

Setting the Board for Success

EVERY MOVE COUNTS





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**SETTING
THE BOARD**

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**FSHD
Advocate**

The FSHD Society is the world's largest research-centered patient advocacy organization focused on facioscapulohumeral muscular dystrophy (FSHD). This genetic condition affects one million people worldwide and causes lifelong, progressive muscle weakness that can result in significant pain and disability. We are accelerating the development of treatments, empowering and activating the FSHD community, and making sure no one has to face this disease alone. The Society does not endorse any of the drugs, procedures, treatments, or products discussed in its reporting. We urge you to consult your physician about any medical interventions.

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Setting the board for success

Every move counts

Developing a new treatment and bringing it to market are not for the faint of heart. Drug development is one of the most complex endeavors known to humanity – a chess grandmaster-level undertaking of tactics and strategies. Drug development demands brilliant insights and flawless execution at every step, from science and technology to navigating the myriad pitfalls in the path from clinical trials to regulatory approval, and, ultimately, delivery of affordable treatments to every person who needs them.

These strategies began with a vision – a dream of something greater. Turning that vision into reality has required much more than just passion. It demands clarity, commitment, and resilience.

Launched 34 years ago, the FSHD Society had a vision of a world free from the devastating effects of FSHD. Back then, little was known and less was being done to discover the cause and cure for the disease.

But a vision, however noble, remains no more than a wish without the determination to follow through. So a small group of thoughtful, committed people came



Mark A. Stone

together to catalyze researchers, clinicians, government agencies, and the biopharma industry. Their passion and persistence have brought us to this place in history.

While the game plan hasn't been easy, the challenges we face together have made us smarter, tougher, and more resilient, allowing us to overcome the inevitable obstacles, disappointments, and setbacks that have assailed us on this journey.

As we reflect on how far we have traveled, we see that in those tough moments, the difference between the vision

and the reality is the will to persist. It's about holding onto the belief that every step brings us closer to our destination.

As you read this issue of *FSHD Advocate*, you will learn about the strategies that guide all our endeavors, along with the can-do attitude that is hard-wired into the FSHD Society. Carrying forward the pioneering spirit of the people who founded the Society, we continue to activate the global FSHD community in our quest to turn the vision – better lives for all families living with FSHD – into a reality.

Mark Stone
President and CEO
FSHD Society

*But a vision, however noble,
remains no more than a wish
without the determination
to follow through.*



Look for us on the Internet at fsbdsociety.org. We thank the FSHD Society staff for their editorial assistance.

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This is how we win

Every move we make drives us toward our goal

BY JUNE KINOSHITA, FSHD SOCIETY

The FSHD Society's approach to combating facio-scapulohumeral muscular dystrophy (FSHD) can feel like a chess game, with many pieces deployed across the board. Each is a critical player working in concert with the others to achieve the ultimate prize: Effective treatments and healthier lives for everyone with FSHD.

Our strategy is two-pronged.

On the one hand, under the banner of the Therapeutic Accelerator, we orchestrate efforts to speed up clinical trials, clear the path to regulatory approval, and steer future treatments past obstacles such as lack of insurance coverage and access to care.

On the other hand, our education and engagement programs are priming our community to mobilize rapidly for natural history studies, clinical trials, and, recently, what we call treatment readiness.

Here's our game plan to ensure that our efforts are effective and target the needs of our families.

The Society's support for the Clinical Trial Research Network and Global Innovation Hub provides the critical infrastructure for clinical research and treatment trials.

This infrastructure makes it possible to conduct efficient research on molecular biomarkers, imaging biomarkers, functional measures, and new treatments.

None of this research can move forward without broad engagement by the community. This is where our chapters, Walk & Rolls, conferences, and Zoom meetings shine.

The community now has powerful new tools with BetterLife FSHD and the Care Connector, which link individuals to valuable resources and enable patients to contribute their data to the collective effort.

These efforts must spread internationally, because FSHD doesn't respect national boundaries – and because solutions require all of us working together. Enter Project Mercury to give patients a powerful voice with regulatory bodies like the Food and Drug Administration and the European Medicines Agency, as well as with insurance payors and healthcare systems.

This is also why we are working on international care guidelines to provide updated medical care for patients everywhere.

Through these coordinated moves, the Society aligns all stakeholders, working together to put FSHD into checkmate. 

Against all odds

The early heroes of the FSHD Society

BY ERIN SAXON, FSHD SOCIETY

In December, I began an exciting project: Capturing the stories of the early members of the FSHD Society. Since then, I've interviewed several remarkable people over Zoom and delved into early issues of *FSH Watch* to uncover the Society's incredible history.

While there are still more stories to collect, each interview sharpens the image of what those first years were like.

Officially formed in 1991, the FSHD Society was started by a handful of people living with FSHD who were determined to solve this disease. Founders like Daniel and Carol Perez, Dr. Stephen Jacobsen, and others created the Society in response to a complete absence of FSHD-specific research and resources.

The Muscular Dystrophy Association existed, but its focus was elsewhere, leaving a critical gap for those with FSHD. The discovery of *DUX4* was still years away, and the challenges seemed insurmountable.

The dedication of this small group was extraordinary. Longtime member and San Diego Chapter Director Amy Bekier explained, "You have to believe that the compounding of different contributions becomes a voice – a loud voice. If we're not out there to be seen, we won't be seen."

Daniel Perez, for example, was the first person to testify on FSHD research before the US Congress, thereby directly influencing the National Institutes of Health to fund its initial FSHD-specific grants. To date, he has testified 65 times.

The Society balanced bold, innovative work with empathy and



Founding members at the first meeting of the FSH Society on July 10, 1992, in Del Mar, California.

connection. Living with FSHD can be isolating, and before the Internet, connecting meant countless phone calls and paper registries.

Carol Perez and others worked tirelessly to build a supportive network. As Deb Calhoun recalled, "Carol would call and say, 'Hey, how are you doing?' or 'I know you have this doctor's appointment coming up. Do you need anything?' She was a really special lady."

In the 1990s, the Society funded research grants, built regional support groups, started researcher-patient conferences, and created the first FSHD educational materials.

