uniQure Announces FDA Breakthrough Therapy Designation for AMT-060 in Hemophilia B

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LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Jan. 30, 2017 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ:QURE), a leader in human gene therapy, today announced that AMT-060, its proprietary, investigational gene therapy in patients with severe hemophilia B, has received Breakthrough Therapy designation by the U.S. Food and Drug Administration (FDA). This designation is based on results from the ongoing, dose-ranging Phase 1-2 study that show sustained increases in Factor IX (FIX), reductions in FIX replacement usage and a near cessation of spontaneous bleeding in patients with severe disease at up to 12 months follow-up.

"We are very pleased the FDA has designated AMT-060 a Breakthrough Therapy for patients with hemophilia B," stated Matthew Kapusta, chief executive officer of uniQure. "The FDA's decision to prioritize and expedite the review of AMT-060 is an important milestone for uniQure and we are committed to working closely with the FDA to rapidly advance our hemophilia B program into late-stage development."

Phase 1-2 Data

Updated clinical data from the ongoing, two-cohort Phase 1-2 trial of AMT-060 were recently presented at the 58th American Society of Hematology (ASH) Annual Meeting. The data included up to 52 weeks of follow-up from the low-dose cohort and up to 31 weeks of follow-up from the second dose cohort.

Data from the second-dose cohort show a dose response with improvement in disease state in all five patients, including the discontinuation of precautionary FIX infusions in all four patients that previously required chronic replacement therapy. As of the data cutoff date for the ASH presentation, only one unconfirmed spontaneous bleed was reported during an aggregate of 94 weeks follow-up after discontinuation of prophylactic FIX replacement therapy.

All five patients in the low-dose cohort, whose bleedings were previously uncontrolled despite being managed with prophylactic therapy, continue to maintain, constant and clinically meaningful levels of FIX activity for up to 52 weeks post treatment, resulting in a complete cessation of spontaneous bleedings in the last 14 weeks of observation.

AMT-060 continues to be well-tolerated, and there have been no severe adverse events. Three out of the total of 10 patients (two in the second-dose cohort and one previously reported from the low-dose cohort) experienced mild, asymptomatic elevations of alanine aminotransferase (ALT) and received a tapering course of corticosteroids per protocol. Importantly, the temporary elevations in ALT were not associated with any loss of endogenous FIX activity or T-cell response to the AAV5 capsid.

No patients across either cohort have developed inhibitory antibodies against FIX and no patients screened in the study tested positive for anti-AAV5 antibodies.

About Breakthrough Therapy Designation

The 2012 Food and Drug Administration Safety and Innovation Act (FDASIA) established the Breakthrough Therapy designation to expedite the development and review of new drugs with preliminary clinical evidence demonstrating that they may offer a substantial improvement over available therapies for patients with serious or life-threatening diseases. The Breakthrough Therapy designation is a distinct status from both accelerated approval and priority review, which can also be granted to the same drug if relevant criteria are met. According to FDA data for its fiscal year 2016, the Center for Biologics Research and Review received a total of 23 requests for Breakthrough Therapy designation with only 4 designations granted - or 17% of all requests.

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 implementation and effects of the Company's new strategic and organizational changes, the development of our gene therapy product candidates, 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 2015 Annual Report on Form 20-F filed with the Securities and Exchange Commission on April 4, 2016. 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:

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

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

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