FSHD Advocate
2024 • ISSUE 2
FSHD SOCIETY

INNOVATIONS

Ingenious solutions to our challenges

FIND YOUR ROPE TEAM • A BIOMARKER AT LAST? • ADVANCES IN HEARING AIDS
LIFTWARE ROBO SPOON • FSHD DAY ON CAPITOL HILL

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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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June Kinoshita, Editor
june.kinoshita@fshdsociety.org

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

Innovating with impact

Our community is a laboratory for innovation

Mark A. Stone

Innovating with impact – three words that apply to our families living with FSHD. Indeed, this is one of their distinguishing hallmarks – the ability to meet the obstacles of life head-on, with steadfast resolve, hopeful perseverance, and innovative solutions.

Author Marilyn Ferguson wrote, "Achievers have an enabling attitude, realism, and a conviction that they themselves are the laboratory of innovation. Their ability to change themselves is central to their success. They have learned to conserve their energy by minimizing the time spent in regret or complaint. Every event is a lesson, every person a teacher." This attitude permeates our community and the FSHD Society itself, as together we innovate to improve lives and stop the relentless progression of FSHD.

As you read through these pages, you'll begin to see the creative approaches the FSHD Society is taking to overcome obstacles in trial recruitment, regulatory approval, and patients' access to treatments. Toward these goals, the FSHD Society is building collaborations, engaging experts to guide us into new territory, and learning lessons from like-minded organizations.

For instance, Project Mercury (p. 18) is an unprecedented approach to bring together stakeholders from many countries to tackle a common set of challenges: Ensuring that there are enough "trial-ready" patients in registries for upcoming clinical trials, and assuring treatments are available to patients.

Your input into the design of clinical trials is helping to drive innovation in blood biomarkers and outcomes suitable for pediatric trials.

Additionally, check out our first-ever FSHD Day on Capitol Hill (p. 8), and familiarize yourself with impactful events like the FDA listening session (p. 23) and our annual Walk & Roll (p. 24), happening throughout North America this fall and comprising thousands of families. More prosaic, but just as important, is our community's never-ending search for inventions to make daily life easier, whether it's through better and cheaper hearing aids or a space-age "robo spoon."

Know that when the FSHD Society launches new initiatives, they are both for you and because of you. You are our inspiration and the focus of all we do. We live with the urgency that something must be done, and if it is to happen, it's up to us.

Mark Stone
President and CEO
FSHD Society

Editor
June Kinoshita

Copy Editor
Elaine Alibrandi

Graphic Design
Leslie Anne Feagley

FSHD Society
(781) 301-6060
info@fshdsociety.org
fshdsociety.org

To send general mail:
75 North Main Street, Suite 1073
Randolph, MA 02368, USA

To mail donations:
PO Box 411617
Boston, MA 02241-1617, USA

FSHD IS NOT FOR COWARDS

There was plenty of ingenuity and grit on display in Denver

BY JUNE KINOSHITA, FSHD SOCIETY

Many scientists who work on FSHD spend their days bent over test tubes and computers, so this year's International Research Congress (IRC), on June 13-14, opened with a bracing talk by Jeff Johnston, who has lived with the diagnosis for the past 25 years.

"We humans value independence so much that we are willing to die for it," Johnston noted. "With FSHD, I've lost a lot of independence. I'm a 54-year-old man who can no longer live by himself." He said the COVID pandemic – the isolation, loss of cherished activities, anxiety about the future – gave ordinary people a taste of "what it's like to live with FSHD."

Johnston's remarks underscored how FSHD is not a benign condition, as it is sometimes depicted in textbooks. As he thanked the audience, he impressed upon them the urgency and life-changing impact of their research.

Clinical trial landscape

The hot news of the IRC was Avidity's announcement of interim data four months into their Phase 1/2 trial of their RNA therapy called "del-brax" (see page 10).

Alexandra Collin de l'Hortet of Epic Bio shared what she called "robust evidence" for EPI-321 as a potential gene therapy for treating FSHD by permanently suppressing *DUX4*. "We intend to submit an IND application [to the FDA] and are looking forward to commencing first-in-human trials in 2024," she said.

Oxana Beskrovnaya, PhD, of Dyne Therapeutics, reported that the company's lead candidate, DYNE-302, "resulted in dose-dependent and robust reduction of the *DUX4* transcriptome … that lasted up to three months, with benefit on muscle structure and function" in a mouse model of FSHD.

DYNE-302 is similar to Avidity's del-brax, in that it employs an antibody to deliver an siRNA (small interfering RNA) to muscle, where it blocks *DUX4* expression. The Avidity findings are a cause for optimism that DYNE-302 will also be beneficial, but its commercial success may depend on additional factors such as safety, tolerability, and how long-lasting the effects are. Dyne has not announced a time frame for starting a clinical trial.

On the horizon

Lindsay Wallace and colleagues at Nationwide Children's Hospital shared progress on an RNA-based therapy combining mi405, identified in their lab as a potent inhibitor of *DUX4*, with AAV-SLB-101, the adeno-associated virus capsid (surface protein) used by Solid Biosciences in their Duchenne gene therapy. The team showed the construct, ARM-201, is a potent reducer of *DUX4* in mice. ARM-201 is the lead FSHD candidate of Armatus Bio.

Sharif Tabebordbar of Kate Therapeutics described creating artificial microRNA sequences, finding the sequence that was most potent at reducing *DUX4* expression, and pairing it with a muscle-targeting Myo-AAV to deliver the miRNA into a mouse model of FSHD. Their compound improved the mice's ability to run on a treadmill, reduced FSHD-like pathology in the muscles, and lowered *DUX4* levels. On the strength of these data, the company plans to keep developing their gene therapy candidate for FSHD.

Anti-inflammatory drugs, specifically those targeting IL-6, were mentioned as potential therapies for FSHD, and one such drug is currently in a clinical trial in France in a study led by Sabrina Sacconi.

Emanuele Mocciaro and Davide Gabellini described small-molecule drugs that silence/target a region of the genome involved in activating *DUX4*. Two of these drugs are currently in clinical trials for leukemia. Safety and tolerability from these trials will no doubt inform whether these drugs would be good candidates for treating FSHD.

Tools for trials

One topic of keen interest is the Reachable Workspace (RWS), which was selected as a primary outcome for Fulcrum Therapeutics' Phase 3 clinical trial. RWS measures how high and how far, back and forth, a person can reach their arms while seated. But regulatory agencies like the FDA will likely demand evidence that changes in RWS line up with changes in patients' quality of life.

Support for this claim was presented by Leo Wang, MD, from the University of Washington. He analyzed data on 168 adults who participated for two years in the ReSolve natural history study of FSHD. The study found a strong correlation between declines in RWS and muscle strength, and a moderate correlation with difficulties in daily activities that require arm mobility.

Chantal Coles at Murdoch Children's Research Institute in Melbourne, Australia, is conducting an analysis of circulating immune cells as the community seeks to under-

"FSHD is often called a mourning disease. It just relentlessly takes from you. The pianist Steve Blier, who also has the condition, told me FSHD is not for cowards."

