Accessing EMFLAZA™ (deflazacort) for Duchenne muscular Dystrophy

PPMD 2017 Connect Conference

June 29, 2017; 4:00pm

Stuart Peltz, PhD

PTC Therapeutics, South Plainfield, NJ

Forward looking statements within the meaning of The Private Securities Litigation Reform Act of 1995

All statements, other than those of historical fact, contained in this presentation, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC; the PDUFA date for the new drug application (NDA) for Translarna for the treatment of nonsense Duchenne muscular dystrophy (nmDMD) as well as the date of the advisory committee meeting; PTC's ability to maintain the current label under the marketing authorization in the European Economic Area (EEA) for Translarna™ (ataluren) for nmDMD; the clinical utility and potential advantages of Translarna (ataluren) and Emflaza™ (deflazacort); PTC's expectations with respect to the future commercial availability of, and access to, Emflaza; PTC's strategy, future operations, future financial position, future revenues or projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: PTC's ability to resolve the matters set forth in the Refuse to File letter it received from the FDA in connection with its NDA for Translarna for the treatment of nmDMD, including whether PTC's filing of the NDA over protest with the FDA will result in a timely or successful review of the NDA, and whether PTC will be required to perform additional clinical and non-clinical trials or analyses at significant cost, which, if successful, could potentially support the approval of the NDA filed over protest or a new NDA submission; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the EEA, including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to enroll, fund, complete and timely submit to the EMA the results of Study 041, which is a specific obligation to continued marketing authorization in the EEA; PTC's scientific approach and general development progress; matters related to PTC's commercial launch of Emflaza in the United States; PTC's ability to realize the anticipated benefits of its acquisition of Emflaza, including the possibility that the expected benefits from the acquisition will not be realized or will not be realized within the expected time period; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's Quarterly Report on Form 10-Q as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna or Emflaza.

The forward-looking statements contained herein represent PTC's views only as of the date of this presentation and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this presentation except as required by law.

PTC THERAPEUTICS

PTC Therapeutics, Inc.

- Founded 20 years ago
- Now has a global footprint with US headquarters located in NJ
- Focused on discovery, development and commercialization of oral therapies for rare diseases
- Multiple programs across genetic disorders and oncology

Our Mission

To leverage our knowledge of **RNA biology** to bring **novel therapeutics** to patients affected by **rare and neglected disorders**

