FAMILY INFORMATION GUIDE

PLAYING LIKE BROTHERS BECAUSE THEY CAN

For you and your child, right now means everything. That's why now is the time to discuss EMFLAZA® (deflazacort)—***THE FIRST*** FDA-approved corticosteroid for boys with Duchenne muscular dystrophy (DMD).

HELP EXTEND *HIS NOW.*

INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA® (deflazacort)

What is EMFLAZA® (deflazacort) used for?

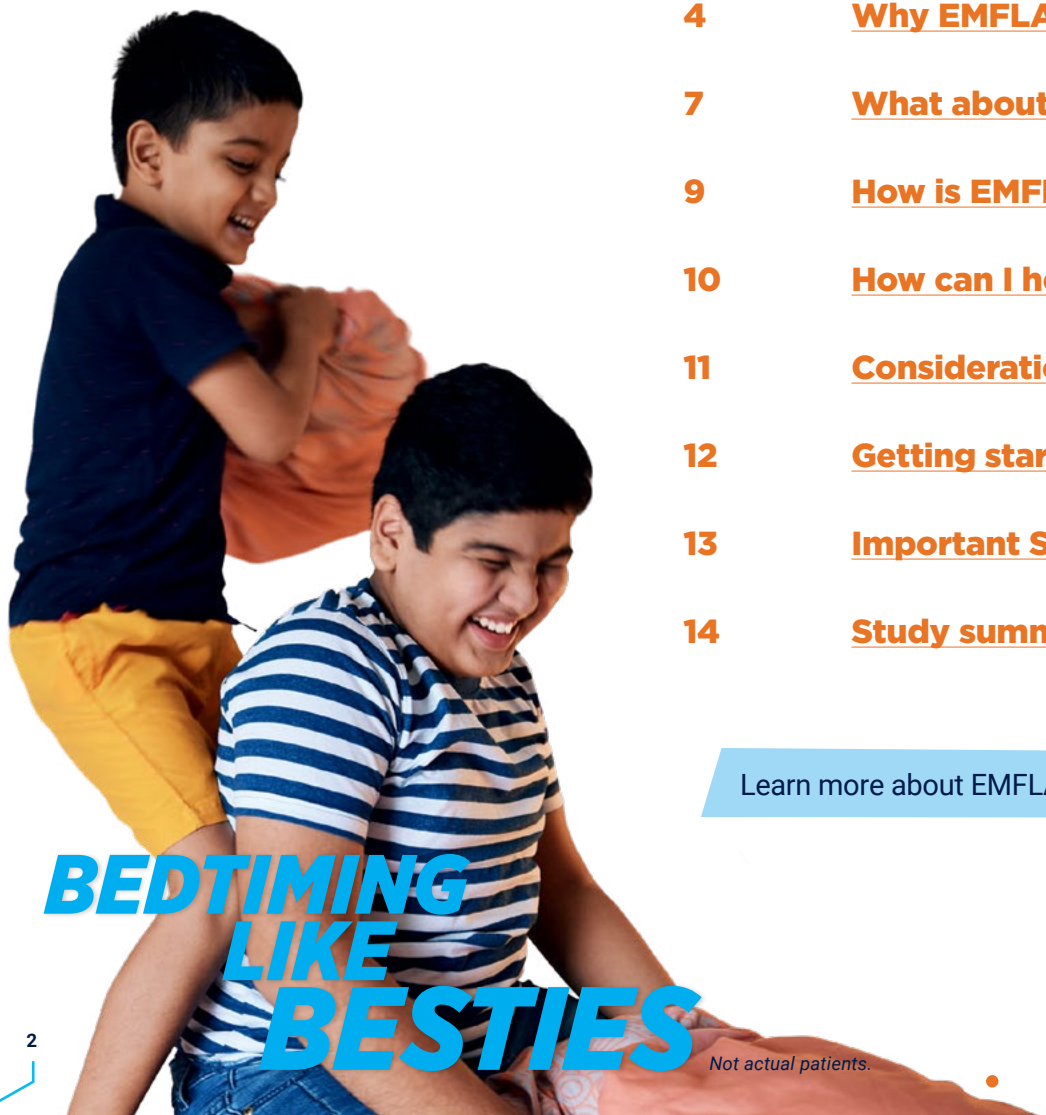
EMFLAZA is a prescription medicine used to treat Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

Please see Indication and Important Safety Information on page 13 and accompanying full Prescribing Information.

