



uniQure Announces Preclinical Proof of Concept for Gene Therapy Approach in Huntington's Disease

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AMSTERDAM, the Netherlands, March 22, 2016 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leader in human gene therapy, today announced the publication of preclinical data supporting its proprietary Huntington's disease gene therapy program, AMT-130. Findings published in the current issue of the peer-reviewed journal Molecular Therapy-Nucleic Acids (www.nature.com/mtna/journal/v5/n3/index.html) provide preclinical proof of concept for uniQure's AMT-130 program and demonstrate the potential of a one-time administration of AAV5-delivered gene therapy into the central nervous system (CNS) to silence the Huntingtin gene (HTT). An inherited, mutated form of HTT causes Huntington's disease, a rare, fatal, neurodegenerative disorder that leads to severe physical and cognitive deterioration.

The paper, titled "*Design, Characterization, and Lead Selection of Therapeutic miRNAs Targeting Huntingtin for Development of Gene Therapy for Huntington's Disease*", was authored by a research team led by Pavlina Konstantinova, Ph.D., Director of Emerging Technologies at uniQure under the direction of Chief Scientific Officer Harald Petry, Ph.D. The publication describes multiple approaches to silencing HTT using expression cassette-optimized artificial microRNAs (miHTTs). Several miHTT scaffolds were incorporated in an AAV5 vector using uniQure's established baculovirus-based manufacturing platform and administered to a humanized mouse model. The data demonstrate strong silencing of mutant HTT and total HTT silencing *in vitro* and *in vivo*. Furthermore, it was shown that HTT knock-down efficiency could be increased to 80% by using optimized miHTT scaffolds. The data published today were in part presented at the 11th Annual CHDI Huntington's Disease Therapeutics Conference on February 24, 2016 by Dr. Konstantinova.

Based on these results, uniQure has initiated further studies of AMT-130 to support the filing of an investigative new drug application with the FDA.

"Huntington's disease devastates families and there is currently no effective disease-modifying treatment," commented Charles W. Richard, M.D., Ph.D., Senior Vice President, Research and Development, Neuroscience at uniQure. "We are excited by the results of this study, and believe this degree of knock-down of mutant Huntingtin protein, if duplicated in our ongoing non-human primate safety toxicology studies and future human clinical trials, could significantly alter the course of the disease."

"Dr. Konstantinova and her team have made significant progress in the search for an effective treatment for this cruel neurodegenerative disorder," said Dan Soland, Chief Executive Officer of uniQure. "AMT-130 now represents our third gene therapy product candidate in the CNS area, in addition to AMT-110 in Sanfilippo B and the NIH-sponsored program in Parkinson's disease. We will continue to leverage our deep experience in the CNS field, as well as our validated manufacturing capabilities and AAV5 technology, to advance AMT-130 towards the clinic."

About Huntington's Disease

Huntington's disease is a severe genetic neurodegenerative disorder causing loss of muscle coordination, behavioral abnormalities and cognitive decline, resulting in complete physical and mental deterioration over a 12-15 year period of time. The disease is caused by an autosomal dominant mutation, a cytosine-adenine-guanine (CAG) expansion, in the first exon of the Huntingtin gene leading to a non-functional, aggregation prone mutated protein. Despite the clear etiology, there are no therapies available to treat the disease, delay onset or slow progression of a patient's decline.

About uniQure

uniQure is delivering on the promise of gene therapy -- single treatments with potentially curative results. We are leveraging our modular and validated technology platform to rapidly advance a pipeline of proprietary and partnered gene therapies to treat patients with CNS, liver/metabolic and cardiovascular diseases. www.uniQure.com

uniQure Forward-Looking Statement

This presentation contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. These forward-looking statements include, but are not limited to, statements regarding the future development of our programs in Huntington's disease, Parkinson's disease and Sanfilippo B, and the progress of any of our ongoing or planned clinical studies and/or development of our product candidates. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with collaboration arrangements, our and our collaborators' clinical development activities, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in uniQure's 2014 Annual Report on Form 20-F filed with the Securities and Exchange Commission on April 7, 2015. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future.

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