

# Fulcrum Therapeutics Reports Recent Business Highlights and Fourth Quarter and Full Year 2020 Financial Results

March 4, 2021 at 7:01 AM EST

- Company on track to present data from Phase 2b ReDUX4 trial with losmapimod in facioscapulohumeral muscular dystrophy (FSHD) in late-2Q 2021 –
- On track to report results from Phase 1 trial in healthy adult volunteers with FTX-6058 for sickle cell disease in mid-2021 –
- Company to discontinue Phase 3 COVID-19 trial (LOSVID) –
- Extended cash runway into 4Q 2022; raised \$50.6 million in gross proceeds from January 2021 public offering –
- Conference call scheduled for 8:00 a.m. ET today –

CAMBRIDGE, Mass., March 04, 2021 (GLOBE NEWSWIRE) -- Fulcrum Therapeutics, Inc. (Nasdaq: FULC), a clinical-stage biopharmaceutical company focused on improving the lives of patients with genetically defined rare diseases, today provided a business update and reported financial results for the fourth quarter and full year of 2020.

"In 2020, we made meaningful progress in advancing our pipeline despite the extraordinary challenges brought on by COVID-19," said Robert J. Gould, Ph.D., president and chief executive officer. "We have laid the foundation to achieve several key milestones in 2021, including a comprehensive assessment of our Phase 2 ReDUX4 trial with losmapimod in facioscapulohumeral muscular dystrophy late in the second quarter and completing our Phase 1 trial in healthy adult volunteers with FTX-6058, a highly potent small molecule EED inhibitor in development for the treatment of select hemoglobinopathies, including sickle cell disease and beta-thalassemia. Additionally, after careful consideration and a strategic review of the COVID-19 landscape, we are discontinuing our LOSVID trial. This enables us to focus on rare diseases. I would like to thank the patients and investigators who participated in this trial and the Fulcrum team who worked tirelessly to rapidly design and launch the LOSVID trial during a global pandemic."

"Furthermore, we have made great progress with our next-generation product engine including new levels of validation in our internal research efforts and externally through our strategic collaborations," continued Dr. Gould. "With the additional capital from our recent public offering, we have extended our cash runway into the fourth quarter of 2022 and we believe that we are well positioned to continue progress on our goal to advance therapies to improve the lives of patients with genetically defined rare diseases."

## **Recent Business Highlights**

- On track to report data from ReDUX4, a Phase 2b trial of losmapimod, a selective p38 $\alpha$ / $\beta$  mitogen activated protein kinase (MAPK) inhibitor, in FSHD late in the second quarter of 2021.
  - Data will include the primary endpoint, reduction from baseline of DUX4-driven gene expression, as well as a pre-specified sensitivity analysis assessing biopsies with the highest pre-treatment level of DUX4-driven gene expression. Additionally, secondary endpoints, including skeletal muscle MRI and exploratory endpoints, including clinical outcome assessments and patient reported outcomes will also be reported.
  - Continued evaluation of the Phase 2 Open Label Study.
- On track to report results from the Phase 1 trial in healthy adult volunteers with FTX-6058 in development for sickle cell disease (SCD) in mid-2021, and to begin dosing patients with SCD by year end.
  - FTX-6058, a highly potent small molecule EED inhibitor, is designed to induce expression of fetal hemoglobin (HbF) in red blood cells following oral administration to compensate for the mutated adult hemoglobin associated with hemoglobinopathies, including sickle cell disease and beta-thalassemia.
  - Preclinical data with FTX-6058 showed an increase in HbF levels up to approximately 30% of total hemoglobin, indicating the potential to have a significant impact on patients with sickle cell disease.
- After careful consideration, Fulcrum is discontinuing LOSVID, a Phase 3 trial with losmapimod for hospitalized subjects with COVID-19, due to significant changes in the COVID-19 treatment paradigm, including new therapeutic options and emerging vaccines.
  - The company has decided to redeploy its resources to other clinical programs and discovery efforts, with a continued focus on rare diseases.
  - Losmapimod was generally well tolerated in LOSVID, and an independent data safety monitoring board did not identify any safety concerns related to losmapimod.
- Multiple scientific meeting presentations
  - Presentation on FulcrumSeek, the company's next generation product engine approach, at the Society for Laboratory Automation and Screening annual meeting in January 2021.
  - Presented multiple posters supporting the potential of FTX-6058 in sickle cell disease at the 62<sup>nd</sup> American Society

of Hematology (ASH) annual meeting, December 5-8, 2020.

