



CORTICOSTEROIDS FOR DUCHENNE MUSCULAR DYSTROPHY

Duchenne muscular dystrophy (DMD) is the most common form of muscular dystrophy in children. DMD is a genetic—*or inherited*—disorder. It is linked to the X-chromosome. DMD mainly affects boys. An estimated one in 3,500 males worldwide has DMD. Around 400 boys in the US are born with DMD each year. Females are rarely affected.

Neurologists from the American Academy of Neurology (AAN) and the Child Neurology Society (CNS) who specialize in diseases of the brain, central nervous system, muscle and nerves, believe you should know about the treatment options for DMD, and their side effects.

There is no cure. But there are a range of complementary and supportive therapies for DMD. The experts from the AAN and the CNS looked carefully at all of the research for *corticosteroids*, one of the treatments for DMD. The experts wanted to find out how safe and effective this treatment is for children with DMD.

Cause of DMD

DMD occurs when *dystrophin*, one of the proteins that help keep muscle cells strong, is missing. Bundles of fibers make up muscle tissue. A group of proteins along the top layer of each fiber helps keep muscle cells intact and working properly. The lack of dystrophin is due to a genetic abnormality.

Symptoms of DMD usually appear between ages two and five. In a child with DMD, the muscles break down over time and muscle strength declines. The first muscles affected are at the hip and thigh, the pelvis, upper arms, and upper legs. Symptoms may include:

- Difficulty running and jumping
- Difficulty climbing steps
- Frequent falls
- Large calf muscles
- Difficulty getting up from a lying or sitting position

Treatment for DMD

In some cases, children may need physical therapy to reduce muscle tightening, orthopedic support devices, and corrective orthopedic surgery to improve their ability to function.

As part of your child's treatment plan, the doctor may also prescribe drugs called corticosteroids. These drugs slow the rate of muscle deterioration. Corticosteroids are the only effective drugs in providing improvements in children with DMD. Experts believe these drugs may help improve muscle strength and delay the progression of DMD.

Corticosteroids are man-made drugs. They are similar to a hormone called cortisone. The body produces cortisone on its own. Research shows that two corticosteroids, prednisone and deflazacort, are effective in DMD. These two corticosteroids have a similar makeup. As of January 2005, only prednisone was available in the US.

The experts found that prednisone helps muscle strength and function and should be offered as a treatment option.

Deflazacort can be prescribed in the countries where it is available.

Taking corticosteroids can have risks. They may cause side effects. Your child's doctor will discuss these risks and side effects with you. The most common side effects are:

- Weight gain
- *Cushingoid* appearance—a rounded face
- Acne
- Cataracts—particularly in patients taking deflazacort
- Excessive hair growth
- Gastrointestinal symptoms
- Irritability or behavior changes

A nutrition plan and exercise program may prevent some of these side effects.

Your child's doctor may perform tests to see if the drugs are having an effect and if your child is having side effects. These may include muscle strength and function tests and lung function tests.

If the side effects outweigh the benefits, your neurologist may lower the dose of the drug. If the dose is decreased, the level of improvement in muscle strength and function may not be as great.

Talk to your primary care doctor or a child neurologist

Together you and your child's health care provider can choose the treatments that will work best for your child. A child neurologist can provide more information and resources to help patients and caregivers make the best treatment choices.

This is an evidence-based educational service of the American Academy of Neurology. It is designed to provide members with evidence-based guideline recommendations to assist with decision-making in patient care. It is based on an assessment of current scientific and clinical information, and is not 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 the circumstances involved. Physicians are encouraged to carefully review the full AAN guidelines so they understand all recommendations associated with care of these patients.



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