Greening the landscape

Living with a rare disease can feel terribly lonely, all the more so when one is faced with the world’s indifference to the patient’s plight. So when Susie Kanewske, a busy fashion stylist from Portland, Oregon, was diagnosed with facioscapulohumeral muscular dystrophy (FSHD) in 2014, “understanding that I had this disease and not knowing if my children or grandchildren might have it, too,” she recalled, “I didn’t think there would be a solution during my life.”

Stunned to learn the cause of the leg weakness that she had first noticed five years earlier, Kanewske searched the Web to learn everything she could about her condition. “A few months later,” she said, “I was at my first FSH Society conference.” The experience changed her life.

The Society’s Patient Connect conference, one of the essential programs made possible by your donations, is the premier networking event for the FSHD community. Every two years, patients, families, researchers, and clinicians gather to learn from one another and emerge with new ideas and a powerful sense of purpose to work toward solutions. For Kanewske, “The Society is a lifeline. It is a place I can go to for answers.”

Your support has enabled the FSH Society to sustain a 26-year effort to transform the FSHD landscape from an outback into an ecosystem ripe with opportunity. Biotechs today see world-class scientists who have made impressive headway toward understanding the disease. They see engaged, motivated patients eager to volunteer for studies. They see essential tools—FSHD cell lines, bioengineered mice, repositories of patients’ tissue and DNA—to aid in testing drugs and gene therapies. And the result is that today more than a dozen companies are pursuing treatments for FSHD. This is unprecedented.

Thank you—our donors, volunteers, Board of Directors, and Scientific Advisory Board—for giving genuine hope to Kanewske and thousands like her. Today, your support is more important than ever. With your generous contributions, the FSH Society can continue to have an outsized impact on FSHD research and on the lives of patients and families everywhere.

June Kinoshita
Executive Director and Chief Operating Officer
Over the past three years, two experimental treatments have been tested in patients, and more than a dozen companies have joined the field to work on the next generation of FSHD drugs, many of which are targeting the DUX4 gene thought to lie at the core of the disease. These are dramatic advances, and they did not happen overnight. They are the result of the FSH Society carefully cultivating and nurturing the conditions needed for research and drug development efforts to thrive. Signature achievements in 2016 include the following:

Support for understanding the cause of FSHD
- A record-setting $1,383,892 in FSH Society commitments to new research projects.
- Coriell Institute makes available a biorepository of FSHD cell lines generated out of 607 samples collected from 43 multi-generational families.
- Creation of a nationwide FSHD tissue donation registry.
- Development of a new FSHD mouse model, now available from Jackson Labs.

Getting ready for clinical trials
- Helping to launch the seven-center FSHD Clinical Trial Research Network (CTRN) through an FSH Society grant, with support from the Geraldi Norton Foundation and an anonymous donor.
- Co-organizing an international workshop on global FSHD patient registries.
- Collaborating with Acceleron Pharma to make sure outcomes measured in Acceleron’s clinical trials will be meaningful to patients.

Building a strong network
- Our first joint FSH Society international research and patient networking conference attended by more than 350 researchers, patients, families, and other stakeholders.
- Participating in three FSHD Family Day conferences at leading centers and organizing member support group meetings at 16 sites across the US.

Clinical Trial Research Network members
The FSH Society’s investment in research is reflected in the steady increase in scientific publications on FSH muscular dystrophy since 1991. A spike in publications occurred in 1995 due to the discovery that contraction of D4Z4 repeats on chromosome 4 causes FSHD, and the value of p13e11 for genetic testing was observed. This came on the heels of the 1990 discovery genetically linking FSHD to region 4q35 on chromosome 4.

Since the FSH Society’s work to pass the MD-CARE Act in 2001, National Institutes of Health funding for FSHD (dark green) has grown from 2 to 11 percent of total NIH funding for muscular dystrophy (light green).
Since 2012, the FSH Society’s revenue has increased steadily. The Society is determined to expand its capacity in fundraising in order to invest in the research and education needed to build upon our track record of progress and success.
The knee of the curve

Progress can seem excruciatingly slow when a technology revolution is viewed in real time. And the fact of the matter is, finding a treatment for FSHD is a daunting task for which there are no shortcuts. Do not be disheartened. It is a heady time for biotechnology in general and FSHD research in particular as we approach the “knee of the curve.” The very nature of the technology acceleration curve is that the seemingly impossible becomes inevitable if you can keep to the course. I thought it to be impossible before, but my true hope today is that in our lifetime, we will be able to look back and reflect on how we all witnessed and profited from an amazing time of progress and success. So stay tuned in, buckle up, and join us in all ways that you can to keep us all on the acceleration curve!