These efforts created a tectonic shift in the FSHD landscape and paved the way for today's therapeutic pipeline, clinical trials, and a Chapter Program that spans the US and Canada.

As current Board Vice-Chair Howard Chabner reflected, "I think that

it's pretty fair to say that if the Society wasn't formed, we would not be anywhere near where we are today."

Some of these early heroes are no longer with us, but their legacy lives on. The work they started continues, and one lesson is clear: Collaboration moves us forward. As effective treatments come into view, we are standing on the shoulders of those who dared not only to dream, but to act.

With every interview, I've asked what kept people going when there was no certainty the work would pay off. Founding member Don Burke put it best: "In terms of what motivated us ... it was knowing that there was going to be no progress unless we made the progress happen. But it was hard."

If you have a story to share about those early days, I'd love to hear it. Please get in touch with me at erin.saxon@fshdsociety.org. 

Big pharma shows growing interest in FSHD

Recent deals could inject up to \$3.5 billion into the space

BY JUNE KINOSHITA, FSHD SOCIETY

The pharmaceutical industry is showing a growing interest in developing therapies for facioscapulohumeral muscular dystrophy (FSHD), a rare genetic disorder affecting an estimated one million people worldwide.

This trend is evident through recent high-profile deals between major pharmaceutical companies and smaller biotech firms specializing in FSHD and other muscle conditions.

This past May, Sanofi, the eighth largest pharmaceutical company in the world, entered into a collaboration and licensing agreement with Fulcrum Therapeutics for the development and commercialization of losmapimod, a potential first-in-class treatment for FSHD.

The deal, worth up to \$1 billion, granted Sanofi rights to losmapimod outside the US, with Fulcrum retaining US rights. Fulcrum halted its FSHD program in September after its Phase 3 trial of losmapimod failed to meet its primary endpoint, but this setback has not dampened big pharma's interest.

Just this past November, pharma giant Novartis made a significant move by acquiring Kate Therapeutics for up to \$1.1 billion, expanding its capabilities to advance gene therapies in neuromuscular conditions.

Kate Therapeutics is developing drug candidates for various muscular dystrophies. Their FSHD candidate aims to deliver a microRNA against *DUX4* to skeletal muscles. Kate's Directed Evolution of AAV Capsid Leveraging *In Vivo* Expression of Transgene RNA (DELIVER) platform develops adeno-associated virus (AAV) to deliver therapeutic molecules selectively to muscle rather than to organs such as the liver.

Similarly, Sarepta Therapeutics has made a substantial investment in FSHD research through a global licensing and collaboration agreement with Arrowhead Pharmaceuticals.

The deal, valued at more than \$1 billion, includes multiple siRNA programs targeting FSHD and other muscle disorders. Arrowhead's lead product for FSHD, ARO-DUX4, is currently in a Phase 1/2 clinical trial.

Sarepta's gene therapy for Duchenne muscular dystrophy, Elevidys®, received FDA approval in 2023. The agreement with Arrowhead will enhance Sarepta's pipeline, complementing the company's existing leadership in Duchenne muscular dystrophy.

Despite the disappointment with losmapimod, Sanofi will also remain active in FSHD through its strategic collaboration with miReculé for the development of an antibody-RNA conjugate (ARC) to treat FSHD.

This deal, worth up to \$400 million, combines miReculé's anti-*DUX4* RNA therapy with Sanofi's muscle-targeted NANOBODY® technology. The resulting therapy aims to selectively target and suppress the underlying cause of FSHD in muscle tissue.

This partnership leverages Sanofi's global reach and expertise in rare diseases to bring an FSHD treatment to patients worldwide.

These investments by major pharmaceutical companies highlight the increasing recognition of FSHD as a significant unmet medical need and the potential for innovative therapies to make a meaningful impact on patients' lives.

These developments also mean there is intensifying urgency to educate individuals with FSHD, patient advocacy groups, and clinicians on the steps they should take to prepare for upcoming clinical trials and treatments. 



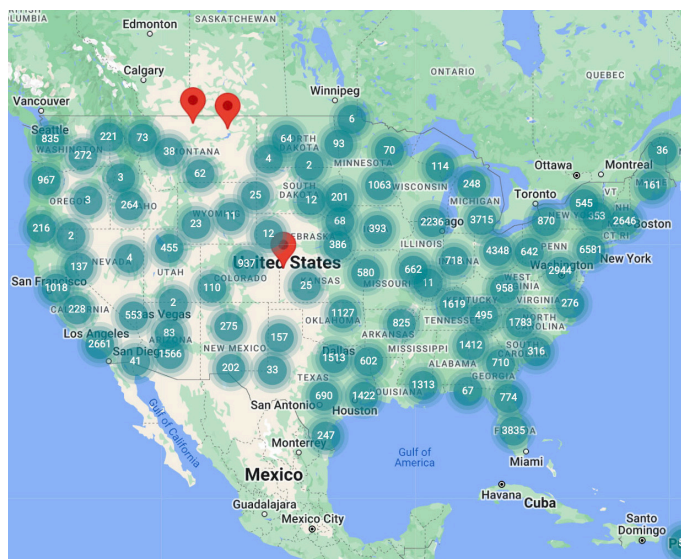
Care Connector

Find resources near you

BY ANNA GILMORE, FSHD SOCIETY

Among the most frustrating experiences you can have with a new healthcare provider is realizing that they don't know anything about your condition. For a long time, the FSHD community has asked for some kind of database of providers who have any level of FSHD knowledge. This year, we are finally rolling out a new tool to help fill this need: The Care Connector.

This tool has been built as a searchable map, where you can set your search area and select what types of providers/resources you'd like to see. The information that pops up on the map comes from a



The Care Connector can be found at fshdsociety.org/care-connector/.

variety of sources and will need to be updated regularly.

If you're using the tool, and you notice some outdated information (or your own doctor is missing!), please take a moment to submit updates. We want to share the most accurate information to help families find the resources and support they need.

Many thanks to Ranae Beeker, Len Goldberg, Lois Gilman, Tara Hill, and other contributors who have worked over the years

to bring this project to fruition. We wouldn't have made it to this point without you! 🙏

Why a new care guideline is needed

Medical care guidelines set the standard for clinicians to follow when caring for their patients. But as we hear from our community, people with FSHD all too often encounter doctors and other providers who know little about the condition or have outdated practices.

