



uniQure Presents Updated Clinical Data in Patients with Severe Hemophilia B Demonstrating up to 9 Months of Sustained Levels of

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--Data from Low-Dose Cohort of AMT-060 Clinical Trial Continues to Show Durability and Therapeutically Relevant Factor IX (FIX) Activity in Severe, Older Patients with Advanced Joint Disease and Frequent Bleeding Episodes--

--Four Patients Remain Free of Prophylactic FIX Infusions, Achieving 82% Reduction in total FIX Usage--

LEXINGTON, Mass. and AMSTERDAM, Netherlands, July 27, 2016 (GLOBE NEWSWIRE) -- uniQure N.V. (Nasdaq:QURE), a leader in human gene therapy, today announced updated clinical data from its ongoing Phase I/II trial of AMT-060, its proprietary, investigational gene therapy, in patients with severe hemophilia B. The updated data show that all patients in the low-dose cohort have sustained improvements in their disease phenotype and continue to maintain durable levels of Factor IX (FIX) gene activity for up to 39 weeks post treatment. Four out of the five patients are over 50 years of age and had advanced joint disease at study entry. These updated data are being presented today in an oral session of the World Federation of Hemophilia (WFH) 2016 World Congress taking place in Orlando, Florida.

"I am very encouraged by the stability of increased FIX activity of AMT-060 and the significant reduction in required infusions of factor replacement," stated Wolfgang Miesbach, M.D., professor of hematology at the University of Frankfurt, Germany. "This effect is particularly important because it is seen in severe patients with established joint disease who experienced a high frequency of joint bleeds despite intense use of prophylactic FIX prior to study entry."

"The majority of patients in this low-dose cohort of AMT-060 are showing FIX activity in the range of 5% of normal, and clinical experience has shown that patients in this range generally do not require prophylactic factor replacement and have a very low frequency of spontaneous joint bleeding episodes," Professor Miesbach added. "Furthermore, at up to 9 months of follow up, AMT-060 continues to be well-tolerated and show durable, clinically relevant levels of FIX activity, which is the key goal of an effective gene therapy approach."

Phase 1/2 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 corticosteroids. The study includes two dose cohorts of five patients each, with the first cohort receiving 5x10¹² gc/kg and the second cohort receiving 2x10¹³ gc/kg. All patients in the trial had documented severe or moderately-severe hemophilia, including documented FIX levels less than 1-2% of normal, and required chronic infusions of prophylactic or on-demand FIX therapy at the time of enrollment. There were no screen failures due to pre-existing anti-AAV5 neutralizing antibodies (NABs) in the study.

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. 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 due to the low prevalence of NABs as evaluated in 25 patients across clinical studies in three different diseases.

Key Data Update from Phase I/II Low-Dose Cohort of AMT-060 in Older Hemophilia B Patients with Severe Joint Disease

Data as of July 22, 2016:

- Through up to 9 months of follow-up, the mean steady-state FIX activity for the four patients that discontinued prophylactic (precautionary) FIX therapy was 5.4% of normal, with a range from 3.1% to 6.7% of normal. This level of FIX activity is consistent with the highest achieved FIX activity 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 were published in the *New England Journal of Medicine* in 2011 and 2014 and demonstrated therapeutically relevant and durable clinical benefits for up to four years as of the time of its last publication. The FIX gene cassette used in the St. Jude study is exclusively licensed by uniQure.
- Total usage of FIX concentrate declined substantially among the four patients that discontinued prophylactic FIX therapy. These patients demonstrated a mean reduction in annualized total FIX usage of 82% after treatment with AMT-060. For all five patients in the low-dose cohort, the mean annualized total FIX usage declined 75% after treatment with AMT-060. The one patient who remained on prophylactic therapy has sustained an improved disease phenotype and also required materially less FIX concentrate after treatment with AMT-060.
- AMT-060 continues to be well-tolerated. As noted previously, one 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 to AAV5 was evident. Per the study protocol, the patient received a short course of prednisolone and rapidly returned to baseline ALT levels with no evidence of loss of FIX expression. No other patients have experienced elevated ALT levels and none of the five patients developed inhibitory antibodies against FIX.
- AMT-060 continues to demonstrate a very low screening failure rate, with all patients screened in the study testing negative for pre-existing anti-AAV5 NABs. To date, 25 patients have been screened for pre-existing anti-AAV5 NABs with

a fully validated assay across several clinical studies with only one patient excluded due to a borderline positive result. This collective data set suggests that a large proportion of the hemophilia patient population may be eligible for treatment with AMT-060.

"Data thus far from this low-dose cohort of AMT-060 have shown that FIX activity in the range of approximately 3% to 7% of normal results in a substantial reduction in the need for FIX replacement therapy for hemophilia patients with severe phenotype, including those with advanced joint disease," stated Deya Corzo, M.D., senior vice president and therapeutic head of Liver/ Metabolism at uniQure. "With our scalable and proven manufacturing platform, we look forward to advancing our hemophilia B program and presenting additional data from all 10 patients in our Phase I/II study before the end of the year."

uniQure previously announced that all five patients in the higher-dose cohort have been treated with a one-time administration of AMT-060 at a dose of 2x1013 gc/kg, which is four times higher than the dose used in the low-dose cohort. Patients in the higher-dose cohort are now in the early stages of follow-up. AMT-060 is being co-developed with Chiesi for Europe.

A summary of the updated, top-line data for the low-dose cohort of AMT-060 presented at WFH:

Patient	Age	Advanced Joint Disease?	Prior to Treatment	Up to 9 Months After AMT-060 Administration					
FIX Activity	Hemophilia Phenotype**	Prophylactic FIX Infusions?	Steady- state FIX Activity***	Latest Measured FIX Activity	Week of Last Measurement	Prophylactic FIX Infusions?			
1	34	No	< 1%	Severe	Yes	6.3%	7.2%	Week 39	No
2	54	Yes	< 1%	Severe	Yes	4.6%	4.7%	Week 39	No
3*	72	Yes	< 1%	Severe	Yes	<2.0%	< 2.0%	Week 26	Yes
4	69	Yes	1.5%	Moderate-severe	Yes	6.7%	6.6%	Week 39	No
5*	71	Yes	< 1%	Severe	Yes	3.1%	2.9%	Week 26	No

*Patients 3 and 5 are siblings.

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

***Steady-state FIX activity was defined as the mean of all levels measured after the discontinuation of prophylactic FIX infusions through the most recent follow-up assessment, with the latter measurement performed at least 10 days after the most recent administration of Factor IX concentrate.

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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