A double-blind, escalating dose, randomized, placebo-controlled study to assess the pharmacokinetics, safety and tolerability of single subcutaneous injections of GSK2402968 in non-ambulant subjects with Duchenne muscular dystrophy

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Description: This study is directed to assess the pharmacokinetics, safety and tolerability of drug GSK2402968 in non-ambulant subjects with Duchenne muscular dystrophy. This drug is designed to induce skipping of exon 51 in the DMD gene. The study will be conducted at Nationwide Children’s Hospital, in Columbus, OH.

To be considered to be part of the trial, subjects must:

1.) Be diagnosed with Duchenne muscular dystrophy.
2.) Have been in a wheelchair full-time for at least one year, but no more than four years. If a boy is walking, or uses a wheelchair only part-time, he will not be a candidate for this study.
3.) Have one of the following out-of-frame deletions in the DMD gene:
   - exons 13-50
   - exons 29-50
   - exons 43-50
   - exons 47-50
   - exons 48-50
   - exons 49-50
   - exon 50
   - exon 52

Subjects will undergo a screening that will include a DEXA scan and cardiology examination, along with blood tests. More than two weeks later, they will receive a subcutaneous injection of the study agent, after which blood samples will be drawn over 24 hours. They will need to return to Nationwide Children’s Hospital for follow-up visits one week and four weeks after the injection for further blood and urine tests, and examination by a study doctor.

Travel costs will be reimbursed under guidelines discussed with subjects before enrollment.

If you are interested in this study, please contact the Study Coordinator:

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