



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

The Duchenne treatment journey starts here

EMFLAZA[®] is the first and only corticosteroid indicated to treat Duchenne muscular dystrophy.

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.

Please see additional Important Safety Information throughout and full [Prescribing Information](#).



Diagnosis and treatment

Duchenne muscular dystrophy (DMD) is a rare disease caused by a genetic mutation that affects up to 1 in every 3600 to 6000 male births.^a Duchenne causes muscles to get weaker over time. Once muscle function is lost, it cannot be regained. That's why it's important to seek treatment options for Duchenne as early as possible. ***On average, signs and symptoms of Duchenne appear when a child is between 2 and 3 years old.***

^aAs referenced in a review article by Bushby K et al. *Lancet Neurol.* 2010;9(1):77.

What you should know about corticosteroids

Corticosteroids are medicines that relieve inflammation in the body. Updated guidelines published by the DMD Care Considerations Working Group recommend starting corticosteroid treatment early for long-term benefits. The US Centers for Disease Control and Prevention (CDC) guidelines recommend a corticosteroid as part of the treatment plan in fighting Duchenne muscular dystrophy. Physical therapy and treatment with corticosteroids are the cornerstones of DMD treatment and should continue even after loss of ambulation.

The DMD Care Considerations guidance was created by a group of experts to determine appropriate and necessary assessments and interventions using clinical scenarios. These guidelines reflect the opinions of the panel of experts based on their understanding and application of the existing scientific literature.

Before beginning treatment with corticosteroids, you should discuss the benefits and risks of treatment with your loved one's healthcare provider. It's also important to discuss your child's treatment plan with other members of his care team.



DMD Care Considerations Article Objective

The DMD Care Considerations guidelines were developed by an expert steering committee from various backgrounds. This group of clinical experts is called the Care Considerations Working Group (CCWG). With improved patient survival and a shift in anticipatory diagnostic and therapeutic strategy, the goal of the CCWG was to update the 2010 Care Considerations guidelines to improve patient care by addressing the needs of patients with prolonged survival and by providing guidance in the advancement and assessment of interventions as well as emerging genetic and molecular therapies for DMD.

Birnkrant DJ, Bushby K, Bann CM, et al; DMD Care Considerations Working Group. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. *Lancet Neurol*. 2018;17(3):251-267.

EMFLAZA® (deflazacort) preserves muscle strength

EMFLAZA® is a corticosteroid that can be prescribed to children with Duchenne muscular dystrophy as young as 2 years of age.

In a clinical trial of 196 boys aged 5 to 15 with Duchenne muscular dystrophy, the effectiveness and safety of EMFLAZA were compared with placebo (sugar pills).

EMFLAZA improved muscle strength at 12 weeks compared with placebo.

**EMFLAZA was numerically favored in timed measures
of patient function in comparison with placebo**

These findings were not considered statistically significant. This means that because the two groups studied were not large enough, the results could have occurred by chance.



***Time to
stand up***



***Time to
climb 4 stairs***



***Time to
walk 30 feet***

IMPORTANT SAFETY INFORMATION (cont'd)

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.

Common Side Effects of EMFLAZA® (deflazacort)

As with any drug, EMFLAZA does have a risk of side effects. Please seek treatment immediately and tell your healthcare provider if your child has any of these symptoms or any other side effects.

Side Effects	EMFLAZA 0.9 mg/kg/d (n=51) % at 12 weeks	Placebo (n=50) % at 12 weeks ^a
Facial puffiness or Cushingoid appearance	33%	12%
Weight increased	20%	6%
Increased appetite	14%	2%
Upper respiratory tract infection	12%	10%
Cough	12%	6%
Frequent daytime urination	12%	2%
Common cold	10%	6%
Unwanted hair growth	10%	2%
Central obesity	10%	4%
Skin redness	8%	6%
Irritability	8%	4%
Runny nose	8%	0%
Abdominal discomfort	6%	2%

^aAt 12 weeks placebo patients were re-randomized to receive either EMFLAZA or an active comparator.

