



## uniQure Announces First Quarter 2020 Financial Results and Highlights Recent Company Progress

April 29, 2020

~ Completed Target Patient Dosing in Phase III HOPE-B Trial of Etranacogene Dezaparvovec in Hemophilia B ~

~ Announced 22 Presentations at Upcoming ASGCT Virtual Annual Meeting ~

LEXINGTON, Mass. and AMSTERDAM, April 29, 2020 (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 first quarter of 2020 and highlighted recent progress across its business.

"Despite the ongoing global crisis caused by the coronavirus pandemic, we ended the first quarter of 2020 in a position of strength and remain well capitalized," stated [Matt Kapusta](#), chief executive officer of uniQure. "I am very proud of the continued dedication of our employees in both the United States and the Netherlands, and thank them for the outstanding work they continue to do in driving forward our clinical programs, research pipeline, and manufacturing and technology operations. We are well positioned for an important second half of the year when we expect to announce new clinical data and advancements from across the business."

### Recent Company Progress

#### • *Advancing late-stage development of etranacogene dezaparvovec (AMT-061) for the treatment of hemophilia B*

- In the first quarter, the Company announced the achievement of targeted dosing of patients in the HOPE-B pivotal trial of etranacogene dezaparvovec (AMT-061), an investigational AAV5-based gene therapy incorporating the patent-protected FIX-Padua variant for the treatment of patients with severe and moderately severe hemophilia B. A total of 54 patients now have received the one-time dose of etranacogene dezaparvovec.
- The Company remains on track to provide 26-weeks of Factor IX data for all 54 patients in the HOPE-B trial before the end of this year and to file an application for marketing authorization in 2021.
- The manufacturing process validation for etranacogene dezaparvovec is ongoing, and the Company believes it is able to produce material at commercial scale.

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

- In March 2020, the Company announced that the first two patients in the Phase I/II clinical trial of AMT-130 in Huntington's disease have been enrolled after successfully meeting all screening and eligibility criteria. Due to the impact of the coronavirus pandemic, the two procedures that were scheduled to occur in late March have been temporarily postponed.
- The Company continues its work to resume treatment in the Phase I/II trial as soon as it is clinically appropriate and will provide further updates on the program at that time.

#### • *Advancing research pipeline of gene therapy candidates and the Company's proprietary technologies*

- Earlier this month, the Company announced its significant scientific presence at the upcoming American Society of Gene and Cell Therapy Virtual Annual Meeting, to be held May 12-15, 2020. In total, 22 data presentations will be delivered by the Company's researchers and scientists, including five oral presentations.
- The data presentations will highlight the Company's industry-leading research and technology capabilities and feature new preclinical findings on the Company's gene therapy candidates for hemophilia A, spinocerebellar ataxia type 3 and Fabry disease.

#### • *Strong cash position to advance the Company's programs*

- As of March 31, 2020, the Company held cash and cash equivalents of \$342.0 million, which is expected to be sufficient to fund the Company's operations into 2022.

### Upcoming Investor Events

- SunTrust Robinson Humphrey (STRH) Virtual Life Sciences Summit, May 5 – 6, 2020.
- American Society of Gene and Cell Therapy Virtual Annual Meeting, May 12 -15, 2020.

- Huntington's Disease Regulatory Science Consortium Virtual Annual Meeting, May 18 – 19, 2020.
- Goldman Sachs Virtual Annual Healthcare Conference, June 9 – 11, 2020.
- Credit Suisse 2020 Summer Biotech Conference, June 22 -24, 2020.

## Financial Highlights

**Cash Position:** As of March 31, 2020, the Company held cash and cash equivalents of \$342.0 million, compared to \$377.8 million as of December 31, 2019. The Company currently expects cash and cash equivalents will be sufficient to fund operations into 2022.

**Revenues:** Revenue for the three months ended March 31, 2020 was \$0.1 million, compared to \$1.1 million for the same period 2019. The decrease in 2020 reflects the reduction of activities following the end of the initial Research Term in May 2019 of our collaboration with Bristol-Myers Squibb ("BMS").

**R&D Expenses:** Research and development expenses were \$26.0 million for the three months ended March 31, 2020, compared to \$20.5 million for the same period 2019. The change was primarily related to increased activities associated with our ongoing clinical studies of etranacogene dezaparovec, the Phase I/II trial of AMT-130, increased share-based compensation and the additional recruitment of personnel to support the development of product candidates.

