

# 2018 FSHD Connect

The FSH Society's International Network Meeting  
for FSHD Families, Clinicians, and Researchers



June 9–10, 2018 | Flamingo Hotel | Las Vegas, Nevada





## Chapter Development Program

There is great power in community. When we come together, each bringing our own unique strengths and skills, we inspire each other to go further and reach higher than we can when we go it alone.

That's what the FSH Society Chapter Program is all about!

Driven by volunteers, supported by staff, the Chapter Program is our greatest opportunity to fund more research, connect more patients, and advance more progress. ***Are you in?***

### Goals:

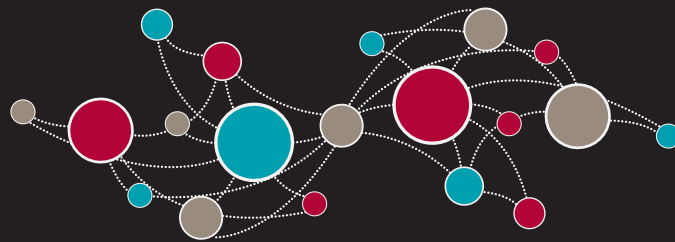
- Educating and empowering patients and families
- Building communities and connectivity
- Fundraising

### Requirements:

- 2+ meetings/events per year. At least one should have an education/empowerment component.
- 1+ fundraiser per year (Walk & Roll to Cure FSHD or other of your choosing)

Together we can do so much

Learn more at [FSHSociety.org/ChapterLaunch](https://FSHSociety.org/ChapterLaunch)



## FSHD CONNECT CONFERENCE DAY 1

9:00 a.m.–1:00 p.m.

### PRE-MEETING ACTIVITIES

**REGISTRATION** (El Dorado Ballroom Foyer)

- 9:30 a.m.–11:00 a.m. **Mindfulness Practice**, Jennifer Egert, PhD (Carson City Ballroom)
- Information table for National FSHD Registry, tissue donation registry, Clinical Trial Research Network, chapter program

1:00–1:15 p.m.

**2018 FSHD CONNECT CONFERENCE DAY 1** (El Dorado Ballroom)

### Welcome

- Jim Chin, *Board Chair, FSH Society*
- Mark Stone, *President & CEO, FSH Society*

1:15–2:30 p.m.

### UNDERSTANDING FSHD

- 1:15–1:40 p.m. **FSHD 101: DUX4—Prince Charming Turned Into Joker**, Alexandra Belayew, PhD
- 1:40–2:05 p.m. **Beyond Evidence-Based FSHD Care Guidelines: What We Know From Clinical Experience**, Rabi Tawil, MD
- 2:05–2:30 p.m. **Q&A**

2:30–2:45 p.m.

### BREAK

2:45–5:00 p.m.

### THE ROAD TO TREATMENTS

- 2:45–3:15 p.m. **Clinical Trial Readiness**, Jeffrey Statland, MD, and Research Volunteer Panel
- 3:15–3:30 p.m. **FSHD Therapies 101**, Scott Harper, PhD
- 3:30–4:30 p.m. **The Pipeline for FSHD Therapies**, Scott Harper, PhD (moderator), and Panel
  1. **Targeting Muscle Growth and Regeneration**  
Peter Zammit, PhD (myogenesis)  
Ken Attie, MD, *Acceleron* (myostatin inhibitor)  
Ryan Wuebbles, PhD, *Strykagen* (small molecule enhancer of muscle growth)  
Chad Heatwole, MD (HGH + testosterone trial)
  2. **Targeting DUX4 Expression**  
Lucienne Ronco, PhD, *Fulcrum Therapeutics* (small molecule)  
Monica Hayhurst Bennett, PhD, *Genea Biocells* (small molecule)  
Fran Sverdrup, PhD (small molecule)  
Charis Himeda (CRISPR-based repression of DUX4)
  3. **Targeting DUX4 mRNA**  
Yi-Wen Chen, DVM, PhD (antisense)  
Scott Harper, PhD (gene therapy)
- 4:30–5:00 p.m. **Q&A**

6:00–10:00 p.m.

