uniQure Announces 2020 Financial Results and Highlights Recent Company Progress

March 1, 2021

~ Six patient procedures now completed in Phase I/II for Huntington's disease, including two procedures conducted in February 2021, with full enrollment of first cohort expected mid-2021 ~

~ Last patient in HOPE-B pivotal study to complete 52-week follow-up visit before end of the first quarter of 2021, with top-line data to be presented in the second quarter of 2021 ~

~ Analysis of the HOPE-B safety event expected to be completed and submitted to the FDA before end of the first quarter of 2021 ~

~ Research & Development Day to be held mid-2021 to announce expanded pipeline and platform advancements ~

LEXINGTON, Mass. and AMSTERDAM, March 01, 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 2020 and highlighted recent progress across its business.

"Despite unprecedented challenges in 2020, the uniQure team demonstrated extraordinary resiliency and dedication to the patients we serve, qualities which have defined our company for more than two decades," stated Matt Kapusta, chief executive officer at uniQure. "I'm very proud of uniQure's accomplishments, including completing patient dosing in the HOPE-B pivotal trial, presenting positive 26-week data on 54 patients that met the study's first primary endpoint, and announcing a landmark transaction with CSL Behring for the global commercialization of etranacogene dezaparvovec, which is expected to close in the second quarter of 2021 following the U.S. antitrust review. We also became the first company to initiate the clinical investigation of an AAV gene therapy for Huntington's disease and have now enrolled six patients in the study."

"Looking at 2021, we are focused on important catalysts as we prepare the filings for regulatory approval of etranacogene dezaparvovec and advance our Phase I/II study of AMT-130 in Huntington's disease, with early biomarker data on the initial patients expected as early as the end of this year. We are also making investments to expand our pipeline of promising gene therapies and further strengthen our AAV manufacturing platform. We look forward to hosting a Research and Development Day mid-year during which we will unveil new research programs and other technology initiatives."

Recent Company Progress

- *Advancing late-stage development of etranacogene dezaparvovec (AMT-061) for the treatment of hemophilia B*
    - In December 2020, initial 26-week Factor IX data in 54 patients in the HOPE-B pivotal trial of etranacogene dezaparvovec in severe to moderately severe hemophilia B patients were presented in a late-breaker oral presentation at the 62nd Annual Meeting of the American Society of Hematology (ASH). The data met the first primary endpoint with mean FIX activity of 37% of normal at 26 weeks. Patients achieved significant increases in FIX activity irrespective of pre-existing neutralizing antibodies. During the 26-week period after dosing, patients reported an 83% reduction in total bleeding events. Only three bleeding events were classified as spontaneous bleeds requiring treatment, representing a reduction of 92% of such bleeds. Mean annualized usage of FIX replacement therapy, a secondary endpoint in the clinical trial, declined by 96%.
    - The Company expects to present top-line data related to additional co-primary endpoints, including 52-week annualized bleeding rates and FIX activity, during the second quarter of 2021.
    - In December 2020, the FDA placed the Company's hemophilia B program on clinical hold following the report of a serious adverse event associated with a diagnosis of hepatocellular carcinoma (HCC), a form of liver cancer, in one patient in the HOPE-B trial. The analysis from the Company's ongoing investigation is expected to be completed shortly and submitted to the FDA during the first quarter of 2021. uniQure expects to meet with the FDA early in the second quarter of 2021 to discuss the analysis and the status of the clinical hold, after which the Company anticipates providing an update.
    - In January 2021, the Company and CSL Behring received requests for additional information by the Federal Trade Commission (FTC) as part of its antitrust review of a global licensing agreement for etranacogene dezaparvovec that was announced in June 2020. The Company expects to fulfill the request by the end of the first quarter of 2021 and, subject to completion of the FTC review, close the transaction during the second quarter of 2021.
- *Advancing the clinical development of AMT-130 for the treatment of Huntington's disease*
    - In February 2021, the independent Data Safety Monitoring Board 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.

- Two additional patient procedures were completed subsequent to the February DSMB meeting and as of March 1, 2021, a total of six patients have been enrolled in the study. The ten-patient cohort is on track to be fully enrolled by mid-2021. Early imaging and biomarker data from this first-in-human AAV-gene therapy trial in Huntington's disease are anticipated by year-end 2021.