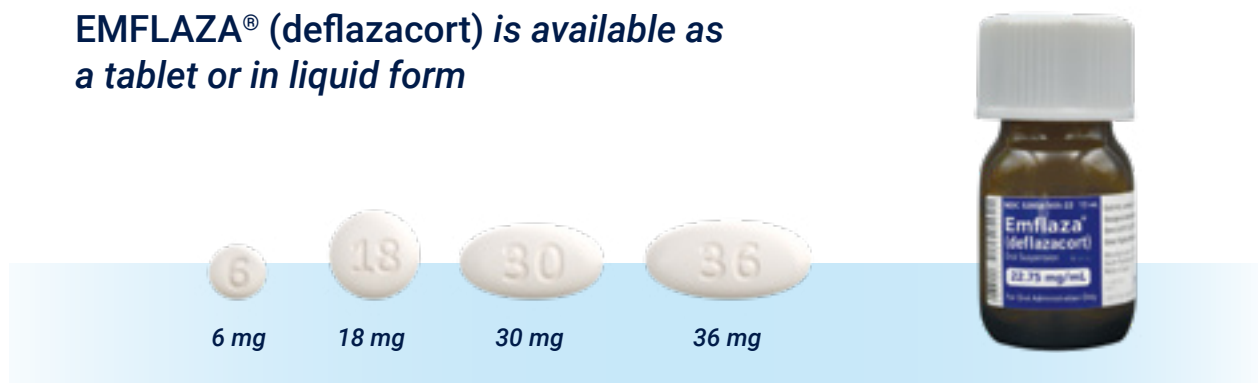
IMPORTANT SAFETY INFORMATION (cont'd)

- **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.

Please see additional Important Safety Information throughout and full [Prescribing Information](#).

Convenient once-daily dosing

EMFLAZA® (deflazacort) is available as
a tablet or in liquid form



Tablets not shown at actual size.

(Recommended dose is approximately 0.9 mg/kg/day)

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

IMPORTANT SAFETY INFORMATION (cont'd)

- **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.

*For patients with Duchenne muscular dystrophy,
accurate dosing makes a difference*

Weight range (kg)	Weight range (lbs)	0.9 mg/kg Daily dose (as calculated)	Daily dose strengths (mg) ^a	
10-13	22-29	9-11.7	6 6	(12 mg)
14-20	31-44	12.6-18	18	(18 mg)
21-26	46-57	18.9-23.4	6 18	(24 mg)
27-33	59-73	24.3-29.7	30	(30 mg)
34-40	75-88	30.6-36	36	(36 mg)
41-46	90-101	36.9-41.4	6 36	(42 mg)
47-53	103-117	42.3-47.7	6 6 36	(48 mg)
54-60	119-132	48.6-54	18 36	(54 mg)
61-66	134-145	54.9-59.4	30 30	(60 mg)
67-73	147-161	60.3-65.7	30 36	(66 mg)

^aPlease visit EMFLAZA.com for oral suspension dosing considerations.

- Mix liquid EMFLAZA well with 3 to 4 oz of juice or milk and give immediately
- EMFLAZA should not be taken with grapefruit or grapefruit juice
- Store at room temperature
- Shake well before each use
- Clean oral dispenser and replace cap tightly after each use
- Discard any unused EMFLAZA after 1 month of first opening the bottle

Important Dosing Considerations

Patients should not stop taking EMFLAZA abruptly or without first checking with their healthcare provider. You may need to gradually reduce the dose rather than stop taking it altogether.

- The dosage of EMFLAZA must be decreased gradually if the drug has been administered for more than a few days
- Please talk to your child's **healthcare provider** before making any dose adjustments

Access and affordability

From financial assistance to navigating the prescription process, PTC *Cares*™ is here for you.



Getting Started

Once your doctor has written a prescription for EMFLAZA® (deflazacort) and submitted a Prescription Start Form (PSF), you are eligible to access PTC products and receive support from PTC *Cares*.



Welcome Call

Once we receive the PSF form and obtain your consent, a PTC *Cares* case manager will call you to confirm all information, answer any questions, and discuss the process and next steps to obtain EMFLAZA®.



Patient Assistance

Your PTC *Cares* case manager will call you to explain insurance benefits, Patient Support Services, and any available resources such as copay assistance or other forms of support to assist in obtaining PTC *Cares* products. PTC *Cares* may discuss financial assistance with patients who qualify.