### CureFSHD RECEPTION AND BANQUET: “RESEARCHER ROULETTE”

Enjoy an evening dining with the rock stars of FSHD research.

## FSHD CONNECT CONFERENCE DAY 2

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8:45–9:00 a.m.

**OPENING REMARKS** (El Dorado Ballroom)

- **Charting a Bold Course**, Mark Stone, *President & CEO, FSH Society*
  - **Sharing Our Stories, Finding Our Voices**, Tim Hollenback, *Host, FSH Society Radio*
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9:00–10:15 a.m.

**CONCURRENT BREAKOUT SESSIONS 1A**

- **Moving Well: Physical Therapy and Bodywork** (El Dorado), Katy Eichinger, PT, PhD, NCS,, *University of Rochester Fields Center for FSHD Research*, Ruth Hereford, *Muscular Dystrophy Support Centre, UK*, and Beatriz Nascimento, *School for Self-Healing, San Francisco*
  - **Caring for Your Emotional and Psychological Health** (Carson City I), Jennifer Egert, PhD, and Kent Drescher, PhD. Self-help, psychotherapy, coaching, holistic treatments—each offers skills and treatments for living with greater emotional and psychological balance and ease. We will discuss the options and explore how you can access providers.
  - **Ask the Scientist** (Carson City II), Michael Kyba, PhD, *University of Minnesota*, will answer your questions about FSHD research—what causes FSHD, what do scientists think is going on with muscle weakness, what types of treatments appear most promising, what is the role of stem cells, gene therapy, gene editing technology, etc.
  - **Eating Safely and Talking About It** (Virginia City I), Kiera Berggren, MA/CCC-SLP, MS, *University of Utah*. An overview of swallowing and communication concerns with tips for managing issues.
  - **Living Well With FSHD: How the Science of Personal Well-Being Can Improve Your Life** (Virginia City II), Ora Prilleltensky, EdD. Note: this session will be held at 9:00 a.m. only. This session will focus on strategies for enhancing well-being in the face of FSHD: how to set and achieve important goals, cultivate positive emotions and manage negative ones, and how to strengthen close relationships and social bonds.
  - **Loving Well: A Session for Friends and Family Members of People Affected With FSHD** (Virginia City III), Isaac Prilleltensky, PhD, *University of Miami*. Addressing the experiences and issues faced by caregivers. The challenge of maintaining a strong, loving relationship while shifting roles and avoiding burnout.
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10:15–10:30 a.m.

**BREAK**

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10:30–11:45 a.m.

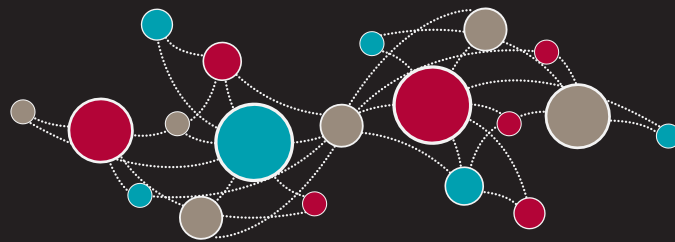
**CONCURRENT BREAKOUT SESSIONS 1B** (repeat of 1A minus Living Well)

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11:45 a.m.–1:00 p.m.

**LUNCH** (El Dorado Ballroom), FSH Society National Chapter Program Launch

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**1:00–2:00 p.m.**

**Let's Activate!** (El Dorado Ballroom)

A workshop to network, answer your questions, and discuss practical steps for moving forward with the FSH Society's National Chapter Program

