**A research study to evaluate eteplirsen in children with Duchenne Muscular Dystrophy (DMD)**

**Purpose of the Study:**

* To find out if a medication called eteplirsen can help boys with Duchenne Muscular Dystrophy (DMD)
* To find out if eteplirsen is a safe medication (few side effects)

**Who qualifies for the study:**

* Boys with DMD, between 7 and 16 years old
* No change in oral corticosteroid dose for at least 24 weeks
* Must be able and willing to follow directions necessary to complete all assessments

**We will investigate two groups:**

* **Treated group**: Boys who have an out-of-frame deletion that may be corrected by exon 51 skipping (deletion of exons 45-50, 47-50, 48-50, 49-50, 50. 52, 52-63)
* **Untreated group**: Boys who have an out of-of-frame deletion that may not be corrected by exon 51 skipping but potentially corrected by skipping another exon (e.g. exons 44, 45, 50, 53)

**What‘s involved:**

* Treated group: weekly intravenous (IV) infusions of eteplirsen, two muscle biopsies
* Untreated group: will not receive eteplirsen or biopsy but will complete all other assessments
* Physical examinations, blood samples and vital signs
* Repeated physical assessments including distance walked in 6 minutes, motor function, pulmonary function and heart echocardiogram

**Time commitment:**

* Study duration is approximately 62 weeks (14 ½ months)
* Physical assessments 2 days in a row, every 12 weeks
* For the treatment group: IV infusion of eteplirsen for 2 hours every week at UCLA

**Who can I contact if I wish to participate in the study?**

* **For more information, please contact Dr. Shieh’s office at (310) 825-3264 and ask for Juan Valderramos, or send an email to** **JValderramos@mednet.ucla.edu**