Fulcrum Therapeutics Announces U.S. Food and Drug Administration Grants Fast Track Designation to Losmapimod for the Potential Treatment of Facioscapulohumeral Muscular Dystrophy (FSHD)

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CAMBRIDGE, Mass., May 12, 2021 (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 the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to losmapimod for the potential treatment of facioscapulohumeral muscular dystrophy (FSHD).

“There are no approved therapies to treat patients with FSHD, and losmapimod is currently the only drug in clinical development for this serious and debilitating disease,” said Judith Dunn, Ph.D., Fulcrum’s president of research and development. “We are pleased that the FDA has granted Fast Track designation, which we believe demonstrates the potential for losmapimod to address unmet medical needs for people living with FSHD.”

Fast Track Designation is intended to facilitate development and expedite the review of drugs that treat serious conditions so an approved product can reach the market expeditiously. It enables the company to have more frequent interactions with the FDA throughout the drug development process and allows for eligibility for priority review and accelerated approval in certain cases, as well as a rolling review.

Fulcrum is on track to report full data from ReDUX4, a Phase 2b randomized, double-blind, placebo-controlled trial of losmapimod in FSHD patients, at the virtual FSHD International Research Congress taking place June 24-25, 2021. Data will include the primary endpoint, reduction from baseline of DUX4-driven gene expression, as well as a pre-specified sensitivity analysis assessing biopsies with the highest pre-treatment level of DUX4-driven gene expression. Additional data to be reported include secondary endpoints evaluating disease progression via skeletal muscle MRI, exploratory endpoints assessing muscle function measures and patient reported outcomes.

Losmapimod previously received Orphan Drug Designation for FSHD.

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 3,600 subjects in clinical trials across FSHD and 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). Fulcrum has also advanced FTX-6058, a small molecule designed to increase expression of fetal hemoglobin for the treatment of sickle cell disease and beta-thalassemia into Phase 1 clinical development.

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, the potential advantages and therapeutic potential of Fulcrum’s product candidates and availability of clinical trial data. 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; initiate and enroll clinical trials on the timeline expected or at all; correctly estimate the potential patient population and/or market for the Company’s product candidates; replicate in clinical trials positive results found in preclinical studies and/or earlier-stage clinical trials of losmapimod, FTX-6058 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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