

DUCHENNE MUSCULAR DYSTROPHY

Duchenne muscular dystrophy (Duchenne) is a **severe form** of muscular dystrophy.

1 of 7,000+ rare diseases with few or no treatment options.¹

Fatal Genetic Disorder
Life expectancy is generally between the late teens and early twenties.²

1 in 5,000 live male births⁴
Duchenne largely occurs in males, although on rare occasions it can appear in females.³

Affects **300,000+** males worldwide, **15,000** in the U.S.^{5,14}

TREATING DUCHENNE

There is not yet a cure for Duchenne, but medical treatments may help slow disease progression.²

A comprehensive multidisciplinary care approach is recommended for Duchenne, but compliance varies.³

Corticosteroids like EMFLAZA™ (deflazacort) have been shown to be effective in slowing the decline in muscle strength and function in Duchenne.^{2,15}

More than 50% of eligible Duchenne patients do not receive corticosteroids.¹²

Other types of interventions help to address symptoms of the disease:³



Physical and occupational therapy



Orthopedic, cardiac, gastrointestinal & pulmonary management



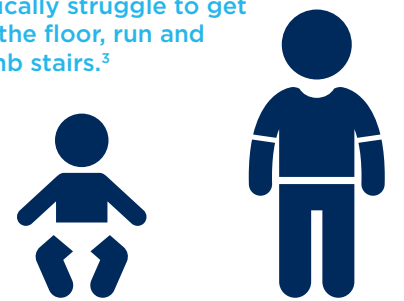
Behavior & learning management

Please see reverse side for Indication and Important Safety Information. Please see www.EMFLAZA.com for full Prescribing Information.



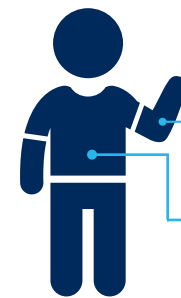
SYMPTOMS AND PROGRESSION

Toddlers with Duchenne tend to sit and stand later than their peers.⁷ Once they begin to walk, they'll typically struggle to get off the floor, run and climb stairs.³



Patients with Duchenne often have trouble moving beginning at an early age.⁷

AGES 11 12 13



As they get older, muscle weakness advances to the skeletal muscles in the arms, legs and trunk.⁷

In their early teens, heart and respiratory muscles often begin to weaken.^{9,10}



Despite advances, patients with Duchenne often die of heart trouble or respiratory complications.^{7,11}

CAUSES OF DUCHENNE



Duchenne is caused by mutations in the **dystrophin gene**. Without dystrophin, a structural protein, muscles are unable to operate properly and suffer progressive damage.³



Patients with Duchenne are born with the genetic mutation. **There is nothing they can do to stop it.**



About **2/3** of patients with Duchenne inherited the mutation from their **mother** through the **X chromosome**.⁷



About **1/3** of patients have Duchenne because of a random spontaneous **genetic mutation**.⁸

1860s

Duchenne was first described.

1980s

The genetic mutation responsible for Duchenne was identified.

2016

The first treatment for Duchenne was approved in the U.S.

2017

EMFLAZA™ (deflazacort) marks the first time that an FDA-approved treatment is available for patients 5 years of age and older with all genetic forms of Duchenne.



Emflaza™
(deflazacort)

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

ABOUT EMFLAZA™ (deflazacort)

EMFLAZA is Indicated for the Treatment of Duchenne Muscular Dystrophy in Patients 5 Years of Age and Older.

EMFLAZA is not for patients who are allergic to deflazacort or any of the inactive ingredients in EMFLAZA.

Patients should not stop taking EMFLAZA, or change the amount they are taking, without first checking with their healthcare provider, as there may be a need for gradual dose reduction to decrease the risk of serious side effects.

Corticosteroids, such as EMFLAZA can cause:

- Hyperglycemia, altered glucose metabolism
- Increased risk of infection
- Changes in cardiovascular/kidney function that could lead to increases in blood pressure, salt, and water retention, and decreases in blood levels of potassium and calcium
- Behavioral and mood changes that could lead to potentially severe psychiatric adverse reactions
- Osteoporosis, decrease in bone mineral density
- Serious skin rashes
- May slow growth and development
- Cataracts or glaucoma

Vaccinations: The administration of live or live attenuated vaccines is not recommended while taking EMFLAZA. Killed or inactivated vaccines may be administered, but the responses cannot be predicted. Patients should discuss their vaccine history with their healthcare provider before starting EMFLAZA, and while taking EMFLAZA, patients should check with their healthcare provider before receiving any new vaccines or booster shots.

