

Co-author of Study on Corticosteroid Side Effects With DMD Finds More Reasons to Use Them

The Duchenne muscular dystrophy (DMD) community has long felt they were in a catch-22 situation: Corticosteroid therapy is the standard of care to slow disease progression, but long-term use of the drugs can come with serious adverse side effects.

Robert Griggs, MD, a professor of neurology, medicine, pediatrics, pathology, and laboratory medicine at the University of Rochester School of Medicine and Dentistry, says this perception hasn't just been a problem in the United States but globally.

"I was an author on a paper published in the New England Journal of Medicine in 1989 ([PMID 2657428](#)) which showed that a dosage of 0.75 mg/kg per day was effective in increased strength and can increase it for many months," he says. "The question has been about side effects."

Dr. Griggs sought to answer that question as co-author of a new paper "Effect of Different Corticosteroid Dosing Regimens on Clinical Outcomes in Boys With Duchenne Muscular Dystrophy," published online by the Journal of the American Medical Association in April ([PMID 35381069](#)).

The study involved 196 boys with DMD, examining side effects when corticosteroids were administered.

"The most important findings of this study included the fact that daily prednisone or deflazacort are both much better than the intermittent regimen," says Dr. Griggs, a principal investigator for the study, which received funding from the National Institutes of Health (NIH) and MDA. "Deflazacort had few side effects — it had less weight gain but did have some growth restriction versus prednisone. But there wasn't any major difference in the benefit of the two drugs. Also worth noting is that prednisone is less expensive than deflazacort."

The study subjects were followed for three years of treatment with corticosteroids. "As far as clinicians are concerned, only three side effects were called serious, and two of the three were an X-ray abnormality in two patients and excess calcium in the urine in one patient — none of which bothered the patients," he says.

This study has the potential to impact DMD treatment across the world, as some providers and families have been avoiding using corticosteroids because of concerns about side effects. The findings reported in this paper should help allay those concerns and give providers data that will justify the use of corticosteroid therapy.

"This treatment needs to be available to many more people," Dr. Griggs says. "We have to keep getting the word out on how it can help."

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