



# CORTICOSTEROID TREATMENT OF DUCHENNE MUSCULAR DYSTROPHY

This is a summary of the American Academy of Neurology (AAN) and Child Neurology Society (CNS) evidence-based guideline, which determines the current best practice for corticosteroid treatment of Duchenne muscular dystrophy (DMD) in children.

The authors of this guideline reviewed available evidence on corticosteroid treatment of boys with DMD to determine whether there are sufficient benefits with limited risks to recommend their use in this condition. This guideline is based on a complete and critical analysis of the published studies to date. This summary is designed to provide a strategy to make decisions in patient care.

*Please refer to the full guideline for detailed findings and supporting evidence at [www.aan.com/professionals/practice/index.cfm](http://www.aan.com/professionals/practice/index.cfm).*

Benefits and side effects of corticosteroid therapy need to be monitored. Timed function tests, pulmonary function tests, and age at loss of independent ambulation are useful to assess benefits. An offer of treatment with corticosteroids should include a balanced discussion of potential risks.

Potential side effects of corticosteroid therapy:

- Weight gain
- Cataracts
- Acne
- Gastrointestinal symptoms
- Cushingoid appearance
- Short stature (i.e., a decrease in linear growth)
- Excessive hair growth
- Behavioral changes

## STRONG EVIDENCE SUGGESTS

Drug	Recommendations and Dose	Side Effects and Dose
<b>Prednisone</b>	Prednisone has been demonstrated to have a beneficial effect on muscle strength and function in boys with DMD and should be offered (at a dose of 0.75 mg/kg/d) as treatment. <b>(Level A)</b>	Maintaining a dosage of 0.75 mg/kg/d is optimal; but, if side effects require a decrease in prednisone, tapering to dosages as low as 0.3 mg/kg/d gives less robust but significant improvement. <b>(Level A)</b>  If excessive weight gain occurs (>20% over estimated normal weight for height over a 12-month period), based on available data, it is recommended that the dosage of prednisone be decreased (to 0.5 mg/kg/d with a further decrease after 3-4 months to 0.3 mg/kg/d if excessive weight gain continues). <b>(Level A)</b>
<b>Deflazacort</b>	Deflazacort (0.9 mg/kg/d) can also be used for the treatment of DMD in countries in which it is available. <b>(Level A)</b>	Patients should be monitored for asymptomatic cataracts as well as weight gain during treatment with deflazacort. <b>(Level A)</b>

View the following additional AAN child neurology guidelines at [www.aan.com/professionals/practice/index.cfm](http://www.aan.com/professionals/practice/index.cfm)

Date	Title
December 2004	Pharmacological Treatment of Migraine Headache in Children and Adolescents
May 2004	Medical Treatment of Infantile Spasms
March 2004	Diagnostic Assessment of the Child with Cerebral Palsy
February 2003	Evaluation of the Child with Global Developmental Delay
January 2003	Treatment of the Child with a First Unprovoked Seizure
September 2000	Evaluating a First Nonfebrile Seizure in Children
August 2000	Screening and Diagnosis of Autism

This guideline summary is evidence-based. The AAN uses the following definitions for the level of recommendation and classification of evidence. **\*Recommendation Level:** "Level" refers to the strength of the practice recommendation based on the reviewed literature. **Level A:** Established as effective, ineffective or harmful for the given condition in the specified population. **Level B:** Probably effective, ineffective or harmful (or probably useful/predictive or not useful/predictive) for the given condition in the specified population. **Level C:** Possibly effective, ineffective or harmful (or possibly useful/predictive or not useful/predictive) for the given condition in the specified population. **Level U:** Data inadequate or conflicting; given current knowledge, treatment is unproven.

This is an 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.

Copies of this summary and additional companion tools are available at [www.aan.com/professionals/practice/index.cfm](http://www.aan.com/professionals/practice/index.cfm) or through AAN Member Services at (800) 879-1960.



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