2015–2016
Year-to-Date

Donor Impact
REPORT

FSH SOCIETY
FACIOSCAPULOHUMERAL MUSCULAR DYSTROPHY

Connecting for a Cure

The woman's eyes welled up with tears the moment she stepped into the Starbucks in a suburb of Chicago, where a dozen FSH Society members had gathered. She had struggled for years with a mysterious malady that had finally been given a name: facioscapulohumeral muscular dystrophy. "My own relatives still don't believe I have a real condition," she wept. But now she had found her FSHD family.

Such moments of connection are life changing, and they happen every day thanks to your support. A connected patient is empowered to advocate for his or her own health and well-being, participate in research, and network with others to drive progress. Connected patients are as vital as any scientist or clinician to the success of our endeavor.

Your support also forges connections on a larger scale. The FSHD Clinical Trial Research Network (CTRN), which launched this year with funding by the FSH Society, is a milestone. The CTRN brings leading FSHD research centers together into a "center without walls," capable of carrying out state-of-the-art clinical trials at the scale required to get a drug approved by the FDA.

The CTRN is essential for future treatments, and it has come about because of the solid foundation the FSH Society has built over the past 25 years. This sustained effort—to unravel genetic mechanisms, create cellular and animal models, and measure the impact of FSHD on patients' strength and function—has brought us to where we are today.

As we focus our efforts on the future, it's instructive to review what we have accomplished in our first 25 years. In the center of this report, we have placed the major milestones on a timeline. It is an astonishing parade of achievements by any standard.

We did this, together.

You—our donors, staff, volunteers, Board of Directors, and Scientific Advisory Board—have been the lifeblood of the FSH Society. As we celebrate our achievements, let us also redouble our commitment to push forward toward a future when FSH muscular dystrophy can be treated, and all who live with this condition can be freed of its burdens.

June Kinoshita
Chief Operating Officer, FSH Society

"Academia and industry are making real progress toward treatments for FSHD. The Clinical Trial Research Network funded by the FSH Society is critical for ensuring the success of these trials."

— PETER JONES, PHD

OUR ACHIEVEMENTS

In 2015-2016 YTD, the FSH Society invested more than $2.3 million in research projects and initiatives that are blazing trials to the future. Highlights include:

Resources and infrastructure

- FSHD tissue donor registry launched.
- FSHD Clinical Trial Research Network launched.
- International workshop organized on global FSHD patient registries.
- NIH funding for FSHD research reached new high estimated at more than $17 million over the most recent two years (2015-2016).

Advancing the frontiers of knowledge

- Launched a genomewide CRISPR knockout strategy to identify genes that modify FSHD.
- Human FSHD muscle was successfully grafted in a mouse from immortalized cell lines.

Paving the path toward treatments

- First use of CRISPR gene-editing technology to silence DUX4 (the gene thought to cause FSHD).
- Antisense technology repressed DUX4 in human FSHD cell lines.
- Gene therapy for FSHD approached readiness for human trials.
- FSHD Health Index clinical trial outcome instrument almost ready to submit to the FDA.
- Candidates for blood biomarkers identified.
- Electrical impedance myography showed promise as noninvasive method to assess muscle condition.

Empowering patients and families

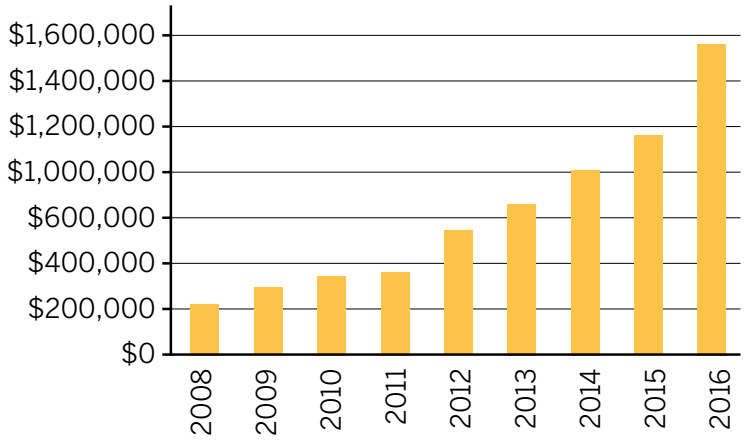
- FSH Society member meetings were held in 16 locations around the U.S.
- FSH Society collaborated on Patient Days with three FSHD research centers.
- The journal *Neurology* published the first evidence-based FSHD care guideline for physicians.

