

uniQure Announces FDA Orphan Drug Designation for AMT-130 in Huntington's disease

~ First Investigational Gene Therapy in Huntington's Disease to Receive Designation ~

Lexington, MA and Amsterdam, the Netherlands, October 6, 2017 — [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 Orphan Drug Designation (ODD) by the U.S. Food and Drug Administration (FDA). 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.

ODD in the United States provides a special status for investigational drugs being developed for rare diseases considered to affect only up to 200,000 people in the U.S. The ODD programs offer product market exclusivity for up to seven years in the U.S. and ten years in the European Union following regulatory approval, along with tax and financial incentives for companies developing medicines for such orphan indications.

"Attaining orphan designation recognizes the potential that AMT-130 holds in delivering meaningful therapeutic benefit to patients suffering from this devastating disease," said [Matthew Kapusta](#), chief executive officer of uniQure. "It supports our ongoing development in Huntington's as we seek to bring the first gene therapy approach to this disease into the clinic next year."

AMT-130 consists of an AAV5 vector carrying an artificial micro-RNA which silences 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.

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

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 August 8, 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.

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