– JEFF JOHNSTON, KEYNOTE SPEAKER

stand the role of the immune system in FSHD. Her "Immune Atlas" will be a valuable tool to support muscle recovery after *DUX4* reduction.

A strong push to characterize pediatric FSHD is underway with three clinical centers worldwide (Kansas; Nice, France; and Melbourne, Australia) carrying out long-term natural history studies combined with MRI imaging and molecular muscle characterization to help researchers understand early disease onset and the impact of FSHD on human development.

Keynote speaker, Silvère van der Maarel, answers a question at this year's International Research Congress on FSHD.

"Find your rope team"

Following the IRC, the FSHD Connect conference convened on July 15-16 with a record 283 in-person and 139 virtual registered attendees. Keynote speaker Command Sergeant Major (ret.) Gretchen Evans got the meeting off to an unforgettable start by sharing her story of resilience in overcoming devastating injuries to lead Team Unbroken. She emphasized how everyone needs a "rope team" – people who can pull you up when you fall, and whom you can support when they need help.

Everyone received a carabiner – the metal clip used by rock-climbing teams – and tags so they could exchange contact information with the people they want on their own rope team.

We got to see a rope team in action on Friday night when a delegate vented his frustration that the hotel was unable to lower the height of his bed so that he could transfer to it from his wheelchair. Immediately, a group around the table went up to his room and removed the box spring so that the bed would be at the right height.

Keynote speaker Command Sergeant Major (ret.) Gretchen Evans.

Taking action

The rope team spirit pervaded the weekend, as people stepped forward to help the cause.

Leading clinicians shared strategies to achieve optimal wellness. Volunteers led sessions to share ideas for healthy diets, treating pain, and managing fatigue.

Many lined up for photo and video shoots that will help get their stories out to the public.

Families with kids talked before a packed room of industry representatives about their desire to participate in clinical trials.

Dozens of volunteers helped researcher Linda Lowes, PhD, of Nationwide Children's Hospital test new methods she is developing to measure strength and functional changes more accurately.

One hundred patients and unaffected family members donated blood samples for Dr. Suja Jagannathan's novel biomarker study. "They helped us take two years off the time it would have otherwise taken us to collect so many samples," marveled Brianna Blume, who helped to coordinate this effort for the FSHD center at the University of Colorado.

These were just a few examples of how this community's can-do spirit is moving mountains to get to treatments and a cure.

From upper left, clockwise: Andrea van Beek leads the "Let's Make Art" session; a young crowd gathers to work on the art project; participants trade ideas for managing fatigue; carabiner and tags given to all attendees; Kurt Spiegel leads a breakout session on managing pain; and Claire Simpson of the University of Colorado demonstrates some assistive technologies for adaptive gaming and computers.

FSHD Day on Capitol Hill

Our nation's lawmakers did not know FSHD. Now they do.

BY ANNA GILMORE, FSHD SOCIETY

Mike Kelly (R-PA) was the first congressman to agree to introduce the resolution for the federal government to recognize June 20 as World FSHD Day.

"Maybe it was because we expressed so much excitement over the Pittsburgh Steelers Terrible Towel displayed in his office," mused Mark Christman, leader of the Western Pennsylvania Chapter, "or maybe it was because my wife Renee and Amanda Hill both cried during my talk about the impact of the disease."

The pivotal meeting with Representative Kelly was part of a whirlwind of activity on May 8, when 12 FSHD families convened in Washington, DC, to advocate for themselves and their community. Our goal was to raise awareness of FSHD and build relationships with key lawmakers.

This event marked the first step toward securing additional research funding, streamlining regulatory decision-making, and advocating for insurance coverage of future treatments.

Twenty-one tenacious people – patients, parents, and spouses – dedicated their time and energy to sharing their experiences living with FSHD, outlining their hopes for the future, and forming new friendships.

Our eternal gratitude to (left to right): Laura Maffei, Amy Lowden, Isla Treonze (front), Chris Lowden, Vera Treonze, Siena Treonze (front), Rich Treonze, Maggie Eggleston, Debbie Eggleston, Lexi Pappas LeVine, (FSHD Society staff: Anna Gilmore, Amanda Hill, Erin Saxon), Gary Lauck, Gregg Lichtenstein, Ellen Lichtenstein, Mollie Garrett, Renee Christman, Mark Christman, FSHD Society CEO Mark Stone, Don Burke, Laura Carrino, and Chris Carrino (not pictured, as they were already in meetings: Sam Ray, Ally Roets). Additional thanks to others who attended Zoom meetings and gave testimony in advance of the event: Len Goldberg, Kristin Zwickau, Roy Stang, Ann Areson, and Jim Wilkinson.

ACTION ITEMS FOR CONGRESS

Our goal was to bring attention to the unique challenges faced by people living with FSHD, and to advocate for awareness and policies that would significantly improve lives. We asked legislators to take action by:

- **Signing on to our resolution recognizing June 20 as World FSHD Day.**
- **Supporting the FAA's reauthorization bill to ensure more accessible air travel – a landmark bill that was passed a week later.**
- **Supporting funding streams directed toward patient access, upper body mobility awareness, and FSHD research.**

Maggie Eggleston shares her experience with her home state congresswoman, Debbie Dingell (MI-6).

"We began the process of building the kinds of relationships we will need to advocate in the future for specific needs."

—GREGG LICHTENSTEIN

"Our goal was to humanize FSHD and express the urgency for a treatment."

—CHRIS CARRINO, THE CHRIS CARRINO FOUNDATION

"It is the first step in the legislative trajectory to ensure a cure is accessible for all affected, including children!"

—VERA TREONZE

Senator Mark Kelly (AZ) meeting with his constituents, Ally Roets and Sam Ray.

Greeting Rep. Doris Matsui (CA-7) before a successful meeting with her staff.

Senator Roger Wicker (MS) pauses to speak with our advocates.

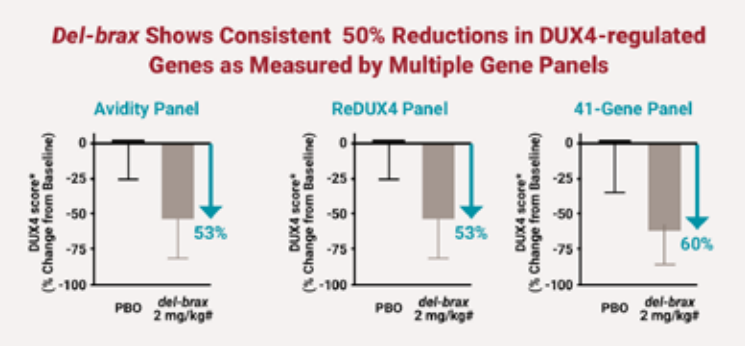
Avidity shares interim data from FSHD clinical trial

"Unprecedented" and "consistent" reductions in DUX4 activity

BY JUNE KINOSHITA, FSHD SOCIETY

Avidity Biosciences, a San Diego-based biopharmaceutical company, announced that its investigational therapy for facioscapulohumeral muscular dystrophy (FSHD) reduced by more than 50% the expression of genes that are regulated by *DUX4* – widely viewed as the gene that triggers muscle weakness and degeneration in FSHD. This is the first time anyone has shown that an experimental therapeutic for FSHD reduces *DUX4* expression in patients.

