

We are conducting a research study in patients with Duchenne Muscular Dystrophy (DMD)

Study 4658-204

Purpose of the Study:

- We are doing the study to find out if a compound called eteplirsen can help people with the muscular disorder called Duchenne Muscular Dystrophy (DMD).
- We also want to find out if eteplirsen is safe to take without causing too many side effects
- This study is sponsored by Sarepta Therapeutics, Inc.

Who qualifies for the study:

- Male between 7 and 21 years old
- Been on a stable dose of oral corticosteroids
- Patients who cannot walk or are not able to walk 300 meters in 6 minutes
- Patients who have an out-of-frame deletion that may be corrected by exon 51 skipping (i.e. deletion of exons 45-50, 47-50, 48-50, 49-50, 50, 52, 52-63)

What's involved:

- We'll check your medical history
- It will involve physical examinations, and vital signs
- We'll evaluate a distance walked in 6 minutes, upper body strength and motor function

Time commitment:

- The total patient participation is approximately 104 weeks

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

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