



**Merriam, Kansas
October 5, 2024**

Hosted by Dr. Jeffrey Statland, the University of Kansas Medical Center, & FSHD Clinical Trial Research Network. Sponsored by the FSHD Society

Agenda

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| 9:00 – 10:00 am | Check in, social time |
| 10:00 – 10:10 am | Welcome! A new era for the FSHD community
Jeffrey Statland, MD, KUMC, director of the CTRN
Beth Johnston, Chief Community Engagement Officer, FSHD Society, |
| 10:10 – 10:35 am | FSHD clinical overview
Constantine Farmakidis, MD, KUMC, Co-director KUMC MDA Clinic |
| 10:35 – 10:55 am | The risk of shoulder injury in FSHD
William Liu, MD candidate, KUMC |
| 10:55 – 11:15 | What are we learning from whole-body MRI?
Doris Leung, MD PhD, Kennedy Krieger Institute |
| 11:15 – 12:15 | Care team panel Q&A
Physical therapy and exercise, genetic testing and counseling, orthotics and assistive devices, nutrition, mental health, accessing services |
| 12:15 – 1:15 pm | Lunch & breakout session on pain and exercise |
| 1:15 – 1:30 pm | Activating the Heartland Community – Beth Johnston |
| 1:30 – 1:40 pm | BetterLife FSHD - Kayleigh Worek, FSHD Society |
| 1:40 – 2:15 pm | Clinical trials and therapeutic approaches , Jeffrey Statland, MD |
| 2:15 – 2:45 pm | Break / Breakout session on managing fatigue |
| 2:45 – 3:05 pm | MOVE+ study overview , Michaela Walker, KUMC & CTRN |
| 3:05 – 3:25 pm | TARGET FSHD – A study of clenbuterol , Rebecca Clay, KUMC |
| 3:25 – 3:40 pm | Avidity data from Del-brax trial , Catherine Woods, Avidity Biosciences |
| 3:40 – 4:10 pm | Industry Q&A - moderated by Jeffrey Statland, MD |
| 4:10 – 4:20 pm | Closing remarks – Carolyn Lloyd, Jeffrey Statland, MD |

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.

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Speaker Bios



Jefferey 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 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 32 institutions in the United States, Canada, UK, European Union, Brazil, and Australia.



Beth Johnston, Chief Community Engagement Officer, FSHD Society. *Beth's journey with the FSHD began shortly after 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 2009-2014. Beth served on the Board of Directors from 2011 to 2014, and then joined the staff of the FSHD Society in 2016. Beth has a Master of Business Administration from the University of Denver. Beth and Jeff have 2 adult daughters, and live in Denver, CO.*



William Liu, MD Candidate, KUMC, is a fourth-year medical student at the University of Kansas School of Medicine. Both a proud KC native and an avid student researcher, he will be pursuing a neurology residency after graduation. Under Dr. Statland's mentorship, he independently developed and conducted a clinical research project to investigate the prevalence of shoulder injuries in individuals with FSHD; it is currently under review for publication. (The FSHD Society helped to recruit participants for the study.)



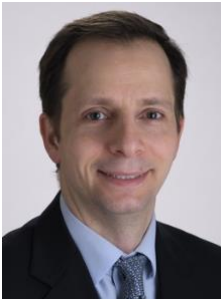
Doris Leung, MD PhD, is Associate Professor of Neurology at the Johns Hopkins University School of Medicine and Director of the Center for Genetic Muscle Disorders at Kennedy Krieger Institute. While completing her post-doctoral clinical research fellowship in muscular dystrophy under Dr. Kathryn Wagner, she obtained her PhD from the Graduate Training Program in Clinical Investigation at the Johns Hopkins Bloomberg School of Public Health. Dr. Leung leads a multidisciplinary practice for patients with all types of genetic muscle disorders. Her research centers on developing imaging biomarkers and clinical outcome measures for muscular dystrophies.



