

# FSH Watch



CONNECTING THE COMMUNITY OF PATIENTS, FAMILIES, CLINICIANS, AND INVESTIGATORS



## ADVOCACY

### Our own American Ninja Warrior

*FSHD gets national exposure on NBC*

by **CARDEN WYCKOFF**  
Atlanta, Georgia

**N**BC's *American Ninja Warrior* is a competitive obstacle course race where people from all over the country apply to take on the challenge. The main purpose behind the show is also to showcase the competitors' stories or causes. My brother Spencer—a 6'7" beast of a human being—has decided his mission is to raise awareness for FSHD, and so when *American Ninja Warrior* put out a call for try-outs in Atlanta, he was on it.

Spencer applied to appear on the show back in January with a video explaining that his reason to compete was to raise awareness and fund a cure for FSHD. Then, in early March during a family night dinner, he got the surprise call notifying him that he had been selected!

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**Spencer and Carden Wyckoff.**



**University of Kansas Clinical Trial Center.**



## RESEARCH

### FSH Society establishes Clinical Trial Research Network for FSHD

*\$121,000 launches initiative to expedite future drug trials*

by **JUNE KINOSHITA**  
FSH Society

**T**he FSH Society has awarded a \$121,000 grant to co-principal investigators Jeffrey Statland, MD, of the University of Kansas Medical Center, and Rabi Tawil, MD, of the University of Rochester Medical Center, New York, to expedite studies of therapies for FSHD by developing a core FSHD Clinical Trial Research Network (CTRN). The FSH Society awards funds to four institutions

that are piloting the CTRN: the University of Rochester, Kennedy Krieger Institute, Ohio State University, and the University of Kansas have all established centers with expertise in FSHD and neuromuscular clinical trials.

Underscoring the keen interest in creating a network for FSHD clinical research, additional funding obtained by Dr. Tawil from a private foundation will enable the CTRN to be expanded to a total of seven sites. The additional sites include the University of California, Los Angeles; the University of Utah; and the University of Washington.

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## Tenacity and teamwork

Dear Friends,  
There's nothing more gratifying for me than meeting FSH Society members in their communities and seeing the impact our work is having on people's lives.

This July, I had the pleasure of participating in an FSHD Patient Day at the University of Kansas Medical Center (KUMC), which was organized by Jeff Statland, MD PhD, and his team. More than 50 patients and their family members attended and soaked up a series of excellent talks. These gatherings are often an intensely emotional experience, with many individuals meeting others with FSHD for the first time in their lives. It is a privilege to help make such moments happen.

Just as gratifying was to hear Dr. Statland describe the very real progress being made toward developing treatments, much of it with support from the FSH Society. On the day before the Patient Day, I had the opportunity to visit the KU Clinical Research Center, a state-of-the-art facility equipped to carry out clinical studies and treatment trials. KUMC is one of four institutions piloting the FSHD Clinical Trial Research Network (CTRN), which was launched this year with a grant from the FSH Society (see our cover story).

The CTRN is a cornerstone of the infrastructure the FSHD field needs for drug development and clinical trials. Another is the FSHD biospecimen registry, also launched this year through an FSH Society grant (see page 4). By joining this registry, individuals with FSHD can arrange for their tissues to be recovered from surgeries or after death, and delivered to any



June Kinoshita

qualified scientific researcher around the world who needs it. Please consider joining the registry. This is the most powerful and precious gift you can leave to future generations.

Research funded by the FSH Society—fueled by your donations—has helped to establish a strong foundation for understanding the molecular basis of FSHD, and today we are seeing the fruits of these pioneering efforts. Dozens of companies, from biotech start-ups to pharmaceutical giants, are now developing therapies aimed at FSHD. Fulcrum Therapeutics is but the latest to join this group (see page 8).

The advances we are seeing in the FSHD field today give us real hope. They are the direct result of so many patients, families, researchers, and clinicians who have worked alongside the FSH Society over the past 25 years, never giving up, and determined to succeed.

Indeed, whether it's the Biviano brothers living in a tent for two years to wring concessions from the Italian government (page 16), Spencer Wyckoff tackling the punishing obstacle course on *American Ninja Warrior* (page 1), or Ken Colosi's dogged pursuit of Chase Bank bureaucrats (page 7), FSHD seems to engender an extraordinary degree of tenacity!

Thank you for your constant and steadfast support, and for your donations in the past. We must continue our work, and I hope you'll give generously at this time to ensure that the great advances and opportunities that we're creating together will reach fruition.

It's an honor to be on your team.



June Kinoshita  
Executive Director, FSH Society

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It is our editorial policy to report on developments regarding FSHD, but we do not endorse any of the drugs, procedures, treatments, or products discussed. We urge you to consult with your own physician about any medical interventions.

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## FSH SOCIETY ESTABLISHES CLINICAL TRIAL RESEARCH NETWORK FOR FSHD

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The CTRN will comprise academic research centers working closely with the FSH Society to collaboratively develop, test, and validate clinical outcome measures and biomarkers. A prospective study of 150 patients followed over 18 months will be needed to validate the outcome measures of the Clinical Trial Research Network.

“This Clinical Trial Research Network is a milestone for the FSHD and muscular dystrophy community,” said Daniel Perez, co-founder, president, and CEO of the FSH Society. “The network will significantly increase the likelihood that promising therapeutic interventions in FSHD can be translated into clinical trials for patients. We are also thrilled that the CTRN will ensure the patient voice is heard and addressed throughout the drug development process via its Patient Engagement Circles.”

Research conducted by the CTRN includes a longitudinal natural history study, which “will help to establish the variability and rate of progression of FSHD in patients,” said Jeffrey Statland, assistant professor of neurology at University of



**Kathryn Wagner, MD, at the Kennedy Krieger Institute.**

Kansas Medical Center. “This information is crucial in clinical trial design, as it allows the estimation of the study sample size and the power to detect a predicted therapeutic effect size.”

The Clinical Trial Research Network members include:

- Rabi Tawil, MD, professor of neurology, pathology, and lab medicine, and head of the Fields Center for FSHD Research

at the University of Rochester Medical Center;

- John Kissel, MD, professor of neurology and chairman of the Department of Neurology at the Ohio State University Medical Center;
- Kathryn Wagner, MD PhD, director of the Center for Genetic Muscle Disorders at the Kennedy Krieger Institute and associate professor of neurology and neuroscience at the Johns Hopkins School of Medicine;
- Jeffrey Statland, MD, assistant professor of neurology at University of Kansas Medical Center in Kansas City;
- Nicholas Johnson, MD, assistant professor of neurology, pediatrics, and pathology at the University of Utah;
- Perry Shieh, MD PhD, associate professor of neurology and director of the neuromuscular fellowship program at the University of California, Los Angeles;
- Leo Wang, MD PhD, assistant professor in the Department of Neurology at the University of Washington Medical Center in New York. [FSHWatch](#)

## FSH Society launches FSHD Biospecimen Registry

### RESOURCE MATCHES TISSUE DONATIONS TO RESEARCHERS WHO NEED THEM

The FSH Society has launched the FSHD Biospecimen Registry, the first nationwide FSH muscular dystrophy tissue registry. The registry, created by the FSH Society in partnership with the National Disease Research Interchange (NDRI), establishes a way for patients to arrange to donate tissue in advance of surgery and after death, and ensures that the tissue will be delivered to qualified FSHD researchers.

“Muscle and other tissues from FSHD patients and relatives are truly precious and vitally important for the advancement of research,” said Daniel Perez, president and CEO of the FSH Society. “Solving FSHD begins with the courage of individuals to donate their tissues to advance science. This registry allows patients to donate such tissue. Without sufficient access to patients’ tissues and cells, scientists will not be able to fully understand the biological mechanisms of FSHD and test strategies to treat the condition.”

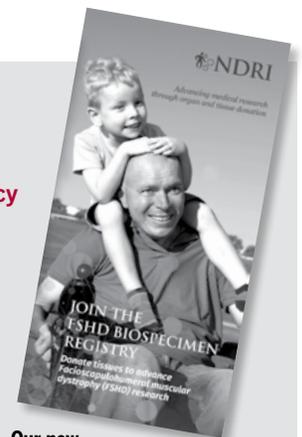
“NDRI is proud to partner with the FSH Society on the launch of the FSHD Biospecimen Registry,” said Bill Leinweber, president and CEO of NDRI. “We are confident this registry will serve as a critical resource to support the advancement of research on FSHD.”

Individuals affected by FSHD and their unaffected family members of all ages are encouraged to donate by registering with NDRI’s Private Donor Program. The Private Donor Program

gives individuals and their families an opportunity to leave a meaningful legacy for their loved ones by providing a simple system through which tissues can be donated for research. NDRI works with patients and their families to obtain authorization for donation of tissues from surgery or postmortem, arranges the recovery, and provides the tissues to approved researchers.

Tissues collected for FSHD will be provided to registered and approved FSHD researchers, who will have access to health information and donated tissues, as well as detailed clinical and genetic information to observe the presence of disease, thus providing targets for the development of new therapies. Personal identifying information about the donor is removed from the medical data to protect the individual’s privacy. Needed specimens include facial muscles, shoulder girdle muscles, arm muscles, hip flexors, back muscles, leg muscles, abdominal muscles, pectoral muscles, diaphragm, cochlea (inner ear), and retina.

For more information about the FSHD Biospecimen Registry, visit <https://www.fshsociety.org/donate-tissue/>.



**Our new brochure about the tissue donation registry.**

# FSH Society commits over \$1.36 million to research in 2016

*Grants approved from February 2016 cycle set new record*

by **JUNE KINOSHITA**  
FSH Society

The FSH Society has committed \$648,774 in funding to five research projects that aim to break new ground in the search for a treatment and cure for FSHD. These grants bring the total research funding committed by the FSH Society in 2016 to **\$1,383,892**, a 46 percent increase from 2015 and a new record for the Society.

