uniQure Announces Hemophilia B Gene Therapy Program To Enter Pivotal Study With FIX-Padua Variant in 2018

~ AMT-060 with the FIX-Padua Modification (AMT-061) Demonstrates Substantial Increase in FIX Activity in Non-human Primates ~

~ Plans to Initiate Pivotal Study with Enhanced AMT-061 in 2018 ~

~ Achieves Alignment with FDA on Streamlined Clinical and Regulatory Strategy for AMT-061, Which Will be Included Under Existing Breakthrough Therapy Designation ~

~ Acquires a Patent Family Covering FIX-Padua in Hemophilia B ~

~ Conference Call Scheduled for Today at 8:30 a.m. ET ~

Lexington, MA and Amsterdam, the Netherlands, October 19, 2017 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that following multi-disciplinary meetings with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA), the company plans to expeditiously advance AMT-061, which combines an AAV5 vector with the FIX-Padua mutant, into a pivotal study in 2018 for patients with severe and moderately severe hemophilia B.

AMT-061 and AMT-060, the latter of which has been tested in 10 patients in an ongoing Phase I/II clinical trial, are identical in structure apart from two nucleotide substitutions in the coding sequence for FIX. The gene variant, referred to as FIX-Padua, expresses a protein with a single amino acid substitution that has been reported in multiple preclinical and nonclinical studies to provide an approximate 8 to 9-fold increase in FIX activity compared to the wild-type FIX protein. All other critical quality attributes of AMT-061 are expected to be comparable to those of AMT-060, as AMT-061 utilizes the same AAV5 capsid and proprietary insect cell-based manufacturing platform.

“Our mission in hemophilia B has always been to develop the safest and most effective gene therapy with the broadest application to patients. We believe AMT-061 moves us closer to this goal, as it has the potential to provide optimized clinical and tolerability benefits to nearly all severe and moderately severe patients with hemophilia B,” stated Matthew Kapusta, chief executive officer of uniQure. “We are delighted to have received constructive guidance from both the FDA and EMA, which we believe allows us to expeditiously advance AMT-061 into a pivotal study next year, as previously planned. In anticipation of this, we have begun GMP production of AMT-061 in our Lexington facility and preparations for the pivotal study are underway.”

“I believe AMT-061 has the potential to be an important gene therapy for patients suffering with hemophilia B,” stated Steven Pipe, M.D., professor of pediatrics and pathology and pediatric medical director of the hemophilia and coagulation disorders program at the University of Michigan. “Based on the data generated to date, AMT-061 may be the first gene therapy to provide durable, curative benefits to nearly all patients with hemophilia B, without the complications associated with capsid-related immune responses. I very much look forward to serving as an investigator in this exciting Phase III program.”

Clinical and Regulatory Pathway for AMT-061

- The FDA has agreed that AMT-061 will be included under the existing Breakthrough Therapy designation and Investigational New Drug (IND) for AMT-060. The EMA also has agreed that AMT-061 will be included under the current PRIME designation.

- The Company achieved general agreement with the FDA and EMA on the proposed pivotal trial plan for AMT-061. The study is expected to be an open-label, single-dose, multi-center, multi-national trial investigating the efficacy and safety of AMT-061 administered to adult patients with severe or moderately severe hemophilia B. The primary objective of the trial is to evaluate AMT-061 for prevention of bleedings. Secondary objectives include additional efficacy and safety aspects. Patients will serve as their own control, with a baseline established during a six-month observational lead-in phase prior to treatment with AMT-061.

- Concurrent with the start of the six-month lead-in phase of the pivotal study, a short dose-confirmation study is expected to begin in the third quarter of 2018. Three patients will receive a single intravenous (IV) dose of AMT-061 at 2 x 10^{13} gc/kg and will be evaluated for a period of approximately six weeks to assess FIX activity levels and confirm the dose. Each patient will continue to be followed longer term, and no lead-in phase is required for the dose confirmation study.

AMT-061 Nonclinical Data Demonstrate Tolerability and Substantial Increases in FIX Activity

- A Good Laboratory Practices (GLP), nonclinical study of AMT-061 has been performed in non-human primates at four different dose levels up to a dose of 9 x 10^{13} gc/kg. The purpose of this study was to compare AMT-061 to AMT-060 with respect to liver transduction, circulating FIX protein levels, circulating FIX activity levels and toxicity, after a single intravenous dose with 13- or 26-week observation periods.

