

MyoKardia and Fulcrum Therapeutics Announce Multi-Target Collaboration to Discover Novel Targeted Therapies for Genetic Cardiomyopathies

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Collaboration will Combine MyoKardia's Leading Precision Cardiovascular R&D Platform with Fulcrum's Proprietary Product Engine to Identify Targets that Modulate Gene Expression

BRISBANE, Calif. and CAMBRIDGE, Mass., July 21, 2020 (GLOBE NEWSWIRE) -- MyoKardia, Inc. (Nasdaq: MYOK), a clinical-stage biopharmaceutical company discovering and developing targeted therapies for the treatment of serious cardiovascular diseases and Fulcrum Therapeutics, Inc. (Nasdaq: FULC), a clinical-stage biopharmaceutical company focused on improving the lives of patients with genetically defined rare diseases, announced today that they have entered into a strategic collaboration and license agreement to discover, develop and commercialize novel targeted therapies for the treatment of genetic cardiomyopathies.

Under the agreement, MyoKardia will access Fulcrum's unique, proprietary target discovery engine to identify therapeutics that control the expression of genes that are known to be underlying drivers of genetic cardiomyopathies. The collaboration focuses joint discovery efforts on certain undisclosed genetic targets. MyoKardia will be responsible for all development and commercialization activities for, and will have global rights to, any potential therapeutics identified through this collaboration.

Fulcrum will receive a payment of \$12.5 million at the close of the transaction and may be eligible to receive research, development and commercial milestone payments and additional research reimbursement of up to \$302.5 million for a first product to progress through development and commercialization. If MyoKardia chooses to develop and commercialize products directed to additional targets under the collaboration, Fulcrum may be eligible for up to \$150.0 million in milestone payments. Fulcrum may also be eligible to receive tiered royalty payments in the mid-single-digit to low double-digit range on net sales for any products under the collaboration that are commercialized.

MyoKardia intends to select targets for further exploration under this collaboration informed by its integrated research and development engine, which includes capabilities in translational research, proprietary and novel disease models, clinical development, and patient engagement and identification. Potential diseases associated with such undisclosed targets are expected to share common characteristics with indications currently being pursued by MyoKardia: strong and genetically-validated mechanistic rationale, high unmet patient need, potentially efficient pathway to approval, and synergy with the commercial organization that MyoKardia is building.

"This partnership is a natural extension of MyoKardia's investments over the last eight years in building a world-leading cardiovascular research, translational, clinical and commercial organization. We believe this collaboration will enable us to leverage our unique strengths to expand thoughtfully in identifying new therapeutic candidates for the potential treatment of heritable cardiomyopathies," said Robert S. McDowell, Ph.D., MyoKardia's Chief Scientific Officer. "We have been impressed by Fulcrum's ability to discover new biology around genetic muscle disorders. By working together, we hope to further our mission to treat patients suffering from serious cardiovascular disease."

"This collaboration highlights the broad applicability of our product engine to discover and develop new treatments in genetically defined rare diseases with high unmet need," said Robert J. Gould, Ph.D., Fulcrum's President and Chief Executive Officer. "We are pleased to partner with MyoKardia, a leader in the field of precision cardiovascular medicine and look forward to leveraging their unique capabilities to rapidly advance potential treatments to serve patients in urgent need."

About MyoKardia

MyoKardia is a clinical-stage biopharmaceutical company discovering and developing targeted therapies for the treatment of serious cardiovascular diseases. The company is pioneering a precision medicine approach to its discovery and development efforts by (1) understanding the biomechanical underpinnings of disease; (2) targeting the proteins that modulate a given condition; (3) identifying patient populations with shared disease characteristics; and (4) applying learnings from research and clinical studies to inform and guide pipeline growth and product advancement. MyoKardia's initial focus is on small molecule therapeutics aimed at the proteins of the heart that modulate cardiac muscle contraction to address diseases driven by excessive contraction, impaired relaxation, or insufficient contraction. Among its discoveries are three clinical-stage therapeutics: mavacamten (formerly MYK-461); danicamtiv (formerly MYK-491) and MYK-224.

MyoKardia's mission is to change the world for people with serious cardiovascular disease through bold and innovative science.

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) and is advancing losmapimod to Phase 3 for the treatment of COVID-19. Fulcrum also anticipates a regulatory filing in the second half of 2020 with FTX-6058 for the treatment of sickle cell disease.

Please visit www.fulcrumtx.com.

MyoKardia Contacts

Michelle Corral
Executive Director, Corporate Communications and Investor Relations
MyoKardia, Inc.
650-351-4690
ir@myokardia.com

Hannah Deresiewicz (investors)
Stern Investor Relations, Inc.

Fulcrum Contacts

Christi Waarich
Director, Investor Relations and
Corporate Communications
617-651-8664
cwaarich@fulcrumtx.com

Stephanie Ascher (investors)
Stern Investor Relations, Inc.

212-362-1200
hannah.deresiewicz@sternir.com

Julie Normant (media)
W2O
628-213-3754
jnormart@w2ogroup.com

212-362-1200
stephanie.ascher@sternir.com

Kaitlin Gallagher (media)
Berry & Company Public Relations
212-253-8881
kgallagher@berrypr.com