Fulcrum Therapeutics Announces Recent Business Highlights and Financial Results for Fourth Quarter and Full Year 2025

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— Announced positive 12-week results from the 20 mg dose cohort of the Phase 1b PIONEER trial of pociredir in sickle cell disease (SCD) —

— 20 mg cohort demonstrated rapid and robust fetal hemoglobin (HbF) induction, with mean absolute HbF increasing by 12.2% from a baseline of 7.1% to 19.3% at Week 12, improvements in markers of hemolysis and anemia, and encouraging trends in vaso-occlusive crisis (VOC) reduction —

— Fulcrum plans to initiate a potential registration-enabling trial in the second half of 2026 —

— Ended 2025 with $352.3 million in cash, cash equivalents, and marketable securities; cash runway into 2029 —

CAMBRIDGE, Mass., Feb. 24, 2026 (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 fourth quarter and full year 2025 and provided a business update.

“Building on the previously reported interim data presented in December at ASH, the positive 12-week data from the complete 20 mg cohort of the PIONEER trial reinforce our conviction in pociredir’s potential to address the underlying biology of sickle cell disease,” said Alex C. Sapir, Fulcrum’s President and Chief Executive Officer. “The magnitude of HbF induction, progression toward pan-cellular distribution, and improvements in markers of hemolysis and anemia observed to date position us well as we prepare for discussions with the FDA regarding the design of the next study. With a strong balance sheet extending our cash runway into 2029, we believe we are well positioned to continue to advance pociredir and our broader benign hematology pipeline.”

Recent Business Highlights

- Announced positive 12-week results from the 20 mg dose cohort (n=12) of the Phase 1b PIONEER trial in SCD, building upon previously reported interim data presented at the 2025 American Society of Hematology (ASH) Annual Meeting. Mean absolute HbF increased by 12.2% from a baseline of 7.1% to 19.3% at Week 12. Results demonstrated progression toward pan-cellular HbF induction, improvements in markers of hemolysis and anemia, and encouraging trends in VOC reduction. Pociredir continued to be generally well-tolerated, with no treatment-related serious adverse events reported as of the December 23, 2025 data cutoff.
- In December 2025, completed an underwritten public offering of common stock and pre-funded warrants raising $164.2 million of net proceeds, strengthening Fulcrum's balance sheet to support advancement of pociredir through the next phase of development among other general corporate purposes.
- Fulcrum expects to provide additional details regarding the design of its next trial in the second quarter of 2026 following receipt of meeting minutes from its End-of-Phase meeting with the FDA. Pending feedback from the FDA, Fulcrum plans to initiate a potential registration-enabling trial in the second half of 2026.
- Activating sites for an open label extension trial for participants in the PIONEER trial to evaluate the safety and durability of response with pociredir.
- Based on results from IND-enabling studies, Fulcrum has decided not to advance its program for bone marrow failure syndromes into clinical development and will focus its resources on advancing pociredir and its core benign hematology programs.

Fourth Quarter and Full Year 2025 Financial Results

- **Cash Position:** As of December 31, 2025, cash, cash equivalents, and marketable securities were $352.3 million, as compared to $241.0 million as of December 31, 2024. The increase of $111.3 million is primarily due to net proceeds of $164.2 million from the December 2025 public offering of our common stock and pre-funded warrants, partially offset by cash used to fund operating activities in 2025.
- **R&D Expenses:** Research and development expenses were $15.4 million for the three months ended December 31, 2025, as compared to $11.7 million for the three months ended December 31, 2024. The increase of $3.7 million was primarily due to increased costs related to the advancement of the PIONEER trial.

Research and development expenses were $56.1 million for the year ended December 31, 2025, as compared to $63.4 million for the year ended December 31, 2024. The decrease of $7.3 million was primarily due decreased costs associated with the discontinuation of our losmapimod program and the reimbursement from the global development cost sharing under the now-terminated collaboration with Sanofi, partially offset by increased costs related to the advancement of the PIONEER trial.

- **G&A Expenses:** General and administrative expenses were $7.3 million for the three months ended December 31, 2025, as compared to $7.7 million for three months ended December 31, 2024. The decrease of $0.4 million was primarily due to decreased professional services costs.

General and administrative expenses were $28.7 million for the year ended December 31, 2025, as compared to $36.4 million for the year ended December 31, 2024. The decrease of $7.7 million was primarily due to decreased professional services costs and decreased employee compensation costs as a result of the reduction in workforce implemented in the third quarter of 2024.

- **Net Loss:** Net loss was $20.3 million for the three months ended December 31, 2025, as compared to a net loss of $16.6 million for the three months ended December 31, 2024.

Net loss was $74.9 million for the year ended December 31, 2025, as compared to $9.7 million for the year ended December 31, 2024.

Cash Runway Guidance

Based on its current operating plans, Fulcrum now expects that its current cash, cash equivalents, and marketable securities will be sufficient to fund its operating requirements into 2029.

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 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 HbF. Pociredir is being developed for the treatment of SCD. In the PIONEER Phase 1b clinical trial in people with SCD, pociredir has demonstrated dose-dependent increases in HbF, pan-cellular HbF induction, and improvements in markers of hemolysis and anemia. Across the 12 mg and 20 mg dose cohorts, pociredir has been generally well-tolerated with up to three months of exposure, with no treatment-related serious adverse events reported through the December 23, 2025 data cutoff date. Pociredir has been granted Fast Track and Orphan Drug Designation from the FDA 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.

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 clinical development of pociredir, including an open-label extension trial and receiving feedback from regulators on trial design and commencing a registrational 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 enable IND filings related thereto; and Fulcrum's 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, including progressing early stage candidates into the clinic; initiating and enrolling clinical trials on the timeline expected or at all; including receiving feedback from, and 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; 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)

	December 31, 2025	December 31, 2024
Cash, cash equivalents, and marketable securities	$ 352,306	$ 241,021
Working capital[(1)]	344,432	238,879
Total assets	366,284	260,718

Total stockholders' equity	349,000	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 December 31,		Year Ended December 31,	
	2025	**2024**	**2025**	**2024**
Collaboration revenue	—	—	—	80,000
Operating expenses:				
Research and development	15,416	11,713	56,103	63,386
General and administrative	7,277	7,716	28,666	36,448
Restructuring expenses	—	—	—	2,063
Total operating expenses	22,693	19,429	84,769	101,897
Loss from operations	(22,693)	(19,429)	(84,769)	(21,897)
Other income, net	2,359	2,861	9,889	12,172
Net loss	$ (20,334)	$ (16,568)	$ (74,880)	$ (9,725)
Net loss per share, basic and diluted	$ (0.31)	$ (0.31)	$ (1.18)	$ (0.16)
Weighted-average common shares outstanding, basic and diluted	65,781	53,954	63,355	61,984

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