**2:00–3:15 p.m.**

**CONCURRENT BREAKOUT SESSIONS 2A**

- **Modern Mobility** (El Dorado), Erik Montville, *Numotion Mobility*. An overview of mobility and assistive equipment to address the needs of people with FSHD. Learn about the Tek Robotic Mobilization Device.
- **Transitions** (Carson City I), Carden Wyckoff and Kent Drescher, PhD. Coping with changing physical ability, figuring out what adaptations and devices you will need, and the psychological process of letting go and accepting your new reality.
- **Respiratory Health: What Everyone With FSHD Should Know** (Virginia City I), Lou Saporito, RRT, *New Jersey Center for Noninvasive Mechanical Ventilation and Pulmonary Rehabilitation*.
- **The Future of Assistive Technologies** (Virginia City II), Nate Phipps, *Harvard Medical School Wyss Institute*, Don Burke, and others. During this session, Nate will explain the Harvard Biodesign Lab's wearable robots. We'll then discuss these soft, textile devices as a group and chat about applications specific to FSHD.
- **Speaking of Family** (Virginia City III), Ora Prilleltensky, EdD, and Jennifer Egert, PhD. A conversation about families and the varied ways in which they cope (or don't) with having members with FSHD. A forum to share stories and advice, and support one another.

**3:15–3:30 p.m.**

**BREAK**

**3:30–4:45 p.m.**

**CONCURRENT BREAKOUT SESSIONS 2B** (repeat of 2A)

**4:45–5:00 p.m.**

**FINALE**

**Closing Remarks**

**5:00 p.m.**

**ADJOURN**

**Wifi network:** FSH Society  
**Password:** curefshd

# 2018 FSHD CONNECT

## ABOUT OUR PRESENTERS

(in alphabetical order)

### **Beatriz Ambrósio do Nascimento, OTR, MA,**

is professor of occupational therapy at the Federal University of San Carlos, Brazil, and senior instructor of the School for Self-Healing, San Francisco, California. An occupational therapist with a master's degree in social sciences, she enrolled in the School for Self-Healing in 1989 to work on her own FSH muscular dystrophy. Results were so amazing, she decided to dedicate her life to teaching the self-healing method for the past 30 years. The method consists of gentle, supportive massage, non-stressful movement techniques, breathing, and visualizations that strengthen fragile, dystrophic muscles. Identifying exercises relevant to the specific condition of each area is very important, as exercises for healthy muscles will not work for sick ones, and may even be detrimental.



**Kenneth Attie, MD,** is vice president of medical research at Acceleron Pharma. He has devoted 30 years to developing investigational drugs for growth disorders and neuromuscular diseases. As vice president at Acceleron since 2009, he has conducted studies in anemia, cancer, and neuromuscular disorders, including Duchenne and FSH muscular dystrophies, and CMT disease. His expertise is in growth hormones and the TGF-beta superfamily. Dr. Attie is board certified in pediatric endocrinology, with more than 50 publications in peer-reviewed journals. He received his education and medical training at the University of Michigan, Ann Arbor; NYU Medical Center; and the University of California, San Francisco.



**Alexandra Belayew, PhD,** is professor emerita, Faculty of Medicine and Pharmacy, University of Mons, Belgium. At a time when the causes of FSHD were still a mystery, she and her team identified the DUX4 gene in repeated DNA elements considered "junk DNA" at the genetic locus of the disease. Together with Frédérique Coppée, her team showed the gene was activated in patient muscle cells, and proposed that the resulting DUX4 protein could disturb gene



regulation in FSHD muscles. The first DUX4 target gene was identified in collaboration with Yi Wen Chen, and DUX4 toxicity was shown in collaboration with Alberto Rosa. Belayew's team further developed antisense agents to prevent DUX4 protein production.

### **Kiera Berggren, MA/CCC-SLP, MS,**

is a speech-language pathologist in the Department of Neurology at the University of Utah. She has worked in rehabilitation for individuals with acquired neurological injuries and in the provision of care for individuals with neurodegenerative diseases. She currently provides swallow, communication, and cognitive support to patients and families in multidisciplinary clinics for MDA, ALS, and Huntington's disease, and conducts research in neuromuscular diseases.

