2014 FSHD Connect
The FSH Society’s Biennial International Network Meeting for FSHD Patients, Families, Clinicians and Researchers

Saturday, August 16 and Sunday, August 17, 2014
The Westin Boston Waterfront Hotel
425 Summer Street, Boston, MA 02210 USA
Westin Boston Waterfront
Conference Level

Men’s Restroom indicated by figure
Women’s Restroom indicated by figure
Dear Friends,

Welcome to the 2014 FSHD Connect International Patient Researcher Networking Conference for Facioscapulohumeral Muscular Dystrophy (FSHD). Thank you for your commitment in working with the FSH Society and the FSHD patient, research and clinical communities to find solutions for FSHD. The FSH Society is a world leader in combating muscular dystrophy. It has provided nearly $5.8 million in seed and start-up grants to pioneering FSHD research. We have provided education worldwide on FSHD and created an international collaborative network of patients and researchers. This year’s program demonstrates the fruits of an ongoing, challenging, and productive dialogue that the FSH Society has facilitated over the years.

During the next two days, you will hear from distinguished speakers on clinical and research topics related to FSHD. In addition to the lecturers and moderators, we have many prominent FSHD researchers and clinicians in the room with us, and we encourage you to reach out and say hello to those sitting next to you, across from you and around the room. Our good colleagues and friends are here to offer support and gain insights. Each new day brings new knowledge and new technologies that will help make for a longer and higher quality of life. We are now 6,000 families strong and our shared experience and constituency allows us to clearly see where the opportunities are and where the deficits are in care for those with FSHD.

Many of you know the extraordinary impact the FSH Society has had in advocacy, education, research, networking, and helping promote solutions. The FSH Society's work in education, advocacy, and training has led to increased funding in the United States and abroad. It was a key participant in drafting the Muscular Dystrophy Community Assistance Research and Education Act of 2001 (MD CARE Act), which in the United States mandates research and investigation into all forms of Muscular Dystrophy. The MD CARE Act has helped provide nearly $462 million dollars in additional NIH funding since its inception and this in turn has fostered billions of dollars in non-federal funding into muscular dystrophy in the past 13 years. We are hopeful that by this meeting the MD CARE Act 2014 will be a continuing reality.

These successes notwithstanding, it remains a daily challenge to attain the funds we need to survive and grow. The Society’s work of the past 23 years has been largely driven by the passion, dedication and extremely long hours given by a very small group of people committed to change. Prof. David Housman, Chairman of the FSH Society Scientific Advisory Board (SAB), and his colleagues on the SAB have given selflessly of themselves, their knowledge and their time at no charge to bring about insight into FSHD to help those suffering from the disease. Our colleagues on the Board of Directors work countless hours without remuneration to guide the organization and fundraise. We stand on the brink of developing new treatments, and now we need all of you more than ever, to help reach our goals. We need you to volunteer for research and raise awareness. And we need your help to raise millions of dollars to sustain the pace of research and prepare for future clinical trials. Please, support the Society generously, and urge friends, family, business colleagues and your communities to do the same.

The FSH Society wishes to thank the 2014 FSHD Connect speakers and all attendees for their contributions to the success of this meeting. We thank our sponsors, including: Association Francaise contre les Myopathies, Cytokinetics, FSHD Global Research Foundation, FSH Society, Genzyme a Sanofi Company, Muscular Dystrophy Campaign (UK), U.S. DHHS NIH University of Massachusetts Medical School Senator Paul Wellstone MD Cooperative Research for FSHD, Quintiles, and Ride-Away for helping to make this meeting possible. We especially thank all of the attendees for supporting this meeting through gifts and registrations and by actively participating.

You should feel good about being here today. You should know that you are not alone and that many talented people are working hard to solve and treat FSHD. We hope you feel empowered by the patient-patient and patient-researcher interactions. The Society is here for you and we count on you to ask others to support the Society and allow us to continue the excellent research and programs.