“These grants reflect the high quality and ambition of grant applications submitted to the FSH Society,” said Daniel Perez, president and CEO of the FSH Society. “None of this would be possible without the past year’s growth in the Society’s revenue, fueled by the generosity of donors at every level and the hard work of FSH Society staff, Board members and fundraising volunteers.”

The following proposals submitted in February 2016 were approved:

► **CRISPR APPROACHES TARGETING DUX4 IN VIVO**

**Peter L. Jones and Robert Bloch, University of Massachusetts Medical School, Worcester**  
\$240,014 for two years

► **NOVEL ROLE FOR REDUCED RNA QUALITY CONTROL IN FSHD PATHOGENESIS**

**Sujatha Jagannathan, Fred Hutchinson Cancer Research Center, Seattle, Washington; Robert Bradley and Stephen Tapscott, mentors**  
\$59,225 for one year

► **STUDY OF THE UNEXPECTED CYTOPLASMIC FUNCTIONS OF DOUBLE HOMEODOMAIN PROTEINS DUX4 AND DUX4C DURING DIFFERENTIATION: FOCUS ON HEALTHY AND PATHOLOGICAL MUSCLE CELLS**

**Eugénie Anseau, University of Mons, Belgium; Frédérique Coppée and Alexandra Belayew, mentors**  
\$124,807 for one year

► **ASSESSING THE PATHOLOGIC ROLE OF DUX4 IN A HUMANIZED MOUSE MODEL OF FSHD**

**Amber Mueller, University of Maryland, Baltimore; Robert Bloch, mentor**  
\$134,728 for three years

► **MICRORNAS AS POTENTIAL MODIFIERS OF FACIOSCAPULOHUMERAL MUSCULAR DYSTROPHY**

**Nizar Saad, Nationwide Children’s Hospital, Columbus, Ohio; Scott Q. Harper, mentor**  
\$90,000 for one year

With these awards, the Society continues to significantly expand funding and the search for treatments and a cure for FSHD, a disease that impacts more than 870,000 individuals worldwide.

In 2015, the Society funded a total of \$949,221, a 16 percent increase over total funding in 2014.

For full details and project summaries on the FSH Society’s grant awards, please visit <http://www.fshsociety.org/funded-grants/>. 



**Robert Bloch**



**Amber Mueller**



**Nizar Saad**



**Jones Lab**



**Sujatha Jagannathan (at left)**

## Q&A with Dr. Rabi Tawil, Part 2

About the FSHD Care Guidelines

This is a continuation of our Q&A session with Rabi Tawil, MD, co-director of the MDA Neuromuscular Disease Clinic at the University of Rochester, New York, and lead author of the first evidence-based care guideline for FSHD, a project the FSH Society supported through consultations and advocacy. Summaries of the guideline can be downloaded at <https://www.fshsociety.org/resources/>.



Rabi Tawil, MD

Part 1 of our session was published in *FSH Watch* Issue 1, 2016.

**Q:** I was wondering if you could give us some options that are available for FSHD Type 1 women who may be interested in starting a family?

**Dr. Tawil:** One of the difficulties with FSHD is that the genetic test for FSHD needs a lot of DNA, and so this precludes doing what we call pre-implantation genetic diagnosis (PGD), except in certain situations.

A pre-implantation diagnosis is when you can take eggs from the female and fertilize them in vitro, and grow the embryos to the eight-cell (day 3) stage. You can then take one cell from each embryo and test it, and you'd be able to determine if that very early embryo has FSHD or not. You can then implant the FSHD-free embryos into the mother, and you can be certain that the baby won't have FSHD.

The problem is that you cannot do standard genetic testing on DNA from just one cell, but you can do it in a different way. You can do PGD based on what we call linkage, and it's a little bit complicated to explain, but for that to happen, you need multiple members of the family who have FSHD—and preferably from more than one generation—to donate blood to get tested, and then by looking at the pattern of the DNA of all the affected family members, you can figure out, based on DNA from one cell, what the probability of that embryo is to develop FSHD. So in FSHD, to do pre-implantation genetic diagnosis, the bottom line is that it only works if you have multiple affected members of the family in more than one generation that can be tested.

**Q:** I understand that the care guidelines are based on FSHD Type 1 (FSHD1). Would it make sense for an FSHD Type 2 (FSHD2) patient to share the guidelines with my care team?

**Dr. Tawil:** Again, the problem is that FSHD2 is really kind of a new type of FSHD that's only been known for the last six or seven years. I think we know that both FSHD Type 1 and 2 eventually have the same mechanism; they're caused by the same thing, even though they're genetically different. Both of them are caused by the expression of DUX4 in muscle cells. What we know is that, in general, FSHD2 looks very much like FSHD1.

What I have not seen in FSHD2 myself or have seen published anywhere are some of the more severe forms of FSHD, like

infantile-onset FSHD, which you see with FSHD1. I don't think it has been reported in FSHD2. Similarly, the complications of hearing loss and retinal vascular disease have not yet been reported in FSHD2.

So I think the care guideline recommendations, by and large, apply for both Types 1 and 2. Some of the predictors—for example, the size of the D4Z4 deletion—obviously do not apply to FSHD2. There may be certain factors that we're going to be able to measure, and which we're looking at now, that may tell us if somebody is more predisposed to getting more severe disease with FSHD2. This has to do with the methylation levels of the DNA. The methylation level basically reflects how tightly bound the DNA is around the D4Z4 repeat; the less tightly bound it is, the more likely you're going to get DUX4 expression. The more tightly bound it is, the more it's suppressed, and so we're looking at identifying the best marker for methylation in FSHD2 and seeing if it may be helpful in making a prognosis in patients who have FSHD2. But again, in general, as far as recommendations like exercise and everything else, I think the guideline recommendations apply for both types of FSHD.

**Q:** Are cardiac arrhythmias and atrial flutter typically related to FSHD, and what's their significance?

**Dr. Tawil:** It's very hard to tease out from the reports of FSHD patients who have these arrhythmias whether they have them because of other cardiovascular risk factors, or whether the cardiac issues are connected to FSHD. But if there's anything that happens with FSHD, it's some of those atrial arrhythmias, and they're usually very benign.

Now, atrial fibrillation is not necessarily benign, because it can predispose to clots, and so somebody who has chronic atrial fibrillation may need to be on anticoagulation drugs. But again, I don't think that there's enough evidence from what we've looked at to suggest that people with FSHD need to be screened for these arrhythmias because most people will have symptoms if they have atrial fibrillation or atrial flutter. Again, while there are potentially dangerous arrhythmias in the long run, they're not as severe as some ventricular arrhythmias.

**Q:** Do you anticipate any blood tests that preclude the need for a muscle biopsy? I think maybe the underlying question really is, Why is the muscle biopsy still being done often as part of the diagnostic workup when there is a genetic test that would definitively tell you if you have FSHD?

**Dr. Tawil:** There is a flow chart in the actual journal article of the guidelines that shows that there's no need for a biopsy. The only time you would do a biopsy is if people looked like they have FSHD, and you do all the genetic testing, and it comes back negative; then you have to do a biopsy to help guide the physician as to where to go next as far as figuring out what you have. So the

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# She sells stem cells by the sea shore

*Muscle biology conference highlights progress in FSHD*

by **AMANDA RICKARD**  
San Diego, California

Attending a muscle-focused scientific conference situated among the pine and oak trees on the Monterey, California, seaside is as close to paradise as is imaginable for a young researcher. This year’s “Frontiers in Myogenesis” meeting at the Asilomar Conference Grounds, presented by the Society for Muscle Biology (<http://www.musclebiology.org/>), was focused on “Molecular Mechanisms Modulating Skeletal Muscle Development and Homeostasis in Health and Disease.”

The FSHD field had its moment in the spotlight when Stephen Tapscott, MD PhD, from the Fred Hutchinson Cancer Research Center in Seattle, Washington, spoke to attendees about the progress made so far in furthering our understanding of the causes and effects of facioscapulohumeral muscular dystrophy (FSHD). His outlook was optimistic as he discussed his thoughts on major therapeutic options, which focus on attacking DUX4, the gene believed to be the linchpin in causing FSHD. These ideas include:

- preventing the genetic code for DUX4 in DNA from being transcribed into DUX4 RNA (the form of genetic material that gets translated into toxic Dux4 protein);
- activating mechanisms to destroy DUX4 RNA; and
- interfering with the cell death and immune responses that DUX4 elicits in diseased muscle.

The first step toward testing treatments in patients is to use cells growing in a laboratory (“FSHD-in-a-dish”) to identify potential therapies. My work at Genea Biocells in San Diego, which I presented at the conference, includes identifying and tracking “disease phenotypes,” which distinguish FSHD-affected cells from healthy ones. At Genea, we use muscle cells made from human FSHD embryonic stem cells donated by patients who have undergone pre-implantation genetic diagnosis (a technology used during in vitro fertility treatments to select for embryos that don’t carry the FSHD mutation).

Phenotypes of FSHD include DUX4 expression (production of DUX4 RNA and protein) and changes in the gene expression, shape, development, and lifespan of mature muscle cells. Our specialty is “high-content screening” (thousands of microscopic wells filled with cells to test many conditions) to identify small molecules that change disease phenotypes to normal levels. This is research we are actively carrying out now using our FSHD-in-a-dish model.

At Asilomar, research on differences between control and FSHD cells was also presented by muscle stem cell expert Peter



**Asilomar Conference Center.**

Zammit, PhD, of King’s College London, who reported that important signaling pathways known to be required for normal muscle development are perturbed in FSHD. Zammit found these changes when he sifted through five different studies that have looked for gene expression differences in FSHD, and he proposed a mechanism by which these changes influence the repair of FSHD muscle and its sensitivity to oxidative stress.