Emflaza®
(deflazacort)

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

CONTENTS



Not actual patients.

Learn more about EMFLAZA, ***here and now.***

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WHAT SHOULD I KNOW ABOUT CORTICOSTEROIDS?

Living with DMD, your son has unique functional and emotional needs; both are meaningful considerations for choosing a corticosteroid.

Corticosteroids are often introduced soon after DMD diagnosis to ease inflammation and preserve muscle strength.

Every boy is different, so there are options when choosing a corticosteroid.

There are two corticosteroids used to treat boys with DMD and they are not the same. You can work with your care team to understand both options, their indications, benefits, side effects, and how they can affect your son differently.

The U.S. Centers for Disease Control and Prevention (CDC) guidelines recommend a corticosteroid as part of the DMD treatment plan.

WHY EMFLAZA® (deflazacort)?

Help boys with DMD preserve their "here and now."

As the FIRST FDA-approved corticosteroid for boys with DMD as young as age 2,
EMFLAZA is designed to help maintain muscle strength and function.

IMPROVED
muscle strength
after 3 months vs placebo

Caleb, Age 8
Actual EMFLAZA patient.

In a 52-week clinical trial of 196 boys aged 5 to 15 years with DMD, the effectiveness and safety of EMFLAZA were compared with placebo (sugar pill). After 12 weeks of treatment, patients taking EMFLAZA had significantly improved muscle strength compared with placebo (0.15 change in strength score vs -0.10 change in strength score).

Make the Most of His Now.

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IMPORTANT SAFETY INFORMATION (cont'd)

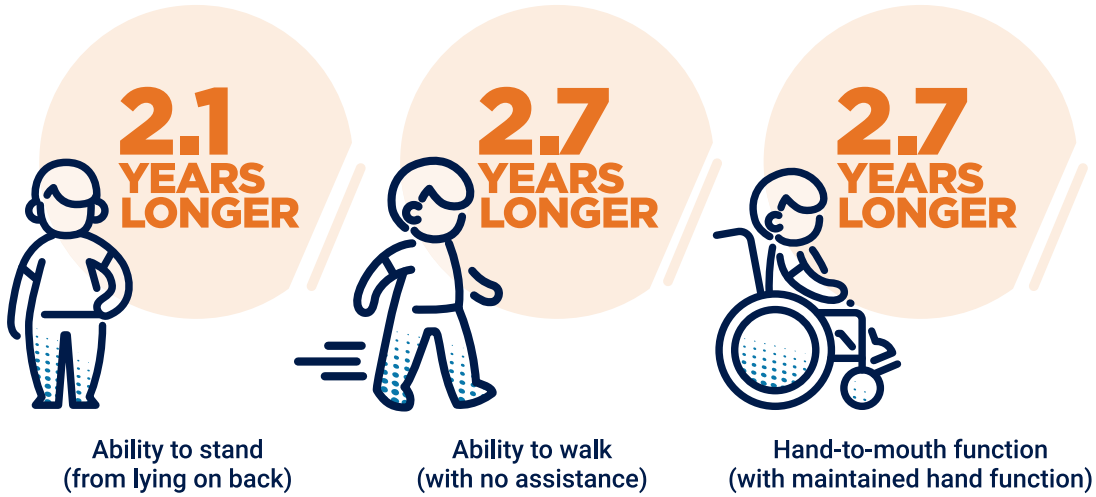
When should I not take EMFLAZA?
Do not use if you have had hypersensitivity, including allergic reactions, to deflazacort or any of the inactive ingredients.

