

## uniQure Announces Third Quarter 2018 Financial Results and Highlights Company Progress

Lexington, MA and Amsterdam, the Netherlands, November 6, 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 third quarter of 2018 and highlighted recent progress across its business.

"We achieved a number of important milestones so far this year, including the initiation of our global HOPE-B pivotal trial in hemophilia B, the dosing of three patients in our dose-confirmation study of AMT-061 and the finalization of our toxicology study for AMT-130 in Huntington's disease," stated Matt Kapusta, chief executive officer of uniQure. "In the new year, we look forward to commencing patient dosing in the treatment phase of our HOPE-B pivotal study, as well as initiating a first-in-human study in Huntington's disease. We also are excited to host a Research & Development Day on November 19, 2018 featuring our newest gene therapy research programs and our recent progress advancing important new technologies."

### Recent Company Progress

- *Continuing to enroll HOPE-B Phase III pivotal study of AMT-061 in hemophilia B; Completed dosing of three patients in Phase IIb dose-confirmation study*
  - The global Phase III HOPE-B pivotal study of AMT-061 in hemophilia B continues to enroll patients with sites now open in the U.S. and Europe. 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.
  - Three patients in the Phase IIb dose-confirmation study of AMT-061 have been treated. The study is an open-label, single-arm, single-dose trial being conducted in multiple centers in the U.S. Three patients received a single intravenous (IV) infusion of  $2 \times 10^{13}$  vc/kg and are currently under evaluation, and the data at approximately six to eight weeks will be used to assess Factor IX (FIX) activity and confirm the dose for the global Phase III HOPE-B study. Top-line data will be made available before the end of this year.
- *Advancing AMT-130 for the treatment of Huntington's disease into a Phase I/II clinical study*
  - The Company recently completed its GLP-safety and toxicology study of AMT-130 in non-human primates. Data from the study demonstrated AMT-130 was generally safe and well-tolerated, and will be incorporated in an Investigational New Drug (IND) application expected to be submitted to the U.S. Food and Drug Administration (FDA) before the end of this year. Following acceptance of this IND, the Company expects to begin clinical development of AMT-130 with the goal of being the first AAV-gene therapy to enter the clinic in Huntington's disease patients in 2019.
- *Completed heart function study of AMT-126 in a preclinical animal model of congestive heart failure*

- The Company and its collaboration partner, Bristol-Myers Squibb, recently completed a heart function proof-of-concept study of AMT-126 in a pre-clinical animal model of heart failure. The study demonstrated DNA delivery and expression of S100A1 in the myocardium, thereby validating uniQure's vector delivery platform in the animal model. The data did not show a benefit on heart function at six months, and consequently, the Joint Steering Committee has chosen to discontinue work on S100A1. Bristol-Myers Squibb intends to replace the S100A1 collaboration target with another cardiovascular target. Bristol-Myers Squibb and uniQure will continue working on the other collaboration targets under the collaboration.
- *Hosting an investor-focused Research & Development Day on November 19, 2018 in New York City*
- uniQure will host a Research & Development Day on Monday, November 19, 2018 from 8:30 a.m.-12:00 p.m. EST. The program will showcase an expansion of the Company's early stage pipeline, targeting both the liver and central nervous system, and highlight advances in its technology platform. The event will be webcast live under the investor relations section of uniQure's website at [www.uniQure.com](http://www.uniQure.com)
- *Presented data on proprietary manufacturing and technology platform at the European Society of Gene and Cell Therapy 26<sup>th</sup> Annual Congress in Lausanne, Switzerland*
- In an oral presentation, the Company presented data on a highly potent, next-generation promoter for liver-directed gene therapies. Two *in-vivo* studies, one in a mouse model and one in non-human primates showed that the optimized promoter was capable of generating up to 40 times more protein expression compared to the reference promoter. Further details on the applicability of this promoter will be announced at the Research & Development Day.
  - uniQure recently initiated the development of a 500-liter stirred tank reactor process that has the potential to significantly increase manufacturing capacity and enhance scalability. Analysis of drug substance produced using the 500-liter stirred tank reactor showed that overall process performance, biological activity, content and product purity were comparable to material produced by smaller scale, wave-based reactors.

### **Expected Near-Term Company Milestones**

- Announce top-line data from the Phase IIb dose-confirmation trial of AMT-061 in hemophilia B before the end of the year
- Present long-term durability, safety and efficacy data from the ongoing Phase I/II study of AMT-060 at the American Society of Hematology Annual Meeting in early December
- Submit the IND application for AMT-130 in Huntington's disease before the end of the year and initiate preparations for clinical development
- Announce new gene therapy research programs and advancements in technology platform at an investor-focused Research & Development Day on November 19, in New York City

## Financial Highlights

**Cash Position:** As of September 30, 2018, the Company held cash and cash equivalents of \$239.5 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 September 30, 2018 was \$3.1 million, compared to \$2.3 million for the comparable period in 2017.