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.

Patients should tell their healthcare provider if they have had recent or ongoing infections, develop a fever, or experience any other side effects.

Please see www.EMFLAZA.com for full Prescribing Information.

You may report side effects to ProPharma Group at 1-866-562-4620 or drugsafety@propharmagroup.com.

You may report side effects to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

REFERENCES

1. Centers for Disease Control and Prevention. (2016, Feb 17). Rare Diseases, Genomics and Public Health: An Expanding Intersection. Retrieved from <https://www.nichd.nih.gov/news/resources/spotlight/Pages/020116-rare-disease-day.aspx>. Accessed December 19, 2016.
2. Balaban B, Matthews DJ, Clayton GH, Carry T. Corticosteroid treatment and functional improvement in Duchenne muscular dystrophy: long-term effect. *Am J Phys Med Rehabil*, 2005;84(11):843-50.
3. Bushby K, Finkel R, Birnkrant D et al. Diagnosis and management of Duchenne muscular dystrophy, Part 1: Diagnosis, and pharmacological and psychosocial management. *Lancet*, published online November 30, 2009.
4. Mendell JR, Lloyd-Puryear M. Report of MDA muscle disease symposium on newborn screening for Duchenne muscular dystrophy. *Muscle Nerve*, 2013;48(1):21-6.
5. Cure Duchenne. (2016). What is Duchenne? Retrieved from <http://www.cureduchenne.org/about-duchenne.html>. Accessed December 19, 2016.
6. Muscular Dystrophy Association. (2016). Duchenne Muscular Dystrophy (DMD). Retrieved from <https://www.mda.org/disease/duchenne-muscular-dystrophy>. Accessed December 19, 2016.
7. NIH National Human Genome Research Institute. (2013, April 18). Learning About Duchenne Muscular Dystrophy. Retrieved from <https://www.genome.gov/19518854/learning-about-duchenne-muscular-dystrophy/>. Accessed December 19, 2016.
8. Aartsma-Rus A, et al. The importance of genetic diagnosis for Duchenne muscular dystrophy. *J Med Genet* 2016;53:145-151.
9. Biggar WD, Harris VA, Eliasoph L, Alman B. Long-term benefits of deflazacort treatment for boys with Duchenne muscular dystrophy in their second decade. *Neuromuscul Disord* 2006; 16: 249-55.
10. Roberto R, Fritz A, Hagar Y, Boice B, Skalsky A, Hwang H, et al. The natural history of cardiac and pulmonary function decline in patients with Duchenne muscular dystrophy. *Spine* 2011;36:E1009-E1017.
11. Moxley RT, Ashwal S, Pandya S, et al., Practice Parameter: Corticosteroid treatment of Duchenne dystrophy. *Neurology*. 2005; 64(1): 13-20.
12. Kim S, Campbell KA, Fox DJ, et al., Corticosteroid treatments in males with Duchenne muscular dystrophy: treatment duration and time to loss of ambulation, *J Child Neurol*, 2015;30(10):1275-1280.
13. U.S. Food and Drug Administration. (2016). FDA grants accelerated approval to first drug for Duchenne muscular dystrophy. Retrieved from <http://www.fda.gov/newsevents/newsroom/pressannouncements/ucm521263.htm>. Accessed December 19, 2016.
14. Coach to Cure MD. (2015). Our Cause: Duchenne Muscular Dystrophy. Retrieved from http://www.parentprojectmd.org/site/PageServer?pagename=CTCMD_cause. Accessed December 19, 2016.
15. Griggs RC, Miller JP, Greenberg CR, et al., Efficacy and Safety of Deflazacort Vs Prednisone and Placebo for Duchenne Muscular Dystrophy, *Neurology*, 2016, Nov 15;87(20):2123-2131.

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