Once we have identified a molecule that restores our FSHD cells to a healthy state in a dish, we’ll need to test the therapy in an animal model of the disease. There is some difficulty there, as Tapscott discussed, because DUX4 DNA does not exist naturally in the animals that science would typically use for a disease model (such as fruit flies, wasps, mice, rats, zebrafish, etc.).

However, researchers are working hard to improve FSHD animal models. Peter Jones, PhD, and Takako Jones, PhD, from the University of Massachusetts, Worcester, presented a poster on a “tunable” DUX4-based FSHD mouse model. They’ve designed mice that make human DUX4 in response to a chemical switch, such that higher doses of the chemical result in more DUX4 expression. Because DUX4 activity and disease severity are quite variable in FSHD patients, the Jones model is an attractive one for therapeutics development, since a scientist can turn DUX4 up or down depending on the needs of each experiment.

Also presenting data at Asilomar were Charles Emerson Jr., PhD, and Meng-Jiao Shi of the University of Massachusetts Medical School. They showed that the Genea muscle-making method can be used to

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## Chasing after a loan

*Dogged persistence gets the largest US bank to change its policy*

by **KEN COLOSI**  
Mentor, Ohio

Just when you think that society has evolved regarding people with disabilities, you come across a prehistoric dinosaur lurking in the shadows.

A year ago September, I purchased a mobility scooter in order to be able to enjoy parks, museums, and other venues that require walking long distances. After months of having my wonderful girlfriend, Sue, assemble and disassemble the scooter to fit into my SUV, I decided that it was time to get an accessible minivan with a ramp to make things easier, as well as allow me to use the scooter at work.

I purchased the vehicle from a fantastic company, Mobility Works, in Akron, Ohio. As many of you know, these converted vehicles are not inexpensive, even when they are a few years old like mine was. I looked to my left, then looked to my right, and LeBron was not around to help me pay for it, so I decided that I would need to get an auto loan for the half that I could not pay for up front.

I have accounts and credit cards through Chase Bank, so I decided to start there for a loan because they had a reasonable rate. I filled out the online application, and they came back and offered me several thousand dollars less

than the requested loan amount—and at almost double the interest rate I had applied for. I immediately called them to inquire why I did not get what I asked for. They told me that I was asking for much more money than a 2012 Dodge Grand Caravan was worth.

Their mistake was understandable. Nowhere in the application did it state that this was a handicap conversion vehicle, which basically doubles the price and value of the vehicle. I thoroughly explained this to them, and told them they could find the true market value on several different websites that



Ken Colosi

advertise these vehicles.

The woman told me she would have to speak with her manager and put me on hold. After a few minutes she came back and said, “I’m sorry, we do not include the additional conversion costs when evaluating the worth of a vehicle, just the value of the minivan itself. Is there anything I can help you with today?”

I was absolutely stunned. “That’s it? You are going to just dismiss me like that?” She reiterated to me that this was Chase’s policy, and there was nothing she could do. You may as well have waved a red flag in front of my bullheaded face. By the way, while I was on hold with her, I filled out an application with Pentagon Federal Credit Union, and after explaining the identical situation to them, they approved my loan in less than 30 minutes (at a better rate).

Now I was on a mission! I was right, and Chase was wrong. I called their customer

blah.... They get really irritated when you accuse them of discrimination.

I called the first guy back again and told them their response was unacceptable, but after conferring with his bosses, he assured me that their policy was not discriminatory. Time to escalate. Federal Consumer Financial Protection Bureau, here I come! I filed an official complaint online through this federal agency. Eventually, Chase responded to it by saying the loan was handled correctly and that there was no discrimination. I learned that the Federal Consumer Financial Protection Bureau does not induce fear and panic in large corporations and really has little power to make anyone change anything.

Time to call corporate headquarters again and speak to the next level up. I told them I was not going away until they recognized their obvious fault with their lending policy and changed it. I may have berated them that they were “too big to fail and too big to care.”

Surprisingly, on one of my many follow-up calls, the woman told me that they were going to review the policy at the corporate level with the counsel of their legal department. Weeks and months passed as I occasionally called on them to check their progress. Yes, I can be a real pain sometimes.

Then one day I received a letter from Chase in the mail. It stated, in part, “We apologize for your experience. Since your loan

application, Chase has enhanced its valuation process for used vehicles with mobility items, and we invite you to reapply.”

Wow! Finally. After months of stating the obvious to the largest bank in the United States and the sixth largest bank in the world, it took an unbelievable amount of persistence to get them to consider just one of the many challenges handicapped people face every day and to do the right thing. I realize this is not going to change the world, but it is one more small step in the right direction. FSHWatch

“After months of stating the obvious to the largest bank in the United States and the sixth largest bank in the world, it took an unbelievable amount of persistence to get them to consider just one of the many challenges handicapped people face every day and to do the right thing. I realize this is not going to change the world, but it is one more small step in the right direction.”

—KEN COLOSI

service number first and got nowhere. Next, I called their corporate offices and pleaded my case there, to someone who obviously had no real authority but at least pretended to care. I was told that my loan had been handled correctly according to their policy. They still didn’t get it. “Your policy is wrong. It discriminates against the handicapped.”

I received a response in the mail that my loan was handled correctly and that Chase does not discriminate against any person based on race, religion, color, blah blah,

# Fulcrum Therapeutics takes aim at FSH muscular dystrophy

Cambridge start-up launched with \$55 million

by JUNE KINOSHITA  
FSH Society

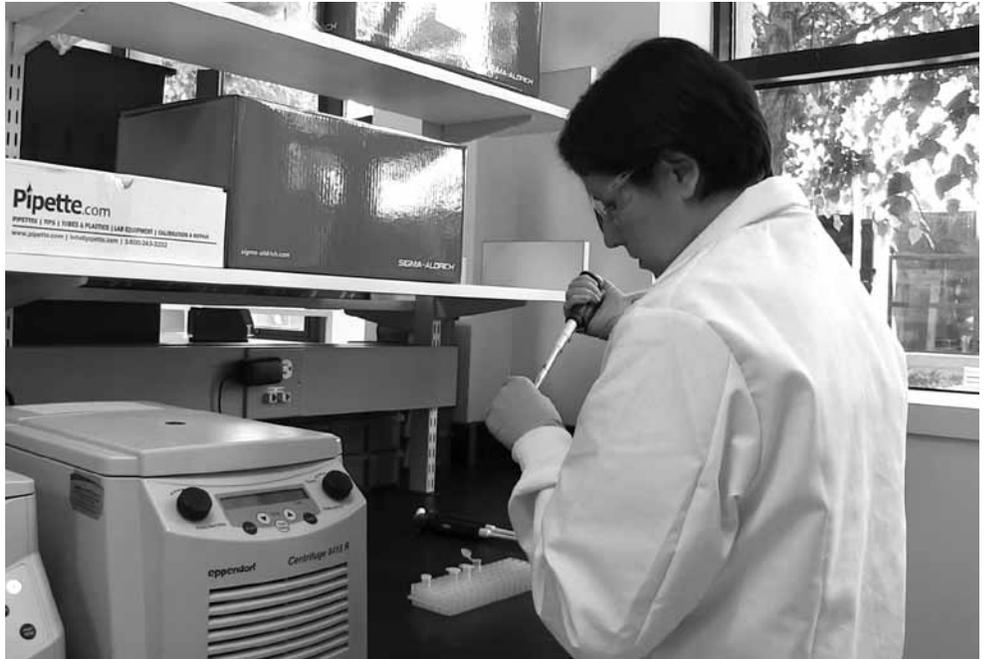
The latest addition to a growing number of biotechs with therapy-development programs targeting FSH muscular dystrophy is Fulcrum Therapeutics, a Cambridge, Massachusetts-based company that was launched in July with \$55 million in funding from Third Rock Ventures, LLC. Fulcrum’s strategy is to focus on “unlocking gene control mechanisms to ... discover and develop small molecules that modulate the on/off control mechanisms that regulate genes,” according to the company.

Fulcrum will initially focus on two severe genetic diseases—FSHD and Fragile X syndrome (FXS). What the two diseases have in common is that each arises from a gene mutation that causes an error in gene regulation. In FSHD, muscle damage results from the activation of a gene called DUX4 that should be silent in adulthood. In the case of Fragile X, the cell is unable to make a protein required for brain function when the FMR1 gene is repressed.

Fulcrum is led by president and chief executive officer Robert J. Gould, PhD, former president and CEO of Epizyme, and a long-time leader in drug discovery and development.

“One of the single most important biologic breakthroughs of the last decade has been the unraveling of gene regulation at a molecular level. For more than a decade we have understood the genetic code—the genome. Now that we are unraveling the way these genes are regulated and put to work in biology, there is an unprecedented opportunity for drug development,” Gould said. “We are excited to build Fulcrum Therapeutics—a company that is capable of translating these discoveries into groundbreaking new therapies that restore balance in patients who currently have no other therapeutic options.”

Fulcrum’s product engine was created to integrate and accelerate the rapid advances that have reshaped a wide range of biological disciplines, including transcription biology, stem cell biology, and computational biology. The integration of these diverse, advanced technologies



Fulcrum Therapeutics is up and running in Cambridge, Massachusetts.

throughout the drug discovery and development process creates the opportunity to deliver on the promise of genomic medicine, namely, to not only discover genetic disease drivers but also to regulate gene expression to restore health.

Fulcrum models gene regulation in disease tissue using patient cells that are

discoveries are brought together with publicly available gene regulatory data to create genomewide maps of gene regulation that enable rapid identification of drug targets for the activation or repression of disease genes.