The FSHD Society aims to remedy this situation by leading an international project to publish an updated care guideline and make sure it is distributed globally..

This revised FSHD care guideline is critical not only to optimize patient care, but also to ensure that our community is ready for clinical trials and new treatments. There is now a robust therapeutic pipeline targeting the underlying causes of FSHD. Some of these therapies are currently in clinical trials or close to being in the clinic. We want to help ensure that doctors and patients are prepared for them.

One of the most important updates in the new guideline is on genetic testing. Genetic testing used to be recommended only if a patient with symptoms had no family history or exhibited signs that weren't quite typical.

In the new guideline, genetic testing is recommended to confirm an FSHD diagnosis in any individual with symptoms, regardless of family history. Such confirmation is required for

participation in clinical trials and for prescribing future treatments that target the genetic cause of FSHD.

The last FSHD care guideline was published in 2015, and the field has made many advances since then. In 2021, the FSHD Society and European Reference Network convened an international team of nearly 50 medical experts and patient advocates to begin drafting an updated international guideline.

People living with FSHD helped to shape the guideline. For example, they requested information on pregnancy and birth because of their personal experiences of not getting much guidance from their own doctors. They also pointed out how things like hand gestures and bowing can make communication difficult in various cultures.

Now being readied to submit for publication to a major medical journal, the care guideline provides updated recommendations on diagnosis; genetic testing; managing muscle weakness, pain, and fatigue; hearing and vision loss; respiratory issues; and surgery. It also covers new topics including mental health; communication; speech and swallowing; cardiac abnormalities; and pregnancy, birth, and parenting.

— by Michelle Mellon, MD

BetterLife FSHD

Real people, real impacts

BY KAYLEIGH WOREK AND AMANDA HILL, FSHD SOCIETY

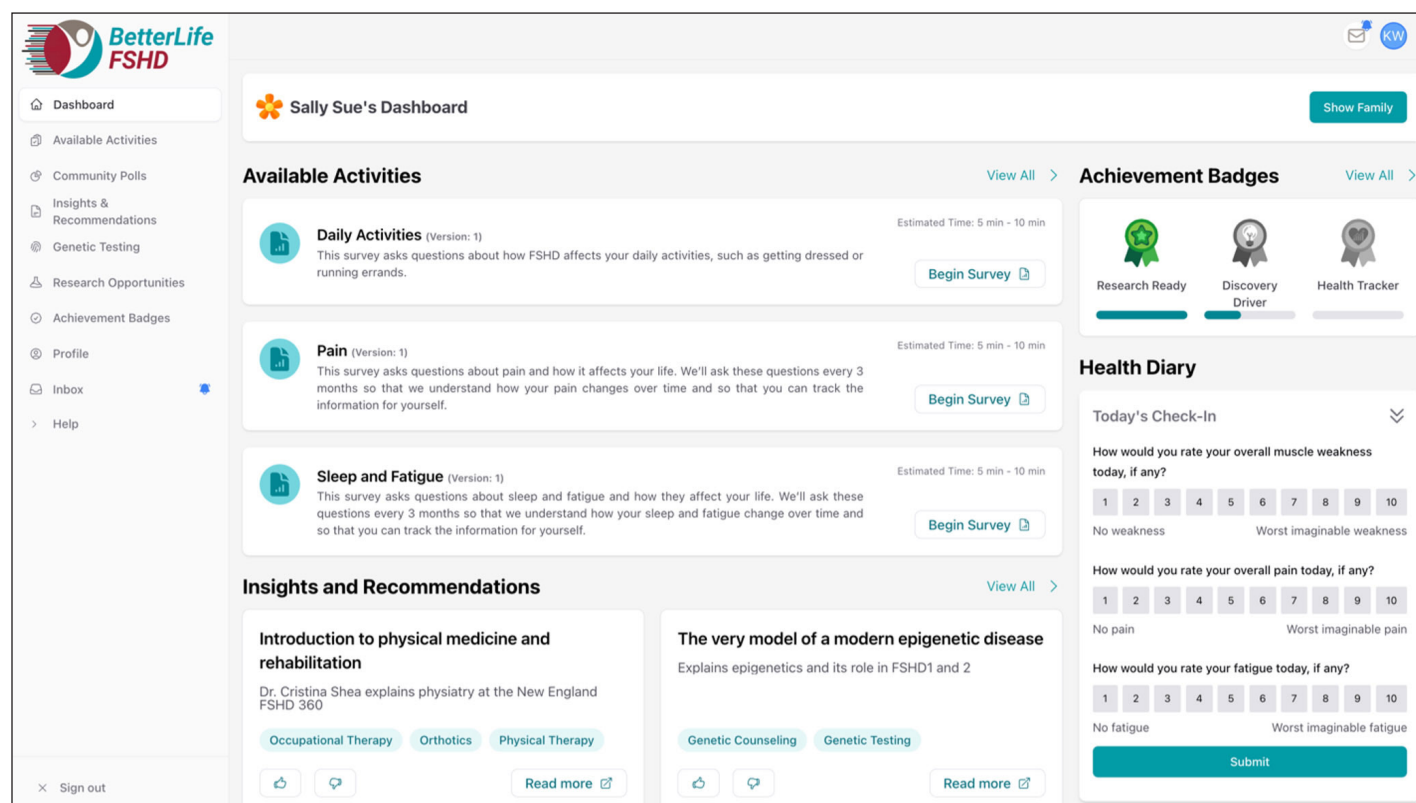


FIGURE 1. The dashboard enables you to quickly and easily navigate around BetterLife and see available activities.

In case you missed it, the long-awaited and first-of-its-kind digital research and health platform, BetterLife FSHD, launched last fall.

BetterLife was designed in partnership with people living with FSHD to be your go-to platform for taking control of your health journey.

After receiving several big feature upgrades in January 2025, BetterLife now serves as a one-stop hub to track symptoms and progression, contribute your data to research anonymously, discover personalized articles relevant to your journey, investigate how your experience compares to that of others, and easily identify research opportunities that may be a good fit for you.

