



uniQure Completes Strategic Review to Refocus its Pipeline, Reduce Operating Costs and Deliver Long-Term Shareholder Value

November 15, 2016

~ Company Structure Simplified; Manufacturing to be Consolidated into Lexington, MA

~ Preparation Underway for Late-Stage Development in Hemophilia B

~ Cost Savings Expected to Extend Cash Resources into 2019

~ Company to Host Investor Conference Call Today at 8:30 a.m. ET

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Nov. 15, 2016 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ:QURE), a leader in human gene therapy, today announced the completion of a company-wide strategic review aimed at refocusing its pipeline, consolidating its manufacturing and enhancing overall execution to drive shareholder value. As a result of this initiative, the Company will prioritize programs in hemophilia B, Huntington's disease and those associated with its landmark collaboration with Bristol-Myers Squib (BMS) in cardiovascular disease. Additionally, the Company will restructure its research and development organization in the Netherlands and consolidate manufacturing in the United States. These actions are expected to reduce operating expenses and create a more efficient company focused on the successful and timely development of gene therapies for patients with serious unmet medical needs.

As part of its effort to focus resources on key priorities, uniQure has initiated discussions with its collaborator regarding the potential discontinuation of licensing discussions for AMT-110 for the treatment of Sanfilippo B, and will pursue partnering opportunities for its academic-sponsored program in Parkinson's disease.

uniQure expects to realize €5 to €6 million of annualized cost savings in personnel and other related operating expenses as a result of the elimination of approximately 50 to 60 positions, or 20% to 25% of global headcount, by the end of 2017. Additionally, the Company expects to further reduce planned operating expenses by €11 to €15 million over the next two years through the focusing of its pipeline. Based on its strong cash position and the above actions, uniQure believes its existing cash resources will be sufficient to fund operations into 2019.

"The strategic restructuring brings enhanced focus to our pipeline, streamlines operations and improves our financial strength," stated Matthew Kapusta, interim chief executive officer of uniQure. "We are committed to the timely development of our programs in hemophilia B and Huntington's disease, as well as the support of our collaboration with BMS. Along with our investigators and collaboration partner, we are enthusiastic about the interim data from our ongoing Phase I/II study of AMT-060 in hemophilia B and will allocate necessary resources to expedite bringing AMT-060 to market, with commercial manufacturing capabilities in our state-of-the-art U.S. facility which is already in place."

"We are confident that these efforts will significantly streamline operations and position us well for the future," Mr. Kapusta added. "We are grateful for the contributions that the employees affected by this outcome have made to uniQure, and we will be supporting them throughout this transition."

Key Outcomes of the Strategic Review

Pipeline Prioritization

uniQure has decided to prioritize the following programs within its gene therapy pipeline:

- Pursue as top priorities the clinical development of its product candidates in hemophilia B and Huntington's disease. The Company intends to initiate a pivotal trial in hemophilia B pending discussions with regulatory authorities and to file an investigational new drug (IND) application for Huntington's disease following the completion of ongoing IND-enabling studies.
- Provide greater focus on new product generation, with an emphasis on liver-directed diseases. This includes the internal development or in-license of new product candidates in rare and orphan diseases that can leverage the Company's next-generation vector and promoter platform and manufacturing capabilities.
- Advance uniQure's collaboration with BMS in cardiovascular diseases as a top research priority, specifically focused on the advancement of S100A1 in congestive heart failure.
- Evaluate options for its gene therapy programs targeting Sanfilippo B and Parkinson's disease, including partnering opportunities. Two of the four patients in the ongoing Phase I/II clinical trial in Sanfilippo B have completed their 30-month evaluations, and the 30-month data on all four patients in the study are expected to be available for presentation in the first quarter of 2017. Regarding the academic-sponsored trial in Parkinson's disease, uniQure will evaluate partnership opportunities to accelerate completion of the ongoing study and further develop the program.

Consolidate Global Manufacturing

- uniQure will consolidate all GMP manufacturing at its Lexington, MA facility. 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.

- The Company will maintain a smaller, but fully integrated research and development organization in Amsterdam, the Netherlands. The team is expected to move into a new facility in the first half of 2017.

Streamline Research and Development

- The previous organizational structure based on therapeutic areas of focus is being eliminated. As a result of this change, Deya Corzo, M.D., senior vice president of the liver and metabolic therapeutic area, and Charles Richard, M.D., Ph.D., senior vice president of the central nervous system therapeutic area are transitioning their responsibilities and will be leaving the Company by the end of the year.
- Christian Meyer, M.D., Ph.D., chief medical officer, will assume full responsibility for advancing the company's hemophilia B gene therapy program.
- Harald Petry, Ph.D., chief scientific officer, will continue to lead uniQure's preclinical research, including IND-enabling studies in Huntington's disease.

"We have simplified the structure of the Company to drive focus and provide for a strong foundation upon which to execute our key priorities," stated Philip Astley-Sparke, chairman of the uniQure Board of Directors. "Further details on how we expect to position our hemophilia program will be provided after we have met with regulatory authorities early next year to discuss our plans for a pivotal study. We will further direct capital into programs that we believe will create near-term value, as well as continue to leverage our core assets, including next-generation vector development and best-in-class manufacturing capabilities."

Conference Call Information

uniQure will host a conference call today, November 15, 2016 at 8:30 a.m. ET to discuss this announcement. To access the live call by phone, dial (877) 280-2296 (United States) or +44 (0)20 3427 1917 (international); the conference ID is 4243017. The call may also be accessed through the Investors section of the Company's website at www.uniQure.com. Following the live webcast, a replay of the call will be available at the same location through November 30, 2016.

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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