The FSH Society has worked with Fulcrum’s founders over the past several years to share knowledge about the FSHD

“One of the single most important biologic breakthroughs of the last decade has been the unraveling of gene regulation at a molecular level. For more than a decade we have understood the genetic code—the genome. Now that we are unraveling the way these genes are regulated and put to work in biology, there is an unprecedented opportunity for drug development.”

ROBERT J. GOULD

either donated through tissue biopsy or derived from skin cells using the technology of induced pluripotent stem cells. It uses screening tools such as CRISPR/Cas9 and chemical probe libraries to dissect gene regulatory mechanisms in cellular models of diseases. These

research community, patients, and clinical trial readiness. The company’s decision to target FSHD is a powerful validation of the Society’s decisions to invest in identifying the genetic mechanism, developing cellular and mouse models, and advancing clinical trial outcome measures. FSHWatch

# Antisense technology muzzles DUX4

Tested in human cells and mouse xenografts

by **JUNE KINOSHITA**  
FSH Society

The cause of facioscapulohumeral muscular dystrophy (FSHD) is thought to center on DUX4, a gene that normally is silent in adult skeletal muscle. When DUX4 gets “expressed,” as happens in FSHD, it activates other destructive reactions in muscle cells and causes muscles to degenerate. Suppressing DUX4 is a logical strategy to treat FSHD, and a new study suggests that this may be achievable using “morpholino antisense oligomers,” a type of synthetic molecule that blocks specific DNA sequences.

Morpholinos are being tested in experimental treatments for other diseases, including spinal muscular atrophy, Duchenne muscular dystrophy, and myotonic dystrophy.

The new FSHD study was the result of a collaboration between academic scientists and Genzyme. The academic researchers are members of the NIH-funded Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center for FSHD and work at the University of Massachusetts Medical School in Worcester, as well as the Kennedy Krieger Institute and Johns Hopkins School of Medicine, both in Baltimore, Maryland.

FSHD results from a perfect storm of things that go awry in the genetic machinery that normally keeps DUX4 safely under lock and key. These



Genzyme offices in Cambridge, Massachusetts.

include the loss of D4Z4 repeat units on a section near the tip of the long arm of chromosome 4, the presence of a “permissive” poly-A haplotype that allows the DUX4 gene to get transcribed into messenger RNA, and hypomethylation of the D4Z4 units. All of these conditions have to be present in order for FSHD to occur in an individual.

The researchers tested various

morpholinos and found that one, code named FM10, which targets the poly-A site, was strikingly effective in knocking down DUX4 expression. They also looked at several genes that are activated by DUX4, such as ZSCAN4 and MBD3L5, and found that their levels were also very low, confirming that DUX4 activity had been suppressed.

In addition, the investigators checked to see that FM10 did not result in potentially detrimental “off target” effects on other genes.

These experiments were done in cells derived from 11 FSHD patients and six first-degree, genetically related, unaffected relatives, as well as in mice that had been engrafted with human FSHD muscle (see “Human Muscle Grows in Mice,” *FSH Watch* Spring 2014). The study acknowledges the FSH Society for assisting with recruiting volunteers to donate muscle biopsies used in the research.

Future work will address whether morpholinos can achieve DUX4 knockdown when given systemically and longer term to a whole animal. Stay tuned. [FSH Watch](#)

## Reference

Chen JC, King OD, Zhang Y, Clayton NP, Spencer C, Wentworth BM, Emerson CP Jr, KR. Morpholino-mediated Knockdown of DUX4 Toward Facioscapulohumeral Muscular Dystrophy Therapeutics. *Mol Ther*. 2016 Jul 5. doi: 10.1038/mt.2016.111.

## Q&A WITH DR. RABI TAWIL, PART 2

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first approach is that you don't need a muscle biopsy to make a diagnosis of FSHD.

**Q:** If there is breakdown of muscle tissue going on in FSHD, does that put a strain on kidneys and other organ systems, and should there be some kind of therapy to support those systems that are being stressed by muscle breakdown?

**Dr. Tawil:** I think that the person is referring to what happens in people who have generalized breakdown of their muscle in what we call rhabdomyolysis. That's when many muscles break down all at the same time, and the muscles leak enzymes and myoglobin proteins which can cause kidney damage. At any one time,

the actual muscle damage is not very high in FSHD, and it's reflected by the CPK enzyme level. Most people with FSHD have either normal or slightly abnormal CPKs, in the 500 to 1,000 range. Those levels don't result in any kidney problems. It's the people who have CPKs of 20,000 to 100,000 who get into trouble.

**Q:** There are some patients who have very severe atrophy, and keeping weight on is a challenge for them. What do you recommend for a patient who just can't put on weight and needs to?

**Dr. Tawil:** I think it's a balance. You don't want to put on too much weight. The question is, Are people who are very thin, who have FSHD

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## Our first meeting

*FSHD folks gather in the Windy City*

by **BRANDI LUKAS**  
*Village of Lakewood, Illinois*

It was such an honor and a pleasure to meet the FSH Society members who attended the first ever meeting in Chicago on July 10. We hope they all enjoyed our time together as much as I did. We're grateful that everyone took the time out of their Sunday to share and connect with us. I am a better person for having met them all, and I cannot wait to see how we change the face of the FSHD community and grow our group.



We collected and shared the phone numbers and emails of the attendees, along with a little snippet of information about each person. We'd love to hear from others in the Chicago area who would like to join our group.

We also created a Facebook group so that our group can stay in touch. As we grow and evolve, the group will grow with us, and we can add new members, distribute information, and stay connected. Look us up on Facebook at "FSHD - Chicago Support Group" (<https://www.facebook.com/groups/601819619993442/>) and request to be added to the group. We encourage you to invite other members to the group as you encounter new people with FSHD, doctors, PTs, friends, or family that are supportive and interested.

Moving forward, my husband David and I will be spearheading



**Our kickoff meeting in Chicago!**

our local gatherings with support from the FSH Society home office in Boston, and with any others in the Chicago area who would like to help. We are already working on our next meeting and will keep in contact as things come together to solicit ideas, dates, and such.

Please, please, please know that your voices are important, and we welcome your thoughts and ideas, and we always love to help! Don't ever hesitate to reach out to us for any reason. Together we can make a difference! **FSHWatch**

**Editor's note:** Contact Robyn O'Leary at the FSH Society at (781) 301-6042 or [robyn.oleary@fshsociety.org](mailto:robyn.oleary@fshsociety.org) to join our group.

## Stem Cells 101 for FSHD research

*San Diego member support group meeting*

by **AMY BEKIER**  
*San Diego, California*

On July 30, 2016, twenty-five FSHD patients, family members, and scientists assembled at Genea Biocells in La Jolla, California, to hear presentations by senior scientists Amanda Rickard and Anabel de la Garza. Their work is aimed at identifying future treatments for FSHD patients.



The company's website states that "Genea Biocells developed the world's first human stem cell model of FSHD which shows cellular hallmarks of the disease. We pursue a small molecule approach to find therapeutic drug candidates that slow, stop, or reverse the disease-related phenotype in skeletal muscle. This project has been supported by

the FSHD Global Research Foundation and Friends of FSH Research.

"Genea Biocells is a neuromuscular disease-focused discovery-stage company using proprietary human pluripotent stem cell technologies. Genea Biocells also provides contract research services to pharma and supplies reagents to strategic academic collaborators to expand their capabilities and further validate their technologies. Genea Biocells has one of the world's largest banks of pluripotent human embryonic stem cells and developed the world's first consistent, scalable, and high-yield differentiation process for functional skeletal muscle cells."

Rickard offered a detailed and engaging explanation of how human embryonic stem cells have been developed into an "FSHD-in-

a-dish" cellular model, and how this system is used to find potential therapies for FSHD. De la Garza presented a review of techniques for human muscle stem cell transplantation, which could potentially present a customized treatment for each FSHD patient.

We all enjoyed a tour of the lab after a lunch hosted by the FSH Society. Thank you to Genea Biocells, and especially to Amanda and Anabel for providing us with such a fascinating and educational afternoon!

The presentations are available on the FSH Society's YouTube channel at <https://www.youtube.com/watch?v=AG-YzAZYDSg>. **FSHWatch**

**Editor's note:** Amy Bekier is a member of the FSH Society Board of Directors and secretary of the FSH Society.

## An exercise therapy targeting individual muscles

Michigan members learn about the work of a “muscle whisperer”

by **AMY TESOLIN-GEE**  
Midland, Michigan

Several FSHD patients, family members, and FSHD-experienced clinicians came together in Ann Arbor on June 4, 2016, for an educational meeting including lots of helpful, informal discussion time. Many people traveled a good distance, and some drove from as far away as northern Michigan and Columbus, Ohio.



Lynnette Rasmussen and Laura Damschroder shared invaluable lessons learned during their four-year experience working together—Laura, as a patient with FSHD, and Lynnette as a certified Pilates and occupational therapist. Their experience has revealed an effective approach to finding muscles Laura had thought were lost. Lynnette has explored ways to target lost muscles to maximize Laura’s independence and mobility.

After working with Lynnette just a handful of times myself, I am inclined to describe her as a muscle whisperer. She combines her 40 years of experience with a keen ability to tune in to her client’s individual needs, effectively tailoring therapies to meet them.

Laura, an internationally known researcher in her field of healthcare implementation science, loves traveling the world and maintains a very active lifestyle in spite of FSHD. She stresses the importance to everyone of moving even just a little bit. For example, start with walking slowly on a treadmill or around your living room for five minutes each day—or whatever you can do—then gradually work up from that.

Through weekly half-hour sessions with Lynnette, Laura has gained subtle increases in strength, particularly in muscles she thought were gone. It should be noted that Lynnette’s approach differs from traditional physical therapy regimens where large muscle groups may be targeted at once. Her approach with Laura involves working the severely affected muscles, such as hamstrings, with subtle and focused exercises to bring them back to life. Even such small functional increases in these and other muscles deemed lost have led to noticeable improvement in Laura’s balance.