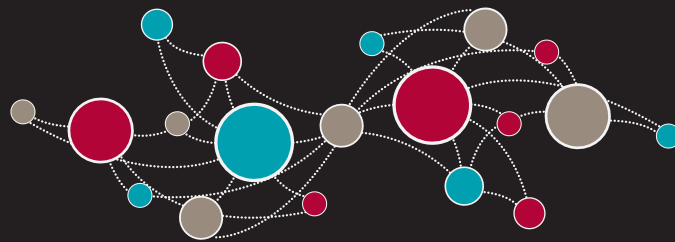


**Yi-Wen Chen, DVM, PhD,** is associate professor of genomics and precision medicine at George Washington University, and is on the faculty at the Center for Genetic Medicine Research, Children's National Health System. She has been studying molecular mechanisms of muscle diseases and how muscles respond to exercises and conditions that cause muscle wasting. Her research in FSHD has led to better understanding of the cause of the disease and how the disease progresses. Recently, Dr. Chen and Jean Mah completed a multicenter collaborative study on the clinical characterization of early-onset FSHD. Currently, Dr. Chen is searching for circulating biomarkers using blood samples collected in this study, which may be important to future clinical trials. She is also investigating several antisense oligonucleotide drugs that target DUX4 for potential treatments of FSHD.



**Kent D. Drescher, PhD,** is a licensed clinical psychologist. He recently retired from many years of service at the National Center for PTSD, which is part of the VA Health Care System. In his role there he conducted research, provided clinical care for veterans,





and educated clinicians about post-traumatic stress. Dr. Drescher has FSHD, and personally understands the physical and emotional impact the disease has had on him and his family. His clinical specialty is acceptance and commitment therapy, which is a transdiagnostic behavioral treatment designed to increase human flourishing in the face of diverse life challenges.

**Jennifer Egert, PhD**, is a clinical psychologist in private practice in New York City offering mindfulness-based psychotherapy, workshops in mindfulness-based cognitive therapy (MBCT), and mindful approaches to stress management. She began her own personal study and mindfulness practice in 2002, exploring the work of Jon Kabat-Zinn, Thich Nhat Hanh, and others. In addition to work with mindfulness interventions, Dr. Egert trained and practiced as a health psychologist at the VA Medical Center in New York City and Montefiore Hospital in the Bronx, working with individuals with medical issues, disabilities, and at end of life to find ways to live more easily in the face of illness. Bringing her experience to the FSHD community is meaningful for her, given her own diagnosis and that of several family members.



**Katy Eichinger, PT, PhD, NCS**, is a physical therapist in the Neuromuscular Disease Center at the University of Rochester Medical Center. She received a PhD in health practice research from the University of Rochester School of Nursing in 2016, and a doctorate of physical therapy from Upstate Medical University in 2007. Eichinger is certified by the American Board of Physical Therapy Specialties in neurologic physical therapy. She is involved in the clinical care of adults and children with neuromuscular conditions, and as part of the neuromuscular research team she conducts natural history studies and clinical trials involving patients with FSHD, myotonic dystrophy, Charcot-Marie-Tooth disease, Duchenne muscular dystrophy, and myasthenia gravis. Her interests are in outcome measures, health and wellness, and balance.



**Scott Q. Harper, PhD**, is principal investigator at the Center for Gene Therapy at the Research Institute at Nationwide Children's Hospital, and associate professor of pediatrics at the Ohio State University College of Medicine, Columbus. He is a molecular biologist who has spent his career working to develop gene therapy approaches for muscular dystrophies. As a PhD student at the University of Michigan, he and his colleagues created "micro-dystrophin" gene therapy to treat Duchenne muscular dystrophy (DMD) in mice. Micro-dystrophin gene therapy is now being independently tested in boys with DMD. The lessons learned in DMD can now be applied to develop gene therapies for FSHD, and this has been a main focus of the Harper lab during the past decade. Dr. Harper was selected as the Outstanding New Investigator by the American Society of Gene and Cell Therapy in 2014.



**Monica Hayhurst Bennett, PhD**, is director of preclinical development at Genea Biocells Inc. She has more than 10 years of translational research experience in the areas of stem cell biology, neuroscience, and muscle biology. Her biotechnology experience includes developing protein, small-molecule, and cellular therapeutics in degenerative diseases of skeletal muscle at Fate Therapeutics and Genea Biocells, and leading research activities in the areas of neuroscience, and metabolic and cardiovascular biology at Orexigen Therapeutics. Dr. Hayhurst Bennett received her PhD from Stanford University with a focus on developmental neurobiology, and completed her postdoctoral training at the Harvard Stem Cell Institute, where she investigated spinal muscular atrophy, a neurodegenerative condition.



