FSHD 360 Conference
The Latest in Health & Research

University of Kansas
November 12, 2022

Merriam Community Center
6040 Slater Street, Merriam, KS

9:00 – 9:55 am	**Check in, social time**
9:55 – 10:00 am	**Why are we here? What is the CTRN?** Mark Stone, FSHD Society
10:00 – 10:20 am	**FSHD Today** Jeffrey Statland, MD, University of Kansas
10:20 – 10:50 am	**FSHD Research: Stem cells / gene therapy** Nizar Saad, PhD, Nationwide Children's Hospital
10:50 – 11:20 am	**FSHD Research: Insights from MRI** Seth Friedman, PhD, University of Washington
11:20 – 11:35 am	**Break**
11:35 am – 12:05 pm	**Care Team panel / Q&A** • Genetic counseling • Physical and occupational therapy • Nutrition & supplements • Accessing services you need • Mental health
12:05 – 1:05 pm	**Lunch and Ice Breaker**
1:05 – 1:40 pm	**Trial readiness for FSHD** • **New genetic diagnostics**, Erin Shehan, CGC, PerkinElmer Genomics • **TestFSHD,** Jamshid Arjomand, CSO, FSHD Society • **Our PACT,** Leigh Reynolds, Chief Program Strategist, FSHD Society
1:40 – 2:00 pm	**FSHD Research: The MOVE study** Michaela Walker, University of Kansas
2:00 – 2:30 pm	**Pain in FSHD** Renatta Knox, MD PhD, Washington University St. Louis
2:30 –2:45 pm	**Break**
2:45 – 3:45 pm	**Drug development in FSHD** • Clinical trial overview and a trial-ready community Jeffrey Statland, MD, and Jamshid Arjomand, PhD • Olga Mitelman, MD, Fulcrum Therapeutics • Haley Arellano, Avidity Biosciences
3:45 – 4:00 pm	**Igniting the community** Kiley Higgs, Kansas City chapter director, Leigh Reynolds
4:00 pm –	**Closing remarks and social time**

FSHD 360 Kansas City
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.

Jeffrey Statland, MD, is a professor of neurology at the University of Kansas Medical Center in Kansas City, Kansas. His specific research interest over the last 10 years has been preparing for clinical trials in FSHD. He has systematically analyzed performance of strength and functional outcomes in prior FSHD clinical trials and compared to performance in a natural history study. He has worked with collaborators to develop new disease-relevant outcome measures to assess patient-reported disease burden, functional impairment, and physiological changes in muscle. He is the co-Principal Investigator for the FSHD Clinical Trial Research Network (CTRN) which is comprised of 21+ institutions in the United States, Canada, UK and European Union.

Nizar Saad, PhD, is an assistant professor at The Ohio State University College of Medicine, and a principal investigator at the Center for Gene Therapy at Nationwide Children's Hospital. Dr. Saad is an experienced RNA biologist and gene therapy specialist. Dr. Saad's growing international reputation is demonstrated by his service on the New Investigator Committee, the Musculo-Skeletal Gene & Cell Therapy Committee and the Oligonucleotide and RNAi Therapeutics Committee of the American Society of Gene and Cell Therapy (ASGCT). Dr. Saad's research interests are in the discovery of circulating exosome-based biomarkers and the development of viral (Adeno-Associated Virus) and non-viral (Exosomes) based therapies towards neuromuscular diseases such as FSHD.

Seth Friedman, PhD, University of Washington, is a medical physicist at Seattle Children's Hospital. He is a member of the imaging task force of the FSHD Clinical Trial Research Network. His research is focused on the potential for magnetic resonance imaging (MRI) as a technique to follow various types of changes in the volume and composition of muscle in FSHD. The hope is that MRI can be validated as a measurement tool for disease progression in future clinical trials.

Erin Shehan, MS, CGC, Genomic Testing Consultant, PerkinElmer Genomics. Erin Shehan is a certified genetic counselor currently residing in Philadelphia, PA. She began her 16 year career path as a genetic counselor working as a clinical prenatal and pediatric GC in NYC. She has since had other roles in the industry space specializing in hereditary cancer and women's health, before joining PerkinElmer Genomics as a Genomic Testing Consultant. In her free time, she loves all things sports, yoga, and cheering on her beloved Villanova Wildcats.

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 working on Huntington disease.

Leigh Reynolds, chief program strategist, FSHD Society. Leigh has worked in non-profit leadership and capacity building for more than 25 years, most often in the patient advocacy space. Immediately prior to joining the FSHD Society, Leigh was the founder and managing director of a private consulting firm that focused on "helping the people that help the world." Before launching her own consulting firm, Leigh served on the leadership and development teams for the Polycystic Kidney Disease (PKD) Foundation and the National Multiple Sclerosis (MS) Society.

Michaela Walker is a research project manager at the University of Kansas Medical Center for the FSHD Clinical Trial Research Network (FSHD CTRN), and is managing MOVE and MOVE+, the largest natural history studies to date of FSHD and why they are so important to the drug development effort. She believes strongly in patient education, advocation, and working to better the care and therapies that are available to patients.

Renatta Knox, MD PhD, is a physician scientist at Washington University in St. Louis where she cares for children in the pediatric neuromuscular clinic and conducts mechanistic and translational research on FSHD. She completed her undergraduate studies at Harvard College where she fell in love with gene regulation, an interest that attracted her to FSHD. She went to UCSF for her MD PhD and did Child Neurology Training at New York Presbyterian-Weill Cornell. She recently completed a pediatric neuromuscular and gene therapy fellowship at The Ohio State University and Nationwide Children's Hospital where she worked with Scott Harper.

Olga Mitelman, MD, is Senior Vice President, Head of Medical Affairs, at Fulcrum Therapeutics. 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.

Haley Arellano joined Avidity Biosciences in May 2022 as a Director of Clinical Operations overseeing the Facioscapulohumeral Muscular Dystrophy (FSHD) program. She has worked in global drug development for nearly 18 years with trial management experience in a wide range of therapeutic areas. For the last seven years, Haley's career has been focused on rare disease research including experience with Myotonic Dystrophy, Rett Syndrome, ATTR amyloidosis, LGMD2i and other orphan conditions. Prior to joining Avidity Biosciences, Haley worked in operational strategy for BridgeBio.

Kiley Higgs, Kansas City chapter director. Kiley is also project manager of the FSHD Clinical Trial Research Network. She says, "I feel incredibly fortunate to work with not only one of the most renowned researchers in FSHD but also with the incredible patients I encounter every day. Everything we accomplish in research is due to the amazing patients and families willing to volunteer their valuable time in pursuit of a cure for FSHD. I feel privileged to be a small part of this incredible journey and feel now is a time of hope and optimism as we are closer than ever to a cure!