC Cares case manager will then contact you 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 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.

**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**
Contraindication: Do not use if you are allergic to deflazacort or any of the inactive ingredients in EMFLAZA.

Do not stop taking EMFLAZA, or change the amount you are taking, without first checking with your healthcare provider, as there may be a need for gradual dose reduction to decrease the risk of adrenal insufficiency and steroid “withdrawal syndrome”. Acute adrenal insufficiency can occur if corticosteroids are withdrawn abruptly, and can be fatal. A steroid “withdrawal syndrome,” seemingly unrelated to adrenocortical insufficiency, may also occur following abrupt discontinuance of corticosteroids. For patients already taking corticosteroids during times of stress, the dosage may need to be increased.
**7 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).

**8 When was EMFLAZA approved for use in 2- to 5-year-olds 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. The safety and effectiveness of EMFLAZA in pediatric patients is supported by studies of almost 200 boys between the ages of 5 and 15. Use of EMFLAZA in patients 2 years to less than 5 years of age is supported by the findings of efficacy and safety in patients 5 years and older with DMD.

**IMPORTANT SAFETY INFORMATION (CONT'D)**

- **Hyperglycemia:** Corticosteroids can increase blood glucose, worsen pre-existing diabetes, predispose those on long-term treatment to diabetes mellitus, and may reduce the effect of anti-diabetic drugs. Monitor blood glucose at regular intervals. For patients with hyperglycemia, anti-diabetic treatment should be initiated or adjusted accordingly.

- **Increased Risk of Infection:** Tell your healthcare provider if you have had recent or ongoing infections or if you have recently received a vaccine or are scheduled for a vaccination. Seek medical advice at once should you develop fever or other signs of infection, as some infections can potentially be severe and fatal. Avoid exposure to chickenpox or measles, but if you are exposed, medical advice should be sought without delay.

- **Alterations in Cardiovascular/Kidney Function:** EMFLAZA can cause an increase in blood pressure, salt and water retention, or a decrease in your potassium and calcium levels. If this occurs, dietary salt restriction and potassium supplementation may be needed.

- **Behavioral and Mood Disturbances:** There is a potential for severe behavioral and mood changes with EMFLAZA and you should seek medical attention if psychiatric symptoms develop.

- **Effects on Bones:** There is a risk of osteoporosis or decrease in bone mineral density with prolonged use of EMFLAZA, which can potentially lead to vertebral and long bone fractures.

- **Effects on Growth and Development:** Long-term use of corticosteroids, including EMFLAZA may slow growth and development in children.

- **Ophthalmic Effects:** EMFLAZA may cause cataracts, ocular infections and glaucoma and you should be monitored if corticosteroid therapy is continued for more than 6 weeks.

- **Vaccination:** The administration of live or live attenuated vaccines is not recommended in patients on EMFLAZA. Live-attenuated or live vaccines can be administered at least 4 to 6 weeks prior to starting EMFLAZA.

- **Serious Skin Rashes:** Seek medical attention at the first sign of a rash.

- **Drug Interactions:** Certain medications can cause an interaction with EMFLAZA. Tell your healthcare provider of all the medicines you are taking, including over-the-counter medicines (such as insulin, aspirin or other NSAIDS), dietary supplements, and herbal products. Alternate treatment, dosage adjustment, and/or special test(s) may be needed during the treatment.

**Common side effects that could occur with EMFLAZA include:** Facial puffiness or Cushingoid appearance, weight increased, increased appetite, upper respiratory tract infection, cough, frequent daytime urination, unwanted hair growth, central obesity, and colds.

**Please see the accompanying 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://www.emflaza.com 1-844-4PTC-CARES (1-844-478-2227)