- Presented target engagement and good tolerability with FTX-6058 in multiple preclinical rodent models with once-a-day oral dosing at the 14<sup>th</sup> Annual Sickle Cell Disease Research & Educational Symposium and 43<sup>rd</sup> National Sickle Cell Disease Scientific Meeting, September 25, 2020.
- Presented multiple posters supporting Fulcrum's integrated approach to the evaluation of FSHD patients during the 25<sup>th</sup> International Congress of the World Muscle Society (WMS), October 1, 2020.
- Advanced FulcrumSeek discovery efforts and strategic collaborations with Acceleron and MyoKardia, a wholly owned subsidiary of Bristol-Myers Squibb Company.
- Raised gross proceeds of \$50.6 million from a public offering in January 2021.
  - The underwritten public offering of 4,600,000 shares of the company's common stock at a public offering price of \$11.00 per share included 600,000 shares issued upon the exercise in full by the underwriters of their option to purchase additional shares at the public offering price.
- Key management updates
  - *CEO transition:* On March 4, 2021, Fulcrum announced that Bryan E. Stuart will be promoted to president and chief executive officer and Robert J. Gould, Ph.D. will retire from his role as president and chief executive officer, each effective March 31, 2021. Mr. Stuart has also been appointed to Fulcrum's Board of Directors. Dr. Gould will continue to serve on Fulcrum's Board and has also been appointed to the Scientific & Technology committee of the Board. Additionally, Mark Levin, Fulcrum's Board chair, will assume the role of executive chair effective upon Dr. Gould's retirement.
  - *CSO transition:* On January 19, 2021, Fulcrum announced that Chris Moxham, Ph.D. was promoted to chief scientific officer and Owen Wallace, Ph.D. stepped down from his role as chief scientific officer, each effective February 5, 2021. Dr. Wallace has been appointed to Fulcrum's Scientific Advisory Board.

#### **Fourth Quarter and Full Year 2020 Financial Results**

- **Cash Position:** As of December 31, 2020, cash, cash equivalents, and marketable securities were \$112.9 million, as compared to \$96.7 million as of December 31, 2019. Based on current plans, the company expects that its cash, cash equivalents, and marketable securities as of December 31, 2020, together with the net proceeds of \$46.4 million from the sale of its common stock in a public offering on January 22, 2021, will be sufficient to enable Fulcrum to fund operating expenses and capital expenditure requirements into the fourth quarter of 2022.
- **Collaboration Revenue:** Collaboration revenue was \$4.2 million for the fourth quarter of 2020, as compared to no revenue recognized during the fourth quarter of 2019. The increase in collaboration revenue was due to the execution of the company's collaboration and license agreements with Acceleron and MyoKardia in December 2019 and July 2020, respectively.

Collaboration revenue was \$8.8 million for the year ended December 31, 2020, as compared to no revenue recognized during the year ended December 31, 2019. The increase in collaboration revenue was due to the execution of the collaboration and license agreements with Acceleron and MyoKardia in December 2019 and July 2020, respectively.

- **R&D Expenses:** Research and development expenses were \$16.1 million for the fourth quarter of 2020, as compared to \$12.1 million for the fourth quarter of 2019. The increase of \$4.0 million was primarily due to increased costs to support the company's ongoing and planned clinical trials and increased personnel-related costs to support the growth of Fulcrum's research and development organization, including increased stock-based compensation expense.

