Fulcrum Therapeutics to Initiate Phase 1 Trial with FTX-6058 for Sickle Cell Disease

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CAMBRIDGE, Mass., Oct. 05, 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 Investigational New Drug application (IND) is now in effect for its Phase 1 trial in healthy adult volunteers with FTX-6058 for sickle cell disease. FTX-6058 is a small molecule designed to increase expression of fetal hemoglobin with the potential to treat hemoglobinopathies such as sickle cell disease and beta-thalassemia.

This Phase 1 trial will evaluate the safety, tolerability and pharmacokinetics of FTX-6058 and will be comprised of four parts. Part A will be a randomized, double-blind, placebo-controlled, single ascending dose (SAD) study in up to six cohorts. Part B will be a randomized, double-blind, placebo-controlled, multiple ascending dose (MAD) study in up to four cohorts dosed once daily for 14 days. Part C will be an open label pilot food effect study in subjects randomized to take FTX-6058 with and without a high-fat meal, and Part D will be an open label study to evaluate the potential of FTX-6058 to induce CYP3A (using midazolam).

"We are pleased to leverage our expertise in the modulation of genetic drivers of disease to expand our clinical development efforts into a third area with sickle cell," said Robert J. Gould, Ph.D., president and chief executive officer of Fulcrum Therapeutics. “We believe FTX-6058 offers a differentiated approach to a potential treatment. A major unmet need remains for many sickle cell patients, and the availability of an effective and safe small molecule treatment option could represent a significant advancement. We are excited about the preclinical data that showed elevations of fetal hemoglobin up to 30% of total hemoglobin. Should this elevation be seen in sickle cell patients, it has the potential to address multiple symptoms, including painful crises and anemia."

About Sickle Cell Disease
Sickle cell disease (SCD) is a genetic disorder of the red blood cells caused by a mutation in the HBB gene. This gene encodes a protein that is a key component of hemoglobin, a protein complex whose function is to transport oxygen in the body. The result of the mutation is less efficient oxygen transport and the formation of red blood cells that have a sickle shape. These sickle shaped cells are much less flexible than healthy cells and can block blood vessels or rupture leading to anemia. SCD patients typically suffer from serious clinical consequences, which may include anemia, pain, infections, stroke, heart disease, pulmonary hypertension, kidney failure, liver disease and reduced life expectancy.

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 Phase 3 for the treatment of COVID-19. Fulcrum is also advancing 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 Company’s initiation of a clinical trial and evaluation, and the potential benefits, of FTX-6058 as a potential treatment for sickle cell disease, the development status of the Company’s product candidates and the potential advantages and therapeutic potential 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; 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 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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