Drug companies wake up to the opportunity

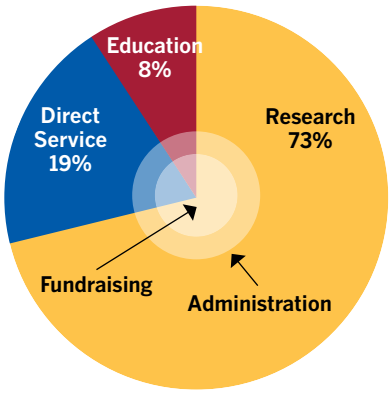
The years of coordinating and investing in basic research are laying the groundwork for industry to launch programs to discover and develop treatments for FSHD. Today, there are more than a dozen companies with FSHD programs. Notable events in 2015-2016 include:

- **aTyr Pharma initiated the first clinical trial for early-onset FSHD.**
- **aTyr Pharma conducted the first industry-sponsored adult FSHD clinical trial.**
- **Acceleron announced plans for a Phase 2 clinical trial in adult FSHD patients.**
- **Facio Therapies developed its FSHD drug-screening platform.**
- **Ultragenyx partnered with an FSH Society-funded investigator.**
- **Fulcrum Therapeutics, with $55 million in start-up funds, set its sights on FSHD.**
- **Idera and Genzyme reported early-stage success with antisense approaches.**

Accelerating investment in research

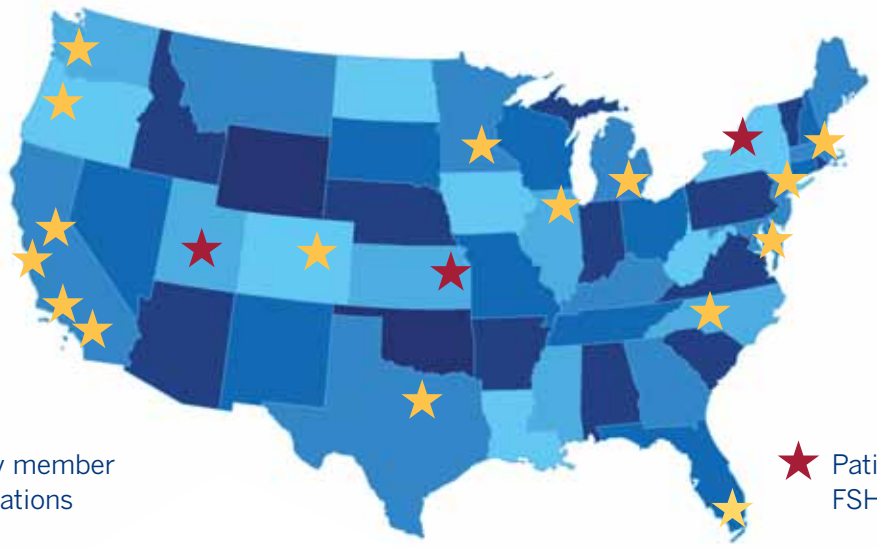


How we invested funds in 2015



Research:	$1,202,949
Direct Service:	$315,100
Education:	$136,178
Administration:	$188,236
Fundraising:	$121,021
TOTAL:	**$1,963,484**

Established 19 meeting sites since 2013



★ FSH Society member meeting locations

★ Patient Days at FSHD clinical centers

FSH SOCIETY 25TH ANNIVERSARY TIMELINE

1 **1991**
FSH Society founded.

2 **1993**
First FSH Society Scientific Advisory Board formed.

3 **1993**
Study of families reveals evidence of genetic heterogeneity.

4 **1994**
First issue of *FSH Watch* published.

5 **1994**
Daniel Perez gives first testimony before the U.S. House of Representatives.

6 **1995**
First FSHD patient brochure published.

7 **1996**
First genetic test for FSHD on limited research basis.

8 **1997**
First International Scientific Symposium on FSHD.

9 **1997**
First FSH Society patient conference, in San Diego, with 80 members.

10 **1997**
David E. Housman becomes Chair of Scientific Advisory Board.

