



Preliminary Agenda

Friday, June 14 – Pre-conference Events

5:30–7:00 p.m. **Registration open**
Lower level 2

6:00–8:00 p.m. **Joint reception for the International Research Congress & FSHD Connect**
The Plaza, outside, main
level

Saturday, June 15 – FSHD Connect Day 1

8:00–9:00 a.m. **Registration and Healthy Start continental breakfast**
Lower level 2

9:00 a.m.–12:15 p.m. **Morning sessions**
Colorado Ballroom E-J

9:00 a.m. **Welcome**

Beth Johnston, chief community engagement officer, FSHD Society
Beth's journey with the FSHD Society began shortly after her husband, Jeff, was diagnosed more than 20 years ago. She began volunteer fundraising for the FSHD Society as the co-chair of New York's "Festive Evening of Song" event from 2009 to 2014. Beth served on the Board of Directors from 2011 to 2014, and then joined the staff of the FSHD Society in 2016. Prior to joining the Society, Beth worked in social media marketing, real estate, and business consulting. She has a master of business administration degree from the University of Denver. Beth and Jeff have 2 adult daughters, and live in Denver, Colorado.

Introductory remarks

Mark Stone, president & CEO, FSHD Society
Mark has served as an executive leader of research-focused patient advocacy nonprofit organizations for the past 18 years, including the past 7 at the FSHD Society. Passionate about helping organizations

work to accelerate treatments and cures in diseases that are both rare and of a genetic origin, he has successfully engaged patients, families, and friends in raising up an “army of activists” while advocating with pharmaceuticals, the National Institutes of Health (NIH), and the FDA to increase funding levels and enlarge the pipeline of potential therapies.

9:10 a.m.

Finding your rope team to overcome any barriers

Command Sergeant Major (Ret.) Gretchen Evans

Texas native Gretchen Evans is one of the most decorated female veterans in US history. She held leadership roles in multiple combat engagements throughout her 27 years in the Army, including overseeing more than 30,000 ground troops in Afghanistan. While she was serving in Afghanistan in 2006, Gretchen’s world changed in a millisecond when a rocket blast caused debilitating injuries, including a traumatic brain injury, internal injuries, and the loss of all hearing – ending her military career. With the help of her guide dog, her “rope team” of friends and supporters, and a healthy dose of grit, Gretchen navigated her way through a changed life and adopted a new mission: To advocate on behalf of wounded veterans. In 2019, she founded Team UNBROKEN, a mixed-ability athletic team, and competed in the World’s Toughest Race, currently airing on Amazon Prime. For her tireless dedication to supporting others, Gretchen received the Pat Tillman Award for Service at the ESPYs on ABC.

10:05 a.m.

Where we are in FSHD and how we got here

Nicholas Johnson, MD, Virginia Commonwealth University

This talk will provide a historical overview of FSHD, focusing on key steps that have brought us to the age of therapeutic trials. We will discuss the current state of the field with an eye toward how the community can prepare for the future. Dr. Johnson is professor and vice chair of research, and director of the Center for Inherited Muscle Research (CIMR) at Virginia Commonwealth University.

10:30 a.m.

A new biomarker for FSHD?

Sujatha Jagannathan, PhD, University of Colorado Anschutz Medical Center Campus

FSHD is characterized by death of muscle cells due to aberrant expression of DUX4. Current methods to monitor progression of FSHD and response to treatments at a molecular level require invasive muscle biopsies. FSHD biomarkers in blood would reduce patient hardship and allow for more frequent tracking of disease status during treatment and clinical trials. We seek to develop FSHD biomarkers from cell-free DNA (cfDNA) – free-floating DNA that arises from genomic DNA shed by cells, which then ends up in blood, saliva,

and urine. We propose to harness information from cfDNA to detect the physiological state of an individual, including FSHD disease state.

10:35 a.m.

