uniQure Announces First Quarter 2021 Financial Results and Highlights Recent Company Progress

May 10, 2021

~ 52-week follow-up data from HOPE-B pivotal study expected to be presented later this quarter ~

~ Announced closing of the global commercialization and license agreement with CSL Behring for hemophilia B gene therapy ~

~ Completed enrollment of first dose cohort of U.S. Phase I/II clinical trial of AMT-130 for Huntington's disease, with initiation of second dose cohort and European Phase Ib/II expected in second half of 2021 ~

~ Pierre Caloz to be appointed Chief Operating Officer ~

~ Research & Development Day to be held Tuesday, June 22, 2021 ~

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, May 10, 2021 (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 2021 and highlighted recent progress across its business.

"We have made great strides with our clinical trial execution across both our hemophilia and Huntington's disease programs, with all patients in the HOPE-B pivotal trial completing their 52-week follow-up visits and all ten patients in the first dose cohort of our U.S. clinical trial in Huntington's disease completing their procedures," stated Matt Kapusta, chief executive officer at uniQure. "Looking at important near-terms catalysts, we look forward to announcing top-line, 52-week follow-up data from the HOPE-B pivotal trial, initiating our collaboration with CSL Behring and holding an R&D Day to provide updates on the research pipeline, including new product candidates," he added. "In the second half of the year, we expect to begin patient enrollment in the second cohort of AMT-130 and to initiate our open-label European study of AMT-130. Later this year, we expect to submit the BLA for etranacogene dezaparvovec and announce early data on the first four patients in the Phase I/II study of AMT-130."

Recent Company Progress

- *Advancing late-stage development of etranacogene dezaparvovec (AMT-061) for the treatment of hemophilia B*
    - The Company expects to announce top-line data during the second quarter of 2021 related to additional clinical trial endpoints in the HOPE-B pivotal trial of etranacogene dezaparvovec in severe to moderately severe hemophilia B patients, including 52-week annualized bleeding rates and FIX activity.
    - The Company announced the closing of the transaction with CSL Behring for its hemophilia B gene therapy following the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 on May 5, 2021. In accordance with the agreement, the upfront payment of $450 million was paid to uniQure on May 7, 2021.
    - In April 2021, the FDA removed the clinical hold on the Company's hemophilia B gene therapy program regarding a diagnosis of hepatocellular carcinoma (HCC), a form of liver cancer, in one patient in the HOPE-B trial. This followed a review of data from a comprehensive investigation that found it was highly unlikely etranacogene dezaparvovec was the cause of the HCC in this patient.
- *Advancing the clinical development of AMT-130 for the treatment of Huntington's disease*
    - The ten-patient, first dose cohort of the Phase I/II clinical trial of AMT-130 was fully enrolled in the second quarter of 2021. The second, higher-dose cohort is expected to begin enrollment in the third quarter of 2021 after a review by the independent Data Safety Monitoring Board (DSMB). Early imaging and biomarker data from the first four enrolled patients in this first-in-human AAV-gene therapy trial in Huntington's disease are anticipated by year-end 2021.
    - In February 2021, the DSMB recommended proceeding with patient enrollment in the Phase I/II clinical trial of AMT-130 after finding no significant safety concerns based on its review of the six-month data from the first two enrolled patients and the 90-day data from the next two enrolled patients.
    - The Company also announced plans to initiate an open label, Phase Ib/II clinical study of AMT-130 in Europe with enrollment beginning in the second half of 2021.
- *Expanding the Pipeline and Strengthening the Platform*

- In February 2021, the Company began an expansion of its Amsterdam site to build additional laboratories to support growing research and development activities, as well as a cleanroom designed for manufacturing cGMP materials at a 500-liter scale.

- The Company plans to host a virtual Research & Development Day on June 22, 2021 to highlight new investments in its expanding gene therapy pipeline focusing on CNS and rare, liver-directed disorders, as well as new advancements in platform technology and manufacturing.

- *Expansion of the leadership team*

  - Pierre Caloz will join the leadership team as Chief Operating Officer, effective May 17, 2021, overseeing all manufacturing operations, global CMC development and innovation, supply chain and facilities. Mr. Caloz joins the Company with nearly 20 years of global operations experience in the biopharma industry, including CSL Behring, Merck-Serono, Abgenix and Amgen. Most recently, he served as Senior Vice President and General Manager of European Union and Asia Pacific Operations at CSL Behring where he managed a team of more than 4,000 FTEs and an operating budget of more than EUR 1 billion. Mr. Caloz earned a BSc degree from the University of Geneva, a MSc degree from Swiss Federal Institute of Technology, and an EMBA from the Ashridge Business School.

- *Strong cash position to advance the Company's programs*

  - As of March 31, 2021, the Company's cash position was $260.8 million, which did not include any financial impact associated with the commercialization and license agreement with CSL Behring. Including the receipt from CSL Behring of the $450 million upfront payment, the Company expects cash and cash equivalents will be sufficient to fund operations into the second half of 2024.

