



uniQure Announces Preliminary Topline Results from Low-Dose Cohort in Hemophilia B Phase I/II Gene Therapy Clinical Trial

January 7, 2016

--Meaningful Factor IX Expression Validates Successful Transduction of the Liver Using uniQure's Proprietary AAV5 Vector--

--Four of Five Patients Have Fully Discontinued Prophylactic Recombinant Factor IX Therapy--

--Conference Call to Discuss Data Scheduled for 8:30 am EST Today, January 7--

AMSTERDAM, the Netherlands, Jan. 7, 2016 (GLOBE NEWSWIRE) -- uniQure N.V. (Nasdaq:QURE), a leader in human gene therapy, today announced preliminary topline results from the low-dose cohort of an ongoing Phase I/II clinical trial being conducted in adult hemophilia B patients treated with uniQure's novel AAV5/FIX gene therapy, AMT-060. All five patients in the low-dose cohort had Factor IX (FIX) phenotypic features of severe or moderately-severe hemophilia including documented Factor IX (FIX) levels less than 1-2% and required chronic treatment with prophylactic recombinant FIX (rFIX) therapy at the time of enrollment.

The first two patients out of five in the low dose cohort have completed at least 20 and 12 weeks of follow up and had central-lab-confirmed FIX expression levels of 5.5% and 4.5% of normal, respectively at the cutoff date of December 16th, 2015. The three additional patients have been dosed, but had not achieved the full 12 weeks of follow-up at the cutoff date. However, as of January 6, 2016, four of the five patients, including the first two patients enrolled in the study, have met a secondary objective in the trial by fully discontinuing prophylactic rFIX. The 12 week follow-up, post AMT-060 administration, marks the period in which investigators in the trial attempt discontinuation of prophylactic rFIX, based on FIX expression levels. The first patient in the low-dose cohort experienced a mild, transient and asymptomatic elevation of transaminase levels at around 10 weeks post treatment. This patient received a short, 8-week course of tapering prednisolone doses with rapid return of transaminase levels to baseline values. No elevated transaminase levels have been observed in the other four patients thus far, with all patients being on therapy for at least 10 weeks as of January 6, 2016.

AMT-060 consists of a codon-optimized wild type FIX gene and the LP1 liver promoter together with the AAV5 viral vector, manufactured using uniQure's proprietary insect cell based manufacturing technology. AMT-060 is administered, without immunosuppressant therapy, through the peripheral vein in one treatment session for approximately 30 minutes. The study includes two cohorts, with the low-dose cohort using 5x10¹² gc/kg and the high-dose cohort using 2x10¹³ gc/kg. Thus far, there have been no patient screening failures due to pre-existing neutralizing antibodies against AAV5 and no patients have developed inhibitory FIX antibodies.

These early data from the low-dose cohort suggest that AMT-060 is generally well-tolerated and capable of successfully transducing the liver resulting in clinically meaningful FIX expression levels. This current trial uses a starting dose of AAV5/FIX gene therapy that is similar to the highest dose of the same FIX gene cassette evaluated in a study conducted by Prof. Amit Nathwani and the St. Jude Children's Hospital using an AAV8 serotype vector, and uniQure's preliminary data are comparable with the endogenous FIX expression levels achieved in the St. Jude study. The results of the St. Jude study, which were published in the *New England Journal of Medicine* in 2011 and 2014, demonstrated that a durable mean FIX expression of 5.1% of normal (range 2.9% to 7.2%) can be achieved with this gene cassette and result in meaningful long-term clinical benefits for patients. In the St. Jude study, four of six patients treated at the high dose had transient elevations of transaminase levels, managed with a tapering prednisolone regimen. The FIX gene cassette used in the St. Jude study is exclusively licensed by uniQure.

"Thus far, the overall tolerability and FIX expression profile in the low-dose cohort is encouraging for patients with hemophilia B and support the continuation of the study," commented Professor Frank W.G. Leebeek, M.D. Ph.D. of the Erasmus Medical Center in Rotterdam, an investigator in the study. "Previous studies have demonstrated that maintaining durable FIX expression around 3% to 5% of normal may have a significant clinical benefit as measured by significant reduction in consumption of units of FIX concentrate and lower risk of spontaneous bleeding episodes."

uniQure intends to present a more complete analysis of these data from this low-dose cohort at a scientific conference in the second quarter of 2016. Subject to the Data Monitoring Committee's approval, the Company also anticipates initiating enrollment of the high-dose cohort this quarter.

"These preliminary topline results support our hypothesis that AAV5/FIX can deliver clinically meaningful expression levels of FIX for patients with hemophilia B," commented Dan Soland, Chief Executive Officer of uniQure. "So far, our AAV5-based gene therapies have been systemically administered to 13 adult patients across two clinical studies in two different disease states, and via direct central nervous system administration in four children in a third study, providing us with a strong safety dataset on the AAV5 vector and our proprietary insect cell based manufacturing technology."

"Today, we are the only AAV gene therapy company in the world with both proprietary, commercial-scale manufacturing capabilities and encouraging clinical data across multiple diseases," continued Mr. Soland. "These preliminary results further support our modular platform approach and leadership in gene therapy."

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. The episodes can cause long-term damage, for example to the joints, and can 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 frequent intravenous administrations of plasma-derived or recombinant hFIX are required to stop or prevent bleeding. Hemophilia B occurs in approximately 1 out of 30,000 live births.

Conference Call and Webcast

uniQure will discuss the data in a webcast conference call at 8:30 am EST on Thursday, January 7, 2016. The webcast and the accompanying slides, which can be downloaded as a PDF file, can be located on uniQure's website at <http://www.uniqure.com/news/calendar-of-events/>. The conference call can be accessed by dialling one of the numbers listed below five minutes prior to the start of the call and providing the confirmation code: **1640435**.

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To ensure a timely connection to the webcast, it is recommended that users register at least 15 minutes prior to the scheduled start time. The webcast replay will be available for at least 72 hours following the call.

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 CNS, liver/metabolic and cardiovascular diseases. www.uniqure.com

uniQure Forward-Looking Statement

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 future development of AMT-060. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with 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 2014 Annual Report on Form 20-F filed with the Securities and Exchange Commission on April 7, 2015. 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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