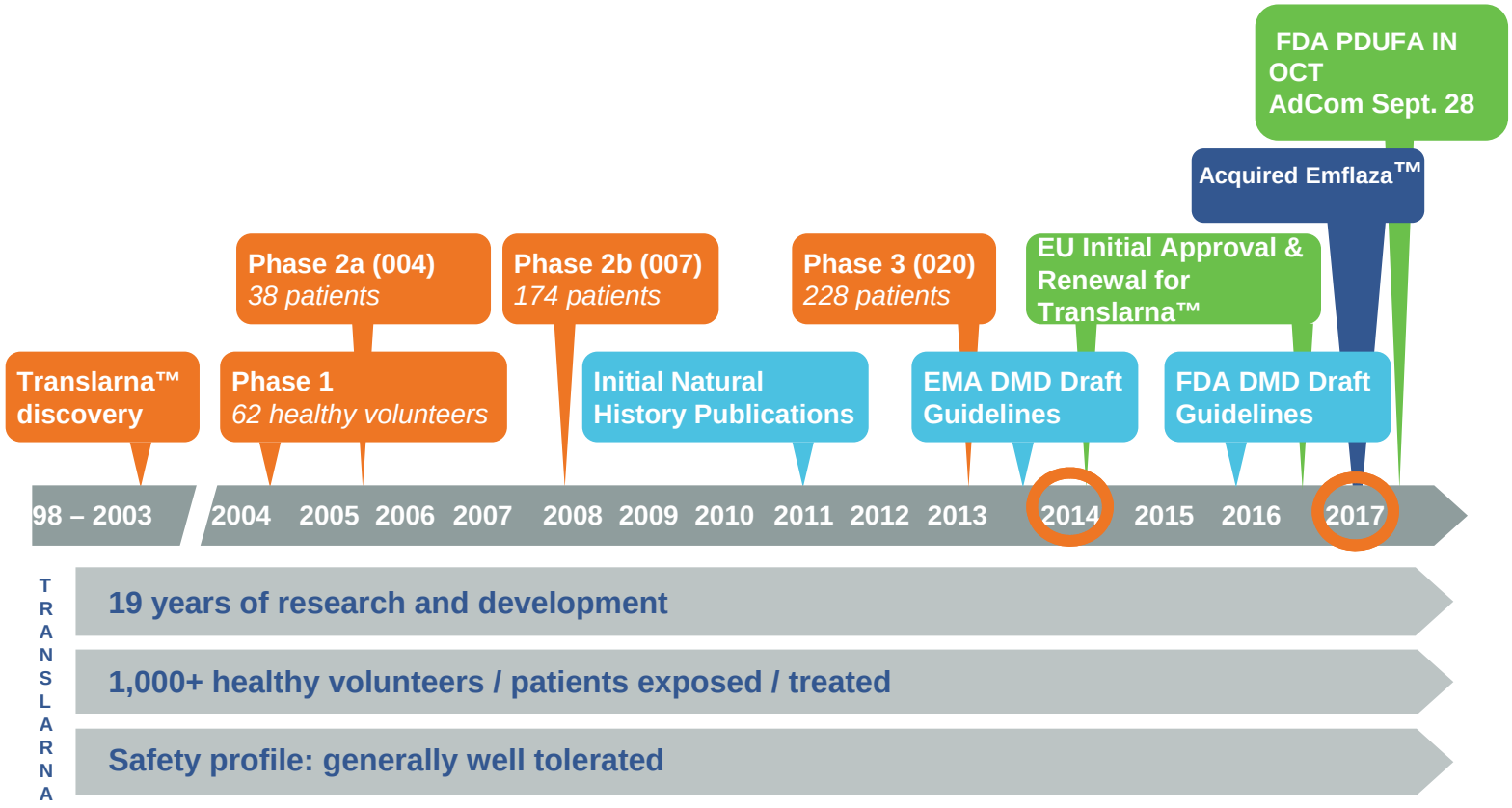
Commitment to patients with rare and neglected diseases






PTC THERAPEUTICS

PTC– A history steeped in commitment to DMD



* Approval is subject to annual review and renewal by the European Commission, following reassement by the EMA of the risk/benefit balance

