uniQure Announces Strategic Reorganization to Reduce Operating Expenses and Support Advancement of Multiple Clinical-Stage Programs

October 5, 2023

~ Reduction of 28% of workforce not related to HEMGENIX® manufacturing obligations; Total cost savings of $180 million to extend cash runway into second quarter of 2027 ~

~ Discontinuing investments in more than half of research and technology projects, centralizing operations, and streamlining organization ~

~ Prioritizing continued development of AMT-130 in Huntington's disease and near-term initiation of clinical trials for AMT-260 in refractory mesial temporal lobe epilepsy, AMT-162 in SOD1-ALS, and AMT-191 in Fabry disease; Multiple potential value drivers expected over next two years ~

~ As a result of reprioritization, Ricardo Dolmetsch, Ph.D. Chief Scientific Officer to depart the Company and Rich Porter, Ph.D. Chief Business Officer will assume responsibilities for research as well as nonclinical and vector development ~

LEXINGTON, Mass. and AMSTERDAM, Oct. 05, 2023 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced a strategic reorganization that will significantly reduce operating expenses while supporting focused execution to rapidly advance multiple clinical-stage programs to proof-of-concept.

"At uniQure, our highest priority is to deliver innovative, life-changing therapies to patients with significant unmet needs. To accomplish our mission and generate near-term value for our stakeholders, we will implement a strategic restructuring of our business," stated Matt Kapusta, chief executive officer of uniQure. "We are taking important actions today to cut operating expenses while ensuring that we have the necessary resources to advance our prioritized clinical-stage programs as rapidly as possible to proof-of-concept. Following an extensive review, we plan to discontinue more than half our research and technology projects and focus our R&D efforts on programs that leverage our CNS and liver-targeted gene therapy expertise, have the potential for expedited clinical proof of concept, and have attractive risk-value profiles. We remain fully committed to carefully managing costs, prudently allocating capital, rigorously assessing our clinical development priorities as new data emerges, and thoughtfully evaluating strategies that can enhance value for shareholders."

"These were difficult but necessary decisions," he added. "I'm truly grateful for the commitment and contributions of our many colleagues, and we remain fully committed to delivering on our promise to patients in need. We also look forward to sharing longer-term, interim data on our Huntington's disease program in late fourth quarter of this year and initiating clinical trials for our other programs as rapidly as possible."

Restructuring Operations and Reducing Expenses

Following an extensive review of the pipeline, the Company will discontinue more than half of its research and technology projects, including AMT-210 for the treatment of Parkinson's disease and multiple undisclosed programs. The company will focus its research efforts on a limited number of projects believed to have optimal risk, value and speed attributes, including AMT-161 for c9orf72 amyotrophic lateral sclerosis (ALS), AMT-240 for autosomal dominant Alzheimer's disease, and next-generation AAV capsid development. As a result of the reprioritization, the Company will be closing a research lab in Lexington and plans to sublease this space.

The Company will also consolidate all GMP manufacturing into its Lexington, MA manufacturing facility and consolidate process and analytical development into its Amsterdam, Netherlands facility. Commercial manufacturing of HEMGENIX® for CSL Behring will be unaffected by these actions.

As a result of the restructuring plan, the Company expects:

- Elimination of 114 positions, which represents 28% of the workforce not committed to HEMGENIX manufacturing obligations, and approximately 20% of the total workforce
- Total cost savings of approximately $180 million over the next three years
- Current balance of cash, cash equivalents and investment securities of $628.6 million as of June 30, 2023 (excludes $100 million milestone payment subsequently received from CSL Behring) to fund operations into the second quarter of 2027
- One-time restructuring costs of approximately $2.3 million, primarily incurred in the fourth quarter of 2023

Prioritizing Clinical-stage Programs

The Company plans to focus resources on driving execution across four clinical-stage programs and expediting near-term proof-of-concept data readouts.

