uniQure

uniQure Announces First Quarter 2019 Results and Highlights Recent Company Progress

~ Presented Updated Clinical Data from Phase IIb Study of AMT-061 in Patients with Hemophilia B Demonstrating Increases in FIX Activity Sustained at up to 51% of Normal at 12 Weeks

~ Achieved IND Clearance and Fast Track Designation for AMT-130 in Huntington's disease

~ Announced 6 Presentations at Upcoming ASGCT Meeting, Including Preclinical Data on Research Pipeline

Lexington, MA and Amsterdam, the Netherlands, April 29, 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 the first quarter of 2019 and highlighted recent progress across its business.

"During the first quarter of 2019, we continued to make excellent progress across our portfolio of gene therapy product candidates," stated Matt Kapusta, chief executive of uniQure. "We remain highly encouraged by the continued follow-up from our Phase IIb study of AMT-061, which shows the potential to normalize FIX activity in patients with hemophilia B. We are now focused on advancing our ongoing Phase III HOPE-B pivotal study of AMT-061 and have made significant progress activating sites and enrolling patients, with a goal of completing patient enrollment by the end of this year. We are also pleased to announce that AMT-061 received Orphan Drug Designation by the U.S. Food and Drug Administration, which, combined with our FIX-Padua intellectual property position and AAV5's potentially favorable immunogenicity profile, may provide a meaningful first-mover advantage."

"With the clearance of our Investigational New Drug application for AMT-130 earlier this year, we are making headway in the preparations for our Phase I/II study of AMT-130 in Huntington's disease. AMT-130, which recently received Fast Track Designation, is the world's first one-time administered therapy for Huntington's disease to enter clinical testing, and we continue to expect patient dosing in this landmark study to begin in the second half of the year."

Recent Company Progress

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

  - Enrollment of patients in the global HOPE-B pivotal trial in hemophilia B is advancing and the Company currently is on track to complete enrollment by the end of 2019. In February, the Company presented updated clinical data on AMT-061 demonstrating sustained increases in Factor IX activity (FIX) up to 51% of normal and mean FIX activity for the three patients of 38% of normal at 12 weeks. None of the patients received Factor infusions, reported bleeding events or required immunosuppression over a combined 42 weeks of observation.

  - On April 17, 2019 the U.S. Food and Drug Administration (FDA) granted AMT-061 Orphan Drug Designation (ODD). ODD in the U.S. provides special status for investigational drugs being developed for rare diseases considered to affect only up to 200,000 people in the U.S. The ODD program offers product market exclusivity for up to seven years in the U.S. following regulatory approval, along with tax and financial incentives for companies developing medicines for such orphan indications.

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

  - In January 2019, the FDA declared effective the Company's Investigational New Drug application (IND) for AMT-130. The Company expects to begin dosing patients in the second half of this year in its 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, and is planning to announce initial safety data on the surgical procedure before the end of this year.

  - Earlier this month, the Company announced that the FDA has granted Fast Track designation for AMT-130. The Fast Track program is designed to facilitate the development of and expedite the review of therapies to treat serious conditions and fill an unmet medical need. A therapy granted Fast Track Designation may be eligible for several benefits, including more frequent meetings and communications with the FDA and, if relevant criteria are met, the potential for Accelerated Approval, Priority Review or Rolling Review of a Biologics License Application (BLA) or New Drug Application (NDA).

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

  - The Company announced the acceptance of six data presentations, including multiple oral presentations featuring preclinical data for its gene therapy candidates in hemophilia A, Fabry disease and Huntington's disease at the upcoming Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) on April 29- May 2, 2019.

Upcoming Events

- 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 May 4-10, 2019.

- Present 26 weeks of follow-up data on the three patients in the Phase IIb dose-confirmation study of AMT-061 in hemophilia B patients at the Hemostasis & Thrombosis Research Society (HTRS) 2019 Symposium on May 10, 2019.