Sincerely,

William R. Lewis, Sr., M.D.
Chairman, Board of Directors
FSH Society

Daniel Paul Perez
President & CEO
FSH Society

June Kinoshita
Executive Director
FSH Society
Friday, August 15

4:00 – 6:00 pm  Registration.  Harbor Ballroom Foyer
5:00 – 6:00 pm  Visit to MIT Media Lab (fully subscribed)
6:30 – 9:00 pm  Informal reception.  Birch Bar, Westin Boston Waterfront lobby

Saturday, August 16

8:00 am  Registration opens.  Harbor Ballroom Foyer.  Breakfast on own.
8:45 – 9:00 am  Welcome and Keynote.  Harbor Ballroom
9:00 – 10:00 am  Research Summit Part 1

- Silvère Van der Maarel, Leiden University Medical Center. "Genetics of FSHD."
- Yi-Wen Chen, National Children’s Hospital. "A multicenter collaborative study on infantile onset facioscapulohumeral muscular dystrophy."
- Peter Jones, University of Massachusetts Medical School. "Genetically similar, epigenetically different: Insights into FSHD from studying discordant families"
- Louis Kunkel, Children’s Hospital of Boston. "Modeling FSHD in Zebrafish for Therapy Development."

10:00 – 10:40 am  Panel discussion and Q&A
10:40 – 11:10 am  Break (Light food, coffee, tea, and juice available).  Harbor Ballroom Foyer
11:10 – 11:55 am  Research Summit Part 2

- Stephen Tapscott, Fred Hutchinson Cancer Research Center. "A consensus model for FSHD identifies opportunities for therapy."
- Michael Kyba, University of Minnesota. "Discovering and testing therapeutics."
- Alexandra Belayew, University of Mons. "Several ways to go DUX hunting."

11:55 am – 12:30 pm  Panel discussion and Q&A
12:30 – 12:35 pm  Brief announcements and housekeeping
12:35 – 2:00 pm  Lunch (included in registration).  Harbor Ballroom Foyer
FSH Society Development Committee: Brainstorming on creative fundraising.  Harbor Ballroom

2:00 – 5:00 pm  Main hall: exhibitors and clinical center tables.  Harbor Ballroom Foyer
Science corner

2:00 – 3:00 pm  Concurrent Patient-led Breakout Sessions – Living with FSHD

- Parents of children with FSHD (Facilitator: Ray Huml)  Carlton room
- Navigating teenage life and transitioning into adulthood (For teens and young adults only)  Facilitators: Ben Brittain & Carden Wyckoff)  Griffin room
- Living with constant change (Facilitator: Daniel Perez)  Burroughs room
- Nutrition 101 (Facilitator: Sabrina Sacconi MD)  Harbor Ballroom III
• Navigating FSHD in relationships for adults (Facilitator: Hilary Michels)  *Lewis room*
• Fundraising and events (Facilitators: Amy Bekier, Ellen Hannan, Beth Johnston)  *Harbor Ballroom I*

3:00 – 3:30 pm  **Break** (Refreshments)  *Harbor Ballroom Foyer*

3:30 – 4:30 pm  **Repeat of Breakout Sessions – Living with FSHD**

• Parents of children with FSHD (Facilitator: Ray Huml)  *Carlton room*
• Navigating teenage life and transitioning into adulthood  *(For teens and young adults only)*
  Facilitators: Ben Brittain & Carden Wyckoff  *Griffin room*
• Living with constant change (Facilitator: Daniel Perez)  *Burroughs room*
• Nutrition 101 (Facilitator: Sabrina Sacconi MD)  *Harbor Ballroom III*
• Navigating FSHD in relationships for adults (Facilitator: Hilary Michels)  *Lewis room*
• Fundraising and events (Facilitators: Amy Bekier, Ellen Hannan, Beth Johnston)  *Harbor Ballroom I*

5:00 – 6:00 pm  **Workshops**
Hands-on Workshop: Mindfulness in managing pain and stress  *Burroughs room*
FSHD Science Journal Club  *Harbor Ballroom III*

6:00 pm  **Adjourn for the day**
Dinner on your own.

*Photography:*  *Staff and participants will be taking photos during the meetings for use in FSH Society print and electronic publications. If you do not wish a photo/video of yourself to be used in this manner, please move out of the picture and/or give one of the Society’s staff a written note with this instruction.*
Sunday, August 17

8:00 am Registration opens. Harbor Ballroom Foyer. Breakfast on own.

9:00 – 9:05 am Day 2 welcome and introduction Harbor Ballroom

9:05 – 10:05 am Medical Summit Part 1

- Jeff Statland, University of Rochester. “Opening the clinical trial toolbox.”
- Sabrina Sacconi, Nice University Hospital. “Advances in the care of FSHD.”
- Baziel Van Engelen, Radboud University Nijmegen Medical Center. “Reducing chronic fatigue through exercise and cognitive behavioral therapy.”