Treatment for FSHD – a broader view

Nicol Voermans, MD PhD, Radboud University Medical Center

It is generally known that there is “no cure for FSHD,” but this does not mean there is no treatment. Treatment and cure are two different things. There are many treatments already for FSHD. Indeed, most of the drugs being developed are not a cure but a treatment. A treatment leads to an improvement in health, even if it may not eliminate, or cure, the disease. Medical professionals use medicine, therapy, surgery, and other treatments to help lessen the symptoms. Dr. Voermans will discuss the multidisciplinary, patient-centered approach at Radboud University Medical Center, a world leader in clinical research and care for neuromuscular disorders.

11:00–11:25 a.m.

Morning break

11:25 a.m.

Care to enhance the quality of life

Molly Morrill, certified nurse practitioner

An ACHPN (Advanced Certified Hospice and Palliative Nurse), Molly is a nurse practitioner at Pathways Hospice & Palliative Care in northern Colorado and has been part of their care network for the past 9+ years. The world of healthcare can be overwhelming, intimidating, and complicated. We will spend some time unpacking the basics of community resources, looking at the differences among care environments, and conclude with a deep dive into hard conversations about advance directives for people with chronic, progressive conditions like FSHD so that you and your families can have the education you need to make the best choices for your care.

11:50 a.m.

FORTITUDE™ Phase 1/2 trial – interim results

Amy Halseth, PhD, Avidity Biosciences

AOC 1020 is an antibody-oligonucleotide conjugate (AOC™) being developed to target the root cause of FSHD. AOC 1020 is currently being evaluated in the FORTITUDE™ study, a Phase 1/2 clinical trial in adults with FSHD. Avidity designed FORTITUDE in partnership with patients, caregivers, advocates, and key physicians in the field to ensure the study best meets the needs of the FSHD community. The primary goal is to evaluate the safety and tolerability of AOC 1020. Participants are randomly assigned to receive 4 doses of either AOC 1020 or a placebo via intravenous infusion during the 12-month treatment period. Preliminary results from FORTITUDE support the continued development of AOC 1020.

12:15–1:00 p.m.

Lunch

Colorado Ballroom E-J

1:00–5:30 p.m.

Colorado Ballroom A-B

My FSHD video shoot (sign up in advance for a time slot)

Jared Jacobsen, videographer

Jared, whose father co-founded the FSHD Society, is a documentary filmmaker whom we've asked to record your stories about the impact FSHD has had on you and your loved ones. One area of focus is the upper body, as many people do not understand how loss of shoulder and arm mobility can lead to loss of independence. Your testimonies will help us deliver a strong message about the urgent need for better treatments to the FDA, policymakers, and the public.

1:00–5:30 p.m.

Lower level 1

Mattie Silks

My FSHD photo shoot (sign up in advance for a time slot)

Jacob Pollock, photographer

Jacob has been working as a professional photographer in the Naples, Florida, area for 7 years. He looks forward to creating portraits of you and your loved ones. "I have worked with the FSHD Society in the past and have a personal connection to the Society through my father, who lives everyday with FSHD," he says. "My father has shown me that despite any setbacks, you can accomplish anything you set your mind to, including his Ride Across America race to raise funds for FSHD research. While I may not ride my bike across the country, I can still be a great photographer!"

1:00–5:30 p.m.

Lower level 1

Afternoon breakout sessions

1:00–2:00 p.m.

Breakout session A1

Matchless

Meet the doctors

With Stacy Dixon, MD; Nick Johnson, MD; Kaity Smith, CGC; Molly Morrill, NP; and Nicol Voermans, MD PhD

Meet the doctors who spoke this morning, along with their colleagues working in pediatric neurology and genetic counseling. They have as much to learn from you as you from them. While they cannot provide specific medical advice (as they are not your physician), they can share their knowledge. This is also an opportunity to share your experiences and observations as people living with FSHD.

Gold Coin

Pediatric and early-onset FSHD forum

Debbie Eggleston and Ally Roets, with Drs. Eva Chin, Linda Lowes, Julie Parsons, Ian Woodcock, and biopharma representatives

This session will be held only once and not be repeated at 2:15.