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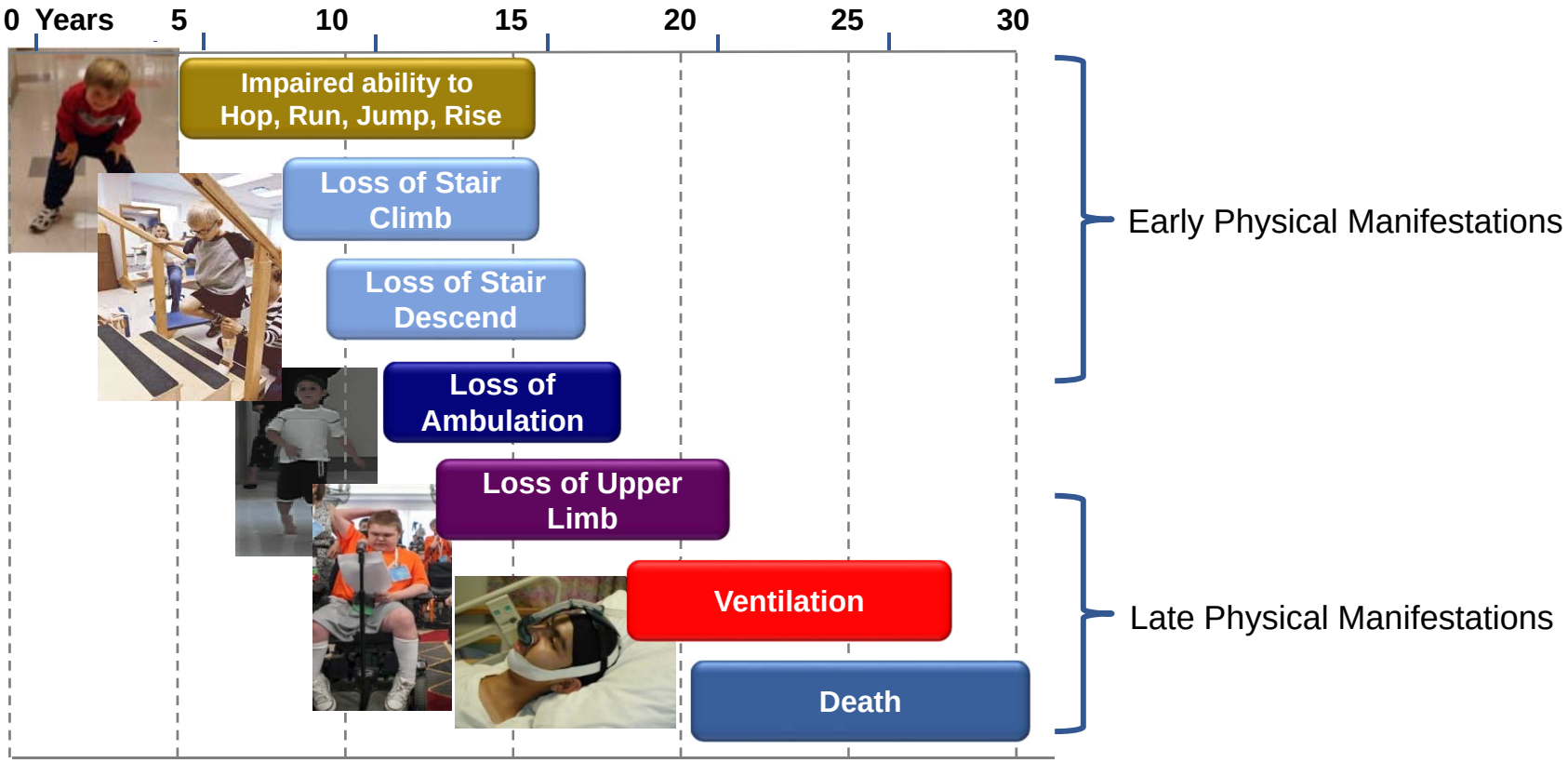
DMD Milestones are Irreversible; drugs that slow disease progression change the course of the disease



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Targeting the inflammatory component of DMD

DUCHENNE MUSCULAR DYSTROPHY

Immune-mediated pathology in Duchenne muscular dystrophy

Amy S. Rosenberg,[1]* Montserrat Puig,[1] Kanneboyina Nagaraju,[2] Eric P. Hoffman,[2] S. Armando Villalta,[3] V. Ashutosh Rao,[1] Lalage M. Wakefield,[4] Janet Woodcock[1]

Immunological and inflammatory processes downstream of dystrophin deficiency as well asmetabolic abnormalities, defective autophagy, and loss of regenerative capacity all contribute to muscle pathology in Duchenne muscular dystrophy (DMD). These downstream cascades offer potential avenues for pharmacological intervention. Modulating the inflammatory response and inducing immunological tolerance to de novo dystrophin expression will be critical to the success of dystrophin-replacement therapies. This Review focuses on the role of the inflammatory response in DMD pathogenesis and opportunities for clinical intervention.

Science Translation Medicine; Volume 7; 5 Aug. 2015; Page 1-12

PTC THERAPEUTICS

Emflaza™ (deflazacort)

Acquired in April, 2017

Acquisition based on our understanding of the results from the placebo arm from our Randomized Control Trials

PTC THERAPEUTICS

EMFLAZA™ is the first FDA approved corticosteroid for treatment of Duchenne muscular dystrophy

- EMFLAZA™ indication is regardless of genetic mutation
- Received FDA approval on February 9, 2017
- EMFLAZA™ Improved muscle strength and slowed disease progression
- We believe that EMFLAZA™ should be available for Duchenne patients as part of their totality of care

EMFLAZA™ (deflazacort)

EMFLAZA is indicated for the treatment of Duchenne muscular dystrophy in patients 5 years of age and older.

