uniQure

uniQure Announces 2018 Financial Results and Highlights Recent Company Progress

~ Initiated Dosing Phase of HOPE-B Pivotal Study of AMT-061 in Hemophilia B ~

~ Reported Updated Clinical Data from Phase IIb Study of AMT-061 Demonstrating Sustained FIX Activity at up to 51% of Normal, with Mean FIX of 38% of Normal at Twelve Weeks After Administration ~

~ Achieved Clearance of Investigational New Drug Application for AMT-130 in Huntington's Disease Providing for Start of Phase I/II Clinical Trial in 2H 2019 ~

~ Announced Expanded Research Pipeline with New AAV Gene Therapy Approaches to Hemophilia A, Fabry Disease and Spinocerebellar Ataxia Type 3 ~

Lexington, MA and Amsterdam, the Netherlands, February 28, 2019 — 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 2018 and highlighted recent progress across its business.

"2018 was a year marked by tremendous progress across all of our programs and gene therapy platform," stated Matt Kapusta, chief executive officer of uniQure. "We're also off to a very strong start in 2019 having achieved a number of value-driving milestones across our programs, including the initiation of the dosing phase of our global HOPE-B pivotal trial of AMT-061 in hemophilia B and the clearance of our IND for AMT-130 in Huntington's disease. Looking ahead in the year, we expect to complete enrollment in the HOPE-B pivotal trial and report longer-term follow up from the ongoing Phase IIb study. We also expect to initiate dosing of a Phase I/II study of AMT-130 and advance our pipeline programs in hemophilia A, Fabry disease and Spinocerebellar Ataxia Type 3. We believe achieving these goals will move us closer to providing potentially transformative therapies to patients and further demonstrate the importance of our industry leading technology platform and AAV manufacturing capabilities."

Recent Company Progress

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

  - The Company announced earlier this month that the first patient has been treated in the global Phase III HOPE-B pivotal study of AMT-061 in hemophilia B. The Company expects to complete the enrollment of approximately 50 patients in the trial by year-end.

  - Also this month, the Company presented updated clinical data on AMT-061 demonstrating increasing and sustained FIX levels of up to 51% of normal after the one-time administration of AMT-061 in the ongoing Phase IIb study. Mean Factor IX (FIX) activity for the three patients at 12 weeks increased to 38% of normal, exceeding threshold FIX levels generally considered sufficient to eliminate or significantly reduce the risk of bleeding events. None of the patients received Factor infusions, reported bleeding events or required immunosuppression over a combined 42 weeks of observation. The second and third patients had previously screen-failed and were excluded from another gene therapy study due to pre-existing neutralizing antibodies to a different AAV vector.

*** Epidemiological data indicate that factor activity above 12% of normal is associated with substantial reduction or elimination of spontaneous bleeds and factor usage. Den Uijl IE et al Haemophilia 2011; 17(6):849-53**

Collectively, these updated data further support AMT-061 as a potential first- and best-in-class gene therapy for hemophilia B.

- *Advancing AMT-130 into clinical development for the treatment of Huntington's disease*

  - In January, the Company announced the clearance of its Investigational New Drug Application (IND) for AMT-130 in Huntington's disease. AMT-130 is set to become the first AAV gene therapy to enter clinical development for Huntington's disease.

  - The Company expects to initiate a dose-escalating, randomized and controlled Phase I/II clinical study to assess the safety, tolerability and efficacy of a one-time treatment of AMT-130 in patients with Huntington's disease. The Company expects to open several clinical sites in the United States and begin dosing patients in the second half of this year, with the potential to report early safety data on initial patients treated in the study before the end of the year.

  - Multiple preclinical data presentations were featured at a recent scientific meeting demonstrating the potential of AMT-130 to restore neuronal function and partial reversal of gliosis as shown by magnetic resonance spectroscopy (MRS).

  - Additional preclinical data were presented on AMT-130 demonstrating restoration of brain cell function and reversal of neuropathology in a Huntington's disease mouse model, knock-down of mutant huntingtin in the cerebral spinal fluid (CSF) of minipigs, and showing low titers of pre-existing neutralizing antibody (NABs) in the CSF, irrespective of the levels of NABs in serum, suggesting the potential of treating a broad patient population with AMT-130.

- *Advancing robust pipeline of novel gene therapy research programs*

  - At its Research and Development Day late last year, the Company unveiled a pipeline expansion of new AAV gene therapy approaches to liver and central nervous system (CNS) disorders, including AMT-180 for hemophilia A, AMT-190 for Fabry disease and AMT-150 for Spinocerebellar Ataxia Type 3 (SCA3). The Company expects to advance the preclinical development of all three programs during 2019, including the initiation of a GLP toxicology study of AMT-180 in the first quarter of 2019.

  - The Company also introduced its proprietary miQURE™ gene silencing platform that has applications across multiple indications, including Huntington's disease and SCA3.

Upcoming Events

- Participate in a fireside chat at the Leerink Partners 8th Annual Global Healthcare Conference on March 1, 2019.

- Present at the Cowen 39th Annual Health Care Conference on March 12, 2019.

- Present at the Barclays Global Healthcare Conference on March 13, 2019.

- Present preclinical data on the Company's pipeline and technology platform at the American Society for Gene and Cell Therapy (ASGCT) Annual Meeting, April 29- May 2, 2019.

