



uniQure Announces Oral Presentation of Updated Results for AMT-060 at the World Federation of Hemophilia (WFH) 2016 World Congre

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AMSTERDAM, The Netherlands, July 11, 2016 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ:QURE), a leader in human gene therapy, today announced that updated data from its ongoing Phase I/II clinical study of AMT-060 in hemophilia B patients will be presented at the World Federation of Hemophilia 2016 World Congress being held July 24-28, 2016 in Orlando, Florida.

The data will be presented by Dr. Wolfgang Miesbach, Professor at the Universitäts Klinikum in Frankfurt, Germany, the principal investigator of the trial, on Wednesday July 27, 2016 during the Late Breaking Session on Gene Therapy. The updated results will include 39 weeks of follow-up and immunogenic data from the five-patient low-dose cohort. AMT-060 is an AAV5-FIX gene therapy manufactured by uniQure using its proprietary insect-cell based technology platform.

The presentation will take place at 2:45 pm EDT in Hall A4 of the Orange County Convention Center.

AMT-060 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 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%, 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.

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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PDF version of release <http://hugin.info/157414/R/2027651/753720.pdf>

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