- **The first FDA-approved corticosteroid indicated for the treatment of Duchenne**
- **Once-a-day dosing regimen taken with or without food**
- **Improved muscle strength and slowed disease progression**

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

Please see Indication & Important Safety Information for EMFLAZA in this deck

PTC THERAPEUTICS

EMFLAZA™ strategic rationale

- Aligns with mission to bring important therapies to patients with rare diseases
- Continues PTC's commitment to DMD patients and families
- Establishes U.S. commercial footprint for potential ataluren launch if FDA approved
- A second commercial DMD therapy that is complementary to ataluren

Emflaza™
(deflazacort)

PTC THERAPEUTICS

Treatment guidelines recommend corticosteroids (CS) as part of standard of care for Duchenne

The Centers for Disease Control 2010 Guidelines urge consideration of corticosteroid therapy for Duchenne patients[1]

CS are the only medication currently available that slows the decline in muscle strength and function in Duchenne muscular dystrophy regardless of mutation

CS help reduce the risk of scoliosis and stabilize pulmonary function; cardiac function may also improve

The American Academy of Neurology 2016 Guidelines urge consideration of corticosteroid therapy for Duchenne patients[2]

CS should be offered for improving strength and pulmonary function in Duchenne patients

CS improve timed motor function, reduce the need for scoliosis surgery, delay cardiomyopathy onset and loss of ambulation and increase survival

1. Bushby K, et al. Lancet Neurol. 2010: 9(1); 77-93. **2.** Gloss D, et al. Neurology. 2016; 86(5); 465-472. **3.** Data on file.

PTC THERAPEUTICS

Emflaza Cares™

A service program providing personalized support and resources to help you gain access to EMFLAZA

PTC THERAPEUTICS

Enroll in EMFLAZA*Cares*™

1 of 2

Emflaza*Cares*™

Prescription Start Form

Phone: 1-844-EMFLAZA (1-844-363-5292) **Fax:** 1-844-322-9980

Step 1: Please complete all fields on this form including the TWO prescriptions to prevent delays in processing.
Step 2: If able, obtain patient's signature for the HIPAA authorization and EMFLAZACares program.
Step 3: Fax this form, along with copies of both sides of insurance and prescription benefit cards to EMFLAZACares.

PATIENT INFORMATION

Patient First Name: _____ Patient Last Name: _____ Date of Birth: _____
Guardian/Caregiver's Name: _____ Relationship: _____
Address: _____ Apt: _____
City: _____ State: _____ ZIP: _____
Home Phone: _____ Mobile: _____
Gender: ☐ Male ☐ Female Email Address: _____
Ok to leave message: ☐ Yes ☐ No Preferred Contact Number: ☐ Home ☐ Mobile
Best time to reach me: ☐ Morning ☐ Afternoon ☐ Evening

INSURANCE INFORMATION

	Primary Insurance	Secondary Insurance
Drug Insurance		
Phone Number		
Policy Number		
Group Number		
Policyholder Name		
Rx Member ID		
Rx BIN (if applicable)		
Rx Group ID		

☐ Patient has no insurance.
☐ Send copy front/back of prescription, medical, secondary insurance cards.

