Letter from the Jain Foundation

Thank you so much for your willingness to participate in this critical study. Your participation directly affects the progress towards future therapies. Without your involvement and continued commitment to this study, there won’t be information needed to run successful clinical trials of therapeutic interventions. The final study results will also provide physicians with important information about this disease and help them better care for you.

This newsletter contains updates on the progress of the research, what we have achieved so far, and how we can work together to make the study as beneficial and successful as possible. Please take time to read it fully.

Thank you again for partnering with us in this pivotal research milestone. This clinical outcome study is the crucial first step that will give us the understanding and knowledge necessary for clinical trials to be developed. You are a hero to the community and are making a difference!

The Jain Foundation Team

Oversight and funding for COS is provided by the Jain Foundation. The Jain Foundation is a small family foundation that focuses solely on identifying treatments for dysferlinopathy (LGMD2B/Miyoshi). Find additional information at www.jain-foundation.org

A first look at the data...

We have already begun to analyze the data we have collected so far and are very pleased with the results we are seeing.

What we’ve learned: There was barely any change in the results between the screening and baseline visits, which is what we expected because there wasn’t much time between these two visits. This is very exciting because it means that we’re on the right track and should be able to get reliable results from the study. The consistency in the data shows that the tests are being carried out to the same high standard.

Participant Quote:

“It has been an awesome heartwarming experience to volunteer and work with experts to research this rare condition. I feel honored to be a part of the study, continuously learning more about the condition while aiming to help come up with potential treatments and a possible cure for later generations. I have a more optimistic view on life and am so hopeful about the future.”

To consider for your next visit...

- The integrity and accuracy of the data we record is incredibly important. The journal provided to you by your COS center is a very useful tool for tracking your activity levels and medications. Please make use of it to ensure that the information you provide is as accurate as possible.

- To ensure similar conditions for each visit, please come to each visit rested and wear similar shoes and bring any necessary walking aids.

Make your data count!!!!
Please be sure to complete all 6 COS visits

All six stages of testing are crucial for the success of the study. To miss a stage would mean that your data could not be used in the final analysis.

Thank you for helping us collect valuable research tools!!!!!

We’d like to give a special thanks to everyone who has donated blood and skin for the biobank. So far, 75% have donated blood and 30% have given skin samples.

All samples are vital given the low number of identified dysferlinopathy patients. Blood analysis will allow us to identify disease markers that can be used for non-invasive monitoring of the disease during clinical trials. The skin samples can be used to study the effects of possible treatments on different types of dysferlin mutations. Currently, researchers only have access to a small number of patient cell samples, so the skin samples collected in COS are very important for studying your mutations. The more samples we have, the better our analysis will be.

It’s not too late to donate. Please consider donating blood and/or skin if you haven’t already.

Want periodic updates from the Jain Foundation?
Go to www.jain-foundation.org/COSupdates and sign up!

Have a question or comment for the Jain Foundation team or a quote for the next newsletter?
Email us at contact@dysferlinoutcomestudy.org
Thank you for registering for the International Dysferlinopathy Registry (IDR). The information in this registry will allow researchers preparing new clinical trials to identify eligible patients. Completing your information in the IDR is the easiest way to allow clinicians determine if you are eligible for their studies.

If you haven’t already filled out the second questionnaire, please go to the website (http://www.dysferlinregistry.org) and log into your account. Once you have logged in, click on “My questionnaires” in the menu on the left and then click the questionnaire 2 link to add your information.

In order to get questionnaire 3 completed for you, please click the “My doctor(s)” link in the menu on the left and add your Clinical Outcome Study doctor. By doing this, your COS physician will be able to provide the medical information necessary for your IDR registration.

Once you have finished questionnaire 2 and added your COS doctor, your registration will be completed for you.

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**Participant Quote:**

“In 1981, when I first started to have symptoms, no genes that caused MD had been discovered yet. Few if any neurologists knew what to make of my symptoms. They seemed to be annoyed at me for being such a confusing case and nobody contemplated any treatments in the foreseeable future.

Today, there's a clinical study of my type of MD involving nearly 200 patients worldwide. It has been a long time (longer than I had wished, of course). But things have come a long way.”

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**Participant Quote:**

“I wanted to thank the Jain Foundation and Dr. Sparks team in Charlotte N.C. for another great experience with participating in the International Dysferlinopathy Clinical Trial. Am very excited to watch what this amazing Foundation can accomplish with the information gathered.”

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**Study Site Location**

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We have recruited 194 participants for COS! Do you know where all 14 study sites are?

We are happy to announce that we have recruited 194 individuals, well surpassing our original goal of 150. This is wonderful news as the more participants we have, the more data we have to analyze. We couldn’t have done it without you! It is amazing that so many people with this rare disease are working together to make this study a success.