



# FY2017

## DONOR REPORT



## FSHD Moonshot

### EYES ON THE ULTIMATE PRIZE

**B**ecause of your generosity, we have seen exceptional progress in worldwide FSHD research and therapeutic development over the past several years. The FSH Society celebrates these advances as we look with great hope toward 2018 and beyond.

You have been the catalyst for these advances! You have enabled the FSH Society to build a launchpad from which we are ready to make our moonshot—treatments for FSH muscular dystrophy—using every tool in the arsenal of modern biotechnology.

Curing a genetic disease is a daunting mission that requires a clear strategy, leaders, warriors, allies, and supplies. With your continuing support, we will have what it takes to reach our goal—treatments and a cure for FSHD.

**We have a sense of urgency.** With every patient who is losing strength and skills, we hear the clock ticking. We must find a way to bring safe and effective treatments to our families much more quickly.

**We have hope.** More than a dozen pharmaceutical companies are working to find solutions. The FSHD field has deepened with new discoveries and, through your ongoing support, we are funding cutting-edge research to fill the pipeline with potential therapies.

**We know what we must do.** It's all about patients and families. We will do what it takes to get us further and faster to interventions that will slow or stop the progression and improve patients' health and well-being.

Your support has brought us far—and we will travel even further, together, all the way to our cherished destination—disease-modifying treatments and ultimately a cure. And you and your family need never endure this disease alone. We will be with you every step of the way.

With gratitude,

Mark Stone  
President and CEO

*"I can see that  
we have hit a  
tipping point.  
All this research  
is going to  
pay off."*

**—Deborah Schwartz,  
NEW YORK CITY**



*“In this life-changing storm, it feels good to have someone like you reach out to us so quickly and competently.”*

—Alex H.,  
DALLAS, TEXAS

## READY TO LAUNCH

A Massachusetts-based biotech has completed the first part of its Phase 2 study and reported that its compound has increased muscle size in FSHD patients by more than 12 percent. Buoyed by these initial results, the company will embark on studies in coming months to learn whether the muscle growth results in improved strength. This compound is a “first-generation” drug targeting general biological processes that regulate muscle growth.

Working in the wings at high speed are more than a dozen companies that are developing the next generation of therapies targeting the DUX4 gene thought to lie at the core of the disease—treatments that could potentially alter the course of the disease itself.

These are dramatic advances, and in 2017 we took bold steps to make sure the field is prepared for the time when these new drugs are ready to be tested in patients:

### Growing the capacity of the organization

- Mark Stone came on board as the FSH Society’s president and CEO, bringing a proven track record of transforming patient advocacy nonprofits into drug development powerhouses.
- We are building capacity around a focused plan of action aimed at achieving our goals.

### Getting treatments to our families faster

- We have sharpened our focus on initiatives, such as preclinical validation of candidate drugs, that will directly speed up treatment discovery and development.
- We are deepening and expanding our involvement with the FSHD Clinical Trial Research Network (CTRN) to make sure we have validated tools to measure outcomes, and an international network of centers ready to run rigorous clinical trials.

### Relentlessly pursuing the scientific breakthroughs we need to cure FSHD

- Understanding in detail how DUX4 expression leads to skeletal muscle weakness in a compelling new mouse model of FSHD.
- Testing an antisense molecule for its ability to knock down DUX4 in a mouse genetically engineered to express the FSHD-causing gene.
- Identifying novel treatment strategies by meticulously unpacking how DUX4 expression is regulated.

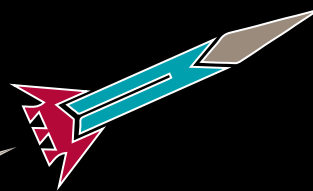
### Building a strong community

- We are establishing a nationwide chapter program to activate and empower patients and families to become research advocates and fundraising leaders in their communities.
- We launched regional FSHD Family Day conferences centered on the Clinical Trial Research Network member institutions, to build trust, communication, and a level of engagement among researchers, clinicians, and families that will be second to none.

### How the FSH Society drives FSHD therapy development

We invest strategically at key stages along the entire drug development trajectory. We invest where we see the need, to move us faster and most efficiently—from fundamental research on disease mechanisms and drug targets all the way to preclinical and clinical validation of new therapies.