**Chad R. Heatwole, MD, MS-CI**, is associate professor of the Neuromuscular Center and associate director of the Center for Health + Technology (CHeT), both at the University of Rochester. He has more than 17 years of clinical experience treating patients with



# ABOUT OUR PRESENTERS

neuromuscular disease. Dr. Heatwole leads an international network for the creation of disease-specific outcome measures and has contributed to the development of more than 30 disease-specific outcome measures, including multiple instruments for FSHD. His other research interests include the development and testing of novel therapeutics for muscular dystrophy. He is the principal investigator of the ongoing STARFISH study, which tests testosterone and rHGH in FSHD, and the CROMFiSH study, an NIH-funded longitudinal study of FSHD patients designed to optimize FSHD clinical trial infrastructure and therapeutic development.

**Ruth Hereford** is chair of trustees at the Muscular Dystrophy Support Centre in the UK. In her role at the small but growing charity, Hereford is spearheading work to develop a multidisciplinary approach and model for providing specialist physical therapies and support for people with muscular dystrophy. A FSHer whose symptoms first appeared at age 11 but were not diagnosed until age 18, Hereford looks forward to sharing experiences and insights into ways therapies can help people with FSHD/MD manage and mitigate muscle deterioration and pain while maintaining mobility. Hereford's accounting work allows her to telecommute and divide her time among the US, UK, and her travels with her British husband and fellow trustee.



**Charis L. Himeda, PhD**, is research assistant professor at the University of Nevada Reno School of Medicine. She has worked in the fields of myogenesis and muscle disease for many years, most recently working with Peter Jones to address fundamental questions in FSHD and explore potential avenues to therapy. Following her 2016 study demonstrating that pathogenic gene expression in FSHD can be repressed using CRISPR technology, she was interviewed by *The Washington Post*, *The Huffington Post*, *The Boston Business Journal*, and Tim Hollenback of FSH Society Talk Radio. She received the inaugural FSH Society Young Investigator of the Year award in 2016. Dr. Himeda is currently studying the



epigenetic networks regulating DUX4 expression in FSHD to identify potent and specific therapeutic targets.

**Michael Kyba, PhD**, is the Carrie Ramey/Children's Cancer Research Fund Endowed Professor in Pediatric Cancer Research at the Lillehei Heart Institute, University of Minnesota. He studies muscle disease, degeneration, and regeneration with skeletal muscle stem cells. He completed his PhD at the University of British Columbia, Vancouver, in 1998 and postdoctoral work at MIT in 2003. He started his laboratory in 2003 at the University of Texas Southwestern Medical Center in Dallas, where he demonstrated that the transcription factor DUX4 causes skeletal muscle pathology, thus providing a mechanism for FSHD. In 2008 he moved to the University of Minnesota, where his laboratory investigates how the DUX4 protein functions, has developed an animal model for FSHD, and pursues an interest in developing DUX4 inhibitors as drugs for FSHD.



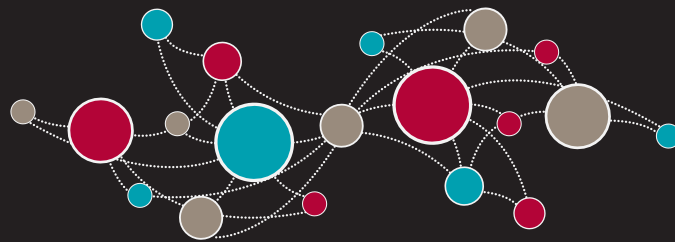
**Erik Montville** is an assistive technology professional at Numotion Mobility. He has 20 years' experience in the complex rehab technology field, starting at ground level building custom wheelchairs and fabricating parts. Today, as a leading ATP in Las Vegas, he specializes in pediatrics and the early-intervention community, but also has had great success and experience with adults with a wide range of diagnoses. He is deeply involved with muscular dystrophy families in Las Vegas and volunteers his time regularly for those who are affected by this disease. Erik loves to share his knowledge and experience to help build a better community.



