SAREPTA THERAPEUTICS

U.S. Patient Affairs

Newsletter for the LGMD community

Summer 2021

Letter to the Community

Dear LGMD Advocacy Leaders,

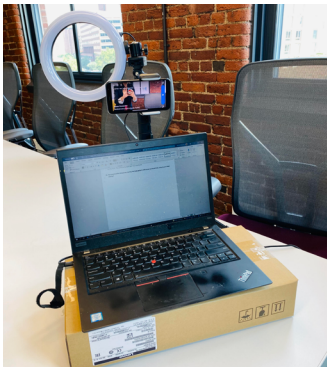
We're pleased to bring you program highlights from winter and spring 2021. While Sarepta's Patient Affairs work continues virtually, we are hopeful for continued connectivity this year and eventual in-person engagements in the future. This is an exciting year for Sarepta and the community; we look forward to the bi-annual LGMD International Conference, engaging in new ways via social media platforms, and continuing to advance our LGMD pipeline. Please feel free to reach out to our team at any time by emailing advocacy@sarepta.com. We would be happy to hear from you.

Wishing you all a wonderful summer.
-The Sarepta US Patient Affairs Team

Social Media Engagement

Sarepta's first-ever FACEBOOK LIVE event!

Sarepta Patient Affairs is pleased to announce that our first Facebook Live Event (ever!) kicked off this summer and we had tremendous engagement on social media from the community. We featured a conversation with Sarepta's Medical Affairs colleague to discuss the topic of genetic testing. This event is living on our Facebook page, and you are free to share it with your communities. We encourage the community to follow us on our social media channels to stay updated on potential future Facebook Live events.

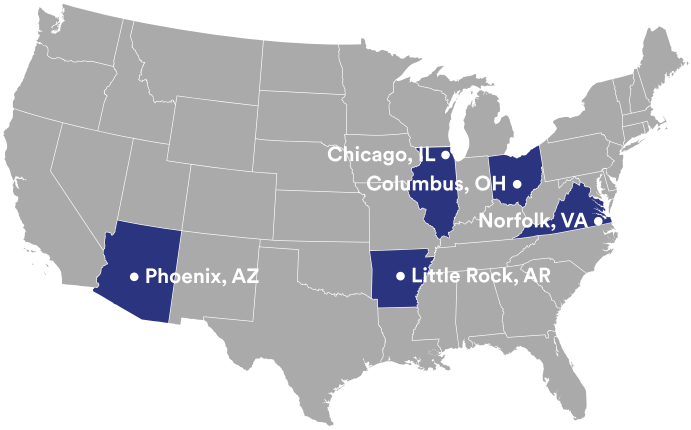


LIVE

LGMD Study Updates

> **"Natural history studies provide foundational understanding of how the disease impacts people to facilitate development, approval, and ultimate availability of meaningful treatments."**
>
> – Donna-Lee Destouche, Director, Clinical Operations at Sarepta

JOURNEY: Sarepta's Study of the Natural History of Patients With LGMD2E/R4, LGMD2D/R3, and LGMD2C/R5, ≥ 4 Years of Age, Who Are Managed in Routine Clinical Practice. New sites in the United States have been activated, and we encourage the advocacy and patient community to visit clinicaltrials.gov to see the full list of sites (NCT04475926). Participating in a potential natural history study allows the participant to be assessed for potential future treatment trials including gene therapy. The map below highlights the sites activated as of this spring 2021 with more global sites to be added in the future. If your organization receives inquiries you are welcome to refer them to email either clinicaltrials@sarepta.com or advocacy@sarepta.com or call 800.690.2003 to learn about how they may potentially enroll.



LGMD Study updates *(cont.)*

Sarepta was pleased to announce LGMD2E/R4 data at the March 2021 Muscular Dystrophy Association (MDA) Conference. Sarepta presented a variety of data at the 2021 Muscular Dystrophy Association (MDA) Clinical and Scientific Conference, including two-year data on gene expression for SRP-9003, our investigational gene therapy for the treatment of limb-girdle muscular dystrophy type 2E (LGMD2E/R4). These data are encouraging and support the continued development of our LGMD2E/R4 research program.

