

Advocacy and Industry Partners Launch New Global Coalition to Speed Delivery of New Therapies for Facioscapulohumeral Muscular Dystrophy (FSHD)

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Project Mercury unites patients, industry, research and clinical communities in groundbreaking initiative

RANDOLPH, Mass. and CAMBRIDGE, Mass., June 20, 2023 (GLOBE NEWSWIRE) -- Today on World FSHD Day, the FSHD Society and Fulcrum Therapeutics (Nasdaq: FULC) announce the launch of Project Mercury, a first-of-its-kind collaboration of global stakeholders committed to identifying and addressing the challenges that could impede the delivery of new medicines for this devastating condition.

Developing and delivering treatments for any rare disease involves unique challenges, including establishing trial outcome measures; enrolling small, and often undiagnosed, patient populations; managing financial risk and incentives; navigating extended diagnostic journeys and fractured care pathways; and generating clear and compelling evidence to support regulatory and reimbursement activities.

"We understand the gaps in improving the lives of rare disease patients, but no patient advocacy organization is capable of solving the myriad of challenges on its own," said Mark Stone, President and CEO, FSHD Society and inaugural chair of the Project Mercury Global Task Force. "By synergizing the expertise, insights and influence of industry, researchers and others, we have greater chance at catalyzing change and meeting the needs of the FSHD community."

Project Mercury consists of collaboration at the global level, through a Global Task Force, and at the local level, through Country Working Groups. Founding Project Mercury Global Task force members include: The FSHD Society, FSHD Canada Foundation and multiple other members of the World FSHD Alliance, Fulcrum Therapeutics, Avidity Biosciences, TREAT-NMD, FSHD patient advocates, leading FSHD clinicians and researchers, and experts in the fields of health economics and patient access.

FSHD is a rare genetic disorder that is estimated to affect 1 in 8,000 people worldwide. People with FSHD experience relentless and accumulating muscle and functional loss, which results in the inability to perform daily life activities due to significant impairment of upper extremity function, loss of mobility, and chronic pain. Typically beginning in the teenage years, around 20 percent of people diagnosed with FSHD will need a wheelchair by age 50. FSHD currently has no effective treatment or cure, however in recent years significant progress has been made, underscored by Fulcrum Therapeutics' initiation of REACH, the first ever Phase 3 clinical trial for FSHD in 2022.

The unrelenting progression of FSHD illustrates the importance of providing rapid and equitable delivery of future treatments. Driven by this sense of urgency, Project Mercury seeks to mobilize all sectors of the FSHD community to unite for the singular purpose of ensuring that the barriers to access are removed, as quickly as possible.

Project Mercury is guided by the principle of shared burden to create shared benefit. Collectively, the members of the Global Task Force aim to accelerate progress in understanding and treating FSHD by:

- building a global cohort of 10,000 clinical-trial-ready, well-characterized patients for participation in clinical trials
- expanding and optimizing the world-wide clinical trial infrastructure
- removing the barriers that delay delivery of therapies once approved

"We are thrilled to be a global sustaining partner in this unique collaboration to ensure that patients benefit from innovation as quickly as possible," said Robert J. Gould, Ph.D., Fulcrum's interim president and chief executive officer. "The Project Mercury model can help align all stakeholders to overcome the barriers and inefficiencies that stand in the way of progress."

Project Mercury's Global Task Force and Country Working Groups are all led by patient advocacy organizations of the World FSHD Alliance. As the co-founding and convening partner, the FSHD Society provides management, resources, and implementation support for the coalition. As a founding and sustaining partner, Fulcrum Therapeutics is supporting Project Mercury by donating resources and support for communications. All members of the Global Task Force commit to sharing resources and dedicating staff to help Project Mercury achieve the greatest impact.

