FSHD 360 Conference
The Latest in Health & Research

Worcester, Massachusetts
November 2, 2024

Co-hosted by the UMass Wellstone Center for FSHD and the FSHD Society

UMass Chan New Education and Research Building, Room N1-1400
366 Plantation St, Worcester, MA 01605

Final Agenda

10:00 – 11:00 am	**Doors open, check in, social time / consenting for blood draw**
11:00 – Noon	**Lab tours and research posters / consenting for blood draw**
Noon – 1:15 pm	**Lunch and Welcome,** June Kinoshita, FSHD Society
	Activating the New England community Anna Gilmore, FSHD Navigator program, FSHD Society Kristin Zwickau, Kathy Senecal, New England chapter co-directors
	Overview of FSHD care and research, Lawrence Hayward, MD, UMass Chan
	Science talks. Katelyn Daman, PhD, & Dongsheng Guo, UMass Chan
1:15 – 1:30 pm	**Physiatry and FSHD** Cristina Shea, MD, Spaulding Rehabilitation, MassGeneral
1:30 – 1:45 pm	**Pediatric neuromuscular care at UMass Chen** Steve Chrzanowski, MD, PhD, UMass Chan
1:45 – 2:00 pm	**Advances in upper-body exoskeltons** Emilia Mann, Harvard Biodesign Lab
2:00– 2:45 pm	**Break and Breakouts: Q&A and conversations** Meet with the speakers above in small groups
	Blood draw for interested volunteers (please get pre-consented in advance of the meeting)
2:45 – 4:00 pm	**Drug development and clinical trials for FSHD** Lucienne Ronco, PhD, chief science officer, FSHD Society **Avidity update**, Eric Horne and Han Cho, Avidity Biosciences **Industry Q&A:** Katherine Beaverson, Dyne Therapeutics; Eric Horne and Han Cho, Avidity Biosciences; and Wes Miller, Epic Bio.
4:00 pm	**Closing remarks and Adjourn**

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.

New England FSHD 360 Conference
Speaker Bios

June Kinoshita, is Senior Director of Research and Education for the FSHD Society. June works at the intersection of research and the FSHD community. With the science team, she advises on strategies and programs to make sure the Society addresses the urgent needs and realities of those who are living with FSHD. With the community, she develops educational programs to support a highly engaged network of patients and family members who are empowered to advocate for their health and well-being and understand their vital role in advancing research. Previously, she has worked at Scientific American, Science, and co-founded the Alzheimer Research Forum and n-of-one.

Anna Gilmore is Director of Patient Engagement for the FSHD Society. Anna has been with the FSHD Society since early 2018. She has had the privilege of working closely with FSHD families through the volunteer-driven Chapter and fundraising programs. Lately, she has been working to build up the newly formed FSHD Navigator program, government advocacy initiatives, and engagements with the FDA. She comes to the organization from Northeastern University, where she worked in the City & Community Affairs division for 7 years. She lives in southern Maine with her family, who frequently guest-star in the background of her Zoom calls.

Lawrence J. Hayward, M.D., Ph.D. is Professor of Neurology and Director of Neuromuscular Division at University of Massachusetts Chan School of Medicine. Dr. Hayward is a physician-scientist and board-certified neurologist who directs the multidisciplinary FSHD clinic at UMass Chan Medical School and participates in basic and translational research as co-director of the UMass Wellstone Center for FSHD. He leads evolving FSHD clinical trials and directs a biomarker study to improve outcome measures for future FSHD trials. He collaborates closely with Dr. Charles Emerson's lab of the Wellstone Center to establish cellular and preclinical models that may accelerate progress toward new FSHD therapies.

Katelyn Daman, PhD, is a senior research scientist in Charlie Emerson's lab at UMass Chan Medical School where her goal is to develop a therapy for FSHD. Katelyn is using cell and animal models which can be used in preclinical FSHD drug development studies. She is currently collaborating with several companies and colleagues in the RNA Therapeutics Institute at UMass to advance lead therapeutic candidates. Katelyn received her Ph.D. in molecular and cell biology from Brandeis University and completed postdoctoral research in the Emerson lab before staying on as a senior researcher.

Dongsheng Guo, PhD, is an instructor at the UMass Chan Wellstone Center for FSHD, specializing in muscular dystrophies, including FSHD and LGMD, using patient-derived induced pluripotent stem cells (iPSCs). He developed a novel technology to generate functional muscle stem cells from human iPSCs, known as iMyoblasts. Dr. Guo continues to utilize this technology to develop universal muscle stem cells and model muscular dystrophies, aiming to understand their molecular pathologies and develop gene editing and drug-based therapeutics for treatment. In addition, he is investigating hearing loss in FSHD through FSHD iPS-derived organoids to explore the underlying mechanisms and develop potential treatments.

Cristina Shea, MD, is a physiatrist (physical rehabilitation doctor) with a particular interest in neuromuscular conditions. Her goal is to help individuals living with physical disability maximize function and quality of life. Dr. Shea completed residency in Physical Medicine and Rehabilitation at Spaulding Rehabilitation Hospital and Neuromuscular Fellowship at MGH/Brigham and Women's Hospital in Boston. During her training, she met many patients with physical symptoms leading to mobility limitation. Now as a physician at Spaulding and the Brigham she hopes to help patients best address these symptoms. This may include combining different types of therapy, medications, mobility aids, bracing, and/or injections.

