

# Third Rock Ventures Launches Fulcrum Therapeutics with \$55 Million Series A Investment

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*Fulcrum Therapeutics to develop new medicines to deliver a new future to patients and their families by transforming gene regulation in disease*

*Industry Leader Robert Gould, Ph.D., named President and CEO*

Cambridge, Mass., July 19, 2016 – Third Rock Ventures, LLC today announced the launch of Fulcrum Therapeutics, a company focused on unlocking gene control mechanisms to develop small molecule therapies. Fulcrum will discover and develop small molecules that modulate the on/off control mechanisms that regulate genes.

Fulcrum was established with \$55 million in Series A financing to further develop a cross-disciplinary product engine to identify and modulate gene regulatory targets core to disease. Through its product engine, Fulcrum will develop a robust pipeline across therapeutic areas, spearheaded by two initial programs in genetic diseases. The company is led by President and Chief Executive Officer Robert J. Gould, Ph.D., former President and CEO of Epizyme, and a long-time leader in drug discovery and development.

“One of the single most important biologic breakthroughs of the last decade has been the unraveling of gene regulation at a molecular level. For more than a decade we have understood the genetic code – the genome. Now that we are unraveling the way these genes are regulated and put to work in biology, there is an unprecedented opportunity for drug development,” Dr. Gould said. “The richness of the human genome comes from the way genes are turned on and off. Through the modulation of these on and off switches we have the potential to transform the treatment of hundreds of serious human diseases. We are excited to build Fulcrum Therapeutics – a company that is capable of translating these discoveries into groundbreaking new therapies that restore balance in patients who currently have no other therapeutic options.”

Fulcrum’s product engine was created to integrate and accelerate the rapid advances that have reshaped a wide range of biological disciplines, including transcription biology, stem cell biology and computational biology. The integration of these diverse advanced technologies throughout the drug discovery and development process creates the opportunity to deliver on the promise of genomic medicine; namely, to not only discover genetic disease drivers, but also to regulate gene expression to restore health.

Fulcrum models gene regulation in disease tissue using patient cells that are either donated through tissue biopsy or derived from skin cells using the technology of induced pluripotent stem cells. It uses screening tools such as CRISPR/CAS9 and chemical probe libraries to dissect gene regulatory mechanisms in cellular models of diseases. These discoveries are brought together with publicly available gene regulatory data to create genome-wide maps of gene regulation that enable rapid identification of drug targets for the activation or repression of disease genes. The combination of patient derived cells, screening tools and genome-wide databases enables Fulcrum’s product engine.

The company believes there is long-term potential to address complex genetic diseases through its approach. The early focus of Fulcrum’s product engine is on two severe genetic diseases: Fragile X syndrome (FXS) and a form of muscular dystrophy called facioscapulohumeral muscular dystrophy (FSHD). Each disease arises from a single gene mutation that creates an error in gene regulation. In the case of Fragile X, the silencing of the FMR1 gene eliminates the cell’s ability to make a protein needed for brain function. In FSHD, the precipitating event is the activation of a gene called DUX4 that should be silent in adulthood. This activation leads to muscle wasting as cells die. Other genetic diseases that can be explored with Fulcrum’s robust product engine include other neurologic disorders, skeletal muscular disorders and cardiac muscular disease.

Expertise spanning core disciplines

The company’s internal discovery and development team is complemented by a group of scientific founders. Together, this combined expertise integrates the elements of the Fulcrum Therapeutics product engine.

Overall leadership is provided by CEO Robert J. Gould, who served as Epizyme’s President and Chief Executive Officer from 2010 to 2015. Prior to joining Epizyme, from 2006 to 2010, he served as Director of Novel Therapeutics at The Broad Institute of MIT and Harvard. Dr. Gould was Vice President, Licensing and External Research and Vice President, Basic Research, Merck Research Laboratories, at Merck & Co., Inc., where he held a variety of leadership positions during his 24-year tenure.

Company founders include:

- Michael Green, M.D., Ph.D.: University of Massachusetts Medical School; The Howard Hughes Medical Institute; National Academy of Sciences; providing expertise in gene regulation
- Danny Reinberg, Ph.D.: New York University School of Medicine; The Howard Hughes Medical Institute; National Academy of Sciences; providing expertise in chromatin structure and function
- Rudolf Jaenisch, M.D.: Whitehead Institute for Biomedical Research; Massachusetts Institute of Technology; Institute of Medicine; National Academy of Sciences; providing expertise in stem cell biology
- Jeannie Lee, M.D., Ph.D.: Massachusetts General Hospital; Harvard Medical School; The Howard Hughes Medical Institute; National Academy of Sciences; providing expertise in gene regulation and X chromosome genetics
- Brad Bernstein, M.D., Ph.D.: Massachusetts General Hospital; Harvard Medical School; Broad Institute of MIT and Harvard; providing expertise in genome-wide characterization of gene regulation.

## About Fulcrum Therapeutics

Fulcrum Therapeutics is a biotechnology company developing new medicines to deliver a new future to patients and their families by transforming gene regulation in disease. Fulcrum’s therapies are based on 3 modulating gene regulation via control of genetic on and off switches of disease genes. Fulcrum was launched in 2016 by Third Rock Ventures, and is headquartered in Cambridge, Mass. For more information please visit [www.fulcrumtx.com](http://www.fulcrumtx.com)

## **About Third Rock Ventures**

Third Rock Ventures is the leading healthcare venture firm focused on disruptive areas of science and medicine to discover, launch and build companies that make a dramatic difference in people's lives. By combining our team's scientific vision, strategic leadership, operational expertise and innovative dealmaking capabilities, we nurture bold ideas that translate into successful business enterprises. Recognizing that the best way to create value for our investors is to create value for patients, our companies are built on a solid foundation of science, medicine, people and business strategy. For more information, please visit [www.thirdrockventures.com](http://www.thirdrockventures.com).

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