JOHNS HOPKINS MEDICINE

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An Open-label, Multi-Center, 48-week study with concurrent untreated control arm to evaluate the efficacy and safety of Eteplirsen in Duchenne Muscular Dystrophy

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Application No.: IRB00040305

The Kennedy Krieger Institute and the Johns Hopkins School of Medicine are recruiting volunteers with DMD to participate in an open-label, Phase 3, multi-center, 48-week study to evaluate the efficacy and safety of a new study drug, eteplirsen, being developed by Sarepta Pharmaceuticals, Inc., in patients with Duchenne muscular dystrophy (DMD) with genetic deletions amenable to correction by exon 51 skipping (treated group). The study will also have a control group which will include DMD patients with genetic deletions not amenable to exon 51 skipping.

Eteplirsen is designed to "skip" a part of the gene that makes dystrophin called exon 51. For people who have changes, called deletions, in certain parts of the dystrophin gene, skipping exon 51 might potentially allow the body to produce a shortened, but still working, form of the dystrophin protein. The research study will test whether eteplirsen works to improve muscle function in people with DMD who have deletions that may be corrected by skipping exon 51.

In addition to other eligibility criteria, participants must be male with a confirmed DMD mutation, be between 7 to 16 years of age and able to walk. The duration of participation in the research study will be approximately 62 weeks and will require weekly out-patient visits along with possible overnight and/or out-of-state travel. Approximately 160 eligible participants will be included in this study at 39 different participating sites in the US.

Eteplirsen Treated Group – DMD males, with genetic deletion that may be corrected by exon 51 skipping, will receive a 30 mg/kg dose of eteplirsen via intravenous infusion once a week for 48 weeks. 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.

Untreated Control Group – DMD males, with genetic deletion that cannot be corrected by skipping exon 51, will not receive eteplirsen but will continue on his standard of care treatment regimen and will complete certain assessments during 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.