10:05 – 10:30 am Panel discussion and Q&A

10:30 – 11:00 am Break (Light food, tea, coffee, and juice). Harbor Ballroom Foyer

11:00 – 11:45 am Medical Summit Part 2

- Shree Pandya, University of Rochester. “Update on physical therapy and exercise recommendations.”
- Kofi Boahene, Johns Hopkins University Medical School. “Surgical and nonsurgical options for facial reanimation in FSHD.”
- Ray Huml, Quintiles. “Clinical trial readiness and the patients’ roles today.”

11:45 – 12:15 pm Panel discussion and Q&A

12:15 – 1:00 pm “Reverse Q&A” – Doctors and scientists ask patients questions.

1:00 – 2:00 pm Lunch (included in registration). Harbor Ballroom Foyer

Advocacy and awareness – The Selfie campaign and beyond. Harbor Ballroom

2:00 – 5:00 pm Main hall: Exhibitors and clinical center tables. Harbor Ballroom Foyer

Science corner

2:00 – 3:00 pm Concurrent Breakout sessions – Living with FSHD 2

- Exercise and athletics (Facilitator: Kristin Duquette) Harbor Ballroom I
- Traveling with FSHD (Facilitators: Cyndi Segroves & Ray Jordan) Harbor Ballroom III
- Genetics – to test or not to test? Families, family planning and beyond (Facilitators: Carol Hoffman, PhD, MS, LGC; & Carol Birnbaum, MD) Burroughs room
- Navigating FSHD in relationships for teens and young adults (For teens and young adults only) (Facilitator: Hillary Michel) Griffin room
- Caregiving (For caregivers only) (Facilitators: Beth Johnston & Fred Thys) Lewis room
- Dress and Fashion (Facilitator: Kristina McMullin) Carlton room

3:00 – 3:30 pm Break (Refreshments). Harbor Ballroom Foyer
2014 FSH Society FSHD Connect Agenda

3:30 – 4:30 pm  Concurrent Breakout sessions – Living with FSHD 2

- Exercise and athletics (Facilitator: Kristin Duquette) Harbor Ballroom I
- Traveling with FSHD (Facilitators: Cyndi Segroves & Ray Jordan) Harbor Ballroom III
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- Caregiving (For caregivers only) (Facilitators: Beth Johnston and Fred Thys) Lewis room
- Dress and Fashion (Facilitator: Kristina McMullin) Carlton room

4:30 – 5:00 pm  Finale. Assessments, feedback and farewell. Harbor Ballroom

Photography: Staff and participants will be taking photos during the meetings for use in FSH Society print and electronic publications. If you do not wish a photo/video of yourself to be used in this manner, please move out of the picture and/or give one of the Society’s staff a written note with this instruction.
**FSHD CONNECT FACULTY DIRECTORY AND BIOGRAPHIES**

**Alexandra Belayew, PhD**
Professor & Laboratory Head  
Department of Molecular Biology  
Université de Mons-Hainaut  
Mons, Belgium

The DUX4 gene that maps in the D4Z4 repeated element was discovered in 1996, by Dr. Belayew’s group in Prof. D. Collen’s Research Center at the University of Leuven, Belgium. The project was continued in her own laboratory established at the University of Mons in September 1999. The Belayew lab has accumulated unusual expertise and developed very sensitive tools to demonstrate expression of the very low abundance DUX4 mRNA and protein. They have developed antisense oligomers in collaboration with Prof. S. Wilton (Molecular Gene Therapy Group, University of South Australia) targeting the DUX4 mRNA to prevent expression of its protein and the resulting toxicity caused to primary myoblast cultures. The Belayew group is also supporting Co-investigator Frédérique Coppée in collaboration with Prof. R. Fitzsimons (University of Sydney) to investigate the deregulations of the WNT pathway in FSHD myotubes; this project is of major interest since it can explain the two phenotypes of FSHD myotubes (atrophic or disorganized) and point to a therapeutic strategy with drugs already available for human use.

