uniQure Strengthens Intellectual Property Portfolio with Granted Patent Claims Covering AMT-130 for Huntington's Disease

~ Issued Patents in the U.S. and EU Cover RNA Construct Specifically Designed to Target Highly Toxic Exon1 Protein ~

Lexington, MA and Amsterdam, the Netherlands, May 22, 2019 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the issuance of two new patents covering AMT-130, the Company's gene therapy candidate for the treatment of Huntington's disease. 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 U.S. Patent and Trademark Office issued U.S. Patent 10,174,321 on January 8, 2019 and the European Patent Office issued EP 3237618 on May 22, 2019. The claims as granted cover the RNA constructs specifically designed to target exon1 and the embedding of these Huntington's disease RNA sequences into the miR451 scaffold, which is exclusively licensed to uniQure from Cold Spring Harbor Laboratory (CSHL). The claims also cover certain expression cassettes comprising the RNA constructs and the use of gene therapy vectors including AAV vectors encompassing the described expression cassettes.

AMT-130 incorporates the Company's proprietary miQURE™ gene silencing technology platform, which is designed to degrade disease-causing genes, without off-target toxicity, and induce silencing of the entire target organ through secondary exosome-mediated delivery. Preclinical studies of miQURE-based gene therapies have demonstrated several important advantages, including enhanced tissue-specificity, improved nuclear and cytoplasmic gene lowering and no off-target effects associated with impact on the normal cellular miRNA or mRNA mechanisms. miQURE technology also has been incorporated in the Company's gene therapy product candidate for spinocerebellar ataxia Type 3, another central nervous system disorder.

"We are very pleased with the continued expansion of our patent portfolio related to AMT-130, which has the potential to be the first one-time treatment for Huntington's disease patients and is expected to enter the clinic this year," stated Sander van Deventer, M.D., Ph.D., chief scientific officer at uniQure. "These patents cover our novel gene therapy approach to Huntington's disease, which we believe is the only investigational therapy specifically designed to silence both mutant huntingtin protein and the highly toxic exon1 protein fragment. In extensive preclinical studies to date, AMT-130 has demonstrated the potential to silence disease-causing protein in the brain, including within the cortex and the deep structures where Huntington's disease is known to manifest."

The U.S. Food and Drug Administration has granted orphan drug designation and Fast Track designation for AMT-130 in Huntington's disease. AMT-130 has received an Orphan Medicinal Product Designation from the European Medicines Agency making it the first investigational AAV-gene therapy in Huntington's disease to receive such designation. The company expects to initiate clinical testing for AMT-130 in the second half of 2019.

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 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 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 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, our ability to initiate dosing of a Phase I/II study of AMT-130 in the second half of 2019 or ever, our ability to open clinical sites for the Phase I/II study in the United States, whether AMT-130 becomes the first one-time administered AAV gene therapy for the treatment of Huntington's Disease, whether AMT-130 is able to successfully target deep brain structures, or alleviate the accumulation of the exon1 HTT fragment, or silence mutant huntingtin protein at levels greater than in other studies or at all, and whether AMT-130 will degrade disease-causing genes without off-target toxicity or induce silencing in the entire target organ through secondary exosome-mediated delivery or through any mechanism. 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, 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 Annual Report on Form 10-K filed on April 29, 2019. 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