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uniQure Presents Clinical Data from Ongoing Phase I/II Study in Hemophilia B Demonstrating 6 Months of Sustained Increases in Fa

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--Data from Low-Dose Cohort Presented at the 21st Congress of the European Hematology Association Show Durable and Therapeutically Relevant Factor IX (FIX) Expression--

--Median FIX Activity for All Five Patients in Low-Dose Cohort of 5.4%--

--Conference Call to Discuss Data Scheduled for 8:00 am EST / 2:00 pm CET Monday, June 13--

AMSTERDAM, the Netherlands, June 11, 2016 (GLOBE NEWSWIRE) -- uniQure N.V. (Nasdaq:QURE), a leader in human gene therapy, announced today that additional data from its Phase I/II clinical trial of AMT-060 in hemophilia B patients were presented at the 21st Congress of the European Hematology Association (EHA) in Copenhagen, Denmark. Study investigator Dr. Frank Leebeek, Professor of Hematology at Erasmus University Medical Center in Rotterdam, gave an oral presentation of a comprehensive overview of results from five patients that received a single administration of uniQure's novel gene therapy, AMT-060, at the initial low dose of $5x10^{12}$ gc/kg. Hemophilia B is a serious and rare inherited disease in males characterized by insufficient blood clotting caused by a mutation in the Factor IX (FIX) gene. The AMT-060 gene therapy consists of a codon-optimized wild type FIX gene cassette, the LP1 liver promoter and an AAV5 viral vector manufactured by uniQure using its proprietary insect cell-based technology platform.

All patients in the low-dose cohort saw improvements in their disease phenotype and achieved sustained increases in FIX activity, with a median of 5.4% (expressed as % of normal) at 6 months post treatment. Prior to the study, all five patients had features of severe or moderately-severe hemophilia, including documented FIX activity levels of ≤1.5%, and required chronic, prophylactic (precautionary) infusions of Factor IX concentrate to manage bleeding. After treatment with AMT-060, total usage of Factor IX concentrate declined substantially, with four patients remaining free of any prophylatic infusions through the April 26, 2016 cut-off date. The oldest patient in the cohort (72 years of age), who had severe joint disease prior to the study and continued prophylactic infusions of Factor IX therapy, also demonstrated improvement in disease phenotype.

"After six months of follow-up, I can say as a clinician who regularly treats hemophilia patients that the impact on the quality of life for these patients treated with AMT-060 is very positive," said Professor Leebeek. "The increases in FIX activity and the overall stability of the activity observed over a 6-month period are cause for optimism, as they are associated with meaningful clinical benefits as well as reduced need for ongoing infusions of recombinant FIX therapy."

AMT-060 has been well-tolerated thus far in the study. As noted previously, the first patient in the low-dose cohort experienced a mild, transient and asymptomatic elevation of alanine aminotransferase (ALT) 10 weeks after treatment. No cellular immune response was evident. Pursuant to the study protocol, the patient received a short course of prednisolone and rapidly returned to baseline ALT levels without any loss of FIX expression. No other patients have experienced elevated ALT levels and none of the five patients developed inhibitory antibodies against FIX.

The 6 months of data from the AMT-060 low-dose cohort are consistent with the highest achieved FIX expression levels observed in a previous study conducted by St. Jude Children's Research Hospital (St. Jude), which utilized the same wild-type FIX gene cassette incorporated in AMT-060. The results of the St. Jude study, which were published in the *New England Journal of Medicine* in 2011 and 2014, demonstrated therapeutically relevant and durable clinical benefits for up to four years, with mean FIX activity of 5.1% (range 2.9% to 7.2%). The FIX gene cassette used in the St. Jude study is exclusively licensed by uniQure.

"We continue to be very encouraged by the data from the low-dose cohort in our Phase I/II study of AMT-060," commented Daniel Soland, Chief Executive Officer of uniQure. "We have so far in the trial achieved our goal for the low-dose cohort, which is to demonstrate safety and therapeutically relevant, durable increases in FIX activity similar to that which was observed in the landmark St. Jude study. The objective for our second cohort is to show meaningful dose-related increases in FIX activity. Based on the results of our non-human primate dose-ranging studies, we are hopeful to accomplish this goal."

On May 31, 2016, uniQure announced that all five patients in the second dose cohort have been treated with a one-time administration of AMT-060 at a dose of $2x10^{13}$ gc/kg, which is four times higher than the dose used in the first cohort. AMT-060 continues to demonstrate a very low screening failure rate, with all patients screened in the study testing negative for pre-existing neutralizing antibodies against AAV5. Patients in the second dose cohort are currently in the early stages of follow-up and initial data are expected to be presented before the end of 2016.

"AMT-060 continues to have multiple distinct advantages," continued Mr. Soland. "It is the only hemophilia gene therapy that combines a gene cassette with clinically proven multi-year durability and an AAV5 vector serotype that has demonstrated safety and broad applicability in more than 25 patients across three clinical studies. We look forward to advancing our clinical program for AMT-060 and sharing additional data from all 10 patients in our Phase I/II study before the end of the year."

Phase I/II Trial Overview

The Phase I/II, open-label, multi-center study includes 10 patients each receiving a one-time, 30-minute, intravenous administration of AMT-060, without the use of immunosuppression therapy. The study includes two dose cohorts of five patients each, with the first cohort receiving $5x10^{12}$ gc/kg and the second cohort receiving $2x10^{13}$gc/kg. All patients in the trial had documented severe or moderately-severe hemophilia, including documented FIX levels less than 1-2%, and required chronic infusions of prophylactic or on-demand Factor IX therapy at the time of enrollment. uniQure will follow all 10 patients in the study for a period of five years to further assess long-term safety and durability.

A summary of the top-line data for the low-dose cohort is as follows:

				Prior to Treatment			**At 6 Months Post AMT-060**		
Patient	Age	Prior hep C infection?	Hemophiliac arthropathy?	FIX activity	Hemophilia phenotype*	Prophylactic FIX Infusions?	FIX activity	Hemophilia phenotype*	Prophylactic FIX Infusions?
1	34	No	No	< 1%	Severe	Yes	6.3%	Mild	No
2	54	Yes	Yes	< 1%	Severe	Yes	5.4%	Mild	No
3	72	Yes	Yes	< 1%	Severe	Yes	<2.0%	Moderate	Yes
4	69	Yes	Yes	1.5%	Moderate-severe	Yes	6.2%	Mild	No
5	71	Yes	Yes	< 1%	Severe	Yes	2.9%	Moderate	No

*based on FIX activity using guidelines as published in Thromb Haemost 2001; 85: 560

Conference Call and Webcast

uniQure will discuss the data in a webcast conference call at 8:00 am EST on Monday, June 13, 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 5314178.

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Investors may also listen to the webcast of the conference call live on the "Events" section of uniQure's website, www.uniQure.com. 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 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.

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 development of our gene therapies 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 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 and its 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.

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