**Kofi Boahene, MD, FACS**  
Associate Professor of Facial Plastic and Reconstructive Surgery  
& Otolaryngology Head and Neck Surgery  
The Johns Hopkins Hospital  
Baltimore, Maryland

Ghana, Russia, Australia and United States are only a few of the countries where Dr. Boahene has lived and studied, and his current approach to facial plastic surgery is a true reflection of his international education. Dr. Boahene’s practice encompasses the entire spectrum of facial plastic and reconstructive surgery, including rhinoplasty, nose reshaping, eyelid surgery, facial rejuvenation surgery, fat transfers, facial augmentation, wrinkle treatment, corrective surgery for congenital facial defects, cleft lip and palate repair, ear reshaping, craniofacial surgery, minimally invasive and endoscopic skull base surgery, microsurgery, reconstruction of cancer patients and extensive post-traumatic deformities. Dr. Boahene has pioneered reconstructive techniques for FSHD patients. He has received numerous awards, including the Distinguished Mayo Brother’s Fellowship Award, a very prestigious award given to only two surgeons in any given year throughout the Mayo Foundation. Recently, Dr. Boahene was awarded the Jack R. Anderson Prize for Scholastic Excellence, for attaining the highest score in the nation on written and oral exams administered by the American Board of Facial Plastic and Reconstructive Surgery.

**Yi-Wen Chen, DVM, PhD**  
Associate Professor of Pediatrics and Integrative Systems Biology  
Children’s National Medical Center  
George Washington University  
Washington, DC

Dr. Chen’s research interests focus on the pathophysiological mechanisms of muscle disorders and molecular responses to muscle disuse and rehabilitation. She started her research in FSHD by studying molecular mechanisms of the disease. In collaboration with Dr. Sara Winokur, UC Irvine, the collaborative group first reported genome-wide gene expression changes in FSHD and proposed potential mechanisms of the disease. Subsequently, in collaboration with Dr. Alexandra Belayew, the group demonstrated aberrant expression of double homeobox protein 4 (DUX4) in FSHD and reported the polyadenylation signal of DUX4 in the genomic region adjacent to the last DUX4 copy. Her group then examined downstream regulatory targets and pathways of DUX4, regulatory roles of miRNA in FSHD, and gene regulatory proteins that modulate DUX4 expression. The most recent focus of her studies is to explore potential therapeutic strategies and identify biomarkers for FSHD. She is currently the co-chair of a multicenter collaborative study on the clinical features, expression profiling, and quality of life of pediatric fascioscapulohumeral muscular dystrophy supported by the FSH Society.
Dr. Raymond Huml has over 23 years of experience in the biopharmaceutical industry. He previously held the position of Head of Global Due Diligence for Quintiles Corporate Development and identified risks associated with Quintiles global, product-based investments. Working in various roles within Quintiles, he has worked on all major investment transactions involving more than $2.9 billion in capital on alliances with biotechnology and pharmaceutical partners of all sizes. Dr. Huml understands the risks involved with biosimilar programs from reviewing over $500 million worth of opportunities and closing over $75m in deals involving biosimilar products. Previously, Dr. Huml worked in the Center for Integrated Drug Development, Biostatistics, Medical Writing, and Regulatory Affairs Departments in Quintiles Clinical Development Services group. Dr. Huml has published over thirty five articles for Regulatory Focus Magazine on topics such as biosimilars and due diligence. In 2013, Dr. Huml co-wrote the chapter on biosimilars for the 7th Edition of Wiley’s The Textbook of Pharmaceutical Medicine.

Dr. Peter Jones first made his mark in the field of epigenetics as a postdoctoral fellow with Dr. Alan Wolffe at NIH in Bethesda, Maryland where he identified the connection between DNA methylation, histone modifying enzymes and a repressive chromatin structure. Dr. Jones’ interest in FSHD began in 2003, when, as an Assistant Professor at the University of Illinois at Urbana-Champaign, a graduate student who had been recently diagnosed with FSHD introduced him to the disease and the possible role of epigenetics. By 2006, the Jones lab was working full-time on multiple FSHD projects thanks to funding from the FSH Society and the National Institutes of Health. In 2008, Dr. Takako Jones joined the lab full-time, bringing great expertise in developmental, cellular and molecular biology. Currently, the Jones’ function as Co-Principal Investigators of their lab at the University of Massachusetts Medical School and are investigating the epigenetics of FSHD families, studying the mechanisms regulating DUX4 expression in muscle and developing FSHD-like animal models for preclinical testing of FSHD therapeutics.

