



## uniQure Announces Third Quarter 2019 Results and Highlights Recent Company Progress

October 28, 2019

~ Announced Completion of Full Patient Enrollment in HOPE-B Pivotal Study of Etranacogene Dezaparvec ~

~ Achieved Site Initiation and Began Patient Screening for Phase I/II Clinical Study of AMT-130 in Huntington's Disease ~

~ Strong Balance Sheet with September 30, 2019 Cash and Cash Equivalents of \$403 Million Expected to Fund Operations into Mid-2022 ~

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Oct. 28, 2019 (GLOBE NEWSWIRE) -- [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 2019 and highlighted recent progress across its business.

"We achieved a major company milestone for 2019 with the earlier than expected completion of full patient enrollment in the HOPE-B pivotal study of etranacogene dezaparvec in hemophilia B, as well as the initiation of clinical sites and patient screening for the Phase I/II clinical study of AMT-130 in Huntington's disease," stated [Matt Kapusta](#), chief executive officer of uniQure. "We also strengthened our balance sheet with new capital that we expect will fund operations into mid-2022 and allows us to deliver on key corporate goals across the business. We look forward to continuing our momentum through the remainder of the year and sharing our progress during what we believe will be a very productive 2020."

### Recent Company Progress

- *Advancing late-stage development of etranacogene dezaparvec (AMT-061) for the treatment of hemophilia B*
  - In early September 2019, the Company announced the achievement of patient enrollment in its HOPE-B pivotal study of etranacogene dezaparvec for the treatment of patients with severe and moderately severe hemophilia B. Due to the high level of interest in the study from both patients and study investigators, the study was over-enrolled with 6 patients, providing a cumulative enrollment of 62 patients across 39 sites in the United States and Europe.
  - With the achieved enrollment of the HOPE-B pivotal study, the Company expects to provide top-line Factor IX (FIX) activity data on all patients before the end of 2020, and to submit a Biologics License Application (BLA) for marketing authorization of etranacogene dezaparvec in 2021.
- *Advancing AMT-130 into clinical development for the treatment of Huntington's disease*
  - The Company continues to make important progress with 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. Several clinical sites are now open for enrollment and patient screening is underway. The Company expects to enroll the first sentinel patient later this year or in early 2020.
- *Advancing research pipeline of gene therapy candidates towards the clinic*
  - Numerous preclinical and nonclinical studies of AMT-180 for the treatment of hemophilia A are underway to support the submission of a clinical trial application next year. AMT-180 incorporates a proprietary, exclusively licensed, modified Factor IX gene that has been demonstrated in preclinical studies to convey Factor VIII-independent activity and circumvent inhibitors to Factor VIII.
  - The Company continues to progress preclinical studies of AMT-150 for the treatment of spinocerebellar ataxia type 3. uniQure recently selected its lead AMT-150 candidate and expects to initiate IND-enabling studies in the first half of 2020. In May 2019, the Company presented data from a preclinical study demonstrating that a single administration of AMT-150 in the cerebrospinal fluid resulted in strong AAV transduction and significant mutant ataxin-3 lowering of up to 65%.
  - The Company is currently conducting preclinical studies of AMT-190 for the treatment of Fabry disease and expects to select a lead candidate in 2020. AMT-190 incorporates a proprietary, exclusively licensed, modified NAGA (ModNAGA) variant that may have several advantages over other therapies for Fabry disease, including higher stability in blood, circumvention of inhibitors, better biodistribution in the target organs, secondary toxic metabolite reduction and improved cross-correction of neighboring cells.

- *Successful follow-on offering funding operations into mid-2022*
  - In September 2019, the Company successfully completed the public follow-on offering of 5.6 million ordinary shares at a public offering price of \$46.00 per ordinary share, raising approximately \$242.8 million in net proceeds.
  - As of September 30, 2019, the Company held \$403.2 million in cash and cash equivalents, positioning the Company to complete the clinical development of etranacogene dezaparvovec in hemophilia B, begin preparations for commercial launch, to fund the Phase I/II clinical study of AMT-130 in Huntington's disease as well as the further development of our other preclinical product candidates.

#### Upcoming Investor and Industry Events

- World Orphan Drug Congress, November 11 – 14, 2019, Washington, D.C.
- Credit Suisse Healthcare Conference, November 12 – 13, 2019, Phoenix, AZ.
- Barclays Gene Therapy Summit, November 13, 2019, New York City, NY.
- 2019 International Ataxia Research Conference, November 13 – 16, 2019, Washington, D.C.
- Stifel Global Healthcare Conference, November 19 – 20, 2019, New York City, NY.
- 29th Annual NECTAR Meeting, November 28 -29, 2019, Cardiff, UK.
- Piper Jaffray, 31<sup>st</sup> Annual Healthcare Conference, December 3, 2019, New York City, NY.
- Evercore ISI 2<sup>nd</sup> Annual HealthCONx Conference, December 4, 2019, Boston, MA.

