**Research Study for Males 7 Years or Older with Duchenne Muscular Dystrophy**

**Investigational Drug for the Early Treatment of Cardiomyopathy in DMD**

**This study is being sponsored by Dr. Subha Raman at Ohio State University (Nationwide Children’s Hospital) with study sites at Cincinnati Children’s Hospital and in Los Angeles at UCLA (Mattel Children’s Hospital)**

|  |
| --- |
| **Frequently Asked Questions (FAQs) About This Study** |

**What is the purpose of the study?**

* The purpose of this study is to determine if eplerenone, a type of medication typically used for advanced heart failure patients, in combination with another medicine (ACE-I or ARB) can prevent heart muscle damage in people with Duchenne Muscular Dystrophy (DMD).

**Who is eligible for the study?**

* If you are a male with DMD, at least 7 years old and:
  + can undergo a cardiac MRI scan without sedation and follow breath-hold instructions
  + no kidney problems
  + not on eplerenone or other drugs like it
  + have preserved left ventricular systolic function
  + participation is open to boys at any stage of mobility if able to transfer into MRI scanner

**How long will this study last?**

* The study lasts for 12 months.

**How many study visits are there?**

* After the initial (baseline) screening visit, there are five study visits during a 12-month period.
* The study visits will happen at 2 weeks, one month, 2 months, 3 months, six months, 9 months, and 1 year.

**Where do the study visits take place?**

* The baseline visit will take place at UCLA. If you have had recent cMRI at Cincinnati Children’s Hospital or Nationwide Children’s Hospital you may be able to complete most study visits at UCLA.

**What will happen during this study?**

* Participants will be randomly assigned to receive either eplerenone (the study drug) or a placebo. A placebo is an inactive substance that looks like the study drug, but contains no medication.
* Participants will take assigned drug every other day for one month.
* If after one month, levels of potassium and creatinine (markers of kidney function) are normal, participants will continue to take the drug once daily for the remainder of the study.
* All participants will have blood drawn at the baseline visit as well as at 2 weeks and months 1, 2, 3, 6, 9 and 12.
* All participants will have a cardiac MRI scan at the baseline, 6 and 12 months.

**Is there payment for participation?**

* There is no payment for participating in the study but you will be reimbursed for parking and receive a gift card to cover expenses.
* The blood draws at 2 weeks, and at months 1, 2,3, 6 and 9 will be paid for by the study.
* The cardiac MRI scan at the 6 month visit will be paid for by the study. The scans at the baseline and 12 month visit may be paid for by the study if your insurance plan will not cover it.
* The study drug will be provided for free.

**Will there be access to the drug once the study has ended?**

* If the results are promising, your physician may choose to prescribe this medication to you or your child after the study has ended.

**Why should I consider participating in this study?**

* While no personal benefit can ever be guaranteed, there are other benefits to participating in a research study. You may be able to:
  + play an active role in your own health care (or that of your child)
  + gain access to new research treatments before they are widely available
  + have access to medical specialists who may not normally be available

and/or

* + help others by contributing to the better understanding and treatment of DMD-associated heart disease.

**Where can I learn more about this study?**

* You can learn more about this study at PPMD.com and/or ClinicalTrials.gov.
* You may also contact: Dr. Nancy Halnon at UCLA, (310) 825-5296 or nhalnon@mednet.ucla.edu

or

Dr. Subha Raman or Beth McCarthy at The Ohio State University, (614) 688-8020 or raman.1@osu.edu