- Present preclinical data on AMT-130 for the treatment of Huntington's disease at the 2019 Associazione Italiana Corea Di Huntington Roma Onlus Annual Meeting on May 18, 2019.

Financial Highlights

Cash Position: As of March 31, 2019, the Company held cash and cash equivalents of $208.8 million, compared to $234.9 million as of December 31, 2018. The Company currently expects cash and cash equivalents will be sufficient to fund operations into 2021.

Revenues: Revenue for the three months ended March 31, 2019 was $1.1 million, compared to $3.5 million during the same period 2018. The decrease reflects the termination of activities associated with S100A1 in our collaboration with Bristol-Meyers Squibb in October 2018.

R&D Expenses: Research and development expenses were $20.5 million for the three months ended March 31, 2019, compared to $17.1 million during the same period 2018. The change was primarily related to increased activities associated with our ongoing Phase III pivotal study of AMT-061 and planned Phase I/II study of AMT-130, increased share-based compensation and the hiring of additional clinical and operations staff at our Lexington site.

SG&A Expenses: Selling, general and administrative expenses were $8.1 million for three months ended March 31, 2019, compared to $6.3 million during the same period 2018. The change was primarily related to increases in personnel and consulting expenses, professional fees and share-based compensation expenses.

Net Loss: The net loss for the three months ended March 31, 2019 was $27.8 million, or $0.74 per share, compared to $18.8 million, or $0.59 per share during the same period 2018.

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 ability of AMT-061 to normalize FIX activity in patients with hemophilia B, our ability to realize a meaningful first-mover advantage, 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, and our ability to report early safety data on initial patients treated in the Phase I/II study before the end of the year. 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.

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UNAUDITED CONSOLIDATED BALANCE SHEETS

	March 31, 2019	December 31, 2018
	in thousands	
Current assets		
Cash and cash equivalents	$ 208,787	$ 234,898
Accounts receivable and accrued income from related party	461	233
Prepaid expenses	3,597	1,116
Other current assets	350	329
Total current assets	**213,195**	**236,576**
Non-current assets		
Property, plant and equipment, net	27,778	29,179
Operating lease right-of-use asset	18,678	-
Intangible assets, net	5,917	5,201
Goodwill	496	506
Restricted cash	2,930	2,444
Total non-current assets	**55,799**	**37,330**
Total assets	**268,994**	**$ 273,906**
Current liabilities		
Accounts payable	$ 3,527	$ 3,792
Accrued expenses and other current liabilities	7,311	8,232
Current portion of operating lease liability	3,808	-
Current portion of deferred rent	-	311
Current portion of deferred revenue	7,772	7,634
Total current liabilities	**22,418**	**19,969**
Non-current liabilities		
Long-term debt	35,630	35,471
Operating lease liability, net of current portion	23,344	-
Deferred rent, net of current portion	-	8,761
Deferred revenue, net of current portion	27,465	28,861
Derivative financial instruments related party	2,603	803
Other non-current liabilities	422	435
Total non-current liabilities	**89,464**	**74,331**
Total liabilities	**111,882**	**94,300**
Total shareholders' equity	**157,112**	**179,606**
Total liabilities and shareholders' equity	**$ 268,994**	**$ 273,906**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Period ended March 31,	
	2019	**2018**
	in thousands, except share and per share amounts	
Total revenues	**$ 1,136**	**$ 3,478**
Operating expenses:		
Research and development expenses	(20,537)	(17,058)
Selling, general and administrative expenses	(8,067)	(6,301)
Total operating expenses	**(28,604)**	**(23,359)**
Other income	313	615
Other expense	(349)	(333)
Loss from operations	**(27,504)**	**(19,599)**
Non operating items, net	(268)	718
Loss before income tax expense	**(27,772)**	**(18,881)**
Income tax benefit	-	92
Net loss	**$ (27,772)**	**$ (18,789)**
Basic and diluted net loss per ordinary share	$ (0.74)	$ (0.59)
Weighted average shares used in computing basic and diluted net loss per ordinary share	37,676,172	31,710,497