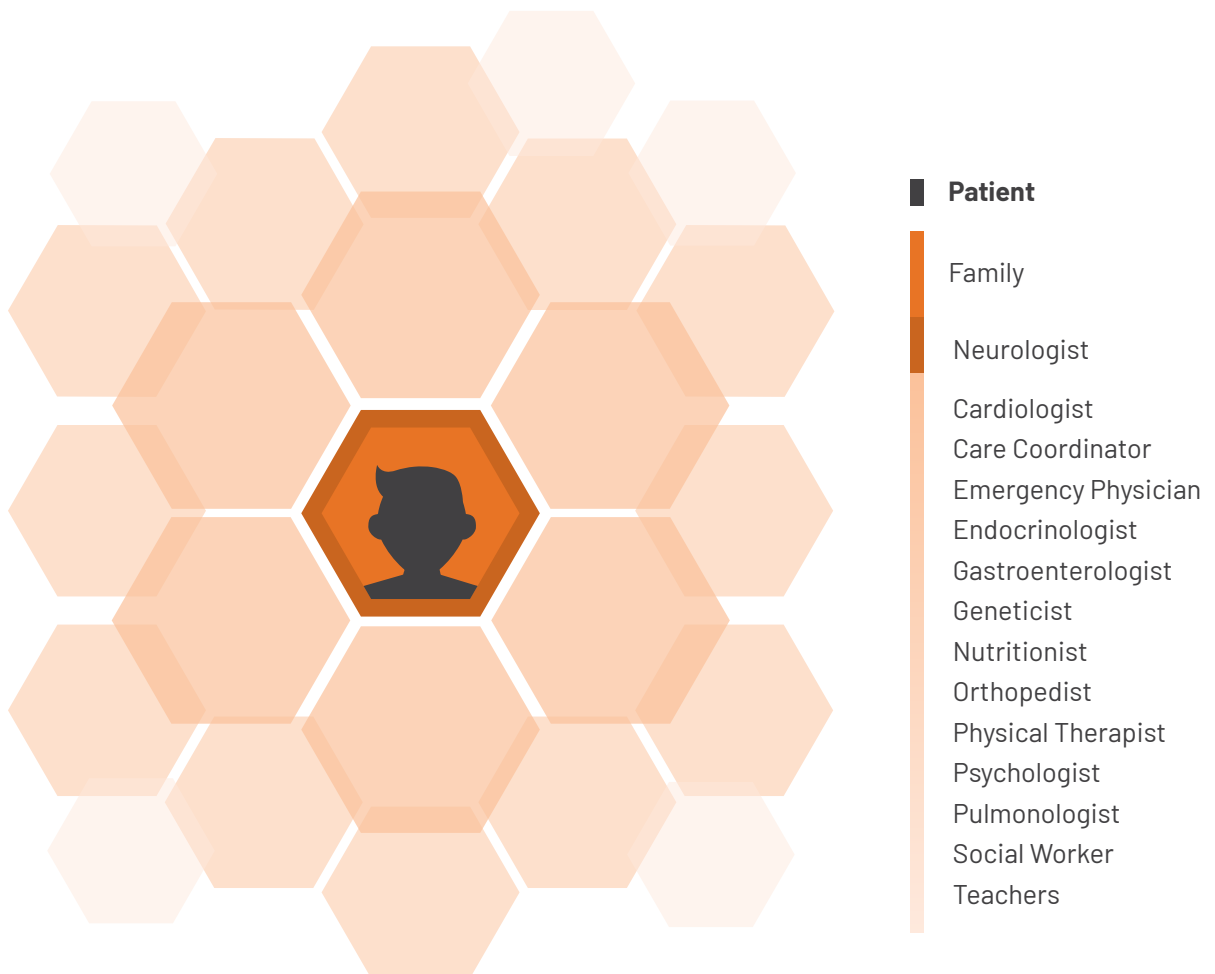
Visit www.ptccares.com or call 1-844-4PTC-CARES (1-844-478-2227) and speak with a PTC Cares case manager 8 AM to 8 PM ET, Monday to Friday.



Caring for a loved one with Duchenne takes a team. And it starts with you.

You are the most important part of your child's care team. You see him every day. And you know him best, so you'll be the first to notice any changes in his health.