**R&D Expenses:** Research and development expenses were \$20.5 million for the three months ended September 30, 2018, compared to \$20.1 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. Research and development expenses for the three months ended September 30, 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 recorded in relation to that acquisition.

**SG&A Expenses:** Selling, general and administrative expenses were \$5.9 million for the three months ended September 30, 2018, compared to \$5.6 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 September 30, 2018, compared to an expense of \$14.2 million for the comparable period in 2017. The decrease was primarily related to income of \$13.8 million recorded in 2017 from the July 2017 termination of the collaboration with Chiesi.

**Net Loss:** The net loss was \$22.0 million, or \$0.59 per share, for the three months ended September 30, 2018, compared to \$10.2 million, or \$0.40 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](http://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 or the confirmation of the dose in that study, the release of top-line clinical data, dosing patients with AMT-061 in our Phase III clinical trial in the first quarter of 2019, any replacement by BMS of the S100A1 therapeutic target or the continued collaboration by BMS on other therapeutic targets pursuant to our collaboration agreement, the filing of an Investigational New Drug (IND) application for AMT-130 by the end of this year or its acceptance by regulatory authorities, initiating a Huntington's*

*Disease clinical program or other program, trial or study that is the first-in-patient or that otherwise begins in 2019, the achievement of any of our planned near term or other milestones, 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. 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 March 14, 2018 and Quarterly Report on Form 10-Q filed on November 6, 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.*

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UNAUDITED CONSOLIDATED BALANCE SHEETS

	September 30, 2018	December 31, 2017
	in thousands, except share and per share amounts	
<b>Current assets</b>		
Cash and cash equivalents	\$ 239,546	\$ 159,371
Accounts receivables and accrued income	552	1,586
Prepaid assets and other current assets	1,982	1,826
<b>Total current assets</b>	<b>242,080</b>	<b>162,783</b>
<b>Non-current assets</b>		
Property, plant and equipment, net	30,802	34,281
Intangible assets and goodwill	5,724	10,100
Restricted cash	2,454	2,480
<b>Total non-current assets</b>	<b>38,980</b>	<b>46,861</b>
<b>Total assets</b>	<b>\$ 281,060</b>	<b>\$ 209,644</b>
<b>Current liabilities</b>		
Accounts payable	\$ 4,115	\$ 2,908
Accrued expenses and other current liabilities	7,098	8,838
Current portion of long-term debt	11,111	1,050
Current portion of deferred rent	1,093	737
Current portion of deferred revenue	8,594	4,613
Current portion of contingent consideration		1,084
<b>Total current liabilities</b>	<b>32,011</b>	<b>19,230</b>
<b>Non-current liabilities</b>		
Long-term debt, net of current portion	9,597	19,741
Deferred rent, net of current portion	8,156	9,114
Deferred revenue, net of current portion	29,849	67,408
Contingent consideration, net of current portion		2,880
Derivative financial instruments related party	1,085	1,298
Other non-current liabilities	510	614
<b>Total non-current liabilities</b>	<b>49,197</b>	<b>101,055</b>
<b>Total liabilities</b>	<b>81,208</b>	<b>120,285</b>
<b>Total shareholders' equity</b>	<b>199,852</b>	<b>89,359</b>
<b>Total liabilities and shareholders' equity</b>	<b>\$ 281,060</b>	<b>\$ 209,644</b>

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	<b>Three months ended September 30,</b>	
	<b>2018</b>	<b>2017</b>
	<b>in thousands, except share and per share amounts</b>	
<b>Total revenues</b>	<b>\$ 3,148</b>	<b>\$ 2,260</b>
<b>Operating expenses:</b>		
Research and development expenses	(20,541)	(20,103)
Selling, general and administrative expenses	(5,898)	(5,584)
<b>Total operating expenses</b>	<b>(26,439)</b>	<b>(25,687)</b>
Other income	557	14,413
Other expense	(490)	(261)
<b>Loss from operations</b>	<b>(23,224)</b>	<b>(9,275)</b>
Non operating items, net	1,157	(1,248)
<b>Loss before income tax expense</b>	<b>(22,067)</b>	<b>(10,523)</b>
Income tax benefit	32	278
<b>Net loss</b>	<b>\$ (22,035)</b>	<b>\$ (10,245)</b>
Basic and diluted net loss per common share	\$ (0.59)	\$ (0.40)
Weighted average shares used in computing basic and diluted net loss per common share	37,247,193	25,632,642