Fulcrum Therapeutics Announces Recent Business Highlights and Financial Results for Second Quarter 2023

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— Screening closed for the Phase 3 REACH pivotal trial of losmapimod in facioscapulohumeral muscular dystrophy (FSHD) —

— Expect to report topline data for REACH in the fourth quarter of 2024 —

— Interactions continue with the U.S. Food and Drug Administration (FDA) to resolve clinical hold for FTX-6058 in sickle cell disease (SCD) —

— Alan A. Musso appointed as chief financial officer —

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

CAMBRIDGE, Mass., Aug. 03, 2023 (GLOBE NEWSWIRE) -- Fulcrum Therapeutics, Inc.® (the “Company”) (Nasdaq: FULC), a clinical-stage biopharmaceutical company focused on improving the lives of patients with genetically defined rare diseases, today reported financial results for the second quarter of 2023 and provided a business update.

“We are encouraged by the continued progress in the first half of 2023 and look forward to continuing to execute on our key priorities for our clinical programs,” said Alex C. Sapir, Fulcrum’s president and chief executive officer. “We closed screening for the Phase 3 REACH trial of losmapimod, and we expect to report topline data in the fourth quarter of 2024. This marks a critical milestone that brings us one step closer to potentially delivering the first FDA-approved therapy for the treatment of FSHD. In parallel, we continue to work diligently with the FDA to resolve the clinical hold for FTX-6058 as soon as possible.”

Recent Business Highlights

- Screening is closed in the REACH Phase 3 pivotal trial evaluating losmapimod in FSHD at sites in the United States, Canada, and Europe. Fulcrum expects to complete enrollment in the third quarter of 2023 and expects to report topline data in the fourth quarter of 2024.
- In partnership with the FSHD Society, Fulcrum announced the launch of Project Mercury, a new global coalition to accelerate the delivery of new therapies for FSHD by uniting and mobilizing multiple sectors of the FSHD community, including advocates, patients, industry leaders, researchers, and clinicians. Fulcrum is the global and sustaining sponsor of Project Mercury.
- Interactions with the FDA to resolve the clinical hold for FTX-6058 are ongoing.
- Obtained an exclusive global license from CAMP4 Therapeutics Corp. (CAMP4) to acquire intellectual property arising from CAMP4’s pre-clinical research program in Diamond-Blackfan Anemia (DBA). Under the terms of the agreement, Fulcrum will advance the discovery, development, and commercialization of novel therapeutic agents against an undisclosed target for the potential treatment of DBA.
- Alex C. Sapir appointed as president and chief executive officer and member of Fulcrum’s board of directors, effective July 1, 2023. Robert J. Gould, Ph.D., transitioned from his role as interim chief executive officer and president and will continue to serve as a member of the board of directors. In addition, Dr. Gould will serve as chair of the science and technology committee.
- Alan A. Musso appointed as chief financial officer effective August 7, 2023.

Second Quarter 2023 Financial Results

- **Cash Position:** As of June 30, 2023, cash, cash equivalents, and marketable securities were $278.2 million, as compared to $202.9 million as of December 31, 2022.
- **Collaboration Revenue:** Collaboration revenue was $0.9 million for the second quarter of 2023 as compared to $1.9 million for the second quarter of 2022.
- **R&D Expenses:** Research and development expenses were $17.8 million for the second quarter of 2023 as compared to $25.0 million for the second quarter of 2022. The decrease of $7.2 million was primarily associated with a $5.0 million milestone achieved during the second quarter of 2022 due to GlaxoSmithKline plc upon the initiation of REACH and decreased costs for FTX-6058 as a result of the clinical hold.
- **G&A Expenses:** General and administrative expenses were $10.3 million for the second quarter of 2023 as compared to $11.1 million for the second quarter of 2022. The decrease of $0.8 million was primarily due to decreased professional services costs.
- **Net Loss:** Net loss was $23.8 million for the second quarter of 2023 as compared to $34.1 million for the second quarter of 2022.

Financial Guidance

Fulcrum expects that its existing cash, cash equivalents, and marketable securities will be sufficient to fund its operating expenses and capital expenditure requirements into mid-2025.

Conference Call and Webcast

Fulcrum Therapeutics, Inc. will host a conference call and webcast today at 8:00 a.m. ET to review the second quarter and 2023 recent business highlights and financial results. Individuals may register for the conference call by clicking the link here. Once registered participants will receive dial-in details and a unique pin which will allow them to access the call. The webcast will be accessible through the Investor Relations section of Fulcrum's website at www.fulcrumtx.com or by clicking here. Following the live webcast, an archived replay will also be available for 90 days.

