uniQure Provides Update on Dose-Confirmation Study of AMT-061 in Patients with Hemophilia B

September 24, 2018

~ Third patient treated in Phase IIb study ~

~ Topline data expected to be available before the end of 2018 ~

LEXINGTON, Mass and AMSTERDAM, the Netherlands, Sept. 24, 2018 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today announced that the third patient has been treated in the Company's 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 Medicine (PRIME) regulatory initiative by the European Medicines Agency.

The Phase IIb dose-confirmation study is an open-label, single-arm, single-dose trial being conducted in the United States to confirm the dose of AMT-061 prior to patient treatment in the ongoing Phase III HOPE-B pivotal trial. Three patients received a single intravenous (IV) infusion of $2x10^{13}$ vc/kg over the past month and are currently under evaluation to assess safety and Factor IX (FIX) activity.

"We are very pleased with the progress we have made in the clinical development of AMT-061, our lead gene therapy product candidate for hemophilia B," stated Matthew Kapusta, chief executive officer of uniQure. "The treatment of the third patient puts us in the position to present topline data and confirm dosing for our Phase III HOPE-B pivotal study by the end of the year."

"We also have made advancements in the execution of the HOPE-B pivotal study, which now includes multiple recruiting sites and enrolled patients," he added. "We believe that AMT-061 has the potential to be a leading gene therapy treatment that provides durable, clinically relevant increases in FIX activity with a favorable immunogenicity profile which could expand patient eligibility for treatment with our gene therapy."

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 comparator. After the six-month lead-in period, patients will go on to 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 our Phase IIb study, the confirmation of the dose for AMT-061, the release of top-line clinical data by the end of this year, the ability for AMT-061 to be a leading gene therapy treatment for hemophilia B patients or to deliver clinically relevant increases in FIX activity or to provide a favorable immunogenicity profile *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 such as the dosing of patients in the HOPE-B pivotal trial, and/or the development and regulatory approval of our product candidates in the United States or in Europe. 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 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.*

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