Joining BetterLife FSHD is easy. After providing consent on a web browser or mobile device and creating a login, BetterLife users are taken to an easy-to-navigate dashboard.

Participants enter information at their own pace by completing surveys, which unlock new features. Your progress is tracked by badges, and you can view how

many other users have achieved the same badges and milestones.

BetterLife follows the highest industry standards for digital security, ensuring that users and their data are protected (see Figure 1).

With hundreds of participants from more than 40 US states, BetterLife brings tangible benefits for people living with FSHD (see Figure 2).

For many, BetterLife offers a sense of belonging. Mollie Garrett shares, “FSHD is a daunting disease to live with, especially when we feel alone on the journey. This website allows me access to reliable information and tools when I need them. I cannot stress how invaluable this is to me!”

Participants note that they enjoy the informative and useful content that is part of a comprehensive and well-catalogued resource library.

Users can also engage with community polls, a forum-style feature that enables curious community members to

see how they compare to others with FSHD in real time and pose their own questions of interest (see Figure 3).

For others, BetterLife provides hope. Kate Brown states, “After navigating my disease alone for so many years, I am thrilled to have a website designed to support me and my family.... I am sure it will become my go-to source for daily life.”

More than half of participants use the daily symptom tracking feature to monitor their pain, fatigue, and muscle weakness, and easily view their trends.

Additionally, families can benefit from linking profiles for affected family members and delegating access to trusted people (such as a spouse).

Finally, BetterLife uses survey answers to match partici-

pants with current research opportunities in FSHD (see Figures 4 and 5).

BetterLife has also made behind-the-scenes impacts by recruiting participants for interviews and focus groups with major pharmaceutical companies.

Soon, BetterLife will unveil a specialized portal for researchers, helping to support research projects and the development of future therapies.

The BetterLife team continues to work hard to provide even more valuable features, which will be released this year to the FSHD community. We’re eager to hear your feedback as we work to make BetterLife the best it can be.

Thank you for joining us on this journey – there is so much more to come! 🎉

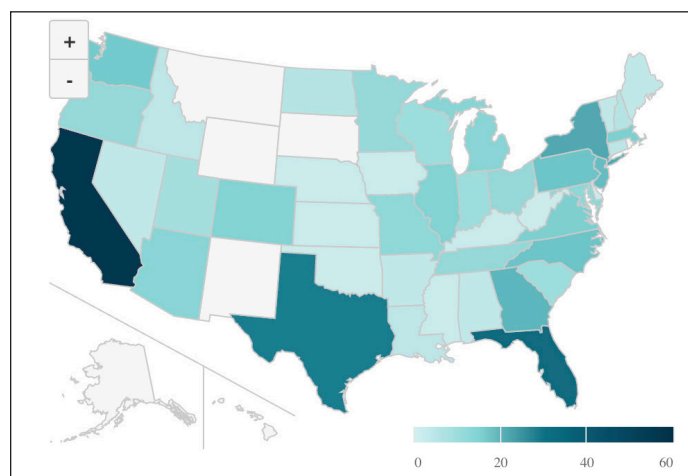


FIGURE 2. Geographic location of BetterLife users as of January 17, 2025. Darker colors indicate higher numbers of participants.

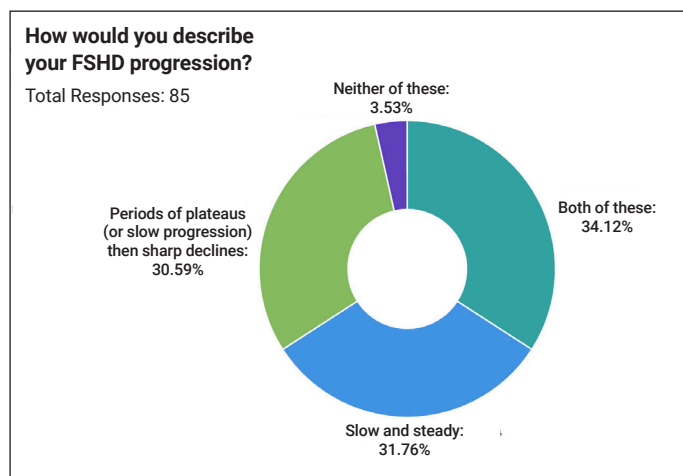


FIGURE 3. Community polls allow users to develop and pose their own questions of interest and see how their answers compare to those of others.

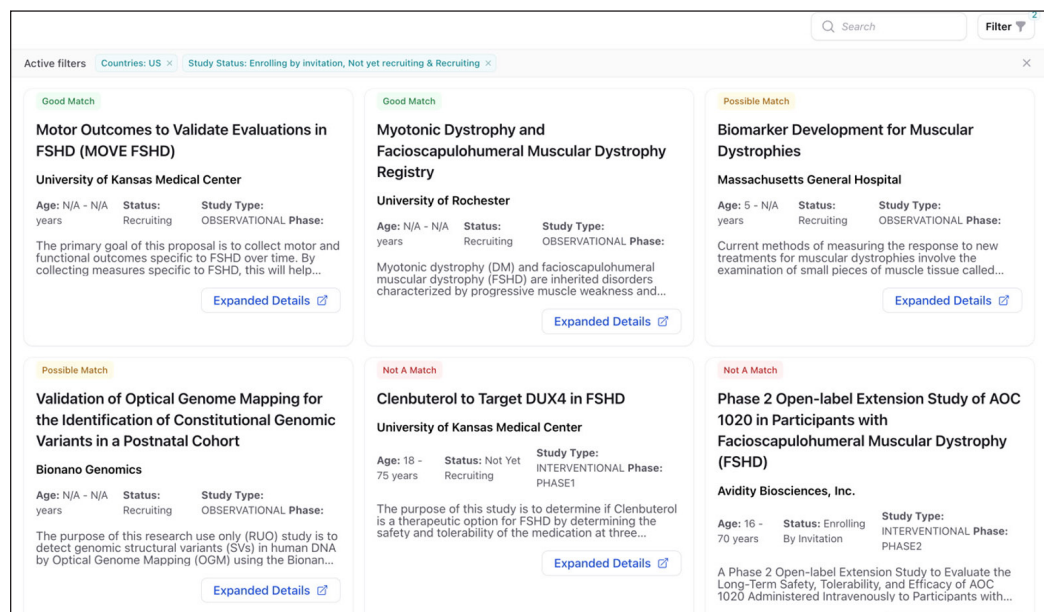


FIGURE 4. One new BetterLife feature compares your survey answers to eligibility criteria to help match you with studies.

