uniQure Presents New Preclinical Data on AMT-130 in Huntington's Disease at the ESGCT 25th Anniversary Congress in Berlin

-- One-time Administration of AMT-130 Demonstrates Survival Benefit and Functional Improvement of Huntington's Disease Symptoms in Preclinical Study --

-- IND-enabling Toxicology Study Initiated --

Lexington, MA and Amsterdam, the Netherlands, October 18, 2017 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today presented new preclinical data on AMT-130, its proprietary gene therapy candidate for the treatment of Huntington's disease (HD), at the European Society of Gene and Cell Therapy (ESGCT) 25th Anniversary Congress in Berlin, Germany.

Data from the study demonstrate that following administration of AMT-130 in Huntington's disease mouse models, significant improvements in both motor-coordination and survival were observed, as well as a dose-dependent, sustained reduction in huntingtin protein. AMT-130 comprises an AAV5 vector carrying a DNA cassette encoding an engineered micro RNA (miHTT) that silences the human huntingtin protein. The study on functional improvement and sustained huntingtin lowering was performed by members of uniQure's research department in collaboration with Charles River Discovery Research Services, Finland.

"We are confident that the combination of suppression of neuronal dysfunction, improvement of Huntington's disease symptoms, extended survival and long-term huntingtin lowering observed in these studies, could translate into patient benefit and improve their quality of life," stated Sander van Deventer, M.D., Ph.D., chief scientific officer at uniQure. "We have now begun our investigational new drug-enabling toxicology studies in rodents and non-human primates that will support an IND application for AMT-130 next year."

Preclinical Data Findings

This study builds on previous data generated at uniQure, demonstrating a long-term significant suppression of mutant huntingtin protein, the cause of Huntington's disease, after a single administration of AMT-130 in the Q175 mouse model of Huntington's disease.

The current study was conducted in the rapidly progressing R6/2 mouse model of Huntington's disease, which is characterized by early onset of motor symptoms and a much reduced life-span. A single administration of AMT-130 into the striatum was followed by a significant improvement of motor symptoms including improved coordination on the rotarod (a rotating cylinder to test coordination, physical condition and motor planning) as well as a significantly increased median survival from 120 to 149 days, compared with the control group (p = 0.0270). The data also demonstrate a significant reduction in expression of mutant huntingtin protein.

The functional improvements observed in these preclinical studies helped support the Orphan Drug Designation granted to AMT-130 by the U.S. Food and Drug Administration earlier this month and reinforce that huntingtin-lowering gene therapy for Huntington's disease could modify the course of this devastating 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 an autosomal dominant mutation, a cytosine-adenine-guanine (CAG) expansion, in the first exon of the huntingtin gene leading to a non-functional, aggregation

prone mutated protein. 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.

uniQure Contacts:

FOR INVESTORS:

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