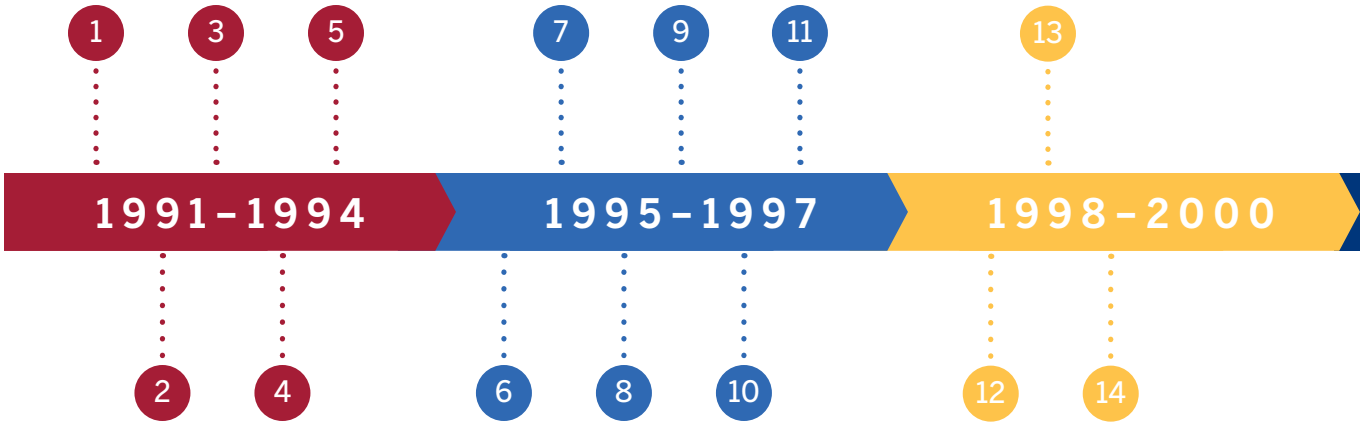
11 **1997**
First major clinical trial for FSHD (albuterol).*

12 **1998**
NIH makes first directed request for grants on FSHD as a result of Society's advocacy.

13 **1998**
First FSH Society grants awarded

- Marjorie Bronfman Postdoctoral Research Fellowship to Silvère M. van der Maarel.
- Marjorie Bronfman Postdoctoral Research Fellowship awarded to Sara T. Winokur.
- Delta Railroad Research fellowship awarded to Alexandra Belayew, PhD, for seminal work leading to discovery of DUX4.

14 **1999**
Daniel Perez becomes Society's first paid employee.



15 **2001**
FSH Society writes MD-CARE Act to include all muscular dystrophies; enacted into law by President George W. Bush.

- NIH announces series of initiatives to accelerate FSHD research.
- MD-CARE Act establishes multiple Wellstone Centers.
- Federal Muscular Dystrophy Coordinating Committee created.

16 **2002**
FSHD is associated with the chromosome 4qA allele.

17 **2003**
Daniel Perez works with Human Genome Project to finish sequencing "junk" DNA at 4q35.1-4q35.2.

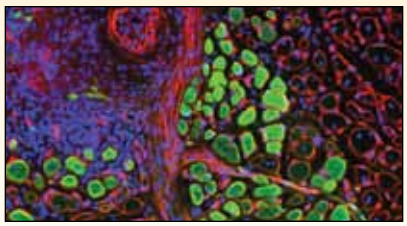
18 **2005**
First FSHD prenatal genetic testing made available in U.S.

19 **2007**
Clinical trial of myostatin inhibitor in FSHD patients.

20 **2009**
First brochure on Physical Therapy & FSHD.

21 **2010**
Genetic mechanism of FSHD Type 1 is published in the journal *Science*.

22 **2012**
International team finds gene for FSHD Type 2.



23 **2012**
FSHD Champions international alliance is established.

24 **2013**
Gene for FSHD2 reported to modify the severity of symptoms in FSHD1.

25 **2013**
First high-throughput screen for drugs targeting the genetic mechanism for FSHD1.

26 **2014**
Facio Therapies biotech founded.*

27 **2015**
CRISPR/dCas9 inactivates DUX4 expression in human FSHD muscle cells.

28 **2015**
FSH Society total lifetime grants awarded exceed $6.85 million.

