

**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**

WASHINGTON, D.C. 20549

**FORM 8-K/A**

**Amendment No. 1**

**CURRENT REPORT**

**Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported): **June 29, 2017**

**PTC THERAPEUTICS, INC.**

(Exact Name of Company as Specified in Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-35969**  
(Commission  
File Number)

**04-3416587**  
(IRS Employer  
Identification No.)

**100 Corporate Court**  
**South Plainfield, NJ**  
(Address of Principal Executive Offices)

**07080**  
(Zip Code)

Company's telephone number, including area code: **(908) 222-7000**

**Not applicable**

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- ☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- ☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- ☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- ☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

## EXPLANATORY NOTE

This Amendment No. 1 on Form 8-K/A (this “Amendment”) is being filed solely to include Exhibit 99.1 to the Current Report on Form 8-K filed by the Company with the Securities and Exchange Commission on June 29, 2017 (the “Original 8-K”), which exhibit was inadvertently omitted from the original filing due to a technical error.

Except as provided herein, the disclosures contained in this Amendment have not been updated to reflect events, results or developments that have occurred since the filing of the Original 8-K.

### Item 7.01. Regulation FD Disclosure.

On June 29, 2017, the Company presented at the PPMD 2017 Connect Conference. The accompanying slide deck from the presentation has been posted on the Events and Presentations page under the Investors section of the Company’s website. A copy of the slide deck is also attached to this Current Report on Form 8-K (this “Report”) as Exhibit 99.1 and is incorporated by reference into this Item 7.01.

Please refer to page 2 of the presentation attached hereto as Exhibit 99.1 for a discussion of certain forward-looking statements included therein and the risks and uncertainties related thereto.

The information set forth in or incorporated by reference into this Item 7.01, including Exhibit 99.1, shall not be deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

### Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

See Exhibit Index attached hereto.

### Signature

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Amendment No. 1 on Form 8-K/A to be signed on its behalf by the undersigned hereunto duly authorized.

