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Continuing to make progress in understanding and treating FSHD

Trials readiness and infrastructure grant awards for 2017

On December 19, 2017, the FSH Society Board of Directors reviewed and approved the Society's Science, Technology and Research (STaR), and, Finance Committees' recommendations for research infrastructure and clinical trials readiness projects and funding. We received one request for bridge funding for the Clinical Trials Research Network (CTRN) for facioscapulohumeral muscular dystrophy (FSHD) through the coordinating center based at the University of Kansas Medical Center, Fairway, Kansas. Based on our ongoing conversations and the noted value of having a standing, research and trial-ready infrastructure for FSHD, investigators were selected to receive a FSH Society bridge grant of one hundred thirty-three thousand two hundred fifty-four dollars (US\$133,254) for six months. Below is a list of the funded project(s), including project description as submitted by the applicant.

We are very pleased to list the projects and grantees funded.

Ad-Hoc 2017 Cycle

1. Facioscapulohumeral Muscular Dystrophy Clinical Trial Research Network (FSHD CTRN)

Jeffrey Statland, MD

Assistant Professor of Neurology

University of Kansas Medical Center, Fairway, Kansas USA

02/01/2018 - 08/31/2018

US\$133,254 for 6 months

FSHS-82017-11 (a continuation of FSH Society Grant FSHS-52016-01)

Project Summary

The overall, long term aim of this application is to expedite the development of new therapies for Facioscapulohumeral Muscular Dystrophy (FSHD) by maintaining an FSHD Clinical Trial Research Network (CTRN). Successful clinical trials depend on several factors including: access to and the ability to recruit patients, a precise understanding of the natural history of the disease and the major contributors to disease variability, and reliable outcome measures that are sensitive to change in FSHD. A major hurdle to development of rational trial strategies and validation of outcome measures for FSHD clinical trials is lack of an existing clinical trial network infrastructure with common standard operating procedures (SOPs). We developed the FSHD CTRN to overcome this hurdle by: 1) creating a streamlined system for regulatory /ethical oversight; 2) developing standards for what data is collected and how it is collected; 3) creating a network of well-trained and knowledgeable clinical evaluators – this is absolutely essential for clinical trials; 4) creating a network of trained study coordinators with strong patient engagement, recruitment, and retention skills; 5) ensuring the participation of all major stakeholders; 6) validating new outcome measures for drug registration studies; and 7) training the next generation of FSHD clinical researchers (Figure 1). Not only will the FSHD CTRN help close gaps in trial readiness, but the CTRN also provides a network of sites with a centralized streamlined regulatory process, specific, common expertise in FSHD, and an engaged patient population ready to conduct efficient, high quality clinical trials.

The Pathway to FSHD Trial Readiness

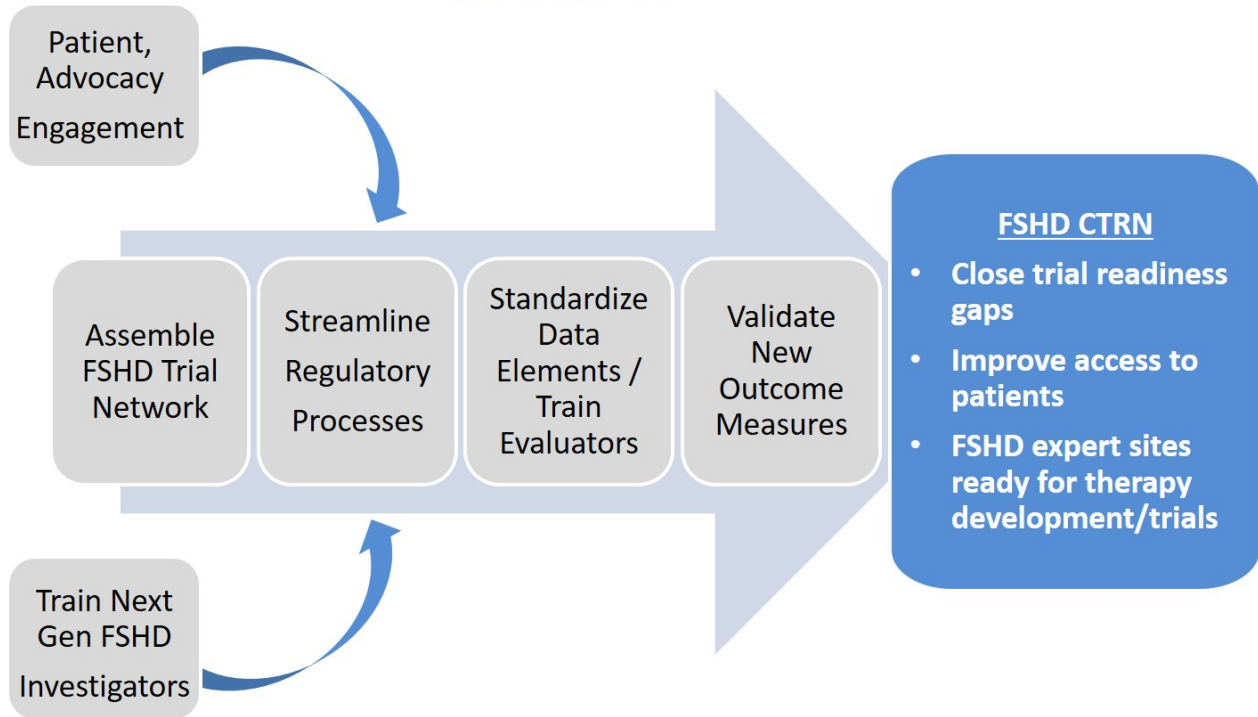


FIGURE 1

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