uniQure Announces Dosing of First Patients in European Open-Label Clinical Trial of AMT-130 Gene Therapy in Huntington's Disease

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LEXINGTON, Mass. and AMSTERDAM, Feb. 07, 2022 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the dosing of the first two patients in its European open-label Phase Ib/II clinical trial of AMT-130, a potential one-time gene-therapy approach for the treatment of Huntington's disease. The clinical trial is taking place at several sites in Poland, the United Kingdom and Germany.

"We are very pleased to expand the clinical development of AMT-130 and to build on our ongoing experience in the Phase I/II clinical trial in the United States," stated Ricardo Dolmetsch, Ph.D., president of research and development at uniQure. "We expect to complete patient enrollment in this European study by the end of the year and to provide safety and target-engagement data from the full 10-patient, low-dose cohort in the U.S. trial in the second quarter of this year."

"The Interventional Neurotherapy Center (INC) at Mazowiecki Szpital Bródnowski Hospital is the first and only center in Europe currently performing MRI-guided infusions of gene therapies," said Professor Miroslaw Zabek M.D. Ph.D., the chairperson of the department of neurosurgery and INC. "Our team is extremely excited to participate in this important Huntington's disease scientific research alongside our colleagues in the U.S. and to dose the first patients in the European clinical trial of AMT-130."

"Since 1995, our center at the Institute of Psychiatry and Neurology (IPiN) has offered genetic testing and clinical care to Polish patients with Huntington's disease," said Dr. Grzegorz Witkowski M.D. Ph.D., IPiN principal investigator. "Our patients have been very interested in the potential for a one-time treatment to stop progression of the disease and, given the recent setbacks in Huntington's disease research it means a lot to the Polish HD Community to be able to enroll the first patients in this first EU gene therapy trial."

The European Phase Ib/II clinical trial of AMT-130 for the treatment of Huntington's disease will explore the safety, proof of concept, and dosing in 15 total patients with early manifest Huntington's disease split into a six person, low-dose open-label cohort, followed by a nine patient, higher-dose open-label cohort. All patients will be dosed with AMT-130.

The multi-center study consists of an initial 6-month post-treatment study period followed by long-term follow-up for five years. Patients will receive a single administration of AMT-130 through MRI-guided, convection-enhanced stereotactic neurosurgical delivery directly into the striatum (caudate and putamen). The study is currently open for recruitment at IPiN and surgery at INC in Poland and is expected to expand to referral and surgical sites in the United Kingdom and Germany. Additional details are available on www.clinicaltrialsregister.eu (EudraCT 2020-001461-36).

About AMT-130

AMT-130 is uniQure's first central nervous system (CNS) focused gene therapy product consisting of an AAV5 vector carrying an artificial micro-RNA specifically tailored to silence the huntingtin gene, leveraging our proprietary miQURE® silencing technology. The therapeutic goal is to inhibit the production of the mutant protein (mHTT). Using AAV vectors to deliver micro-RNAs directly to the brain for non-selective knockdown of the huntingtin gene represents a highly innovative and promising approach to treating Huntington's 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, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 temporal lobe epilepsy, Alzheimer's, Parkinson's and ALS. 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 we will complete patient enrollment in the European study by the end of this year or ever, whether we will be able to provide safety and target-engagement data from the low-dose cohort in the U.S. trial in the second quarter of this year, whether we will be able to explore safety, tolerability and efficacy signals in either cohort of the study. 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 Commercialization and License Agreement with CSL Behring, our and our collaborators' clinical development activities, clinical results, collaboration arrangements, corporate reorganizations and strategic shifts, 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 periodic securities filings, including its Annual Report on Form 10-K filed March 1, 2021 and Quarterly Report on Form 10-Q filed on October 25, 2021. 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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