uniQure Announces First Patient Treated in Dose-Confirmation Study of AMT-061 in Patients with Hemophilia B

~ Topline data expected to be available in the fourth quarter of 2018 ~

Lexington, MA and Amsterdam, the Netherlands, August 23, 2018 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today announced that it has treated the first patient in its Phase IIb dose-confirmation study of AMT-061, an investigational AAV5-based gene therapy incorporating the FIX-Padua variant for the treatment of patients with severe and moderately severe hemophilia B. AMT-061 has been granted Breakthrough Therapy Designation by the United States Food and Drug Administration and access to Priority Medicines (PRIME) regulatory initiative by the European Medicines Agency.

"The initiation of the AMT-061 dose-confirmation study is an important step toward our goal of advancing a potentially life-changing treatment for patients with hemophilia B," said Robert Gut, M.D., Ph.D., chief medical officer of uniQure. "I am extremely proud of the efforts made by the uniQure team to initiate this study, the objective of which is to demonstrate meaningful increases in FIX activity using the Padua variant and confirm dosing for the HOPE-B pivotal trial initiated this past June. We look forward to completing patient enrollment shortly and providing top-line data before the end of the year."

"As a one-time administered therapy, AMT-061 has the potential to transform the treatment paradigm for hemophilia B patients," said Annette von Drygalski, M.D., associate clinical professor at the University of California San Diego and director of its hemophilia and thrombosis treatment center. "By incorporating both AAV5 and the FIX-Padua variant, AMT-061 has the potential to deliver clinically relevant increases in FIX activity with low risk of cellular immune responses, which could expand patient eligibility for treatment with gene therapy. I greatly appreciate the opportunity to participate in the AMT-061 clinical program and view the initiation of the Phase IIb and Phase III studies as important milestones in the development of this potentially important therapy for patients with hemophilia B."

The Phase IIb dose-confirmation study is an open-label, single-arm, single-dose trial being conducted in the United States. Approximately three patients are expected to receive a single intravenous (IV) infusion of $2x10^{13}$ vc/kg and be evaluated for a period of approximately six to eight weeks to assess Factor IX (FIX) activity.

Phase III HOPE-B Pivotal Trial

Patient enrollment is also underway in the global Phase III HOPE-B clinical trial to evaluate the safety and efficacy of AMT-061. Approximately 50 adult hemophilia B patients classified as severe and moderately-severe will be enrolled in a six-month observational period during which time they will continue to use their current standard of care to establish a baseline control. After the six-month lead-in period, patients will go onto receive a single intravenous administration of AMT-061. Dosing of patients in the HOPE-B pivotal trial is expected to start early in the first quarter of 2019.

About AMT-061

AMT-061 consists of an AAV5 viral vector carrying a gene cassette with the Padua variant of Factor IX (FIX-Padua). FIX-Padua has been reported to provide an approximate 8 to 9-fold increase in FIX activity compared to the wild-type FIX protein, as used in AMT-060. AAV5-based gene therapies have been demonstrated to be safe and well-tolerated in a multitude of clinical trials, including three uniQure trials conducted in 22 patients in hemophilia B and other indications. No patient treated in clinical trials with the Company's AAV5 gene therapies has experienced any cytotoxic T-cell-mediated immune response to the capsid.

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 completion of enrollment in our Phase IIb study, the release of top-line clinical data, the ability to transform the treatment paradigm for hemophilia B patients or to deliver clinically relevant increases in FIX activity or to provide a low risk of cellular immune responses or to expand patient eligibility for treatment with gene therapy, the achievement of any of our planned near term or other milestones, 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. 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, 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.

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