What's more, the company reported trends showing improvement in muscle strength and reachable workspace (a way of measuring the range of arm movement). Participants and clinicians reported positive improvements. Importantly, the therapy has favorable safety and tolerability, and no severe adverse events have been reported.



The data come from Avidity's FORTITUDE™ trial, a Phase 1/2 clinical trial of delpacibart braxlosiran, or "del-brax" for short (formerly AOC 1020), a molecule that targets the messenger RNA of *DUX4*. Researchers analyzed data from 12 participants who have been either on a placebo or on del-brax for four months (out of a 48-week dosing period).

The study's details were presented at the FSHD Society's International Research Congress on June 14 in Denver, Colorado, by Jeffrey Statland, MD, professor of neurology at the University of Kansas Medical Center and a FORTITUDE trial investigator.

Amy Halseth, PhD, the FSHD lead at Avidity, shared the results the following day at the FSHD Connect patient conference, where the news was greeted with applause and many a damp eye.

Biomarker found

Researchers have been seeking a reliable "circulating biomarker," a substance in the blood that would indicate whether or not *DUX4* is active. So it caused a bit of a stir when Avidity announced it had found one.

Avidity did not provide details in its press release, and we are eager to learn more.

Avidity also reported that creatine kinase levels were reduced by 25% or more in individuals on del-brax as compared to the placebo group. Creatine kinase is a well-established indicator of muscle damage, so a decrease is a good sign, but it is not specific to the FSHD disease process.

What this means

"These early data would support the notion that del-brax has the potential to change the course of disease for people living with FSHD," said Statland.

It's exciting that positive trends in functional improvements were seen only four months into dosing. Such a fast response raises the hope that Avidity may be able to shorten its trial and move into Phase 3 sooner than it had anticipated. The U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) have granted Orphan designation for del-brax and the FDA has granted del-brax Fast Track designation.

Avidity plans to accelerate initiation of registrational cohorts in the FORTITUDE trial starting in the second half of this year. The Phase 1/2 cohorts of the FORTITUDE trial are fully enrolled. Data generated from the upcoming registrational cohorts will be used as part of the data package evaluated by regulatory agencies for new drug approval. Avidity plans to share additional information about the upcoming trial cohorts with the FSHD community as it becomes available.

We are deeply grateful for the hard work and sacrifice of thousands of individuals living with FSHD who gave blood and muscle samples, filled out countless registry questionnaires, volunteered for natural history studies, lay inside MRI scanners, tested devices, and showed up in all kinds of ways. It's your dedication, your faith in the future, and your refusal to give up hope that have blazed the trail.

Muscle regeneration

A complementary approach for FSHD therapeutics

BY JUSTIN COHEN, PHD, NEW HAVEN, CONNECTICUT

This is an exciting time for facioscapulohumeral muscular dystrophy (FSHD) patients, with many therapeutics entering clinical trials. Most of these focus on targeting DUX4, the toxic protein that causes the disease. However, other approaches are also entering the clinic, including muscle regeneration to help regrow wasted muscle – something particularly useful for more severely affected patients.

Visualize FSHD muscle as a wooden boat, and *DUX4* as a hole with water coming in. For some patients, *DUX4*-specific therapies plug the hole, and that is all that will be needed because the wood is still sound. However, some may have significant water damage that "rots" the wood. This is where therapeutics for muscle regeneration come in, providing new wood in the rotted areas.

The major tool to accomplish this is stem cells – special cells that have the potential to become any cell in the body. However, stem cells in adults are difficult to grow and limited in what they can become. To overcome these obstacles, many studies use induced pluripotent stem cells (iPSCs), which are fully differentiated adult cells that have been reprogrammed to behave like fetal stem cells, with the potential to become any type of cell in the body.

Initial approaches in FSHD involved generating iPSCs from a patient's own cells to avoid immune rejection, correcting the mutation, and transplanting the cells back into the patient's body. However, this is costly and unscalable. Researchers are now focusing on generating healthy cells from a single donor with immune-evading modifications that can be banked and given to all patients.

While several labs including the Perlingeiro group at the University of Minnesota are studying this, Vita Therapeutics has recently announced plans to conduct a trial using this method in FSHD patients in approximately two years if this approach continues to be viable.

Other innovative studies on regeneration are also in development. The Badylak group at the University of Pittsburgh examines the extracellular matrix (ECM), a mixture of compounds that provide structural and biochemical support to cells, including stem cells. First demonstrated to restore some muscle lost to soldiers with war wounds, preliminary ECM studies in FSHD mice showed promising improvement in muscle function. The group is now following up by adding matrix-bound nanovesicles (MBV), signaling molecules that can "enhance" the impact of ECM, potentially through immune modulation.

Lastly, FSHD Canada Foundation, together with the SMA Foundation and Myologica, LLC, is pursuing an intriguing collaboration. In this "kitchen sink" approach, various potential muscle regeneration compounds being screened for spinal muscular atrophy are also being tested in FSHD. Most of these compounds enhance stem cells and are already being used in other diseases, which would reduce clinical trial time. One such compound, developed by Satellos Biosciences, Inc., increased muscle force in FSHD and Duchenne muscular dystrophy (DMD) mouse models. A clinical trial in DMD will begin this year.

Overall, there is intriguing progress on muscle regeneration, which has the potential to help the more severely affected FSHD patients. While not a cure, muscle regeneration holds promise and, in the future, may be combined with DUX4-targeting therapies to maximize benefits.

Visualize FSHD muscle as a wooden boat, and DUX4 as a hole with water coming in. For some patients, DUX4-specific therapies plug the hole. However, some may have significant water damage that "rots" the wood. This is where therapeutics for muscle regeneration come in, providing new wood in the rotted areas.

– JUSTIN COHEN

Why put off getting hearing aids?

They're now more affordable and available

BY RICH HOLMES, SANDWICH, MASSACHUSETTS

If you're straining to hear what people say, or find yourself cranking up the volume on your TV to the point others find loud, you probably need hearing aids.

High-frequency hearing loss, often mild, may occur in more than half of people with FSHD, but estimates vary. Hearing loss can be more profound in the early-onset form.

It's difficult to determine if hearing loss in an adult with FSHD resulted from dystrophy, other causes, or a combination, as hearing loss is common in the general population.

More than 15% of Americans over 18 have hearing loss, according to the National Health Interview Survey. The problem increases with age. Almost 80% of people over 70 have hearing loss, says the National Council on Aging.

The good news is that hearing aids have become easier to obtain and cheaper to buy. In August 2022, the US Food and Drug Administration issued a rule allowing direct sale of hearing aids to consumers without requiring a medical exam and doctor's prescription or a fitting by an audiologist.

