

Cure FSHD For All Newsletter

Summer 2026

2026 IRC Conference

For years, early-onset FSHD wasn't talked about nearly enough. Now, we're seeing more research focused on kids and people with more advanced disease progression. That's progress, but as a teen living with FSHD, I know we need more than presentations and posters.

We need action.

This June, the International Research Congress on FSHD included several presentations on early-onset FSHD, including four posters presented by FSHD moms Debbie Eggleston and Ally Roets. Research on kids and non-ambulatory individuals is important. We need that research to move forward, and we need clinical trials that include kids and non-ambulatory people with FSHD.

Ally will continue working with the CTRN team toward publishing pediatric and non-ambulatory data from the MOVE study. Contact ally@fshdcure.org about the analysis of pediatric or non-ambulatory MOVE data.

Contact debbie@fshdcure.org with any questions or input about a retrospective natural history of childhood-onset FSHD or video assessment.

Mission

Cure FSHD For All is dedicated to advocating for access to treatments for all individuals with FSHD, regardless of age of onset, severity, or ambulation status.

2026 Connect Conference

At Connect, patients and their family members had the chance to come together and share their personal experiences with researchers and have their voices heard. Kids and young adults spent time with pharmaceutical company representatives while solving an escape room together. Later, we were on a panel to share messages from our FDA testimonies. We had the opportunity to go to the Shedd Aquarium, where we had a penguin encounter. We got to pet the adorable (and very loud) penguins, and learn about their training.

We chatted with other kids with FSHD. It was a unique and important opportunity to hang out with others who understand what we deal with and who go through similar experiences. The two of us have been working together for a while on advocacy efforts, but the Connect Conference was the first time we actually met in person. We had a lot of fun chatting and joking together.

We also had the chance to learn from adults who spoke to us about finding jobs and building a career while living with FSHD. This gave us real examples of people who show that FSHD does not stop us from reaching our goals or finding a career we enjoy.

Quote from a Professional

"Children and adolescents with early-onset FSHD should not be an afterthought in drug development. From the beginning, clinical development programs should consider how younger patients can be included in trials and what is needed to make that inclusion possible. If we wait until therapies are approved for adults before beginning to study them in children, we delay access for the patients who may have the most to gain from an intervention. Early-onset FSHD needs to be part of the clinical development strategy from day one: with appropriate endpoints, study designs, and regulatory planning built in from the start."

-Michelle Mellion, MD



Spotlight

Luna Klein Heerenbrink

Luna Klein Heerenbrink is 15 years old and from a small village in the Netherlands called Spijk. She likes drawing and baking and wants to study criminal psychology someday. She grew up familiar with FSHD because her mother and grandmother both have it. She was diagnosed in 2020.

When Luna was in fourth grade, she started tripping on her own feet. She would say, "I will trip over my shoelaces even when I'm wearing velcros." Then, she started having a little bit of scapular winging. Now, her shoulder and scapular weakness affects almost everything that she does.

The symptoms that bother Luna the most are the weakness in her hands, shoulders, and neck. Her hands tire quickly, so it is hard to hold heavy objects and sometimes she drops things. She can't raise her arms very high so doing anything above shoulder height is difficult or impossible. Her neck muscles get extremely tired due to compensating for her shoulder loss. Sometimes after leaning her head back, she can't lift it back up without help.

Even though FSHD is tremendously difficult, Luna has grown in several skills due to that difficulty. She has learned to set her boundaries and advocate for herself.

There are no clinical trials in the Netherlands for people of Luna's age. Luna wants pharma representatives and researchers to visit FSHD Days and listen to the patients.

COMING SOON: MyFSHD Podcast Episode

Last summer, we had the opportunity to join Peter Jones and a few other teens on the My FSHD Podcast to talk about our experiences living with FSHD as teens.

We just recorded another episode together! We're grateful to Peter for this opportunity and for being such a great advocate for those of us with early-onset FSHD, as well as the FSHD community as a whole.

The new episode will air soon!

CTRN Meetings

This week, my family members with FSHD are heading to San Antonio to volunteer at the FSHD CTRN clinical evaluator training. If you're attending the Investigator Meeting, I'd love to meet you! Please come say hi to me and a few other kids who are traveling to Texas to support FSHD research. We'll be at the taco bar gathering! -Maggie

2026 Connect Conference



FDA Early-Onset Listening Session

On July 1, after months of preparation, we joined fellow advocates in an FDA Patient Listening Session on early-onset FSHD. The FDA heard from people living with early-onset FSHD, two mothers (one of whom lost her daughter to the disease), and a pediatric neurologist. The testimony was candid and detailed. It included both data and personal experience.

Ally Roets led this advocacy effort, making clear to the FDA that early-onset FSHD is both burdensome and life-threatening. Dr. Butterfield added his perspective as a pediatric neurologist who still has no treatment to offer his patients with FSHD.

We are grateful for this opportunity to educate and advocate, and we thank everyone who shared their powerful stories. The FDA will post its report on its website. If you'd like a summary emailed to you, please contact ally@fshdcure.org.

Education

In the summer of 2024, I came close to entering a clinical trial through expanded access. After months of waiting, my doctor called while we were flying home from vacation to tell us the FDA had approved me. For the first time, I had real hope that we could slow or even stop this disease, and we began planning our trip to Rochester to start treatment. But the study was pulled just as we were preparing to go. That was two years ago, on August 25th, 2024. I was 13. Since then, we have repeatedly asked for patients like me to have a chance, but the answer has always been no, while I continue to lose muscle and independence. I understand that companies face difficult decisions and that clinical trials have rules, but getting a drug approved should not have to mean leaving patients behind. Expanded access could give patients like me hope while also helping researchers learn about people with FSHD who are not represented in traditional trials. There are scientific, business, and human reasons to study us, and we are running out of time and function while we wait. We are asking one company to be brave enough to create a responsible expanded access pathway and study patients who do not fit neatly into existing trials. I don't want special treatment; I want the opportunity to fight for the future every 15-year-old should get to imagine. Two years ago, I came close enough to that hope to almost touch it. **I am still waiting for one company to be brave enough to give it back.** -Kate



For questions, input, or collaboration opportunities, please email communications@fshdcure.org

www.curefshdforall.com

Signing off for the season,

Maggie Kate

