Fulcrum Therapeutics Announces Multiple Presentations at the 2020 American Academy of Neurology (AAN) Science Highlights Virtual Platform

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CAMBRIDGE, Mass., May 21, 2020 (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 multiple presentations at the 2020 American Academy of Neurology (AAN) Science Highlights Virtual Platform. AAN's annual meeting was cancelled due to COVID-19 and the virtual platform allows for all accepted oral and poster presentations to be presented. The materials linked below will be available for approximately 10 months.

"We are pleased by the breadth of losmapimod data selected for presentation, which highlights the progress we've made to develop a treatment for patients with facioscapulohumeral muscular dystrophy (FSHD) and our approach to treating the root cause of genetically defined rare diseases," said Diego Cadavid, M.D., Fulcrum's senior vice president, clinical development. "In particular, we have identified a set of stable DUX4-regulated gene transcripts that provide a pharmacodynamic biomarker endpoint to measure the treatment effect of losmapimod in FSHD. Additionally, in collaboration with AMRA Medical, we have created a standardized whole-body magnetic resonance imaging (MRI) protocol to evaluate skeletal muscle composition in FSHD patients. I would like to thank the patients and their families who have participated in our trials, and who inspire us every day."

2020 AAN Virtual Platform Presentation and Poster Details

Phase 1 Clinical Trial of Losmapimod in Facioscapulohumeral Muscular Dystrophy (FSHD): Safety, Tolerability, and Target Engagement

https://cslide-us.ctimeetingtech.com/aan2020/attendee/eposter/poster/2192

Design of a Phase 2, Randomized, Double-Blind, Placebo-Controlled, 48-Week, Parallel-Group Study of the Efficacy and Safety of Losmapimod in Treating Subjects with Facioscapulohumeral Muscular Dystrophy (FSHD): ReDUX4

https://cslide-us.ctimeetingtech.com/aan2020/attendee/eposter/poster/2193

Development of an Optimized Timed Up and Go (oTUG) for Measurement of Changes in Mobility Impairment in Facioscapulohumeral Muscular Dystrophy (FSHD) Clinical Trials

https://cslide-us.ctimeetingtech.com/aan2020/attendee/eposter/poster/1638

A Biomarker of DUX4 Activity to Evaluate losmapimod Treatment Effect in FSHD Phase 2 Trials

https://cslide-us.ctimeetingtech.com/aan2020/attendee/eposter/poster/2196

An In-Home Study of Facioscapulohumeral Muscular Dystrophy (FSHD) Patients using Contactless Wireless Sensing and Machine Learning

https://cslide-us.ctimeetingtech.com/aan2020/attendee/eposter/poster/1721

Development and Evaluation of a Whole-body MRI Imaging Protocol and Analysis Algorithms to Measure Changes in Skeletal Muscle in FSHD

https://cslide-us.ctimeetingtech.com/aan2020/attendee/eposter/poster/2176

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 p38α/β mitogen activated protein kinase (MAPK) inhibitor that was exclusively in-licensed from GSK by Fulcrum Therapeutics following Fulcrum's discovery of the role of p38α/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 p38α/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 rare diseases in areas of high unmet medical need. Fulcrum's proprietary product engine identifies drug targets which can modulate gene expression to treat the known root cause of gene mis-expression. The company has advanced losmapimod to Phase 2 clinical development for the treatment of facioscapulohumeral muscular dystrophy (FSHD) and has completed extensive pre-clinical research for FTX-6058 for the treatment of sickle cell disease and beta-thalassemia.

Please visit www.fulcrumtx.com.

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, including statements regarding the development status of the Company’s product candidates. All statements, other than statements of historical facts, contained in this press release, including statements regarding the Company’s strategy, future operations, future financial position, prospects, plans and objectives of management, are forward-looking statements. 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, risks associated with Fulcrum’s ability to obtain and maintain necessary approvals from the FDA and other regulatory authorities; continue to advance its product candidates in clinical trials; replicate in later clinical trials positive results found in preclinical studies and early-stage clinical trials of losmapimod and its other product candidates; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; obtain, maintain or protect intellectual property rights related to its product candidates; manage expenses; and raise the substantial additional capital needed to achieve its business objectives. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the Company’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 the Company’s most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company’s views as of the date hereof and should not be relied upon as representing the Company’s views as of any date subsequent to the date hereof. The Company anticipates that subsequent events and developments will cause the Company’s views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so.

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