- *Expanding the Pipeline and Strengthening the Platform*

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

  - In September 2020, the Company initiated a safety and toxicology study of AMT-150, a novel gene therapy for the treatment of Spinocerebellar Ataxia Type 3 (SCA3). SCA3 patients experience brain degeneration that results in movement disorders, rigidity, muscular atrophy and paralysis. There is currently no treatment available that slows the progressive course of this lethal disease. AMT-150 is a one-time administered AAV gene therapy incorporating the Company's proprietary miQURE™ silencing technology, similar to the technology used in the Company's AMT-130 program for the treatment for Huntington's disease, and is designed to halt ataxia in early manifest SCA3 patients.

  - In September 2020, the Company selected a lead gene therapy candidate (AMT-190) for the treatment of Fabry disease to advance into IND-enabling studies. The lead candidate is a one-time administered AAV5 gene therapy incorporating an α-galactosidase A (GLA) transgene. In preclinical studies comparing multiple product candidates, AMT-190 demonstrated the most robust and sustained increases in GLA activity.

- *Milestone achieved in research collaboration with Bristol Myers Squibb*

  - In December 2020, the Company earned a $4.4 million milestone payment from Bristol Myers Squibb for achieving a pre-specified milestone under the companies' collaboration and license agreement (CLA) of 2015. The milestone payment is related to Bristol Myers Squibb's decision to select a gene therapy product candidate for IND-enabling studies. Under the terms of the CLA, the Company is entitled to receive up to $217.0 million in aggregate milestone payments for each collaboration target, as well as royalties on net product sales.

  - Also, in December 2020, the Company and Bristol Myers Squibb amended the CLA to limit Bristol Myers Squibb to four existing collaboration targets. The amendment also terminated two warrants previously granted to Bristol Myers Squibb to increase Bristol Myers Squibb's ownership in the Company up to 19.9%. The companies agreed that upon the consummation of a change of control transaction of the Company, uniQure will make a pre-specified payment to Bristol Myers Squibb, subject to certain terms and conditions.

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

  - In January 2021, the Company amended its current debt facility with Hercules Capital from $35 million to $135 million and drew down $35 million of that principal, bringing the total outstanding debt to $70 million.

  - As of December 31, 2020, the Company's cash position was $244.9 million, which together with the Company's current debt facility, is expected to fund operations into the second half of 2022. This does not include any financial impact associated with the pending collaboration and license agreement with CSL Behring. Assuming the receipt from CSL Behring of the $450 million payment due at the closing of the transaction, 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)

- Cowen 41st Annual Health Care Conference, March 1-4, 2021
- H.C. Wainwright Global Life Science Conference, March 9-10, 2021
- Stifel CNS Day, March 31, 2021
- Guggenheim Healthcare Talks - 2021 Genomic Medicines & Rare Disease, April 1, 2021
- Wells Fargo Annual Biotech Corporate Access Days, April 6 & 8, 2021
- 20th Annual Needham Virtual Healthcare Conference, April 12-15, 2021

- Chardan 5th Annual Genetic Medicines Manufacturing Summit, April 26-27, 2021

Financial Highlights

Cash Position: As of December 31, 2020, the Company held cash and cash equivalents of $244.9 million, compared to $377.8 million as of December 31, 2019.

Revenues: Revenue for the year ended December 31, 2020 was $37.5 million, compared to $7.3 million during the same period in 2019. The change was primarily related to an increase in non-cash license revenue that we recognized as of the December 1, 2020 effective date of the amended Bristol-Myers Squibb collaboration and license agreement, as well as from revenue that we recognized in December 2020 following achievement of a research milestone for one of the four Collaboration Targets.

R&D Expenses: Research and development expenses were $122.4 million for the year ended December 31, 2020, compared to $94.7 million during the same period in 2019. The change was primarily related to increased activities associated with our ongoing clinical studies of etranacogene dezaparvovec and AMT-130, increased share-based compensation, increased license expenses and the recruitment of personnel to support the development of our product candidates.

SG&A Expenses: Selling, general and administrative expenses were $42.6 million for the year ended December 31, 2020, compared to $33.5 million during the same period in 2019. The change was primarily related to increases in personnel and consulting expenses to support our growth, as well as increases in professional fees.