These hearing aids are only for people with mild to moderate hearing loss.

The rule aims to lower prices through marketplace competition, and you've probably seen the TV commercials for these devices.

Coverage and price

Price has been a major obstacle to obtaining hearing aids, which many private health insurance plans and standard Medicare don't cover. However, some Medicare Advantage plans do cover them. And for children up through age 21 enrolled in Medicaid, hearing aids are covered in about half of the nation's states. If you're eligible for Veterans Affairs benefits, you may obtain hearing aids, be fitted by an audiologist, and have them maintained at no cost.

If you're considered disabled because of FSHD but wish to work, you may qualify for government assistance to obtain hearing aids, as well as other assistive devices or services. In Massachusetts, where I live, MassAbility (formerly the Massachusetts Rehabilitation Commission) oversees this function.

Some nonprofit organizations recycle hearing aids and may pay for a one-time service, such as an audiology appointment. Financial eligibility requirements may apply.

According to the National Council on Aging, the new over-the-counter (OTC) hearing aids cost about $1,600 on average. Some cost less and go for hundreds of dollars. In contrast, models available through audiologists cost $5,000 to $7,000.

Whatever the price, models vary in design (behind the ear, in the ear, hidden in the ear canal) and options such as Bluetooth connection to a phone app, battery lifespan, and whether batteries are rechargeable or not, as well as availability of online support. Do some research and read independent reviews before you buy.

Although buying a set of OTC hearing aids doesn't require first seeing an otolaryngologist or an audiologist, it's still a good idea to have your hearing evaluated by a professional if you can afford to do so.

Testing by a medical professional determines the degree and range of hearing loss, and an initial test establishes a baseline that future tests can be checked against to see if your loss is progressing and in what areas. Audiologists can clean and repair your hearing aids, and may check your ear canals for any wax buildup.

Hearing loss hurts

Maybe you think that you don't need the expense and bother of hearing aids, and can do just fine without them. Think again.

Hearing loss is associated with greater risk of dementia. It may cause affected people to interact less with others, leading to isolation that also contributes to developing dementia and poor mental health. Depression, anxiety – even a greater chance of falls – are associated with hearing loss, according to the National Council on Aging.

Fortunately, regular use of hearing aids can improve a person's psychological and social condition, says the

continued on page 18...

It's alive! It's alive!

A space-age solution for eating soup

BY AMY BEKIER, SAN DIEGO, CALIFORNIA

Hello, my fellow slurpy, sloppy FSHD superhero eaters!

For many of us with FSHD, as our hands, wrists, and arms grow weaker, it becomes quite difficult to raise an eating utensil to our mouths. Even when we succeed by using both hands, the utensil tends to turn, causing that tasty morsel to drop back onto the plate or splash into the bowl.

Our stained, battle-worn clothing gets more nutrients than we do.

You will often see us faceplanting our heads into the plate to bring the food to our mouths. Add the viscosity of a liquid or slippery meal and you might as well be trying to eat soup with a fork. Six hours later we're still slurping away, yet sadly little of it has landed in the intended pie hole.

Are you tired of launching peas across a crowded room? Don't want to look like a toddler while trying to impress a date?

"I am determined to eat independently for as long as I can."

–AMY BEKIER

There's got to be a better way

There are many of us who have reached a point where we need feeding assistance from a caregiver, but I felt that if there was a way to continue eating on my own, especially since I live alone, it was worth investigating. I am determined to eat independently for as long as I possibly can.

Many have tried to invent a better way. There are fat utensils, squishy utensils, utensils with handles, sippy cups, and plates with dividers. But none of these are completely effective in bringing food to one's mouth.

Then there are bulky, motorized arms for feeding, but they are over-the-top expensive and not easily transportable.

Without useful assistive devices for eating, FSHD folks tend to avoid certain foods or stick with finger foods.

I have become a master at searching the web and letting my fingers do the walking. While shopping online, I came across *liftware.com*. They have a video showing the utensil adapting and adjusting its angle no matter how the wrist is twisted.

Although skeptical and wary of the high price ($195), I bought one, figuring it is a one-time investment that is much less expensive than paying a caregiver to assist with feeding. I'm happy to report that my Liftware utensil has paid for itself through the years, multiple times over.

It's alive!

Liftware Level is a fat-handled, cool-looking, lightweight utensil with advanced sensor and motor-based self-leveling technology. No matter how you lift it using its self-charging handle, it will twist snakelike so that it always stays level facing upward to prevent food from falling off. It's as if it's alive. The added accessory can be a fork or spoon, but I find I only need to use the spoon.

The handle is springy, so I don't find it effective for anchoring a piece of steak while cutting it, but once the food is in bite-size pieces, the spoon works great.

At first, even though it kept the food level, I still had issues getting it up to my mouth. By experimenting I found that if I hold it from the very bottom of the handle, I can easily twist one hand, holding one hand with the other, to extend the length of the handle and the spoon closer to my mouth.

The device works not only for soup but for pasta, peas, cereal, or any small-particled tasties. There is even a different device for someone with tremors, such as Parkinson's, called Liftware Steady.

I am more confident when I go out to eat, and always receive questions and awe when using the device. Often people will approach my table to request a referral for a friend or family member.

Wouldn't you like to wow your friends with sleek, sexy, futuristic, space-age technology that is sure to impress and create conversation?

May the fork be with you!

Note: Search YouTube for Liftware to see the device in action. This article is for educational purposes. The FSHD Society does not endorse specific products.

Muscle loss with Ozempic® and similar drugs

What's the risk to people with muscular dystrophy?

BY RICH HOLMES, SANDWICH, MASSACHUSETTS

Heard about Ozempic, Mounjaro®, and related drugs and wonder if one might help you lose weight? That's a decision you should make with your doctor, but be aware of three points:

- Weight loss typically includes both fat and some muscle.
- How much muscle a person with FSHD might lose when taking one of these drugs isn't known.
- The severity of your FSHD, your age, and your general health should be considered.

The weight-loss drugs known as GLP-1 agonists include Ozempic and Wegovy® (both semaglutide), and Mounjaro (tirzepatide). They work like a hormone in your body named GLP-1 (glucagon-like peptide 1), one of a group of gut peptides called incretins that are secreted when you eat. GLP-1 agonists (drugs that stimulate GLP-1) cause the pancreas to make insulin and slow stomach emptying, as well as increasing muscle uptake of glucose and reducing liver creation of glucose, according to a January 2023 article in the National Library of Medicine. They've been used to treat type 2 diabetes and obesity, and are being eyed for other uses.

For a typical overweight or obese person who loses weight by dieting, about 20% to 30% of the loss may be lean tissue, according to a May 2017 article in *Advances in Nutrition*. But what about weight loss via one of these GLP-1 agonists?

"I think as of now data are really limited regarding the GLP-1 agonists and effects on muscle wasting in muscular dystrophy or FSHD," wrote Jeffrey Statland, MD, in a recent email. Dr. Statland is a professor of neurology and co-director of MDA clinics at the University of Kansas Medical Center in Lawrence. "There are articles suggesting some portion of the weight loss is lean muscle mass, likely due to the rapid weight loss, which may be exaggerated in people who already have a low lean muscle mass (e.g., aging or muscular dystrophy)."

