For patients living with Duchenne muscular dystrophy, early, long-term treatment can HELP HIM CREATE HIS STORY

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.

Please see Indication and Important Safety Information on pages 14 and 15 and accompanying full Prescribing Information.
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To learn more about EMFLAZA visit www.EMFLAZA.com
EARLY TREATMENT OF DUCHENNE MUSCULAR DYSTROPHY IS IMPORTANT

Duchenne muscular dystrophy (DMD) is a rare disease that causes muscles to get weaker little by little over time. Duchenne is caused by a genetic mutation that affects up to 1 in every 3,600-6,000 live male births.¹⁻⁴

Early diagnosis and the start of a coordinated treatment plan, which includes physical therapy with an emphasis on passive stretching of the muscles surrounding the knee, is important to keeping muscle strength for as long as possible at every stage of the disease.⁵

Adding corticosteroids to therapy has been shown to be effective in keeping muscle strength.⁶ Ask your healthcare provider about including EMFLAZA in your child’s treatment plan.

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YOU SHOULD KNOW THAT CORTICOSTEROIDS:

- Are medicines that relieve inflammation in the body\(^7\)
- Are recommended by the American Academy of Neurology (AAN) and the U.S. Centers for Disease Control and Prevention (CDC) guidelines as a building block of treatment of Duchenne muscular dystrophy\(^1,8\)
- Must be given as directed by your prescriber
- May, like certain drugs, cause drug interactions. Tell your healthcare provider what other medicines your child is taking
- Should not be stopped without asking your prescriber first

Talk to your provider about CORTICOSTEROIDS

Discuss the benefits of corticosteroids with your healthcare team. Long-term corticosteroid treatment has been shown effective in maintaining muscle function but not in recovering function once it is lost. That's why early intervention is important.\(^9\)
MAKE IT A TEAM EFFORT
A strong team is key to effective treatment of Duchenne muscular dystrophy. This team includes your primary care provider and staff but also you, your family, friends, or other caregivers who offer valuable support throughout the treatment journey.

Be sure to help your child stick to a healthy lifestyle: a well-balanced diet, watching sugar and salt, and adding vitamin supplements when needed.

Get your team to work early and be sure you keep them involved, particularly your neurologist, to meet the needs of your child.

Assemble a Team for Support and Care
In addition to corticosteroids and working closely with your neurologist, it is important to seek care from a range of specialists from various disciplines. They can help carefully manage a patient living with Duchenne, including monitoring any potential side effects.
EMFLAZA CAN BE AN IMPORTANT PART OF YOUR DUCHENNE TREATMENT PLAN

EMFLAZA® (deflazacort) is a corticosteroid that works through receptors in the body to effectively inhibit inflammation. Clinical studies demonstrated that, compared to placebo, EMFLAZA:

- Improved the muscle strength of Duchenne patients.
- Slowed the loss of muscle strength over time.
- Improved time to stand up, climb 4 stairs, and walk 30 feet.

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ARE THERE ANY SIDE EFFECTS WITH EMFLAZA?

There is a risk of side effects with any drug. Common side effects with EMFLAZA include:\n\n- Facial puffiness (sometimes called Cushingoid appearance)
- Increased weight
- Increased appetite
- Upper respiratory tract infection
- Cough
- Frequent daytime urination
- Colds
- Unwanted hair growth
- Central obesity
- Patchy reddening of the skin
- Irritability
- Runny nose
- Abdominal discomfort

Please seek treatment immediately and tell your healthcare provider if you have any of these symptoms or any other side effects.

If you have a medical question regarding the use of EMFLAZA, please ask your healthcare provider. Discuss your child’s immunization schedule with your provider before starting EMFLAZA.

Drug Interactions

When starting any new treatment, it is important to know about any potential interactions that may occur. That’s why it is so important to let your healthcare provider know about all the medications your child is taking, including over-the-counter and prescription medicines, dietary supplements, and herbal products.