Other non-operating items, net: Other expenses were $16.0 million for the year ended December 31, 2020, compared to other expenses of $3.1 million during the same period in 2019 primarily related to foreign exchange results related to changes in the exchange rates between the Euro and the U.S. Dollar.

Net Loss: The net loss for the year ended December 31, 2020 was $125.0 million, or $2.81 loss per share, compared to $124.2 million, or $3.11 loss per share during the same period in 2019.

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 about its cash runway, the timing of submitting data to the FDA or providing an update related to the investigation of any safety event related to the HOPE-B study, the enrollment of patients in, or Data Safety Monitoring Board review of, our Phase I/II gene therapy clinical trial of AMT-130 in Huntington's disease, the timing of the announcement of data in our Phase I/II gene therapy clinical trial of AMT-130, the timing of the announcement of data in our HOPE-B pivotal trial of etranacogene dezaparvovec, the timing of filing for regulatory approval of etranacogene dezaparvovec, the timing of regulatory approval of our agreement with CSL Behring, the potential financial compensation we may be paid pursuant to our agreement with CSL Behring, 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, the regulatory approval of that transaction, 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.

uniQure Contacts:

FOR INVESTORS:

Maria E. Cantor
Direct: 339-970-7536
Mobile: 617-680-9452
m.cantor@uniQure.com

Chiara Russo
Direct: 617-306-9137
Mobile: 617-306-9137
c.russo@uniQure.com

FOR MEDIA:

Tom Malone
Direct: 339-970-7558
Mobile:339-223-8541
t.malone@uniQure.com

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UNAUDITED CONSOLIDATED BALANCE SHEETS

	December 31, 2020	December 31, 2019

	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 244,932	$ 377,793
Accounts receivables	6,618	-
Accounts receivable from related party	-	947
Prepaid expenses	4,337	4,718
Other current assets	3,024	748
Total current assets	**258,911**	**384,206**
Non-current assets		
Property, plant and equipment, net	32,328	28,771
Operating lease right-of-use assets	26,086	26,797
Intangible assets, net	3,361	5,427
Goodwill	542	496
Restricted cash	2,748	2,933
Deferred tax asset	16,419	-
Total non-current assets	**81,484**	**64,424**
Total assets	**$ 340,395**	**$ 448,630**
Current liabilities		
Accounts payable	$ 3,772	$ 5,681
Accrued expenses and other current liabilities	18,038	12,457
Current portion of operating lease liabilities	5,524	5,865
Current portion of deferred revenue	-	7,627
Total current liabilities	**27,334**	**31,630**
Non-current liabilities		
Long-term debt	35,617	36,062
Operating lease liabilities, net of current portion	30,403	31,133
Deferred revenue, net of current portion	-	23,138
Derivative financial instruments related party	-	3,075
Other non-current liabilities	3,136	534
Total non-current liabilities	**69,156**	**93,942**
Total liabilities	**$ 96,490**	**$ 125,572**
Shareholders' equity		
Total shareholders' equity	**243,905**	**323,058**
Total liabilities and shareholders' equity	**$ 340,395**	**$ 448,630**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Years ended December 31,		
	2020	**2019**	**2018**
	(in thousands, except share and per share amounts)		
Total revenues	**$ 37,514**	**$ 7,281**	**$ 11,284**
Operating expenses:			
Research and development expenses	(122,400)	(94,737)	(74,809)
Selling, general and administrative expenses	(42,580)	(33,544)	(25,305)
Total operating expenses	**(164,980)**	**(128,281)**	**(100,114)**
Other income	3,342	1,888	2,146
Other expense	(1,302)	(2,028)	(1,548)
Loss from operations	**(125,426)**	**(121,140)**	**(88,232)**
Non-operating items, net	(16,017)	(3,061)	5,159
Loss before income tax income / (expense)	**(141,443)**	**(124,201)**	**(83,073)**
Income tax income / (expense)	16,419	-	(231)
Net loss	**$ (125,024)**	**$ (124,201)**	**$ (83,304)**
Basic and diluted net loss per ordinary share	$ (2.81)	$ (3.11)	$ (2.35)
Weighted average shares used in computing basic and diluted net loss per ordinary share	44,466,365	39,999,450	35,639,745