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

Please see Indication and Important Safety Information on page 13 and accompanying full Prescribing Information.

HELP DELAY DMD PROGRESSION, WHEN IT MATTERS MOST

In a 10-year follow-up study measuring 3 important DMD events,
deflazacort showed meaningful differences over time compared to prednisone:



Ability to stand
(from lying on back)

Ability to walk
(with no assistance)

Hand-to-mouth function
(with maintained hand function)

In a study of 440 males aged 2 to 28 years with DMD, the effectiveness and safety of deflazacort were compared with prednisone.

Please see pages 14 and 15 to learn about studies that support information in this brochure not included in the approved label for EMFLAZA® (deflazacort).

Please talk to your son's healthcare provider if you have any questions.

IMPORTANT SAFETY INFORMATION (cont'd)

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.

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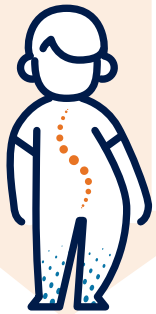
HELP SUPPORT HIM AS HE GROWS

Once your son starts taking deflazacort, it's important for him to stay on treatment as it was shown to preserve muscle function throughout his body—not just in his arms and legs.

In a 13-year study, scoliosis was delayed and lung function was preserved for longer in boys taking deflazacort.

DELAYED ONSET OF SCOLIOSIS (CURVED SPINE)

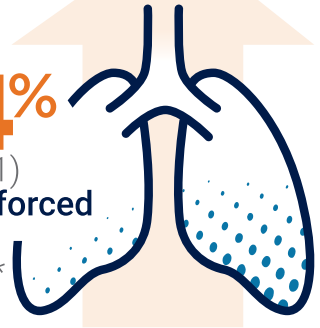
LOWERED RISK OF SCOLIOSIS
compared to prednisone



Over 11.9 years, **17.9% of patients developed scoliosis taking prednisone** vs 7.9% taking deflazacort.

PRESERVED LUNG FUNCTION

9.24%
(CI: 3.06–15.41)
greater mean forced vital capacity
vs prednisone*



*Forced vital capacity is a type of test that measures the amount of air your child can inhale and exhale.

A 13-year study of 435 boys with DMD assessed patients' ages simultaneously with DMD progression events, including scoliosis onset and decrease in lung function. Please see pages 14 and 15 to learn about studies that support information in this brochure not included in the approved label for EMFLAZA® (deflazacort).

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IMPORTANT SAFETY INFORMATION (cont'd)

- 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 corticosteroids to avoid exposure to chickenpox or measles and to alert their healthcare provider immediately if they are exposed.

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WHAT ABOUT SAFETY AND SIDE EFFECTS?

COMMON SIDE EFFECTS WITH DEFLAZACORT

% OF PATIENTS TAKING DEFLAZACORT 0.9 mg/kg/day AT 12 WEEKS

Facial puffiness or Cushingoid appearance **33%**	Frequent daytime urination **12%**	Common cold **10%**	Abdominal discomfort **6%**
Weight increased **20%**	Upper respiratory tract infection **12%**	Irritability **8%**	
Increased appetite **14%**	Unwanted hair growth **10%**	Runny nose **8%**	
Cough **12%**	Central obesity **10%**	Skin redness **8%**	

Please seek treatment immediately and tell your son's healthcare provider if your child has any of these symptoms or any other side effects.

IMPORTANT SAFETY INFORMATION (cont'd)

- 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.

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CONSIDER HIS BEHAVIOR AND WEIGHT—HERE, NOW, AND TOMORROW

The deflazacort molecule is different from other corticosteroids and was developed to provide a more tolerable treatment option.

In a 52-week clinical trial of 196 boys aged 5 to 15 years with DMD, the effectiveness and safety of EMFLAZA® (deflazacort) were compared with prednisone. In comparing adverse events between deflazacort 0.9 mg/kg/day and prednisone 0.75 mg/kg/day, the following was found:

Dunky, Age 6 Caleb, Age 8

Actual EMFLAZA patients.