**Nathan Phipps** is an industrial designer at the Harvard Biodesign Lab's Wyss Institute. He studied industrial design at the Rhode Island School of Design, with a focus on human-centered methods and prototyping. Working in the outdoor recreation industry, he developed a







knowledge of textiles, inflatable devices, and physiology. His work in the Biodesign Lab prioritizes understanding of user needs to create wearable robots that assist with human movement.

**Isaac Prilleltensky, PhD**, is professor of educational and psychological studies, vice provost for institutional culture, the Erwin and Barbara Mautner Chair in Community Well-Being, and former dean of the School of Education and Human Development at the University of Miami. He has published 10 books and more than 130 scholarly papers, book chapters, and book reviews dealing with individual, organizational, and community well-being. Dr. Prilleltensky is the recipient of multiple awards from the American Psychological Association. He is a vegan, fitness aficionado, and humor writer. In 2015, he received an award from the National Newspaper Association for his humor writing. His newest book is *The Laughing Guide to Well-Being: Using Humor and Science to Become Happier and Healthier*.



**Ora Prilleltensky, EdD**, is a former clinical assistant professor at the University of Miami. She has a doctorate in counseling psychology from the University of Toronto, and is interested in disability and well-being. Prilleltensky has FSHD and uses a power wheelchair. She is the author of four books, including *Motherhood and Disability: Children and Choices*; *Promoting Well-Being: Linking Personal, Organizational, and Community Change*; *The Laughing Guide to Change*; and *The Laughing Guide to a Better Life*.



**Lucienne V. Ronco, PhD**, is vice president of translational medicine at Fulcrum Therapeutics. Dr. Ronco joined Fulcrum at the company's launch in 2016. She brings more than 20 years of industry experience in translational medicine, drug discovery, biotechnology, and pharmaceuticals. Prior to joining Fulcrum, Dr. Ronco was a director within the Center for the Development of Therapeutics at Broad



Institute of MIT and Harvard. Previously, she has held positions as president of research at Catabasis Pharmaceuticals; at AstraZeneca Boston she was the global director of translational medicine, as well as the local director for the departments of in vivo pharmacology, informatics, and disease area research. She was also the team scientific lead in breast disease at AstraZeneca and senior research investigator at the Pfizer Discovery Technology Center. Dr. Ronco completed postdoctoral research at Harvard Medical School and holds a PhD in biological chemistry from the University of California, Los Angeles.

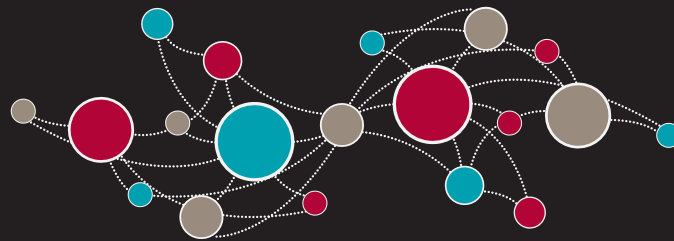
**Lou Saporito, BA, RRT**, is a New Jersey-licensed respiratory care practitioner and adjunct instructor at Rutgers Medical School and University Hospital, Newark, New Jersey. Saporito has 30 years of experience in the evaluation and education of patients with respiratory difficulty due to neuromuscular disease at the Muscular Dystrophy Association Clinic at Rutgers Medical School. He is a respiratory home care consultant to Millennium Respiratory Services and is cofounder of BreatheNVS.



**Jeffrey M. Statland, MD**, has just been promoted to associate professor of neurology with tenure at the University of Kansas Medical Center. His research has centered on describing the natural history of and response to therapy of neuromuscular diseases. He currently serves as principal investigator for research studies in FSHD, DMD, SMA, and myotonic dystrophy. His specific research interest during the past eight years has been in preparing for clinical trials in FSHD. Dr. Statland has worked with collaborators to develop new disease-relevant outcome measures to assess biomarkers, physiological changes in muscle structure or function, and patient-reported FSHD impact. He has recently assembled an FSHD Clinical Trial Research Network comprising seven academic centers and a research team with decades of experience in conducting clinical trials, with the overarching goal of hastening therapeutic development for FSHD.



