uniQure Announces Second Quarter 2017 Financial Results and Recent Company Progress

~ Advancing Gene Therapy Programs in Hemophilia B and Huntington's Disease ~
~ Provides Update on Research Collaboration with Bristol-Myers Squibb ~
~ Makes New Appointments to Company Leadership and Nominations to Board of Directors ~

Lexington, MA and Amsterdam, the Netherlands, August 8, 2017 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today reported its financial results for the second quarter of 2017 and highlighted recent progress across its business.

"We had a highly productive second quarter and past few weeks in which we announced substantial progress in advancing our lead program in hemophilia B through commercial-scale manufacturing and CMC accomplishments, regulatory preparation and the receipt of broader patent protection around our insect-cell platform technology," stated Matthew Kapusta, chief executive officer of uniQure.

"We presented long-term clinical data in patients with severe hemophilia B demonstrating safety and durable clinical benefits, as well as the potential to treat nearly all patients suffering from this life-changing disorder. We now have full, unencumbered global rights to our hemophilia B program creating value for shareholders and opening the door to new potential opportunities, and we are advancing our Huntington's disease program into an IND-enabling toxicology study in the fall," he added. "Furthermore, based on recent encouraging data, uniQure and Bristol-Myers Squibb intend to advance our S100A1 product candidate into additional preclinical studies, including a therapeutic heart failure study that we expect to initiate as soon as possible. We are embarking on a very busy and equally productive second half of the year, and we look forward to providing additional company updates."

Second Quarter 2017 and Recent Company Progress:

- Successful development and scale up of manufacturing processes for hemophilia B and other gene therapy programs
  - Developed and optimized a reproducible, commercial scale, manufacturing process for producing its hemophilia B gene therapy in accordance with Good Manufacturing Practices (GMP). This process will be leveraged across each of the Company's programs, including AMT-130 in Huntington's disease for which uniQure expects to file an investigational new drug (IND) application in 2018. A comparability protocol also has been finalized, and the Company is planning to meet with U.S. and European regulators in the early fall to further discuss its plans to begin a pivotal program in hemophilia B in 2018.

- Continued progress on research collaboration with Bristol-Myers Squibb (BMS) in congestive heart failure
  - Today, uniQure announced that preliminary data from a study in large animals demonstrated both DNA delivery and human S100A1 expression in the myocardium after treatments with product produced from uniQure's proprietary insect cell, baculovirus manufacturing process. Based on this finding and others, BMS and uniQure intend to advance the product candidate into further preclinical studies, with a goal of initiating a preclinical therapeutic heart failure study as soon as possible.

- Presentation of updated, long-term clinical data from ongoing Phase I/II trial of AMT-060 in patients with severe hemophilia B
  - Clinical data presented at the International Society on Thrombosis and Hemostasis (ISTH) annual congress on up to eighteen months of follow-up from the study's low-dose cohort and up to twelve months of follow-up from the study's second, higher-dose cohort.
  - All 10 patients in the study demonstrated improvements in their disease state as measured by reduced FIX replacement therapy and bleeding frequency. Across both dose cohorts, cumulative annualized FIX consumption decreased by 79%, and in the second-dose cohort, no spontaneous bleeds were reported in the last six months of follow-up - with a reduction in the annualized spontaneous bleed rate of 84% compared to the one-year period prior to gene transfer.

- Presentation of new clinical data at ISTH demonstrating the potential to expand the applicability of AAV5-based gene therapy to nearly all patients suffering from hemophilia B
  - New clinical data demonstrated efficacy of AAV5 gene therapy in the presence of pre-existing neutralizing antibodies (NABs). All three patients with detected anti-AAV5 NABs according to the highly sensitive luciferase-based (LUC) NAB assay, presented increases in FIX expression. To date, 18 patients across two clinical studies have received intravenous, systemic administration of AAV5-based gene therapies without any observed T-cell activation.
  - These data suggest AAV5 may have a superior immunogenicity and safety profile compared to other AAV vectors.

- Expansion of intellectual property portfolio with newly issued patent providing broad protection of insect cell-based AAV manufacturing
  - Patent broadly covers an important component of insect cell-based AAV manufacturing technology. The newly issued Hermens '627 patent significantly expands uniQure's leading intellectual property portfolio related to large-scale, highly reproducible manufacturing of AAV in insect cells. The technology covered in the Hermens '627 patent family, which is currently widely applied in insect cell-based AAV manufacturing, significantly increases the value of uniQure's patent portfolio and strengthens the company's leadership in the production of AAV-based gene therapies.

- Reacquired development and commercial rights to hemophilia B program from Chiesi
  - uniQure now has full, unencumbered global rights to its hemophilia B gene therapy program with clinical proof-of-concept and is positioned to accelerate clinical development, maximize shareholder value and take advantage of new, strategic opportunities related to the program.

- Presentation of new preclinical data at the American Society of Clinical and Gene Therapy (ASGCT)
  - Data demonstrated successful and effective transduction of AAV5 in non-human primates with pre-existing anti-AAV5 neutralizing antibodies (NABs). At all observed levels, pre-existing neutralizing antibodies for AAV5 did not impact the transduction effectiveness of the AAV5 vector. This suggests a much broader potential population of eligible patients than previously expected for AAV5-based gene therapies, including uniQure's investigational gene therapy in patients with severe hemophilia B.
  - Data demonstrated successful readministration of gene therapy in non-human primates and successful transduction of AAV5 in the presence of pre-existing NABs. Effective repeated hepatic gene delivery with uniQure's AAV5 vector was demonstrated after a proprietary immunoadsorption procedure in non-human primates (NHPs).

