



FREQUENTLY ASKED QUESTIONS FOR PATIENTS AND FAMILIES

Will my insurance cover EMFLAZA® (deflazacort)?

Each patient may have a different insurance plan. In order to determine the insurance benefit for any patient, a completed EMFLAZA Prescription Start Form must be signed and submitted to PTC *Cares*™. This form is available at [PTCCares.com](https://www.ptccares.com).

After the EMFLAZA Prescription Start Form is submitted, your PTC *Cares* Case Manager will work with your insurance plan on your behalf to verify your insurance benefits.

Insurance providers may require more information from your physician about the medical necessity for EMFLAZA. Your PTC *Cares* Case Manager will work directly with your healthcare provider to submit all information necessary to answer questions from your insurance provider. We expect that going through this process with your insurance company may vary from days to several weeks, depending on your insurance plan.

After insurance coverage is approved, how long will the prescription continue?

The length of time the prescription is active will vary by insurance provider. Most insurance providers authorize prescriptions for 6 months to 1 year. Your PTC *Cares* Case Manager will monitor your prescription and will apply for reauthorization before the prescription expires.

How much will I have to pay for my prescription?

Each patient may have a different insurance plan. The specific benefits of that plan determine the amount paid by your insurance provider and the amount that you are responsible to pay.

In order to determine the insurance benefit for any patient, a completed EMFLAZA Prescription Start Form must be submitted to PTC *Cares*.

At that point, your PTC *Cares* Case Manager will work with your insurance plan on your behalf to verify your insurance benefit to determine coverage, including any cost assigned to the patient. The type of out-of-pocket expenses may include a copay, coinsurance, and/or deductible.

PTC has created comprehensive patient assistance programs to provide you with the financial support needed to ensure access to EMFLAZA.

Your PTC *Cares* Case Manager will determine which patient assistance program is right for you, based on your type of insurance.



PTC is committed to minimizing or eliminating patient out-of-pocket expenses.

Private or Commercial Insurance

If you have private or commercial insurance, your PTC *Cares*[™] Case Manager will automatically enroll you into a program that will eliminate your cost in most cases. Some state regulations mandate a patient pays a small amount (such as \$10) each month. In those cases, patients may have a small monthly payment.

Public or Government Insurance

If your insurance is provided by the government (like Medicaid or Medicare), you may have to pay a certain amount out-of-pocket. In these cases, your PTC *Cares* Case Manager will refer you to a third-party patient foundation that will work with you to provide financial assistance based on your eligibility.

No Insurance

If you do not have insurance coverage, your PTC *Cares* Case Manager will offer EMFLAZA[®] (deflazacort) free of charge if you meet eligibility criteria.

REMEMBER: In order for your PTC *Cares* Case Manager to help, a completed Prescription Start Form must be submitted to the PTC *Cares* program. The form can be found on PTCCares.com.

If a patient is already on deflazacort, including as part of a clinical trial, will he be required to discontinue deflazacort or switch to prednisone because of insurance requirements?

PTC is committed to continuity of care. We have a Bridge Program in place so that any patient currently taking deflazacort can continue on EMFLAZA while waiting for insurance verification of coverage for EMFLAZA. In order to start this process, there must be a Prescription Start Form submitted. This form can be found at PTCCares.com.

What is the difference between EMFLAZA and prednisone?

EMFLAZA is approved by the US Food and Drug Administration (FDA) for Duchenne muscular dystrophy in patients 5 years and older. Prednisone is not FDA approved for Duchenne muscular dystrophy.

Can you explain weight-based pricing?

The recommended oral dosage of EMFLAZA is approximately 0.9 mg/kg/day once daily. In other words, the dose of EMFLAZA is calculated based upon the weight of the patient. Once a dose is determined, the necessary tablet or suspension strength(s) is used to reach that dose.



Will getting insurance coverage of EMFLAZA® (deflazacort) cause my insurance premiums to rise?

While it is impossible for us to predict what factors influence healthcare premiums, the impact of EMFLAZA to an insurance plan's budget is negligible. It is not expected that premiums for prescription coverage will increase due to EMFLAZA.

How will you supply EMFLAZA to patients who have received imported deflazacort?

PTC has an EMFLAZA Bridge Program in place to provide deflazacort to anyone who has imported deflazacort. Under the EMFLAZA Bridge Program, patients will receive EMFLAZA free of charge while they are waiting for insurance approval.

REMEMBER: To get started, make sure that both you and your healthcare provider sign the Prescription Start Form and submit the form to PTC Cares™. This form is available on [PTCCares.com](https://www.ptccares.com).

How do I find out the status of my approval?

Your PTC Cares Case Manager will keep you informed of the progress of your application. You may call 1-844-4PTCCARES (1-844-478-2227) for an update.

How will I get EMFLAZA?

Once approved for EMFLAZA, the pharmacy (US Bioservices) will call you to arrange delivery. EMFLAZA will be delivered to your home, office, or other location of your choice. They will also call you each month to manage your refills and confirm delivery prior to shipments.

How much EMFLAZA will be shipped to me?

Generally, insurance companies authorize shipments of a 30-day supply. However, some insurers will approve a 90-day supply. You may discuss the shipment approved by your insurance company with your Case Manager.

How do I order refills?

The pharmacy will call you to schedule your refill. You can also call 1-844-4PTCCARES (1-844-478-2227) if you have any questions about your refills.



6 mg | 18 mg | 30 mg | 36 mg tablets
22.75 mg/mL oral suspension



What if I don't want the services of PTC Cares™?

If you choose not to sign the Prescription Start Form and enroll in PTC Cares, your prescription will go directly to the pharmacy (US Bioservices). The pharmacy will determine your insurance coverage but will not be able to help you with appeals if coverage is denied. They will not be able to enroll you in any of the programs PTC has in place to reduce out-of-pocket expenses.

Where can we get more information?

More information can be found on [PTCCares.com](https://www.ptccares.com). Case Managers are able to assist you and can be contacted at 1-844-4PTCCARES (1-844-478-2227). They are available 8:00 AM – 8:00 PM EST, Monday – Friday.



INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA[®] (deflazacort)

INDICATION

EMFLAZA[®] is indicated for the treatment of Duchenne muscular dystrophy in patients 5 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.

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



INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA[®] (deflazacort) (cont.)

- **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](tel:1-866-562-4620) or email at usmedinfo@ptcbio.com.

You may also report adverse events directly to FDA at [1-800-FDA-1088](tel:1-800-FDA-1088) or www.fda.gov/medwatch.