FSHD 360 Conference
The Latest in Health & Research

Virginia Commonwealth University
October 30, 2021

VCU Larrick Center
900 Turpin Street, Richmond, VA 23284

9:30 – 10:00 am	**Check in**
10:00 – 10:05 am	**Welcome**. Mark Stone, CEO, FSHD Society
10:05 – 10:45 am	**FSHD Disease and Research Overview** Jeffrey Statland, MD, University of Kansas Medical Center
10:45 – 11:45 am	**Multidisciplinary panel** • Physical therapy – Mandy Butler, PT DPT • Speech-language and swallow issues – Kiera Berggren, CCC-SLP • Mental Health – Kent Drescher, PhD (virtual) • Q&A
11:45 am – 12:30 pm	**Lunch break and networking**
12:30 – 1:30 pm	**FSHD Clinical Care and Genetic Testing** • FSHD Clinical Care – Nicholas Johnson, MD • Genetic Testing – Matthew Herzog • Q&A
1:30 – 3:00 pm	**Drug Development Roundtable** • The drug development ecosystem – Jamshid Arjomand, PhD • Drug Development Panel Presentations ◦ Elizabeth Ackerman PhD, Avidity Biosciences (virtual) ◦ Maria Naylor, Dyne Therapeutics ◦ Olga Mitelman MD, MBA, Fulcrum Therapeutics • Q&A
3:00 – 3:30 pm	**Activating the Virginia community –** Beth Johnston, Anna Gilmore **Overview of the Virginia chapter –** Gary Lauck • Q&A and discussion
3:30 pm	**Closing remarks** Mark Stone, CEO, FSHD Society

FSHD SOCIETY
LIGHTING THE WAY TO A CURE

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FSHD 360 Conference VCU
Speaker Bios

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. Prior to joining the FSHD Society, he was the chief executive officer of NephCure Kidney International. From 2004-2011, he was executive vice president and COO of the Polycystic Kidney Disease (PKD) Foundation. 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.

Jeff Statland, MD, is a professor of neurology at the University of Kansas Medical Center and a leading researcher in the field of facioscapulohumeral muscular dystrophy (FSHD). He is the co-principal investigator for ReSolve (Clinical Trial Readiness to Solve Barriers to Drug Development in FSHD), an ongoing observational study that aims to validate new clinical outcome assessments and evaluate physiological biomarkers to support the design and implementation of future clinical trials. In addition to co-leading the ReSolve natural history study, Dr. Statland, along with his collaborators at the University of Rochester Medical Center, is developing a disease-specific patient reported health inventory and molecular and neuroimaging biomarkers of disease activity for future FSHD clinical trials.

Mandy Butler, PT DPT, has worked as a full-time physical therapist through the VCU Health System for over 19 years. She has worked with pediatric patients in the educational, early intervention, outpatient, long term care and inpatient rehab settings. After working in the pediatric neuromuscular clinic for several years, she became the consulting physical therapist in the adult neuromuscular and ALS clinics at VCU in 2009. In 2016, Mandy took on the role as a clinical evaluator with neuromuscular research for both adult and pediatric patients. The neuromuscular research has expanded to over 15 studies and is continuing to grow.

Kiera Berggren, CCC-SLP, is a research speech-language pathologist (SLP) in the department of neurology at VCU. Berggren was previously part of the FSHD Clinical Trial Research Network (CTRN) site at the University of Utah and moved to VCU as part of Nicholas Johnson's team. Berggren trained as a chemist before embarking on a second career as an SLP in neurological rehabilitation. She discovered a passion for working with individuals with neurodegenerative diseases and currently provides swallow, communication, and cognitive support to patients and families in multidisciplinary clinics for ALS and the MDA. She is also be active in research in neuromuscular diseases including myotonic dystrophy, FSHD, ALS, and others where she has been looking at orofacial strength, swallow function, and changes in speech.

Kent Drescher, PhD, is a California licensed clinical psychologist who provided clinical services, education, and research at the National Center for PTSD for more than 27 years before retiring in 2017. His primary areas of expertise include the intersection of trauma and spirituality, and moral injury. He has been an early advocate for the use of evidence-based Acceptance and Commitment Therapy (ACT) for veterans struggling with moral challenges following military service. Dr. Drescher was diagnosed with FSHD at age 28 in 1983, about 10 years after his first symptoms appeared. He and his wife currently volunteer as chapter directors for the FSHD Society chapter for the San Francisco Bay area.

Nicholas E. Johnson, MD, MSCI, FAAN is associate professor of Neurology and Human and Molecular Genetics and vice chair of research in Neurology at Virginia Commonwealth University with a focus in inherited neuromuscular disorders. He received his medical degree at the University of Arizona and completed his neurology residency and combined fellowship in neuromuscular medicine and experimental therapeutics at the University of Rochester. His laboratory is focused on identifying the pathogenesis of limb girdle muscular dystrophy, myotonic dystrophy, and FSHD and identifying appropriate clinical endpoints for these conditions. Johnson conducts therapeutic trials in many other inherited nerve and muscle disorders.