- **Fewer boys taking deflazacort experienced abnormal behavior compared to those taking prednisone**
  - Deflazacort: 8.8% (n=6/68); prednisone: 14.3% (n=9/63)
- **Boys taking deflazacort experienced less weight gain compared to those taking prednisone**
  - Deflazacort: 27.9% (n=19/68); prednisone: 34.9% (n=22/63)
- **More boys taking deflazacort experienced cataracts compared to those taking prednisone (4.4% vs 1.6%)**
  - No cataracts were considered serious events

Please see pages 14 and 15 to learn about studies that support information in this brochure not included in the approved label for EMFLAZA.
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IMPORTANT SAFETY INFORMATION (cont'd)

- 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.
- 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

Please see Indication and Important Safety Information on page 13 and accompanying full Prescribing Information.

HOW IS EMFLAZA® (deflazacort) TAKEN?

Convenient once-daily dosing to help him stay engaged in the now.

EMFLAZA is available in tablet and liquid forms and can be taken with or without food.

IMPORTANT DOSING CONSIDERATIONS

Do not stop taking EMFLAZA abruptly or without first checking with your son's 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 taken for more than a few days.
- Weight gain should be assessed by your child's healthcare provider to determine whether the dose is appropriate.

Available in tablet and liquid forms
Tablets can be taken in 4 different strengths (6 mg, 18 mg, 30 mg, and 36 mg).

Learn more about dosing.

Dunky, Age 6
Actual EMFLAZA patient.

IMPORTANT SAFETY INFORMATION (cont'd)

EMFLAZA. Live-attenuated or live vaccines should not be used in patients taking EMFLAZA.

- 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:

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HOW CAN I HELP MY SON?

Your child's healthcare provider may turn to corticosteroids soon after DMD diagnosis; however, the steroid your child starts on may not always be the best to stay on.

Your son's healthcare provider may need to reevaluate or increase the dosage as your child grows. If your child is in one of the stages shown below and you're not sure if they are receiving all they should from their current treatment, talk to their healthcare provider.

EARLY AMBULATORY

- Childhood
- Moving slower or with difficulty, frequent falls, muscle weakness, enlarged calves
- It's important to track developmental milestones and health status

LATE AMBULATORY

- Late childhood/adolescent/young adult
- Difficulty walking, may walk off-balance, weakened hands
- A scooter or wheelchair may help decrease fatigue

EARLY NON-AMBULATORY

- Adolescent/young adult
- Unable to walk, scoliosis (curved spine), muscle pain, weakness in arms
- This may be the time to track respiratory function twice a year

Continuing corticosteroids through all stages of DMD can help him stay involved with friends and family for longer.

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IMPORTANT SAFETY INFORMATION (cont'd)

- 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.

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CONSIDERATIONS FOR SWITCHING CORTICOSTEROIDS

If your son is currently on corticosteroids, tracking his physical and emotional development can help with making treatment decisions. As his symptoms change over time, asking yourself these questions may be helpful.

STRENGTH	Has there been a shift in his muscle strength or function?
WEIGHT	Do the gains feel excessive?
INDEPENDENCE	Has his disease progressed?
TIME	Are activities taking longer than before (eg, walking, standing, etc.)?
COMMUNICATION	Has his emotional state shifted?
HEALTH STATUS	Is he maintaining his quality of life?

Ask your son's healthcare provider about making a change.

COOKING LIKE A PRO

Dunky, Age 6
Actual EMFLAZA patient.

IMPORTANT SAFETY INFORMATION (cont'd)

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.

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GETTING STARTED WITH PTC *Cares*™

Once you and your healthcare provider have decided to start EMFLAZA® (deflazacort), simply fill out a prescription form to begin the journey.

STEP 1

Obtaining a Prescription

Fill out the **Prescription Start Form** with your healthcare provider.

STEP 2

Explanation of Benefits

Your dedicated Case Manager will walk you through the details of your insurance benefits and any additional financial assistance programs that may be available to you.

