uniQure Receives Orphan Medicinal Product Designation in Europe for AMT-130 in Huntington's disease

January 22, 2018

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Jan. 22, 2018 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ:QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that AMT-130, its proprietary gene therapy candidate for Huntington's disease, has received an Orphan Medicinal Product Designation (OMPD) from the European Medicines Agency. AMT-130 represents the first investigational AAV-gene therapy in Huntington's disease to receive such designation. In October 2017 the company announced that the U.S. Food and Drug Administration granted orphan drug designation for the same indication. There are currently no approved medical treatments aimed at addressing the underlying cause of Huntington's disease, and AMT-130 has the potential to play a role in this area of high unmet medical need.

AMT-130 consists of an AAV5 vector carrying an engineered micro-RNA specifically designed to silence the huntingtin gene. The therapeutic goal is to inhibit the production of the mutant protein. Using AAV vectors to deliver micro-RNAs directly into the brain represents a highly innovative approach to treating Huntington's disease.

"The granting of orphan drug designation in Europe represents another important milestone for our AMT-130 program," said Matthew Kapusta, chief executive officer of uniQure. "Huntington's disease affects approximately 70,000 people in the U.S. and Europe[1], making this one of the largest clinical unmet needs in the rare disease field. We expect to file an Investigational New Drug application later this year and be the first AAV-gene therapy to enter clinical development for Huntington's disease."

To qualify for OMPD in Europe a therapy must be intended for the treatment of a disease that is life-threatening or chronically debilitating and have a patient prevalence in the European Union of no more than 5 in 10,000. OMPD offers product market exclusivity for ten years in the European Union following regulatory approval, along with tax and financial incentives for companies developing medicines for such orphan indications.

About Huntington's disease
Huntington's disease is a rare, inherited neurodegenerative disorder that leads to loss of muscle coordination, behavioral abnormalities and cognitive decline, resulting in complete physical and mental deterioration over a 12- to 15-year period of time. The disease is caused by the expansion of CAG trinucleotide in exon 1 of a multifunctional gene coding for protein called huntingtin. 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 hemophilia, Huntington's disease and cardiovascular diseases.
www.uniQure.com

1. http://www.ema.europa.eu

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, the development of our gene therapy product candidates, the success of our collaborations and the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies and/or development of our product candidates, and the scope of protection provided by our patent portfolio. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our and our collaborators' clinical development activities, 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 Quarterly Report on Form 10-Q filed on November 1, 2017. 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.

uniQure Contacts

For Investors:

Maria E. Cantor
Direct: 339-970-7536
Mobile: 617-680-9452
m.cantor@uniQure.com

Eva M. Mulder
Direct: +31 20 240 6103
Mobile: +31 6 52 33 15 79

e.mulder@uniQure.com

For media:

Tom Malone
Direct: 339-970-7558
Mobile: 339-223-8541
t.malone@uniQure.com