# ABOUT OUR PRESENTERS



**Fran Sverdrup, PhD**, is a research fellow in the Edward A. Doisy Department of Biochemistry and Molecular Biology at Saint Louis University School of Medicine. He spent 11 years in the pharmaceutical industry, where he focused on identifying and validating drug targets for inflammatory diseases. Additionally, he utilized animal models to evaluate the potential therapeutic efficacy of drug candidates. He has applied this same skill set to drug discovery for FSHD at Saint Louis University. Dr. Sverdrup is currently working with Ultragenyx Pharmaceuticals to identify and evaluate inhibitors of DUX4 expression for therapeutic development in FSHD.



**Rabi Tawil, MD**, is professor of neurology at the University of Rochester Medical Center. He is a clinician-researcher with long-standing involvement in FSHD clinical care and research. He has organized and chaired several international FSHD meetings, including workshops to establish care guidelines in FSHD as well as clinical trial readiness. Dr. Tawil directs the Richard Fields Center for FSHD Research, an ongoing productive collaboration since 2007 with Silvère van der Maarel in Leiden, the Netherlands, and Stephen Tapscott at the Fred Hutchinson Cancer Research Institute in Seattle. He is currently collaborating with Jeffrey Statland at the University of Kansas Medical Center on a multicenter study to validate new clinical outcomes for future clinical trials.



**Ryan Wuebbles, PhD**, is the CSO of Strykagen Corporation and research assistant professor at the University of Nevada, Reno (UNR). His career interest has been in the field of muscular dystrophy since the age of 20, when he was diagnosed with FSHD. At the University of Illinois, Urbana-Champaign, he received a PhD in cell biology with Peter Jones. He joined the Department of Pharmacology at the UNR to work on muscular dystrophy therapeutics with Dean



Burkin. A primary project has been to optimize and run a cell-based assay for small-molecule enhancers of alpha-7 Integrin expression, and help transition them into mouse model studies. In order to commercialize the technologies they have developed, Burkin and Wuebbles cofounded Strykagen in 2013.

**Carden Wyckoff** is on the FSH Society Board of Directors, Development Committee, and Patient Connect Steering Committee. She is a long-time advocate/community member for the FSH Society and was elected last year as the youngest director. Wyckoff was diagnosed with infantile-onset FSHD at the age of nine and has a spontaneous mutation. This unique genetic occurrence has empowered her to take a stand against the disease. She transitioned in early adolescence from running to walking, to limping, to using AFOs, to a scooter, then to a “spaceship” wheelchair. Her knowledge, coping strategies, and best practices acquired along her journey can help benefit the newly diagnosed or those who are anxious about what the next stage holds.



**Peter S. Zammit, PhD**, is professor of cell biology at the Randall Centre for Cell and Molecular Biophysics, King’s College London. He completed a PhD in muscle regeneration at King’s College London before working on gene regulation and heart development with Margaret Buckingham at the Pasteur Institute (Paris). Next, he worked with Terence Partridge investigating muscle stem cells at Imperial College London before starting his own group at King’s College London in 2005. Dr. Zammit’s core research involves understanding how muscle stem cells are regulated in healthy, aged, and diseased skeletal muscle. Current research includes investigating the contribution of stem cell dysfunction and mechanisms of disease progression in Emery-Dreifuss muscular dystrophy and FSHD, together with developing potential therapies.

