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An exploratory study to assess two doses of GSK2402968
in the treatment of ambulant boys with
Duchenne Muscular Dystrophy (DMD)

The Kennedy Krieger Institute and the Johns Hopkins School of Medicine are recruiting volunteers with DMD to participate in a multi-center, phase II trial to test the safety of study drug, GSK2402968, at different doses, and its potential effectiveness in preventing loss of strength. The trial is sponsored by GlaxoSmithKline which designed the study drug, GSK2402968, to “skip over” exon 51 in the gene for dystrophin.

Male participants must be between 5-18 years of age, able to walk and have a mutation for which skipping exon 51 would predict restoration of the reading frame (deletion of exons 13-50, 29-50, 43-50, 45-50, 47-50, 48-50, 49-50, 50, or 52).

Participation in the research study requires out-patient visits. There will be a total of 30 visits within a 48-week period. After a 2-day screening to determine eligibility to the study, the participant will be randomized to receive either the study drug or placebo. Weekly injections will be administered for 24 weeks followed by a post- treatment phase. There will be tests to determine safety of the drug (including blood and urine tests, cardiac tests, renal ultrasound) and its effectiveness (muscle strength and function tests, pulmonary function, muscle biopsy of the leg, and MRI of the mid-thigh). Travel funds are available.



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Taking part in this research study may or may not prevent loss of muscle strength and 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 email bibat@kenedykrieger.org

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