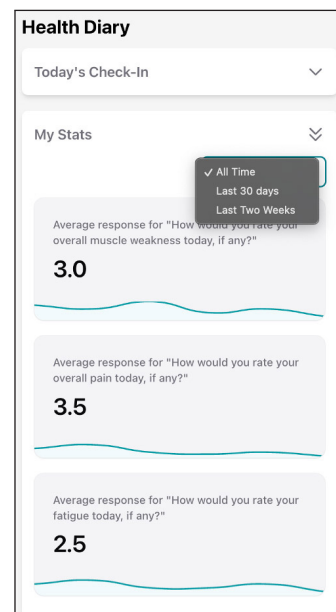


FIGURE 5. Users can view their pain, muscle weakness, and fatigue information over 2 weeks, 30 days, or all time.

2025 Volunteer Leadership Summit

Powered by love and resilience

BY BETH JOHNSTON, FSHD SOCIETY



A record number of people attended the 2025 Volunteer Leadership Summit both in-person and on Zoom.

For the seventh consecutive year, the FSHD Society brought together its volunteer leaders from across the United States and Canada for the annual Volunteer Leadership Summit. Held on February 1 and 2, 2025, this hybrid event combined in-person gatherings at Chicago's Hilton O'Hare with virtual participation via Zoom. The weekend was a dynamic mix of learning, collaboration, camaraderie, and even a touch of fun.

"Powering the Path Forward"

The summit's theme, "Powering the Path Forward," underscored the pivotal year ahead for FSHD clinical research, community engagement, and advocacy efforts. The event began with inspiring talks from Archer Sverdrup and Tim Hollenback, who shared their personal journeys and encouraged attendees to reflect on their own paths within this mission-driven community.

Educational highlights

Participants engaged in a variety of educational sessions designed to equip them with tools and insights for advancing FSHD initiatives. Topics ranged from "building a treatment-ready ecosystem" to updates on the latest therapy developments and clinical trials.

Afternoon breakout sessions offered opportunities for smaller groups to delve deeper into subjects such as "Government Advocacy in 2025," "Using BetterLife," storytelling techniques, and collaborative roundtable discussions aimed at generating fresh ideas.

A heartfelt closing

The summit concluded on an emotional note with remarks from Jack Gerblich, who reminded volunteer leaders of their profound impact. "Never forget that one can inspire many," he said. "You are the reason families find hope. You are the

Setting the Board

reason voices are heard. Together, we are not just dreaming of a cure; we are building a legacy of love and resilience.”

This year’s summit reinforced the collective strength of the FSHD Society’s network of leaders. As they move forward into 2025, these dedicated volunteers remain united in their mission to drive progress and bring hope to families

affected by facioscapulohumeral muscular dystrophy.

We have all types of roles for volunteers – to suit any level of time, skill, or desire. Contact Beth Johnston at beth.johnston@fshdsociety.org to explore these opportunities. Maybe we will see you at the Volunteer Leadership Summit next year! 🎉

“Seeing so many faces (on my Zoom screen!) with enthusiasm, joy, and eagerness ... was just the uplift that I needed. I’m ready to roll forward.”

– RANAE BEEKER, WASHINGTON



From left, Jacob and Anna Wise from Minnesota, Mollie Garrett from Virginia, and Heloise Hoffmann from California.



Marisa Spain shares her “Aha!” moment as her husband, Aaron Rose, looks on.

“It was humbling to hear the stories of those impacted by this disease and connect with so many amazing people.”

– JANE ROBERTS, ALBERTA, CANADA

“It’s not just about giving back – it’s about growing, having fun, and being part of something truly impactful.”

– FROM VOLUNTEER SURVEY



From left: Karen Camarta, Jane Roberts, and Samantha Johnston.

We need YOU – to write to your senators

Aiming to access fresh funding for FSHD research

BY ANNA GILMORE, FSHD SOCIETY



Last year was an exciting one for our community as we launched our first FSHD Day on Capitol Hill! A small but mighty group of advocates succeeded in getting a House Resolution introduced with bipartisan sponsorship. They built valuable relationships with lawmakers who continue to support our community.

In partnership with the Muscular Dystrophy Association (MDA), this year we are building on our momentum to make a very specific request.

The Peer Reviewed Medical Research Program (PRMRP) is a federal grant program that supports scientific research. There is a list of diseases and conditions that researchers must be working on to apply. Becoming one of the eligible diseases for this grant would open up a new source of much-needed funding for basic research. We've come such a long way in recent years, but there is still so much we don't know about FSHD.

The only way to get onto the list

of eligible diseases for the PRMRP is to have a United States senator advocate for it on our behalf.

We are launching a campaign this spring, and we will need everyone's help! If you would like to write a letter or email to your senator, join a Zoom meeting with legislative staffers, or maybe get even more involved, please let us know right away!

Visit our Advocacy webpage to learn more: fsbdsociety.org/our-impact/advocacy/. 

Together, we are changing the future of FSHD

In 2024, the FSHD Society led bold initiatives that are bringing us closer than ever to life-changing treatments. We united and empowered our community, drove critical research forward, and engaged top regulatory agencies to accelerate progress.

One breakthrough: In just a few months, 477 individuals enrolled in our new BetterLife FSHD platform, providing invaluable real-world data to advance research and improve patient care.

Your support is fueling these milestones. Scan the QR code or visit bit.ly/FSHDimpact2024 to explore the full 2024 Impact Report and see how your generosity is making a difference.

2024 IMPACT REPORT

Scan to see the impact you made



bit.ly/FSHDimpact2024



Hope in the laboratory

Taking back control of my destiny through research

BY HELOISE HOFFMANN, STANFORD UNIVERSITY

For the past year, I have been working alongside a team of fellow undergraduates at Stanford University to research a therapeutic approach for FSHD. Directing this project has taught me unique lessons in hope, resilience, collaboration, and the goodness of people.