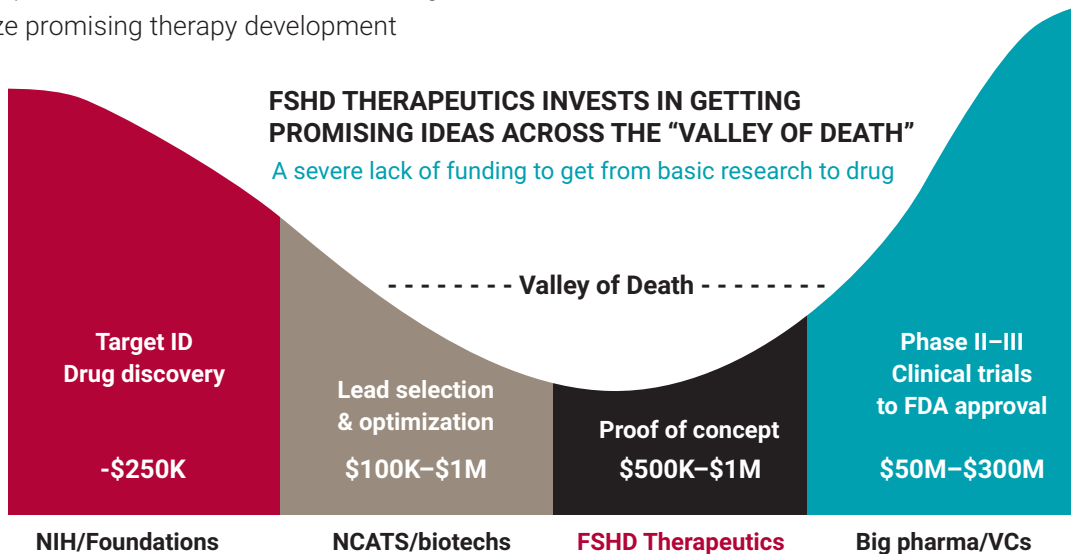


# FSHD THERAPEUTICS

A therapeutics accelerator initiative of the FSH Society

## OUR CHALLENGES

- Reduce the barriers to drug development in FSHD
- Better understand disease progression
- Make it easier to enroll volunteers in clinical trials
- Use the power of data to unlock breakthroughs
- Catalyze promising therapy development



## OUR PURPOSE

Accelerate the development of treatments and discover a cure for FSHD.

## OUR GOAL

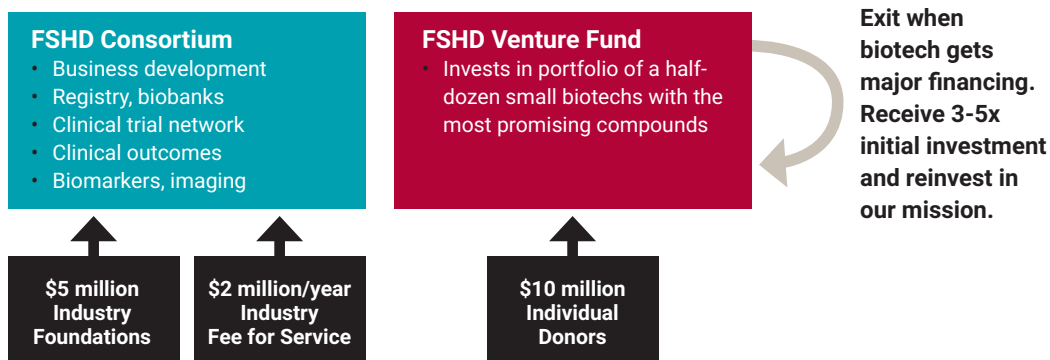
Deliver disease-modifying therapies to our families by the **YEAR 2025.**

## OUR PLAN

Raise \$15 million for capital investment in:

- Pre-clinical, pre-competitive infrastructure for clinical trials via an industry consortium, including a clinical trial network, patient registries, natural history studies, clinical outcome measures, biomarkers, imaging markers, FDA guidance.
- Venture philanthropy fund to invest in proof-of-concept and early clinical development of the most promising treatments.

## FSHD THERAPEUTICS STRUCTURE



**Interested in advancing the conversation? Please contact:**

Mark Stone, President & CEO • FSH Society • (781) 301-7323 • [mark.stone@fshsociety.org](mailto:mark.stone@fshsociety.org)

Learn more at [FSHSociety.org/FSHDTX](http://FSHSociety.org/FSHDTX)

The FSH Society would like to thank our Gold Sponsors:



**JAMES CHIN, SR.,  
SCHOLARSHIP FUND**

The FSH Society would like to thank our Silver Sponsors:



The FSH Society would like to thank for in-kind support:



Special thanks to our steering committee:

Carden Wyckoff	Nancy Payton
Carole Baas	James Rice
Frank Kolakowski, PhD	Neil Solomon, MD
Stuart Lai	

