



FACIOSCAPULOHUMERAL  
MUSCULAR DYSTROPHY

# 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.

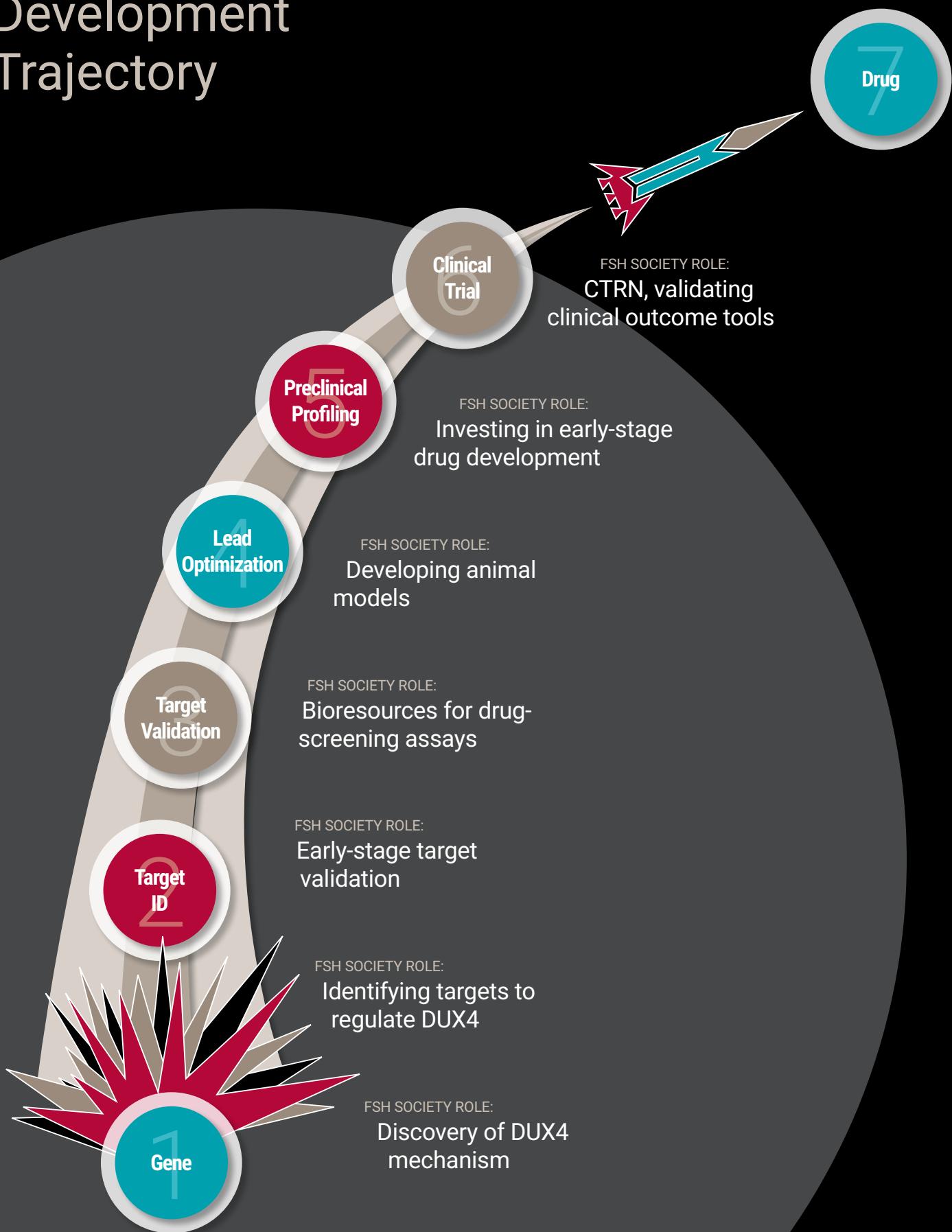
## Building a strong community

- 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



# 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 Ruekert,  
CASTLE ROCK, COLORADO

# 2017 BENEFACTORS

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

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

Duncan & William R. Lewis Sr., MD

**\$100,000-\$99,999**

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



## 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

## 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

## 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