uniQure Announces Achievement of Planned Enrollment in HOPE-B Pivotal Trial of AMT-061 (Etranacogene Dezaparvovec) in Patients with Hemophilia B

Lexington, MA and Amsterdam, the Netherlands, September 3, 2019 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today announced that the planned enrollment of 56 patients has been achieved in the HOPE-B pivotal trial of etranacogene dezaparvovec (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. Due to the high level of interest in the study from both patients and study investigators, uniQure expects to over-enroll up to six additional patients before the end of September. Etranacogene dezaparvovec 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 extremely pleased to reach this important milestone in our ongoing development of etranacogene dezaparvovec, which we believe has the potential to be the first and best-in-class gene therapy for patients with hemophilia B," stated Matt Kapusta, chief executive officer of uniQure. "We appreciate the tremendous support from the hemophilia patient community in achieving this important goal ahead of schedule and look forward to sharing top-line data from the Phase III trial, which we expect to do next year."

The pivotal Phase III HOPE-B study builds on the success of the interim 36-week results of the Company's ongoing Phase IIb study of etranacogene dezaparvovec, which demonstrated that a single administration of the investigational gene therapy resulted in sustained increases in Factor IX (FIX) levels up to 54% of normal, and a mean FIX level of 45% of normal. During that time, no patient reported any bleeding events or required any infusion of FIX replacement therapy for bleeds or experienced any material loss of FIX activity. Additionally, an ongoing Phase I/II study of AMT-060, the Company's first-generation gene therapy for the treatment of hemophilia B, demonstrated that all 10 patients continue to show sustained and stable increases in FIX activity and long-term clinical benefits at up to 3.5 years of observation.

"We are very excited and proud to have achieved the targeted patient enrollment in the HOPE-B study in just a little more than one year from study initiation," added Robert Gut, M.D., Ph.D., chief medical officer at uniQure. "This multi-center, multinational trial involves 39 clinical sites across 9 countries, and highlights the outstanding effort and passion of our clinical operations, clinical development, medical affairs and project management teams. We would like to thank all study participants, advisors, primary investigators and the whole study staff in the United States and Europe for their great contribution and support."

About the Pivotal Phase III HOPE-B Study

The pivotal Phase III HOPE-B trial is a multinational, open-label, single-arm study to evaluate the safety and efficacy of etranacogene dezaparvovec. Adult hemophilia B patients classified as severe or moderately severe are enrolled in a six-month observational period during which time they will continue to use their current standard of care to establish a baseline control. After the six-month lead-in period, patients will receive a single intravenous administration of etranacogene dezaparvovec at the $2x10^{13}$ gc/kg dose. Dosing of patients in the HOPE-B pivotal trial was initiated in January 2019.

The study's primary endpoint is the assessment of Factor IX activity 26 weeks after dosing. Secondary endpoints 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.

Patients enrolled in the HOPE-B pivotal trial will be tested for the presence of pre-existing neutralizing antibodies to AAV5 but will not be excluded from the trial based on their titers. Previous studies performed by uniQure suggest that AAV5-based gene therapies may be viable treatments for at least 97% of patients.

About Etranacogene Dezaparvovec (AMT-061)

Etranacogene dezaparvovec, also known as AMT-061, consists of an AAV5 viral vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX-Padua). AAV5-based gene therapies have been demonstrated to be safe and well tolerated in many 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-based 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 gene therapies to treat patients with hemophilia B, hemophilia A, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other 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 ability of etranacogene dezaparvovec to be a first-in-class or best-in-class gene therapy for patients with hemophilia B, the ability to add up to six additional patients before the end of September 2019, and whether top-line data from the Phase III trial can be shared next year or ever. 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 July 29, 2019. 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:

Maria E. Cantor
Direct: 339-970-7536
Mobile: 617-680-9452
m.cantor@uniQure.com

Eva M. Mulder
Direct: +31 20 240 6103
Mobile: +31 6 52 33 15 79
e.mulder@uniQure.com

FOR MEDIA:

Tom Malone
Direct: 339-970-7558
Mobile: 339-223-8541
t.malone@uniQure.com