FSHD 360 Conference
The Latest in Health & Research

Columbus, Ohio
November 12, 2023

Presented by the FSHD Society, The Ohio State University, and Nationwide Children's Hospital

Agenda

9:00 – 10:00 a.m.	**Check in, social time**
10:00 – 10:05 a.m.	**Welcome! What is a 360? What is the CTRN?** Anna Gilmore, FSHD Society
10:05 – 10:25 a.m.	**FSHD 101 and overview of research in Columbus** Bakri Elsheikh, MD, OSU FSHD Center
10:25 – 10:35 a.m.	**FSHD genetics and genetic counseling** Jennifer Roggenbuck, LGC, OSU
10:35 – 11:20 a.m.	**Q&A with the OSU Care Panel** Jennifer Roggenbuck, LGC; Marco Tellez, Sr. CRC; Andrea Jaworek, PT; Mike Isfort, MD; Laura Domrose, RDN CSO LD; and Kinsey Ludt, LSW
11:20 – 11:30 a.m.	**Break**
11:30 – 11:45 a.m.	**FSHD history and the Center for Gene Therapy** Kevin Flanigan, MD, Nationwide Children's Hospital
11:45 – noon	**Pediatric FSHD studies and future trials** Linda Lowes, PT, PhD
noon – 1:00 p.m.	**Q&A followed by Lunch**
1:00 – 1:20 p.m.	**Clinical trial overview and trial readiness for FSHD** Jamshid Arjomand, PhD, FSHD Society
1:20 – 2:05 p.m.	**Drug development Q&A panel** Amy Halseth (Avidity) and Mihaela Levitchi Benea (Fulcrum)
2:05 – 2:15 p.m.	Break
2:15 – 3:00 p.m.	**Future directions for therapy and trials** Scott Harper, PhD, Nizar Saad, PhD
3:00 – 3:25 pm	**MOVE and MOVE + natural history studies.** **How to enroll in current studies and trials** Mike Isfort, MD, Andrea Jaworek, PT, & Marco Tellez
3:25 – 3:35 pm	**Activating the local community** Sue Aumiller, Ohio chapter leader, and Anna Gilmore, FSHD Society

We thank our 360 sponsors, Avidity Biosciences and Fulcrum Therapeutics. *Disclaimer: This is an educational conference. The inclusion of information about therapies and products does not imply an endorsement by the FSHD Society or by any participating research or medical institutions. Always consult your personal medical provider before trying out a novel treatment.*

Ohio FSHD 360 Conference
Speaker Bios

Anna Gilmore is Director of Patient Engagement for the FSHD Society. She joined the FSHD Society in March 2018. After spending a wonderful few years getting to know FSHD families as a part of the community engagement team, Anna is now working to open up even more avenues through which people can access information and make connections. 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. She lives in Wells, Maine with her awesome family.

Bakri Elsheikh, MBBS. Dr. Bakri Elsheikh is a Professor of Neurology, the Director of the Neuromuscular Medicine Division, and the Director of the OSU Muscular Dystrophy Association Care Center. He also serves as the site principal investigator for The Facioscapulohumeral Muscular Dystrophy (FSHD) Clinical Trial Research Network (CTRN) and has been involved in numerous FSHD natural history and drug treatment trials.

Jennifer Roggenbuck, MS, CGC is a genetic counselor and Associate Professor in the Division of Genetics, Departments of Internal Medicine and Neurology, where she specializes in the genetic evaluation of neuromuscular disorders such as muscular dystrophies, hereditary neuropathies, and motor neuron diseases. She works to bring genetic testing technology to patient care for noninvasive genetic diagnosis, genetic counseling, and gene-targeted therapy.

Marco Tellez, Lead Clinical Research Coordinator, has twelve years of clinical experience in neuromuscular diseases and four years of research involvement. Marco works with patients with rare diseases such as FSHD, ALS (amyotrophic lateral sclerosis), SMA (spinal muscular atrophy), Friedreich's Ataxia, Myasthenia Gravis, and Myopathies such as IMNM (Immune-meditated necrotizing myopathy). Specializing in personalized care, he has been awarded two institutional Bravo Awards and is a contributor for numerous collaborative publications. He loves to work on DIY home projects in the winter and spends most of his free time outdoors and traveling, learning new cultures. Fun fact: Marco has 12 chickens!

Andrea Jaworek, PT, has been a physical therapist for 13 years and participates in neuromuscular research. Since 2017, Andrea has been a clinical evaluator on 34 clinical trials, and currently serves on the training team of the FSHD Clinical Trial Research Network (CTRN). She has been a co-author on multiple abstracts and publications. Andrea is driven to help patients live their best quality of life and be as functional as they can be. She is passionate about aquatic physical therapy and exercise and loves participating in research to help further pharmaceutical advancements to help individuals with neuromuscular diseases be less impaired by their conditions and hopefully find a cure one day.

