uniQure Outlines Key Initiatives for 2017 and Vision for 2018

~ Preparations Underway for Late-Stage Development in Hemophilia B ~

~ Three Clinical-Stage Programs by 2018 Including One Pivotal and Two Proof-of-Concept Studies ~

~ Company Strengthened Following Strategic Review and New Executive Leadership ~

Lexington, MA and Amsterdam, the Netherlands, January 9, 2017 — uniQure N.V. (NASDAQ: QURE), a leader in human gene therapy, today announced its key corporate initiatives for 2017 and vision for 2018 following the completion of a company-wide strategic review late last year that refocuses its pipeline, streamlines operations, improves its financial position and enhances overall execution and shareholder value. uniQure management is meeting with investors and analysts this week in conjunction with the 35th Annual J.P. Morgan Healthcare Conference taking place in San Francisco. An updated corporate presentation is posted to the investor page of the Company's website, www.uniqure.com

"As we begin 2017, we expect each of our core programs to achieve important milestones that will bring us closer to delivering life-transforming gene therapies for patients," stated Matthew Kapusta, chief executive officer of uniQure. "Our key focus is to rapidly advance our hemophilia B program into late-stage development. By 2018, we expect to have three clinical-stage programs, including a pivotal study for AMT-060 in hemophilia B, and two clinical proof-of-concept studies for congestive heart failure and Huntington's disease. We believe our financial position is strong and capable of funding the accomplishment of these objectives."

Recent Accomplishments

- *Hemophilia B* – Presented positive new and updated clinical data on AMT-060 at the 58th American Society of Hemophilia (ASH) Annual Meeting demonstrating clinically significant and sustained increases in FIX activity, substantial reductions in FIX replacement usage and a near cessation of spontaneous bleeding in patients with severe disease at up to 12 months follow-up.

- *Huntington's disease* – Published in a peer-reviewed journal preclinical data showing sustained and strong wild-type HTT protein silencing in humanized control mice, including knock-down efficiency up to 80% using optimized miHTT scaffolds.

- *Collaboration with Bristol-Myers Squibb (BMS)* – Made significant progress in transferring S100A1 to uniQure's proprietary insect-cell, baculovirus platform, as well as conducting preclinical dose-ranging analyses and comparability studies.

- *Strategic review* – Announced the completion of a company-wide strategic review to refocus its pipeline, streamline operations, reduce operating costs and enhance execution and long-term shareholder value.

- *Leadership* – Appointed Matthew Kapusta as Chief Executive Officer in December 2016.

Key Corporate Initiatives for 2017

- *Advance hemophilia B program towards late-stage clinical development* – The Company intends to initiate a pivotal trial for AMT-060 in hemophilia B pending discussions with regulatory authorities that are expected to take place in early 2017.

- *Advance AMT-130 in Huntington's disease program towards IND filing* – The Company plans to file an investigational new drug (IND) application for Huntington's disease following the completion of ongoing IND-enabling studies, including the initiation of additional safety and toxicology studies.

- *Advance AMT-120 in heart failure towards IND filing* – uniQure's collaboration with BMS in cardiovascular diseases is a top research priority, specifically focused on the advancement of S100A1 in congestive heart failure. The Company expects to initiate preclinical functional and safety studies for S100A1 in 2017, as well as conduct additional research activities for two additional product candidates designated by BMS.

- *Presentation of preclinical and clinical data* – Data presentations on programs in hemophilia B and Huntington's disease are expected in 2017, including longer-term safety and efficacy data on AMT-060 from the ongoing Phase I/II clinical trial and preclinical data on AMT-130 in Huntington's disease.

- *Initiate manufacturing campaigns* – uniQure expects to initiate cGMP manufacturing campaigns for all anticipated clinical programs in its Lexington, MA facility in 2017. The Lexington facility is fully operational and has the capability of scaling up to 2,000L capacity, which uniquely positions the Company for late-stage development and commercialization of its gene therapy products, importantly AMT-060.

uniQure had approximately €140 million ($158 million) in cash and cash equivalents as of September 30, 2016, which is expected to fund operations into 2019.

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 hemophilia, Huntington's disease and cardiovascular diseases. www.uniQure.com

uniQure 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 implementation and effects of the Company's new strategic and organizational changes, the development of our gene therapy product candidates, 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 corporate reorganizations and strategic shifts, 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

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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