uniQure Announces Second Quarter 2018 Financial Results and Highlights Company Progress

~ Patient Enrollment Underway in Global Phase III HOPE-B Pivotal Study of AMT-061

~ First Patient Successfully Screened into Phase IIb Dose-Confirmation Trial of AMT-061

~ Robert Gut, M.D., Ph.D., Named Chief Medical Officer and Other Appointments to Clinical Development Leadership

~ Ended Second Quarter with $259 Million in Cash and Cash Equivalents; Expected to Fund Operations into 2021

Lexington, MA and Amsterdam, the Netherlands, August 8, 2018 — 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 2018 and highlighted recent progress across its business.

"We have made tremendous progress over the past several months advancing our gene therapy programs in hemophilia B and Huntington's disease, as well as in expanding our intellectual property portfolio and executing on our manufacturing plan," said Matt Kapusta, chief executive officer of uniQure. "Our Phase III pivotal trial of AMT-061 is actively enrolling patients, and we have consented and successfully screened the first of three patients in our Phase IIb dose-confirmation study."

"The initiation of our registrational study provides the opportunity for uniQure to be first to market with a one-time treatment that has the potential to transform the standard of care for patients with hemophilia B," Mr. Kapusta added. "We continue to work toward what we expect will be an equally productive second half of 2018 with the achievement of multiple value-creating catalysts across the company, including top-line data from our dose-confirmation study, the submission of an Investigational New Drug application for AMT-130 in Huntington's disease, and hosting a Research and Development Day that will feature expansion of our early-stage pipeline."

Recent Company Progress

- *Advancing AMT-061 into late-stage clinical development for the treatment of hemophilia B*

  - The first patient in the Phase IIb dose-confirmation study of AMT-061 in hemophilia B has consented and successfully screened into the study and is expected to be treated shortly. The Phase IIb study is an open-label, single-arm, single-dose trial being conducted in multiple centers in the U.S. Three patients will receive a single intravenous (IV) infusion of $2x10^{13}$ vc/kg and be evaluated for a period of approximately six to eight weeks to assess Factor IX (FIX) activity and confirm the dose for the global Phase III HOPE-B study. Top-line data are expected to be made available by the end of this year.

  - Patient enrollment has commenced for the global Phase III HOPE-B pivotal study. This registrational study includes a six-month lead-in phase to collect baseline data as patients will serve as their own control. The Company expects to begin dosing patients with AMT-061 in the first quarter of 2019.

- Clinical supplies for the AMT-061 Phase IIb dose-confirmation study have been successfully shipped to the site in anticipation of the first patient treatment. Additionally, the production of clinical batches is underway in the Company's Lexington, MA manufacturing facility for the Phase III HOPE- B study.

- The U.S. Patent and Trademark Office granted Patent Number 9,982,248 which broadly covers methods of treating bleeding disorders including hemophilia B, using AAV gene therapy with nucleic acid encoding the hyperactive Factor IX (FIX) Padua variant. This is the second U.S. patent to issue in a patent family that uniQure acquired from the inventor, Professor Paolo Simioni, a renowned hemophilia expert at the University of Padua, Italy. The '248 patent was prosecuted by uniQure and granted after consideration of the prior art.

➢ *Advancing AMT-130 for the treatment of Huntington's disease into a Phase I/II clinical study*

- The six-month in-life portion of a GLP-safety and toxicology study on AMT-130 in non-human primates has been completed and all study reports are expected to be finalized in the third quarter of 2018. Data from this study will be used in support of an Investigational New Drug (IND) application for AMT-130 which is expected to be submitted to the U.S. Food and Drug Administration (FDA) later this year. Following acceptance of this IND, the Company expects to advance AMT-130 with the goal of being the first AAV-gene therapy to enter the clinic in Huntington's disease patients.

- In June 2018, previously presented preclinical data on AMT-130 were published in the journal, *Molecular Therapy*. Researchers investigated the feasibility, efficacy and safety of AMT-130 in a Huntington's disease transgenic minipig model. At both three and six months after treatment, widespread, dose-dependent distribution of the vector was observed throughout the minipig brain that correlated strongly with human mutant huntingtin (mHTT) lowering. Specifically, six months after treatment at the high dose, researchers observed a mean mHTT lowering of 85.3% in the putamen, 75.0% in the caudate nucleus, 73.7% in the thalamus and 52.0% in the cortex.