Besides exercises, Lynnette uses kinesio tape to stabilize and activate muscle groups. Laura noted that sensory feedback she received from the taping of her hamstrings helped her feel more grounded when she left her workout sessions. It is thought, Lynnette explained, that the action (coils) of the tape help to recruit the various muscles of the taped area.

Laura shared that in 2010 a doctor recommended she get a scooter, and all indications were that she would soon need a wheelchair. However, she has remained stable since working with Lynnette over the past four years, even improving measurably on some of her muscle strength assessments during annual neurology visits. It is important to note that even though the measured improvements in her large-scale strength assessments have been subtle, she feels and functions considerably better than she did in 2010, and her disease progression appears stable. [FSH+Watch](#)

To learn more, visit the FSH Society’s YouTube channel (<https://www.youtube.com/user/FSHSociety>), where we have posted a video of Lynnette’s presentation.

Double the fun with Michelle and Dave!



This summer, FSH Society Board member Michelle Mackay and her husband Dave offered an exceptional matching gift challenge to thank supporters of their Lake Party. After pledging a dollar-for-dollar match for every gift donated during the campaign, they then upped the ante and pledged \$2 for every \$1 given by a first-time donor! The campaign resulted in a total of \$120,427.50 for the FSH Society. Talk about making a splash! We are so deeply grateful to the Mackays for their generosity and leadership, and to all who responded to the challenge.

## SHE SELLS STEM CELLS BY THE SEA SHORE

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generate FSHD muscle cells from induced pluripotent stem cells (iPSCs) that are derived from adult cells donated by FSHD patients. They took these FSHD-affected muscle cells and transplanted them into mice, where the researchers could measure DUX4 expression and see evidence of its downstream effects, including expression of its target genes. This mouse allows us to see exactly how DUX4 expression changes human muscle in a live organism, making it a great resource for FSHD studies and for testing treatments.

Overall, I was very glad to learn about a variety of topics related to muscle development and muscle disease, meet some of the top-tier scientists who study FSHD, and have a chance to present my work to a curious and thought-provoking audience. It is wonderful to be reminded both of the progress made so far in understanding FSHD pathogenesis and the many outstanding scientists who are dedicating their lives to finding a treatment or cure for muscle disease.

**Editor’s note:** Amanda Rickard is a senior scientist at Genea Biocells, Inc., a San Diego-based biotechnology company specializing in using disease-affected embryonic stem cells for therapeutics discovery. [FSH+Watch](#)

\* The FSH Society has helped to seed and initiate projects by Emerson, Jones, and Zammit.

## My sitpoint of view

*Reflections on the first Miami patient meeting*

by **ORA PRILLELTENSKY**  
Miami, Florida

On Sunday, May 15, 2016, we held the first South Florida meeting for individuals and family members affected by FSH muscular dystrophy. My husband Isaac and I were pleased to host this meeting at our home in Coral Gables. Eleven people were in attendance including June Kinoshita, executive director of the FSH Society, who traveled to Miami especially for this meeting.

We were a diverse bunch: women and men, young and not so young, from various professional backgrounds and having varying levels of impairment due to FSHD. Some of us were diagnosed long ago, while others have recently learned that they have FSHD. Despite these differences, and the fact that most of us had never met before, two hours seemed to fly by. We introduced ourselves and shared our FSHD journeys, stories that spoke of struggles and challenges but also strength and resilience.

People also shared practical information regarding providers, equipment, and ways of addressing challenges. June Kinoshita spoke about the work of the FSH Society and shared some research highlights. The consensus at the end of the meeting was that knowing others who share our diagnosis is an important source of emotional and instrumental support. At people's request, I sent out a list with names and contact details so that folks can keep in touch.

Writing about our meeting for the *FSH Watch* newsletter provides me with an opportunity to share my personal experience and outlook on life with FSHD. I am very interested in well-being for people with disabilities, both as someone who uses a motorized wheelchair due to this condition



**Ora Prilleltensky (center) with her son Matan (left) and husband Isaac (right).**

***There is no doubt that the progressive, at times unpredictable, nature of FSHD can present formidable challenges to well-being. It is also true, however, that it is possible to live a meaningful and productive life, disability and all.***

and as a psychologist and former professor with expertise in the field.

There is no doubt that the progressive, at times unpredictable, nature of FSHD can present formidable challenges to well-being. It is also true, however, that it is possible to live a meaningful and productive life, disability and all. Savoring positive experiences, counting our blessings, and cultivating meaningful relationships with others are all conducive to well-being. So is contributing to the life of others and involving ourselves in something we are passionate about and which builds on our strengths. There are losses and hardships, for sure, but also opportunities for deep relationships, meaning-

ful endeavors, and good times.

The main goal of the FSH Society is to raise funds for research that can one day lead to a cure. In the meantime, there is a lot that can be done to improve quality of life for people living with FSHD and other disabilities. Attitudes and behaviors are important, but so are life circumstances and access to resources and supports.

My main blessing in life is my family: my loving husband and best friend Isaac, our son Matan, and his wife Elizabeth. I'm also fortunate to have resources that expand the range of options available to me. I am among the 20 percent of individuals with FSHD who require a wheelchair. Mine has seat elevation, which is the only way for me to get out of the chair on my own to perform basic self-care. I also have a wheelchair-accessible home, a reliable van with a ramp, and the ability to pay for needed assistance that is not covered by my insurance.

These are luxuries that most people with disabilities do not have; in fact, people with disabilities constitute the poorest, most disadvantaged minority group in society. I share this because

I believe that activism should also focus on policies and practices that can improve quality of life for people living with FSHD and other disabilities. It is particularly important to target individuals who are unable to pay for services, devices, and other resources that can meaningfully improve the quality of their lives.

Beyond fighting for a future cure, a lot can be done to change lives today. I end this with a quote from the famous anthropologist Margaret Mead: "Never doubt that a small group of thoughtful, committed citizens can change the world. Indeed, it is the only thing that ever has." 

# The magical universe of Belinda Miller

*FSHD hasn't limited author's imagination*

by **EMMA YOUNG**  
Dumfries, Maryland

**B**elinda Miller has created her own world. Filled with magic, wonder, gnomes, mermaids, pirates, fantastical creatures, and abounding adventures, this world comes to life in the author's two-book series, *Phillip's Quest* and *The Ragwort Chronicles*.

*Phillip's Quest*, a seven-book fantasy series geared toward middle-school readers, describes the perils and feats of Phillip, a gnome living in Twistedoak, his best friend, Edward, and Ava Dreamspinner, a dinosaur, as they seek to defeat the Red Witch of Winterfrost Castle.

*The Ragwort Chronicles*, a three-book fantasy series written for a third-grade reading level, describes the Ragworts, a family of warrior gnomes, and their escapades getting to and into Brokenfell, a village near Twistedoak.

"The books are very 'Middle-Earth' [described by Tolkien in *The Lord of the Rings* trilogy] versus 'Narnia' [written in *The Chronicles of Narnia* created by C. S. Lewis]," Miller stated, comparing her series to these classic good-versus-evil tales. "They are values based," with true friendship, loyalty, and courage shown during the characters' adventures, she said, describing her books as "exciting and profound."

Weaving tales has come naturally for the gifted author and Manassas, Virginia, resident. "I've written all my life," Miller said. "It's what I do. I love it. I love literature. I love to read. And I love children's books."

## Facing challenges

Having a passion for writing doesn't mean the actual writing process is easy for Miller. She has facioscapulohumeral muscular dystrophy (FSHD), a progressive neuromuscular disease. Diagnosed at age 22, she explains, "Over the past 43 years, I have watched myself atrophy. It gives me limitations. I don't walk anymore. I'm limited in my arm usage. My arms and hands are very weak. I've had a difficult time pronouncing words. I tire much more quickly, and sometimes, I'm in pain."

Miller outlined the physical process she undergoes to see her fantastical worlds come to life. "My right hand is pretty much gone. I can only use one finger to type," she said. "My left hand is weak, but I can type for maybe a couple of hours. I get cramps in my hand, and



Author Belinda Miller with a feline friend.

***"I want kids to understand the joy of reading and what using the imagination can do. I've done these sorts of things all my life. It was what made me a successful teacher—that I could integrate all the senses."***

BELINDA MILLER

I get tired. It takes me a long time to type." What about modern technologies, such as voice recognition software, that translate the spoken word to text? "Because I don't have a normal speech pattern [due to weakening across facial muscles], it's very difficult for them to translate into the written word," Miller explained.

Yet the irrepressible Miller has always continued to press forward. "[Writing] is what my calling is and what I'm meant to do. I do what I can, when I can," Miller said. "Some days I can go on and on, and some days I'm just not going to move. You have to take the good with the bad.

"I have a life, and I was not going to put that on hold until the disease caught up with me. Until I'm to the point where I totally cannot

do anything, I'm going to do as much as I can. Everyone is dealt certain obstacles," Miller said. "It's up to you whether to feel sorry for yourself. You're either going to do it, or you're going to make an excuse for not doing it. Every day is a challenge, but every day I wake up and do what I can."

## Beyond writing, creating an interactive world

Belinda and her 17-year-old cat, Sambucca Miller, certainly can do a lot. The world she has created extends beyond the written word. A former school teacher with a master's degree in education, Miller witnessed the power of imagination and experiential learning in her classroom. She integrates the senses into her stories and creates treasures that supplement the books, bringing them to life.

Each book includes special recipes related to the story for children and families to try in their own homes, such as Roscoe Ragwort's favorite snicker doodle cookies. "When he saw them at Lilly Willowflower's wedding," writes Miller in the appendix of *The Ragwort Chronicles, Along the Way: Roscoe's Journey*, "he stuffed them in every pocket he could. They were the only thing he had to eat on his first night in the Dark Forest. Don't overbake them, or they'll be as hard as a rock in Bandersnatch Swamp!"

