WEIGHT-BASED DOSING COUDE FOR STARTING, OR

STAYING ON TREATMENT

Mikey, Age 9

Actual EMFLAZA patient

Reid

INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA® (deflazacort)

What is EMFLAZA® (deflazacort) used for?

EMFLAZA is a prescription medicine used to treat Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

When should I not take EMFLAZA?

Do not use if you have had hypersensitivity, including allergic reactions, to deflazacort or any of the inactive ingredients.

Please see <u>Indication and Important Safety Information</u> throughout and accompanying full <u>Prescribing Information</u>.



AS YOUR SON GROWS, WEIGHT-BASED DOSING ADJUSTMENTS MAY BE NEEDED

Dosing considerations

Your child should not stop taking EMFLAZA abruptly or without checking with their healthcare provider. You may need to gradually reduce the dose rather than discontinue treatment altogether.

The dosage of EMFLAZA must be decreased gradually if your child has taken it for more than a few days

Weight Range	kg	10-13	14-20	21-26	27-33	34-40	41-46	47-53	54-60	61-66
	lbs	22-29	31-34	46-57	59-73	75-88	90-101	103-117	119-132	134-145
0.9 mg/kg Daily Dose (as calculated)		9-11.7	12.6-18	18.9-23.4	24.3-29.7	30.6-36	36.9-41.4	42.3-47.7	48.6-54	54.9-59.4
Daily Dose Strengths (mg)		(12 mg)	(18 mg)	(24 mg)	(30 mg)	(36 mg)	(42 mg)	(48 mg)	(54 mg)	(60 mg)
		•		6 13	30	36	6	6 6	33 (6) (3)	30

Recommended dosing is approximately 0.9 mg/kg/day

Tablets not shown at actual size.

Annual dose adjustments that align with annual weight gain may not be enough

- Regularly monitoring weight is important especially in younger DMD patients

IMPORTANT SAFETY INFORMATION (cont'd)

What warnings should I know about EMFLAZA?

- EMFLAZA can cause changes in endocrine function. 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 Acute adrenal insufficiency can occur if corticosteroids are withdrawn abruptly, and can be fatal.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.
- There is an increased risk of infection when taking EMFLAZA. Tell the healthcare provider if the patient
 has had recent or ongoing infections or if they have recently received a vaccine. Medical advice should
 be sought immediately if the patient develops fever or other signs of infection. Patients and/or caregivers
 should be made aware that some infections can potentially be severe and fatal. Warn patients who are on

Please see Indication and Important Safety Information throughout and accompanying full Prescribing Information.

HELP HIM MAKE THE MOST OF HIS NOW WITH CONVENIENT ONCE-DAILY DOSING OPTIONS

Tablets are available in 4 different strengths (6 mg, 18 mg, 30 mg, 36 mg)

- EMFLAZA can be taken with or without food
- Tablets may be given whole or crushed and can be taken immediately after mixing with applesauce
- You can combine multiple tablets to add up to the prescribed dosing, rounding up to the nearest dose amount

Also available in liquid form

- Round up to the nearest tenth of a milliliter (mL)
- 13 mL in a 30-mL bottle with two 1-mL oral dispensers included
- Mix EMFLAZA well with 3-4 oz of juice or milk and give immediately
- Store at room temperature

Corticosteroid considerations

• If your son is currently on corticosteroids, tracking his physical development can help with future treatment decisions. If you notice any weight gain, ask your son's healthcare provider to determine if the gains are excessive.

IMPORTANT SAFETY INFORMATION (cont'd)

- corticosteroids to avoid exposure to chickenpox or measles and to alert their healthcare provider immediately if they are exposed.
- EMFLAZA can cause an increase in blood pressure and water retention.
 If this occurs, dietary salt restriction and potassium supplementation may be needed.
- There is an increased risk of developing a hole in the stomach or intestines in patients with certain stomach or intestine disorders when taking corticosteroids like EMFLAZA.
- EMFLAZA can cause severe behavioral and mood changes. Seek medical attention from the health care provider if any behavioral or mood changes develop.
- There is a risk of osteoporosis with prolonged use of EMFLAZA, which can lead to vertebral and long bone fractures.
- EMFLAZA may cause cataracts or glaucoma and a health care provider should monitor for these conditions if corticosteroid therapy is continued for more than 6 weeks.







Bottle not shown at actual size.

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LET THE DOSING ASSISTANT HELP

Dosing support at your fingertips

 You can calculate dosing according to weight using a simple dosing tool. Just provide your son's body weight to see the recommended daily dose. Remember to speak to your son's healthcare provider before making any dosing changes.



Scan the QR code to use the dosing assistant or learn more at <u>www.emflaza.com/dosing</u>

IMPORTANT SAFETY INFORMATION (cont'd)

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 Immunizations should be up-to-date according to immunization guidelines prior to starting therapy with EMFLAZA. Live-attenuated or live vaccines should be administered at least 4 to 6 weeks prior to starting EMFLAZA. Live-attenuated or live vaccines should not be used in patients taking EMFLAZA.

Not an actual patient.

- EMFLAZA can cause serious skin rashes. Seek medical attention at the first sign of a rash.
- Rare instances of anaphylaxis have occurred in patients receiving corticosteroid therapy, including EMFLAZA.

What should I tell my health care provider?

Tell the health care provider about all medical conditions, including if the patient:

- is pregnant or planning to become pregnant. EMFLAZA® (deflazacort) can harm your unborn baby.
- is breastfeeding or planning to breastfeed. EMFLAZA may appear in breastmilk and could affect a nursing child.

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.

What are the side effects of EMFLAZA?

The most common side effects of 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. These are not all of the possible side effects of EMFLAZA. Call your doctor for medical advice about side effects.

To report an adverse event, please call <u>1-866-562-4620</u> or email at <u>usmedinfo@ptcbio.com</u>. You may also report side effects to FDA at <u>1-800-FDA-1088</u> or at <u>www.fda.gov/medwatch</u>.

Please see Indication and Important Safety Information throughout and accompanying full Prescribing Information.

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