Susan Whitacre is a registered dietitian. She did her training at KU Medical Center while getting a Master's in Dietetics in 2000. Susan has worked for the MDA clinic at KU Med Center from 2000-2003 and 2005 to present. Susan helps patients with weight loss, weight gain/maintenance, swallowing issues, and tube feeding.



Mary Crouch Young, PT DPT (she/her), received her doctorate of physical therapy from the University of Kansas Medical Center (KUMC) and her master of arts in Theological Studies from the Claremont School of Theology. As a faculty member in the KUMC Department of Physical Therapy she provides clinical services for individuals with progressive neuromuscular conditions; assists with teaching neurological, cardio-pulmonary, and ethics coursework; and acts as coordinator for student integrated clinicals in the department's hybrid learning program. Dr. Crouch Young also provides home health PT for survivors of TBI and stroke.



Constantine Farmakidis, MD, is an associate professor of neurology at the University of Kansas Medical Center. He sees patients with a broad spectrum of neuromuscular diseases and has special interests in myasthenia gravis and inherited muscle and nerve diseases. He is also active in the electromyography laboratory, where he performs nerve conduction and EMG diagnostic studies. Dr. Farmakidis obtained his medical degree and neurology residency training at the University of Rochester School of Medicine. He completed a fellowship in clinical neurophysiology/EMG at Columbia University.



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, advocacy, and working to better the care and therapies that are available to patients. Michaela is also co-leader with Patrick McKinney of the FSHD Society's Kansas City chapter.



Rebecca Clay is a Senior Research Coordinator and Assistant Project Manager to the FSHD CTRN. She has a Bachelor of Science in Human Biology with a focus in Genetics and Reproduction as well as two minors in Kinesiology and Biology. Currently, she is working in the Neuromuscular Research Division in the Department of Neurology at University of Kansas Medical Center under Dr. Jeffrey Statland. She has been working with Dr. Statland and Michaela Walker as her mentors since January of 2020. Her passion is primarily in helping others and seeing the medical field grow to assist all in need.



Patrick McKenney is Dr. Statland's administrative assistant, as well as the co-chair of the Heart of America FSHD Society Chapter. He is a graduate of Loyola University Chicago and DePaul University.



Brian McHugh, Pharm.D., is a pharmacist by training and has spent the past two years working as a Medical Science Liaison (MSL) at Genentech, specializing on Neurological Rare Diseases. Drawing on his extensive background in clinical pharmacy, Brian works to bridge the gap between scientific research and clinical practice, partnering with healthcare professionals to enhance patient care and drive progress in innovative therapies for rare neurological disorders.



Catherine Woods, MSN, APRN, FNP-C, is Senior Medical Science Liaison at Avidity Biosciences. She is a Nurse Practitioner by training and was in clinical practice for over 15 years prior to shifting over to medical affairs. After finishing her nursing degree at Wichita State University, she started her clinical career at KUMC. She obtained her MSN from University of Kansas Medical Center. In her clinical career, she focused on rare genetic diseases including extensive work as a patient health care champion.



Kayleigh Worek is the Program Manager for BetterLife FSHD at the FSHD Society. She holds a master's degree in medical Speech Language Pathology and has five years of experience as a speech therapist, where she enjoyed supporting families from premature infants in NICUs to older adults in nursing homes. In 2020, she transitioned to clinical research, where she worked with biobanks, patient registries, investigator-initiated trials, and numerous global pharmaceutical-sponsored clinical trials across a variety of therapeutic areas.



Carolyn Lloyd joined the FSHD Society in September of 2023 as Chief Development Officer. She is an innovative development executive with over 20 years of experience in healthcare and higher education fundraising. Carolyn's strengths included development structure and strategy, principal and major gifts, corporate relations, committee and board management, community engagement and team building. She is a seasoned big-picture leader with a personal passion and commitment to serving those who suffer from chronic illness and their families. Carolyn lives in Kansas City Missouri with her husband Marc and rescue dog Tally.