Transcript Details
This is a transcript of an educational program. Details about the program and additional media formats for the program are accessible by visiting: https://reachmd.com/programs/Audioabstracts/global-infrastructure-fshd-trials/54642/

ReachMD
www.reachmd.com
info@reachmd.com
(866) 423-7849

Strengthening Global Infrastructure for FSHD Trials

Dr. Maeusli:

You're listening to *AudioAbstracts* on ReachMD, and I'm Dr. Mimi Maeusli. As potential therapies for facioscapulohumeral muscular dystrophy, or FSHD, move closer to clinical testing, an important question is emerging: how do we ensure that clinical trial sites around the world are ready to participate?

FSHD is a rare neuromuscular disorder with a growing number of treatments in development. However, research in rare diseases presents unique challenges, including the need to rapidly establish and coordinate trial sites across multiple countries. In a paper published in the *Journal of Neuromuscular Diseases* in October 2025, investigators describe an international initiative designed to address these challenges. The program, called Project Mercury, was launched by the FSHD Society to strengthen the global infrastructure needed to conduct FSHD clinical trials.

Project Mercury brings together researchers, clinicians, industry partners, and patient advocacy groups from around the world. Its organizational structure includes a Global Task Force and several Country Working Groups led by organizations within the World FSHD Alliance. The initiative aims to assemble a global cohort of approximately 10,000 well-characterized patients who are ready for trial participation, expand international research capacity, and address obstacles that can delay patient access to therapies.

An important focus of this effort is supporting clinical centers that have limited prior experience in FSHD research. By sharing expertise and resources, the initiative aims to help these sites develop the capabilities needed to participate in upcoming studies.

Because most clinical studies follow similar operational phases, the authors suggest using a trial roadmap to guide the process. This framework outlines the typical stages of a study, including pre-start-up, start-up, active trial conduct, and study close-out. During the preparation phase, sites complete feasibility assessments, negotiate budgets, and obtain regulatory approvals. Once a study begins, investigators focus on patient recruitment, protocol-driven assessments, and ongoing monitoring. After the trial concludes, activities shift to data analysis, reporting of findings, and secure archiving of study documentation.

Another theme highlighted in the paper is the importance of collaboration with sponsors. Many FSHD trials are initiated by pharmaceutical companies, meaning investigators work closely with sponsors and contracted vendors throughout the study. Successful partnerships require mutual understanding of priorities, such as ensuring participant safety, maintaining efficient enrollment, and generating reliable clinical data.

The authors also provide practical recommendations for new principal investigators. Leading a study requires familiarity with regulatory responsibilities under Good Clinical Practice standards, along with strong financial and operational oversight. Investigators are encouraged to establish clear standard operating procedures, develop relationships with key institutional partners, such as ethics committees, pharmacy teams, and laboratory services, and build a capable research team. Recruiting an experienced study coordinator and maintaining communication with referring clinicians and patient advocacy groups can also support effective patient recruitment.

Several practical considerations are also discussed for the day-to-day execution of FSHD trials. These include engaging patient representatives during the planning stages, maintaining proactive communication with participants and healthcare providers, and ensuring facilities accommodate patients with mobility limitations. Operational adjustments can also improve participant comfort. For example, MRI protocols may be adapted by limiting scan sessions to roughly 30 minutes with scheduled breaks. Additional clinical considerations include using cystatin-C instead of creatinine to evaluate renal function in individuals with muscle wasting, and recognizing that incomplete right bundle branch block may occur in patients with FSHD but is generally considered benign. The authors

also recommend systematically gathering feedback from trial participants and reinforcing strict privacy practices, including advising participants not to share study details on social media to avoid compromising trial integrity.

Looking ahead, the authors outline several ways future FSHD trials could become more inclusive and patient-centered. One priority is increasing diversity and broadening eligibility criteria so study populations better represent the wider FSHD community. This could include enrolling both treatment-naïve individuals and patients previously exposed to investigational or approved therapies, as well as groups frequently excluded from trials, such as presymptomatic individuals, wheelchair users, and patients with D4Z4 repeat size 10. The authors also encourage stronger integration of patient-centered outcomes, including tools like Goal Attainment Scaling and systematic collection of participant feedback regarding the trial experience. Finally, they highlight growing interest in decentralized trial models that incorporate remote monitoring of motor function, as well as the exploration of combination therapeutic approaches.

So with multiple therapies under investigation and potentially thousands of participants expected to enroll in clinical trials over the next several years, expanding global research capacity will be essential. Initiatives such as Project Mercury aim to ensure that clinical trial infrastructure evolves alongside therapeutic innovation.

This has been an *AudioAbstract*, and I'm Dr. Mimi Maeusli. To access this and other episodes in our series, visit ReachMD.com, where you can Be Part of the Knowledge. Thanks for listening!