STEP 3

Getting Your Medication

Your medication will be delivered to you directly by a specialty pharmacy. Your Case Manager will coordinate with you and your pharmacy to help make sure deliveries arrive where and when you need them.

Support From Someone Who's Been There

Learning how others have managed challenges and achieved successes can help you gain insight into decisions you may need to make for your family. The Peer Navigator Program can connect you with other families who understand your journey. Your family can choose to speak with English- or Spanish-speaking navigators, according to your preferences.

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INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA® (deflazacort)

What is EMFLAZA® (deflazacort) used for?

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When should I not take EMFLAZA?

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What should I tell my health care provider?

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

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STUDY SUMMARIES

Scientific publications can provide helpful information when you are looking to learn more about a topic that is meaningful to you or your family's needs. The following studies support information in this brochure not included in the approved label for EMFLAZA® (deflazacort). Please talk to your son's healthcare provider if you have any questions.

2016 STUDY

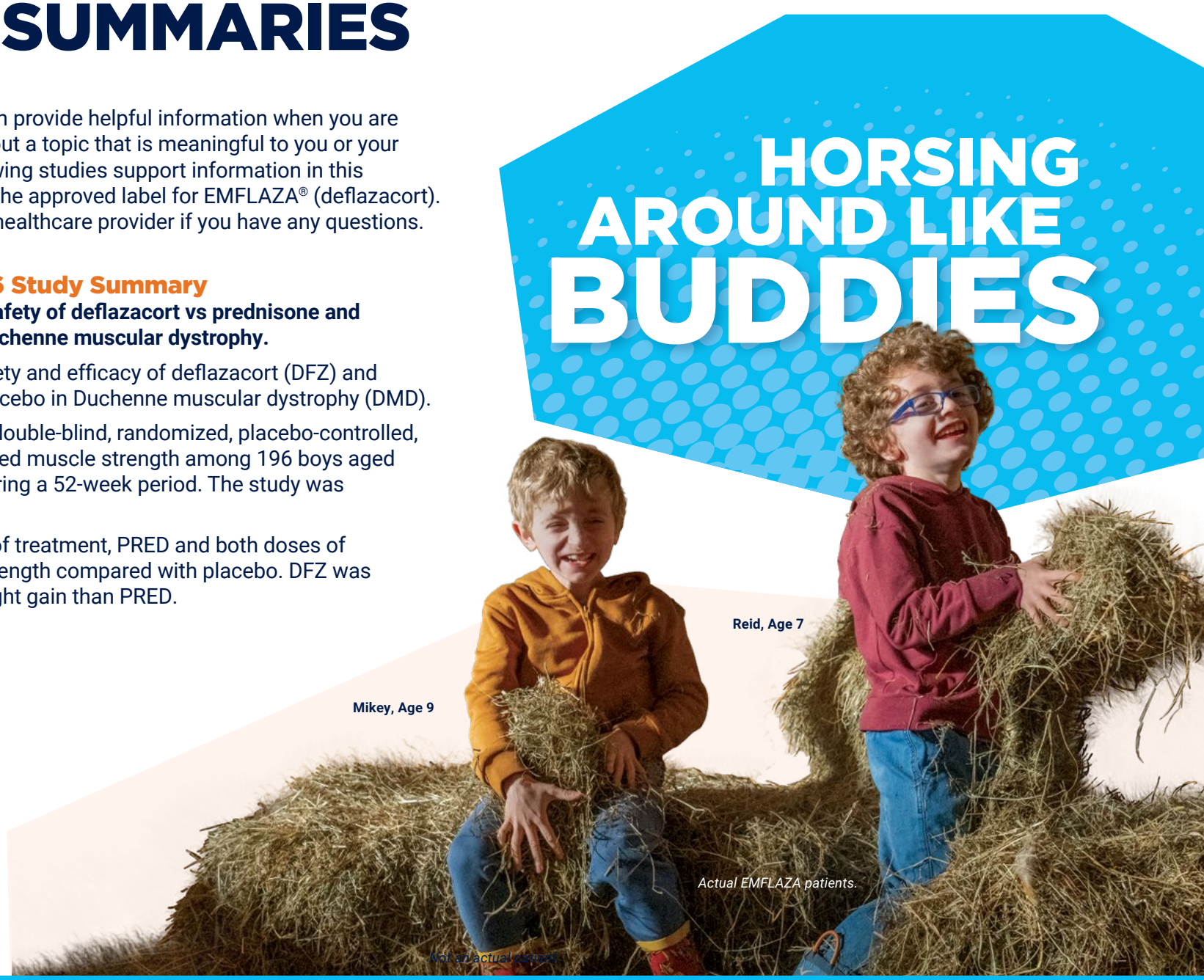
Griggs 2016 Study Summary

Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy.

Objective: To assess safety and efficacy of deflazacort (DFZ) and prednisone (PRED) vs placebo in Duchenne muscular dystrophy (DMD).

Methods: This phase III, double-blind, randomized, placebo-controlled, multicenter study evaluated muscle strength among 196 boys aged 5 - 15 years with DMD during a 52-week period. The study was completed in 1995.

Results: After 12 weeks of treatment, PRED and both doses of DFZ improved muscle strength compared with placebo. DFZ was associated with less weight gain than PRED.



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STUDY SUMMARIES

2018 STUDY

McDonald 2018 Study Summary

Long-term effects of glucocorticoids on function, quality of life, and survival in patients with Duchenne muscular dystrophy: a prospective cohort study.

Objective: This study examined the long-term effects of glucocorticoids on milestone-related disease progression across the lifespan and survival in patients with Duchenne muscular dystrophy.