# Drug Development Trajectory



6  
Clinical  
Trial

FSH SOCIETY ROLE:  
CTRN, validating  
clinical outcome tools

5  
Preclinical  
Profiling

FSH SOCIETY ROLE:  
Investing in early-stage  
drug development

4  
Lead  
Optimization

FSH SOCIETY ROLE:  
Developing animal  
models

3  
Target  
Validation

FSH SOCIETY ROLE:  
Bioresources for drug-  
screening assays

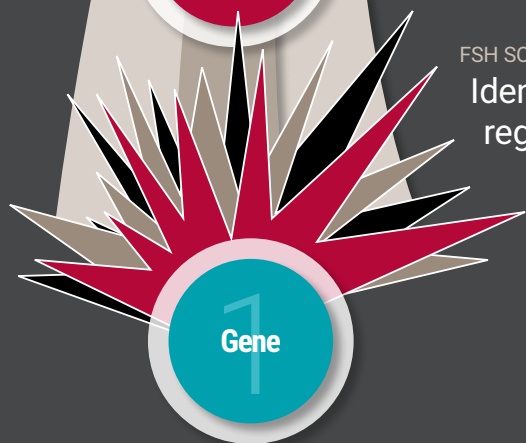
2  
Target  
ID

FSH SOCIETY ROLE:  
Early-stage target  
validation

FSH SOCIETY ROLE:  
Identifying targets to  
regulate DUX4

1  
Gene

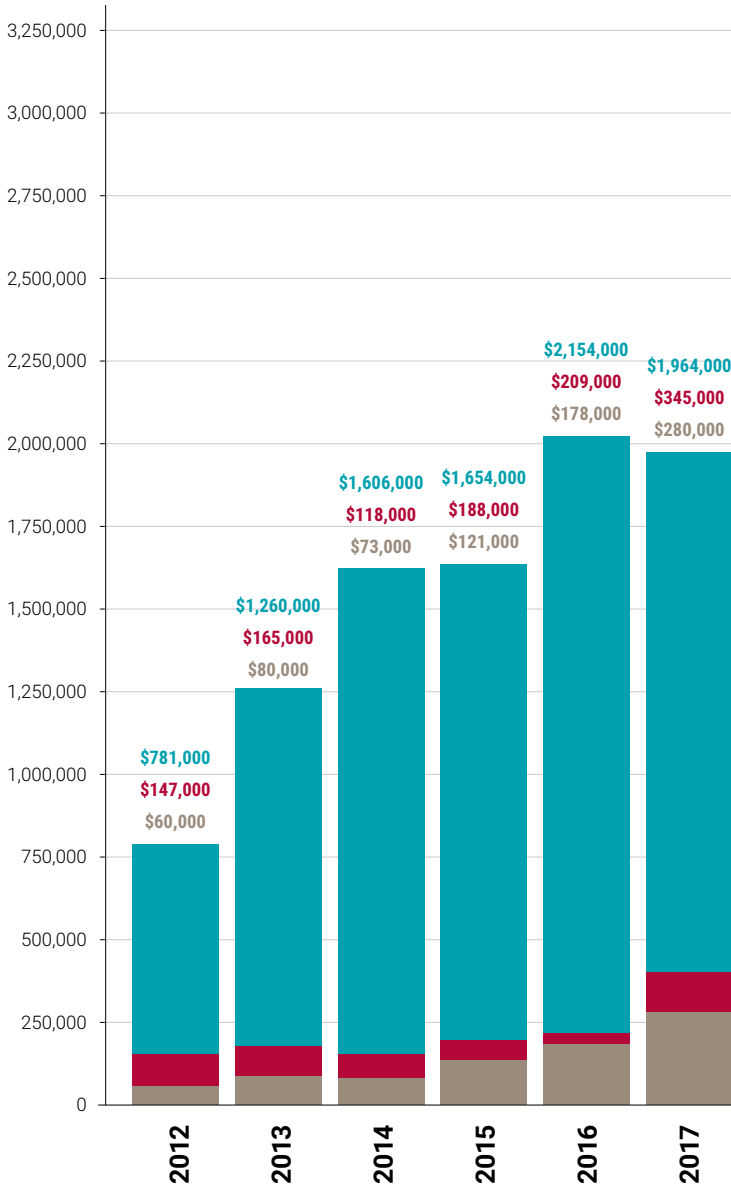
FSH SOCIETY ROLE:  
Discovery of DUX4  
mechanism



