



uniQure Announces FDA Clearance of Investigational New Drug Application for AMT-130 in Huntington's Disease

January 22, 2019

~ AMT-130 Poised to Become First AAV Gene Therapy to Enter the Clinic for Huntington's Disease ~

~ Lead Product Candidate from Company's Proprietary miQURE™ Gene Silencing Platform ~

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Jan. 22, 2019 (GLOBE NEWSWIRE) -- [uniQure N.V.](#) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the U.S. Food and Drug Administration (FDA) has completed its review of the Company's Investigational New Drug (IND) application for [AMT-130](#), and the IND is now effective allowing uniQure to begin its planned Phase I/II study. AMT-130 comprises a recombinant AAV5 vector carrying a DNA cassette encoding a microRNA that non-selectively lowers or knocks-down human huntingtin protein in [Huntington's disease](#) patients.

"The FDA's clearance of our IND for AMT-130 is a significant milestone for Huntington's disease patients and an important event in the field of gene therapy," said [Matt Kapusta](#), chief executive officer at uniQure. "We expect that AMT-130 will be the first one-time administered AAV gene therapy to enter clinical testing for the treatment of Huntington's disease, a devastating neurodegenerative disorder for which there is no approved disease-modifying treatment.

"AMT-130 also represents the first clinical-stage AAV-based therapy specifically designed to silence an abnormal gene in the brain with a single administration, and we believe our proprietary miQURE™ gene silencing platform has the potential to be applied to many other diseases, such as spinocerebellar ataxia type 3 (SCA3)," continued Mr. Kapusta. "This achievement is a major milestone for uniQure's research organization, who have dedicated years of effort with the hope we can one day offer treatment for the many patients waiting generations for an effective therapy."

FDA clearance of the IND enables uniQure to initiate its planned dose-escalating, randomized and controlled Phase I/II clinical trial to assess the safety, tolerability and efficacy of a one-time treatment of AMT-130 in patients with Huntington's disease. uniQure expects to open several clinical sites in the United States and begin dosing patients in the second half of this year.

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. The disease is caused by an autosomal dominant mutation, CAG repeat expansion in the first exon of the huntingtin gene, that leads to the production of a mutated protein that aggregates in the brain. Despite the clear etiology of HD, 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 other severe genetic 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, the achievement of any of our planned near term or other milestones, our ability to become the first AAV Gene therapy for Huntington's Disease to begin clinical trials, our ability to initiate our planned dose-escalating, randomized and controlled Phase I/II clinical trial, our ability to open several clinical sites in the United States and begin enrolling patients in the second half of this year or ever, the development of our gene therapy product candidates, the ability to achieve therapeutic or curative effects in human patients in any of our product candidates, whether our proprietary miQURE™ gene silencing platform can be applied to any other diseases, such as spinocerebellar ataxia type 3 (SCA3), the ability to produce a product candidate that is safe and effective, the ability to obtain regulatory approval for any of our product candidates, 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. 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 collaboration activities, product development activities, 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 Annual Report on Form 10-K filed on March 14, 2018 and Quarterly Report on Form 10-Q filed on November 6, 2018. 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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