

EMFLAZA (deflazacort) FAQ

September 2025

- **What is EMFLAZA (deflazacort)?**
 - EMFLAZA (deflazacort) is an oral [corticosteroid](#) that works by reducing inflammation which can help protect muscles from damage. Deflazacort is the generic form and [EMFLAZA](#) is the brand name.
- **Who is eligible?**
 - [EMFLAZA](#) is available for people ages 2 years and up with a diagnosis of Duchenne muscular dystrophy.
 - Deflazacort is available for people ages 5 years and up with a diagnosis of Duchenne muscular dystrophy.
- **What is the difference between EMFLAZA and deflazacort?**
 - EMFLAZA is the brand name and deflazacort is the generic form. Generic and brand medications have the same active ingredient, but are made by different companies and may have different “inert” or inactive ingredients. You can learn more about the differences between generic and brand name [here](#).
 - Deflazacort currently has 2 manufacturers:
 - [Cranbury Pharmaceuticals](#) (Tris Pharma) – oral suspension (liquid) only
 - [Aurobindo Pharma](#) -6 mg, 18 mg, 30 mg, and 36 mg tablets only
 - EMFLAZA is manufactured by [PTC Therapeutics](#).
- **Important things to know:**
 - Because EMFLAZA & deflazacort are [steroids](#), you should never stop taking or change the amount taken, without first checking with your healthcare provider.
 - It's important to understand the potential side effects of any treatment. Be sure to talk with your neuromuscular team about risks, benefits, and precautions before starting any medications.
- **What are the possible side effects of EMFLAZA (deflazacort)?**
 - Common side effects include:
 - Cushingoid features (round face, increased fat around base of neck), increased weight, increased appetite, changes in endocrine function, immunosuppression, effects on bone health, ophthalmic (eye) effects, effects on growth, and high blood pressure.
- **What precautions are needed while taking EMFLAZA (deflazacort)**
 - Because EMFLAZA and deflazacort are [steroids](#), it suppresses the adrenal glands. Because of this, suddenly discontinuing EMFLAZA or deflazacort can be extremely dangerous and cause [adrenal crisis](#), which can be life threatening. Talk to your neuromuscular doctor about what to do if you run out of medication, or are too sick to take your medication. Learn more about adrenal suppression, adrenal crisis, when stress dosing is needed, and more in [PJ's Protocol](#).
- **How does my child get access to EMFLAZA (deflazacort)?**
 - It's important to discuss with your neuromuscular provider which steroid is right for your child. If you and your neuromuscular team decide on Emflaza (deflazacort) your doctor

will write a prescription and send it to a specialty pharmacy that will mail the medication to your home.

- If you and your neuromuscular team decide that the brand name EMFLAZA is the best choice for you/your child, your doctor will include a [“Dispense as Written” \(DAW\)](#) code on the prescription so it may not be substituted for a generic.
- Learn more about access & coverage resources for EMFLAZA (deflazacort) [here](#).

- [Learn more about clinical trials of EMFLAZA \(deflazacort\)](#)

- **Video Library**

- [Webinar: EMFLAZA Landscape in 2024](#)
- [Webinar: Navigating Steroid Choices - Understanding the Process, Pathways, and Policies](#)
- [PPMD's 2021 Annual Conference: In the Pipeline: Reducing Inflammation - PTC Therapeutics](#)
- [PPMD's 2019 Conference: Inflammation and Fibrosis](#)