- Data from the study demonstrated a strong correlation between dose and human FIX (hFIX) expression levels, as well as biological activity of the expressed hFIX protein. At equal doses, circulating vector DNA plasma levels, liver distribution, liver cell transduction and hFIX protein expression were comparable for both AMT-060 and AMT-061. Additionally, AMT-061 demonstrated substantial increases in hFIX clotting activity compared to AMT-060, consistent with those previously reported for FIX-Padua.

- Based on a statistical analysis of the AMT-061 and AMT-060 non-human primate data, as well as the clinical data from the Phase I/II trial of AMT-060, the Company believes that AMT-061 administered at a dose of 2 x 10^{13} gc/kg may lead to mean FIX activity of approximately 30 to 50 percent of normal.

- The study also examined toxicology of AMT-061, including liver enzyme activity, coagulation biomarkers and other safety parameters. Data from the study demonstrated that AMT-061 was well-tolerated with no evidence of any significant toxicological findings. There was no increased thrombin generation or increased fibrin formation or degradation detected during the six months of follow-up. No increase in immunogenicity is expected with AMT-061, as there are no changes in the AAV5 capsid.

AMT-061 Continues to Leverage AAV5's Favorable Tolerability and Immunogenicity Results

- AAV5-based gene therapies have been demonstrated to be generally safe and well-tolerated in a multitude of clinical trials, including three uniQure trials conducted in 22 patients in hemophilia B and other indications.

- In contrast to data reported using other AAV capsids delivered systemically via IV infusion, no patient treated in clinical trials with the Company's AAV5 gene therapies has experienced any confirmed, T-cell-mediated immune response to the capsid or material loss of FIX activity.

- An independent clinical trial has demonstrated that AAV5 has the lowest prevalence of preexisting neutralizing antibodies (NAb) compared to other AAV vectors. Data from the Phase I/II study of AMT-060 also demonstrated clinical proof-of-concept in the presence of preexisting NAb to AAV5, suggesting that all, or nearly all hemophilia B patients may be eligible for treatment with AMT-061.

Commercial-scale, GMP Manufacturing of AMT-061 Clinical Material Underway

- uniQure has initiated production of multiple clinical-grade batches of AMT-061 in its state-of-the-art Lexington, MA manufacturing facility. Material is being produced at commercial scale and utilizing current Good Manufacturing Practices (cGMP). uniQure expects to begin releasing product for the pivotal trial by the first quarter of 2018. The manufacturing process, controls and methods utilized for AMT-061 are consistent to those previously used for AMT-060.

- The Company has achieved alignment with the FDA and EMA on its plan to establish comparability between AMT-061 and AMT-060. uniQure expects to complete its ongoing comparability analysis and plans to submit the data to the agencies for review in the first quarter of 2018. Data reviewed to date support comparability between AMT-061 and AMT-060.

Exclusive Patent Covers the Use of Padua in Gene Therapy for Hemophilia B

- In a separate press release, uniQure today announced that it has acquired a patent family that broadly covers the FIX-Padua variant and its use in gene therapy for the treatment of coagulopathies, including hemophilia B. This family includes a patent issued in the U.S., as well as pending patent applications in Europe and Canada. uniQure recently filed divisional patent applications that would further strengthen its intellectual property position related to the FIX-Padua variant.

- The patent family was acquired from Professor Paolo Simioni, a renowned hemophilia expert at the University of Padua, Italy, who is widely recognized as the first to identify the mutation. Professor Simioni is serving as an advisor and consultant exclusively to uniQure for the development of gene therapy products using his invention. He is expected to assist the Company in its discussions with regulators, investigators and key opinion leaders throughout the clinical development of AMT-061.

Conference Call Information

uniQure will host a conference call today, October 19, 2017 at 8:30 a.m. ET to discuss this announcement. To access the live call by phone, dial (877) 280-2296 (United States) or +44 (0)20 3427 1900 (international); the conference ID is 2516119. The call may also be accessed through the Investors section of the Company's website at www.uniQure.com. Following the live webcast, a replay of the call will be available at the same location through November 2, 2017.

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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