Meet with some of our most influential clinicians and FSHD patients from the early-onset and pediatric communities. We will host a conversational session where children and teens with FSHD, people

(of all ages) with early-onset FSHD, and their families can share their stories, ask and answer questions about their experiences, and discuss hopes for future treatments. The pediatric and early-onset FSHD community will raise awareness about the issues that are unique to them. If you are a young person with FSHD or an adult who was diagnosed as a child, please join us!

Independence

Managing and reducing FSHD pain

Kurt Spiegel, presenter and discussion moderator

Many people with FSHD experience some degree of pain, but struggle with moderating and reducing the suffering. By examining the causes of the pain and evaluating options to address both mind and body, we can begin the journey to better health. Therapies range from pharmaceuticals to mindfulness and will even factor in what some might consider controversial therapies such as THC and CBD from marijuana. Applying available therapies and using a proven methodology, we can identify solutions that can become part of our daily regimen and help us take action toward well-being and living our best lives.

Kurt Spiegel hails from Evergreen, Colorado, and serves as the Colorado Chapter director of the FSHD Society. He is an outdoor enthusiast but had to give up being an endurance biker, snowboarder, and runner due to his FSHD symptoms. Experiencing pain from FSHD, he applied his computer science background and problem-solving skills to develop strategies for mitigating chronic pain. With constant improvement, he hopes to ride once again.

Penrose Ballroom 2

Orthotic interventions for improved mobility

Caitlin Prechel, certified prosthetist orthotist

Caitlin practices at Rise Prosthetics and Orthotics in Denver. The use of orthotic bracing, mainly ankle-foot orthoses, can greatly improve mobility and safety during ambulation by increasing stability at the ankle. There are several orthotic options that can be pursued based on each individual's muscle strength, range of motion, ankle position, and goals.

Penrose Ballroom 1

Accessible vans

Dylan Murray, certified mobility consultant, and James White, service manager, MobilityWorks, Denver

Dylan Murray is a leading expert in mobility equipment and vehicles. He will be discussing the technology and options that exist in the wheelchair-accessible vehicle space. James White will be discussing the importance of maintaining your wheelchair-accessible vehicle, and tips and tricks to get your vehicle in peak condition. Attendees will be escorted to a hotel parking area to see one of the

MobilityWorks vans. An open Q&A session will follow their presentation.

2:00–2:15 p.m.

Break/switch rooms

2:15–3:10 p.m.

Breakout session A2

Matchless

Meet the doctors – *See description of 1:00 p.m. session*

Gold Coin

Let's make art!

Debbie Eggleston, Ally Roets, and Andrea Vanbeek

Join us for this all-ages session, where our young attendees can join forces with folks from biopharmaceutical companies and build community over a collaborative art project. In this hands-on activity, we will create a lasting piece that displays our collective ingenuity and imagination. Get your creative juices flowing and show the world what we can do together!

Independence

Pain management – *See description of 1:00 p.m. session*

Penrose Ballroom 2

Orthotics – *See description of 1:00 p.m. session*

Penrose Ballroom 1

Accessible vans – *See description of 1:00 p.m. session*

3:10–3:25 p.m.

Break/switch rooms

3:25–4:20 p.m.

Breakout session B1

Matchless

Meet the scientists

Sujatha Jagannathan, PhD, University of Colorado Anschutz Medical Center Campus

Here's your chance to discuss the science of FSHD with one of the leading researchers in the field. Have your questions answered about the DUX4 gene, figure out the difference between FSHD type 1 and 2, learn about what scientists know and don't know regarding the symptoms that you live with every day. Sujatha will help you to understand. If you want someone to critique your pet theory, now's your chance. Maybe you'll even brainstorm some new ideas for future research.