Louis M. Kunkel is internationally recognized in the field of human genetics and the genetics of muscular dystrophy in particular. Over the past three decades, Dr. Kunkel has devoted his career to understanding the molecular basis and developing a therapy for neuromuscular disorders and muscular dystrophy. His major accomplishments include the discovery of the gene that causes Duchenne muscular dystrophy (1986) and being the first to pinpoint that gene’s importance in producing the critically needed muscle protein, dystrophin (1987). In the 1990s, he demonstrated that injection of either blood stem cells or muscle stem cells can partially restore dystrophin in affected skeletal muscles—a finding that may lead to treatments for many types of muscle diseases. Dr. Kunkel has authored over 200 journal articles and 20 book chapters, and received more than 20 awards and honors for scientific leadership and achievement, including memberships in prestigious academies. He holds various director and chairman roles and is a professor of pediatrics and genetics at Harvard Medical School.
Dr. Kyba's laboratory studies the regulation of skeletal muscle stem cells, and possible defects in skeletal muscle regeneration in FSHD. He has ongoing research projects studying the DUX4 protein, identifying new drugs targeting DUX4, developing animal models for FSHD and using gene-targeting / genetic correction in FSHD cells. The DUX4 gene has similarity to a gene called Pax7. Dr. Kyba's lab has shown that DUX4 and Pax7 can compete for control of expression of myogenetic regulators such as MyoD, and are testing the hypothesis that DUX4, expressed in satellite cells (muscle progenitor cells), interferes with the formation of muscle cells in FSHD-affected muscle. Because satellite cells are rare and difficult to access, the Kyba lab generated “induced pluripotent stem cells” from FSHD patients, and is studying chromatin changes and gene expression at 4qter that occur with the development of the muscle lineage and skeletal muscle stem cells in vitro. Using a cellular model of FSHD, the Kyba lab recently published the first high-throughput drug screening study of compounds that can block DUX4 toxicity. Dr. Kyba is active in the International Society for Stem Cell Research, American Society of Hematology and the American Heart Association.

Shree Pandya has been involved in clinical care, research and education in neuromuscular disorders for the past thirty years. Dr. Pandya’s areas of expertise in research are related to outcome measures, natural history studies, clinical trial design and implementation. She has been involved in multicenter studies of FSHD as well as other muscular dystrophies, Inclusion Body Myositis and several other rare neuromuscular disorders. Currently Dr. Pandya serves as co- principal investigator in New York for the Muscular Dystrophy Surveillance and Research Network (MD STARnet) funded by the Centers for Disease Control and Prevention (U.S. CDC). Dr. Pandya’s clinical interests are related to physical therapy assessments and interventions targeted to maximize function and maintain health and wellness in patients with neuromuscular disorders. She has worked with various advocacy organizations – MDA, Myotonic Dystrophy Foundation and FSH Society – providing guidance in the areas of physical therapy management. Dr. Pandya has also been involved in providing continuing education to physical therapists in the area of neuromuscular disorders at the state, national and international levels.

Since 2004, Dr. Sabrina Sacconi has had a permanent position as a Neurologist in Nice University Hospital, where she developed clinical and basic research on neuromuscular diseases and contributed in creating the Neuromuscular Diseases Specialized Center for the diagnosis and management of rare neuromuscular disorders. Dr. Sacconi is the coordinator of a Research Group on Neuromuscular Disorders at the CNRS UMR 6543, at the University of Nice and coordinator of the clinical activity on neuromuscular diseases in Nice University Hospital. Her main fields of investigation are myopathy and neurogenic disorders, and more specifically, she works on facioscapulohumeral muscular dystrophy physiopathology, genetics of mitochondrial diseases and cell therapy for muscular dystrophies.
Dr. Jeffrey Statland has both clinical and research training in neuromuscular diseases, with a primary research interest in FSHD. Recent advances in our understanding of the genetic mechanism behind FSHD have led to the identification of potential therapeutic targets, resulting in a pressing need to develop sensitive, disease-relevant outcome measures for clinical trials. Dr. Statland’s current research includes an FSHD disease-specific patient reported health inventory, an FSHD-specific functional rating scale, and the reliability of electrical impedance myography. With Dr. Rabi Tawil at the University of Rochester Medical Center, he is also attempting to develop molecular and neuro-imaging biomarkers of disease activity for future FSHD clinical trials. “By working hand in hand with the basic scientists and our international collaborators we not only learn about how to measure the disease progression in FSHD, but we also gain insight into the molecular underpinnings of FSHD, and the corresponding clinical expression in patients. Together we can develop the institutional infrastructure and tools to run high quality, efficient clinical trials.”