Sarepta's Qualitative Dyadic ("Dyad") Interviews with the Sarcogycanopathy Patient/Caregiver Community has Fully Recruited all Patient Participants!

In January 2021, Sarepta launched Qualitative Interview Study with patients (LGMD2C/R5, LGMD2D/R3, or LGMD2E/R4 subtypes) and their caregivers to help us better understand the impact of living with an LGMD diagnosis. This type of interview has the potential to generate a richer understanding of experiences and perspectives of those living with a rare disease such as limb-girdle muscular dystrophy. It may also help inform optimal patient-centered outcome assessment strategies for the evaluation of symptoms, disease impacts and health-related quality of life in LGMD clinical trials. We are grateful for the collaboration with the advocacy community to recruit individuals to participate in this work. We are pleased to share we had many inquiries and we look forward to analyzing the data.

Pipeline Update

We continue to advance our gene therapy pipeline for LGMD. Our clinical work will advance for subtypes LGMD2E and LGMD2B, and our toxicology work for the LGMD2C and LGMD2D programs will also advance. Apart from our clinical and toxicology work, Sarepta continues to dedicate resources to advancements in gene therapy manufacturing elements that will impact our entire LGMD portfolio. These commercial manufacturing capability developments are critical for both our Duchenne and limb-girdle muscular dystrophy programs. Sarepta's work to develop best-in-class gene therapy manufacturing aims to ensure utility of the clinical data and aims to address key issues according to FDA. We look forward to sharing an update on our work at the LGMD International Conference this September.

> **"While every aspect of gene therapy product development is critical, FDA has identified product manufacturing as the most crucial issue to be addressed."**
>
> – Peter Marks, MD, PhD.
> Director, CBER, FDA.
> May 2019

PROGRAM NAME	DISCOVERY	PRE-CLINICAL	CLINICAL	COMMERCIAL
SRP-9003 (LGMD2E β-sarcoglycan)			■	
SRP-9004 (LGMD2D α-sarcoglycan)			■	
SRP-9005 (LGMD2C γ-sarcoglycan)		■		
SRP-6004 (LGMD2B Dysferlin)			■	
SRP-9006 (LGMD2L Anoctamin 5)		■		
Calpain 3 (LGMD2A)		■		

Meet the Sarepta LGMD Team

We were thrilled to have a conversation with Donna-Lee Destouche, Director in Clinical Operations to learn more about natural history studies and how she is passing the time during the pandemic!

Why is the Natural History Study important, especially when researching rare diseases such as LGMD?

The Natural History Study of a rare disease like LGMD is important because this data has the potential to help drug developers understand how the disease may impact people over time using clinical data.

How does a Natural History Study advance LGMD research?

There are two major ways: one is that the data from the Natural History Studies may be used to design drug studies focused to collect specific evidence.The second is that the Natural History Study data may be used to compare how a therapy might change the progression of disease versus not having any therapy.

What is your favorite thing about working in clinical development on the Natural History Study specifically?

From a Clinical Development perspective, I like working together with a team towards a singular goal of ensuring the patient comes first, that we complete high quality studies while maintaining data integrity with the goal of bringing treatments to patients. I enjoy Natural History studies because of the scientific knowledge that can be added in a specific therapeutic area that can impact how treatments are developed. Natural History studies are typically conducted in disease states that do not have a significant amount of information available, so they have potential to have a big impact.

JOURNEY is the name of the Sarepta Natural History Study; can you share a little background on why the team chose this name?

JOURNEY represents understanding the course of Limb Girdle Muscular Dystrophy through time. By including people at different stages of the disease, the study aims to chart what the disease trajectory is for someone with LGMD at each stage in life. The team would like to understand the Journey that people with LGMD are undertaking to make sure we understand and can design studies and provide critical data. *(cont.)*

Meet the Sarepta LGMD Team *(cont.)*

Wrap up questions

What has been your favorite pandemic pastime?
Drawing has been my favorite re-discovered activity. I had not done this in such a long time and started again during the pandemic.

What are you most looking forward to this summer?
I am looking forward to being outside more since the weather is nice. I enjoy nature so I am planning to find places that I can sit and draw outside.