Miller has created action figures and dolls of the characters that can be purchased, giving an opportunity for imaginative play time

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# FSHD Connect Overview

THE FSH SOCIETY'S ANNUAL MEETING FOR FSHD PATIENTS, CLINICIANS, AND RESEARCHERS

<p>Research Connect November 10-11, 2016</p>	<p>Patient Connect November 11-12, 2016</p>	<p>CureFSHD Gala Evening of November 11, 2016</p>
<p><b>The FSH Society's annual international research conference and priority-setting workshop.</b></p> <p>Thursday, November 10 8:00 a.m.–6:00 p.m. Scientific talks and poster sessions.</p> <p>Friday, November 11 8:00 a.m.–12:45 p.m. Research priority-setting workshop.</p> <p><b>Organizers:</b> Daniel P. Perez, President &amp; CEO, FSH Society. Co-Chairs: Stephen J. Tapscott, MD PhD; Silvère van der Maarel, PhD; Kathryn Wagner, MD PhD.</p> <p><b>Who should attend:</b> Basic, translational, and clinical researchers from academia and industry; funding agencies.</p> <p><b>Expected attendance:</b> 100-120</p>	<p><b>Our networking conference brings together patients, families, clinicians, and researchers.</b></p> <p>Friday, November 11 1:00 p.m.–5:00 p.m. A world-class faculty presents the latest in our understanding of genetics, diagnosis, symptoms, progression, clinical trial readiness, therapeutic targets, and ongoing clinical trials. Q&amp;A to learn <i>from</i> patients.</p> <p>Saturday, November 12 8:00 a.m.–5:00 p.m. Patient-focused workshops on a variety of topics on managing FSHD-related health conditions, living and thriving with FSHD, becoming an empowered patient.</p> <p><b>Who should attend:</b> FSHD patients, family members, clinicians, researchers from academia and industry; advocacy organizations.</p> <p><b>Expected attendance:</b> 200-250</p>	<p><b>The FSH Society's inaugural national gala. Cocktail reception, banquet, and musical performance.</b></p> <p>Friday, November 11 6:00 p.m.–10:00 p.m. An imaginative, science-themed evening to celebrate the scientific achievements of the FSH Society and honor the researchers, clinicians, major donors, and patients who are transforming the landscape for FSHD patients around the world.</p> <p>Concert performance by renowned pianist Steven Blier and guest vocalist.</p> <p><b>Who should attend:</b> Patients, families, friends, researchers, and clinicians; leaders in research, medicine, biotech, and philanthropy.</p> <p><b>Expected attendance:</b> 150-200</p>
<p><b>Registration* by Friday, November 4:</b> Academic, nonprofit, government, \$250; graduate student, postdoctoral researcher, \$150; corporate, \$400.</p> <p><b>Register at:</b> <a href="https://www.fshsociety.org/fshevents/2016-international-researchconsortium-workshop/">https://www.fshsociety.org/fshevents/2016-international-researchconsortium-workshop/</a>.</p> <p><small>* Registration fee waived for presenters.</small></p>	<p><b>Registration by Friday, November 4:</b> Per adult, \$245 (member); \$300 (nonmember); per young adult, \$175 (age 12–18); and no charge for children under 12.</p> <p>Family rate for 3+ attendees, \$595 (member); \$660 (non-member). Membership: \$50.</p> <p><b>Register at:</b> <a href="https://www.fshsociety.org/fsh-events/2016-fshd-connect-conference/">https://www.fshsociety.org/fsh-events/2016-fshd-connect-conference/</a>.</p>	<p><b>Early Bird Registration by Friday, October 21:</b> Gala ticket, \$150; VIP ticket, \$350.</p> <p><b>Registration by Friday, November 4:</b> Gala ticket, \$200; VIP ticket, \$400.</p> <p>Table hosts and sponsor deadline for inclusion in event invitation: August 26.</p> <p>Deadline for inclusion in Gala program: October 20.</p> <p><b>Register at:</b> <a href="https://www.fshsociety.org/fsh-events/fsh-society-national-gala/">https://www.fshsociety.org/fsh-events/fsh-society-national-gala/</a>.</p>

# ABLE Act's tax-advantaged accounts for the disabled

*New legislation would raise age of onset of disability*

by **ANN BIGGS-WILLIAMS**  
Brewton, Alabama

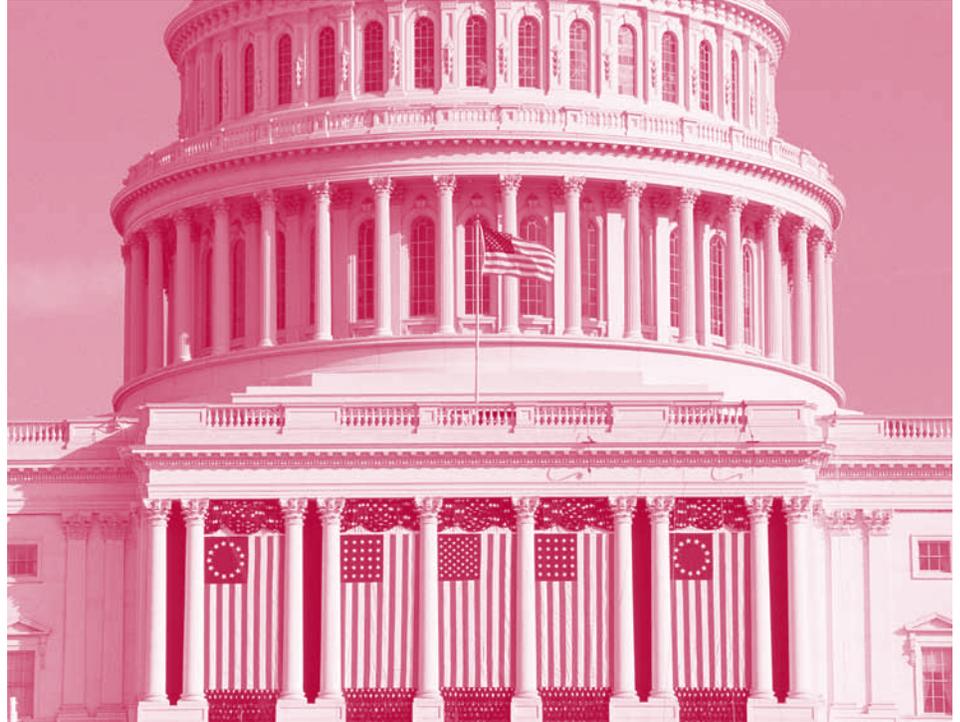
**H**ave you heard about the new ABLE accounts and want to learn more about them? I signed up for a webinar which is currently available online and found it very educational.

The ABLE Act (Achieving a Better Life Experience) of 2014 allowed individuals to establish tax-advantaged savings accounts for the benefit of a disabled family member, similar to college savings accounts. Currently, the age of onset of the disability must be before the age of 26, but now legislation (the ABLE Age Adjustment Act S.2704/H.R.4795) has been introduced in Congress to raise this threshold to 46. This is particularly relevant for many individuals with FSHD, because oftentimes their condition is not diagnosed until they are well past the current cut-off age.

Additional proposed legislation to expand the ABLE Act includes the ABLE to Work Act (S.2720/H.R.4795), which would allow those individuals who are working to save more than \$14,000 each year, and the ABLE Financial Planning Act (S.2703/H.R.4794), which would allow the rollover of 529 educational accounts to a 529A ABLE account.

Costs for items pertaining to "a qualified disability expense" such as education, employment training, an accessible house and transportation, personal assistive services, assistance technology, and healthcare not covered by other plans may be paid for from these accounts. Income earned by these accounts will not be taxed.

The online webinar explores the ABLE accounts now available in several states including Tennessee, Ohio, Florida, and



Nebraska. I was surprised to learn that you can establish an account in a state other than the one in which you live, although the state of Florida does require Florida residency. Present contributions can total up to \$14,000 a year without risking loss of any Medicaid and SSI benefits.

The trio of ABLE Act bills is sponsored by US Senators Richard Burr, R-North Carolina, and Bob Casey, D-Pennsylvania, in the Senate and Representatives Ander Crenshaw, R-Florida; Chris Van Hollen, D-Maryland; and Cathy McMorris Rodgers, R-Washington, and Peter Sessions, R-Texas, in the House of Representatives.

Please consider writing to your sena-

tors and representatives in support of the ABLE Act bills.

To learn more or to view the webinar, visit <http://www.ablenrc.org/>, or contact the National ABLE Resource Center, 1667 K Street, NW Suite 640, Washington, DC 20006, phone (202) 296-2040 or email [info@ablenrc.org](mailto:info@ablenrc.org) (<mailto:info@ablenrc.org>.) You can join an email list to obtain updates, or visit <http://www.facebook.com/theABLENRC> or <https://www.youtube.com/user/theABLENRC> for more information. **FSH Watch**

**Editor's note:** Ann Biggs-Williams is a founding member of the FSH Society Board of Directors.

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## Q&A WITH DR. RABI TAWIL, PART 2

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because there's a lot of muscle wasting, but nutritionally they're eating appropriately? Then I'm not sure that gaining weight in those situations is beneficial for them, especially if they are very weak, because they would have to carry more weight.

But you want to make sure that they're not continuing to lose weight because of what we call a catabolic process. You can do

some blood studies, look at the albumin level, and see if it's appropriate. If somebody's not getting enough nutrition, then it would be reflected in some of the blood tests that you can do. **FSH Watch**

**Editor's note:** We thank Dr. Tawil and dozens of FSH Society members for participating in the live chat.



## World FSHD Day backstory

*Italian brothers' ordeal sparks a global movement*

by JUNE KINOSHITA  
FSH Society

This June 20 saw patients, families, and supporters around the world sharing the World FSHD Day logo across thousands of social media pages to raise awareness of facioscapulohumeral muscular dystrophy.