About FSHD

Facioscapulohumeral muscular dystrophy (FSHD) is a genetic disorder characterized by relentless and accumulating muscle and functional loss, which results in the inability to perform daily life activities due to significant impairment of upper extremity function, loss of mobility and chronic pain. FSHD strikes men, women, and children of all races and ethnicities, and strikes an estimated 1 in 8,000 individuals, or almost 1 million people worldwide. Typically beginning in early teenage years with the loss of muscles in the face (facio), shoulders (scapula), upper arms (humerus), legs or core, FSHD can spread to any muscle. Ten percent of FSHD patients develop symptoms before age 10, and around 20 percent will need a wheelchair by age 50. Over 70 percent experience debilitating pain and fatigue. The condition is inherited and can affect many family members across generations. Some 30 percent of FSHD cases arise in families with no prior history.

About Project Mercury

Project Mercury is an open collaboration among stakeholders from across the globe coming together to overcome the challenges that could slow or prevent effective FSHD therapies from getting to patients everywhere. This collaboration takes place at the global level through a Global Task Force and at the local level, through Country Working Groups. The Task Force and the Working Groups are all led by patient advocacy organizations of the World FSHD Alliance. This global-local approach ensures customization of Project Mercury's work at the local level while sharing resources at the global level. Members include: FSHD UK, FSHD Canada Foundation, FSHD Europe, FSHD Global Research Foundation, FSHD Society, FSHD Stichting, Avidity Biosciences, Fulcrum Therapeutics, EURO-NMD, FSHD European Trial Network, TREAT-NMD, various experts in the field of patient access, health economics and leading FSHD clinicians and researchers. More information about Project Mercury can be found at <https://www.projectmercuryfshd.org>.

About The FSHD Society

The FSHD Society is the world's largest research-focused patient organization for facioscapulohumeral muscular dystrophy (FSHD), one of the most prevalent forms of muscular dystrophy. The Society has catalyzed major advancements and is accelerating the development of treatments and a cure to end the pain, disability, and suffering endured by one million people worldwide who live with FSHD. The FSHD Society has transformed the landscape for FSHD research and is committed to making sure that no one faces this disease alone. The Society offers a community of support, news, and information through its website at <https://www.fshdsociety.org>.

About Fulcrum Therapeutics (NASDAQ: FULC)

Fulcrum Therapeutics is a clinical-stage biopharmaceutical company focused on improving the lives of patients with genetically defined rare diseases in areas of high unmet medical need. Fulcrum's two lead programs in clinical development are losmapimod, a small molecule for the treatment of facioscapulohumeral muscular dystrophy (FSHD), and FTX-6058, a small molecule designed to increase expression of fetal hemoglobin for the treatment of sickle cell disease (SCD) and other hemoglobinopathies, which is currently under a full clinical hold issued by the U.S. Food and Drug Administration. The company's proprietary product engine, FulcrumSeek™, identifies drug targets that can modulate gene expression to treat the known root cause and consequences of gene mis-expression. Fulcrum is a founding and sustaining global task force member of Project Mercury. For more information, visit www.fulcrumtx.com and follow us on Twitter @FulcrumTx and LinkedIn. To learn more about the REACH Phase 3 clinical trial, please visit: <https://beta.clinicaltrials.gov/study/NCT05397470>

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this press release are forward-looking statements, including statements regarding Project Mercury's ability to speed delivery of new therapies, improve access to therapies and meet the needs of the FSHD community, among others. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, Fulcrum's ability to continue to advance its product candidates in clinical trials; initiate and enroll clinical trials on the timeline expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; replicate in clinical trials positive results found in preclinical studies and/or earlier-stage clinical trials of losmapimod, FTX-6058 (if resumed) and any other product candidates; obtain, maintain or protect intellectual property rights related to its product candidates; manage expenses; realize the anticipated benefits of the strategic realignment; manage executive and employee turnover; and raise the substantial additional capital needed to achieve its business objectives, among others. For a discussion of other risks and uncertainties, and other important factors, any of which could cause Fulcrum's actual results to differ from those contained in the forward-looking statements, see the "Risk Factors" section, as well as discussions of potential risks, uncertainties, and other important factors, in Fulcrum's most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent Fulcrum's views as of the date hereof and should not be relied upon as representing Fulcrum's views as of any date subsequent to the date hereof. Fulcrum anticipates that subsequent events and developments will cause Fulcrum's views to change. However, while Fulcrum may elect to update these forward-looking statements at some point in the future, Fulcrum specifically disclaims any obligation to do so.

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