Matthew Herzog, MS CGC, is a Senior Genomic Testing Consultant at PerkinElmer Genomics and a licensed certified genetic counselor based in Charlotte, NC. Prior to joining PerkinElmer Genomics he was a lead study coordinator and genetic counselor at the UCLA Health site of the NIH Undiagnosed Diseases Network.

Jamshid Arjomand, PhD, chief science officer, FSHD Society. A neuroscientist with more than 15 years of pharmaceutical and biotechnology experience in chronic pain, neurodegeneration, neuromuscular disorders and human stem cell disease modeling, Jamshid came to the FSHD Society from Genea Biocells, a San Diego-based biotechnology company where he served for five years as Vice President of Business Development. Genea's pipeline included FSHD for which their lead asset, GBC0905, received orphan drug designation by the FDA in May 2018. From 2005 to 2013, he served as Director of Basic Research at CHDI Foundation. There he designed and managed a complex portfolio of academic, clinical and industry driven projects, primarily related to biomarker discovery, stem cell development, and target discovery and validation efforts for Huntington disease.

Elizabeth Ackerman, PhD. Elizabeth joined Avidity in January 2020 and serves as SVP Clinical Development, Neuromuscular Therapeutic Area Head. She brings extensive experience in research and development of RNA therapeutics with a focus on neurology and rare diseases. Most recently she served as Vice President of Clinical Development at Otonomy Inc. and prior to Otonomy served as the Chief Science Officer at the Myotonic Dystrophy Foundation. Elizabeth worked at Ionis Pharmaceuticals for over 8 years where she led the development of Tegsedi® (an approved treatment for TTR Amyloidosis with Polyneuropathy) and several other rare disease programs.

Maria L. Naylor, PhD, is an Executive Medical Director at Dyne Therapeutics (Waltham, MA), where she is leading the clinical development program for FSHD. She obtained her PhD in Genetics from the Division of Medical Sciences at Harvard Medical School. Prior to joining Dyne, she worked at Biogen, where she led the development and execution of Multiple Sclerosis (MS) studies to address key unmet medical needs, including COVID-19 outcomes and vaccination responses in people with MS. She was also a member of a working group to increase clinical trial participation in underrepresented populations and remains committed to the goal of health equity and improving patient outcomes.

Olga Mitelman, MD, has recently joined Fulcrum Therapeutics as SVP, Head of Medical Affairs. She has twenty years of experience in medical affairs at such companies as Johnson & Johnson, Merck, Biogen, and Sarepta, leading both global and US functions. She 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.

Beth Johnston, chief community development officer, FSHD Society. Beth joined the FSHD Society staff in August of 2016. Her long history with the Society began as a volunteer shortly after her husband was diagnosed with FSHD. She continues her commitment to raising awareness of the disease and building an "army of activists" that will help us achieve our mission of treatments and a cure. Prior to joining the Society, she was Founder & CEO of Social Bridges, a Denver-based social media marketing agency. She has also worked in information technology, project management, high-technology & telecommunications consulting, and real estate.

Anna Gilmore is regional director of community engagement at the FSHD Society. She comes to the organization from Northeastern University, where she worked in the City & Community Affairs division for the last 7 years. Her projects there included implementing co-curricular volunteering programs, and facilitating student leadership development and training with a focus on civic engagement and social justice.

Jodie Howell, co-director, Virginia chapter. Jodie has been a certificated clinical research nurse for over 10 years working in investigational drug studies at Virginia Commonwealth University. Prior to getting involved with research, she worked as a endoscopic and floor nurse with Sentara's Hospital for several years. She believes in empowering patients to be their best advocate.

Gary Lauck, co-director, Virginia chapter. Gary was diagnosed with FSHD1 in November 2018, and through serendipity, learned about and was accepted to the ReSOLVE study. He greatly appreciated the opportunity to meet and interact with the exceptional staff. "In a short amount of time, I overcame the initial shock and anxiety that accompanied my diagnosis and learned a lot about the disease," he says. "It feels great to be an active part of the solution, which is why I volunteered to help start the Virginia chapter."

June Kinoshita, director of research and patient engagement, FSHD Society. Previously, June co-founded and served as Executive Editor of the Alzheimer Research Forum, the pre-eminent Web community for researchers in neurodegenerative disorders. June has worked on initiatives for multiple sclerosis, schizophrenia, amyotrophic lateral sclerosis, Parkinson's disease, and other disorders. She co-founded N-of-One, Inc., a pioneering targeted oncology company. June began her career as a science journalist, working as a writer and editor for *Scientific American, Science*, *The New York Times Magazine,* and many other national publications.