uniQure Announces First Patient Treated in HOPE-B Pivotal Trial of AMT-061 in Patients with Hemophilia B

February 4, 2019

Full Patient Enrollment in Study Expected by Year-End 2019

LEXINGTON, Mass. and AMSTERDAM, The Netherlands, Feb. 04, 2019 (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 it treated the first patient in its HOPE-B pivotal trial of AMT-061, an investigational AAV5-based gene therapy incorporating the patent-protected 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.

“We are very pleased to have successfully administered AMT-061 to the first patient enrolled in the HOPE-B pivotal trial and mark this as a milestone for the field in advancing a potential one-time 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 advance this study, and we plan to complete full patient enrollment in this study before the end of this year.”

“Our mission in hemophilia B has always been to be first to market with a best-in-class gene therapy,” said Matt Kapusta, chief executive officer. “With the initiation of the dosing phase of our pivotal study for AMT-061 and the data generated to date in our Phase IIb study, we believe that we are on track toward our goal of developing the first gene therapy that provides durable, functionally-curative benefits to nearly all patients with hemophilia B, without the complications associated with capsid-related immune responses or the need for immunosuppression therapy.”

The global HOPE-B Phase III clinical trial will evaluate the efficacy and safety of AMT-061. Approximately 50 adult patients with hemophilia B classified as severe or 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 prospective comparator. After the six-month lead-in period, patients will receive a single intravenous administration of AMT-061. The primary outcome measure is the assessment of Factor IX activity 26 weeks after AMT-061 dosing. Secondary outcome measures include annualized bleeding rate (ABR) and usage of Factor IX replacement therapy over a 52-week time frame, as well as other efficacy and safety aspects. Post-treatment, patients will be followed for 5 years.

About AMT-061

AMT-061 consists of an AAV5 viral vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX-Padua). uniQure holds multiple issued patents in the United States and Canada broadly covering methods of treating bleeding disorders, including hemophilia B, using AAV gene therapy with the FIX-Padua variant. Additional patents are pending in the European Union.

AAV5-based gene therapies have been demonstrated to be safe and well-tolerated in a multitude of clinical trials, including four uniQure trials conducted in 25 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. Additionally, preclinical and clinical data show that AAV5-based gene therapies may be clinically effective in patients with pre-existing antibodies to AAV5, thereby potentially increasing patient eligibility for treatment compared to other gene therapy product candidates.

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 other severe genetic 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 completion of full patient enrollment for the HOPE-B Phase III pivotal trial of AMT-061 before the end of this year, our ability to be first to market, our ability to develop a best-in-class gene therapy, our ability to develop a gene therapy that provides durable, functionally-curative benefits to nearly all patients with hemophilia B, without the complications associated with capsid-related immune responses or the need for immunosuppression, the ability of AMT-061 to be a successful gene therapy treatment or to deliver sustained increases in FIX activity or to provide a favorable immunogenicity profile or to eliminate the risk of an immune response, the determination that any of AMT-060, AMT-061 and the AAV5 capsid used in those product candidates are safe or effective or will be determined by any regulatory body to be safe and effective, 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 in the first quarter of 2019 or at any time, 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 November 6, 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:		FOR MEDIA:
Maria E. Cantor	**Eva M. Mulder**	**Tom Malone**
Direct: 339-970-7536	Direct: +31 20 240 6103	Direct: 339-970-7558
Mobile: 617-680-9452	Mobile: +31 6 52 33 15 79	Mobile: 339-223-8541
m.cantor@uniQure.com	e.mulder@uniQure.com	t.malone@uniQure.com