I was diagnosed with FSHD at age 13, after three long years of prodding and poking and yearning for an answer to my symptoms – a path well trodden in the FSHD community. The initial years after my diagnosis left me feeling diminished, shattered, and isolated. But getting involved in advocacy, and now research, has been a life-changing way of taking back control of the disease that once controlled me.

My latest journey started with an impulsive decision to apply for Stanford iGEM (International Genetically Engineered Machine), a global synthetic biology competition enabling undergraduates to pursue student-driven research at the cutting edges of bioengineering.

My teammates – Alice Finkelstein, Michael Liu, Mafer Velasquez, Vanessa Chiprez, Katherine Xu, Amanuel Geremew, and Ayushi Mohanty – jumped on board and helped me transform what started as a mere idea into an adventure.

FSHD is caused by faulty expression of a gene called *DUX4*, which activates a cascade of other genes that is toxic to muscles. Our scientific approach centers around harnessing the inherent DNA-binding properties of one part of the *DUX4* protein and repurposing it to silence, rather than activate, its targets.

Excitingly, we found promising results through our early experiments. My team presented them at the annual iGEM conference in Paris in November, raising awareness about FSHD on the global stage.

Our team competed in a therapeutic research program through which we secured \$50,000 to fund the next steps of our project.

Power of the patient voice

We are now gearing up for new experiments by which we hope to demonstrate that our therapy might work in the clinic.

The past year has been exhausting and frustrating at times, as we worked long hours, encountered problems with our cells, and struggled to get the right DNA sequences. But I have never felt so exhilarated, empowered, and galvanized than when I was in the lab. Looking through a microscope lens at my glowing cells, I thought, “Wow, this could really work!”

As my teammate Alice said, “Analyzing the results of one of our first transfections [inserting new DNA into cells], where we first discovered promising preliminary results from our approach, was magical. The months of effort we had put into planning our project and performing the

series of experiments leading up to that moment – from the background research, to cloning and assembling our DNA constructs, to designing the transfection conditions – suddenly paid off, and in that instant, the potential for our idea to be a novel therapeutic approach for FSHD turned real.”

Above the exciting research, one of our main priorities was elevating patient voices. As a patient, I know how frustrating it can be to feel left out from the research and the exciting advances being made in the field.

Thanks to exceptional efforts by my teammates and collaboration with the whole FSHD community, we interviewed more than 20 clinicians, researchers, and other professionals, and, more importantly, consulted with dozens of patients to ensure their voices are heard in the research process.

“Handling the patient interviews was the experience I value the

*“Hope” is the thing with feathers –
That perches in the soul –
And sings the tune without the words –
And never stops – at all –*

*And sweetest – in the Gale – is heard –
And sore must be the storm –
That could abash the little Bird
That kept so many warm –*

*I’ve heard it in the chilliest land –
And on the strangest Sea –
Yet – never – in Extremity,
It asked a crumb – of me.*

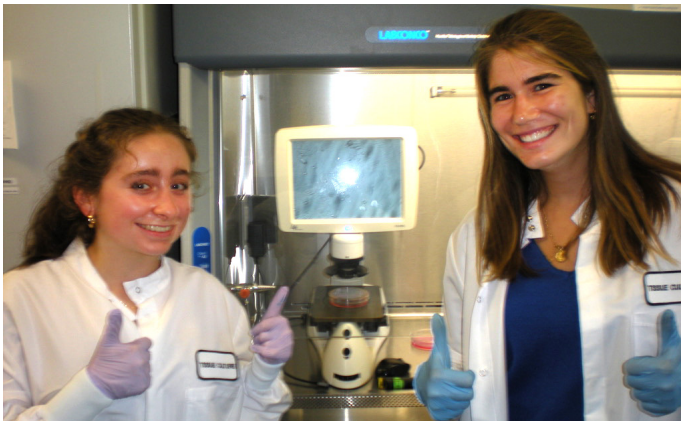
– Emily Dickinson



Heloise Hoffmann



Our iGEM team, from left: Drew Endy (mentor), Amanuel Geremew, Michael Liu, Alice Finkelstein, Mafer Velasquez, Ayushi Mohanty, Katherine Xu, Vanessa Chiprez, Phillip Kyriakakis (mentor).



Alice Finkelstein and Heloise Hoffmann giving a thumbs up.

most out of everything I did during the summer,” said Mafer. “Listening to stories, understanding the multifaceted nature of FSHD, and knowing the special cases of each of our conversations shed light onto multiple aspects of our project. But more than that, it gave us the determination to keep working to make this project an accessible reality.”

Community is our superpower

Working as a team to unpack FSHD, which is commonly characterized by scientists as one of the most complex genetic disease mechanisms, has been beyond inspiring and empowering.

“It was incredible to see how our research could help uncover new possibilities for treating FSHD and give hope to those living with this condition,” enthused Katherine. “Working alongside such passionate teammates has shown me how powerful collaboration and innovation can be in tackling real-world challenges. Not only was it an exciting intellectual challenge to work on computational models in a



The iGEM team in their lab at Stanford University.

field in which I was not an expert, but the connections built through this experience were what inspired me the most.”

Going into 2025, I am more excited and hopeful than ever about what’s coming our way. We are at a pivotal moment in FSHD history; we know the gene causing FSHD and how to stop it, and there is more interest than ever from researchers to tackle it.

Above all, we have a secret superpower: A community of patients, their families, and their cheerleaders, with a greater sense of grit, resilience, and adaptability than I’ve encountered anywhere else.

So let’s roll up our sleeves, put our heads together, and use our superpower to once and for all show FSHD who’s in control!

In addition to my wonderful teammates, I would love to thank our iGEM mentors at Stanford, Helen Blau, Michael Kyba, June Kinoshita, and all the others involved in shaping our project. Without their mentorship and encouragement, we could not have had the same impact! 🙌

Clinical trial snapshot



To learn about clinical trial phases, visit fshdsociety.org/clinical-trials/.

For easy-to-understand details about each trial, visit fshdsociety.org/for-patients-families/clinical-trials/.

