JOHNS HOPKINS MEDICINE

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A PHASE 2 RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED, MULTIPLE ASCENDING DOSE STUDY TO EVALUATE THE SAFETY, EFFICACY, PHARMACOKINETICS AND PHARMACODYNAMICS OF PF-06252616 IN AMBULATORY BOYS WITH DUCHENNE MUSCULAR DYSTROPHY (DMD)

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Application No.: IRB00049415

The Kennedy Krieger Institute and the Johns Hopkins School of Medicine are recruiting volunteers with DMD to participate in a multi-center, phase II randomized, two-period, double-blind, placebo-controlled, multiple ascending dose study to evaluate the safety, efficacy, pharmacokinetics, and pharmacodynamics of the study drug, PF-06252616. The research study is sponsored by Pfizer, Inc. The study drug, PF-06252616, will to be evaluated to determine its ability to reduce the levels of muscle growth inhibitor, myostatin, and to increase muscle mass and function in DMD males.

Male participants with confirmed DMD mutation must be between 6 to less than 10 years of age and able to walk. Participation in the research study requires out-patient visits.

Approximately 105 eligible participants will be randomly assigned to 1 of 3 sequence groups for approximately 96 weeks (two treatment periods of 48 weeks). Three dose levels (5, 20, 40 mg/kg) administered every 28 days will be investigated in a dose escalating fashion in each study participant. At each dose level, dosing will be administered via a 2-hour IV infusion every 4 weeks for a total of 16 weeks (4 doses).

Taking part in this research study may or may not improve muscle mass or 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@kennedykrieger.org