



Approved January 24, 2013

Duchenne Muscular Dystrophy: Double-Blind Randomized Trial to Find the Optimum Steroid Regimen (FOR-DMD)

The Kennedy Krieger Institute and the Johns Hopkins School of Medicine are recruiting volunteers to participate in a multi-center phase III trial which will compare three ways of giving corticosteroids to boys with Duchenne muscular dystrophy (DMD). The trial is sponsored by the National Institute of Neurological Disorders and Stroke (NINDS) and aims to determine whether daily oral corticosteroid dosing (prednisone or deflazacort) is superior to intermittent corticosteroid dosing (prednisone) in terms of muscle strength and side effects.

We are looking for male participants who are between ≥ 4 - < 8 years of age, have a confirmed diagnosis of DMD, can stand up from the floor without assistance, and have not been on oral corticosteroids for more than 4 consecutive weeks.

Participation in the research study requires multiple clinic visits. Eligibility for the study will be determined with a screening evaluation. If eligible, each volunteer will be randomly assigned to receive one of three drug treatments for the duration of the study (up to 60 months). Follow-up visits will be scheduled after 3 months then every 6 months for the next 36 to 60 months (depending on when your child enters the study). Multiple tests will be done to determine the safety and effectiveness of the drug. These tests are routinely performed as part of the standard medical care for DMD.

Taking part in this research study may or may not improve your child's muscle function. There may be risks associated with the study drug and the tests performed in the study.

If you are interested in learning more about the research study, please contact Genila Bibat, MD, Research Trial Coordinator, at #443-923-2697 or e-mail bibat@kennedykrieger.org.

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Protocol Number: NA_00077118