Fulcrum Therapeutics Receives Orphan Drug Designation for Losmapimod in Facioscapulohumeral Muscular Dystrophy (FSHD) and Announces Expanded Patent Portfolio

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CAMBRIDGE, Mass., Jan. 29, 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 that the United States Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to losmapimod, a selective p38α/β mitogen activated protein kinase (MAPK) inhibitor for the treatment of facioscapulohumeral muscular dystrophy (FSHD). Fulcrum also announced the issuance of U.S. patent 10,537,560 with claims covering the use of other p38 kinase inhibitors for the treatment of FSHD.

"We are pleased to have been granted Orphan Drug Designation for losmapimod in FSHD as it underscores the critical need for treating this rare muscular dystrophy that has no approved therapies," said Robert J. Gould, Ph.D., Fulcrum's president and chief executive officer. "We believe losmapimod represents a promising, novel approach to treat the known root cause of FSHD and remain on-track to announce data from the Phase 2b ReDUX4 clinical trial in the third quarter of 2020. Our recently issued patent also expands our intellectual property protection relating to the use of other clinical-stage p38 inhibitors for the treatment of FSHD, strengthening our position as a leader in the treatment of genetically defined diseases."

In October 2019, the Company announced preliminary results from a Phase 1 clinical trial of losmapimod in patients with FSHD, which indicated that losmapimod was generally well tolerated and achieved dose-dependent concentrations in plasma and muscle believed to be adequate for efficacy based on preclinical pharmacology studies. Additionally, losmapimod has shown adequate safety and tolerability in over 3,500 patients and healthy volunteers across multiple indications, with no safety signals attributed to the drug in those trials.

The patent announced today is in addition to U.S. patent 10,342,786, which covers the method of using losmapimod for the treatment of FSHD. These two patents each provide protection through 2038.

About Orphan Drug Designation
The FDA Office of Orphan Products Development grants orphan status to support development of medicines that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases that affect fewer than 200,000 people in the U.S. Among the benefits of orphan designation in the U.S. are seven years of market exclusivity following FDA approval if received, exemption of FDA application fees and tax credits for qualified clinical trials. For more information about orphan designation, please visit the FDA website at www.fda.gov.

About Losmapimod
Losmapimod is a selective p38α/β mitogen activated protein kinase (MAPK) inhibitor that was exclusively in-licensed 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 had never previously been explored in muscular dystrophies, it had 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 and the timing of 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; 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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