Research and development expenses were \$59.0 million for the year ended December 31, 2020, as compared to \$71.1 million for the year ended December 31, 2019. Research and development expenses for the year ended December 31, 2019 include \$25.6 million of one-time costs incurred associated with the issuance of Series B convertible preferred stock under the company's license agreement with GSK for losmapimod and \$2.5 million of one-time costs incurred associated with the achievement of a milestone under the company's license agreement with GSK for losmapimod. Excluding these one-time costs, the increase of \$16.0 million was primarily due to increased costs to support the company's ongoing and planned clinical trials and increased personnel-related costs to support the growth of Fulcrum's research and development organization, including increased stock-based compensation expense.

- **G&A Expenses:** General and administrative expenses were \$5.9 million for the fourth quarter of 2020, as compared to \$4.4 million for the fourth quarter of 2019. The increase of \$1.5 million was primarily due to increased costs associated

with operating as a public company and increased personnel-related costs to support the growth of the organization, including increased stock-based compensation expense.

General and administrative expenses were \$21.4 million for the year ended December 31, 2020, as compared to \$13.1 million for the year ended December 31, 2019. The increase of \$8.3 million was primarily due to increased costs associated with operating as a public company and increased personnel-related costs to support the growth of the organization, including increased stock-based compensation expense.

- **Net Loss:** Net loss was \$17.7 million for the fourth quarter of 2020, as compared to a net loss of \$16.1 million for the fourth quarter of 2019.

Net loss was \$70.8 million for the year ended December 31, 2020, as compared to \$82.7 million for the year ended December 31, 2019.

#### **Conference Call and Webcast**

Fulcrum Therapeutics, Inc. will host a conference call and webcast today at 8:00 a.m. ET to discuss the company's fourth quarter and full year 2020 financial results and recent business highlights. The webcast will be accessible through the Investor Relations section of Fulcrum's website at [www.fulcrumtx.com](http://www.fulcrumtx.com). Following the live webcast, an archived replay will also be available.

#### **Dial-in Number**

U.S./Canada Dial-in Number: 800-527-6973

International Dial-in Number: 470-495-9162

Conference ID: 6376419

Replay Dial-in Number: 855-859-2056

Replay International Dial-in Number: 404-537-3406

Conference ID: 6376419

#### **About FSHD**

FSHD is characterized by progressive skeletal muscle loss that initially causes weakness in muscles in the face, shoulders, arms and trunk, and progresses to weakness throughout the lower body. Skeletal muscle weakness results in significant physical limitations, including an inability to smile and difficulty using arms for activities, with many patients ultimately becoming dependent upon the use of a wheelchair for daily mobility.

FSHD is caused by mis-expression of DUX4 in skeletal muscle, resulting in the presence of DUX4 proteins that are toxic to muscle tissue. Normally, DUX4-driven gene expression is limited to early embryonic development, after which time the DUX4 gene is silenced. In people with FSHD, the DUX4 gene is turned "on" as a result of a genetic mutation. The result is death of muscle and its replacement by fat, leading to skeletal muscle weakness and progressive disability. There are no approved therapies for FSHD, one of the most common forms of muscular dystrophy, with an estimated patient population of 16,000 to 38,000 in the United States alone.

#### **About Losmapimod**

Losmapimod is a selective p38 $\alpha$ / $\beta$  mitogen activated protein kinase (MAPK) inhibitor that was exclusively in-licensed from GSK by Fulcrum Therapeutics following Fulcrum's discovery of the role of p38 $\alpha$ / $\beta$  inhibitors in the reduction of DUX4 expression and an extensive review of known compounds. Utilizing its internal product engine, Fulcrum discovered that inhibition of p38 $\alpha$ / $\beta$  reduced expression of the DUX4 gene in muscle cells derived from patients with FSHD. Losmapimod has been evaluated in more than 3,600 subjects in clinical research across multiple indications, including in several Phase 2 trials and a large Phase 3 trial in acute myocardial infarction. No safety signals were attributed to losmapimod in any of these trials. In 2020, the company received U.S. and European Orphan Drug Designation for losmapimod for the treatment of FSHD. Fulcrum is currently conducting Phase 2 trials investigating the safety, tolerability, and efficacy of losmapimod to treat the root cause of FSHD.

