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 FIX Activity Persisting At Up To 18 Months of Follow-Up –

-- Second-dose Cohort Demonstrates Dose Response Up to One Year, with 84% Reduction in Spontaneous Bleeds and All Patients Free of Prophylactic FIX Replacement Therapy –

-- No Activation of T-Cell Responses or Loss of FIX Activity in Any Patient Up To 18 Months –

Lexington, MA and Amsterdam, the Netherlands, July 10, 2017 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, will today announce updated results from its ongoing, dose-ranging Phase I/II trial of AMT-060, its proprietary, investigational gene therapy in patients with severe hemophilia B. The data includes up to 18 months of follow-up from the low-dose cohort and up to one year of follow-up from the second dose cohort.

The AAV5-based AMT-060 remains safe and well-tolerated with up to a year and a half of follow-up, with no serious adverse events and no development of inhibitors. All patients are now past one year of follow up with no loss of Factor IX (FIX) activity and no capsid-specific T-cell activation.

One-year follow-up data from the second-dose cohort continue to show a dose response 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 84% to a mean of 0.5 annual bleeds after gene transfer. During more than 1,700 cumulative patient days of observation, only one patient in the second cohort reported two unconfirmed spontaneous bleeds, and no such bleeds were reported by any patient during the last six months of observation.

These clinical data will be presented today in an oral presentation at the 26th Biennial Congress of the International Society on Thrombosis and Haemostasis (ISTH), taking place this week in Berlin, Germany.

“We continue to observe a therapeutic benefit from AMT-060 that is clearly superior to their previous prophylactic FIX replacement therapy regimen, even in patients with advanced joint disease who experienced a high rate of bleeds prior to gene transfer,” stated Professor Wolfgang Miesbach, M.D., of the University Hospital Frankfurt, Germany.

“Importantly, AMT-060 appears to be safe and well-tolerated, and quite differentiated, with no loss of FIX activity, no activation of T-cell response and no development of inhibitors for any of the ten patients in the study. The safety profile observed in this study suggests that the AAV5 vector offers long-term safety, efficacy and the potential for broad application in hemophilia B patients,” he added.

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. It is the only hemophilia gene therapy that combines a gene cassette with clinically proven multi-year durability, now out more than five years, and an AAV5 vector serotype that has demonstrated superior safety and broad applicability due to the low prevalence of clinically-relevant titers of neutralizing antibodies (NABs) as evaluated in more than 20 patients across clinical studies in three different diseases.

- 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 5x1012 gc/kg and the second cohort receiving 2x1013 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.

- Patients in the low-dose cohort were characterized by poorly controlled bleeding manifestations despite use of high-dose FIX replacement therapy during the year prior to study compared to the second-dose cohort.

- All but one patient in the study across both cohorts required chronic infusions of prophylactic FIX therapy at the time of enrollment. The remaining patient, who is in the second dose cohort, used FIX therapy on demand.

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

Data as of May 12, 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 were reported in the last six months of follow-up, with a reduction in the annualized spontaneous bleed rate of 84% compared to the one-year period prior to administration of AMT-060. Total bleeds were reduced by 64%.

- 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 79%, from 2.64 million to 544,741 IU.

- Through up to 12 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 (52 weeks) was 8.82%, ranging from 5.2% to 10.7%.

- AMT-060 continues to be well-tolerated, and there have been no severe adverse events.

- In both dose cohorts, FIX activity remained consistent and stable through up to 18 months of follow-up with no emergence of late immune response or loss of FIX activity in any of the patients.

- As previously announced, three patients experienced mild, asymptomatic elevations of alanine aminotransferase (ALT) soon after administration. For these patients, ALT levels returned to their baseline readings, no recurrence of ALT elevation has occurred, and no loss of FIX activity was observed.

- No patients across either cohort have developed inhibitory antibodies against FIX, or demonstrated sustained AAV5 capsid-specific T-cell activation.

"The data from our Phase I/II study demonstrate that AMT-060 continues to deliver sustained and significantly improved clinical benefits over the long term to patients suffering from severe hemophilia B," stated Matt Kapusta, chief executive officer of uniQure. "Our AAV5-based gene therapy has been clinically demonstrated to be safe, effective and durable, with no loss of efficacy at up to 18 months of observation and no cellular immune responses in any patient. Moreover, our use of an AAV5 construct may enable us to offer the promise of gene therapy to nearly all patients suffering from hemophilia B. We continue to make significant progress preparing for our manufacturing campaign and regulatory interactions to support a potential pivotal study, and look forward to providing an update later this year."

AMT-060 is being co-developed with Chiesi for Europe.

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, statements regarding the development of our gene therapy product candidates, including the future development of AMT-060, 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 corporate reorganizations and strategic shifts, collaboration arrangements, our and our collaborators' clinical development activities, 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 2016 Annual Report on Form 10-K filed on March 15, 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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