# ACCOUNTABILITY

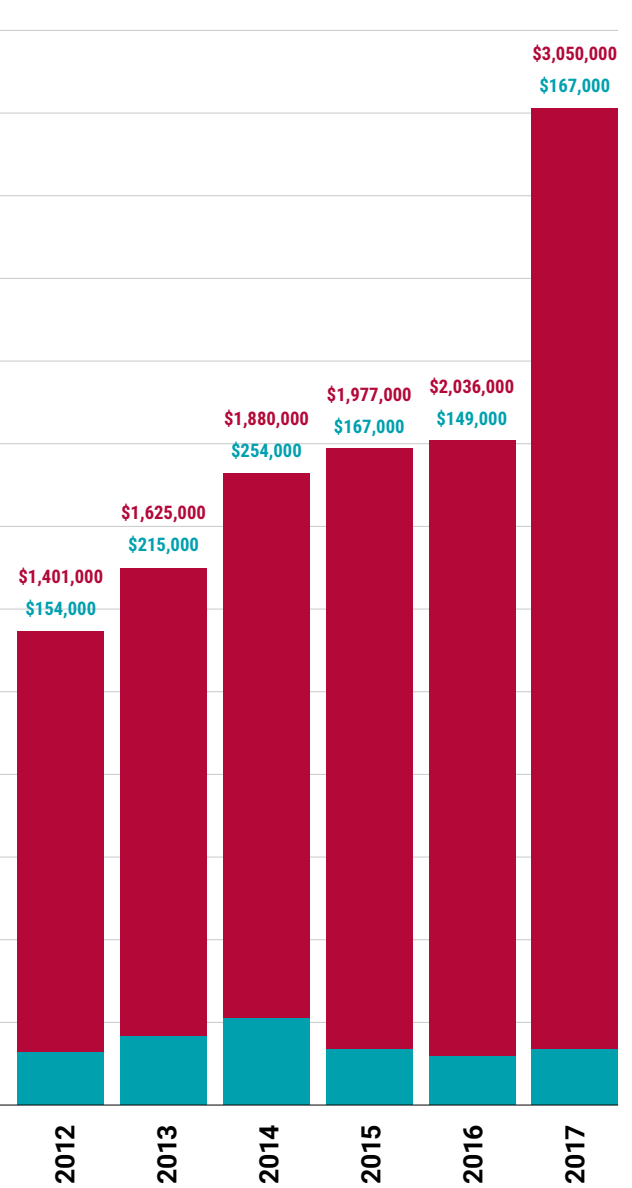
### How we invested our funds (2012–2017)

- Administration
- Fundraising
- Research & education programs



### Sources of support & revenues (2012–2017)

- Support & revenues
- Donated goods & services



# THE POWER OF GRASSROOTS



Team FSHD Cycling rockets out of the starting gate at the 36th annual Race Across America. The team's mission—to raise awareness and funds for the FSH Society. Nearly 400 donors pitched in to raise \$107,000, and all eight riders finished the 3,000-mile race safely together. FSHD patient and team leader George Pollock Jr. said, "This was truly a life-changing and inspiring expedition."



Our second annual Colorado Walk & Roll to Cure FSHD was another wonderful and successful event, raising \$31,500 to benefit the FSH Society. The day was surrounded by the peaceful Colorado blue sky and sunshine, and the event was overflowing with family, friendship, connection, encouragement, hope, and support.



*"I feel an exciting shift happening!"*

—Katie Ruckert,  
CASTLE ROCK, COLORADO

## 2017 BENEFACTORS

*Individual, corporate, and foundation gifts of \$2,500+*

**\$1,000,000+**

Duncan & William R. Lewis Sr., MD

**\$100,000-\$999,999**

S & L Marx Foundation\*

James Chin Sr.

New York Community Trust

**\$50,000-\$99,999**

SHIFT Communications

**\$25,000-\$49,999**

Anonymous

Delta Railroad Construction, Inc.

Andy Johnson-Laird & Kay

Kitagawa

Michelle & David Mackay

Bruce & Jeralyn Ryskamp

Wasily Family Foundation

**\$10,000-\$24,999**

Acceleron Pharma, Inc.

James W. & Mary B. Albert Jr.

Anonymous

Anonymous

Apple, Inc.

