uniQure Announces Achievement of Target Patient Dosing in HOPE-B Pivotal Trial of AMT-061 (Etranacogene Dezaparvovec) in Hemophilia B

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Provides Update on Phase I/II Clinical Trial of AMT-130 in Huntington's Disease and Response to COVID-19 Pandemic

LEXINGTON, Mass. and AMSTERDAM, March 26, 2020 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today announced it has achieved the targeted dosing of patients 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. The targeted number of patients to be dosed per the clinical trial protocol was 50. In total, 54 patients have received the one-time dose of etranacogene dezaparvovec.

"I am very proud of the entire uniQure team, our investigators, study coordinators and the hemophilia patient communities who helped make this major milestone possible," stated Matt Kapusta, chief executive officer of uniQure. "With target patient dosing now finished, we are closely monitoring the trial and working within guidance provided by the FDA regarding COVID-19 to minimize any risk or disruption in patient follow-up visits. We continue to expect top-line data from the Phase III trial before the end of this year, which we believe will support a BLA submission in 2021."

The pivotal Phase III HOPE-B trial follows the Company's ongoing Phase IIb trial of etranacogene dezaparvovec, in which we have previously reported that, after 52 weeks of follow-up data, all three patients had stabilized and sustained FIX activity at therapeutic levels after the one-time administration of etranacogene dezaparvovec. Additionally, in an ongoing Phase I/II trial of AMT-060, the Company's first-generation gene therapy for the treatment of hemophilia B, all 10 patients continued to show sustained and stable increases in FIX activity and long-term clinical benefit, including improved disease phenotype and substantial reductions in spontaneous bleeds at up to 4 years of observation.

Update on the Phase I/II Clinical Trial of AMT-130 in Huntington's Disease

The first two patients in the Company's Phase I/II clinical trial of AMT-130 in Huntington's disease have been enrolled after successfully meeting all screening and eligibility criteria. These patients were scheduled to have their procedures on March 24 and 25 at the Ohio State University. Due to the expanding impact of the COVID-19 coronavirus pandemic, these procedures have been temporarily postponed. The decision to postpone treatment in the trial follows the COVID-19-related State of Emergency declarations in the United States, where the trial is taking place.

"We are following all federal and local regulations including the FDA guidance on clinical trial conduct during the COVID-19 pandemic and we are working very closely with trial investigators to ensure that patient safety remains our top priority," stated Robert Gut, M.D., Ph.D., chief medical officer at uniQure. "Despite this unexpected postponement, we are encouraged by the progress that we have made with this trial since the beginning of the year, and the fact that we have patients who are highly motivated to participate. We will continue our work to resume treatment in the Phase I/II trial as soon as it is clinically appropriate."

uniQure's Response to COVID-19

The Company is continuing its operations under guidance from national, state and local authorities in the Netherlands and in the United States, including the US Food and Drug Administration (FDA) and Centers for Disease Control and Prevention (CDC). In this regard, uniQure is informed by the direction and flexibility provided by the FDA in its March 18, 2020 Guidance entitled **"FDA Guidance on Conduct of Clinical Trials of Medical Products during COVID-19 Pandemic"**. Ongoing clinical research activities are being closely monitored to minimize any risk, disruption or delay in either patient dosing or follow-up visits.

uniQure is focused on ensuring the health and wellbeing of its global workforce. As of March 13, the Company mandated a work-from-home policy for all non-essential employees. As a biopharma research and development company, uniQure is deemed to provide Essential Services under the "stay at home" advisory that was issued by the Governor of Massachusetts on March 23, 2020 and is maintaining its mission-critical activities that include commercial-scale manufacturing operations.

About the Pivotal Phase III HOPE-B Trial

The pivotal Phase III HOPE-B trial is a multinational, open-label, single-arm study to evaluate the safety and efficacy of etranacogene dezaparvovec in 50 patients. 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 trial'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. 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.

About AMT-130

AMT-130 is our novel gene therapy candidate for the treatment of Huntington's disease, a severe genetic neurodegenerative disorder causing loss of muscle coordination, behavioral abnormalities and cognitive decline, often resulting in complete physical and mental deterioration over a 12 to 15-year period. AMT-130 utilizes our miQURE™ proprietary, gene-silencing platform and incorporates an AAV vector carrying a microRNA specifically designed to silence the huntingtin gene and the potentially highly toxic exon 1 protein fragment. AMT-130 has received orphan drug and fast track designations from the FDA and Orphan Medicinal Product Designation from the EMA.

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, whether we will obtain top-line data from the HOPE-B Phase III trial before the end of this year or ever, whether such data will support a BLA submission in 2021, whether we will be able to resume treatment in the Phase I/II trial as soon as it is clinically appropriate, and our expectation to continue our operations during the COVID-19 pandemic. 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 Annual Report on Form 10-K filed on March 2, 2020. 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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