Gold Coin

Energy self-management

Ria de Haas, PhD, project manager FSHD Europe/FSHD European Trial Network

As there is no cure for most neuromuscular diseases (NMDs), including FSHD, managing the symptoms is essential to participate in daily activities. More than 60% of patients with NMD report fatigue

as their most disabling symptom. A self-management outpatient group program called Energetic has been developed to improve the social participation and physical endurance of patients with NMD and chronic fatigue. This program combines aerobic exercise training (AET), education about AET and energy conservation management (ECM), with relapse prevention and implementation in daily life. The Energetic program can be implemented in different healthcare settings and proved to be a facilitator for improving self-management.

Independence

Better nutrition for FSHD

Facilitators: Selina Lai and Kurt Spiegel

We want to help the FSHD community understand that research suggests that your diet is “medicine” for FSHD. The foods you eat impact your disease and can support improved muscle function in combination with supplementation and exercise. Using real FSHD patient food records, we will explore the journey to better nutrition for FSHD and discuss the dietary choices that we make each day. Selina Lai, from the Seattle area, has a registered dietitian credential and has practiced in nutrition for physical fitness and weight management for seniors and young children. She has been learning about the nutrition needs of FSHD patients, as several of her family members have FSHD. Kurt Spiegel hails from Evergreen, Colorado, and serves as the Colorado Chapter director of the FSHD Society. While not classically trained in nutrition sciences, he did spend 7 years as a professional chef, conferred with a registered dietitian to maximize athletic endurance performance, and wrote a sports nutrition cookbook. Always an advocate of healthy living and eating flavorful foods, he has managed to leverage his daily meals to get the best nutrition to combat the symptoms of FSHD.

Penrose Ballroom 2

FSHD and the role of physical therapy

Robert Will, DPT, University of Colorado School of Medicine
Physical therapy’s goal is to help maintain people’s independence through evidence-based care. Physical therapy works best as a collaborative partnership between patients and clinicians. This talk will focus on current physical therapy research, exercise recommendations, and shared decision-making processes with the goal of helping people with FSHD get the most out of their interactions with physical therapists and other rehabilitation professionals.

Penrose Ballroom 1

Assistive technology for computer access and adaptive gaming

Claire Simpson, MS, OTR/L, University of Colorado

Claire is a research instructor with the Center for Inclusive Design and Engineering. She has worked as an occupational therapist in pediatric and adult settings. This session will demonstrate various assistive technologies that can be used as alternate means of accessing a computer, phone, tablet, or video gaming technology. Efficient and effective access to computers and mobile technology is increasingly important in everyday life for education, employment, communication, access to goods and services, and more. However, some individuals with FSHD may not be able to use the computers outfitted with the standard keyboard, mouse, or touchscreen. Assistive technologies can provide alternate means of using computers with the hands, or with other body parts if functional use of the hands and arms is limited.

4:35–5:30 p.m.	Break/switch rooms Breakout session B2
Matchless	Meet the scientists – <i>See description of 3:20 p.m. session</i>
Gold Coin	Energy self-management – <i>See description of 3:20 p.m. session</i>
Independence	Better nutrition for FSHD – <i>See description of 3:20 p.m. session</i>
Penrose Ballroom 2	FSHD and physical therapy – <i>See description of 3:20 p.m. session</i>
Penrose Ballroom 1	Assistive technology for computer access and adaptive gaming – <i>See description of 3:20 p.m. session</i>
5:30–7:00 p.m.	Break
7:00–9:30 p.m. Colorado Ballroom E-J	Banquet and social time

Sunday, June 16 – FSHD Connect Day 2

8:00–9:00 a.m.

Registration and Healthy Start continental breakfast

9:00 a.m.–12:25 p.m.

Morning Sessions

Colorado Ballroom E-J

9:00 a.m.

Welcome to Day 2

Beth Johnston, chief community engagement officer, FSHD Society

9:05 a.m.

Where are we in our understanding of FSHD and of our therapeutic trials?