Patient HIPAA Authorization and Program Participation

I have read and agree to the following HIPAA Authorization to Share Health Information and participate in the EMFLAZACares™ program. I authorize my healthcare providers and health plans to disclose personal and medical information related to my use or potential use of EMFLAZA™ (deflazacort) to PTC Therapeutics, Inc. and its agents and contractors including, but not limited to, US Bioservices specialty pharmacy and Lash/TheraCom and authorize PTC Therapeutics and its agents to use such information to: 1) determine benefit eligibility; 2) communicate with my healthcare providers and health plans about benefit, coverage and medical care; 3) provide me with support services for EMFLAZA™ (deflazacort); 4) contact me and leave messages about EMFLAZA™ (deflazacort); 5) provide me with information or materials related to EMFLAZA™ (deflazacort) or my relevant medical conditions; and 6) contact me about the EMFLAZACares™ program, which may include patient services such as education, training, nurse and pharmacy support. PTC Therapeutics will maintain the confidentiality of my personal and medical information in accordance with its privacy policy and will use this information only for the purposes described above or as permitted by law. However, I understand that personal and medical information disclosed to PTC Therapeutics pursuant to this authorization may be subject to re-disclosure, and privacy laws may no longer restrict its use or disclosure. I further understand that I may refuse to sign this authorization and that my refusal to sign this authorization will have no impact on my eligibility to receive health plan benefits or treatments from my health care providers, but I will not have access to support services from the EMFLAZACares™ program. I understand that I have the right to revoke this authorization at any time in the future, except to the extent that actions have been taken in reliance on the authorization, by submitting a written notice to PTC Therapeutics via fax to 1-908-222-7231 or by mail to PTC Therapeutics, Inc., Attention: Compliance Officer, 100 Corporate Court, South Plainfield, NJ, 07080-2449. I understand that after I have revoked my authorization, PTC Therapeutics will stop using the personal and medical information already obtained for the purposes described above. I am entitled to a copy of this authorization, which expires 10 years from the date it is signed by me (unless earlier termination is required by applicable state law). The personal, insurance and health information I have provided on this form is complete and accurate to the best of my knowledge. I will update my information promptly if any of the information reflected on this form changes by contacting EMFLAZACares™ at 1-844-363-5292.

Patient/Guardian Signature: _____
Relationship: _____ Date: _____

Please see www.EMFLAZA.com for full Prescribing Information.

Emflaza™ (deflazacort)

- **Healthcare provider completes the Prescription Start Form, which serves as the prescription**
- **Patient/guardian signs the consent section of the Prescription Start form**
- **The form is faxed to EMFLAZA*Cares*™ with copies of both sides of insurance and prescription benefit cards**
- **The Prescription Start Form can be found at Emflaza.com**

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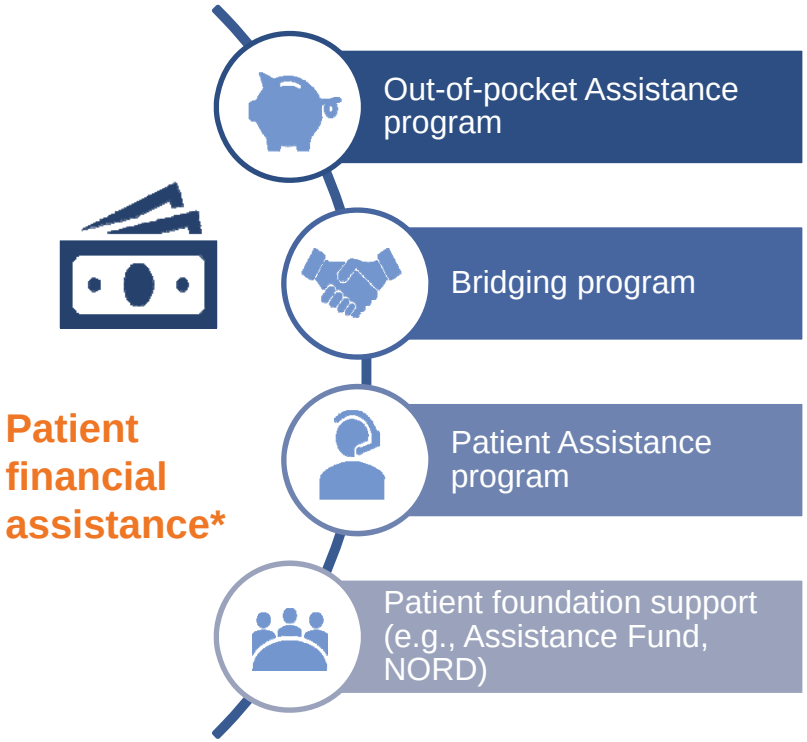
Work with an EMFLAZA*Cares*™ Case Manager