But you are not alone in caring for your child. You have a larger team supporting you. This team may include family and friends. In addition to your child's primary healthcare provider, it may also include a group of healthcare specialists from different disciplines, such as a neurologist, a cardiologist, and many others.



Find a support group in your community

There are programs and organizations that were created to help families living with Duchenne. Take a moment to check out the ones below:

PTC Peer Navigator Program

This program connects you with other families familiar with DMD who can provide you with support and guidance based on their personal journey. To be connected with a PTC Peer Navigator, email peernavigator@ptcbio.com or call **1-866-282-5873**.

CureDuchenne

This nonprofit organization supports Duchenne-related research and features events, workshops, and webinars on their site. Visit www.cureduchenne.org to learn more.

Muscular Dystrophy Association (MDA)

There are more than 150 MDA care centers and affiliates in the United States and Puerto Rico with each location home to a full team of healthcare professionals. To find a specialized MDA clinic near you, visit www.MDA.org/locate or call **1-800-572-1717**.

Parent Project Muscular Dystrophy (PPMD) Certified Duchenne Care Centers

PPMD centers provide comprehensive care and services specifically for people living with Duchenne muscular dystrophy. These centers strive to provide accessible and comprehensive care to as many patients and families as possible. Centers certified by PPMD can be found at www.parentprojectmd.org/carecenters.

PTC does not warrant, whether expressed or implied, that the list of centers is accurate or complete. PTC does not recommend or endorse any center, hospital, or healthcare professional included above for any purpose, nor is the list to verify a center's, hospital's, or specific healthcare professional's credentials or qualifications. It is your responsibility to select a center, hospital, and/or healthcare professional appropriate for your needs, and you assume full responsibility for your interactions with any center, hospital, or healthcare professional you contact through the center list. PTC is not responsible for the medical care or advice given by any center, hospital, or healthcare professional you choose to contact.

Take the time to have a discussion with your child's healthcare provider.

It's important to communicate early and often with your child's healthcare provider and care team. Below, we've provided some helpful questions to ask at his next appointment.

- *Other than my pediatrician, are there any specialists we should see?*
- *How often should we visit the doctor?*
- *What is a care plan, and can we create one?*
- *Are there medications that can help my child with Duchenne?*
- *How will I know if a medication is helping my child?*
- *What if my child experiences side effects from medication?*
- *Are there resources available to help me and my child?*

[illegible]

EMFLAZA[®] (deflazacort) frequently asked questions

Below are answers to common questions about EMFLAZA[®] and starting treatment.

Q: Is there a specific age range for which EMFLAZA may be used?

A: EMFLAZA is approved for use in patients with Duchenne muscular dystrophy who are 2 years of age and older.

Q: What do I need to know about EMFLAZA and my child's immunizations?

A: Before taking EMFLAZA, it is important that your child has received all immunizations according to immunization guidelines. For certain vaccines (live or live-attenuated vaccines), your child will need to wait 4 to 6 weeks prior to starting EMFLAZA.

Q: Can I get EMFLAZA at my local pharmacy where I get all my other prescriptions?

A: EMFLAZA is not available at local pharmacies. It is a prescription medication available only through a specialty pharmacy. EMFLAZA is delivered right to your door through a PTC Therapeutics specialty pharmacy partner.

Q: How much does EMFLAZA cost?

A: Once your healthcare provider has made the decision to prescribe EMFLAZA, your specialty pharmacy will do a benefit verification with your insurance company to identify your out-of-pocket costs related to EMFLAZA. Call PTC Cares[™] at **1-844-4PTC-CARES** (1-844-478-2227) to learn about your eligibility options and assistance.

Q: If I have questions about how to take EMFLAZA, where can I get answers?

A: Please review the complete Instructions for Use included in each EMFLAZA shipment. The Prescribing Information is also enclosed in the pocket of this brochure. If you have trouble finding what you need, you can also contact your loved one's healthcare provider or PTC Cares for assistance.

Q: Can my child stop taking EMFLAZA at any time?

A: Do not stop giving your child EMFLAZA without first consulting his healthcare provider. EMFLAZA should be taken once a day, every day, unless otherwise directed by a healthcare provider.

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- **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 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 the FDA at **1-800-FDA-1088** or www.fda.gov/medwatch.

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To learn more about treatment with EMFLAZA[®],
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