

## Fulcrum Therapeutics to Host Key Opinion Leader Breakfast Symposium on Facioscapulohumeral Dystrophy (FSHD), on November 7, 2019

October 31, 2019

CAMBRIDGE, Mass., Oct. 31, 2019 (GLOBE NEWSWIRE) -- [Fulcrum Therapeutics, Inc.](#) (Nasdaq: FULC), a clinical-stage biopharmaceutical company focused on improving the lives of patients with genetically defined rare diseases, today announced that it will host a key opinion leader (KOL) breakfast symposium focused on understanding Facioscapulohumeral Dystrophy (FSHD) genetics, biology, and clinical implications, on Thursday, November 7, 2019 from 8:30 a.m. to 10:30 a.m. ET in New York, NY. Fulcrum is currently conducting Phase 2 trials investigating the safety, tolerability, and efficacy of losmapimod to treat the root cause of FSHD.

Guest speakers scheduled to present at the event include:

- **Kathryn Wagner, M.D., Ph.D.**, Professor of Neurology and Neuroscience at Johns Hopkins School of Medicine and Director of the Center for Genetic Muscle Disorders Kennedy Krieger Institute
- **Peter Jones, Ph.D.**, Mick Hitchcock, Ph.D. Endowed Chair in Medical Biochemistry and Associate Professor of Pharmacology at University of Nevada, Reno School of Medicine
- **Fran Sverdrup, Ph.D.**, Associate Professor of Biochemistry and Molecular Biology at Saint Louis University, School of Medicine

A live webcast of the presentation will be available through the investor relations section of the Company's website at <https://ir.fulcrumtx.com/events-and-presentations>. Following the live webcast, an archived replay will also be available.

### About FSHD

FSHD is characterized by progressive skeletal muscle loss that initially causes weakness in muscles in the face, shoulders, arms and trunk, and progresses to weakness throughout the lower body. Skeletal muscle weakness results in significant physical limitations, including an inability to smile and difficulty using arms for activities, with many patients ultimately becoming dependent upon the use of a wheelchair for daily mobility.

FSHD is caused by mis-expression of DUX4 in skeletal muscle, resulting in the presence of DUX4 proteins that are toxic to muscle tissue. Normally, DUX4-driven gene expression is limited to early embryonic development, after which time the DUX4 gene is silenced. In people with FSHD, the DUX4 gene is turned "on" as a result of a genetic mutation. The result is death of muscle and its replacement by fat, leading to skeletal muscle weakness and progressive disability. There are no approved therapies for FSHD, one of the most common forms of muscular dystrophy, with an estimated patient population of 16,000 to 38,000 in the United States alone.

### About Losmapimod

Losmapimod is a selective p38 $\alpha$ /β mitogen activated protein kinase (MAPK) inhibitor that was exclusively in-licensed by Fulcrum Therapeutics following Fulcrum's discovery of the role of p38 $\alpha$ /β inhibitors in the reduction of DUX4 expression and an extensive review of known compounds. Utilizing its internal product engine, Fulcrum discovered that inhibition of p38 $\alpha$ /β reduced expression of the DUX4 gene in muscle cells derived from patients with FSHD. Although losmapimod has never previously been explored in muscular dystrophies, it has been evaluated in more than 3,500 subjects in clinical trials across multiple other indications, including in several Phase 2 trials and a Phase 3 trial. No safety signals were attributed to losmapimod in any of these trials. Fulcrum is currently conducting Phase 2 trials investigating the safety, tolerability, and efficacy of losmapimod to treat the root cause of FSHD.

### About Fulcrum Therapeutics

Fulcrum Therapeutics is a clinical-stage biopharmaceutical company focused on improving the lives of patients with genetically defined diseases in areas of high unmet medical need, with an initial focus on rare diseases. Fulcrum's proprietary product engine identifies drug targets which can modulate gene expression to treat the known root cause of gene mis-expression. Please visit [www.fulcrumtx.com](http://www.fulcrumtx.com).

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