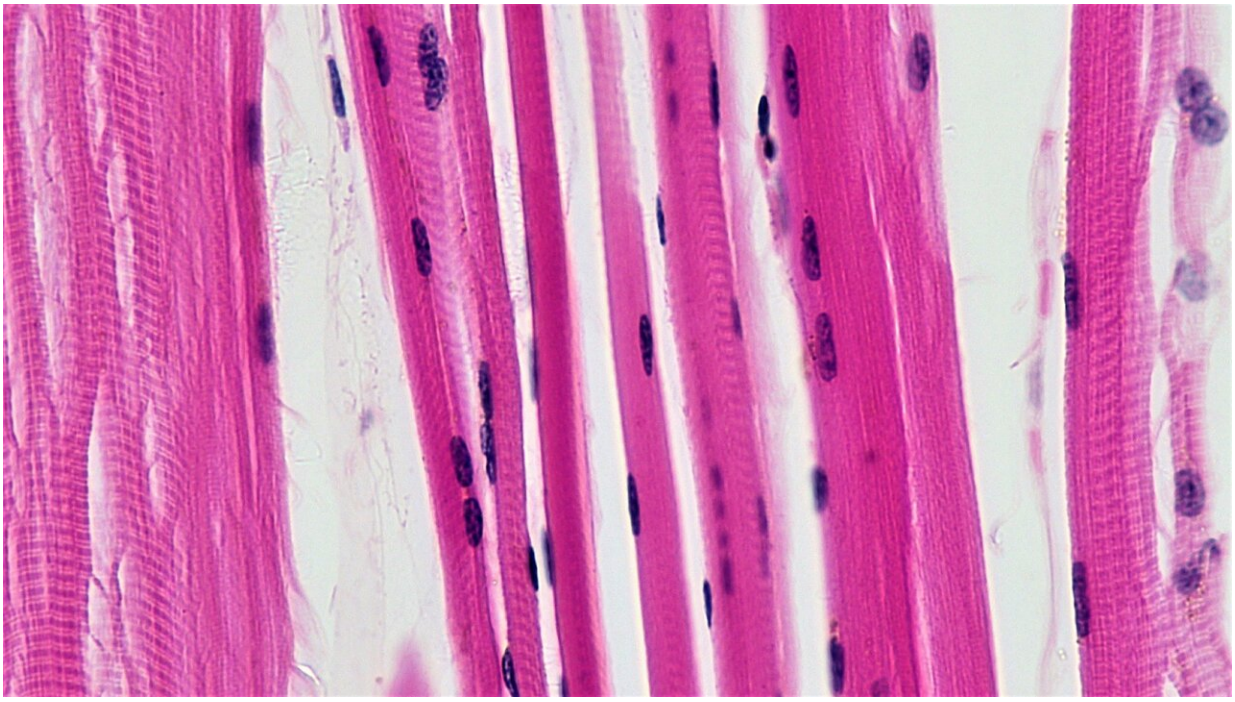
Study finds daily steroids safe and slow the progression of Duchenne muscular dystrophy

May 4 2022, by Mark Michaud



Skeletal muscle fibers. Credit: Berkshire Community College Bioscience Image Library / Public domain

New research published in *JAMA* recommends daily steroid doses for children with Duchenne muscular dystrophy (DMD), marking a significant change in how the disease is treated. University of Rochester Medical Center (URMC) neurologist Robert Griggs, M.D., and Michela Guglieri, M.D., with Newcastle University in the U.K., led the study,

which was conducted by a global team of researchers dedicated to improving care for this fatal disease.

"Corticosteroids are likely to remain the main treatment for DMD for the foreseeable future and worldwide, so it is critical that we establish a standard of care that is backed by scientific evidence," said Griggs. "This study shows that health concerns over the daily use of corticosteroids are overstated and that there is a clear benefit in terms of improved motor and pulmonary functions. These findings clearly support the daily regimen over an intermittent one as an initial treatment for boys with DMD."

DMD is a condition found almost exclusively in boys, and is characterized by muscle weakness, which appears at age 3-4 and progresses rapidly, leading to significant disability. The symptoms eventually spread to the heart and muscles responsible for breathing, and the disease is often fatal by the time the boy reaches his late teens. An estimated 28,000 people in the U.S. suffer from the disease.

While corticosteroids prednisone and deflazacort are known to improve muscle strength and function in patients with DMD and have been a frontline treatment for years, there is currently no universally accepted standard for steroid use in DMD. A global survey of physicians who treat DMD found 29 different regimens, with the most common being ten days on and ten days off. This intermittent dosing regimen was put in place in an effort to limit the potential side effects associated with prolonged steroid use in children, such as weight gain, stunted growth, and loss of bone density.

The Finding the Optimum Regimen of Corticosteroids for DMD (FOR-DMD) study was launched in 2013 to compare daily and intermittent steroid use and establish, from a clinical benefit and safety perspective, the most beneficial regimen for DMD patients. Griggs and Kate Bushby,

M.D. with Newcastle University initiated the phase 3 clinical trial conducted through the Muscle Study Group, an international network of muscular dystrophy researchers that Griggs helped create in 1997 to advance clinical research in neuromuscular disorders, including DMD.

The new study recruited 196 boys with DMD at 32 research sites across North America and Europe and followed them for three years. Participants were assigned to three groups consisting of daily regimens of prednisone or deflazacort, or intermittent prednisone, and followed for three years. The researchers found that the daily regimens of both drugs significantly slowed disease progression as measured by strength testing and muscle function, as compared to the intermittent group. While the daily regimen increased side effects overall, there were minimal serious side effects.

More information: Michela Guglieri et al, Effect of Different Corticosteroid Dosing Regimens on Clinical Outcomes in Boys With Duchenne Muscular Dystrophy, *JAMA* (2022). DOI: 10.1001/jama.2022.4315

Provided by University of Rochester Medical Center