Fulcrum Therapeutics Enters into a Collaboration and License Agreement with Sanofi for the Development and Commercialization of Losmapimod in Facioscapulohumeral Muscular Dystrophy

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— Sanofi receives exclusive rights to commercialize losmapimod in all territories outside the U.S.; Fulcrum retains full U.S. commercialization rights —

— Fulcrum will receive an upfront payment of $80.0 million, and is eligible to receive $975.0 million in potential milestones, plus royalties on ex-U.S. product sales; parties will share future global development costs 50:50 —

— Conference call and webcast scheduled for 8:00 a.m. ET today to discuss the collaboration and other recent corporate developments, in conjunction with the first quarter 2024 financial results —

CAMBRIDGE, Mass., May 13, 2024 (GLOBE NEWSWIRE) -- Fulcrum Therapeutics, Inc.® (Fulcrum) (Nasdaq: FULC), a clinical-stage biopharmaceutical company focused on developing small molecules to improve the lives of patients with genetically defined rare diseases, today announced that it has entered into a collaboration and license agreement with Sanofi (Nasdaq: SNY) for the development and commercialization of losmapimod, an oral small molecule being investigated for the treatment of facioscapulohumeral muscular dystrophy (FSHD). Under the collaboration and license agreement, Sanofi obtains exclusive commercialization rights for losmapimod outside of the U.S.

The collaboration and license agreement combines Fulcrum's expertise in FSHD with Sanofi's global reach and unparalleled commitment to treating patients with rare diseases. Losmapimod is currently being evaluated in a global Phase 3 clinical trial for the treatment of FSHD, a chronic and progressive genetic muscular disorder that is characterized by significant muscle cell death and fat infiltration into muscle tissue. Results from ReDUX4, the Phase 2 clinical trial evaluating losmapimod for the treatment of FSHD, demonstrated a slowing of disease progression and improved muscle health. Fulcrum expects to report topline data from REACH, the global Phase 3 clinical trial, in the fourth quarter of 2024. Following positive data from the Phase 3 trial, Fulcrum and Sanofi plan to submit marketing applications in the U.S., Europe, Japan, and other geographies.

"Sanofi is a proven leader in developing therapeutics for rare neuromuscular diseases and is the ideal partner to maximize the opportunity and reach of losmapimod outside the U.S.," said Alex C. Sapir, Fulcrum's president and chief executive officer. "This deal aligns with our core strategy, allowing Fulcrum to remain focused on preparations for commercialization of losmapimod in the U.S., while leveraging Sanofi's exceptional global commercial capabilities and established infrastructure in key markets around the world. We are excited about the potential to provide the first approved treatment for FSHD patients, and we look forward to working with Sanofi to bring losmapimod to patients globally."

"This partnership provides an exciting opportunity to expand Sanofi's rare disease franchise and deliver the first approved FSHD treatment to patients with the strength and reach of our commercial organization," said Burcu Eryilmaz, Sanofi's Global Head of Rare Diseases. "Losmapimod has shown meaningful clinical benefits that underscore the disease-modifying potential and opportunity to address the high unmet need for a safe and effective drug that slows disease progression. With a deep commitment to bringing hope and new treatment options to patients, we look forward to working closely with Fulcrum as losmapimod advances through late-stage development."

Per the terms of the agreement, Fulcrum will receive an upfront payment of $80.0 million and is eligible to receive up to an additional $975.0 million in specified regulatory and sales-based milestones, along with tiered escalating royalties starting in the low-teens on annual net sales of losmapimod outside the U.S. In addition, Fulcrum and Sanofi will equally share future global development costs.

Conference Call and Webcast
Individuals may register for the conference call by clicking the link here. Once registered, participants will receive dial-in details and unique PIN which will allow them to access the call. An audio webcast will be accessible through the Investor Relations section of Fulcrum's website at www.fulcrumtx.com or by clicking here. Following the live webcast, an archived replay will also be available.

About Losmapimod
Losmapimod is a selective p38α/β mitogen activated protein kinase (MAPK) inhibitor. Fulcrum exclusively in-licensed losmapimod from GSK following Fulcrum's discovery of the role of p38α/β inhibitors in the reduction of DUX4 expression and an extensive review of known compounds. Results reported from the Phase 2 ReDUX4 trial demonstrated a slowing of disease progression and improved function, including positive impacts on upper extremity strength and functional measures supporting losmapimod's potential to be a transformative therapy for the treatment of FSHD. Although losmapimod had never previously been explored in muscular dystrophies, it had been evaluated in more than 3,600 subjects in clinical trials across multiple other indications, with no safety signals attributed to losmapimod. Losmapimod has been granted U.S. Food and Drug Administration (FDA) Fast Track designation and Orphan Drug Designation for the treatment of FSHD. Losmapimod is currently being evaluated in a Phase 3 multi-center randomized, double-blind, placebo-controlled, 48-week parallel-group study in people with FSHD (NCT05397470).

About FSHD
FSHD is a serious, rare, progressive and debilitating disease for which there are no approved treatments. It is characterized by fat infiltration of skeletal muscle leading to muscular atrophy involving primarily the face, scapula and shoulders, upper arms, and abdomen. Impact on patients includes profound decreases in the ability to perform activities of daily living, loss of upper limb function, loss of mobility and independence and chronic pain. FSHD is one of the most common forms of muscular dystrophy and has an estimated patient population of 30,000 in the United States alone.

About Fulcrum Therapeutics
Fulcrum Therapeutics is a clinical-stage biopharmaceutical company focused on developing small molecules to improve 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 in development for the treatment of facioscapulohumeral muscular dystrophy (FSHD), and pociredir (formerly known as FTX-6058), a small molecule designed to increase expression of fetal hemoglobin and in development for the treatment of sickle cell disease (SCD). Fulcrum uses proprietary technology to identify drug targets that can modulate gene expression to treat the known root cause of gene mis-expression. For more information, visit www.fulcrumtx.com and follow us on Twitter/X (@FulcrumTx) and LinkedIn.

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 express or implied statements regarding Fulcrum's collaboration and license agreement with Sanofi and receipt of the upfront payment thereunder; its ability to receive the milestone and royalty payments thereunder and achieve benefits therefrom; timing of data from REACH and its ability to support submission of marketing applications for losmapimod; and Fulcrum's ability to deliver an FDA-approved therapy for FSHD patients; 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, risks associated with Fulcrum's ability to continue to advance its product candidates in clinical trials; initiating and enrolling clinical trials on the timeline expected or at all; obtaining and maintaining necessary approvals from the FDA and other regulatory authorities; replicating in clinical trials positive results found in preclinical studies and/or earlier-stage clinical trials of losmapimod, pociredir and any other product candidates; obtaining, maintaining or protecting intellectual property rights related to its product candidates; managing expenses; managing executive and employee turnover, including integrating a new CMO; and raising 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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