CRISPR Therapeutics and collaborators at the University of Florida awarded Target ALS Grant to develop CRISPR/Cas9-based approaches for ALS

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BASEL, Switzerland, CAMBRIDGE, Mass. and GAINESVILLE, Fla., May 16, 2017 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (NASDAQ:CRSP), a biopharmaceutical company focused on creating transformative gene-based medicines for serious diseases, today announced that Target ALS Foundation, a non-profit organization dedicated to accelerating new treatments for amyotrophic lateral sclerosis (ALS), has awarded a two-year grant to CRISPR Therapeutics and its collaborators to support preclinical discovery and validation of CRISPR/Cas9-based therapeutic approaches directed to amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD). CRISPR Therapeutics will collaborate with Dr. Laura Ranum and Dr. Eric Wang, researchers at the University of Florida in the Center for NeuroGenetics and the Department of Molecular Genetics & Microbiology, to test CRISPR/Cas9 gene-editing strategies in disease models developed by Dr. Ranum and Dr. Wang. The CRISPR Therapeutics and University of Florida consortium was one of four awardees selected by the independent scientific review committee out of a large field of highly competitive applications.

“We are pleased to support this collaborative consortium between CRISPR Therapeutics and the University of Florida. Dr. Laura Ranum and Dr. Eric Wang are two of the leading researchers globally in ALS and similar disorders, and it brings them together with the leading gene-editing company to accelerate the path to clinic for CRISPR-based therapies in ALS,” said **Manish Raisinghani, President of the Target ALS Foundation**.

“We are delighted to partner with Dr. Ranum and Dr. Wang to translate our *in vivo* gene-editing platform into potential therapies that address the underlying cause of ALS and FTD. The advances we make on ALS could pave the way for CRISPR/Cas9-based therapies in other CNS indications as well,” said **Chad Cowan, Head of Research, CRISPR Therapeutics.**

“Building on our expertise in repeat expansion diseases and our development of a robust mouse model of C9orf72 ALS/ FTD we are uniquely poised to test the efficacy of CRISPR/Cas9-based therapeutic strategies and their potential to prevent or reverse disease. My colleague Eric Wang and I are excited to embark on this collaboration to advance the development of CRISPR-based therapeutics,” said **Laura Ranum, Kitzman Family Professor of Genetics and Microbiology and Director of the Center for NeuroGenetics, University of Florida.**

ALS and FTD are incurable diseases, certain forms of which are caused by pathologic expansion of a naturally occurring short DNA sequence repeat in specific genes. The CRISPR/Cas9-based approaches that will be the focus of this collaboration have the potential to correct these mutations at the molecular level to address the underlying cause of disease.

About CRISPR Therapeutics

CRISPR Therapeutics is a leading gene-editing company focused on developing transformative gene-based medicines for serious diseases using its proprietary CRISPR / Cas9 gene-editing platform. CRISPR/Cas9 is a revolutionary technology that allows for precise, directed changes to genomic DNA. The company's multi-disciplinary team of world-class researchers and drug developers is working to translate this technology into breakthrough human therapeutics in a number of serious diseases. Additionally, CRISPR Therapeutics has established strategic collaborations with Bayer AG and Vertex Pharmaceuticals to develop CRISPR-based therapeutics in diseases with high unmet need. The foundational CRISPR / Cas9 patent estate for human therapeutic use was licensed from the company's scientific founder Emmanuelle Charpentier, Ph.D. CRISPR Therapeutics AG is headquartered in Basel, Switzerland, with its wholly-owned U.S. subsidiary, CRISPR Therapeutics, Inc., and R&D operations based in Cambridge, Massachusetts. For more information, please visit www.crisprtx.com.

About the University of Florida Center for NeuroGenetics

The Center for NeuroGenetics (CNG), in the College of Medicine at the University of Florida, uses molecular, genetic and clinical research to define the causes of neurodegenerative disease, including ALS, and to develop effective treatment strategies. The goal of our Center is to advance our understanding of these genetic disorders so that we can develop rational therapeutic strategies for patients. Key aspects of the Center’s approach are to partner with affected families, non-profit organizations like Target ALS, and industry to understand these diseases using both clinical and basic science approaches. The Center for NeuroGenetics was founded in 2010 by Dr. Laura Ranum, PhD (Director) and Dr. Maurice Swanson, PhD (Associate Director). For more information, please visit www.neurogenetics.med.ufl.edu.

About Target ALS Foundation

Target ALS Foundation, Inc. (www.targetals.org) is a non-profit organization with the overall goal of accelerating development of new treatments for ALS. We drive emergence of novel ALS drug discovery programs in industry by funding collaborative consortia focused on development of novel therapeutic targets. To ensure that all new ideas get tested, we make essential tools and resources openly available to all—especially young investigators—with no embargo or strings attached.

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