#### **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. SCD patients 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 FTX-6058**

FTX-6058 is a highly potent small molecule inhibitor of Embryonic Ectoderm Development (EED) capable of inducing robust HbF protein expression in cell and murine models. Fulcrum believes the pharmacokinetics and human dose simulations support that FTX-6058 may be given as a once daily oral compound. The validation of EED as a target for sickle cell disease and the discovery of FTX-6058 as a novel HbF-inducing small molecule were conducted using Fulcrum's proprietary product engine. Preclinical data with FTX-6058 showed an increase in HbF levels up to approximately 30% of total hemoglobin. Fulcrum has initiated a Phase 1 trial with FTX-6058 in healthy adult volunteers.

#### **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 proprietary product engine identifies drug targets which can modulate gene expression to treat the known root cause of gene mis-expression. The company has advanced losmapimod to Phase 2 clinical development for the treatment of facioscapulohumeral muscular dystrophy (FSHD). Fulcrum has also advanced FTX-6058, a small molecule designed to increase expression of fetal hemoglobin for the treatment of sickle cell disease and beta-thalassemia into Phase 1 clinical development.

Please visit [www.fulcrumtx.com](http://www.fulcrumtx.com).

## 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, including statements regarding the development status of the Company’s product candidates, the potential advantages and therapeutic potential of Fulcrum’s product candidates, initiation and enrollment of clinical trials and availability of clinical trial data, and the Company’s ability to fund its operations with cash on hand. All statements, other than statements of historical facts, contained in this press release, including statements regarding the Company’s strategy, future operations, future financial position, prospects, plans and objectives of management, are forward-looking statements. 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 obtain and maintain necessary approvals from the FDA and other regulatory authorities; continue to advance its product candidates in clinical trials; initiate and enroll clinical trials on the timeline expected or at all; correctly estimate the potential patient population and/or market for the Company’s product candidates; 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; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; 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 the Company’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 the Company’s most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company’s views as of the date hereof and should not be relied upon as representing the Company’s views as of any date subsequent to the date hereof. The Company anticipates that subsequent events and developments will cause the Company’s views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so.

### Fulcrum Therapeutics, Inc.

#### Selected Consolidated Balance Sheet Data

(In thousands)

(Unaudited)

	December 31, 2020	December 31, 2019
Cash, cash equivalents, and marketable securities	\$ 112,914	\$ 96,713
Working capital <sup>(1)</sup>	92,785	87,943
Total assets	129,577	110,439
Total stockholders’ equity	95,181	87,153

(1) We define 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,	
	2020	2019	2020	2019
Collaboration revenue	\$ 4,225	\$ —	\$ 8,823	\$ —
Operating expenses:				
Research and development	16,145	12,087	59,042	71,072
General and administrative	5,867	4,403	21,392	13,145
Total operating expenses	22,012	16,490	80,434	84,217
Loss from operations	(17,787)	(16,490)	(71,611)	(84,217)
Other income, net	67	367	792	1,540
Net loss	\$ (17,720)	\$ (16,123)	\$ (70,819)	\$ (82,677)

Cumulative convertible preferred stock dividends	—	—	—	(7,128)
Net loss attributable to common stockholders	<u>\$ (17,720)</u>	<u>\$ (16,123)</u>	<u>\$ (70,819)</u>	<u>\$ (89,805)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.64)</u>	<u>\$ (0.71)</u>	<u>\$ (2.79)</u>	<u>\$ (8.13)</u>
Weighted average number of common shares used in net loss per share attributable to common stockholders, basic and diluted	<u>27,537</u>	<u>22,610</u>	<u>25,354</u>	<u>11,046</u>

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