



FOR IMMEDIATE RELEASE

uniQure Acquires Cardiology Gene Therapy Company InoCard

--Combination of uniQure Leadership and Fundamental InoCard Discovery May Enable Transformative Gene Therapy Treatment of Congestive Heart Failure--

Amsterdam, the Netherlands, and Heidelberg, Germany, August 11, 2014 — uniQure N.V. (NASDAQ: QURE), a leader in human gene therapy, today announced the acquisition of InoCard GmbH, an innovative, early-stage biotechnology company focused on the development of gene therapy approaches for cardiac disease. InoCard has developed a novel gene therapy to preclinical proof of concept, for the one-time treatment of congestive heart failure (CHF), a rapidly progressing disease affecting 26 million people worldwide. InoCard founders Prof. Patrick Most and Prof. Hugo Katus will join uniQure as Managing Director of uniQure in Germany and Chairman of the Scientific Advisory Board, for Cardiovascular Diseases, respectively.

InoCard's lead program is a gene therapy to express the calcium-binding protein S100A1, which has been shown by Profs. Most and Katus to be a master regulator of myocardial function. The therapy was developed based on their fundamental discovery that S100A1 is downregulated in CHF and administration of S100A1 has demonstrated *in vivo* beneficial effects on contractile force, growth control of heart muscle cells and rhythm stability of the heart and is also able to adapt the heart's energy supply to increased cardiac output. In a porcine heart failure model, treatment with InoCard's gene therapy AAV-S100A1 demonstrated a 12-month survival rate of 90%.

"The acquisition of InoCard is a further demonstration of uniQure's strategy to access the best early-stage programs in our industry and accelerate their development by the application of our proven modular platform," commented Jörn Aldag, uniQure's Chief Executive Officer. "There is strong scientific rationale that addressing calcium dysregulation leads to an astounding effect in congestive heart failure. We are very excited to have Patrick Most and Hugo Katus join us. Their groundbreaking research and expertise in heart failure is a great contribution to uniQure, and we believe that together we will deliver the best-in-class treatment for congestive heart failure."

"Despite the continuously growing prevalence of CHF, there have been no therapeutic innovations in decades. InoCard has successfully laid the basis for the development of a long-term, causative treatment of this devastating disease," said Prof. Patrick Most, founder of InoCard. "We believe that combining our promising S100A1 therapy with uniQure's capabilities in innovating safe and effective gene therapies has the potential to transform the treatment of cardiovascular diseases."

Under the terms of the agreement, InoCard shareholders will receive an upfront payment of €3 million (€1.5 in cash and €1.5m in uniQure stock), and certain success-based milestones and royalties.

Conference Call and Webcast

uniQure and InoCard Management will discuss the acquisition in a conference call at 08.30 EDT on Tuesday August 12, 2014. The conference call can be accessed using the information below. To ensure a timely connection to the webcast, it is recommended that users register at least 15 minutes prior to the scheduled start time. Investors may listen to the webcast of the conference call live on the "Events" section of uniQure's website, www.uniQure.com. The webcast replay will be available for at least 72 hours following the call.

Conference Call Information:

Confirmation Code: 5826325

Local - London, United Kingdom: +44(0)20 3427 1917

Local - New York, United States of America: +1212 444 0896

Local - Berlin, Germany: +49(0)30 3001 90539

Local - Amsterdam, Netherlands: +31(0)20 713 2789

Local - Geneva, Switzerland: +41(0)22 567 5432

About AAV-S100A1 Gene Therapy

AAV-S100A1 is a gene therapy product designed to selectively restore cardiac S100A1 deficiency. It is a one-time therapeutic intervention in advanced CHF patients, who often continue to decline despite standard of care treatment. AAV-S100A1 has proven long-term therapeutic efficacy, safety and reduced mortality in a human-relevant *in vivo* heart failure model compatible with clinical drug regimens. The first human trial is expected to start in 2016.

About Congestive Heart Failure

Congestive heart failure (CHF) is the disability of the cardiac muscle to provide sufficient circulatory support both at rest and during exercise. CHF is a rapidly progressing disease affecting 26 million people worldwide, with patients suffering from severe heart failure facing a 5-year mortality rate of over 50%. According to the American Heart Association, the prevalence of CHF is expected to double or triple by 2030. Currently, there is no effective long-term or causative treatment for this disease.

About Gene Therapy

Gene therapy offers the prospect of long-term and potentially curative benefit to patients with genetic or acquired diseases by directing the expression of a therapeutic protein or restoring the expression of a missing protein through a single administration. Genes are the specific areas of DNA that provide the blueprint used by the body's cellular machinery to make proteins. A defect or mutation in a specific gene can result in the inability or reduced ability to express a protein, or the reduced functionality of a protein. Gene therapy seeks to treat the causes of diseases by enabling patients to effectively express a missing or deficient protein through the delivery of the needed gene into cells.

About uniQure

uniQure is delivering on the promise of gene therapy through single treatments with potentially curative results. We have developed a modular platform to rapidly bring new disease-modifying therapies to patients with severe disorders. We are engaged in multiple partnerships and have obtained regulatory approval of our lead product, Glybera, in the European Union for a subset of patients with LPLD. www.uniQure.com

About InoCard

InoCard GmbH is a privately held early-stage biotechnology company based in Heidelberg, Germany. The company has developed an innovative AAV-based gene therapy approach for the long-term treatment of congestive heart failure, targeting the calcium-binding protein S100A1. InoCard was founded in December 2013 as a spin-off of the University of Heidelberg by Prof. Patrick Most und Prof. Hugo Katus, key opinion leaders in molecular and clinical cardiology, respectively. InoCard was advised on this transaction by Dr. Claudia Ulbrich. InoCard's business concept aided by Dr. Marc Lerchenmüller was awarded with the Health Axis Europe Accelerator prize 2013.

FORWARD-LOOKING STATEMENTS

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 risk of cessation or delay of any of the ongoing or planned clinical studies and/or development of our product candidates, the risk of delay or failure to successfully commercialize or obtain further regulatory approval of our products, and the risk that our collaborations or our other collaboration partners will not continue or will not be successful. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our clinical development activities, regulatory oversight, product commercialization, intellectual property claims, and the risks, uncertainties and other factors described under the heading "Risk Factors" in uniQure's Form 20-F filed with the Securities and Exchange Commission dated April 25, 2014. 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.

uniQure:

Aicha Diba

Investor Relations

Direct: +31 20 566 8014

Main: +31 20 566 7394

IR@uniQure.com

Media inquiries:

Gretchen Schweitzer

MacDougall Biomedical Communications

Direct: +49 172 861 8540

Main: +1 781 235 3060

gschweitzer@macbiocom.com