Robin Armour

Barbara Finlay Estate

Sanford Batkin

Betty Jane Schechter Estate

Finkelstein Foundation, Inc.

FSHD Canada Foundation

Roslyn Goldstein

Glenn & Petrina Hasman

Alex & Anne-Catherine Hoffmann

Mark Jagiela

Karen & Darrin Jewell

Stuart Lai

Local Independent Charities  
of America

William A. Maclean

William Michael

Jai & Jennifer Narayan

Michael & Melissa Penwell

Jane & Paul Rittmaster

Beverly & Mike Rowlett

Rebecca J. Ruosso

James & Emilie Ryley

Marsha & Jerry Seslowe

Allan C. Silverstein, DDS, & Pauli  
Overdorff

Neil Solomon, MD, & Paula

Birnbaum

Gregory Stucky and Gracia

Toubia

University of Massachusetts

Medical School

Madeleine & Lawrence

Weinstein, MD

William J. Conners III, & Barbara

S. Conners Charitable

Foundation

Helen & Marc Younger

**\$5,000-\$9,999**

AFM Telethon

Amy Bekier

Powell Brown

Deborah A. & Bradley Calhoun

Peter H. Catterall & Kimberly

Toskey

CIT Bank

Celeste A. Clark

Elizabeth & Joe Conron

Danziger & Markhoff, LLP

Deltex Associates, Ltd. LLC

DFORCE Holdings, Inc.

Julie & Mitchell Dye

Mohammad Ehteshami

Christopher Eklund

Christine & James Ford

GE Foundation Matching Gifts

Program

Goode Family Charitable

Foundation

Nils Hakansson

Sandra & Michael Hecht

Ida Laurello

Kim & C. Larry Laurello Jr.

Lawrence & Agnes Liptak

Kevin Monahan & Ellen Hannan

Adam Manacher & Carol S.

Birnbaum, MD

Marie & Landon Morrell

Susan Nordeen

Nu Way Tobacco Company

Gloria & Burton Richards

San Francisco Foundation

Scott & Lois Schultz

Deborah Schwartz

Kim Small & Norm Wesley

Southern Seafood Market

Susan & David Tunnell

Aileen & Ken Wyckoff

**\$2,500-\$4,999**

Anonymous

Margaret Bath

Jane Bernstein & Robert Ellis

Carter/Validus REIT Investment

Management Company, LLC

Howard Chabner & Michele

De Sha

Malissa Chavarria

Arlene & Gary Cohen, MD

Cytokinetics, Inc.

Alfonso A. & Josephine Greco

Dato

Todd & Sian Defren



## VISIONARY LEADERS ARE GAME-CHANGERS

In the spring of 2017, FSH Society Board chair **William R. Lewis Sr., MD**, and his wife **Duncan** announced a gift of \$1 million to be invested in our research programs. Dr. Lewis has been a member of the FSH Society Board of Directors since 1991 and chair since 2006. He is also a member of the Society's Scientific Advisory Board. As a neurological surgeon with more than four decades of experience—and the husband and father of FSHD patients—Dr. Lewis brings his medical expertise and compelling personal interest to his role at the FSH Society.

The Society also received a three-year grant from the **Sylvia and Leonard Marx Foundation** in the amount of \$120,000 annually, or \$360,000 in total, to support two postdoctoral fellowships per year. Our fellowship program has an enduring impact on the FSHD research community because these grants invest in new talent. The Society's fellowships have launched the careers of many of today's research leaders.

We thank **Bill and Duncan Lewis** and the **Sylvia and Leonard Marx Foundation** for their generous, visionary support.

Thomas Dempsey  
Susan Egert & Bill Milling  
Betty Ettinger  
Lora & Frank Ferguson  
James Fox  
Leonard & Linda Goldberg  
Google Matching Gift Program  
Herbert & Nell Singer Foundation  
William Herzberg & Judy  
Marantz-Herzberg  
Colette Hollander & Daniel  
Wheeler  
Christine & Jeffrey Jacobs  
Timothy Kerrigan & Catherine  
Devine

Joshua Knox  
Sidney & Ruth Lapidus  
Linda & Shane Laurello-  
Bambarger  
Harry & Pamela Lewis  
Lightning Foundation  
William Luby  
David Mazzarelli  
McKesson Foundation c/o JK  
Group  
Robert Melnyk  
Melanie & Norman Mintz  
Janet A. & S. Yegna Narayan  
Lynn & Matthew O'Meara  
Nancy Payton