Steve Chrzanowski, MD, PhD, is an Assistant Professor at the UMass Chan Medical School in Neurology and Pediatrics and currently serves as the co-director of the UMass Duchenne Program. Previously, he completed Pediatric and Child Neurology residencies (Boston Children's Hospital), a Neuromuscular Fellowship (Mass General Brigham), and MD-PhD degrees (University of Florida). Dr. Chrzanowki's interests revolve around earlier identification and intervention to alter long-term outcomes via the development and quantification of novel therapeutics. His research team attempts to answer the question if earlier diagnosis and non-invasive longitudinal monitoring will lead to improved clinical outcomes.

Emilia K Mann is a Research Fellow in functional apparel design at the Harvard Biodesign Lab. The Harvard Biodesign Lab brings together researchers from the engineering, industrial design, medical, and business communities to develop robots and smart medical devices that are specifically intended for interacting and cooperating with humans. As a functional apparel designer, Mann designs and constructs textile based wearable robots intended to both assist and facilitate therapeutic recovery of populations with upper limb impairment.

Lucienne Ronco, PhD, is chief science officer at the FSHD Society. Dr. Ronco earned her PhD at UCLA spent time in academia at Harvard and the MIT-Harvard Broad Institute. In industry, she has worked at Astra Zeneca and Fulcrum Therapeutics. Throughout her career, Ronco has navigated the complex landscapes of both pharmaceutical giants and biotech start-ups, rising to leadership positions that have shaped the direction of drug development. Dr. Ronco will discuss the genetic and molecular basis of FSHD, including the role of the DUX4 gene. She will review novel findings and concepts in our understanding of FSHD, describe the drug development and clinical trial process, and review current trials.

Wes Miller, MD, is chief medical officer at Epic Bio (EpiCRISPR Biotechnologies). A Stanford University graduate, he received his MD at Louisiana State University School of Medicine and was an associate professor at the University of Minnesota specializing in pediatric hematology and oncology and blood stem cell transplantation. In 2018 he joined industry, working at Sangamo Therapeutics, Astellas Gene Therapies and Graphite Bio before coming to Epic Bio. He has drug development experience in gene modified hematopoietic stem cell therapies and in vivo gene editing and gene addition therapies for various rare diseases, including muscle disorders.

Katherine Beaverson, MS, is Vice President of Global Patient Advocacy at Dyne Therapeutics, guiding the strategic planning and implementation of collaborations with patient advocacy groups and patient communities to integrate patient experience and expertise into the medicines development and delivery continuum. She held similar roles in industry with Pfizer, Inc., Boehringer Ingelheim and Amicus Therapeutics. A professionally trained genetic counselor, she spent 10 years at both New York Hospital-Weill Cornell Medical Center and Memorial Sloan-Kettering Cancer Center. She received her BA from Swarthmore College and her MS in human genetics from Sarah Lawrence College. She is the Former Chair of the IRDiRC Companies Constituent Committee and a member of the NYU Pediatric Gene Therapy and Medical Ethics Working Group.

Eric Horne, PhD, is a pharmacologist by training with a passion for communicating how the knowledge of biologic mechanisms leads to drug discovery and development. He has been working in Medical Affairs for the past 12 years supporting multiple drug development programs and post-approval educational initiatives. Eric obtained his PhD in Pharmacology from the University of Colorado School of Medicine and received additional training as a Post-doctoral Fellow at the University of Washington. He is currently a Senior Director of Medical Affairs at Avidity supporting the neuromuscular clinical development programs.

Han Cho, PharmD, is a pharmacist by training with experience in clinical practices and publication management. After completing his pharmacy degree at Midwestern University and master's degree in drug development at UCSD, he has been with Avidity Biosciences supporting publication development. He is currently a medical science liaison with the Medical Affairs team at Avidity supporting clinical development programs in DM1, FSHD, and DMD.

Kathy Senecal has been the New England Chapter volunteer director since 2017. In addition to convening virtual and in-person gatherings, Kathy organizes Walk & Roll to Cure FSHD teams. Kathy and her husband raised their family in Connecticut and after retirement, have enjoyed traveling and family vacations along the coast of Maine. "It's an honor and privilege to be a part of this vibrant community as we continue to move forward with advocacy and potential treatments," she says.

Kristin Motta Zwickau is an accomplished executive leader in talent management, currently serving as VP of Talent Attraction at Veeva Systems, a life sciences technology company. In addition, Kristin is a passionate advocate for patients with early-onset FSHD. She actively supports the Early Onset Chapter of the FSHD Society and was recently appointed Secretary of the FSHD Cure Initiative, a foundation dedicated to expanding clinical trial access for this underserved population in the FSHD community.

UMass Chan MEDICAL SCHOOL

UMASS CHAN MEDICAL SCHOOL
UNIVERSITY CAMPUS

Site Plan - May 2024