Methods: For this prospective cohort study, male patients aged 2 - 28 years with Duchenne muscular dystrophy were enrolled at 20 centers in nine countries. Patients were followed up for 10 years. The study measured the progression of nine mobility and upper limb milestones to compare no glucocorticoid treatment or cumulative treatment duration of less than 1 month versus treatment of 1 year or longer.

Results: 440 patients were enrolled during two recruitment periods (2006 - 09 and 2012 - 16). Time to all disease progression milestone events was significantly longer in patients treated with glucocorticoids for 1 year or longer than in patients treated for less than 1 month or never treated (log-rank). Glucocorticoid treatment for 1 year or longer was associated with increased median age at loss of mobility milestones by 2.1 - 4.4 years and upper limb milestones by 2.8 - 8.0 years compared with treatment for less than 1 month. Deflazacort was associated with increased median age at loss of three milestones by 2.1 - 2.7 years in comparison with prednisone or prednisolone (log-rank).

2020 STUDY

Marden 2020 Study Summary

Real-world outcomes of long-term prednisone and deflazacort use in patients with Duchenne muscular dystrophy: experience at a single, large care center.

Objective: To assess outcomes among patients with Duchenne muscular dystrophy receiving deflazacort or prednisone in real-world practice.

Methods: Clinical data for 435 boys with Duchenne muscular dystrophy from Cincinnati Children's Hospital Medical Center were studied retrospectively using time-to-event and regression analyses.

Results: Median ages at loss of ambulation were 15.6 and 13.5 years among deflazacort- and prednisone-initiated patients, respectively. Deflazacort was also associated with a lower risk of scoliosis, improved ambulatory function, greater % lean body mass, shorter stature, and lower weight, after adjusting for age and steroid duration. No differences were observed in whole body bone mineral density or left ventricular ejection fraction.

Not an actual patient.

WINNING LIKE A GAMER

Learn more here and now at EMFLAZA.com.

Get your child started on EMFLAZA® (deflazacort) by completing the **Prescription Start Form**.

To report SUSPECTED ADVERSE REACTIONS, contact PTC Therapeutics, Inc. at 1-866-562-4620 or Pharmacovigilance@PTCBio.com or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

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US-EMF-0296 12/23

PTC THERAPEUTICS™

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