29 **2015**
aTyr Pharma launches Phase 1 clinical trial of ResolarisTM.*

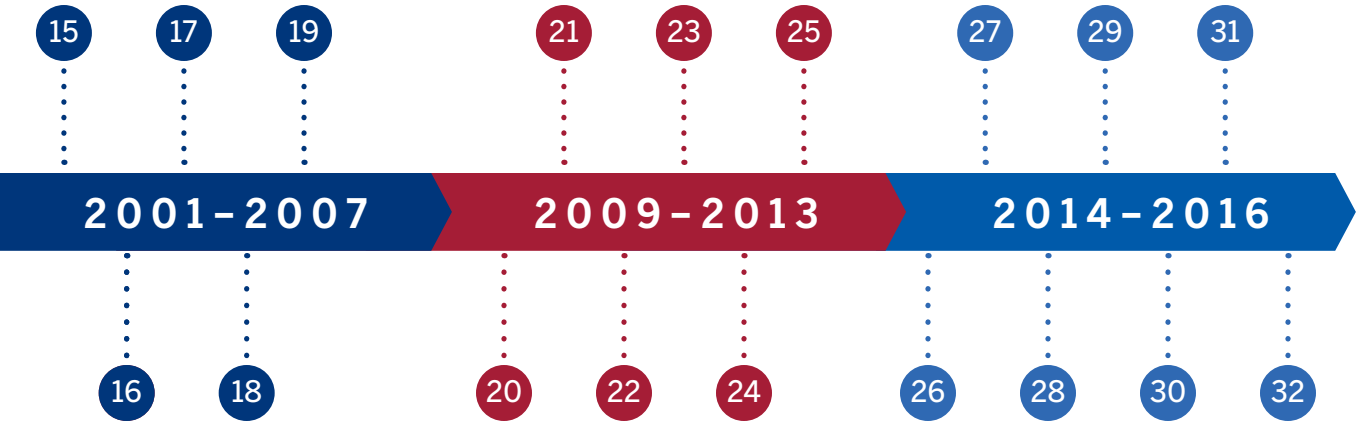
30 **2016**
Acceleron Pharma announces clinical trial of ACE-083.*

31 **2016**
Fulcrum biotech founded; FSHD among initial targets.*

32 **2016**
First "World FSHD Day™" observed on June 20.

WORLD FSHD DAY
Unite to find a cure

* *Drug company milestone*



2015 DONOR REPORT

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

$100,000+

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The most precious gift

"She's fading." It's the call we dread getting—news that one of our FSH muscular dystrophy family is approaching the end. And then we're blown away by what comes next: "She wants to donate her remains to science."

For a condition like FSHD, such occasions arise only a few times a year. It's the most precious gift to advance researchers' understanding of FSHD.

And so the call triggers urgent messages to family members, doctors, lawyers, hospitals, funeral homes, and researchers, as attempts are made to collect consent forms, arrange for transport of the remains, identify a team to retrieve the donor's tissues and deliver them to a waiting laboratory—all in a dauntingly brief time.

Not surprisingly, such efforts fail more often than they succeed. It's heartbreaking for everyone who has worked feverishly to make the donation happen. There had to be a better way, and fortunately, we found it: the National Disease Research Interchange (NDRI), a little-known nonprofit set up precisely to carry out this mission. Modeled on the nationwide network for retrieving donated organs, the NDRI is our partner in establishing the first nationwide FSHD tissue donation registry, which we launched this year.

The registry also arranges for tissue donations by patients who are planning to undergo surgery such as scapular fixation. The operation requires the removal of muscles damaged by FSHD. This tissue is invaluable for research.

The FSHD tissue donation registry arrives not a moment too soon. Scientists in academia and industry have an urgent need for tissue samples from individuals with FSHD to ensure that they thoroughly understand the genes and markers in actual human patients. Such insights will help them develop the model systems ("FSHD-in-a-dish") they need to search for potential treatments.

To join the registry, call the NDRI at (800) 222-6374.

INVEST IN OUR FUTURE

Your gift matters more than ever. Our entire Board of Directors has pledged a total of **$400,300** and challenges you to match this. From now through December 31, 2016, your gift will be counted toward our year-end challenge. Please stretch a little—13 percent over last year's gift—so we can achieve our goals!

FSH SOCIETY
FACIOSCAPULOHUMERAL MUSCULAR DYSTROPHY

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Four Star Charity

YOUR TRUSTED PARTNER. The FSH Society has earned its eighth consecutive 4-star award from Charity Navigator, placing us among the top 2 percent of U.S. charities.

Thank you for helping to restore our lives

I was 13 when I was diagnosed with FSH muscular dystrophy. Since then I have been faced with the struggle of adapting to a changed lifestyle, losing my identity and discovering a new one, and finding the strength to battle obstacles presented to me.

Throughout my diagnosis, the FSH Society has been a rock that has helped me find others who understand my struggles, and who are also motivated to help in any way possible to find a cure for this debilitating disease.

I want to convey my greatest thank you to all of the donors who have seen the importance in funding this cause. The impact that you all are having on hundreds of thousands of lives is immeasurable and will lead to these lives being restored to what they could and should be.

Thank you for your kindhearted support.

—Sincerely, Sarah Geissler