Night owl or morning bird?
Morning bird, I enjoy waking up and completing tasks earlier in my day so I am free later to relax.

LGMD Announcements

International LIMB GIRDLE MUSCULAR DYSTROPHY Conference

A Virtual International Event

September 17-20, 2021

We look forward to sharing updates on our LGMD pipeline and movement in our preclinical and clinical work. Sarepta will be presenting at the Speak Foundation's International LGMD Conference in September 2021. We are honored to be a Platinum Level Sponsor and look forward to engaging virtually with the advocacy and patient community throughout the 3-day event.

Gene Therapy Frequently Asked Questions

Check out our Gene Therapy Frequently Asked Questions Video Series on Sarepta social media pages. This work kicked off in 2020 and we are excited to continue to provide video content in 2021 to educate the community on the most common questions. You can find the Season 1 GT-FAQ playlist on Sarepta's YouTube channel online at Sarepta Gene Therapy FAQs.

Community update: Rare Lessons Award Winners

In July 2020, Sarepta launched Rare Lessons, a rare disease lesson plan contest to promote rare disease learning in the K-12 classroom. In February 2021, we announced the 4 winning lesson plans and published them on sharemylesson.com. Each winning lesson plan author received a $2500 award and $2500 was awarded to their affiliated educational institution. Learn a little about our winners below. We were thrilled to have representation from the LGMD community as one of our esteemed judges and have an individual living with LGMD, Jessica Keogh, be awarded one of the four prizes for her work on creating the "Empathy" lesson plan.

LESSON PLAN:
"Not Weird, Just Different"

"Children are inherently curious and kind and the more they know about rare disease the easier it is for them to show kindness and acceptance toward someone who has a rare disease."

Kaitlin Payne
K-6 Teacher, South Topsail Elementary School, NC

LESSON PLAN:
"The Mystery of Rare Disease"

"As a teacher, advocate, and Charcot Marie Tooth disease advisory board member, I'm proud to find ways like these to help improve the school and life experiences of students of all walks of life and abilities!"

Sarah Kesty
Education Specialist, Castle Park Middle School, CA

LESSON PLAN:
"Rare Diseases Lesson"

"Only by educating and bringing these cases to the forefront, can we progress to create a fairer and more equal society. That is why I chose to enter this competition."

Laura Doyle
Middle School Science Teacher
Atlanta International School, GA

LESSON PLAN:
"Empathy"

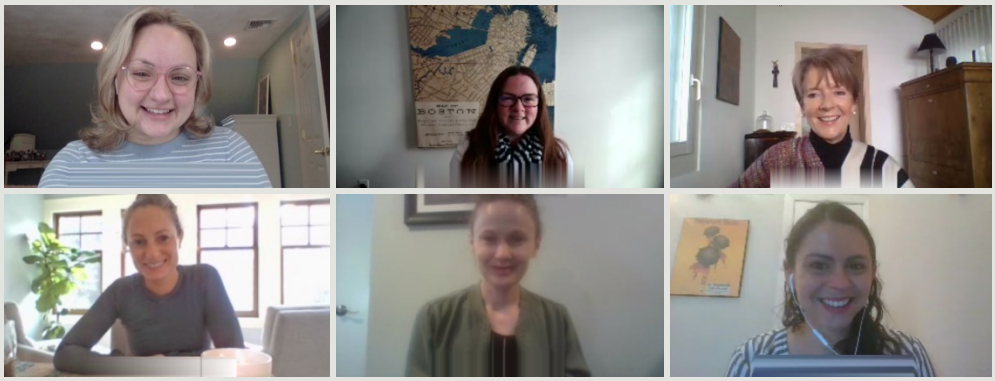
"Including disability as part of the curriculum is essential in creating a more inclusive and empathetic world. Representation matters!"

Jessica Keogh
Special Education Teacher, Middle School
West Chester Area School District, PA

Check out sharemylesson.com to view and download the Rare Lessons winning plans!

Let's Stay in Touch!

Please feel free to reach out to us at advocacy@sarepta.com with any questions or concerns.



Members of the Sarepta Patient Affairs team wearing stripes to recognize Rare Disease Day 2021