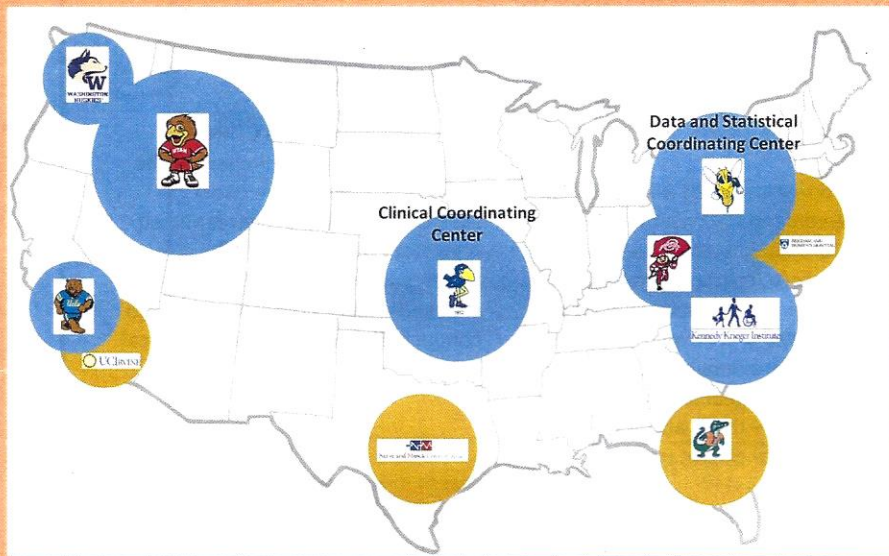


FSHD CTRN

FSHD Clinical Trial Research Network



*Map of FSHD CTRN participating sites. Back Up sites are in yellow.

**The FSHD CTRN is supported from a grant from the FSH Society (FSHS-52016-01), MDA Clinical Research Network Grant (573783) and Private Philanthropy*

What is the FSHD CTRN?

The FSHD CTRN is comprised of 7 academic centers across the United States with a large number of patients with FSHD.

A major hurdle to development of 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

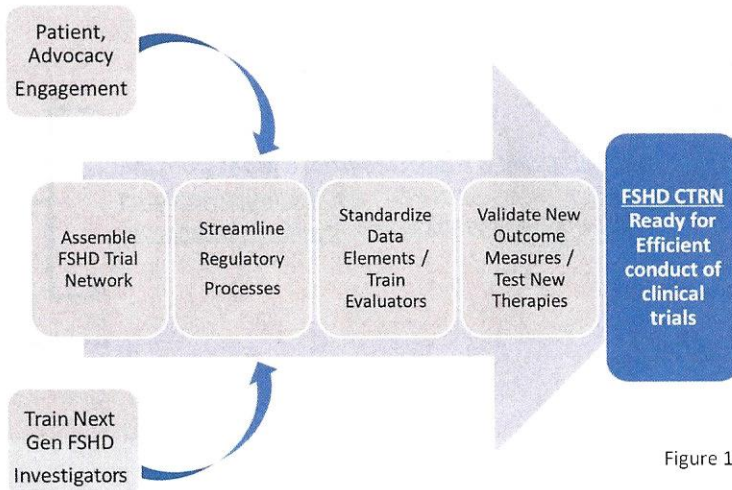


Figure 1

What is the Goal of FSHD CTRN?

The goal of the FSHD CTRN is to hasten therapeutic development – this includes both drugs and therapeutic approaches which may improve quality of life.

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.

Ultimately all information collected as part of the FSHD CTRN will be made available in a de-identified fashion to any researcher or pharmaceutical company with a goal of developing therapies to benefit people with FSHD.

How can I help?

For each CTRN project, we will form committees which include major stakeholders: academic researchers, industry, advocacy groups, and people with FSHD. The CTRN will need volunteers for future focus groups, committees and participants in additional clinical studies.

Currently there is 1 active clinical study in the FSHD CTRN– Clinical Trial Readiness to Solve Barriers to Drug Development in FSHD (ReSolve FSHD). More coming soon!!

If interested in more information or participating in the FSHD CTRN, please contact the FSHD CTRN project manager:

Kiley Higgs

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913-945-9922

Who makes up the FSHD CTRN?

- **University of Washington: Seattle, Washington**
– Dr. Leo Wang
- **University of Utah: Salt Lake City, Utah**
– Dr. Nicholas Johnson
- **University of California Los Angeles: Los Angeles, California** – Dr. Perry Shieh
- **University of Kansas: Kansas City, Kansas**
– Dr. Jeffrey Statland
- **Ohio State University: Columbus, Ohio**
– Dr. Samantha LoRusso
- **University of Rochester: Rochester, New York**
– Dr. Rabi Tawil
- **Kennedy Krieger Institute, Baltimore: Maryland**
– Dr. Kathryn Wagner

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