**PTC Therapeutics, Inc.**

Date: June 30, 2017


By: /s/ Christine Utter

Name: Christine Utter

Title: Principal Financial Officer

## EXHIBIT INDEX

Exhibit No.	Description
99.1	Presentation - PPMD 2017 Connect Conference



**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, 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

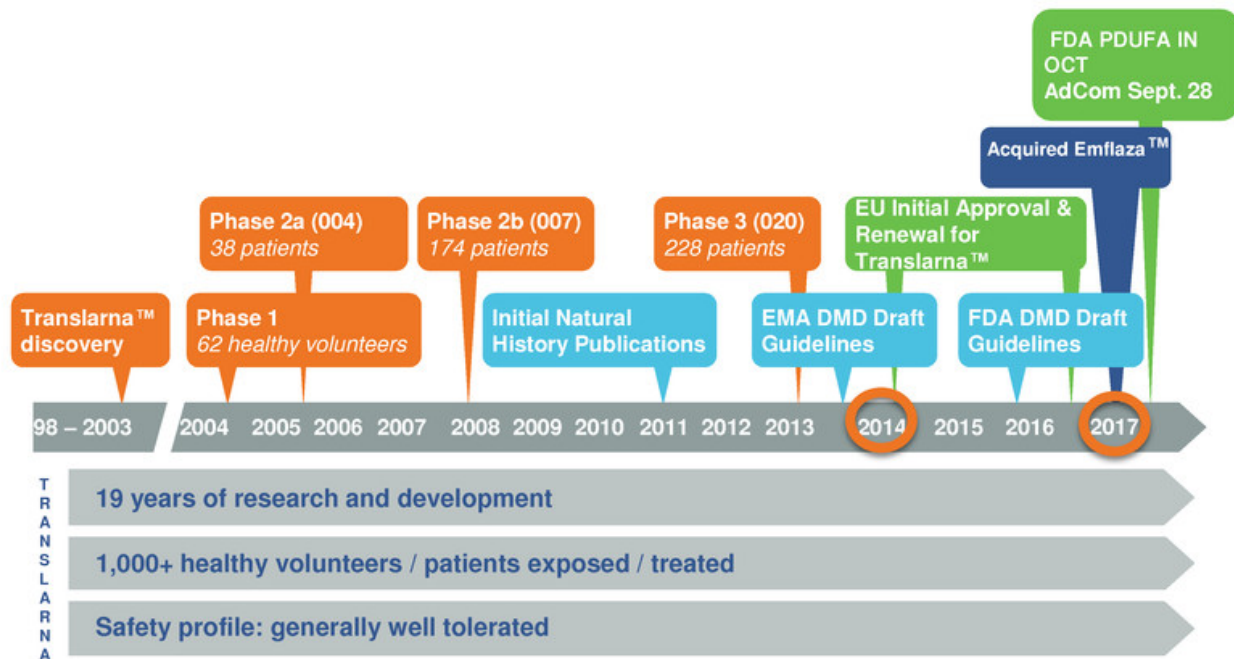


To leverage our knowledge  
of **RNA biology** to bring  
**novel therapeutics** to patients affected  
by **rare and neglected disorders**

## Commitment to patients with rare and neglected diseases



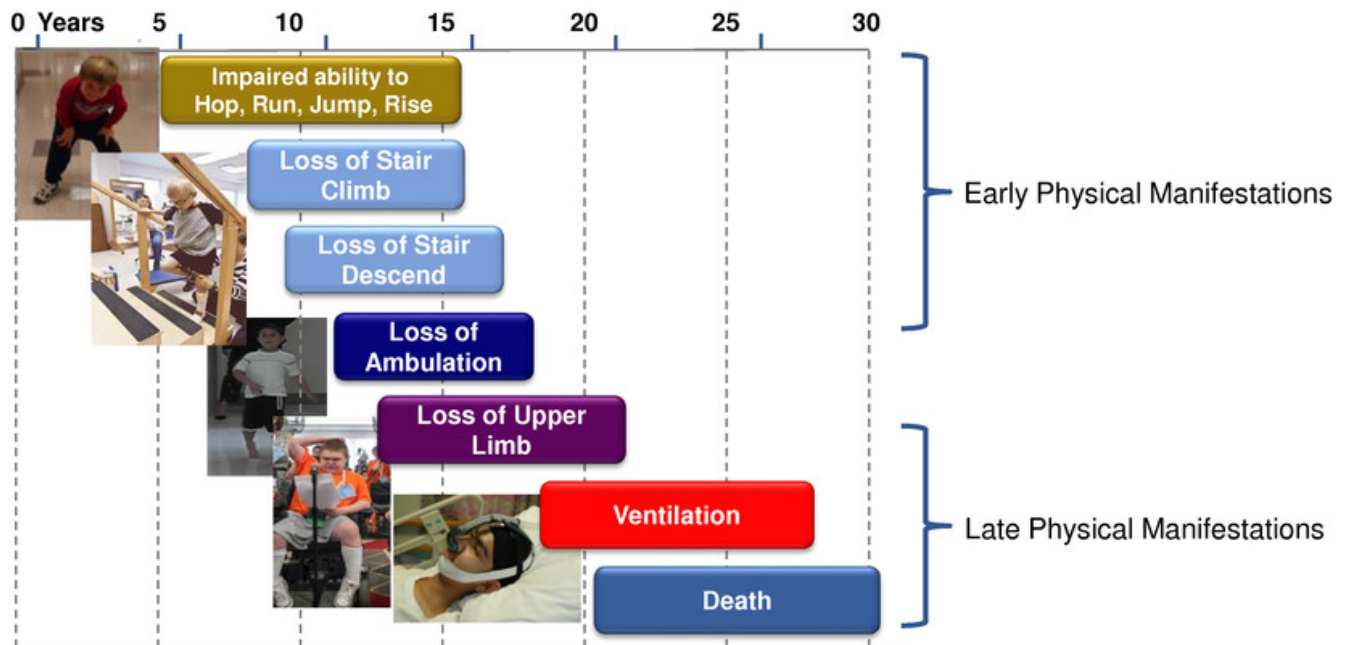
# PTC– A history steeped in commitment to DMD