Clinical trials have shown that these drugs can cause muscle loss:

- A 68-week-long trial of semaglutide involving 140 participants found each had an average loss of 23 pounds of fat and 15 pounds of lean muscle, according to a September 2023 *Fortune Well* article.
- A clinical trial involving Eli Lilly, the maker of Mounjaro (tirzepatide), reported in October 2023 that MRIs of 296 participants with type 2 diabetes not well controlled with metformin found that those given tirzepatide had more fat-free muscle loss but less fat infiltration of muscles than those given insulin.

Eli Lilly has pursued limiting muscle loss from these drugs by studying a drug called bimagrumab, an antibody that promotes muscle growth. Last August, the pharmaceutical giant announced it had completed acquisition of Versanis Bio, the company that makes bimagrumab. According to Eli Lilly's statement, the drug was being tested alone and with semaglutide in overweight and obese people, and bimagrumab and related drugs were being considered for use in combination with tirzepatide and other gut hormones.

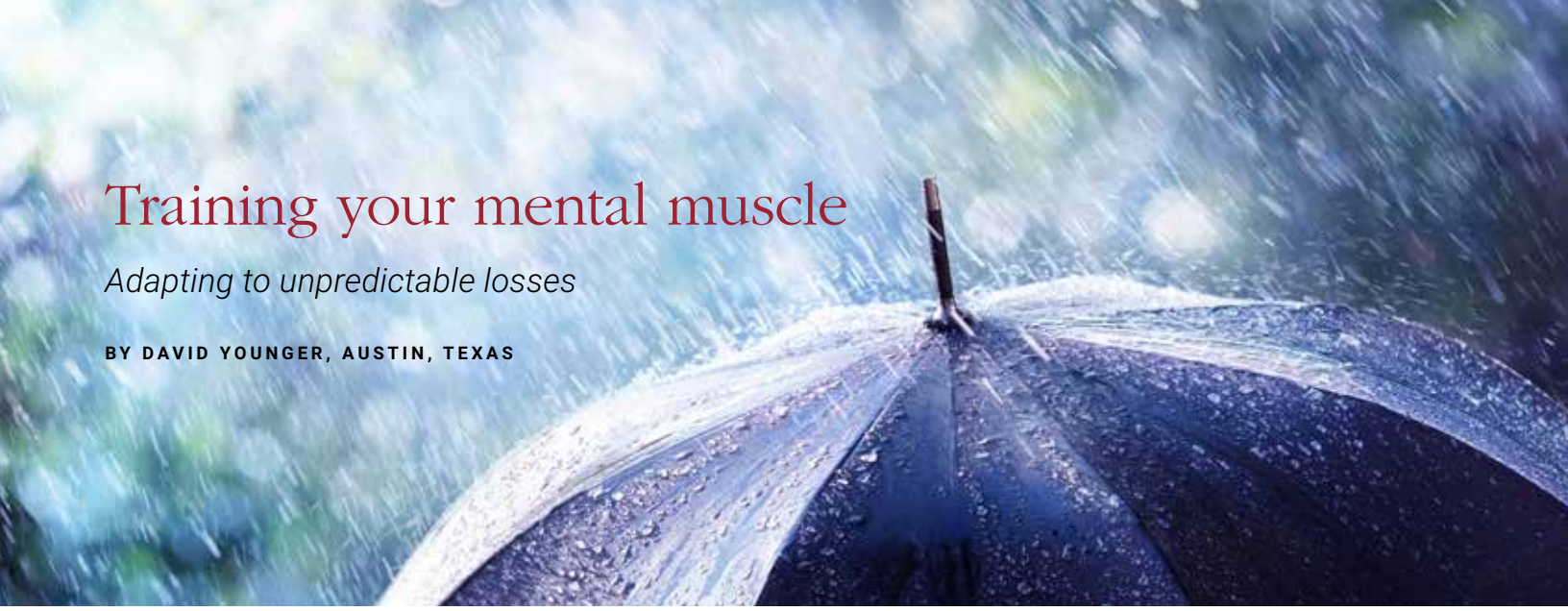
Fellow drug makers are taking similar steps, according to a March article in *Nature Biotechnology*. Regeneron is studying employing two of its antibodies with Ozempic, made by Novo Nordisk. Clinical trials start this year on drugs to target myostatin, a substance in our bodies that restricts muscle growth and other biological pathways, and to increase muscle stem cells. Drug developers hope to cash in on potentially treating both obesity and age-related muscle loss, known as sarcopenia.

Interestingly, a drug targeting myostatin is currently in a Phase 1/2 clinical trial for FSHD. It is intended to help slow or reverse muscle loss in the disease. (See trial update, page 18.)

GLP-1 agonists and age-related muscle loss

While we may not understand how these drugs affect people with FSHD, some research has been done on GLP-1 agonists and age-related muscle loss. This condition raises the likelihood of developing type 2 diabetes, as muscle uses most of the body's glucose (sugar), and significant muscle loss increases the risk of insulin resistance, according to

continued on page 22...



Training your mental muscle

Adapting to unpredictable losses

BY DAVID YOUNGER, AUSTIN, TEXAS

There's a Buddhist teaching or parable that I absolutely love called the Two Arrows Parable.

The first arrow is what life slings at us. Things like muscular dystrophy. Things that are completely outside of our control. The first arrow often doesn't kill us, but it can cause an awful lot of pain.

The second arrow we fire ourselves – at ourselves – in response to the first arrow. It encompasses all the ways we meet the inevitable first arrow when something is presented to us that we, to put it mildly, would rather send packing.

It's the second arrow that we fire in response to the first arrow that makes the pain unbearable. Unlike the first arrow, though, the second arrow is our own doing. We may not be consciously choosing to fire it, but it's a choice, nonetheless. We have the power and ability to break that second arrow.

There's a helpful acronym I use and share with all the people I treat in my psychology practice. It is RAIN, popularized by meditation teacher and author Tara Brach, who wrote a great book called *Radical Acceptance*.

The R of RAIN is Recognize. You need to start paying attention. If you're operating on autopilot, caught in an endless pattern of stimuli and responses, there's no room for learning, growth, and change. The R is about committing to practice paying attention. This alone can transform your life regardless of whether you have muscular dystrophy.

When you're having a reaction to something, and you notice that reaction and simply name it sadness, pain, frustration, you're practicing the R of RAIN, and you're pausing and creating the space for something new.

The A of RAIN is Acceptance. I'm feeling sad right now. I don't have to do anything about it. I don't have to get rid of it. It's okay to feel sadness. I'm not fighting it. I'm recognizing and accepting that it's there in this moment. Trying to deny it only makes it worse. That's a second arrow.

The I of RAIN is Investigating with compassion. Do I feel the sadness in my body? Are there thoughts arising around the sadness? Did something happen that prompted it? Is it reminding me of something? Are there any narratives I have about myself in the world that are getting activated around the sadness? All this investigation is with compassion and open-hearted curiosity. I want to understand the sadness so that I can best take care of myself.