- Present preclinical data on AMT-150 for the treatment of Spinocerebellar Ataxia Type 3 (SCA3) at the 2019 American Academy of Neurology (AAN) Annual Meeting in May 4-10, 2019.

Financial Highlights

Cash Position: As of December 31, 2018, the Company held cash and cash equivalents of $234.9 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 2018 was $11.3 million, compared to $13.1 million for 2017 and $ 25.1 million in 2016. The decrease in collaboration revenue was primarily due to the termination of the Chiesi co-development agreement in July 2017, as well as nonrecurring revenue recognized in the prior year period associated with the production of research supplies in support of the Company's collaboration agreement with Bristol Myers-Squibb.

R&D Expenses: Research and development expenses were $74.8 million for the year ended 2018, compared to $72.2 million 2017 and $ 72.5 million in 2016. The increase was primarily related to costs incurred preparing for the initiation of the AMT-061 pivotal study as well as costs related to the GLP toxicology study completed in 2018 for AMT-130. Research and development expenses for the year ended December 31, 2018 include a $5.4 million noncash impairment loss on an in-process research asset acquired in the 2014 acquisition of the InoCard business, as well as $3.8 million of noncash income from the full release of contingent consideration previously recorded in relation to that acquisition.

SG&A Expenses: Selling, general and administrative expenses were $25.3 million for 2018, compared to $24.6 million for 2017 and $26.0 million for 2016. In 2016, the Company incurred nonrecurring costs related to its conversion from a foreign private issuer to a U.S. domestic filer.

Other income, net: Other income was $0.6 million for 2018, compared to $12.4 million for 2017 and $ 1.5 million for 2016. In 2017, the Company recorded other income of $13.8 million related to the July 2017 termination of the collaboration with Chiesi.

Net Loss: The net loss for the full years 2018, 2017 and 2016 was $83.3 million, or $2.34 per share, $79.3 million, or $2.94 per share, and $73.4 million, or $2.93 per share, respectively.

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 achievement of any of our planned near term or other milestones, our ability to provide further clinical updates on the Phase IIb study in 2019 or at any time, our ability to complete enrollment in our pivotal Phase III trial of AMT-061, the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies, our ability to initiate dosing of a Phase I/II study of AMT-130 in the second half of 2019 or ever, our ability to open several clinical sites for the Phase I/II study in the United States, our ability to report early safety data on initial patients treated in the Phase I/II study before the end of the year, our ability to advance our pipeline programs in hemophilia A, Fabry disease and Spinocerebellar Ataxia Type 3, our ability to move closer to providing potentially transformative therapies to patients and further demonstrate the importance of our industry leading technology platform and AAV manufacturing capabilities, and/or the development and regulatory approval of our product candidates in the United States or in Europe. 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, clinical results, 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 February 28, 2019. 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

	December 31, 2018	December 31, 2017
	in thousands	
Current assets		
Cash and cash equivalents	$ 234,898	$ 159,371
Accounts receivable and accrued income from related party	233	1,586
Prepaid expenses	1,116	1,139
Other current assets	329	687
Total current assets	**236,576**	**162,783**
Non-current assets		
Property, plant and equipment, net	29,179	34,281
Intangible assets, net	5,201	9,570
Goodwill	506	530
Restricted cash	2,444	2,480
Total non-current assets	**37,330**	**46,861**
Total assets	**$ 273,906**	**$ 209,644**
Current liabilities		
Accounts payable	$ 3,792	$ 2,908
Accrued expenses and other current liabilities	8,232	8,838
Current portion of long-term debt	-	1,050
Current portion of deferred rent	311	737
Current portion of deferred revenue	7,634	4,613
Current portion of contingent consideration	-	1,084
Total current liabilities	**19,969**	**19,230**
Non-current liabilities		
Long-term debt, net of current portion	35,471	19,741
Deferred rent, net of current portion	8,761	9,114
Deferred revenue, net of current portion	28,861	67,408
Contingent consideration, net of current portion	-	2,880
Derivative financial instruments related party	803	1,298
Other non-current liabilities	435	614
Total non-current liabilities	**74,331**	**101,055**
Total liabilities	**94,300**	**120,285**
Shareholders' equity		
Total shareholders' equity	**179,606**	**89,359**
Total liabilities and shareholders' equity	**$ 273,906**	**$ 209,644**

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Years ended December 31,		
	2018	**2017**	**2016**
	in thousands, except share and per share amounts		
Total revenues	**$ 11,284**	**$ 13,107**	**$ 25,098**
Operating expenses:			
Research and development expenses	(74,809)	(72,172)	(72,510)
Selling, general and administrative expenses	(25,305)	(24,635)	(25,999)
Total operating expenses	**(100,114)**	**(96,807)**	**(98,509)**
Other income	2,146	15,430	1,465
Other expense	(1,548)	(3,073)	-
Loss from operations	**(88,232)**	**(71,343)**	**(71,946)**
Non operating items, net	5,159	(8,116)	(283)
Loss before income tax expense	**(83,073)**	**(79,459)**	**(72,229)**
Income tax (expense) / benefit	(231)	199	(1,145)
Net loss	**$ (83,304)**	**$ (79,260)**	**$ (73,374)**
Basic and diluted net loss per ordinary share	$ (2.34)	$ (2.94)	$ (2.93)
Weighted average shares used in computing basic and diluted net loss per ordinary share	35,639,745	26,984,183	25,036,465