Limb-girdle Community Bulletin

Addressing top community questions after Sarepta's first-ever Facebook Live event

Dear Limb-girdle muscular dystrophy Community,

On Tuesday, July 27th, Sarepta hosted a Facebook Live event dedicated to genetic testing education. We were pleased to receive many comments and questions and are providing a community bulletin to give responses to common questions. Some of the questions are also addressed in the Facebook Live video, and we encourage you to check it out if interested. The event is still available on our Sarepta Facebook Page. *As a disclaimer, please refer to your physician/healthcare provider or www.clinicaltrials.gov if you wish to learn more about Sarepta's clinical trials and studies.*

I was genetically tested years or decades ago for LGMD; should I be re-tested?

This is a very important question that the LGMD community is consistently asking. There have been improvements in genetic testing methods therefore it may be worth testing again to receive more current results. In some cases, there may now be more information available for a particular result (or variant) in terms of whether it causes a particular condition. So, for example, what was previously classified by the lab as a Variant of Uncertain Significance (VUS) could be reclassified from uncertain to known significance. If a variant classification changes or improves, a genetic testing program typically follow up with the ordering healthcare provider through an updated report. For a list of LGMD genetic testing resources, US community members are encouraged may visit www.limbgirdle.com/genetic-testing. International patients may send a note to Advocacy@Sarepta.com to learn about genetic testing and advocacy resources to help patients navigate testing.

I received a diagnosis for LGMD as a teenager but did not have genetic testing to determine my subtype. Would I meet the criteria for genetic testing through one of these programs? Yes. These programs test for all the known LGMD genes to try to determine subtype.

My child had an elevated CK test last month and I'm wondering if that is enough to access testing? Yes, an elevated CK result is considered an indication for testing and as we mentioned earlier, important for your child's doctor to note any other relevant clinical findings.

I was tested through a testing program the MDA more than 5 yrs. ago and got an uncertain result, does retesting make sense? Yes, test methods and variant information have evolved in the last 5 years and the programs also have clearer next steps to take when test results are uncertain.

What is the status of Sarepta's clinical trials and studies? The focus of the Facebook Live event was on genetic testing education; however, we understand that many in the LGMD community have questions on Sarepta's pipeline. Individuals impacted by LGMD and their families wishing to learn more about work are encouraged to join us on Saturday, September 18th 2021 at 12pm EDT. Sarepta is a proud sponsor of the 2021 LGMD International Conference and Executive Vice President and Chief Scientific Officer, Louise Rodino-Klapac, Ph.D., be presenting a 60-minute

presentation our work. If you wish to join us please register on the conference's website: https://www.eventbrite.com/e/2021-international-limb-girdle-muscular-dystrophy-conference-registration-142222189397.

Sarepta was thrilled to have community engagement during the Facebook Live event. In case members of the patient, caregiver or advocacy community missed the event they may visit our Facebook Page: https://www.facebook.com/sareptatherapeutics/

Feel free to send us your feedback and any ideas for future events that you'd like to see. You may contact us at Advocacy@Sarepta.com.

Additionally, if you are interested in receiving Sarepta news in the future, consider following us on social and joining our email list-serve at <limbgirdle.com/stay-connected> *This website is intended for U.S. audiences.*