That self-care is the N of RAIN. Nurturing. What does the sadness need right now to be honored, held, loved, and cared for?

So I'm recognizing that there's sadness. I'm accepting that it's there. I'm investigating it with compassion to understand it, and I'm nurturing the part of me that's feeling sad.

That's it. It's simple and straightforward, and you rinse and repeat throughout the day as different feelings and emotions arise. It's a practice. It's like a muscle you need to slowly strengthen and develop, and which is impervious to the muscular dystrophy.

The more you practice RAIN, the more aware you become of firing that second arrow. The more you're able to break it.

This is something no one can do for you but you. No one and nothing can take it from you. It's free and does not require any equipment other than your heart and your mind. I hope this inspires you to embrace the possibilities that are out there and within you to let go of what does not serve you and welcome more peace and equanimity.

Note: David Younger, PhD, was diagnosed with FSHD as a child. He received his doctorate in psychology from University College London and has a 100% online video-based practice. He lives in Austin with his wife and two children. Visit *dbyounger.com* for more information.

Why I exercise

To keep myself fit, ready, and aware of changes in my body

BY FRANK HANLEY, BOLIVIA, NORTH CAROLINA

This is an exciting time for us. The research, clinical trials, and recent announcements by pharma companies bring increasingly optimistic news that treatment for FSHD is getting closer by the day.

As research and development move forward utilizing innovative ways to provide treatment, what can we, the patients, do to prepare for the day a treatment arrives?

My neurologist told me, "Whatever you are doing, don't stop; it's working."

That was decades ago, when I saw the doctor at Johns Hopkins University. So what was I doing? I was exercising – qigong practice each morning and kung fu class three times a week. Qigong (pronounced chee gong) is a system of exercise rooted in Chinese medicine. Qigong promotes wellness by strengthening the flow of qi (life energy) through breathing, posture, and gentle movement.

I continue to exercise. Qigong every morning. I cut back kung fu to twice a week. I added weight training at the gym three days a week.

Here is why I exercise

- **To be ready.** With all the recent developments around clinical trials, treatments, and a potential cure, I want to be ready. I want to be in the best shape that I can be, both physically and mentally.
- **For prevention.** I'm afraid. I'm afraid that tomorrow I will wake up to find out that FSHD took an arm, or my other leg, or my diaphragm, or my smile. I exercise to keep myself as strong as possible and optimistic that what I am doing is working.
- **Self-awareness.** My exercise routine keeps me in constant contact with my physical condition. I continually monitor myself for changes, looking for weakness as an indicator of a possible problem.
- **I really enjoy it.** I started practicing kung fu when I was in my 20s, long before I knew anything about FSHD. Even then, I was struggling with weakness in my left shoulder, but I did not know it was FSHD. I started a family, so I stopped training for a while. In my early 30s, I developed foot drop and was diagnosed with FSHD. The doctor told me, "It won't kill you, and there's no cure." I have what I consider a mild case, so I got on with my life and learned to live with it.

In 2009 I joined a new kung fu school, where I was introduced to qigong. The new workouts really helped me. In weeks, I noticed improvements in my strength, coordination, and balance. I felt the training was helping me fight back against muscular dystrophy, both physically and mentally.

Feeling Fit with FSHD streams live on the second and fourth Thursday of each month at 12 p.m. EDT.

Feeling Fit with FSHD

I got excited when I was asked to help develop the Feeling Fit with FSHD program with the FSHD Society. Here is a chance for me to share the exercises that I find work for me. It is an opportunity to reach out to others and share my workouts.

The Feeling Fit with FSHD program is offered through the Society's Gathering Place online community. Join us and find some exercises that you like and that work for you.

Feeling Fit with FSHD streams live on the second and fourth Thursday of each month at 12 p.m. Eastern time. Register on the Society's website to get the Zoom link, and check out the recordings on the Society's YouTube channel.

Note: Frank Hanley is a martial artist, author, and teacher of kung fu, taijiquan, and multiple qigong styles and practices. Frank has been practicing martial and energy arts for more than 30 years and presents workshops at local, national, and global wellness events and conferences. He created and hosts the FSHD Wellness Channel Facebook group, and is a frequent host and presenter on the FSHD Society's Feeling Fit with FSHD Zoom group. Frank can be contacted at *qigongforfshd@gmail.com*.

The FSHD Clinical Trial Research Network

A GAME CHANGER – HERE'S WHAT YOU HELPED LAUNCH

Founded in 2016 with a cornerstone grant from the FSHD Society, the FSHD Clinical Trial Research Network (CTRN) has grown quickly. Starting from four sites in the US, the network has expanded to 32 sites and 11 countries to meet global needs. Funding partners include: Avidity Biosciences, Dyne Therapeutics, Friends of FSH Research, FSHD Canada Foundation, FSHD Society, Fulcrum Therapeutics, Muscular Dystrophy Association, and National Institute of Neurological Disorders and Stroke, and anonymous donor. Current sites are listed below.

United States

- Duke University, NC: Natalie Katz, MD PhD
- Kennedy Krieger Institute, Baltimore, MD: Doris Leung, MD PhD
- Nationwide Children's Hospital, Columbus, OH: Linda Lowes, PT PhD
- Stanford University School of Medicine, CA: John W. Day, MD PhD
- The Ohio State University Medical Center, Columbus: Bakri Elsheikh, MD
- University of California Los Angeles: Perry Shieh, MD PhD
- University of California San Diego: Chamindra Laverty, MD
- University of Colorado: Stacy Dixon, MD
- University of Florida Gainesville: S. H. Subramony, MD
- University of Iowa: Katherine Mathews, MD
- University of Kansas Medical Center, Kansas City: Jeffrey Statland, MD
- University of Rochester Medical Center, NY: Johanna Hamel, MD
- University of Texas at Austin: Yessar Hussain, MD
- University of Texas at San Antonio: Matthew Wicklund, MD
- University of Texas Southwestern Medical Center: Jaya Trivedi, MD
- University of Utah, Salt Lake City: Russell Butterfield, MD
- University of Washington, Seattle: Leo H. Wang, MD PhD
- Virginia Commonwealth University, Richmond: Nicholas Johnson, MD

Canada

- McGill University: Erin O'Ferrall, MD; Angela Genge, MD
- University of Calgary (pending): Lawrence Korngut, MD
- University of Ottawa: Hanns Lochmuller, MD

Europe

- Institute of Myology, Paris, France: Guillaume Bassez, PhD
- Münich University, Germany: Bendikt Schoser, MD
- Radboud University, Nijmegen, Netherlands: Nicol Voermans, MD PhD
- St. George's University Hospitals, UK: Emma Matthews, MBChB
- University College London, UK: Enrico Bugiardini, MD
- University of Copenhagen, Denmark: John Vissing, MD
- University of Milan, Italy: Valeria Sansone, MD
- University of Nice, France: Sabrina Sacconi, MD PhD
- University of Sheffield, UK: Channa Hewamadduma, MD

South America and Australia

- University of Melbourne, Australia: Ian Woodcock, MD
- University of São Paolo, Brazil: Cristiane Moreno, MD PhD

Project Mercury progress report

BY KEN KAHTAVA, FSHD SOCIETY

The FSHD Society is the program manager for Project Mercury, the global collaboration initiative to speed therapies to people affected by FSHD. Below, we update you on progress in Project Mercury's three workstreams: Patient Access, Clinical Trial Readiness, and Sustainability. Read more at *projectmercuryfshd.org*.

