



Summary of Practice Guideline for **Patients** and their **Families**

## Duchenne Muscular Dystrophy: Treatment with Corticosteroids

This information sheet is provided to help you understand the evidence for treating Duchenne muscular dystrophy (DMD) with corticosteroids. This sheet is a service of the American Academy of Neurology (AAN).

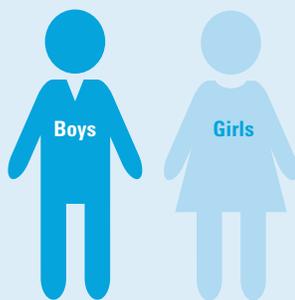
The AAN is the world's largest association of neurologists and neuroscience professionals. Neurologists are doctors who identify and treat diseases of the brain and nervous system. The AAN is dedicated to promoting the highest quality patient-centered neurologic care.

Experts from the AAN carefully reviewed the available scientific studies on treating DMD with corticosteroids. The following information\* is based on evidence from those studies. The information summarizes the main findings of the 2016 AAN guideline on treating DMD with corticosteroids. The 2016 guideline is an update to the 2005 AAN guideline on this topic.

To read the full 2016 guideline, visit [AAN.com/guidelines](http://AAN.com/guidelines).

### What is muscular dystrophy? What is DMD?

Muscular dystrophy (MD) is a group of several different genetic diseases. It causes muscle wasting (damage) and weakness. This affects the muscles of the arms and legs. In some cases it may affect muscles of the face and muscles that control breathing and swallowing.



#### DMD is a type of MD that mainly affects boys

DMD affects around **1.5/10,000** live male births\*

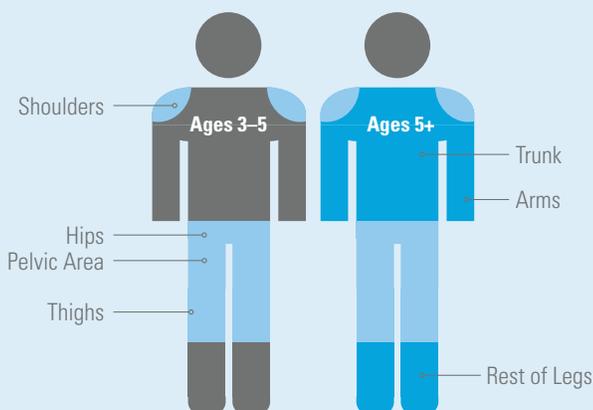
\*Although DMD can affect females, this is rare.

The muscle weakness is progressive. This means the muscle damage gets worse and spreads over time to involve other muscles. Muscle weakness can make it difficult to move or to lift objects. It also affects posture or the ability to hold the body upright.

The rate of ongoing muscle damage depends on both the person and the subtype of the disease. Some subtypes can shorten lifespan. However, in many subtypes, the disorder progresses slowly.

A common sign is unusually large calf muscles. This is because the muscle tissue is abnormal. It may contain scar tissue, making the muscle bulkier. Children with DMD often start walking later than normal. Young children with DMD may seem clumsy and fall often. They may have trouble with:

- Climbing stairs
- Getting up from the floor
- Running



#### Muscle groups affected by DMD

In DMD, symptoms usually appear between ages three and five. Muscle weakness is an early symptom. It can set in as early as age three.

Eventually, DMD leads to:

- Impaired (damaged) functioning of:
  - The lungs and muscles that control breathing
  - The heart
- Inability to walk without an aid (such as a wheelchair)
- Scoliosis (a curved or crooked spine)
- Death by early adulthood

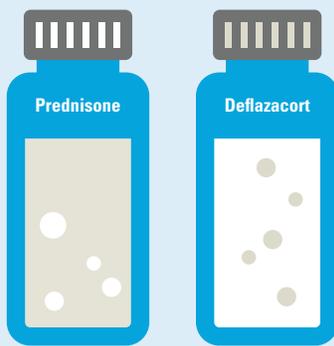
At this time, there is no cure for DMD. However, treatment can reduce some signs and symptoms.

## How is DMD treated? Are there prescription drugs that can help?

DMD typically is treated with drug therapy and cross-specialty care. Certain prescription drugs, called corticosteroids, can help treat some symptoms of DMD.

It is important to know that these drugs can cause serious side effects. For this reason, doctors prescribe low doses. They aim for a dose that is strong enough to be effective while causing the least amount of side effects.

Moderate evidence\* shows that prednisone 0.75 mg/kg/day is the preferred dose. There is moderate evidence\* that prednisone 10 mg/kg/weekend is equally effective over 12 months. However, there is not enough information to show if children will have better outcomes long-term.



