# Fulcrum Therapeutics Announces Recent Business Highlights and Financial Results for First Quarter 2025

May 1, 2025 at 7:00 AM EDT

*— Enrollment complete in the 12 mg dose cohort (n=16) of the Phase 1b PIONEER trial of pociredir in sickle cell disease (SCD); initiated the 20 mg dose cohort —*

*— On track to provide clinical data from the 12 mg dose cohort in early Q3 2025 and the 20 mg dose cohort by the end of 2025 —*

*— Ended Q1 2025 with $226.6 million in cash, cash equivalents, and marketable securities; cash runway into at least 2027 —*

*— Conference call and webcast scheduled for 8:00 a.m. ET today —*

CAMBRIDGE, Mass., May 01, 2025 (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 reported financial results for the first quarter of 2025 and provided a business update.

“We are pleased with the continued momentum of our lead clinical program, pociredir, in development for the treatment of sickle cell disease, including the recent initiation of the 20 mg dose cohort” said Alex C. Sapir, Fulcrum’s president and chief executive officer. “The level of engagement that we’ve observed in the PIONEER trial is highly encouraging, with 16 patients enrolled in the 12 mg dose cohort and enrollment underway in the 20 mg dose cohort. We look forward to sharing data from the 12 mg dose cohort in early Q3 2025, and from the 20 mg dose cohort by the end of 2025. We believe that pociredir has the potential to increase fetal hemoglobin to levels that could ameliorate SCD symptomology and transform the standard of care with a once daily oral treatment option.”

**Recent Business Highlights**

- Patient enrollment continues to progress in the Phase 1b PIONEER trial evaluating pociredir in patients with SCD. Enrollment is complete in the 12 mg dose cohort (n=16) with greater than 90% rates of adherence to study drug and no patient discontinuations to date. Based on the recommendation of the data monitoring committee, Fulcrum has initiated the 20 mg dose cohort and plans to share data from the 12 mg dose cohort in early Q3 2025 and from the 20 mg dose cohort by the end of 2025.
- Two abstracts have been accepted for presentation at the 2025 European Hematology Association (EHA) Congress in Milan, Italy, taking place on June 12-15, 2025. The abstracts, which will be published online on May 15, 2025, highlight preclinical target engagement and gene expression reversibility data of pociredir and clinical data from our previously completed Phase 1 healthy volunteer study.
- Fulcrum continues to advance its program for the potential treatment of inherited aplastic anemias, such as Diamond-Blackfan anemia (DBA), Shwachman-Diamond syndrome, and Fanconi anemia, and plans to submit an investigational new drug application (IND) for DBA during the fourth quarter of 2025.
- Appointed Dae Gon Ha, Ph.D., as senior vice president, head of strategy and business development effective May 1, 2025.

**First Quarter 2025 Financial Results**

- **Cash Position:** As of March 31, 2025, cash, cash equivalents, and marketable securities were $226.6 million, as compared to $241.0 million as of December 31, 2024. The decrease of $14.4 million is primarily due to cash used to fund operating activities in 2025.
- **R&D Expenses:** Research and development expenses were $13.4 million for the three months ended March 31, 2025, as compared to $19.8 million for the three months ended March 31, 2024. The decrease of $6.4 million was primarily due to decreased costs associated with the discontinuation of our losmapimod program and the reimbursement from the global development cost sharing under the collaboration with Sanofi, partially offset by increased costs related to the advancement of the Phase 1b PIONEER trial of pociredir.
- **G&A Expenses:** General and administrative expenses were $7.0 million for the three months ended March 31, 2025, as compared to $10.1 million for three months ended March 31, 2024. The decrease of $3.1 million was primarily due to decreased employee compensation costs as a result of the reduction in workforce implemented in the third quarter of 2024.
- **Net Loss:** Net loss was $17.7 million for the three months ended March 31, 2025, as compared to a net loss of $26.9 million for the three months ended March 31, 2024.

**Cash Runway Guidance**

Based on its current operating plans, Fulcrum continues to expect that its current cash, cash equivalents, and marketable securities will be sufficient to fund its operating requirements into at least 2027.

**Conference Call and Webcast**

Fulcrum Therapeutics, Inc. will host a conference call and webcast today at 8:00 a.m. ET to review the first quarter 2025 financial results and recent business highlights. 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 the company's website at www.fulcrumtx.com or by clicking here. Following the live webcast, an archived replay will also be available.

**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 fetal hemoglobin (HbF) 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.

**About Pociredir**

Pociredir is an investigational oral small-molecule inhibitor of Embryonic Ectoderm Development (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 fetal hemoglobin (HbF). Pociredir is being developed for the treatment of SCD. Initial data in SCD demonstrated proof-of-concept and achieved absolute levels of HbF increases associated with potential overall patient benefit. In clinical trials conducted prior to the clinical hold, which was lifted by the FDA in August 2023, pociredir was generally well-tolerated in people with SCD with up to three months of exposure, with no serious treatment-related adverse events reported. Pociredir has been granted FDA Fast Track designation and Orphan Drug Designation for the treatment of SCD. To learn more about these trials please visit ClinicalTrials.gov.

**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. 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.

**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 Phase 1b PIONEER clinical trial of pociredir, including enrollment progress, number of patients per cohort and planned data announcement for such trial; the potential of pociredir to increase HbF to levels that could ameliorate symptoms of SCD and transform the standard of care; Fulcrum's ability to progress its early stage development programs and planned IND filings related thereto; and its projected cash runway, 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; obtaining, maintaining or protecting intellectual property rights related to its product candidates; managing expenses; realize the anticipated benefits of the workforce reduction and strategic realignment and managing risks associated therewith; 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.

**Fulcrum Therapeutics, Inc.**
**Selected Consolidated Balance Sheet Data**
**(In thousands)**
**(Unaudited)**

| | March 31, 2025 | December 31, 2024 |
|---|---|---|
| Cash, cash equivalents, and marketable securities | $ 226,603 | $ 241,021 |
| Working capital[(1)] | 224,456 | 238,879 |
| Total assets | 242,649 | 260,718 |
| Total stockholders' equity | 228,486 | 243,034 |

(1) Fulcrum defines working capital as current assets minus current liabilities.

**Fulcrum Therapeutics, Inc.**
**Consolidated Statements of Operations**
**(In thousands, except per share data)**

**(Unaudited)**

| | Three Months Ended March 31, | |
|---|---|---|
| | **2025** | **2024** |
| Operating expenses: | | |
| Research and development | 13,404 | 19,773 |
| General and administrative | 6,999 | 10,061 |
| Total operating expenses | 20,403 | 29,834 |
| Loss from operations | (20,403) | (29,834) |
| Other income, net | 2,748 | 2,964 |
| Net loss | $ (17,655) | $ (26,870) |
| Net loss per share, basic and diluted | $ (0.28) | $ (0.43) |
| Weighted-average common shares outstanding, basic and diluted | 62,479 | 61,984 |

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