



uniQure Receives European Medicines Agency Priority Medicines (PRIME) Designation for AMT-060 in Hemophilia B

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LEXINGTON, Mass. and AMSTERDAM, the Netherlands, April 25, 2017 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ:QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that AMT-060, its proprietary, investigational gene therapy in patients with severe hemophilia B, has received PRIME designation by the European Medicines Agency (EMA). This designation is based on results from the ongoing, dose-ranging Phase 1-2 study that show a near cessation of spontaneous bleeding in patients with severe disease at up to 12 months follow-up, clinically significant and sustained increases in Factor IX (FIX) and substantial reductions in FIX replacement usage.

"We are very pleased to have AMT-060 for hemophilia B accepted into the PRIME program," stated Matthew Kapusta, chief executive officer of uniQure. "Similar to the Breakthrough Therapy designation that AMT-060 received from the U.S. Food and Drug Administration earlier this year, we look forward to this enhanced collaboration with the EMA to advance the clinical development of this potentially transformative therapy for hemophilia B patients."

Phase 1-2 Data

Updated clinical data from the ongoing, two-cohort Phase 1-2 trial of AMT-060 were presented last December 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 substantial 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. To date, only one spontaneous bleed was reported 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 robust, constant and clinically meaningful levels of FIX activity for up to 52 weeks post treatment, with 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.

No patients across either cohort have developed inhibitory antibodies against FIX, or demonstrated sustained AAV5 capsid-specific T-cell activation.

About PRIME Designation

The PRIME program was launched by the EMA in March 2016, and the designation is designed to aid and expedite the regulatory process for investigational medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients without treatment options. To be accepted, an investigational medicine must show the potential to benefit patients with unmet medical needs based on early clinical data. Medicines accepted into the PRIME program are considered priority medicines within the European Union (EU).

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 winding down of our Glybera program, the development of our other 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 2016 Annual Report on Form 10-K filed on March 15, 2017. 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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