EmflazaCares Case Manage



Patient financial assistance*

**to eligible participants*

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Prescription Assistance Program

- For patients with commercial insurance and out-of-pocket costs associated with EMFLAZA who qualify, EMFLAZA*Cares*™ offers a copay assistance program

- For patients who need additional assistance or are recipients of state or federal insurance and qualify for assistance, EMFLAZA*Cares*™ will direct them to alternative funding, such as independent charitable patient assistance foundations that may be able to help with out-of-pocket costs, as determined solely by the independent charitable foundation

- For patients who have no insurance, EMFLAZA*Cares*™ offers a Free Drug Program for those who qualify

- If insurance decision is delayed, EMFLAZA*Cares*™ offers a Bridge Program so that no qualified patient has a gap in treatment

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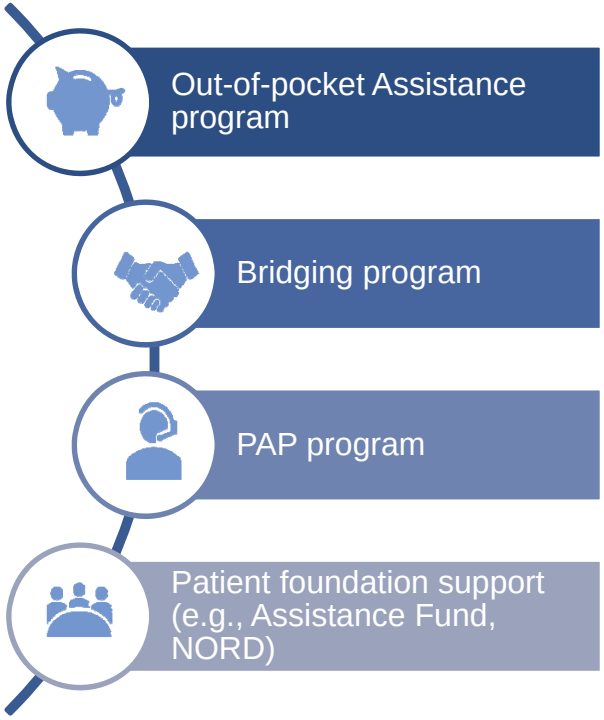
Successfully delivering EMFLAZA to patients since May

Growing number of prescriptions

- Average commercial out-of-pocket close to $0
- Current deflazacort patients eligible to bridge
- Medicaid coverage in ~45 States
- Patients in 40 states receiving drug



~700 patients have received EMFLAZA via either commercial or bridge supply

When EMFLAZA™ treatment is appropriate… Prescribing and access takes 4 steps

1. **Fill out the Prescription Start Form with signatures from both the healthcare provider and patient/guardian and fax to EMFLAZA*Cares*™**

2. **Case manager at EMFLAZA*Cares*™ works with insurers and patient assistance programs to get coverage with little to no out-of-pocket cost**

3. **EMFLAZA is delivered to the patient's home by a specialty pharmacy**

4. **Refills of EMFLAZA are automatic. EMFLAZA*Cares*™ will contact the patient/guardian to schedule a delivery**

Emflaza *Cares*™

A service program providing personalized support and resources to help you gain access to EMFLAZA

Contact EMFLAZA*Cares*™

Phone: 1-844-EMFLAZA (1-844-363-5292)
Fax: 1-844-322-9980
EMFLAZA.com

PTC THERAPEUTICS

EMFLAZA™ 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.
- 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.

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EMFLAZA™ important safety information

- 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 or 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. Killed or inactivated vaccines may be administered, but the responses cannot be predicted.
- 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.
- You may report adverse events to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

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