- Key management appointments and Board of Director nominations further expanding the Company's capabilities across manufacturing, research and clinical development

- Sander van Deventer, M.D., Ph.D., appointed as Chief Scientific Officer; Scott McMillan, Ph.D., appointed as Chief Operating Officer; and Steven L. Zelenkofske, D.O., appointed as Chief Medical Officer.
- Christian Klemt promoted to Chief Accounting Officer.
- Madhavan Balachandran and Jeremy P. Springhorn, Ph.D., nominated to the Board of Directors for approval at the Extraordinary General Meeting of Shareholders (EGM) on September 14, 2017. Mr. Balachandran and Dr. Springhorn are seasoned industry executives who served at Amgen Inc., and Alexion Pharmaceuticals Inc., respectively.
- As previously disclosed, Will Lewis and Sander van Deventer will resign from the Board of Directors effective at the Company's EGM.

Upcoming Anticipated Milestones

- Regulatory meetings with U.S. Food and Drug Administration and European Medicines Agency regarding late- stage clinical development in hemophilia B
- Completion of comparability testing in hemophilia B and technology transfer from Amsterdam to Lexington facility
- Initiation of IND-enabling safety toxicology study of AMT-130 in Huntington's disease
- Initiation of preclinical therapeutic heart study for S100A1 gene therapy targeting congestive heart failure
- Presentation of non-human primate data in Huntington's disease
- Ongoing longer-term follow up and durability data on AMT-060

Financial Highlights

Cash Position: As of June 30, 2017, the Company held cash and cash equivalents of $104.1 million, compared with $132.5 million as of December 31, 2016. The decrease in cash was primarily related to the advancement of its clinical and preclinical gene therapy targets, general corporate activities and capital expenditures related to its facilities. The Company intends to significantly reduce capital expenditures in 2017 and 2018 and realize operational cost savings from the strategic restructuring initiated in November 2016 and the withdrawal of Glybera in October 2017. As a result of these initiatives, the Company expects its cash on hand will be sufficient to fund operations into 2019.

Revenues: Revenues for the three months ended June 30, 2017 were $4.9 million compared to $4.5 million for the same period in 2016. Collaboration revenues for the second quarter of 2017 were $4.2 million, compared to $3.2 million for the comparable period in 2016.

R&D Expenses: Research and development expenses for the three months ended June 30, 2017 were $16.9 million compared to $19.2 million for the same period in 2016. The decrease was primarily related to lower costs in relation to the manufacturing of drug substance to supply our programs.

SG&A Expenses: Selling, general and administrative expenses for the three months ended June 30, 2017 were $5.4 million compared to $7.8 million for the same period in 2016. The second quarter 2016 includes one-off costs of $1.9 million related to the Extera arbitration and $0.9 million of costs associated with the Glybera global registry and Phase IV study, partially offset by higher share-based compensation expense during the second quarter of 2017.

Other Expense: Other expense for the three months ended June 30, 2017 were $2.6 million, compared to zero for the same period in 2016. The second quarter 2017 includes $1.7 million of one-off costs related to the withdrawal of Glybera in 2017 and $0.9 million of costs associated with the exit from our previous Amsterdam facilities.

Net Loss: The net loss for the second quarter of 2017 was $21.3 million, or $0.83 per share, compared to $21.1 million, or $0.84 per share, for the second quarter of 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 liver/metabolic, central nervous system 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, 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, and the scope of protection provided by our patent portfolio. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our and our collaborators' clinical development activities, collaboration arrangements, corporate reorganizations and strategic shifts, 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 Quarterly Report on Form 10-Q filed on May 9, 2017. 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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UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS

	June 30, 2017	December 31, 2016
	in thousands, except share and per share amounts	
Current assets		
Cash and cash equivalents	$ 104,087	$ 132,496
Accounts receivables and accrued income	5,817	9,180
Prepaid assets and other current assets	1,521	2,270
Total current assets	**111,425**	**143,946**
Non-current assets		
Property, plant and equipment, net	35,410	35,702
Intangible assets and goodwill	9,251	8,789
Other non-current assets	1,879	1,828
Total non-current assets	**46,540**	**46,319**
Total assets	**$ 157,965**	**$ 190,265**
Current liabilities		
Accounts payable	$ 3,458	$ 5,524
Accrued expenses and other current liabilities	9,440	9,766
Current portion of long-term debt	4,319	605
Current portion of deferred rent	710	684
Current portion of deferred revenue	5,203	6,142
Total current liabilities	**23,130**	**22,721**
Non-current liabilities		
Long-term debt, net of current portion	16,153	19,631
Deferred rent, net of current portion	8,494	6,781
Deferred revenue, net of current portion	78,728	75,612
Contingent consideration	2,415	1,838
Other non-current liabilities	1,759	51
Total non-current liabilities	**107,549**	**103,913**
Total liabilities	**130,679**	**126,634**
Total shareholders' equity	**27,286**	**63,631**
Total liabilities and shareholders' equity	**$ 157,965**	**$ 190,265**

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UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended June 30,	
	2017	**2016**
	in thousands, except share and per share amounts	
Total revenues	**$ 4,942**	**$ 4,451**
Operating expenses:		
Research and development expenses	(16,866)	(19,221)
Selling, general and administrative expenses	(5,410)	(7,834)
Total operating expenses	**(22,276)**	**(27,055)**
Other income	266	475
Other expense	(2,640)	-
Loss from operations	**(19,708)**	**(22,129)**
Non operating items, net	(1,561)	716
Loss before income tax expense	**(21,269)**	**(21,413)**
Income tax benefit / (expense)	-	333
Net loss	**$ (21,269)**	**$ (21,080)**
Basic and diluted net loss per common share	$ (0.83)	$ (0.84)
Weighted average shares used in computing basic and diluted net loss per common share	25,560,348	25,077,350