➢ *Appointed proven leaders to clinical and medical organization, including Robert Gut, M.D., Ph.D. as Chief Medical Officer*

- Robert Gut, M.D., Ph.D. has been appointed Chief Medical Officer effective August 20, 2018 and will be responsible for leading all clinical development, medical affairs and related functions. Dr. Gut will be based in Lexington, MA and report directly to Mr. Kapusta. He succeeds Steven Zelenkofske, D.O., who will leave the Company due to personal family reasons that preclude him from relocating to the Boston area and require him to be closer to his home in Philadelphia, PA. Dr. Zelenkofske has agreed to continue to serve as a consultant to the Company through the end of the third quarter of 2018 to assist with the transition.

  Dr. Gut has 20 years of experience in the biopharmaceutical industry leading clinical development, regulatory and medical affairs activities in hematology and other therapeutic areas. Dr. Gut most recently served as Chief Medical Officer of Versartis, Inc. until its merger with Aravive Biologics in June 2018. For the majority of his career, Dr. Gut served as Vice President, Clinical Development & Medical Affairs at Novo Nordisk Inc., where he headed the company's U.S. Biopharm Medical

organization with leading products in hemophilia, including NovoSeven® (Factor VIIa), NovoEight® (Factor VIII), Tretten® (Factor XIII) and Factor IX. Dr. Gut's contributions helped achieve nine different FDA approvals and the successful launches of those products.

"I'm extremely pleased to join the uniQure management team at such an exciting time and help advance its potentially transformative gene therapy programs, including AMT-061 in hemophilia B and AMT-130 in Huntington's disease," stated Dr. Gut. "In my career, I have been keenly focused on developing and delivering innovative medicine to patients, including those with hemophilia. I very much look forward to leveraging my experiences and relationships with the hemophilia community to progress AMT-061 through the HOPE-B pivotal study."

Dr. Gut has served as a non-executive member of the uniQure Board of Directors since June 2018, and the Company plans that he will continue to serve on the Board as an executive director.

- Alison Long, M.B.B.Ch., Ph.D., has been appointed vice president, clinical development, hemophilia. She is an accomplished medical professional with both patient management experience and extensive biopharmaceutical R&D experience spanning multiple therapeutic areas, including hematology. Dr. Long most recently served as vice president, head of clinical research at Aegerion Pharmaceuticals where she led both the clinical operations and clinical development programs. Prior to joining Aegerion, she had been medical director of hematology clinical development at Biogen, where she delivered strategic medical leadership for their hemophilia clinical programs.

- Dr. Long joins Joseph Higgins, M.D., who joined uniQure earlier this year as vice president of clinical development, Huntington's disease. Dr. Higgins most recently served as the medical director of neurology for Quest Diagnostics and the CLIA laboratory director for Athena Diagnostics where he managed all neurological diagnostic testing at Quest. He will serve as the lead for the Company's Huntington's disease clinical program.

Near-Term Company Milestones

The company expects to achieve the following milestones in the second half of 2018:

- Complete the dosing of approximately three patients in the dose-confirmation study of AMT-061 and announce top-line data.

- Complete the GLP-safety and toxicology study of AMT-130 in Huntington's disease and submit the IND.

- Host a Research & Development Day in New York City to feature expansion of the Company's early-stage research pipeline and progress on current pipeline candidates.

- Complete the heart function study of AMT-126 in a diseased minipig model of congestive heart failure.

Financial Highlights

The company strengthened its financial position through completion of an underwritten public offering in May 2018. The gross proceeds from the offering, before deducting underwriting discounts and commissions and estimated offering expenses payable by uniQure, were $147.5 million.

Cash Position: As of June 30, 2018, the Company held cash and cash equivalents of $259.2 million, compared to $159.4 million as of December 31, 2017. The Company currently expects cash and cash equivalents will be sufficient to fund operations into 2021.

Revenues: Revenue for the three months ended June 30, 2018 was $3.1 million, compared to $4.9 million for the comparable period in 2017. Collaboration revenue for the three months ended June 30, 2018 was $0.9 million, compared to $4.2 million for the comparable period in 2017. The decrease in collaboration revenue was primarily due to the termination of the Chiesi co-development agreement in July 2017.