Dosage adjustment may be needed during treatment.\(^{13}\)
YOUR CHILD TAKES EMFLAZA JUST ONCE A DAY

EMFLAZA Is Available in Tablet Form

• EMFLAZA can be taken with or without food
• Tablets can be given whole or crushed, mixed with applesauce, and taken immediately
• Prescribed dosage strengths can be achieved by taking multiple tablets in combination, rounding up to the nearest dose

EMFLAZA® (deflazacort) tablets (Recommended dose approximately 0.9 mg/kg/day)

4 tablet strengths

6 mg 18 mg 30 mg 36 mg

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To learn more about EMFLAZA visit EMFLAZA.com
EMFLAZA Is Available in Liquid Form

- Round up to the nearest tenth of a milliliter (mL)
- Available in 13 mL bottle, oral dispenser included
- Mix EMFLAZA well with 3-4 oz. of juice or milk and administer immediately
- Do not mix EMFLAZA with grapefruit juice
- Replace cap tightly on bottle and clean oral dispenser after each use
- Discard any unused EMFLAZA after 1 month of first opening the bottle
- EMFLAZA should not be taken with grapefruit or grapefruit juice

**EMFLAZA oral suspension**
(Recommended dose approximately 0.9 mg/kg/day)

![EMFLAZA bottle](image)

22.75 mg/mL
Bottle not shown at actual size.

Important Dosing Considerations

Patients should not stop taking EMFLAZA abruptly or without first checking with their healthcare provider as there may be a need for gradual dose reduction to decrease the risk of adrenal insufficiency.

- Dosage of EMFLAZA must be decreased gradually if the drug has been administered for more than a few days
- Weight gain should be reassessed by a physician to determine appropriate dosing
PTC Cares™ MAKES IT EASY TO START THERAPY

PTC Cares is a support program dedicated to helping patients, caregivers, and prescribers understand the prescription process, financial assistance, and insurance information. Personal case managers and the specialty pharmacy work together to assist you through every step of the treatment journey.

Prescription written
You and your healthcare provider complete and submit a Prescription Start Form, which acts as a prescription and consent. This starts your PTC Cares support and gives you access to PTC products.

Welcome call
Your PTC Cares case manager will call to confirm your insurance and other information, assist with questions you may have about the medication or your medical condition, and discuss the process of getting treatment.

Insurance benefits investigation
Your PTC Cares case manager will conduct a benefits investigation and offer prior authorization support.

Explanation of patient assistance
Your PTC Cares case manager will explain insurance benefits, Patient Assistance Programs, and any available resources, like copay assistance or other forms of support, to help you start your EMFLAZA® (deflazacort) prescription.

Specialty pharmacy delivery
Your PTC Cares case manager will call to confirm your delivery information. You can request a tracking number so you can follow your delivery during the shipping process.
NEED A REFILL?
Your PTC Cares team will confirm your prescription receipt and call you to schedule the next refill to ensure it arrives on time.

Ongoing personal support assures on-time refills

Stay connected to support

Call
1-844-4PTC-CARES
(1-844-478-2227)
Speak with a PTC Cares case manager 8 AM – 8 PM EST Monday – Friday.

Visit
PTCCares.com
For information and to download the Prescription Start Form.
ADVOCACY GROUPS ARE AVAILABLE FOR SUPPORT

We know that there is more to treatment than just taking care of symptoms. You and your family also have your own individual interests and support needs. Advocacy groups play an important role in helping you connect and bond with other people affected by Duchenne as well as giving patients and their families a public voice. Advocacy groups in your community include but aren’t limited to:

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FIND A CERTIFIED DUCHENNE CARE CENTER

Parent Project Muscular Dystrophy (PPMD) Certified Care Centers provide comprehensive care and services specifically for people living with Duchenne muscular dystrophy. The goal of these centers is to make comprehensive care available and accessible to as many patients with Duchenne and their families as possible. The centers that have been certified by PPMD can be found on their website: parentprojectmd.org/carecenters.

The Muscular Dystrophy Association (MDA) has a network of 200 specialized clinics across the United States and Puerto Rico, each with a team of healthcare professionals all at one location. Find a clinic near you by visiting mda.org/locate or by calling (800) 572-1717.
INDICATION & IMPORTANT SAFETY INFORMATION
FOR EMFLAZA® (deflazacort)

INDICATION

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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:** 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** or email at usmedinfo@ptcbio.com.

You may report adverse events directly to FDA at **1-800-FDA-1088** or **www.fda.gov/medwatch**.
SEE HOW EMFLAZA CAN HELP

• EMFLAZA® (deflazacort) has been shown to help slow muscle weakness in children with Duchenne muscular dystrophy.

• EMFLAZA is a convenient, once-daily medication available in pill or liquid form. It can be taken with or without food.

• PTC Cares™ is your go-to patient service program for questions and support regarding treatment and prescription access.

Call 1-844-4PTC-CARES (1-844-478-2227)
8 AM – 8 PM EST Monday – Friday

To learn more about how EMFLAZA may help your child, visit EMFLAZA.com