#### Financial Highlights

**Cash Position:** As of September 30, 2019, the Company held cash and cash equivalents of \$403.2 million, compared to \$184.1 million as of June 30, 2019. The Company currently expects cash and cash equivalents will be sufficient to fund operations into 2022.

**Revenues:** Revenue for the three months ended September 30, 2019 was \$1.0 million, compared to \$3.1 million during the same period 2018. The decrease reflects the October 2018 termination of the development of S100A1 under our collaboration with Bristol-Myers Squibb.

**R&D Expenses:** Research and development expenses were \$23.6 million for the three months ended September 30, 2019, compared to \$20.5 million during the same period 2018. The change was primarily related to increased activities associated with our ongoing clinical studies of etranacogene dezaparvovec, the 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 \$8.9 million for three months ended September 30, 2019, compared to \$5.9 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 September 30, 2019 was \$23.6 million, or \$0.58 per share, compared to \$22.0 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 gene therapies to treat patients with hemophilia B, hemophilia A, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other 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, our ability to provide further clinical updates and data with respect to our pivotal Phase III study of etranacogene dezaparvovec before the end of 2020 or ever, the ability to submit a BLA for marketing authorization of etranacogene dezaparvovec in 2021, or ever, the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies, our ability to enroll the first sentinel patient in our AMT-130 Phase I/II clinical study in early 2020 or ever, and our ability to fund our operations into mid-2020. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our clinical development activities, clinical results, collaboration arrangements, 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 October 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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[t.malone@uniQure.com](mailto:t.malone@uniQure.com)**uniQure N.V.****UNAUDITED CONSOLIDATED BALANCE SHEETS**

	<b>September 30, 2019</b>	<b>December 31, 2018</b>
	<b>(in thousands, except share and per share amounts)</b>	
<b>Current assets</b>		
Cash and cash equivalents	\$ 403,163	\$ 234,898
Accounts receivable and accrued income from related party	101	233
Prepaid expenses	3,820	1,116
Other current assets	<u>558</u>	<u>329</u>
<b>Total current assets</b>	<b>407,642</b>	<b>236,576</b>
<b>Non-current assets</b>		
Property, plant and equipment, net of accumulated depreciation of \$26.9 million as of September 30, 2019, and \$22.9 million as of December 31, 2018, respectively.	28,278	29,179
Operating lease right-of-use assets	26,852	-
Intangible assets, net	5,424	5,201
Goodwill	482	506
Restricted cash	<u>2,914</u>	<u>2,444</u>
<b>Total non-current assets</b>	<b>63,950</b>	<b>37,330</b>
<b>Total assets</b>	<b>\$ 471,592</b>	<b>\$ 273,906</b>
<b>Current liabilities</b>		
Accounts payable	\$ 3,782	\$ 3,792
Accrued expenses and other current liabilities	12,702	8,232
Current portion of operating lease liabilities	4,436	-
Current portion of deferred rent	-	311
Current portion of deferred revenue	<u>7,527</u>	<u>7,634</u>
<b>Total current liabilities</b>	<b>28,447</b>	<b>19,969</b>
<b>Non-current liabilities</b>		
Long-term debt	35,928	35,471
Operating lease liabilities, net of current portion	31,242	-
Deferred rent, net of current portion	-	8,761
Deferred revenue, net of current portion	23,882	28,861
Derivative financial instruments related party	1,354	803
Other non-current liabilities	<u>507</u>	<u>435</u>
<b>Total non-current liabilities</b>	<b>92,913</b>	<b>74,331</b>
<b>Total liabilities</b>	<b>121,360</b>	<b>\$ 94,300</b>
<b>Total shareholders' equity</b>	<b>350,232</b>	<b>179,606</b>
<b>Total liabilities and shareholders' equity</b>	<b>\$ 471,592</b>	<b>\$ 273,906</b>

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	<u>Three months ended September 30,</u>	
	<u>2019</u>	<u>2018</u>
	in thousands, except share and per share amounts	
<b>Total revenues</b>	<b>\$ 1,046</b>	<b>\$ 3,148</b>
<b>Operating expenses:</b>		
Research and development expenses	(23,554)	(20,541)
Selling, general and administrative expenses	(8,929)	(5,898)
<b>Total operating expenses</b>	<b>(32,483)</b>	<b>(26,439)</b>
Other income	453	557
Other expense	(342)	(490)
<b>Loss from operations</b>	<b>(31,326)</b>	<b>(23,224)</b>
Non operating items, net	7,722	1,157
<b>Loss before income tax expense</b>	<b>(23,604)</b>	<b>(22,067)</b>
Income tax expense	-	32
<b>Net loss</b>	<b>\$ (23,604)</b>	<b>\$ (22,035)</b>
Basic and diluted net loss per ordinary share	\$ (0.58)	\$ (0.59)
Weighted average shares used in computing basic and diluted net loss per ordinary share	40,738,938	37,247,193