PROJECT MERCURY
The Global Initiative to Speed the Delivery of Therapies for FSHD

Patient Access

Ensuring patients can access treatments once they become available requires that regulators and payors have a common understanding of how FSHD progresses. A working group of experienced FSHD clinicians across North America and Europe has been formed, and the research is underway. Their work is expected to take approximately 18 months to complete and will be incorporated into an FSHD data package, which will be made available to drug developers to expedite their own work with regulators and payors.

Clinical Trial Readiness

A partnership between two of the advocacy groups on the Global Task Force (FSHD Society and FSHD Canada Foundation); a healthcare data and clinical trial innovation group called Lumiio; and a global provider of clinical trial services, TRiNDS, has been formed to accelerate and improve clinical trials. Called Global FSHD Innovation Hub, it provides biopharma industry companies and healthcare technology providers an end-to-end solution where biopharma can accelerate trials for their therapeutics anywhere in the world, and where Health IT companies can validate innovative approaches to trial assessments and outcomes (e.g., wearable devices).

Sustainability

Patient advocacy organizations lead countrywide working groups in Canada, the US, the United Kingdom, Australia, Brazil, and many countries in Europe. These working groups are essential to all aspects of Project Mercury's work, especially in areas of patient engagement in research. Recent additions include Spain and Italy. Updates on country working group activities can be found on *projectmercuryFSHD.org*.

... *from page 12*

Why put off getting hearing aids?

American Speech-Language-Hearing Association.

Hearing aids can't restore hearing. But boosting the volume of the frequencies you're missing makes a huge difference.

A doctor told me years ago after viewing my hearing test results that I probably had trouble differentiating F from S sounds, or understanding people with higher-pitched voices (typically, women and children). In fact, I frequently misheard or misunderstood what my wife was saying. Trying to hold up my end of a conversation in a noisy restaurant was challenging. Getting hearing aids has greatly enhanced my life.

On average, Americans with hearing loss wait 10 years before seeking help. Reasons for stalling can be cost, vanity, or just being unaware of the options. Given the downsides of going without treatment – and the lower-cost, sleeker options now available – it makes sense to address the problem once you recognize it – not later. Help is out there.

Clinical trial snapshot

JUNE KINOSHITA AND AMANDA HILL, FSHD SOCIETY

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 July 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.

REACH sponsored by Fulcrum Therapeutics

QUICK FACTS	
Drug	Losmapimod
How Is It Given?	Pill, taken twice a day
Phase	3
Participants	260
Placebo	Yes, 1:1
Genetic Testing	Required, provided by study
Rx Duration	48 weeks in the double-blind stage
Study Visits	6 + screening and follow-up
Notable Activities	MRI
Open-Label Extension	Yes

STATUS	
Enrollment	Completed
Data Expected	Q4 2024
Locations	US, Canada, Denmark, France, Germany, Italy, Netherlands, Spain, UK
Learn More	*clinicaltrials.gov/study/NCT05397470* *fshdsociety.org/fulcrum-reach-trial*

WHO CAN PARTICIPATE?

- Age 18-65
- FSHD1 or FSHD2
- Ricci score 2-4 (cannot be dependent on wheelchair or walker for activities)
- Reachable Workspace total RSA 0.2-0.7
- Must be able to do MRI

Fulcrum Therapeutics

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

QUICK FACTS	
Drug	Satralizumab
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

WHO CAN PARTICIPATE?

- Age 18-65
- FSHD1
- Ricci score 2-4, able to walk without support
- Must be able to do MRI

STATUS	
Enrollment	Currently enrolling
Data Expected	After 2027
Locations	Ottawa, Canada; Nice, France
Learn More	*clinicaltrials.gov/study/NCT06222827*

FORTITUDE sponsored by Avidity Biosciences

QUICK FACTS	
Drug	Del-brax (previously AOC 1020)
How Is It Given?	Intravenous infusion
Phase	1/2a
Participants	72
Placebo	Yes, 2:1
Genetic Testing	Required, provided by study
Rx Duration	5 doses over 9 months
Study Visits	~20, some may be virtual
Notable Activities	MRI, leg muscle biopsy
Open-Label Extension	Yes

WHO CAN PARTICIPATE?

- Age 18-65
- 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

STATUS	
Enrollment	Currently enrolling
Data Expected	Preliminary data announced in Q2 2024 (see p. 10)
Locations	US, Canada, UK
Learn More	*fortitude-study.com* *clinicaltrials.gov/study/NCT05747924* *fshdsociety.org/avidity-fortitude-trial/*

MANOEUVRE sponsored by Hoffmann-La Roche

QUICK FACTS	
Drug	GYM329 (aka RO7204239)
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

WHO CAN PARTICIPATE?

- Age 18-65
- FSHD1 or FSHD2
- Ricci score: ≥ 2.5 and ≤ 4 (must be able to walk unassisted)
- Must be able to do MRI

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/*

ARO-DUX4 trial sponsored by Arrowhead Pharmaceuticals

QUICK FACTS	
Drug	ARO-DUX4
How Is It Given?	Intravenous injection
Phase	1/2a
Participants	52
Placebo	Yes, 3:1
Genetic Testing	Required, provided by study
Rx Duration	Part 1: duration 3 months Part 2: 2 or 4 doses over 1 year
Study Visits	~20
Notable Activities	MRI, leg muscle biopsy
Open-Label Extension	Yes

WHO CAN PARTICIPATE?

- Age 18-70
- FSHD1
- Clinical Severity Scale 3-8
- Must have leg muscle suitable for biopsy and be able to do MRI

STATUS	
Enrollment	Currently enrolling
Data Expected	TBD
Locations	New Zealand, Canada (TBC)
Learn More	*clinicaltrials.gov/study/NCT06131983* *fshdsociety.org/clinical-trials/arrowhead-trial/*

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:

... from page 14

Muscle loss with Ozempic® and similar drugs

a review of research on diabetes drugs and sarcopenia published in November 2021 in *World Journal of Clinical Cases*. Obese older people with type 2 diabetes experience more sarcopenia than those without diabetes.

The review found mixed results for GLP-1 agonists. On the positive side, it found two drugs countered muscle atrophy in mice. One, dulaglutide, restored muscle mass and function in mice used as a model for Duchenne muscular dystrophy, a finding originally reported in an August 2019 article in *Journal of Cachexia, Sarcopenia and Muscle.*

A second drug, exendin-4, increased muscle mass and function in mice with muscle atrophy induced by dexamethasone, a glucocorticoid. The review also found that in a very small study, nine obese diabetic patients treated for 24 weeks with liraglutide, another GLP-1 agonist, lost fat while their skeletal muscle mass apparently stabilized.