- *AMT-130 for the treatment of Huntington's disease* : In the fourth quarter of 2023, the Company plans to provide a clinical update from both the U.S. Phase I/II study of AMT-130, including 18- and 30-month follow-up data from the treated patients in the high- and low-dose U.S. cohorts, respectively, and for the first time, the European Phase I/II study. In the first quarter of 2024, the Company expects to meet with the FDA to review the data and discuss future development of

AMT-130. Also in 2024, the Company will present up to 3-year follow-up data on all patients in the U.S. and European Phase I/II trials, including at least 2-year follow-up data on more than half of patients treated with AMT-130.

- *AMT-260 for the treatment of refractory mesial temporal lobe epilepsy*: In the third quarter of 2023, the Company announced the clearance of an investigational new drug (IND) for the Phase I/IIa clinical study of AMT-260. Screening and patient enrollment is expected to begin in the fourth quarter of 2023 with first patient dosing occurring in the first quarter of 2024.

- *AMT-162 for the treatment of SOD1-ALS*: The Company expects to initiate patient screening in the fourth quarter of 2023 with first patient dosing occurring in the first quarter of 2024.

- *AMT-191 for the treatment of Fabry disease*: The Company continues to expect to submit an IND in the fourth quarter of 2023 and to begin patient dosing in 2024.

Aligning Leadership Team

Changes on the Company's leadership team are aligned with this strategic reorganization and pipeline prioritization. Walid Abi-Saab, M.D., who joined the Company as Chief Medical Officer in June 2023, will continue to lead development of all clinical-stage programs. Due to the significant reduction in research activities, Ricardo Dolmetsch, Ph.D., the Company's current chief scientific officer, is departing the Company and will remain as a scientific consultant through the end of the year. Richard Porter, Ph.D., will assume responsibilities for research, as well as non-clinical and vector development in his new role as Chief Business and Scientific Officer. He will continue to oversee business development and product planning.

Dr. Porter has more than 25 years of neuroscience leadership in the biopharma industry and joined uniQure in June 2021 through the acquisition of Corlieve Therapeutics, where he was founder and chief executive officer.

"I want to express my sincere gratitude to Ricardo for his leadership in advancing our research and development programs over the last several years and for bringing innovative ideas and energy to the Company," added Matt Kapusta. "I look forward to working with Rich in his expanded role as he advances our research and technology efforts and continues to lead our business development activities."

About uniQure

uniQure's mission is to reimagine the future of medicine by delivering innovative cures that transform lives. The recent approvals of our gene therapy for hemophilia B – a historic achievement based on more than a decade of research and clinical development – represents a major milestone in the field of genomic medicine and ushers in a new treatment approach for patients living with hemophilia. We are now leveraging our modular and validated technology and manufacturing platform to advance a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory mesial temporal lobe epilepsy, amyotrophic lateral sclerosis (ALS), Fabry disease, and other severe 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," "establish," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "seek," "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 that the Company's restructuring will have anticipated cost savings of approximately $180 million through 2027 and will extend the Company's cash runway to the second quarter of 2027, statements regarding the timing, expectations and sufficiency of our clinical trials, patient enrollment of our current and planned clinical trials and the timing thereof. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the regulatory approval and commercial launch of HEMGENIX®, material changes to our interim or preliminary data, our clinical trial for Huntington's disease, the impact of financial and geopolitical events on our Company and the wider economy and health care system, our Commercialization and License Agreement with CSL Behring, our clinical development activities, clinical results, collaboration arrangements, regulatory oversight, product commercialization and intellectual property claims and ongoing litigation, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in the Company's periodic securities filings, including its Annual Report on Form 10-K filed February 27, 2023 and the Quarterly Report on Form 10-Q filed August 1, 2023. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and the Company assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

uniQure Contacts:

FOR INVESTORS:

Maria E. Cantor

Direct: 339-970-7536
Mobile: 617-680-9452
m.cantor@uniQure.com

Chiara Russo

Direct: 617-306-9137
Mobile: 617-306-9137
c.russo@uniQure.com

FOR MEDIA:

Tom Malone

Direct: 339-970-7558
Mobile:339-223-8541
t.malone@uniQure.com

uniQure