



CRISPR Therapeutics Awarded Grant from Friedreich's Ataxia Research Alliance to Collaborate with University of Alabama at Birmingham on Gene-edited Treatments for Friedrich's Ataxia

October 13, 2017

-FARA and CRISPR Celebrate Annual rideATAXIA Event-

ZUG, Switzerland and CAMBRIDGE, Mass., Oct. 13, 2017 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (NASDAQ:CRSP), a genome editing company focused on creating transformative medicine for serious diseases, today announced the receipt of the Kyle Bryant Translational Research Award from Friedreich's Ataxia Research Alliance (FARA), a non-profit organization that is focused on curing Friedreich's Ataxia (FA). The grant is awarded to fund research on *in vivo* CRISPR/Cas9-based gene-editing approaches to treat FA, which will be performed in collaboration with Dr. Marek Napierala at University of Alabama at Birmingham. This announcement coincides with FARA's rideATAXIA Philadelphia event, a lead location in an annual bike ride program founded by patient Kyle Bryant, that increases FA awareness and raises funds to treat and cure FA through research.

"FARA is thrilled to celebrate rideATAXIA Philly with the announcement of the Kyle Bryant Translational Research Award to CRISPR Therapeutics. We are grateful to all of the FA families that fundraise and the CureFA Foundation for providing the resources to advance research," said Jennifer Farmer, Executive Director of FARA. "We believe that the combination of CRISPR's pioneering gene-editing capabilities with Dr. Marek Napierala's deep expertise in FA may accelerate the development of new therapies for FA."

Under the terms of the grant, CRISPR Therapeutics will develop gene-editing reagents with the potential to address the underlying genetic causes of FA. Dr. Napierala and colleagues will then evaluate the reagents in humanized animal models of FA to demonstrate preclinical proof-of-concept. Upon conclusion of these studies, CRISPR Therapeutics will have the option to advance the compounds into clinical trials.

"With our growing understanding of the genetic and molecular underpinnings of FA in patients and animal disease models, the time is right to develop a CRISPR/Cas9-based therapeutic approach. I am excited to join CRISPR Therapeutics and FARA in attempting to develop a cure for this devastating disease," said Marek Napierala, Ph.D., Associate Professor, Department of Biochemistry and Molecular Genetics; Stem Cell Institute, University of Alabama at Birmingham.

"Our partners' understanding of disease biology and patient needs are critical to support drug development for FA. We are delighted to partner with Dr. Napierala and FARA on this journey to harness the potential of CRISPR/Cas9 gene editing for patients with FA. We believe that our gene editing technology is uniquely suited to target the underlying genetic causes of genetic disorders such as FA," commented Tony Ho, MD, Head of Research & Development, CRISPR Therapeutics.

This is the second grant in neurologic disorders received by CRISPR Therapeutics in 2017. In May, the company announced award of a Target ALS Foundation grant to support collaboration with Dr. Laura Ranum of University of Florida on CRISPR/Cas9 based therapeutic strategies to be directed to amyotrophic lateral sclerosis and frontotemporal dementia.

About rideATAXIA

rideATAXIA is a nation-wide program of bike rides that welcomes people of all abilities to ride, and to raise funds for FARA's mission to treat and cure FA through research. FARA hosts rideATAXIA cycling events throughout the year in the following area locations: Southern California, Dallas, Northern California, Chicago, Philadelphia and Orlando. To participate in or support rideATAXIA please visit: rideataxia.org.

About FARA

The Friedreich's Ataxia Research Alliance (FARA) is a 501(c)(3), non-profit, charitable organization dedicated to accelerating research leading to treatments and a cure for Friedreich's ataxia. FA is the most common inherited ataxia affecting one in 50,000 people causing a debilitating loss of mobility and life-shortening cardiac conditions. FARA focuses on grant making for FA research and building collaborations in the scientific community and promoting public-private partnerships. www.curefa.org

About University of Alabama at Birmingham

Known for its innovative and interdisciplinary approach to education at both the graduate and undergraduate levels, the University of Alabama at Birmingham is an internationally renowned research university and academic medical center, as well as Alabama's largest employer, with some 23,000 employees, and has an annual economic impact exceeding \$5 billion on the state. The five pillars of UAB's mission include education, research, patient care, community service and economic development. UAB is a two-time recipient of the prestigious [Center for Translational Science Award](#). Learn more at www.uab.edu. [UAB: Knowledge that will change your world.](#)

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 Zug, Switzerland, with its wholly-owned U.S. subsidiary, CRISPR Therapeutics, Inc., and R&D operations based in Cambridge,

Massachusetts. For more information, please visit <http://www.crisprtx.com>.

CRISPR Forward-Looking Statement

Certain statements set forth in this press release constitute "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, but not limited to, statements concerning: the therapeutic value, development and the commercial potential of CRISPR/Cas9 gene editing technologies. You are cautioned that forward-looking statements are inherently uncertain. Although the company believes that such statements are based on reasonable assumptions within the bounds of its knowledge of its business and operations, the forward-looking statements are neither promises nor guarantees and they are necessarily subject to a high degree of uncertainty and risk. Actual performance and results may differ materially from those projected or suggested in the forward-looking statements due to various risks and uncertainties. These risks and uncertainties include, among others: uncertainties inherent in the initiation and conduct of preclinical and clinical studies for the company's product candidates; availability and timing of results from preclinical and clinical studies; whether results from a preclinical study or clinical trial will be predictive of future results in connection with future trials or use; expectations for regulatory approvals to conduct trials or to market products; our ability to obtain and maintain proprietary intellectual property protection on key products and technologies; uncertainties regarding actual or potential legal proceedings and those risks and uncertainties described in Item 1A under the heading "Risk Factors" in the company's annual report on Form 10-K, and in any other subsequent filings made by the company with the U.S. Securities and Exchange Commission (SEC), which are available on the SEC's website at <https://www.sec.gov>. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date they are made. The information contained in this press release is provided by the company as of the date hereof, and, except as required by law, the company disclaims any intention or responsibility for updating or revising any forward-looking information contained in this press release.

Media Contacts:

Jennifer Paganelli
WCG on behalf of CRISPR
1 347-658-8290
jpaganelli@wcgworld.com

Investor Contact:

Chris Erdman
chris.erdman@crisprtx.com
617.307.7227

Chris Brinzey
Westwicke Partners for CRISPR
339-970-2843
chris.brinzey@westwicke.com



CRISPR Therapeutics AG