uniQure Announces Updated, Long-Term Clinical Data from Ongoing Phase I/II Trial of AMT-060 In Patients with Severe Hemophilia B

-- Clinical Benefit Maintained in All Patients, with Durable Increases in FIX Clotting Activity at Up to Two Years of Follow-Up –

-- Second Dose Cohort Demonstrated 89% Reduction in Spontaneous Bleeds, with No Confirmed Bleeds Reported in Last 12 Months –

-- AAV5-Based Gene Therapy Continues to Show Favorable Safety Profile, with No Immune Responses or Loss of FIX Activity in Any Patient –

-- Company Preparing to Initiate Pivotal Study with the FIX-Padua Modification (AMT-061) in 2018 --

Lexington, MA and Amsterdam, the Netherlands, December 11, 2017 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced updated results from its ongoing, dose-ranging Phase I/II trial of AMT-060, its investigational gene therapy in patients with severe hemophilia B. The data includes up to two years of follow-up from the low-dose cohort and up to 18 months of follow-up from the second, higher-dose cohort.

The AAV5-based AMT-060 remains safe and well-tolerated with up to two years of follow-up, with no new serious adverse events and no development of inhibitors. No patient in the study has had any loss of Factor IX (FIX) activity or capsid-specific, T-cell-mediated immune response.

Eighteen-month follow-up data from the second-dose cohort continue to show stable FIX activity with substantial improvement in disease state in all five patients, including the discontinuation of routine prophylactic FIX infusions in all patients that previously required chronic replacement therapy. The annualized spontaneous bleeding rate for the second dose cohort declined 89% to a mean of 0.3 bleeds after gene transfer. In the last year of follow-up, no patient in the second cohort has reported any spontaneous bleeds.

These clinical data were presented this morning in an oral presentation at the 58th American Society of Hematology (ASH) Annual Meeting taking place in Atlanta, Georgia.

"We continue to observe a therapeutic benefit from AMT-060 that is clearly superior to patients' previous prophylactic FIX replacement therapy regimen, with stable elevations in Factor IX levels and a cessation of spontaneous bleeds," stated Professor Frank W.G. Leebeek, M.D. Ph.D. of the Erasmus University Medical Center in Rotterdam, the Netherlands.

"Most importantly, the AAV5-based AMT-060 remains safe and well-tolerated, with no loss of FIX activity, no activation of T-cell response and no development of inhibitors for any of the 10 patients in the study, up to two years

after treatment. The safety profile observed in this study continues to suggest that the AAV5 vector offers long-term safety, efficacy and the potential for broad application in hemophilia B patients."

uniQure announced in October that, following meetings with the FDA and EMA, it plans to initiate a pivotal study in 2018 with AMT-061, which combines an AAV5 vector with the FIX-Padua mutant. AMT-061 and AMT-060 are identical in structure apart from two nucleotide substitutions in the coding sequence for FIX. The gene variant, referred to as FIX-Padua, has been reported in multiple preclinical and nonclinical studies to provide an approximate 8 to 9-fold increase in FIX clotting activity compared to the wild-type FIX gene. 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.

"These data give us continued confidence that our AAV5-based gene therapies offer multi-year durability, superior safety and broad applicability as a result of a favorable immunogenicity profile compared to other AAV vectors," stated Matthew Kapusta, chief executive officer of uniQure. "We believe AMT-061 has the potential to provide curative benefits to nearly all hemophilia B patients, without the complications associated with capsid-related immune responses. Preparations for the pivotal study are underway and the manufacturing of AMT-061 for clinical use has been initiated."

Phase 1/2 Trial Overview

The AMT-060 gene therapy consists of a codon-optimized wild type FIX gene cassette, the LP1 liver promoter and an AAV5 viral vector manufactured by uniQure using its proprietary insect cell-based technology platform.

- The Phase I/II, open-label, multi-center study includes 10 patients each receiving a one-time, 30-minute, intravenous administration of AMT-060, without the prophylactic use of corticosteroids.

- The study includes two dose cohorts of five patients each, with the first cohort receiving $5x10^{12}$ gc/kg and the second cohort receiving $2x10^{13}$ gc/kg.

- Nine patients in the trial were classified as having severe (<1% FIX activity) hemophilia. One patient in the low-dose cohort had a moderate/severe (1.5% FIX activity) phenotype.

Data Update from Phase I/II Clinical Trial of AMT-060 in Hemophilia B Patients

Data as of October 26, 2017:

- All 10 patients in the study have demonstrated improvements in their disease state as measured by reduced FIX replacement therapy and bleeding frequency.

- In the second-dose cohort, no spontaneous bleeds have been reported in the last year of follow-up, with a reduction in the annualized spontaneous bleed rate of 89% compared to the one-year period prior to administration of AMT-060. Total bleeds were reduced by 75%.

- As previously announced, eight of the nine patients that required chronic FIX infusions prior to administration of AMT-060 have discontinued prophylaxis after treatment. All eight patients remained prophylaxis-free at the last follow-up.

- Across both dose cohorts, cumulative annualized FIX consumption decreased by 84%, from 2.64 million to 428,554 IU.

- Through up to 18 months of follow-up among the five patients in the second-dose cohort, the mean steady-state FIX activity persisted at approximately 7% of normal. The mean FIX activity at the last follow-up (18 months) was 8.1%, ranging from 4.2% to 11.1%.

About Hemophilia B

Hemophilia B is a serious and rare inherited disease in males characterized by insufficient blood clotting. The condition can lead to repeated and sometimes life-threatening episodes of external and internal bleeding following accidental trauma or medical interventions. Severe hemophilia is characterized by recurrent episodes of spontaneous joint bleeds, that cause long-term damage to the joints resulting in disabling arthropathy. Bleeds may be fatal if they occur in the brain. The deficient blood clotting results from the lack of functional human Factor IX, or hFIX. Treatment of hemophilia B today consists of prophylactic or on-demand protein replacement therapy, in which one to three times weekly intravenous administrations of plasma-derived or recombinant hFIX are required to prevent bleeding and once daily infusions in case bleeding occurs. Hemophilia B occurs in approximately 1 out of 30,000 live births.

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 transition to our AMT-061 product candidate, 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 November 1, 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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