Fulcrum Therapeutics to Present New Clinical Data from the PIONEER trial of Pociredir in Sickle Cell Disease at the 67th American Society of Hematology Annual Meeting

November 3, 2025 at 4:45 PM EST

— Pociredir Phase 1b PIONEER Trial Data in Sickle Cell Disease to be Presented and Published, Including 12 mg and 20 mg Cohorts —

— Live and Webcast Investor Event with Fulcrum Leadership and Medical Experts will be Hosted Onsite on Sunday, December 7 —

CAMBRIDGE, Mass., Nov. 03, 2025 (GLOBE NEWSWIRE) -- Fulcrum Therapeutics, Inc.® (the “Company”) (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 new data from the Phase 1b PIONEER trial of pociredir in sickle cell disease (SCD) will be presented at the 67th American Society of Hematology (ASH) Annual Meeting. Additionally, Fulcrum will present preclinical data highlighting its calmodulin pathway modulator program for the potential treatment of bone marrow failure syndromes.

“We are looking forward to presenting new clinical data from the PIONEER trial at ASH, including full data from the 12 mg dose cohort and initial data from the 20 mg dose cohort,” said Alex C. Sapir, Fulcrum’s President and Chief Executive Officer. “These data will further characterize pociredir’s potential as a disease-modifying therapy for sickle cell disease, and we look forward to discussing the results in greater detail during our investor event at ASH.”

Presentation details are as follows:

Title: Pociredir, a novel oral once-daily fetal hemoglobin inducer: Results from the Phase 1b PIONEER study in adult participants with severe sickle cell disease and hydroxyurea intolerance or unresponsiveness
Format: Poster
ID: 1157
First Author: Dr. Sheinei Alan, UVA School of Medicine
Presentation Session Date and Time: Saturday, December 6, 2025, between 5:30 – 7:30 PM ET

Title: First-in-class small molecule calmodulin pathway modulators attenuate excess p53 activity and correct erythropoietic defects in models of diamond-blackfan anemia (DBA)
Format: Poster
ID: 1441
First Author: Avik Choudhuri, Senior Scientist, Hematology, Fulcrum
Presentation Session Date and Time: Saturday, December 6, 2025, between 5:30 – 7:30 PM ET

In addition, the pociredir poster has been selected for inclusion in the Poster Walk on Novel and Emerging Therapeutics in Erythrocyte and Iron Disorders Hosted by Blood Red Cells & Iron, which will take place on Sunday, December 7, from 11:15 a.m. to 12:15 p.m. ET. The ASH Poster Walks highlight cutting-edge emerging science in hematology presented in the poster sessions during the meeting.

Following congress publication, the posters will be available on the Publications & Presentations Page of Fulcrum’s website at https://www.fulcrumtx.com/publications-presentations.

Investor Event at ASH 2025
Fulcrum will host a live and webcast investor event featuring company leadership and medical experts on Sunday, December 7, 2025, at 7:00 a.m. ET in Orlando. A live webcast will be accessible on the Investor Relations section of Fulcrum’s website (www.fulcrumtx.com) under Events and Presentations, with a recording available following the event. Further details on specific location to follow.

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 lead clinical program is pociredir, a small molecule designed to increase expression of HbF for the treatment of 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 X (@FulcrumTx) and LinkedIn.

About Pociredir
Pociredir is an investigational oral small-molecule inhibitor of EED that was discovered using Fulcrum’s proprietary discovery technology. Inhibition of EED leads to potent downregulation of key fetal globin repressors, including BCL11A, thereby causing an increase in HbF. Pociredir is being developed for the treatment of SCD. Initial data in SCD in the PIONEER Phase 1b clinical trial showed proof-of-concept and achieved absolute levels of HbF increases associated with potential overall patient benefit. Through the completion of the 12 mg dose cohort, pociredir was demonstrated to be generally well-tolerated in people with SCD with up to three months of exposure, with no treatment-related SAEs reported. Pociredir has been granted FDA Fast Track designation and Orphan Drug Designation for the treatment of SCD. To learn more about clinical trials of pociredir please visit ClinicalTrials.gov.

About 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. People with SCD 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.

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