

Research Study for Males 10 Years or Older with Duchenne Muscular Dystrophy

Research Study for the Early Treatment of Cardiomyopathy in DMD

Frequently Asked Questions (FAQs) About This Study

What is the purpose of the study?

- The purpose of this study is to determine if eplerenone or spironolactone, medications typically used for advanced heart failure patients, can reduce heart muscle damage in people with Duchenne Muscular Dystrophy (DMD) who are not taking steroids.

Who is eligible for the study?

- If you are a male with DMD, at least 10 years old and:
 - no kidney problems
 - not on eplerenone or spironolactone (but may be taking other heart medications)
 - not taking oral steroid medications (prednisone or deflazecort)
 - can undergo a cardiac MRI scan without sedation and follow breath-hold instructions
 - have preserved left ventricular systolic function

How long will this study last?

- The study lasts for 12 months.

How many study visits are there?

- There are up to 8 study visits during a 12-month period.

Where do the study visits take place?

- The baseline and 12 month visits will take place at UCLA and include heart imaging (MRI).
- Blood draw at 2 weeks, and 1, 3, 6, and 9 months can be done at a local laboratory where you reside.

What will happen during this study?

- Participants will be randomly assigned to receive a study drug, either eplerenone or spironolactone to be taken daily for one year.
- All participants will have blood drawn at the baseline visit as well as at 2 weeks, and months 1, 3, 6, 9 and 12.
- All participants will have a cardiac MRI scan at the baseline and 12 months (to be performed at UCLA).
- All participants will have pulmonary function tests at baseline and at 12 months.
- At the end of the 12 study we will ask you to provide information about your medical status for an additional year.

Is there payment for participation?

- There is no payment for participating in the study but you will be reimbursed for travel, food and lodging expenses for visits to UCLA for MRI.
- The blood draws at 2 weeks and months 1, 3, 6 and 9 will be paid for by the study and may be done locally.
- The cardiac MRI scan at the initial and 12 month visit will be paid for by the study.
- Pulmonary function tests performed at UCLA will be paid for by the study
- 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)
 - 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 ClinicalTrials.gov.
- If you are interested in participating or have questions, you may contact:
Dr. Nancy Halnon or Vanessa DePaz at the Children's Heart Center at UCLA, (310) 267-7667
- You may also contact the study sponsor Dr. Subha Raman at The Ohio State University, (614) 688-8020