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 (SCD) and other hemoglobinopathies, which is currently under a full clinical hold issued by the U.S. Food and Drug Administration. The company'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**.

About FTX-6058
FTX-6058 is an investigational oral small-molecule inhibitor of Embryonic Ectoderm Development (EED) that was discovered using FulcrumSeek™, Fulcrum's proprietary discovery engine. Inhibition of EED leads to potent downregulation of key fetal globin repressors, including BCL11A, thereby causing an increase in fetal hemoglobin (HbF). FTX-6058 is being developed for the treatment of sickle cell disease (SCD) and other hemoglobinopathies. Initial data in SCD demonstrated proof-of-concept and achieved absolute levels of HbF increases associated with potential overall patient benefit. Through the March 2023 data cutoff date, FTX-6058 has been generally well-tolerated in people with SCD with up to three months of exposure with no serious treatment-emergent adverse events reported. FTX-6058 has been granted U.S. Food and Drug Administration (FDA) Fast Track designation and Orphan Drug Designation for the treatment of SCD. FTX-6058 is currently under a full clinical hold issued by the FDA.

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.

About Losmapimod
Losmapimod is a selective p38α/β mitogen activated protein kinase (MAPK) inhibitor. Fulcrum exclusively in-licensed losmapimod from GSK following Fulcrum's discovery of the role of p38α/β inhibitors in the reduction of DUX4 expression and an extensive review of known compounds. Results reported from the Phase 2b ReDUX4 trial demonstrated slowed disease progression and improved function, including positive impacts on upper extremity strength and functional measures supporting losmapimod's potential to be a transformative therapy for the treatment of FSHD. Although losmapimod had never previously been explored in muscular dystrophies, it had been evaluated in more than 3,600 subjects in clinical trials across multiple other indications with no safety signals attributed to losmapimod. Losmapimod has been granted U.S. Food and Drug Administration (FDA) Fast Track designation and Orphan Drug Designation for the treatment of FSHD. Losmapimod is currently being evaluated in a Phase 3 multi-center randomized, double-blind, placebo-controlled, 48-week parallel-group study in people with FSHD (NCT05397470).

About FSHD
FSHD is a serious, rare, progressive and debilitating disease for which there are no approved treatments. It is characterized by fat infiltration of skeletal muscle leading to muscular atrophy involving primarily the face, scapula and shoulders, upper arms, and abdomen. Impact on patients includes relentless and accumulating muscle and functional loss impacting their ability to perform activities of daily living, loss of upper limb function, loss of mobility and independence, and chronic pain. FSHD is one of the most common forms of muscular dystrophy and has an estimated patient population of 16,000 to 38,000 in the United States alone.

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 trials, including completion of enrollment in REACH and timing of topline data; Fulcrum's ability to deliver an FDA-approved therapy for FSHD patients; the clinical hold on FTX-6058, including Fulcrum's interactions with the FDA and ability to resolve such hold; Fulcrum's activities under its recent license agreement with CAMP4; and Fulcrum's 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 resolving the clinical hold on FTX-6058 and responding to FDA's requests; 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 of losmapimod, FTX-6058 (if resumed) and any other product candidates; obtaining, maintaining or protecting intellectual property rights related to its product candidates; managing expenses; managing executive and employee turnover, including integrating a new CEO and CFO; 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)

	June 30, 2023	December 31, 2022
Cash, cash equivalents, and marketable securities	$ 278,164	$ 202,921
Working capital[(1)]	268,143	190,794
Total assets	300,332	226,685
Total stockholders' equity	275,428	198,942

(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 June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Collaboration revenue	$ 880	$ 1,882	$ 1,175	$ 4,474
Operating expenses:				
Research and development	17,849	25,019	34,564	42,850
General and administrative	10,323	11,098	21,843	21,857
Total operating expenses	28,172	36,117	56,407	64,707
Loss from operations	(27,292)	(34,235)	(55,232)	(60,233)
Other income, net	3,509	165	6,670	235
Net loss	$ (23,783)	$ (34,070)	$ (48,562)	$ (59,998)
Net loss per share, basic and diluted	$ (0.38)	$ (0.83)	$ (0.80)	$ (1.47)
Weighted-average common shares outstanding, basic and diluted	61,794	40,890	60,764	40,768

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