On the negative side, the review found a six-month treatment of dulaglutide with insulin in diabetics receiving dialysis shrank both fat and muscle mass. The review concluded it is uncertain whether GLP-1 drugs are beneficial or not regarding muscle mass and strength.

A November 2023 article in *Drug Discovery & Development* reported some well-designed studies have shown that the weight loss from these drugs includes a substantial amount of lean tissue – ranging from 20% to 50% of the total number of pounds shed. According to the article, these results are similar to those from weight loss through dieting or bariatric surgery. It noted that a review of research indicates these drugs may improve body composition and possibly offer some protection against muscle wasting.

Comments from FSHD experts

Physicians familiar with FSHD who were contacted for this story haven't yet seen many patients taking one of these drugs. Some said exercise might help limit drug-related muscle loss. While cautious about the risk, they recognized the considerable benefits weight loss could bring.

"The subject has come up in regard to other patients with muscular dystrophy. There is not much evidence that these meds lead to increased weakness. No good, large study has been done, of course (e.g., a randomized controlled trial of GLP-1 vs. no such treatment in patients with dystrophy). So now it is anecdotal. But from my limited experience, I have not seen any significant trend to worsening weakness," wrote Anthony A. Amato, MD, neurology department vice chair and distinguished chair in neurology at Brigham and Women's Hospital's Neurosciences Center in Boston.

"I follow a couple of patients taking these; one feels the weight loss has contributed significantly to improved mobility. But I agree that muscle loss in those more severely affected could be a big concern," wrote Lawrence J. Hayward, MD PhD, professor of neurology and director of the Neuromuscular Division and FSHD Clinic at the UMass Chan Medical School in Worcester, Massachusetts.

Dr. Hayward suggested that, in light of the lack of research specific to people with FSHD, those who take these drugs should be closely followed by their doctors.

"Balancing weight loss with increasing activity as tolerated may be helpful to maintain muscle mass," he added.

"Looks like GLP-1 agonists are very effective in lowering insulin levels and reducing fat. There is potential concern the drug can cause loss of bone density and loss of muscle mass (sarcopenia). However, studies in mice suggest that GLP-1 agonists may actually increase muscle mass," wrote Rabi N. Tawil, MD, co-director (until his recent retirement) of the MDA Neuromuscular Disease Clinic at the University of Rochester.

According to Dr. Tawil, a clinic patient who reported taking a GLP-1 agonist for six months experienced significant weight loss and improved motor function.

"Whether the improved function is due to loss of weight or stronger muscles remains to be seen," he added.

Dr. Statland urged patients to discuss the pros and cons with their physician, "weighing the risk of rapid weight loss and loss of lean mass against the functional improvement people may experience with weight loss and increased mobility.

"I agree certainly a protein-rich diet and aerobic exercise likely will help to some degree mitigate this – although there have not been studies verifying this," he continued. "My recommendation is if weight loss is a goal and someone has had difficulty with weight loss using diet and exercise – they could try the drugs. But they should develop a plan to monitor with their physician or neurologist: A physical regimen with an activity they can monitor while taking the drug, maintain a high-protein diet, and if they feel they notice muscle wasting, let their doctor know.

"In the meantime, on the medical side, we are trying to collect the experience of our patients taking this class of medication and follow their outcomes," Dr. Statland wrote. "Hopefully we will have more detailed guidance in the not-too-distant future."

Noteworthy

Rabi Tawil steps down from a storied career

Rabi Tawil, MD, a pioneering leader in FSHD research, retired this summer after a 36-year career at the University of Rochester Medical Center. He was co-director of the MDA Neuromuscular Disease Clinic. As director of the Neuromuscular Pathology Laboratory, he was responsible for the interpretation of all muscle biopsy samples used for diagnosing muscle diseases. Together with Jeffrey Statland, he co-founded the FSHD Clinical Trial Research Network (CTRN), which has created 32 international sites for FSHD research. Dr. Tawil also established the largest FSHD biorepository in the world, which has been essential in facilitating many discoveries in FSHD. Dr. Tawil's seminal role in the field was recognized by the FSHD Society's Pioneer Award.

FDA listening session this August

The FSHD Society has secured an FDA listening session on the topic of upper-body mobility, which will be held this August. Our purpose is to impress upon the FDA the outsized impact that shoulder and arm weakness has on quality of life, activities of daily living, and ability to maintain independence. A deeper understanding of this aspect of FSHD will lead to more effective review and regulation of future therapies. We also want the FDA to recognize upper-body weakness as a benchmark of disease progression.

In addition, by having this discussion, we will be further educating the FDA on the FSHD patient experience, and what is important to the FSHD community.

Upcoming

FUNDRAISERS

August 18: Falmouth Road Race. Support our team by making a donation!

September & October: Walk & Roll to Cure FSHD. Our flagship grassroots fundraiser will be rolling across North America this September and October. Visit *fshdsociety.org/walkroll* for locations and dates.

CONFERENCES

October 5: FSHD 360, Kansas City

November 2: New England Chapter patient day, Worcester, MA

WEBINARS

Please register in advance to get the link.

August 29: BetterLife FSHD

September 19: Insurance coverage for costly rare-disease treatments

October 17: Good bad things

November 21: ReSolve – what we have learned

December 19: Annual drug development update

FSHD SOCIETY RADIO

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 fall, 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.

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.

Early-Onset Parent Roundtable
Third Tuesday of every month at 8 p.m. ET | 7 p.m. CT | 6 p.m. MT | 5 p.m. PT.

August 20 **October 15**
September 17 **November 19**

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

August 27 **October 29**
September 24 **November 26**

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

August 12 **October 14**
September 9 **November 11**

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.

August 7 **October 2**
September 4 **November 6**

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

August 8 & 22 **October 10 & 24**
September 12 & 26 **November 14 & 26 (Tuesday)**

Young Adults
A Zoom community for empowering young adults impacted by FSHD. Meeting time has changed to the third Monday of each month at 8 p.m. ET | 7 p.m. CT | 6 p.m. MT | 5 p.m. PT.

August 19 **October 21**
September 16 **November 18**

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

FSHD Advocate

FSHD Society
75 North Main Street
Suite 1073
Randolph, MA 02368

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WALK & ROLL TO CURE FSHD

The Walk & Roll to Cure FSHD funds FSHD research, raises awareness, builds community, makes a meaningful impact, and creates lasting memories. We'd love to have YOU be a part of this effort to move us all closer to a cure.

MOVING CLOSER TO A CURE

Walk & Roll to Cure FSHD events are happening across North America this fall! Register now, and then invite your family, friends, and colleagues to join you!

Scan the QR code or visit *FSHDSociety.org/WalkRoll* – find a Walk & Roll near you, or join our virtual event today!

REGISTER TODAY!

FSHDSociety.org/WalkRoll