Daniel Paul & Susan Perez  
Rose & David Pfanzelter  
George & Jane Pollock Jr.  
Tom & Vicky Reynolds  
Andrew & Sarah Rosenbach  
Judith & Kenneth Seslowe, MD  
Jeffrey Sherman  
Rod & Leigh Shuster  
State Street Matching Gift  
Program  
Monti Staton  
Gale Tunnell  
VX-Platform LLC

*\*From a three-year \$360,000 grant*

## Legacy Circle

*These individuals have made provisions in their wills or estate plans for a gift to the FSH Society. Their generous foresight ensures that we will be here to serve the next generation. They have our deep appreciation.*

Michael & Ellen Aeling  
Anonymous  
Sanford Batkin  
James A. Baxter  
Amy Bekier  
Ellen Schechter Berger & Jeffrey  
Berger, MD  
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  
Susie Kanewske  
George T. Kuykendall  
Duncan & William R. Lewis Sr., MD  
Susan Lipari  
Donald & Judith Lokerson  
William A. Maclean  
Joseph J. Matt III

Jennifer Moore  
Roy & Kristy Neilson  
Bruce & Jeralyn Ryskamp  
Elizabeth Schrauder  
Deborah Schwartz  
Patti Smith  
Robin Stemple  
Thomas & Kathy Stewart  
Madeleine & Lawrence  
Weinstein, MD  
Helen & Marc Younger



## WHY I MADE THE FSH SOCIETY A BENEFICIARY IN MY WILL

I've seen incredible leaders rise through the FSHD community. When one passes, another steps forward to take his or her place. We have a legacy of helping each other up.

I am eternally grateful to my father for giving me the financial means to take care of myself and to help others. The FSH Society has supported me, so I have supported the Society. I have designated it as a beneficiary in my will, to ensure that there will be treatments for this disease. I want to make sure there will always be someone to help the next person up.

— Deborah Schwartz,  
NEW YORK CITY

## EVERY GIFT HAS AN IMPACT!

***Whatever amount you are able to give means so much to us. You have our heartfelt thanks!***

GIFT LEVEL	NUMBER OF DONORS	TOTAL GIFTS
\$1,000-\$2,499	174	\$215,000
\$500-\$999	212	\$120,000
\$250-\$499	266	\$77,000
\$100-\$249	697	\$86,000
Under \$100	1,002	\$37,000

To learn more about joining the Legacy Circle, please visit <https://www.fshsociety.org/planned-giving/>.



## Help us lift off in 2018

Your spring campaign gift empowers the FSH Society to launch into the ambitious trajectory we have set for ourselves. Our 2018 Matching Gift Circle has pledged to **match your donations up to a total of \$100,000** during our spring campaign. Help us rise to this fundraising challenge!

*Spring matching gift challenge deadline: **May 31, 2018***

## We empower you to attain your philanthropic goals

The FSH Society invests in initiatives that help you meet your goal: to deliver treatments as quickly and responsibly as possible to our families.

- We evaluate projects to make sure they fit into our overall strategy of speeding up the development of drugs and therapies.
- Projects are also rigorously vetted for need, relevance, stellar science, sound finances, and excellent business management.
- Grant recipients must reach milestones in order to continue to receive funding.
- We steward the research resources and intellectual property resulting from funded work to make sure they will maximally benefit the community.
- We provide good governance, transparency, and accountability.
- We report back to you on the impact of your philanthropy.

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

To learn more about investing in the FSH Society's work, contact Mark Stone, president and CEO of the FSH Society, by phone at (781) 301-7323 or email [mark.stone@fshsociety.org](mailto:mark.stone@fshsociety.org).

## 2017 Matching Gift Circle

These pioneering benefactors enabled us to put up a total of \$66,500 in matching gift challenges in 2017, which inspired almost \$200,000 in donations. We thank them all, and especially Jim Albert for proposing this idea to leverage individuals' donations.

Mary & James Albert Jr.

Kay Kitagawa & Andy Johnson-Laird

William A. Maclean

William Michael

Jai & Jennifer Narayan

Beverly & Mike Rowlett

Allan C. Silverstein, DDS, & Pauli Overdorff

Madeleine & Lawrence Weinstein, MD