The campaign extended to the non-virtual world, with FSHD awareness days gaining official recognition in Massachusetts, New Jersey, and the city of Seattle, thanks to the efforts of advocacy groups including the FSH Society, Chris Carrino Foundation for FSHD, and Friends of FSH Research.

Others held events ranging from a canal swim in the Netherlands to a cocktail party in Sydney to thank supporters of the FSHD Global Research Foundation's annual Chocolate Ball.

We'd like to share the backstory of how June 20 came to be World FSHD Day. The suggestion came from our colleague Fabiola Bertinotti of FSHD Europe. Fabi, who lives in Rome, first told us about Marco and Sandro Biviano, two young brothers with FSHD from a family living on Lipari, a 14.9-square-mile island north of Sicily. Their two sisters also are affected.

Unable to obtain proper care for their FSHD, in 2013 the wheelchair-bound brothers traveled to Rome and camped out in a tent in Piazza Montecitorio in front of the Italian Chamber of Deputies to demand change. After 700 days of demonstrations, the Italian Ministry of Health agreed to establish a telemedicine system and home care to enable the Biviano siblings to receive medical care in their remote village. The system is being offered to all FSHD patients in Italy who are "machine dependent."

The government also established June 20, 2015, as the first FSHD Day in Italy in honor of the birthday of the mother of the Biviano siblings.

In a statement to the press, Minister of Health Beatrice Lorenzin announced: "The project makes available for the Biviano family the most advanced technology, which is personalized for the various and unpredictable dysfunctions of the nervous system. The system will be controlled by an operations center that will manage 24-hour monitoring of the vital signs and will maintain appropriate levels of medical therapies in the form of medicines and physical therapy. This system represents the most innovative operation supporting people suffering from progressive and slowly disabling illnesses.

"The tent they had assembled in Montecitorio for almost 700 days, which is the symbol of a fight encouraged by unrealistic hopes—this tent is now taken apart; Sandro and Marco can finally go home where, together with their sisters Palmina and Elena, they can receive all the possible therapies, the same therapies that their territory had denied. This is the kind of answer that patients look for."

We salute the Biviano brothers for their incredible perseverance and courage. The idea for World FSHD Day crystallized at the 2015 FSHD Champions annual meeting organized by the FSH Society. We thank Natalie Moss of FSHD Global Research Foundation and Fabiola Bertinotti of FSHD Europe, who worked with us and SHIFT Communications on the design of the World FSHD Day campaign. [FSHWatch](#)



**The Biviano brothers in Rome, Italy, protesting the lack of adequate medical care for people in remote areas with FSH muscular dystrophy.**

## THE MAGICAL UNIVERSE OF BELINDA MILLER

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with a moveable figure. Each comes with a description and is personally made by Miller.

A jewelry line inspired by characters and settings in her fantastical stories is available as well. Graced with beautiful detail, these handmade creations are one of a kind.

The prodigious creator also includes instructions for fun children's crafts based on the book series in an e-newsletter. Simply sign up on her website (<https://belindamiller.me/>).

Miller enlisted the help of Lorraine Gonda, a Las Vegas-based illustrator, to ensure her fantastical world is beautifully depicted throughout her books. From fun crafts and food to taste to beautiful jewelry, action figures, and stunning visuals, the creative process of bringing the books to life seems almost endless. "I want kids to understand the joy of reading and what using the imagination can do," Miller said. "I've done these sorts of things all my life. It was what made me a successful teacher—that I could integrate all the senses."

### The future

Miller is currently hoping to publish a coloring book later this year and is finishing her first adult-audience novel, *The Centurion's Lance*, which chronicles the whereabouts and interesting history of the spear that reportedly pierced Christ's side, according to Biblical accounts. "I'm having a good time with that," Miller stated. "It's fascinating."

Will the irrepressible Miller ever stop? Miller's enthusiasm for life shines through in this response: "I'm doing what I love to do. I love my writing. I love living where I live. I'm happy. Life is good. This is cool."

Both the Phillip's Quest book series and the Ragwort Chronicles book series are available now on Amazon.com, wherever books are sold online, or at Miller's website, <https://belindamiller.me/>. In addition, four books have been translated into Braille. They are available through AIM-VA at <https://aimva.org/>. [FSHWatch](#)

**Editor's note:** Reprinted with permission from *Prince William Living*. Our version was edited for length. Emma Young ([eyoung@princewilliamliving.com](mailto:eyoung@princewilliamliving.com)) is a stay-at-home mother and freelance writer residing in Dumfries. We thank Belinda Miller for donating to the FSH Society \$1 for every copy sold of her Ragwort Chronicles book, *Along the Way: Roscoe's Story*, a finalist for the 2016 Next Generation Indie Book Awards. Be sure to use AmazonSmile when you shop for it!



## Meet our newest Board of Directors members

*Board also elects new secretary*

by **JUNE KINOSHITA**  
FSH Society

The FSH Society Board welcomes two new members, who were elected at the May 2016 Board meeting. They bring considerable business, financial, governance, and marketing knowledge with them, along with the deep commitment that comes from having loved ones who are affected by FSHD.

Christine Ford is a business graduate of California State University, Sacramento. She earned her CPA license in 1984 and worked as an auditor with the California State Auditor. She then moved on to various management positions with the California Lottery and the California State Teachers' Retirement System, where she was the CFO and then chief of staff. Chris serves on the finance committee for the FSH Society.

Since retiring in 2012, Chris has been able to more vigorously pursue her interests in travel and volunteering. She has held volunteer positions with her city's personnel board and as a tax preparer with AARP. She currently volunteers in a library program, assisting grade school children and their parents in understanding California's new core curriculum.

Having a sister who has been affected by FSHD for over 30 years, Chris is very committed to furthering the Society's education, research, and development goals so that there can be a future with treatments and a cure. Chris belongs to the Sacramento FSH Society member network, where she continues to educate herself about FSHD and reach out to others who may be newly diagnosed. Chris, a native New Yorker, currently resides in Roseville, California, with her husband Jim.



**Christine Ford**

Tom Ruekert is vice president for Palo Duro Hardwoods. He assisted with the development of a new charter school in his community that opened in 2013, and he now sits on the governing board. Tom spends his free time coaching his children in sports. He aims to work closely with our development and investment committees to bring awareness and additional funds to FSH Society programs.



**Tom Ruekert**

Tom's wife Katie was diagnosed with FSHD in 2011 and has become a strong advocate for the FSH Society. She founded and co-chaired the Walk & Roll to Cure FSHD in Castle Rock, Colorado, on September 10.

Amy Bekier, who joined the FSH Society Board of Directors in 2014, was elected secretary of the Board in May 2016. Amy has stepped ably into the role vacated when Beth Johnston retired from the Board earlier this year.

Linda Laurello-Bambarger stepped off the Board in June 2016. Linda took considerable time and effort to serve on the finance committee while juggling the growing demands of her position as CFO for Delta Railroad Construction and parenting a toddler and a new infant. During her tenure on the Society's Board of Directors, Linda co-organized the Cosie Laurello 10K memorial run in honor of her late grandfather and attended the 2015 Ms. Wheelchair USA competition as the Society's representative in support of FSH Society ambassador Skylar Conover, who won the contest.

We are deeply grateful to Linda for her service, and we look forward to a new chapter in the Society's long-standing relationship with the Laurello family. [FSH Watch](#)



## We welcome three new staff members

*Includes first full-time development position*

by **JUNE KINOSHITA**  
FSH Society

The FSH Society is experiencing unprecedented growth and opportunities to advance our mission, and we are excited to announce three new staff hires who bring superlative skills, commitment, and energy to help us achieve our goals.

Lisa C. Schimmel joined the FSH Society in April 2016 as director of finance and administration. Lisa is a CPA and has a wealth of experience in small- to medium-size nonprofits as well as for-profit companies. She brings to the FSH Society her expertise in



**Lisa C. Schimmel**

accounting, reporting, and strategic planning.

Prior to joining the FSH Society, she was the chief financial officer at Diagnostics for All (DFA). Previously, she served as the director of Finance and Human Resources at the Rose Fitzgerald Kennedy Greenway Conservancy.

Lisa taught financial and managerial accounting at Boston College from 1992 to 2011. While teaching, she also served on several nonprofit boards and finance

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## ICONIC SAN FRANCISCO SETTING FOR FSH SOCIETY BENEFIT CONCERT

What a spectacular gala our San Francisco crew put together! The Third Annual Songs in the Key of Steven Blier was a big hit. It was held in Grace Cathedral of San Francisco on an unusually warm day. The sun was shining, and people brought their good spirits and winning smiles to support our great cause. New friendships were made, old ones were revived, and we could hear laughter and merriment at every corner.

Baritone Theo Hoffman, a Grand Finalist in the 2016 Metropolitan Opera National Council Auditions, and Steve Blier, an extraordinary pianist, put together an unforgettable show. They were witty yet serious, silly yet sensible, and just plain captivating. The duo tugged on every heart string with their tender “Maria” from Leonard Bernstein’s *West Side Story*, and then had everyone rocking out to Bruce Springsteen’s “I’m on Fire.”

The open-air buffet spread on the beautiful courtyard of



Theo Hoffman, baritone  
extraordinaire!

the cathedral delighted even the pickiest of eaters—oh, and that dessert table after the concert!—a succulent spread of petits fours, cupcakes, opera cakes, tartlets, and much more. The indigo and gold table linens and coordinated floral arrangements echoed the colors of the evening sky and San Francisco skyline awash in the light of the setting sun.

