

# Fulcrum Therapeutics Announces Completion of IND-enabling Safety Studies for FTX-6058 in Sickle Cell Disease

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CAMBRIDGE, Mass., April 13, 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 it is on track for submission of an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) in the second half of 2020 for FTX-6058 following the completion of preclinical safety studies and Good Laboratory Practices (GLP) toxicology work.

"Completed preclinical studies illustrate that FTX-6058, a small molecule upregulator of fetal hemoglobin, has the potential to provide distinct advantages over biologics and gene therapies currently being used or developed for the treatment of sickle cell disease," said Owen B. Wallace, Ph.D., Fulcrum's chief scientific officer. "Based on its attractive profile in preclinical *in vitro* and *in vivo* models and its preclinical safety profile, we believe that FTX-6058 has the potential to be an effective oral therapy for people living with sickle cell disease."

In pre-clinical studies, treatment with FTX-6058 was shown to significantly increase HbF levels up to approximately 30% of total hemoglobin as measured by HPLC and mass spectrometry methods in erythroid progenitor cells from multiple human donors. FTX-6058 also elevated HbF *in vivo* in efficacy models at plasma concentrations reasonably expected to be achieved in humans. Fulcrum believes these results indicate that FTX-6058 could play a role in alleviating the burden of disease in people living with this devastating disorder.

## 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 cells. 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 has completed extensive pre-clinical research for FTX-6058 for the treatment of sickle cell disease and beta-thalassemia.

Please visit [www.fulcrumtx.com](http://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, including the timing of submission of the Company's IND. 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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