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



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



# Targeting the inflammatory component of DMD

## DUCHENNE MUSCULAR DYSTROPHY

### Immune-mediated pathology in Duchenne muscular dystrophy

Amy S. Rosenberg,<sup>1\*</sup> Montserrat Puig,<sup>1</sup> Kanneboyina Nagaraju,<sup>2</sup> Eric P. Hoffman,<sup>3</sup> S. Armando Villalta,<sup>3</sup> V. Ashutosh Rao,<sup>1</sup> Lalage M. Wakefield,<sup>4</sup> Janet Woodcock,<sup>1</sup>

Immunological and inflammatory processes downstream of dystrophin deficiency as well as metabolic 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.



**Acquired in April, 2017**

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

# 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**

## 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



## 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<sup>1</sup>

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<sup>2</sup>

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.



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





# Enroll in EMFLAZACares™

1 of 2

**EmflazaCares™**

**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		
Provider 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 provider and health plan 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 Bioscience specialty pharmacy and Lash/Theracort 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 1-800-322-9980 or by mail to PTC Therapeutics, Inc., Attention: Compliance Officer, 550 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 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.

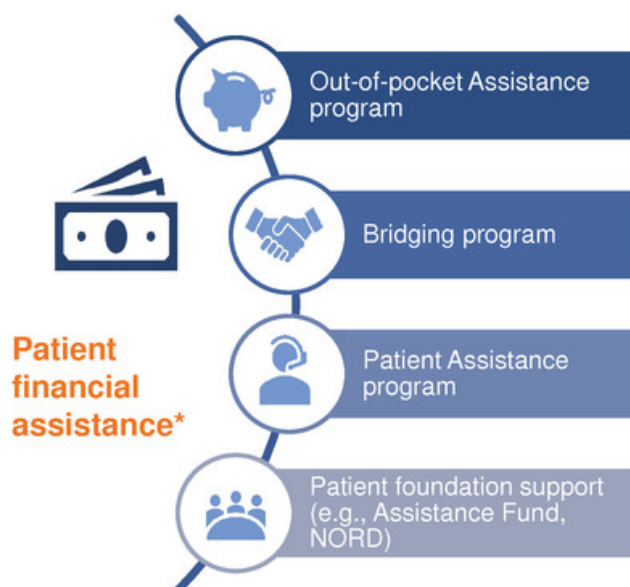
Patient/Guardian Signature: \_\_\_\_\_ Date: \_\_\_\_\_  
 Relationship: \_\_\_\_\_

Please see [www.EMFLAZA.com](http://www.EMFLAZA.com) for full Prescribing Information.

**Emflaza (deflazacort)**  
 Keep this drug out of the reach of children and away from heat.

- 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 EMFLAZACares™ with copies of both sides of insurance and prescription benefit cards
- The Prescription Start Form can be found at [Emflaza.com](http://Emflaza.com)

# Work with an EMFLAZACares™ Case Manager



*\*to eligible participants*

# Prescription Assistance Program



- For patients with commercial insurance and out-of-pocket costs associated with EMFLAZA who qualify, EMFLAZACares™ offers a **copay assistance program**
- For patients who need additional assistance or are recipients of state or federal insurance and qualify for assistance, EMFLAZACares™ 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, EMFLAZACares™ offers a **Free Drug Program** for those who qualify
- If insurance decision is delayed, EMFLAZACares™ offers a **Bridge Program** so that no qualified patient has a gap in treatment

# 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



Out-of-pocket Assistance program



Bridging program



PAP program



Patient foundation support (e.g., Assistance Fund, NORD)



**~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 EMFLAZACares™



2

Case manager at EMFLAZACares™ 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. EMFLAZACares™ will contact the patient/guardian to schedule a delivery





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**



## 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.





## 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](http://www.fda.gov/medwatch).

EMFLAZA™ and EMFLAZACares™ are registered trademarks of PTC Therapeutics, Inc.







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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**