The information shared here is accurate as of February 2024. For status updates on specific locations, search on trial keywords at clinicaltrials.gov.

An open-label extension (OLE) is a study in which participants of a placebo-controlled trial are offered the option to continue to be studied after the trial has been completed. All participants in the OLE receive the active drug, so the study is no longer blinded or placebo controlled.

REINFORCE by Centre Hospitalier Universitaire de Nice, principal investigator Sabrina Sacconi, funded by Hoffmann-La Roche

QUICK FACTS		WHO CAN PARTICIPATE?
Drug	Satralizumab	<ul style="list-style-type: none"> • Age 18-65 • FSHD1 • Ricci score 2-4, able to walk without support • Must be able to do MRI
How Is It Given?	Injection under skin	
Phase	2	
Participants	40	
Placebo	Yes	
Genetic Testing	Required	
Rx Duration	Double-blind phase, at weeks 0, 2, 4, and every 4 weeks thereafter for 48 weeks; open-label phase, same dosing for 48 weeks + follow-ups; total 116 weeks	
Study Visits	~16	
Notable Activities	MRI	
Open-Label Extension	Yes	
STATUS		 
Enrollment	Began January 2024	
Data Expected	Mid-2026	
Locations	Ottawa, Canada; Nice, France	
Learn More	clinicaltrials.gov/study/NCT06222827	

FORTITUDE sponsored by Avidity Biosciences

QUICK FACTS		WHO CAN PARTICIPATE?
Drug	Del-brax (AOC 1020)	<ul style="list-style-type: none"> • Age 16-70 • FSHD1 or FSHD2 • FSHD clinical score of 2-14 • Able to walk 10 meters without assistance • Reachable Workspace score • Must have leg muscle suitable for biopsy and be able to do MRI
How Is It Given?	Intravenous infusion	
Phase	1/2	
Participants	84	
Placebo	Yes, 2:1	
Genetic Testing	Required, provided by study	
Rx Duration	8 doses	
Study Visits	~15, some may be virtual	
Notable Activities	MRI, leg muscle biopsy	
Open-Label Extension	Yes	
STATUS		
Enrollment	Currently enrolling	
Data Expected	Second half of 2026	
Locations	US, Canada, UK	
Learn More	fortitude-study.com clinicaltrials.gov/study/NCT05747924 fshdsociety.org/avidity-fortitude-trial/	

ARO-DUX4 trial sponsored by Arrowhead Pharmaceuticals

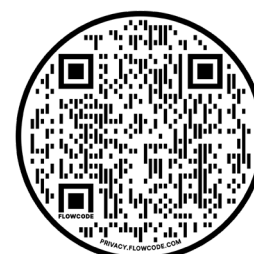
QUICK FACTS		WHO CAN PARTICIPATE?
Drug	ARO-DUX4	<ul style="list-style-type: none"> • Age 18-70 • FSHD1 • Clinical Severity Scale 3-8 • Must have leg muscle suitable for biopsy and be able to do MRI
How Is It Given?	Intravenous infusion	
Phase	1/2a	
Participants	60	
Placebo	Yes, 3:1	
Genetic Testing	Required, provided if needed	
Rx Duration	Part 1: duration 3 months Part 2: 4 doses over 1 year	
Study Visits	~20	
Notable Activities	MRI, leg muscle biopsy	
Open-Label Extension	Yes, if warranted by study results	
STATUS		
Enrollment	Currently enrolling	
Data Expected	TBD	
Locations	New Zealand, Australia, Canada, Europe, and Thailand	
Learn More	fshdsociety.org/arrowhead-trial clinicaltrials.gov/study/NCT06131983	

What you can do to be trial-ready

These steps will reduce delays and improve your chances of enrolling in a trial. You will also need to meet the eligibility criteria for the specific trial. There's no guarantee, as many factors must be considered in selecting people for a trial, but these measures can help.

- Make sure you have been diagnosed by a doctor.
- Get a genetic test confirming FSHD.
- Become a patient at an FSHD Clinical Trial Research Network location.
- Enroll in the MOVE or MOVE+ study.
- See your neurologist every year.
- Follow exercise recommendations and be as healthy as possible.
- Sign up to get FSHD Society email alerts.

Learn more:



MANOEUVRE sponsored by Hoffmann-La Roche

QUICK FACTS		WHO CAN PARTICIPATE?
Drug	GYM329 (aka R07204239)	<ul style="list-style-type: none"> • Age 18-65 • FSHD1 or FSHD2 • Ricci score: ≥ 2.5 and ≤ 4 (must be able to walk unassisted) • Must be able to do MRI
How Is It Given?	Injection under skin	
Phase	2	
Participants	48	
Placebo	Yes, 1:1	
Genetic Testing	Required, talk to your local site	
Rx Duration	Every 4 weeks for 52 weeks	
Study Visits	At least every 4 weeks	
Notable Activities	Wearable device, MRI	
Open-Label Extension	Yes, for 52 weeks	
STATUS		
Enrollment	Complete. Study is active.	
Data Expected	Q3 2026	
Locations	US, Denmark, Italy, UK	
Learn More	forpatients.roche.com clinicaltrials.gov/study/NCT05548556 fshdsociety.org/roche-manoeuvre-trial/	

32nd annual FSHD International Research Congress: Leading FSHD researchers will meet in Amsterdam

The FSHD Society is hosting its annual International Research Congress (IRC) on June 11 and 12 in Amsterdam. We expect close to 200 attendees. This will be an exceptionally festive time in the Dutch city, as it will be in the middle of a year-long celebration of its 750th anniversary.



Nicol Voermans, MD PhD, and Stephen Tapscott, MD PhD, will co-chair our meeting this year.

The IRC has had an outsized impact on the FSHD field. As the premier global scientific platform to present, share, debate, and strategize on therapy development, the IRC has helped to bring the field to its current exciting state, with multiple clinical trials and high stakes around improving trial outcomes. Recent IRC meetings have also broadened the community's focus to include pediatric, early-onset, and non-ambulatory patients.

This year's IRC will be followed by the first-ever FSHD Connect Europe meeting, hosted by FSHD Europe (see below). 



