uniQure Announces First Two Patients Treated in Phase I/II Clinical Trial of AMT-130 for the Treatment of Huntington's Disease

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~ Milestone Marks the First-in-Human AAV Gene Therapy Trial for Huntington's Disease ~





LEXINGTON, Mass. and AMSTERDAM, June 19, 2020 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that the first two patients in the Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease have been treated. The Phase I/II study is a double-blind, randomized clinical trial being conducted in the United States, with now one patient treated with AMT-130, and one patient who received the imitation surgery.

"For years, uniQure has had an unwavering commitment to advance this first-in-human AAV gene therapy for Huntington's disease into clinical testing, and this moment marks an important milestone for our company now that we have two AAV gene therapy candidates in clinical development," said Matt Kapusta, chief executive officer of uniQure. "With the first two patients treated in this trial, we have taken a significant step forward in advancing AMT-130 closer to our goal of developing a therapy that inhibits the production of the mutant huntingtin protein. We are delighted to be working with leading experts in the field to evaluate this promising candidate."

The Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease will explore the safety, tolerability, and efficacy signals in 26 patients with early manifest Huntington's disease randomized to treatment with AMT-130 or an imitation (sham) surgery. The five-year, multi-center trial consists of a blinded 18-month core study period followed by unblinded long-term follow-up. Patients will receive a single administration of AMT-130 through MRI-guided, convection-enhanced stereotactic neurosurgical delivery directly into the striatum (caudate and putamen). Additional details are available on www.clinicaltrails.gov (NCT04120493).

The first two patients will be observed for an initial period of 90 days, followed by a meeting of the Data Safety Monitoring Board (DSMB). The DSMB will review the data on the first two patients and make a determination about continued dosing of the next patients.

AMT-130 is uniQure's first clinical program focusing on the central nervous system (CNS) incorporating its proprietary miQURE™ platform.

"There is an urgent need for disease-modifying options to treat Huntington's disease, and we're excited to have an investigational gene therapy now available for HD patients," stated George Yohrling, chief scientific officer and chief mission officer at Huntington's Disease Society of America. "Based on the promising preclinical data presented on AMT-130 over the years, we are optimistic about its potential to alter the course of this devastating disease."

About Huntington's Disease
Huntington's disease is a rare, inherited neurodegenerative disorder that leads to motor symptoms including chorea, and behavioral abnormalities and cognitive decline resulting in progressive physical and mental deterioration. The disease is an autosomal dominant condition with a disease-causing CAG repeat expansion in the first exon of the huntingtin gene that leads to the production and aggregation of abnormal protein in the brain. Despite the clear etiology of Huntington's disease, there are no currently approved therapies to delay the onset or to slow the disease's progression.

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 gene therapies to treat patients with hemophilia B, hemophilia A, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other diseases. www.uniQure.com

uniQure Forward-Looking Statements
This press release 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, whether AMT-130 will prove to be a promising treatment or alter the course of Huntington's disease, whether the DSMB will authorize treatment of additional patients, and whether we will be able to treat 26 patients under the clinical trial. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the impact of the ongoing COVID-19 pandemic on our Company and the wider economy and health care system, our clinical development activities, clinical results, collaboration arrangements, 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 Quarterly Report on Form 10-Q filed on April 29, 2020. 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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A photo accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/53dbf6e5-2c8c-447c-abd9-a9d5714f2a8c

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