Sincere thanks to my fellow committee members, Joyce Hakansson, Barbara Kweller, Ru Paster, and Christopher Wiseman for their selfless hard work to ensure that every detail of the event was attended to. Don Sebastiani & Sons and Jackson Family Wines donated the excellent libations. The Junior League of San Francisco provided a wonderful corps of volunteers.

Our biggest thanks of all to the amazing Lewis family, to whom we dedicated the concert. As the event’s “FSHD Hero Sponsors” over the past three years, they have been instrumental in their leadership and generosity to the success of our efforts and to the FSH Society as a whole. We all owe them the most profound debt of gratitude. We hope you will join us next year for yet another unforgettable event!

—Nez Bennouna-Zhar

## OUR OWN AMERICAN NINJA WARRIOR

... from page 1

Two weeks later, he competed at the Braves Stadium in downtown Atlanta. Spencer was shown the obstacle course when he arrived that day, but none of the competitors were allowed to try it out in advance. He was only going to have one shot to get through it.

That night, 30 of our closest friends joined our family in cheering from the sidelines. Spencer was told to show up at 8:30 p.m. for a 9:30 p.m. run. However, he didn’t go on until 2:00 a.m.! Imagine the adrenaline running through his body all day long!

A few weeks after the shoot, we were informed that NBC wanted to do a spotlight story about us. We spent a whole day filming out in the woods and in our house, answering hundreds of questions about FSH muscular dystrophy and what it has been like growing up with it.

In June of this year, *American Ninja Warrior*’s Atlanta episode aired on NBC, and again we had our family and 30 of our closest friends watching at my parents’ house, all huddled around the big screen. The anticipation that night was unreal, as we had no idea what the final product looked like, nor did we know when



Spencer’s segment would run. Turns out he was the last one to go on. Saving the best for last! While it was sad to see him fall on the last obstacle, we were still amazed and in awe of his accomplishment.

Wow! What a humbling journey this has been! Our family would like to thank everyone from the bottom of our hearts for supporting us through *American Ninja Warrior*.

I don’t think we can wrap our brains around how far out this coverage went. Since the airing, our whole family has received over a thousand Facebook messages, Instagram direct messages, tweets, shout outs at the mall, park, and grocery store, written letters, and donations. To date we have raised \$14,882.50!

These 15 minutes of fame, however, proved to be more than just about raising awareness for the FSH muscular dystrophy community. They have turned into an incredible catalyst for people who have opened up and shared their personal battles with me, ranging from paralyzing car accidents to spina bifida. I couldn’t feel more honored. I have met the most incredible, inspiring warriors who overcome personal struggles daily.

This experience has shown me not only that people are willing to see a story on TV and donate to a worthy cause, but are also willing to take it a step further and be vulnerable by opening up and sharing their own stories.

So what’s next for our family? Spencer hopes to compete again next year in *American Ninja Warrior*, and in late October we are planning to hike the Georgia segment of the Appalachian Trail with Spencer carrying me piggyback for all 89 miles. We have partnered with Vestigo, an outdoor adventure company, to guide us, and another company, ENO, to design a harness.

Together we believe in overcoming FSHD, and we won’t stop until we find a cure! [FSHWatch](#)



## 2016 Fall Event Calendar

Check our website at <https://www.fshsociety.org/events> for updates, details, and maps.

**Saturday, September 10, 2016**

9:00 a.m.

Walk & Roll to Cure FSHD

Phillip S. Miller Park, Castle Rock, CO

**Saturday, September 17, 2016**

12:00 p.m. – 3:00 p.m.

Mid-Atlantic Member Meeting

Children's National Medical Center, Main Campus  
6th Floor Conf. Room, Washington, DC

**Saturday, September 17, 2016**

1:00 p.m.

Brother John's BBQ, with Ray Jordan & Cyndi Segroves

1801 North Stone Avenue, Tucson, AZ

**Saturday, September 24, 2016**

10:00 a.m. – 12:00 p.m.

Western Washington FSH Community  
Luther's Table, Renton, WA

**Saturday, September 24, 2016**

11:00 a.m. – 2:00 p.m.

North Carolina Member Meeting  
Triangle Presbyterian Church, Durham, NC

**Saturday, October 15, 2016**

7:00 p.m. – 10:00 p.m.

Kicking off a cure with cocktails  
Maddry Residence, Atlanta, GA

**Saturday, October 22, 2016**

10:00 a.m. – 12:00 p.m.

Western Washington FSH Community  
Luther's Table, Renton, WA

**Sunday, October 30, 2016**

5:30 p.m. – 10:00 p.m.

Ghostly Gala to Vanish FSHD  
Cicada Club, Los Angeles, CA

**Saturday, November 5, 2016**

10:00 a.m. – 5:00 p.m.

2nd Annual Inherited Neuromuscular Disorders  
Family Conference  
University Park Marriott, Salt Lake City, UT

**Thursday, November 10, 2016 –**

**Friday, November 11, 2016**

FSHD International Research Connect  
Conference  
Westin Copley Place, Boston, MA

**Friday, November 11, 2016 –**

**Saturday, November 12, 2016**

2016 FSHD Patient-Researcher Connect  
Conference  
Westin Copley Place, Boston, MA

**Friday, November 11, 2016**

6:00 p.m.

FSH Society CureFSHD National Gala  
Westin Copley Place, Boston, MA



## WE WELCOME THREE NEW STAFF MEMBERS

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committees. Prior to her teaching experience she was a manager at the Arthur Andersen Enterprise Group.

Robyn O'Leary joined the FSH Society in May 2016 as executive assistant to provide administrative support and coordinate the Society's meetings and events. Before joining the Society, she was senior education coordinator and marketing compliance liaison at Dentsply Sirona, where she worked in a broad range of administrative and marketing roles over 15 years. Robyn lives in Billerica, Massachusetts, with her husband and two children.

Our most recent hire is someone already well known to many in our community. Beth Johnston joined the FSH Society in August of 2016 as development officer to assist the executive director and the leadership team to create, execute, and grow the Society's fundraising plan.



**Robyn O'Leary**

Beth's long history with the Society includes serving as co-chair of the Festive Evening of Song benefit concert, secretary of the Society's Board of Directors, and grant writer. She will continue her commitment to raising awareness of both the disease itself and the function of the Society in this new development role.

Prior to joining the Society, Beth was founder and chief executive officer of Social Bridges, a Denver-based social media marketing agency. She has also worked in information technology, project management, high-technology consulting, telecommunications, and real estate.

The Colorado native received a BS in business management and finance from Colorado State University and an MBA from the University of Denver. Beth resides in Denver, Colorado, with her husband Jeff and two daughters, Samantha and Nicole. FSH Watch



**Beth Johnston**



## Many ways to support us!

### MAKE A GIFT @ FSH SOCIETY

Make your gift online at <https://www.fshsociety.org/make-gift/> or by phone at (781) 301-6042. Having a well-networked community is essential for making progress toward treatments and a cure.

### COMBINED FEDERAL CAMPAIGN (CFC)

Federal employees can enroll in workplace giving from September 1 to December 15. The FSH Society's CFC identification number is 10239.

### FREE MONEY! CORPORATE MATCHING GIFTS FOR EMPLOYEES

Many organizations will match employees' charitable donations, or will donate if employees volunteer their time to a charity. Don't leave this opportunity to donate on the table! Ask employers about their charitable gift programs.



### GIVE WITH A SMILE THROUGH AMAZON

Amazon will donate 0.5 percent from your eligible purchases to the FSH Society whenever you shop on AmazonSmile. To get started, register here: <http://smile.amazon.com/ch/52-1762747>.



### RAZOO ONLINE FUNDRAISING

Razoo makes it easy to create an online campaign. Your donors will enjoy the convenience, knowing that their gifts will go directly to the FSH Society. With Razoo you can easily promote your campaign over Facebook, Twitter, and other social media. <http://www.razoo.com/story/Facioscapulohumeral-Society>



### OUR EBAY CHARITY AUCTION SITE

The FSH Society is registered (as "FSH Muscular Dystrophy Society") on eBay's charity auction site. If you have an eBay seller's account, you can list items and direct from 10 to 100 percent of the proceeds to the Society. <http://givingworks.ebay.com/charity-auctions/charity/fsh-muscular-dystrophy-society/76296/>



### NOT GETTING OUR EMAIL NEWS?

Sign up right on our website by clicking "JOIN." If you are certain you are on our email list, please check your spam or junk folder.



### GET SOCIAL!



Find our Facebook, Twitter, and Yahoo! Groups by visiting <https://www.fshsociety.org> and clicking on the logos in the right-hand margin.

Our online communities are great sources of news, advice, and social support. The FSH Society Yahoo! Groups forum, online since the 1990s, has tens of thousands of searchable posts. Bookmark these pages and come back often. Use your account privacy settings to limit who can see your posts.

### HAVE YOU MADE A GIFT TO THE SOCIETY IN 2016?

Thanks to the support from members like you, the FSH Society is a world leader in combating muscular dystrophy. **Your donations are tax deductible, and they make a real difference.** Please send your gift in the enclosed envelope. Or contribute online at <https://www.fshsociety.org>. Thank you!



### CHARITY NAVIGATOR TOP PERFORMER

The FSH Society has been awarded its eighth consecutive 4 Stars by Charity Navigator, placing us among the top 2 percent of US charities for fiscal responsibility and governance.



## Information at your fingertips

The FSH Society is dedicated to making sure you have accurate, useful information to help improve the quality of your healthcare and daily life. Our publications are created and reviewed by patients and experts. Visit our website (<https://www.fshsociety.org/>) and go to Understanding FSHD/Brochures & More to download:

- About FSHD
- Physical Therapy and FSHD
- FSHD: A Guide for Schools
- FSHD and Social Support: A guide for friends and family
- Evidence-based FSHD care guideline—Summary for clinicians
- Evidence-based FSHD care guideline—Summary for patients & families

You can also request printed copies by contacting us at:

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