Lucienne Ronco, PhD, interim chief science officer, FSHD Society

Dr. Ronco will discuss the genetic and molecular basis of FSHD, including the role of the DUX4 gene. She will review novel findings and concepts in our understanding of FSHD, describe the drug development and clinical trial process, and review current trials.

9:45 a.m.

Participating in clinical trials

Emily Hyslop, BS CCRP, senior research service professional

Alyssa Avilez, BS, research services professional

Have you ever wondered how those medications on commercials get tested, how anyone determines if they are safe, or if they even work? Join our FSHD research coordinators, Emily Hyslop and Alyssa Avilez from the University of Colorado as they explain what it is like to be that person trying new treatments for FSHD. They will go over what kinds of tests you might expect to have done, time commitments, questions you should ask before agreeing to be in a trial, and if you are interested, how to find and get involved in a clinical trial near you!

10:00 a.m.

Q&A with biopharmaceutical companies

Moderated by Lucienne Ronco

Companies that have FSHD therapies in various stages of development will answer questions posed by the audience (submitted in advance).

Panelists

Dillon Chen, MD PhD, is the medical monitor for neurology and neuromuscular-related programs at Arrowhead Pharmaceuticals, and leads the Phase 1/2a clinical trial on ARO-DUX4 in patients with FSHD1. Before joining Arrowhead, Dillon was an assistant professor and attending physician in the Department of Neurosciences, and Division of Child Neurology at the University of California, San Diego. As a board-certified child neurologist, he cared for children with neuromuscular issues, including pediatric FSHD.

Angela Dodman, PhD, is a lead clinical scientist at Roche (Basel, Switzerland). Angela works in the neuromuscular franchise, where she focuses on FSHD and the MANOEUVRE study. She is a knowledgeable drug development professional with 14+ years' experience in early- and late-phase clinical development in pharmaceutical and biotech companies. Angela is proud to have contributed to the clinical development of 3 approved medicines (so far!), including most recently, Roche's Evrysdi® for spinal muscular atrophy (SMA). She earned a PhD in neurobiology and behavior from Columbia University.

Katherine Beaverson, MS, is Vice President of Global Patient Advocacy at Dyne Therapeutics, guiding the strategic planning and implementation of collaborations with patient advocacy groups and patient communities to integrate patient experience and expertise into the medicines development and delivery continuum. She held similar roles in industry with Pfizer, Inc., Boehringer Ingelheim and Amicus Therapeutics. A professionally trained genetic counselor, she spent 10 years at both New York Hospital-Weill Cornell Medical Center and Memorial Sloan-Kettering Cancer Center.

Amy Halseth, PhD, is the FSHD development lead at Avidity Biosciences, based in San Diego, California. She is relatively new to the FSHD world but has worked on drug development for more than 20 years. Her favorite part of her job is knowing that what she is working on has a real opportunity to make an impact to people living with FSHD and their families. In her spare time, Amy enjoys spending time with her dog Bondi, running, gardening, and reading. Amy holds a PhD from Vanderbilt University.

Christopher Kurtz, MD, chief medical officer at Kate Therapeutics, is a seasoned drug developer, and joined Kate Therapeutics from Amgen, where he led a team of physician scientists in the development and approval of medicines to treat cardiac and metabolic diseases as vice president of global development.

Wes Miller, MD, is chief medical officer at Epic Bio (EpiCRISPR Biotechnologies). He has a clinical background in pediatric hematology and oncology and blood stem cell transplantation. Dr. Miller has drug development experience in gene-modified hematopoietic stem cell therapies and in-vivo gene editing and gene addition therapies for various rare diseases, including muscle disorders.

Olga Mitelman, MD, is senior vice president, head of medical affairs, at Fulcrum Therapeutics. She has 20 years of experience in medical affairs at such companies as Johnson & Johnson, Merck, Biogen, and

Sarepta, leading both global and US functions. Olga has worked in the therapeutic areas of neurology, psychiatry, and hemophilia with exposure to rare disease and orphan indications. She received her MD from the University of Pennsylvania.