Upcoming Investor Events (each to be conducted virtually)

- 2021 RBC Capital Markets Global Healthcare Conference, May 18-20, 2021
- Jefferies Virtual Healthcare Conference, June 1-4, 2021
- 42nd Annual Goldman Sachs Global Healthcare Conference, June 8-10, 2021
- Virtual Raymond James Human Health Innovations Conference, June 21-23, 2021
- uniQure Research & Development Day, June 22, 2021

Financial Highlights

Cash Position: As of March 31, 2021, the Company held cash and cash equivalents of $260.8 million, compared to $244.9 million as of December 31, 2020. In January 2021, the Company and Hercules entered into an amended debt facility agreement increasing the aggregate principle to up to $135.0 million, of which the Company drew down an additional $35.0 million for a total of $70.0 million outstanding under the facility as of March 31, 2021. In March 2021, the Company entered into an Open Market Sale Agreement with SVB Leerink LLC providing for the sale of up to $200.0 million of its ordinary shares from time to time in 'at-the-market' offerings under a shelf registration statement. Through March 31, 2021, the Company sold 859,885 ordinary shares pursuant to this agreement for gross proceeds of approximately $28.7 million.

Revenues: Revenue for the three months ended March 31, 2021 was $0.5 million, compared to $0.1 million during the same period in 2020.

R&D Expenses: Research and development expenses were $32.7 million for the three months ended March 31, 2021, compared to $26.0 million during the same period in 2020. The change was primarily related to increased activities associated with our ongoing clinical studies of Huntington's disease, increased activity in programs in preclinical development and the recruitment of personnel to support the development of our product candidates.

SG&A Expenses: Selling, general and administrative expenses were $12.4 million for the three months ended March 31, 2021, compared to $9.1 million during the same period in 2020. The change was primarily related to increases in personnel and consulting expenses to support our growth, increases in professional fees, and increased share-based compensation.

Other non-operating items, net: Other income, net was $3.1 million for the three months ended March 31, 2021, compared to other income, net of $6.5 million during the same period in 2020. The decrease is primarily due to the termination of the Bristol-Myers Squibb warrants in December 2020, resulting in no recognition of changes in fair value of the derivative financial liability in the current period compared to a recognized in the same period in 2020.

Net Loss: The net loss for the three months ended March 31, 2021 was $41.6 million, or $0.91 loss per share, compared to $28.0 million, or $0.63 loss per share during the same period in 2020.

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, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other 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, uniQure's expectations its plans to announce data from the HOPE-B pivotal trial, patient enrollment in the second cohort of AMT-130, the initiation of an open-label European study of AMT-130, the announcement of data on the first four patients in the Phase I/II study of AMT-130, the submission of the regulatory filing for marketing approval of etranacogene dezaparvovec, the employment of a Chief Operating Officer, the potential financial compensation we may be paid pursuant to our agreement with CSL Behring, the planned R&D Day or other updates on the research pipeline, and our plans to advance or expand our pipeline, accelerate research, identify business development opportunities, invest in technology, or scale our manufacturing capabilities. uniQure's 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 Commercialization and License Agreement with CSL Behring, 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 periodic securities filings, including its Annual Report on Form 10-K filed March 1, 2021. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and uniQure assumes 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, 2021	December 31, 2020
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 260,813	$ 244,932
Accounts receivables	5,445	6,618
Prepaid expenses	9,186	4,337
Other current assets	6,886	3,024
Total current assets	**282,330**	**258,911**
Non-current assets		
Property, plant and equipment, net	33,862	32,328
Operating lease right-of-use assets	25,313	26,086
Intangible assets, net	2,908	3,361
Goodwill	518	542
Restricted cash	2,716	2,748
Deferred tax asset	16,206	16,419
Total non-current assets	**81,523**	**81,484**
Total assets	**$ 363,853**	**$ 340,395**
Current liabilities		
Accounts payable	$ 5,749	$ 3,772
Accrued expenses and other current liabilities	20,896	18,038
Current portion of operating lease liabilities	5,457	5,524
Total current liabilities	**32,102**	**27,334**
Non-current liabilities		
Long-term debt	70,467	35,617
Operating lease liabilities, net of current portion	29,487	30,403
Other non-current liabilities	3,107	3,136
Total non-current liabilities	**103,061**	**69,156**
Total liabilities	**$ 135,163**	**$ 96,490**

Shareholders' equity		
Total shareholders' equity	**228,690**	**243,905**
Total liabilities and shareholders' equity	**$ 363,853**	**$ 340,395**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended March 31,	
	2021	**2020**
	(in thousands, except share and per share amounts)	
Total revenues	**$ 454**	**$ 104**
Operating expenses:		
Research and development expenses	(32,656)	(26,013)
Selling, general and administrative expenses	(12,375)	(9,072)
Total operating expenses	**(45,031)**	**(35,085)**
Other income	352	857
Other expense	(233)	(339)
Loss from operations	**(44,458)**	**(34,463)**
Non-operating items, net	3,115	6,464
Loss before income tax expense	**(41,343)**	**(27,999)**
Income tax expense	(213)	-
Net loss	**$ (41,556)**	**$ (27,999)**
Basic and diluted net loss per ordinary share	$ (0.91)	$ (0.63)
Weighted average shares used in computing basic and diluted net loss per ordinary share	45,468,485	44,279,456