

Fulcrum Therapeutics to Present Initial Data from Phase 1b Trial of FTX-6058 in Adults Living with Sickle Cell Disease at the European Hematology Association (EHA) Hybrid Congress in Vienna, Austria

May 12, 2022 at 5:09 PM EDT

- FTX-6058 is the only oral hemoglobin F (HbF) inducer in clinical development

-Data will include initial HbF changes, safety, tolerability, pharmacokinetics, and other pharmacodynamic measures from the ongoing 6 mg dose cohort of FTX-6058 in adults with sickle cell disease (SCD)

- Investor webcast to be hosted on June 10th at 8 a.m. EDT

CAMBRIDGE, Mass., May 12, 2022 (GLOBE NEWSWIRE) -- [Fulcrum Therapeutics, Inc.](https://www.fulcrumtx.com)[®] (Nasdaq: FULC), a clinical-stage biopharmaceutical company focused on improving the lives of patients with genetically defined rare diseases, today announced that it will be presenting two posters at the European Hematology Association (EHA) Hybrid Congress which will take place June 9-12, 2022, in Vienna, Austria. A third abstract has also been accepted for publication. The accepted abstracts are now available online through the EHA website at www.ehaweb.org.

"HbF is the only mechanism that has shown the ability to broadly improve clinical outcomes for patients with SCD— including anemia, vaso-occlusive crises, pain, fatigue, and acute chest syndrome," said Judy Dunn, Ph.D., president of research and development at Fulcrum. "This Phase 1b study was designed to provide proof-of-concept that FTX-6058 produces increases in HbF and could potentially be the first oral HbF inducer to address critical unmet needs in this population."

- FTX-6058 is an investigational oral small-molecule Embryonic Ectoderm Development Inhibitor (EEDI) that has demonstrated robust induction of fetal hemoglobin (HbF) in human cells and animal models of sickle cell disease (SCD).
- Clinically, in a multiple ascending dose (MAD) Phase 1 study in healthy volunteers, FTX-6058 induced HbG (hemoglobin subunit γ) mRNA in a dose-dependent manner. Translation of HbG mRNA is responsible for the production of HbF protein.

In addition to the poster presentation, Fulcrum will host a virtual investor event on June 10th at 8 a.m. EDT to review these initial FTX-6058 data and provide a program update.

Poster Presentation Information

Date: June 10, 2022

Time: 4:30 p.m. CEST/10:30 a.m. EDT

Title: *Interim results of safety, tolerability, pharmacokinetics, and pharmacodynamics from an ongoing open-label study investigating FTX-6058 in adults living with sickle cell disease*

Presenter: Julie Kanter, M.D., Director of the UAB Adult Sickle Cell Clinic and Associate Professor of Hematology and Oncology

Title: *Inhibition of polycomb repressive complex 2 through EED induces fetal hemoglobin in healthy and sickle cell disease models*

Presenter: Billy Stuart, Ph.D., Fulcrum Therapeutics

Publication Information

Title: *A Systematic Literature Review Describing Protective Effects of HbF in Sickle Cell Disease Outcomes*

Authors: Olga Mitelman, J. Barry, J. Bernhard, P. Bruno, C. Morabito, S. Snedecor, S. Ronnebaum

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 two lead programs in clinical development are losmapimod, a small molecule for the treatment of facioscapulohumeral muscular dystrophy (FSHD), and FTX-6058, a small molecule designed to increase expression of fetal hemoglobin for the treatment of sickle cell disease and other hemoglobinopathies, including beta-thalassemia. Fulcrum's proprietary product engine, FulcrumSeek[™], identifies 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 @FulcrumTx and [LinkedIn](https://www.linkedin.com/company/fulcrum-therapeutics).

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 statements regarding the planned REACH trial including its expected start date and enrollment target, presentation of data from first dose cohort in Phase 1b trial of FTX-6058 and the second dose cohort, the clinical development plan for FTX-6058 as well as timing for expansion into other hemoglobinopathies and initiation of registrational trial for sickle cell disease, nomination of additional development candidates and timing of fourth IND, the sufficiency of Fulcrum's cash resources, losmapimod's potential as a therapy for FSHD and the ability of the selected endpoints of the REACH trial to support regulatory approval. 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; initiate and enroll clinical trials on the timeline expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; 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; 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 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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