THE. FUTURE. IS. NOW.

2019

Clinical trial research network expands by three

The first-ever FSHD Society volunteer leadership summit brought together chapter directors and Walk & Roll leaders from across the U.S.

Twelve chapters across the United States held FSHD Society-sponsored Walk & Rolls raising more than $400,000 for advocacy and research. In addition, grassroots community initiatives, like Team FSHD Skyland Trail in Race Across America which raised more than $200,000, are also getting us closer to a cure

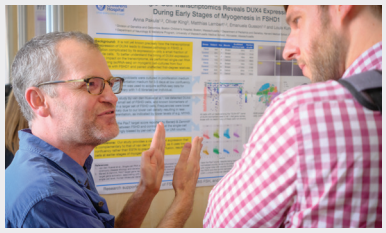
White paper on clinical trial readiness launched

The 26th annual International Research Congress brought nearly 200 researchers together from around the world

ReDUX4 Phase 2B clinical trial launched

In just one year, we launched 26 chapters, spreading information and support to families all across the United States

Innovative research continues to attract interest from drug companies and the NIH. Without support from donors, key initiatives might never have the chance to begin



This year, you were the catalyst that empowered the FSHD Society to launch an aggressive plan to accelerate therapeutic development for FSHD. We brought together pharmaceutical companies, researchers, government agencies, and families in a working collaborative with the goal of delivering disease-modifying therapies by 2025.

We have accomplished much together. Your support, activity, perseverance, and faithfulness have allowed us to be here, at this moment in history. And now, the future is in the same powerful hands … yours.

The visionary computer scientist Alan Kay famously said, *The best way to predict the future is to create it.*

Together, by taking up the mantle of leadership, and building and empowering a global FSHD infrastructure, we are creating a future free of the devastating effects of FSHD. Recognizing that if we don't act now it could take a decade or longer to bring treatments onto the market, we have charted a bold course to overcome the obstacles for therapeutic development and approval.

New Projects to Address Critical Barriers to Success

GENETIC DIAGNOSTICS

We are identifying state-of-the-art technologies and expertise to develop a faster, more cost-effective genetic test that will ease the financial burden on families and enhance clinical trial recruitment.

MOLECULAR BIOMARKERS

We are working with companies and government agencies to create a blood test that biopharmas can rely on to measure the effectiveness of therapies targeting DUX4, the toxic gene associated with FSHD.

INTEGRATION OF PATIENT REGISTRIES/NATURAL HISTORY STUDY

By tracking symptoms and rates of progression, patient registries play an essential role in therapeutic development. Registries have been established in 13 countries, but getting these invaluable data out of their "silos" has been challenging. We are working on ways to aggregate and analyze data, including novel approaches to "big data" analysis and integration, such as machine learning and artificial intelligence.

PATIENT-FOCUSED DRUG DEVELOPMENT MEETING

The Food and Drug Administration (FDA) encourages input from the patient community to ensure that treatments address the patients' primary needs. To facilitate this process, the FDA created the invitation-only Patient-Focused Drug Development (PFDD) meeting. In August, we received an invitation from the FDA to host a meeting this spring. This is a vital opportunity for families to voice their experience with FSHD, disease burden, and preference on treatment thresholds, both through public testimony to the FDA and with surveys representing our broader community.

Strategic Initiatives

We have built consensus on priorities and have enhanced our ability to lead such an endeavor. We are strong enough to:

ACCELERATE

Catalyze the global community to speed up therapeutic development, getting treatments to our families faster.

BROKER

Work with more than 20 companies either considering or actively pursuing therapy development in FSHD.

INFLUENCE

Positively influence regulatory agencies to ensure that safe and effective treatments move through the approval process unhindered.

GROW

Establish more than 25 volunteer-led chapters to provide a local presence while enhancing our global impact.

GLOBALIZE

Magnify the patient voice through an active international alliance of FSHD communities speaking with one voice, one million strong.

The $500,000 Challenge

Our Board of Directors has risen to the task, pledging $500,000 toward our year-end goal and challenging the rest of the community to match it. **Will you deepen your commitment to our collective mission by considering an "over and above" gift to help us reach this goal?** Donate at FSHDSociety.org, call 781.301.6060, or fill out and mail the form below.

2019 EOY

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Please make checks payable to the **FSHD Society**. The Society was incorporated in 1991 as a 501(c)3 charity. All gifts are tax-deductible.

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