—Stuart Lai, co-founder, REDI trading systems
FSH Society Board of Directors

Add value to your philanthropic investment

The FSH Society empowers you to invest in the best scientific and medical research for a cause that is important to you: FSH muscular dystrophy. You can rely on the FSH Society to provide the following valuable services to our donors:

- Solicitation of research proposals from hundreds of researchers worldwide.
- Rigorous scientific review of grant applications by world-class experts.
- Administration of grant contracts with research institutes.
- Progress reports from grant recipients.
- Reporting back to you on the impact of your philanthropy.
- Requiring grant recipients to publish their data.
- Publicity for research findings and funding sources.
- Networking with academia and industry to advance the research.
- Stewardship of research resources resulting from funded work.
- Stewardship of intellectual property.
- Good governance, transparency, and accountability.

With the FSH Society as your partner, your philanthropy will have a powerful impact on our shared quest to improve health and find effective treatments for all who live with FSH muscular dystrophy.

TO LEARN MORE about how to have an impact with your philanthropic investment, contact June Kinoshita, Executive Director and COO of the FSH Society, by phone at (781) 301-6649 or email (june.kinoshita@fshsociety.org).
$2,500+ individual, corporate, and foundation donors

We are grateful for each and every gift. Large and small, they add up to a powerful force!

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Delta Railroad Construction, Inc.
Michelle & David Mackay

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Arlene & Dr. Gary Cohen
Stuart Cohen
Legacy Circle

These individuals have arranged for a gift to the FSH Society in their wills or other estate plans. Their generosity helps to ensure that the Society will be able to sustain its mission for future generations. They have our deepest appreciation.

- Michael & Ellen Aeling
- Anonymous
- Barbara Finlay Trust
- Sanford Batkin
- James A. Baxter
- Amy Bekier
- Greg & Tanya Bergstein
- Joette A. Black
- Barbara & James Chin Sr.
- Michele De Sha & Howard Chabner
- Thomas Dempsey
- JoAnn P. Forance
- James Fox
- Barbara Fulton & Ralph Bladt
- William Herzberg & Judy Marantz-Herzberg
- Christine & Jeffrey Jacobs
- George T. Kuykendall
- Duncan & William R. Lewis Sr., MD
- Susan Lipari
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- Roy & Kristy Neilson
- Betty Schechter
- Ellen Schechter Berger & Jeffrey Berger, MD
- Elizabeth Schrauder
- Deborah Schwartz
- Patti Smith
- Robin Stemple
- Thomas & Kathy Stewart
- Madeline & Dr. Lawrence Weinstein
- Helen & Marc Younger

We are grateful to all of our donors. No matter how small or large, your gift has an impact!

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Double the impact of your gift!

Your donations are vital for helping the FSH Society overcome its challenges and meet its goals. We must invest in deciphering FSH muscular dystrophy so that we can aim treatments at the correct targets, even as we build the infrastructure to ensure that clinical trials will have meaningful results. A group of generous benefactors has pledged to match your donations up to a total of $50,000 during our spring campaign. Help us rise to this fundraising challenge!

Spring matching gift campaign deadline: June 20
World FSHD Day

Unite to find a cure

Barbara Chin’s gift to the future

We at the FSH Society were deeply moved and inspired to receive a magnificent bequest of $50,000 from the Barbara A. Chin Trust. This gift will enable the Society to fund work that Barbara cared deeply about: education and support for people with FSH muscular dystrophy, like herself and her late son Jimmy, and research toward treatments and a cure.

Barbara and her husband Jim Sr., who serves on the Society’s Board of Directors, have supported the Society not only financially but through countless hours of volunteer service. Barbara changed the lives of many patients and families through her kindness and compassion as a peer counselor. The entire FSHD community owes her so much.

Barbara’s bequest helps to ensure that future generations will enjoy lives free of the burdens of this disease. Individuals and families even of modest means can leave a powerful legacy by including the FSH Society in their wills or estate plans.

TO LEARN MORE please visit https://www.fshsociety.org/planned-giving/.

“After fighting this disease for 44 years, many times feeling like I was doing it alone, I now know that there are warriors out there to fight with me. I cannot express my gratitude and love for all of you who are in this battle.”

— BELINDA MILLER, Author