

Cure FSHD For All Newsletter



Spring 2026

Happy World FSHD Day!

Our Advocacy Work

Maggie: Empowering the Next Generation

- **Connect Conference:** Leading my second year of kids' activities to build resilience in our youngest members and give them a chance to meet scientists who are working towards a cure
- **Capitol Hill:** Met with lawmakers to ensure rare disease funding remains a top federal priority and encouraged them to create a resolution declaring June 20th as FSHD Day
- **FDA Listening Series:** Currently writing and sharing my personal story for the FDA to ensure the "Voice of the Patient" guides decisions
- **FSHD Teen Group:** Working with the FSHD Society to create a Gathering Space group for teens with FSHD to connect and support each other

Kate: Making the Invisible, Visible

- **"Against the Current":** Featured in a documentary on FSHD and sports, which has already raised over \$10,000 for the adaptive sports community.
- **Media & Industry:** Interviewed by NBC Boston News and spoke at **Dyne Family Day** to humanize the patient experience for industry leaders.
- **FDA Advocacy:** Joining Maggie in the **FDA Listening Series** to share my hopes and the daily realities of FSHD with those who approve new treatments.

We are both active participants in the **Move Peds** study. We don't just talk about progress; we provide the data to make it happen.

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.

How you can help

The clinical trial model feels like it was built for a different time. We've been hearing about recent FDA guidance that supports more decentralized and patient-focused clinical trials—like using remote monitoring, wearable devices, telemedicine, and doing more visits from home instead of always going into a clinic. To us, this feels like a real step toward making trials match how people actually live now.

We're curious how sponsors and researchers are actually using this in upcoming FSHD trials—whether it's changes to existing studies or how new ones are being designed.

We're wondering:

- Will things like wearable data or remote monitoring actually be used in FSHD trials? (A lot of us already wear devices every day, so this feels like a pretty natural fit.)
- Which parts of trial visits could realistically happen at home instead of in a clinic?
- How do we, as a community, make sure data from wearables is strong enough for the FDA to accept?

Digital and decentralized trials can move faster, make it easier to stay in studies, and include more people who don't live near major hospitals. But most importantly, they let us be part of research in a way that actually fits our real lives—school, friends, family, and everything we're already juggling.

We'd really love to hear how you're actually putting this into practice. Email us your thoughts or questions—we want to help move this forward together.

FDA Early Onset Listening Session

On July 1st, 9 participants will be partaking in an FDA Listening Session, with the purpose of spreading awareness of early onset FSHD. We will be discussing that FSHD is not a disease that is easy to tolerate. Early onset patients are the most severe and are at risk of respiratory compromise and early death. Therefore, inclusive trials and programs like expanded access are absolutely essential. This is not an adult disease, and those who are burdened with symptoms since childhood have the worst to bear and the most to lose. Our hope is that our stories help the FDA understand the importance of inclusive clinical trials, and inspire them to give you the conviction to create them. Please let us know if there is anything else that you feel is important for us to mention when speaking with the FDA.



Spotlight

Dr. Justin Cohen, PhD



Living with Early Onset FSHD is terrifying. Dr. Justin Cohen is proof that people with our condition have to work a second, full-time job just coordinating the logistics of daily life—mapping out accessible routes, fighting for basic inclusion, and planning every movement before we can even think about just being a student. Dr. Cohen faced those hurdles when he left home for Tufts University. However, he never let Boston's brutal winters or inaccessible historic buildings stop him; he advocated for himself, worked with the university to clear his specific wheelchair paths, and proved that independence doesn't mean doing everything alone; it means having the courage to direct your own path. When muscle weakness in his upper body threatened his dream of working in medicine, Dr. Cohen pivoted to biomedical research and completely redefined what it means to work in a laboratory. While earning his Ph.D. at Drexel University, he utilized a lab technician to act as his physical hands, allowing his mind to focus on experimental design and complex data analysis. Today, as a researcher at Yale, he actively hunts for a cure for the very disease we share. Hearing his story showed us that while our bodies might face physical boundaries, our goals don't have to.

Justin's story made us realize the clock is ticking fast for early-onset patients. Traditional clinical trial designs are shutting us out, even though our rapid progression means we could be the key to helping prove treatment efficacy much faster than traditional adult-onset patients. All patients with FSHD, no matter the severity, have muscle to lose. True inclusion requires absolute urgency from pharma executives and researchers to think out of the box to find accommodations that will create success for us all. Because of Dr. Cohen, we are less afraid of college and living on our own, and his accomplishments gave us inspiration to dream big about our own future—but it is up to the medical world to ensure we actually live to see it.

Years ago, a doctor told Justin's parents not to expect much from him. Today, he is at Yale rewriting the future of genetic medicine. His story is proof that our condition does not define our capability. We are ready to fight for our education, our independence, and our dreams, just like Justin did. Our only question left for the medical community is simple: Are you ready to fight just as hard for us?

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

www.curefshdforall.com

Signing off for the season,

Maggie Kate