R&D Expenses: Research and development expenses were $18.5 million for the three months ended June 30, 2018, compared to $16.9 million for the comparable period in 2017. The increase was primarily related to costs incurred preparing for the initiation of the AMT-061 pivotal study and continued IND-enabling nonclinical studies of AMT-130.

SG&A Expenses: Selling, general and administrative expenses were $5.9 million for the three months ended June 30, 2018, compared to $5.4 million for the comparable period in 2017.

Other income, net: Other income, net was an income of $0.1 million for the three months ended June 30, 2018, compared to an expense of $2.4 million for the comparable period in 2017.

Net Loss: The net loss was $20.6 million, or $0.57 per share, for the three months ended June 30, 2018, compared to $21.3 million, or $0.83 per share, for the comparable period in 2017.

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 completion of our Phase IIb study, the release of top-line clinical data, the opportunity to be first to market with a Hemophilia B product, the filing of an IND or other regulatory filings or their acceptance by regulatory authorities, initiating a Huntington's Disease program or other program, trial or study that is the first in the clinic, completion of our GLP safety and toxicology study, our heart function study or other studies, the achievement of any of our planned near term or other milestones, the development of our gene therapy product

candidates, the transition to our AMT-061 product candidate, 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 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 Annual Report on Form 10-K filed on April 30th, 2018. 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 Contacts:

FOR INVESTORS:
Maria E. Cantor
Direct: 339-970-7536
Mobile: 617-680-9452
m.cantor@uniQure.com

Eva M. Mulder
Direct: +31 20 240 6103
Mobile: +31 6 52 33 15 79
e.mulder@uniQure.com

FOR MEDIA:
Tom Malone
Direct: 339-970-7558
Mobile: 339-223-8541
t.malone@uniQure.com

uniQure N.V.

UNAUDITED CONSOLIDATED BALANCE SHEETS

	June 30, 2018	December 31, 2017
	in thousands, except share and per share amounts	
Current assets		
Cash and cash equivalents	$ 259,180	$ 159,371
Accounts receivables and accrued income	1,037	1,586
Prepaid assets and other current assets	2,489	1,826
Total current assets	**262,706**	**162,783**
Non-current assets		
Property, plant and equipment, net	32,126	34,281
Intangible assets and goodwill	11,249	10,100
Restricted cash	2,458	2,480
Total non-current assets	**45,833**	**46,861**
Total assets	**$ 308,539**	**$ 209,644**
Current liabilities		
Accounts payable	$ 3,866	$ 2,908
Accrued expenses and other current liabilities	8,920	8,838
Current portion of long-term debt	8,028	1,050
Current portion of deferred rent	1,082	737
Current portion of deferred revenue	8,463	4,613
Current portion of contingent consideration	1,081	1,084
Total current liabilities	**31,440**	**19,230**
Non-current liabilities		
Long-term debt, net of current portion	12,840	19,741
Deferred rent, net of current portion	8,464	9,114
Deferred revenue, net of current portion	32,853	67,408
Contingent consideration, net of current portion	2,704	2,880
Derivative financial instruments related party	1,309	1,298
Other non-current liabilities	513	614
Total non-current liabilities	**58,683**	**101,055**
Total liabilities	**90,123**	**120,285**
Total shareholders' equity	**218,416**	**89,359**
Total liabilities and shareholders' equity	**$ 308,539**	**$ 209,644**

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended June 30,	
	2018	**2017**
	in thousands, except share and per share amounts	
Total revenues	**$ 3,050**	**$ 4,942**
Operating expenses:		
Research and development expenses	(18,493)	(16,866)
Selling, general and administrative expenses	(5,896)	(5,410)
Total operating expenses	**(24,389)**	**(22,276)**
Other income	565	266
Other expense	(429)	(2,640)
Loss from operations	**(21,203)**	**(19,708)**
Non operating items, net	972	(1,561)
Loss before income tax expense	**(20,231)**	**(21,269)**
Income tax expense	(361)	-
Net loss	**$ (20,592)**	**$ (21,269)**
Basic and diluted net loss per common share	$ (0.57)	$ (0.83)
Weighted average shares used in computing basic and diluted net loss per common share	36,205,061	25,560,348