Anthony Saleh, PhD, is a biotech entrepreneur who founded and currently serves as chief executive officer of miRecule, Inc., a biotechnology company focused on advancing next-generation RNA therapeutics. Dr. Saleh led an effort that resulted in miRecule entering into a strategic collaboration and exclusive license agreement with Sanofi to develop and commercialize a best-in-class antibody-RNA conjugate (ARC) for the treatment of FSHD. Dr. Saleh holds a PhD in biochemistry and molecular biology from Johns Hopkins University.

10:30–10:45 a.m.
Lower level 2 foyer

Coffee break

10:45 a.m.–12:15 p.m.

How the FSHD Society is serving the community

10:45 a.m.

An overview

Amanda Hill, director of clinical research, FSHD Society

11:00 a.m.

BetterLife FSHD

Amanda Hill, director of clinical research, FSHD Society

We are thrilled to announce the premiere of BetterLife FSHD, a pioneering platform dedicated to helping you take control of your well-being and advance FSHD research. With BetterLife FSHD, you can track your symptoms, access personalized resources tailored to your needs, and be matched to clinical trials and other research studies. In this session, Amanda will provide a live demonstration of BetterLife FSHD and share how your experiences and health data can drive us toward treatments and a cure faster than ever before. Are you ready to take control of your FSHD story?

11:20 a.m.

The FSHD Navigator program

Anna Gilmore, director of patient engagement, FSHD Society

The FSHD Navigator is a service dedicated to helping families with FSHD find what they need to live their best lives. Through this program, you can get connected to the information, resources, or contacts you're looking for. This is going to be a very conversational, relationship-focused program. In this session, you will learn how to access the FSHD Navigator.

11:35 a.m.

Hill Day and advocacy

Anna Gilmore, director of patient engagement, FSHD Society

The FSHD Society is entering a new era in its advocacy work, and the field is evolving rapidly. We will report on our inaugural Day on Capitol Hill, which took place in May. A dozen FSHD families advocated for their interests to their elected officials, and made great strides in raising awareness about FSHD and how the disease impacts daily life. This August, we will be speaking to the FDA on the topic of upper-body mobility. These events, combined with other initiatives, will ensure that the voices of the FSHD community are heard in areas of funding, research, regulatory affairs, and insurance.

11:45 a.m.

Celebrating our volunteer leaders

Beth Johnston, chief community engagement officer

Nearly 7 years ago, we began to reimagine and re-engineer the FSHD Society toward one goal – “Therapies to our Families by 2025.” We knew we needed an army of activists to participate in that goal, so in 2018 we launched our Chapter Program and asked volunteers to step forward to help us build this community. Since then, leaders from across the US, Canada, and beyond have done just that – and have beautifully prepared us for the last mile. Today, we celebrate these volunteer leaders, without whom we would not be able to accomplish our mission.

12:00 p.m.

Q&A with our panelists

June Kinoshita, senior director of research and education

12:15 p.m.

Let’s imagine

Jack Gerblick, Atlanta Chapter director and Board of Directors member, FSHD Society

Jack was diagnosed with FSHD in 1990 with a mild to moderate severity level. One of his 2 daughters has recently tested positive for the disease. He retired in 2020 after successfully selling the last of 3 businesses he founded over the past 17 years. Jack is on the Board of the FSHD Society, chair of the Community Development and Engagement Committee, and the director of the FSHD Society’s Atlanta Chapter. He and his wife Renee live in Dunwoody, Georgia, and enjoy spending time with their 4 children and 9 grandchildren. “I decided to get involved with the FSHD Society to continue building, broadening, and strengthening our FSHD community,” he says. “Working toward the mission of finding a cure that will directly, tangibly, and positively impact the lives of FSHD patients and their families. Delivering the message that no one is alone, every person counts, and there is hope.”

12:30 p.m.

Adjourn. See you in 2026!!