In case you missed them... YouTube

The FSHD Society's YouTube channel is a treasure trove of great seminars and conversations. Here are some recent ones we don't want you to miss:

- "We don't grow in the good times. We grow during challenges," with Jennifer Egert, on FSHD Straight Talk
- "Progress and hope in FSHD research," with Lucienne Ronco, PhD, on FSHD Straight Talk
- "Service animal resources," on the Parents' Roundtable
- "FSHD science with Heloise Hoffmann," on the Parents' Roundtable
- "Annual drug development update," with Nicholas Johnson, MD, on FSHD University 

FSHD Connect Europe Meeting 2025

We are thrilled to announce that FSHD Europe, in collaboration with the FSHD Society, is organizing the very first FSHD Connect Europe Meeting in Amsterdam, the Netherlands, from June 13 to 15.



Last year, I had the incredible opportunity to attend the FSHD Connect Meeting in Denver with Prof. Nicol Voermans. It was inspiring to see so many FSHD patients and their families together in an atmosphere of unity and shared purpose. We left Denver with a clear mission: To bring this experience to Europe and create a similar FSHD Connect Meeting for our community.

FSHD Europe, a nonprofit umbrella organization, bringing together 14 patient organizations from 12 countries, has never organized such an event before. We are very grateful for the support and collaboration of the FSHD Society to make our mission possible!

Planning the program has been one of the biggest challenges so far. There's so much happening in FSHD research, from basic research and drug development to clinical trials. The global Project Mercury initiative is making great progress, and it's important for patients to be informed on the importance of patient registries and participation in clinical trials. Beyond research updates, we will also focus on practical ways to manage symptoms and improve quality of daily life.

We hope this event will be a fantastic opportunity for FSHD patients and their families from across Europe to connect, share insights, and build a strong, empowered community.

Can't wait to welcome everyone to Amsterdam and experience this exciting journey together!

For more information, visit fshd-europe.info/fshd-connect-europe/. 

— Ria de Haas, FSHD Europe



Exciting changes are coming



The FSHD Society has always been about progress: Driving breakthroughs in research, uniting our community, and advocating for effective therapies. We've made incredible progress together, and later this spring, we'll unveil a fresh look that reflects where we are headed.

From our founding in 1991, when a handful of individuals came together to address the absence of FSHD-specific research and resources, to today, our mission has been driven by boldness, grit, and collaboration.

With multiple therapies in clinical trials and the first treatments on the horizon, the FSHD Society is evolving to meet new challenges. This transformation will help ensure that our identity reflects the groundbreaking work happening now and builds on our story of a grassroots movement that has grown into a global effort.

You'll see a new look along with a redesigned website that is more user-friendly and makes it easier for individuals, families, clinicians, and researchers to find the resources they need.

We are also hard at work on refining our voice so that we can better connect with the FSHD community and amplify awareness. Together, we're powering the path forward, and we can't wait to show you what's next! 

Upcoming

WEBINARS



FSHD University webinars

Advance registration required.

March 20: Raise your voice!

Advocacy call to action

April 17: Exercise & physical therapy for FSHD, with Ulrike Uta

May 16: The Pediatric MOVE study, with Natalie Katz, MD, and Katy de Valle, PhD

June 26: Breathing with FSHD, with Joshua Benditt, MD

July 17: The role of the physiatrist in managing FSHD, with Nassim Rad, PhD

THE GATHERING PLACE



All of the Zoom groups listed below meet on a regular monthly schedule. For details about topics and speakers, consult the Events Calendar. To join, register once and you'll receive the link and monthly reminders.

CarePartner Hour

Last Tuesday of every month at 8 p.m. ET | 7 p.m. CT | 6 p.m. MT | 5 p.m. PT

Everything Early Onset

Second Monday of every month at 7 p.m. ET | 6 p.m. CT | 5 p.m. MT | 4 p.m. PT

Feeling Fit with FSHD

Second and fourth Thursday of every month (unless a major holiday) at noon ET | 11 a.m. CT | 10 a.m. MT | 9 a.m. PT | 6 p.m. CET | 5 p.m. GMT

Parents' Roundtable

Third Tuesday of every month at 8 p.m. ET | 7 p.m. CT | 6 p.m. MT | 5 p.m. PT

Wellness Hour

Second Monday of every month at 5 p.m. ET | 4 p.m. CT | 3 p.m. MT | 2 p.m. PT

Women on Wellness

First Wednesday of every month at 5 p.m. ET | 4 p.m. CT | 3 p.m. MT | 2 p.m. PT

Young Adults

Third Monday of every month at 8 p.m. ET | 7 p.m. CT | 6 p.m. MT | 5 p.m. PT

FSHD SOCIETY STRAIGHT TALK

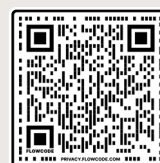


On the second and fourth Tuesday of every month, listen for a new episode of FSHD Straight Talk with Tim Hollenback. These episodes highlight members of our community who are living rich, full lives and diving into their experiences with FSHD. The podcasts are available to stream on-demand on major streaming platforms.

CHAPTER MEETINGS



Our 30+ chapters are busy planning meetings for this year, so please visit our Events Calendar for updates. Many meetings are virtual or hybrid. All are welcome to join from anywhere. Please pay attention to the time zones.



Visit our Events Calendar for updates and to register for events at fshdsociety.org/fshd-events-calendar.



FSHD Society

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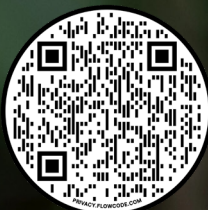
Support today, change tomorrow

Here at the FSHD Society, we have one foot in the present and one in the future, supporting quality of life for those affected by FSHD today and working feverishly to advance initiatives that will improve tomorrow.

Every donated dollar helps us do both by funding:

- Advocacy and education efforts with entities like the FDA and front-line clinicians;
- Our Chapter Program, creating community for those living with FSHD;
- BetterLife, a first-of-its-kind patient-centered FSHD research and resource platform;
- Our global capacity, enabling us to draw from worldwide resources to tackle challenges and develop strategies.

That is a powerful investment. That is what your dollars can do. Make a gift to support today and change tomorrow.



give.fshdsociety.org/donate