

Fulcrum Therapeutics Appoints Renowned Experts in CNS Disorders and Muscular Dystrophies to Serve on Newly Formed Scientific Advisory Board

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Cambridge, Mass., December 7, 2016 – Fulcrum Therapeutics, a company focused on discovering and developing small molecule therapies to unlock gene control, today announced that it has formed a Scientific Advisory Board (SAB) comprised of renowned experts in the biology of central nervous system (CNS) disorders and muscular dystrophies to help guide the development of the company's emerging therapeutic programs.

Fulcrum was launched earlier this year with a group of world-leading scientific founders to translate new genomic discoveries into medicines that regulate gene expression to restore health. Fulcrum's founders provide deep expertise in transcription biology, stem cell biology and computational biology. The integration of these disciplines creates the Fulcrum Product Engine to advance new programs through drug discovery and development to deliver on the promise of genomic medicine.

As Fulcrum advances its lead programs in Fragile X syndrome (FXS) and facioscapulohumeral muscular dystrophy (FSHD) it is establishing CNS and muscular dystrophy franchises. Fulcrum's newly formed SAB adds extensive expertise in disease biology to complement the scientific founders.

Each member of the Fulcrum SAB has contributed important new discoveries to the understanding of these and other related conditions, and has worked to pioneer novel therapeutic strategies to address them.

- **Peter L. Jones, Ph.D.**, an Associate Professor in Cell Biology and Neurology at the University of Massachusetts Medical School is a leader in genetic muscle diseases, with a particular focus on understanding the mechanisms of misregulated gene expression in FSHD.
- **Leslie Leinwand, Ph.D.**, a Professor in the Department of Molecular, Cellular and Developmental Biology and Chief Scientific Officer of the BioFrontiers Institute at the University of Colorado, Boulder, is a renowned muscle physiologist with broad expertise in the genetic underpinnings of heart and skeletal muscle defects.
- **Steve Warren, Ph.D.**, Chairman of the Department of Human Genetics and a Professor of Biochemistry and Pediatrics at Emory University in Atlanta, has made seminal discoveries related to FXS and its potential treatment. He led an international group that first identified the *FMR1* gene, the misregulation of which causes this syndrome, and is now a leader in the effort to identify new genomically focused therapeutic approaches to treating this condition.
- **Marius Wernig, M.D., Ph.D.**, an Associate Professor of Pathology and Chemical and Systems Biology and Assistant Professor at the Institute for Stem Biology and Regenerative Medicine at Stanford University, is an expert in the molecular mechanisms that determine specific cell fates. His laboratory recently identified a pool of transcription factors that are sufficient to convert skin fibroblasts directly into functional neuronal cells.

Additional background on each of these SAB members can be found here: <https://www.fulcrumtx.com/about/>.

"We are thrilled to bring together this distinguished group of scientific leaders on our SAB, each of whom has contributed vital discoveries that have shed light on the role of gene regulation in the development of Fragile X, FSHD and other diseases of interest," said Robert J. Gould, Ph.D., Fulcrum's President and Chief Executive Officer. "The collective perspectives of this group form a powerhouse SAB that bring disease relevant expertise to complement our Founders Board. They will be an invaluable resource to our management team as we build a CNS franchise and a muscular dystrophy franchise composed of new medicines to transform gene regulation in disease."

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 modulating gene regulation via control of genetic on and off switches of disease genes. Fulcrum, headquartered in Cambridge, Mass., was launched by Third Rock Ventures in 2016 and named a "Fierce 15" company later that year. For more information, please visit www.fulcrumtx.com.

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