uniQure

uniQure Presents Preclinical Data on AAV Re-Administration, Regulated Gene Expression and Total Brain Transduction at ASGCT

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AMSTERDAM, the Netherlands, May 9, 2016 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leader in human gene therapy, today announced the data from several preclinical research programs focused on the development and validation of new technologies to improve gene therapy as a therapeutic approach. The results were presented in poster sessions at the American Society of Gene and Cell Therapy (ASGCT) 19th Annual Meeting in Washington D.C. and highlight the progress uniQure has made in establishing optimized gene therapy delivery systems, re-administration protocols and vector technologies specifically in its key focus areas, liver/metabolism and CNS.

"The data presented at the ASGCT demonstrate the significant progress we have made in both our research programs and for our technology platform," said Dr. Harald Petry, Chief Scientific Officer of uniQure. "uniQure has made several important advances in the design and optimization of gene therapy technology that we believe will significantly expand the application and overall effectiveness of our modular platform. We've applied these novel technologies – including optimized gene therapy delivery systems, re-administration protocols, regulated gene expression and improved vector design – across a number of preclinical programs targeting liver and CNS diseases, and remain committed to making gene therapy available to more patients across the globe."

Each of the scientific posters from the ASGCT conference are available on the uniQure website.

Summary of ASGCT presentations:

Liver/Metabolic Disease

- ***Changing the Route of Administration to Improve Liver Transduction by Recombinant AAV-Based Vectors:*** This study, conducted in non-human primates (NHPs) with uniQure's partners at the Centro de Investigación Médica Aplicada (CIMA) in Pamplona, Spain, highlights the potential of alternative administration routes to improve delivery into the liver to achieve higher transduction efficiency and expression levels. High expression levels are crucial for the treatment of diseases associated with a deficiency of enzymatic activity in the liver such as Hemophilia A and B. The results demonstrated that direct administration via the hepatic artery with balloon occlusion of the suprahepatic vein can achieve higher transgene expression and transduction efficiency compared to standard administration techniques.
- ***Successful Repeated Hepatic Gene Delivery by Sequential Administration of AAV5 and AAV1 Vector Serotypes:*** This study, using different AAV serotypes (AAV5 and AAV1) for repeated liver-targeted gene delivery in NHP's, demonstrated that combining different AAV serotypes in a potential therapeutic regimen is a successful strategy for re-administration.
- ***Development of MFP-Inducible System for AAV5 Gene Therapy of Chronic Diseases in the Liver:*** The study conducted in cell culture and mice provides a proof of concept for repeated regulation of transgene expression and a novel vector system for future gene therapy development in disease states where modification of the therapeutic level of gene expression would be advantageous.

CNS Disease

- ***Directional Transduction of AAV5 Vectors in the NHP Brain:*** The goal of this study was to evaluate the transduction efficiency and vector distribution pattern following MRI-guided delivery to improve delivery of a gene therapy into specific regions of the brain. The results indicate a dose dependent distribution profile which could be modified to mediate expression patterns as a potential therapeutic strategy to treat a broad range of neurodegenerative diseases.

Gene Therapy Technology

- ***Successful In Vivo Re-Administration of AAV with the Use of Two-Step AAV injection:*** uniQure researchers evaluated the feasibility of a re-administration protocol utilizing a two-step AAV injection approach to decrease levels of circulating neutralizing antibodies (NAbs) in mice. Results demonstrated a significant dose-dependent reduction in anti-AAV5 antibodies following AVV 'decoy' injection.
- ***Analysis of AAV5 Biodistribution and Viral Shedding in the Presence or Absence of Neutralizing Antibodies*** **:** This study investigated the effect of the presence of neutralizing antibodies on gene therapy efficiency administered to mice. Results indicated that for patients with already existing NAbs, or for the purpose of re-administration, alternative administration routes are needed to strengthen gene therapy application. The study showed significant decreases in the biodistribution of AAV5 in the presence of NAbs. Furthermore, the presence of NAbs increased following infection without a cellular immune response against the vector.

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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