**There is moderate evidence\* that prednisone can help improve muscle strength and lung function in DMD. Weak evidence\* shows deflazacort may help with these symptoms.**

These two drugs have been studied for use in DMD. In some places, only one drug—prednisone or deflazacort—is available.

## What are the possible side effects of these corticosteroids?

Before beginning therapy, patients and their families should discuss the use of these drugs with their doctors. The possible side effects can be significant. Be sure to weigh the risks and benefits carefully.

- Prednisone 0.75 mg/kg/day is probably linked with significant risk of weight gain, excessive hair growth, and puffiness of the face
- Deflazacort may increase the risk of cataracts

The tables below present the guideline findings.

## Tables: Evidence for Prednisone and Deflazacort in DMD

### Disease Progression

Strength of Evidence*	Evidence
<b>Moderate</b>	Prednisone probably improves strength and lung function
<b>Weak</b>	Prednisone may improve timed motor function (functioning during a timed test of movement)
	Deflazacort may improve strength, lung function, and timed motor function
	Deflazacort may delay time to loss of ability to walk
	Deflazacort may increase survival over 5 to 15 years of treatment
	Prednisone or deflazacort may slow the development of scoliosis (a crooked/curved spine)
	Prednisone or deflazacort may reduce the need for scoliosis surgery by 18 years of age
	Prednisone or deflazacort may delay the onset of heart muscle disease by 18 years of age
<b>Not Enough Evidence</b>	Benefit for increasing survival with prednisone
	Benefit for improving quality of life with prednisone or deflazacort
	Benefit for increasing survival with bisphosphonates as add-on therapy to corticosteroids

## Side Effects

Strength of Evidence*	Evidence
<b>Moderate</b>	Prednisone probably linked to significant risk of weight gain, excessive body hair growth, and puffiness of the face (cushingoid appearance)
	Prednisone probably causes shortness of height, behavioral changes, fractures (bone breaks), and cataracts (an eye defect)
<b>Weak</b>	Prednisone may be linked to weight gain in the first year of treatment compared with deflazacort
	Deflazacort may be linked to increased risk of cataracts (a type of eye defect)
<b>Not Enough Evidence</b>	Link between deflazacort and increased risk of weight gain, excessive body hair growth, and puffiness of face

## Comparative Effectiveness

Strength of Evidence*	Evidence
<b>Weak</b>	Prednisone and deflazacort may be equivalent for improving motor (movement) function
<b>Not Enough Evidence</b>	A direct comparison of how effective either prednisone or deflazacort is for improving heart function

## Dosing

Strength of Evidence*	Evidence
<b>Moderate</b>	Prednisone 0.75 mg/kg/day and 10 mg/kg/weekend are probably equally effective at 12 months and probably cause similar side effects over 12 months
<b>Weak</b>	Prednisone 0.75 mg/kg/day and 1.5 mg/kg/day may be equally effective and may cause similar side effects
	Prednisone 0.75 mg/kg/day may be more effective than 0.3 mg/kg/day and may have a greater rate of side effects
<b>Not Enough Evidence</b>	Difference in side effects between prednisone doses taken daily and doses taken every other day
	Preferred dose of deflazacort
	Significant difference in side effects at different doses of deflazacort

## Bone Health

Strength of Evidence*	Evidence
<b>Not Enough Evidence</b>	Benefit for bone health with adding calcifediol and bisphosphonates (alendronate) to prednisone therapy

## This guideline was endorsed by the American Academy of Pediatrics, the American Association of Neuromuscular & Electrodiagnostic Medicine, and the Child Neurology Society.

Some information on disease background was obtained from the Muscular Dystrophy Association at [MDAUSA.org](http://MDAUSA.org).

This statement is provided as an educational service of the American Academy of Neurology. It is based on an assessment of current scientific and clinical information. It is not intended to include all possible proper methods of care for a particular neurologic problem or all legitimate criteria for choosing to use a specific procedure. Neither is it intended to exclude any reasonable alternative methodologies. The AAN recognizes that specific patient care decisions are the prerogative of the patient and the physician caring for the patient, based on all of the circumstances involved.

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\*After the experts review all of the published research studies, they describe the strength of the evidence supporting each recommendation:

*Strong* evidence = more than one high-quality scientific study

*Moderate* evidence = at least one high-quality scientific study or two or more studies of a lesser quality

*Weak* evidence = the studies, while supportive, are weak in design or strength of the findings

*Not enough* evidence = either different studies have come to conflicting results or there are no studies of reasonable quality

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