EXPLORE DMD

A Seroprevalence Study

WHAT IS THE PURPOSE OF THIS STUDY?

EXPLORE DMD will enroll 100 individuals affected by Duchenne to see how many of these individuals have specific types of antibodies (protein) in their blood. This information may help Sarepta understand how many people to expect to be able to treat in the future with certain medical (gene) therapies developed by Sarepta.

What is an antibody?

Antibodies are proteins in your blood that the body develops to help fight off an infection or other particles introduced into your body that are sensed as harmful. Antibodies are an important part of building immunity. Once they develop, antibodies often remain to some extent in the person's body and could help fight off the same or similar infection/particle in the future.

Why is Sarepta performing this study?

Sarepta is currently researching investigational gene therapies that utilize the AAVrh74 vector. A vector is a component of gene therapy that helps deliver a gene therapy into a cell. The EXPLORE DMD study will test for antibodies associated only with this vector. This will provide valuable information on the rate of antibodies to AAVrh74 vector in people with Duchenne who have never had gene therapy. This study will also provide additional data that Sarepta will use for future development of research programs.

Why would I participate in this study?

In addition to supporting research & development in the gene therapy space, some individuals have expressed a curiosity if they may have antibodies to the AAVrh74 vector. Having an antibody to AAVrh74 vector is not harmful to yourself or others. Study participants who have pre-existing antibodies to the AAVrh74 vector may not be able to participate in clinical studies or approved therapies using this specific vector.

Will the study tell me if I have antibodies to vectors being studied in other gene therapy programs?

No, the study is specifically looking at antibodies to the AAVrh74 vector that is utilized with the gene therapies in development at Sarepta. Currently, there is not a single test that screens for all antibodies to vectors that may be used in other gene therapies in development

How do I qualify for the study?

The study is enrolling boys (ages 4-<18) with a confirmed diagnosis of Duchenne muscular dystrophy (any mutation). Individuals residing in the U.S.A are eligible for participation.

You would not qualify for the study if you:

- Have received prior treatment with any gene transfer therapy
- Live with a person who has been exposed to any gene transfer therapy

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How does the study work?

VIRTUAL STUDY: No travel or in-person visits required. The study will be completed online and managed by a single nationwide site called the Virtual Research Coordination Center (VRCC).

QUESTIONNAIRES: You will provide your written consent (approval to participate in the study) and answer a few questions about yourself to the study team at the VRCC. The study team at the VRCC is available to answer any questions you have about your participation.

MEDICAL INFORMATION: Your physician will be asked by the study team at the VRCC to provide medical information about you and your health, including your diagnosis and your genetic test results.

SINGLE BLOOD DRAW: To be completed at a local laboratory or within your home (you choose).

How will I find out about my results?

Results of the blood test will be available approximately 3 weeks after you provide a blood sample.

You will receive an email alert that your results are available.

Your referring physician will receive the results and will review them with you. You may also elect to not learn your results from the study.

You may decide to include a community support specialist from Parent Project Muscular Dystrophy (PPMD) when you receive your results from your physician. While including a community support specialist is not required as part of the study protocol, PPMD is providing you the option of this service to help you and your physician better understand your results and potential impact on future treatment options. While you may elect to have a PPMD community support specialist join you and your physician for the results conversation, this may not be necessary if your referring physician is a neuromuscular specialist with gene therapy expertise. Participants who have completed the EXPLORE DMD study and discussed results with their physician also have the option to schedule a separate, follow-up call with a community support specialist from PPMD. The PPMD community support specialist may be a certified genetic counselor, a nurse, or researcher from PPMD.

To learn more about participation in the study contact the Virtual Research Coordination Center:

Study Physician: Jeffrey J. Klein, MD
Email: Explore.DMD.sm@ppd.com
Telephone: 1-877-302-2876

Is my participation in the study linked to any current or future clinical studies?

No, participation in EXPLORE DMD does not guarantee eligibility or ability to participate in current or future clinical studies regardless of your study results. It should be noted that your antibody status may change over time, and therefore if you are considering other clinical studies or approved gene therapy utilizing the AAVrh74 vector, you will need additional antibody testing to determine your eligibility.

Parent Project Muscular Dystrophy

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EXPLORE DMD is sponsored by Sarepta. Sarepta has partnered with PPMD to provide additional support and information to families who request it.

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