Fulcrum Therapeutics Recognizes Rare Disease Day 2021

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Theme of global unity highlights critical role of building communities of support for patients and families affected by rare diseases around the world

CAMBRIDGE, Mass., Feb. 26, 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 announced the company's recognition of Rare Disease Day 2021.

"While COVID-19 has had an impact on most people around the world, it has put a new spotlight on the importance of connectivity and community for people affected by rare diseases who are at a higher risk of feelings of isolation and can face significant challenges in accessing information and healthcare services," said Robert J. Gould, Ph.D., Fulcrum's president and chief executive officer. "We have been privileged to work with many patients and caregivers affected by genetically defined diseases including facioscapulohumeral muscular dystrophy and sickle cell disease. They have bravely shared their stories in efforts to help others better understand these diseases and access information and support that can help."

Rare Disease Day (www.rarediseaseday.org) was established by EURODIS in 2008 and is held on the last day of February each year in an effort to build awareness of rare diseases and the impact they have on patients and their families. The theme for Rare Disease Day 2021 highlights the need to provide members of the rare disease community around the world with new opportunities to meet, share insights and support and join together in efforts in education and advocacy. This year will mark the first all-digital Rare Disease Day involving interactive online events planned by hundreds of international advocacy, research and patient care organizations.

During February, Fulcrum Therapeutics supported a range of efforts to help build broader awareness of facioscapulohumeral muscular dystrophy (FSHD) and sickle cell disease (SCD) by presenting information from patients and caregivers about the challenges they face. The company will also invite employees to join a virtual meeting and Q&A session where members of the FSHD and SCD communities will talk about how they have addressed different challenges and the opportunities for collaboration between patients and companies working to develop new treatments. Company team members will also take part in the virtual 5k race sponsored by the National Organization for Rare Disorders (NORD). And Fulcrum is launching a new corporate Facebook page that will present perspectives from patients and caregivers about their experiences and hopes for the future.

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 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 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 Phase 3 for the treatment of COVID-19. 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.

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. 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. 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.

Contacts:

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

Stephanie Ascher
Stern Investor Relations, Inc.

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

Media:
Kaitlin Gallagher
Berry & Company Public Relations
kgallagher@berrypr.com
212-253-8881