The Tapscott lab focuses on gene transcription in a chromatin context in normal development and disease. In addition, the lab studies gene expression in rhabdomysarcomas (cancers with characteristics of skeletal muscle) and human muscular dystrophies. For more than two decades, researchers have known that FSHD is caused by a genetic mutation, but no one understood the mechanics of the disease. After several years of intensive research, a team led by Dr. Tapscott broke through the decades-long stalemate with FSHD. Dr. Tapscott’s lab collaborates with GlaxoSmithKline to develop muscular dystrophy therapeutics. The goal of the new agreement is to develop a small-molecule-based medicine to potentially reverse FSHD by inhibiting the activity of a protein that is incorrectly expressed by the DUX4 gene in people with the disease.

Dr. Van der Maarel’s scientific interests focus on the genetic and epigenetic regulation of repetitive DNA in the human genome in relation to disease. His main interest is the genetic and epigenetic basis of facioscapulohumeral muscular dystrophy (FSHD) type 1, an adult muscle disease caused contraction of the D4Z4 macrosatellite repeat on the tip of the long arm of chromosome 4. He also studies other epigenetic diseases affecting the chromatin structure repetitive DNA, resulting the identification of mutations in ZBTB24 as the cause for the primary immunodeficiency type 2 ICF syndrome and mutations in SMCHD1 underlying type 2 FSHD.
Baziel G.M. van Engelen, MD, PhD
Chair in Neuromuscular Disorders, Neuromuscular Centre Nijmegen
Department of Neurology (HP 935) Radboud University Nijmegen Medical Centre, Nijmegen, The Netherlands

Baziel van Engelen studied medicine at the Radboud University Nijmegen Medical Centre and studied philosophy at the University of Amsterdam which he finished cum laude in 1992. He did his Neurology residency at the Universities of Berlin, Germany and Nijmegen, followed by a research fellowship in the Mayo Clinic and Mayo Foundation in Rochester Minnesota, USA. He became associate professor for neuromuscular diseases at the Radboud University Nijmegen Medical Centre in 2000, full professor in 2003, and European Neuromuscular Centre research director in 2010. He has published over 300 articles in his research field. His focus is on bedside-to-bench translational research on neuromuscular disorders. Starting from the individual patients' clinical question, multidisciplinary research is performed with a wide array of other disciplines, from histology, genetics, cell biology, physiology, and imaging to psychology, rehabilitation, and health technology assessment. Ultimate goal is to understand the mechanisms of disease for optimal management and treatment of each specific patient. Main diseases of interest are facioscapulohumeral muscular dystrophy (FSHD), neuralgic amyotrophy, the myotonic dystrophies, and inclusion body myositis.

Kathryn R. Wagner, MD, PhD
Director, The Center for Genetic Muscle Disorders, Kennedy Krieger Institute
Associate Professor, Departments of Neurology and Neuroscience
Co-Principal Investigator, NIH Wellstone Center for FSHD Research
The Johns Hopkins School of Medicine Baltimore, Maryland

Dr. Kathryn R. Wagner treats patients with muscular dystrophies in a multidisciplinary clinic, addressing the multiple medical and social issues affecting individuals and families. Dr. Wagner conducts clinical trials and studies in FSHD currently including a bone health study, a biomarker study and a clinical trials outcome measures study. Dr. Wagner’s laboratory focuses on developing methods to promote muscle regeneration. A major emphasis of her laboratory has been on modulating myostatin, an endogenous regulator of muscle. Dr. Wagner’s laboratory has shown that inhibition of myostatin stimulates muscle stem cells, improving muscle regeneration while reducing fibrosis in animal models of muscular dystrophy. Current efforts include collaborations with industry to combine stem cell and pharmacological therapies for enhanced regeneration. She is an advisor for the FSH Society, Parent Project Muscular Dystrophy and the TREAT-NMD Advisory Committee for Therapeutics.
Sponsors of the Boston International Patient and Researcher Network Meeting 2014:

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