Michael Isfort, MD, grew up in Cincinnati, Ohio and completed his undergraduate and medical school education at The Ohio State University. As a long time Buckeye, he is proud to be a part of the Ohio State University Wexner Medical Center (OSUWMC) Neuromuscular Division. He has a dedication for treating patients with muscular dystrophies including FSHD and hereditary neuropathies including Charcot-Marie-Tooth disease (CMT) and familial amyloid polyneuropathy. He is a member of the Muscular Dystrophy Association clinic, the OSU CMT Center of Excellence, and the Comprehensive Amyloidosis Clinic at OSUWMC. Additionally, he part of the research team conducting FSHD clinical trials at OSUWMC.

Laura Domrose, RDN, CSO, LD joined neurology at OSU in July 2023, however, has been working for OSUMC for the past eight years. She completes consultations within the MDA, ALS and MS clinics. She completed her Bachelor's degree at Miami of Ohio University and fulfilled a dietetic internship through The Ohio State University. Prior to the neurology clinic, she worked primarily with cancer patients at The James, where she obtained a board certification in oncology nutrition. She often talks with patients about healthy diet and lifestyle changes, vitamins, supplements and enteral feeding tubes for nutrition support.

Kinsey Ludt, MSW, LSW, joined the department of Neurology in July 2021. She earned her master's degree from The Ohio State University with a focus on healthcare and mental health. Kinsey practices according to the National Association of Social Workers (NASW) code of ethics, especially the patient's right to self-determination while respecting the dignity and worth of the person. She believes that a collaborative approach to delivering healthcare reduces barriers to care and creates opportunities for more effective outcomes.

Kevin Flanigan, MD, is the Director of the Center for Gene Therapy at the Abigail Wexner Research Institute of Nationwide Children's Hospital (NCH). He has been a member of the Executive Board of the World Muscle Society since 2001 and is a past Chair of the Executive Committee of TREAT-NMD, the international alliance directed toward establishing the infrastructure to ensure that promising new therapies reach patients as quickly as possible. His laboratory focuses on the molecular characterization and therapy of neuromuscular diseases, and the identification of genetic modifiers of disease. He has conducted multiple clinical trials of gene modifying gene transfer therapies in Duchenne muscular dystrophy as well as the childhood neurodegenerative disorders mucopolysaccharidosis types 3A and 3B.

Linda P Lowes, PT PhD, is a Professor of Pediatrics at The Ohio State University and a principal investigator in the Center for Gene Therapy at Nationwide Children's Hospital. Her research interests focus on optimizing functional outcomes in adults and children with neuromuscular disorders. She is also interested in promoting clinical trial readiness through data driven outcome measure selection. To facilitate clinical trial readiness, she has conducted numerous natural history studies. She is proud to have worked on three trials that have led to FDA approval of new treatments for individuals with neuromuscular disorders.

Jamshid Arjomand, PhD, is chief science officer at the 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.

Amy Halseth, PhD, is Executive Director, Clinical Development, Avidity Biosciences. She joined Avidity in March 2022 and serves as the Program Lead for the company's FSHD program. She brings extensive experience in clinical development and medical affairs across a number of therapeutic areas, including diabetes, obesity, osteoarthritis, and pain. She has held leadership positions in industry at Amylin Pharmaceuticals, Genentech, Orexigen Therapeutics, and Biosplice. Amy holds a Ph.D in Molecular Physiology and Biophysics from Vanderbilt University.

Mihaela Levitchi Benea, MD, holds the position of Executive Director, Neuromuscular, Medical Affairs at Fulcrum Therapeutics. Her career spans over two decades in biotech and pharmaceutical industry focused primarily on neurology, including neurodegenerative and neuromuscular disorders. Mihaela graduated in Medicine and completed her residency in Neurology in Cluj-Napoca, Romania and completed a Master of Science in Pharmacology at University of Montreal, Canada.

Scott Harper, PhD, is a principal investigator in the Center for Gene Therapy at the Abigail Wexner Research Institute at Nationwide Children's Hospital and a professor of Pediatrics at the Ohio State University College of Medicine. His primary research focus at Nationwide Children's has been developing adeno-associated virus (AAV) based gene therapies to treat neuromuscular and neurological disorders, including muscular dystrophy (FSHD, LGMD1A), peripheral neuropathy (CMT1A, CMT2D, CMT4B3) and dominant epilepsy. His lab has also focused on developing models and studying the pathogenesis of facioscapulohumeral muscular dystrophy (FSHD).

Nizar Saad, PhD, is a research assistant professor at The OSU College of Medicine and a principal investigator at the Center for Gene Therapy at the Abigail Wexner Research Institute at Nationwide Children's Hospital. An experienced RNA and gene therapy scientist, he serves on multiple committees of the American Society of Gene and Cell Therapy (ASGCT). He seeks to understand the pathobiology of inherited neuromuscular disorders, identify circulating exosome-based biomarkers, and develop viral (Adeno-Associated Virus) and non-viral (Exosomes) based gene therapies towards FSHD and other rare genetic diseases (e.g., Progeria and other Laminopathies).

Sue Aumiller is the Columbus chapter and Walk & Roll leader for the FSHD Society. She joined the Society in 2017 after her son and husband were diagnosed with FSHD. She is an indefatigable advocate for the community, organizing local meetings and raising funds to accelerate the development of treatments and a cure.