**SG&A Expenses:** Selling, general and administrative expenses were \$9.1 million for the three months ended March 31, 2020, compared to \$8.1 million for the same period 2019. The change was primarily related to increases in personnel and contractor related expenses.

**Other income, net:** Other income was \$6.5 million for the three months ended March 31, 2020, compared to other expense of \$0.3 million for the same period 2019, primarily reflecting foreign currency gains and a gain related to the change in fair value of the BMS warrants.

**Net Loss:** The net loss for the three months ended March 31, 2020 and March 31, 2019, was \$28.0 million, or \$0.63 loss per share and \$27.8 million, or \$0.74 loss 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 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 top-line data or any further clinical updates and data with respect to our pivotal Phase III trial of AMT-061 before the end of 2020 or ever, our ability to submit a BLA for marketing authorization of etranacogene dezaparovec in 2021, or ever, our ability to resume treatment or to dose the first two patients in the AMT-130 Phase I/II clinical trial, our ability to fund operations into 2022, and our ability to announce new clinical data and advancements from across the business later in 2020. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the impact of the ongoing COVID-19 pandemic on our Company and the wider economy and health care system, 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 our Quarterly Report on Form 10-Q filed on April 29, 2020. 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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## uniQure N.V.

## UNAUDITED CONSOLIDATED BALANCE SHEETS

March 31, 2020	December 31, 2019
(in thousands, except share and per share amounts)	

<b>Current assets</b>		
Cash and cash equivalents	\$ 342,029	\$ 377,793
Accounts receivable and accrued income from related party	258	947
Prepaid expenses	5,738	4,718
Other current assets	1,576	748
<b>Total current assets</b>	<b>349,601</b>	<b>384,206</b>
<b>Non-current assets</b>		
Property, plant and equipment, net of accumulated depreciation of \$29.8 million as of March 31, 2020 and \$28.6 million as of December 31, 2019 respectively	27,736	28,771
Operating lease right-of-use assets	26,288	26,797
Intangible assets, net	7,211	5,427
Goodwill	486	496
Restricted cash	2,921	2,933
<b>Total non-current assets</b>	<b>64,642</b>	<b>64,424</b>
<b>Total assets</b>	<b>\$ 414,243</b>	<b>\$ 448,630</b>
<b>Current liabilities</b>		
Accounts payable	\$ 4,989	\$ 5,681
Accrued expenses and other current liabilities	9,507	12,457
Current portion of operating lease liabilities	5,900	5,865
Current portion of deferred revenue	6,732	7,627
<b>Total current liabilities</b>	<b>27,128</b>	<b>31,630</b>
<b>Non-current liabilities</b>		
Long-term debt	36,209	36,062
Operating lease liabilities, net of current portion	30,518	31,133
Deferred revenue, net of current portion	23,713	23,138
Derivative financial instruments related party	1,004	3,075
Other non-current liabilities	524	534
<b>Total non-current liabilities</b>	<b>91,968</b>	<b>93,942</b>
<b>Total liabilities</b>	<b>\$ 119,096</b>	<b>\$ 125,572</b>
<b>Total shareholders' equity</b>	<b>295,147</b>	<b>323,058</b>
<b>Total liabilities and shareholders' equity</b>	<b>\$ 414,243</b>	<b>\$ 448,630</b>

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#### UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended March 31,	
	2020	2019
	(in thousands, except share and per share amounts)	
<b>Total revenues</b>	\$ 104	\$ 1,136
<b>Operating expenses:</b>		
Research and development expenses	(26,013)	(20,537)
Selling, general and administrative expenses	(9,072)	(8,067)
<b>Total operating expenses</b>	<b>(35,085)</b>	<b>(28,604)</b>
Other income	857	313
Other expense	(339)	(349)
<b>Loss from operations</b>	<b>(34,463)</b>	<b>(27,504)</b>
Non operating items, net	6,464	(268)
<b>Net loss</b>	<b>\$ (27,999)</b>	<b>\$ (27,772)</b>
Basic and diluted net loss per ordinary share	\$ (0.63)	\$ (0.74)
Weighted average shares used in computing basic and diluted net loss per ordinary share	44,279,456	37,676,172