uniQure Announces Third Quarter 2020 Financial Results and Highlights Recent Company Progress

October 27, 2020

~ Enrolled First Four Patients in Phase I/II Clinical Trial of AMT-130 in Huntington's Disease ~

~ Top-Line Data from HOPE-B Pivotal Trial in Hemophilia B Expected Before Year End ~

~ Initiated IND-enabling Studies for AMT-150 in Spinocerebellar Ataxia Type 3 (SCA3) ~

LEXINGTON, Mass. and AMSTERDAM, Oct. 27, 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 third quarter of 2020 and highlighted recent progress across its business.

"We made excellent progress during the third quarter in our Phase I/II gene therapy clinical trial of AMT-130 in Huntington's disease as the first four patients have now been enrolled in the study, and we look forward to completing enrollment of our first dose cohort by mid-2021," stated Matt Kapusta, chief executive officer at uniQure. "Regarding our hemophilia B gene therapy program, all 54 patients in the HOPE-B pivotal trial of etranacogene dezaparvovec have now completed their 26-week follow-up visits, and we remain on track to announce top-line data from this registration-enabling study before the end of this year. This is expected to be the first data from a pivotal study of a hemophilia B gene therapy, as well as the first clinical data from a large-scale study evaluating gene therapy in patients irrespective of their preexisting AAV neutralizing antibodies. We also continue to work toward the regulatory approval and closing of our landmark licensing agreement for etranacogene dezaparvovec with CSL Behring."

"I'd like to specially thank all our employees for their tireless focus and dedication during these challenging times," he added. "We've made very strong progress thus far in 2020, and I look forward to ending the year on a strong note."

Recent Company Progress

- *Advancing late-stage development of etranacogene dezaparvovec (AMT-061) for the treatment of hemophilia B*
    - Patient dosing was completed in the HOPE-B pivotal trial of etranacogene dezaparvovec in March, 2020, and 26-week follow up on all patients has now been achieved. The Company remains on track to report 26-week Factor IX data for all 54 patients enrolled in the global clinical trial before the end of this year.
    - In June 2020, the Company and CSL Behring entered into a licensing agreement providing CSL Behring with exclusive global rights to etranacogene dezaparvovec. The agreement is one of the largest gene therapy deals announced to date and leverages CSL Behring's global hematology capabilities and infrastructure to benefit hemophilia B patients around the world. Under the terms of the agreement, which is subject to regulatory review in the United States, Australia and the United Kingdom, the Company will receive a $450 million upfront cash payment and be eligible to receive up to $1.6 billion in payments based on regulatory and commercial milestones. The Company will also be eligible to receive tiered double-digit royalties in a range of up to a low-twenties percentage of net product sales arising from the collaboration. Under the terms of the licensing agreement, CSL Behring is responsible for the submission of a Biologics License Application, which is expected in the second half of 2021.
- *Advancing AMT-130 into clinical development for the treatment of Huntington's disease*
    - In September 2020, the independent Data Safety Monitoring Board (DSMB), which is responsible for evaluating patient safety in the Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease, reviewed the 90-day follow-up data on the first two patients and observed no significant safety concerns to prevent further dosing in the study.
    - In early October 2020, the next two patient procedures were completed in the clinical trial. A total of four patients have been enrolled to date in this randomized, double-blinded study - two patients receiving AMT-130 and two patients who have received imitation (sham) surgery.
    - In accordance with study protocol, patient enrollment is expected to continue after a DSMB meeting to review the 90-day follow-up data on the two patients enrolled in October 2020 and the 6-month data on the two patients enrolled in June 2020. The Company expects this DSMB review will take place early next year and that the remaining six patients in the 10-patient first dose cohort will be enrolled by mid-2021.

- *Advancing research pipeline of gene therapy candidates toward the clinic*

    - 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). This study is expected to support the submission of an IND application in 2021. 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, including constructs incorporating a modified alpha-N-acetylgalactosaminidase transgene (modNAGA), AMT-190 demonstrated the most robust and sustained increases in GLA activity.

    - The Company continues to plan to aggressively advance and expand its pipeline by accelerating internal research with a focus on CNS disorders and other rare, liver-directed disorders, as well as by evaluating business development opportunities. Additionally, the Company continues to plan to invest in technology innovation and continue to further scale manufacturing capabilities.

- *Planned leadership team transition*

    - In August 2020, Ricardo Dolmetsch, Ph.D. was appointed as President, Research and Development. Dr. Dolmetsch joins the Company from Novartis Institutes for Biomedical Research (NIBR), the research arm of Novartis AG, where he served as the Global Head of Neuroscience since 2013. Prior to NIBR, Dr. Dolmetsch was a Professor at Stanford Medical School and a Senior Director at the Allen Institute for Brain Science in Seattle, Washington. He obtained his Bachelor's degree with honors from Brown University, earned his Ph.D. in neuroscience from Stanford University and conducted his postdoctoral training at Harvard University Medical School and Children's Hospital Boston.

    - Following the start of Dr. Dolmetsch's employment, Robert Gut, M.D., Ph.D. transitioned out of his roles as Chief Medical Officer and executive director of the Company. The Company intends for Dr. Gut to return to the Board as a non-executive director following shareholder approval, which is required under Netherlands law and expected to take place before the end of this year.

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

    - As of September 30, 2020, the Company's cash position was $279.5 million, which is expected to fund the Company's operations into 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)

- 29th Annual Credit Suisse Virtual Healthcare Conference, November 9-12, 2020
- Barclay's Gene Editing & Gene Therapy Summit, November 16, 2020
- Stifel 2020 Virtual Healthcare Conference, November 16-18, 2020
- Evercore ISI 3rd Annual HealthCONx, December 1-3, 2020

Financial Highlights

Cash Position: As of September 30, 2020, the Company held cash and cash equivalents of $279.5 million, compared to $314.3 million as of June 30, 2020.

Revenues: Revenue for the three months ended September 30, 2020 was $1.8 million, compared to $1.0 million during the same period in 2019.

R&D Expenses: Research and development expenses were $36.3 million for the three months ended September 30, 2020, compared to $23.6 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 $10.8 million for the three months ended September 30, 2020, compared to $8.9 million during the same period in 2019. The change was primarily related to increases in personnel and consulting expenses and share-based compensation expenses.

Other non-operating items, net: Other expense was $9.3 million for the three months ended September 30, 2020, compared to other income of $7.7 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 three months ended September 30, 2020 was $53.8 million, or $1.21 loss per share, compared to $23.6 million, or $0.58 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 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 HOPE-B pivotal trial of etranacogene dezaparvovec, the regulatory approval of our agreement with CSL Behring, the potential financial compensation we may be paid pursuant to our agreement with CSL Behring, the potential filing of an IND application for our AMT-150 product candidate for Spinocerebellar Ataxia Type 3 (SCA3), our plans to advance or expand our pipeline, accelerate research, identify business development opportunities, invest in technology, or scale our manufacturing capabilities, our plans to nominate and appoint Robert Gut to our Board. 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 2, 2020 and Quarterly Report on Form 10-Q filed on October 27, 2020. 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

	September 30, 2020	December 31, 2019
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 279,493	$ 377,793
Accounts receivable and accrued income from related party	223	947
Prepaid expenses	4,168	4,718
Other current assets	3,496	748
Total current assets	**287,380**	**384,206**
Non-current assets		
Property, plant and equipment, net	29,636	28,771
Operating lease right-of-use assets	26,207	26,797
Intangible assets, net	4,052	5,427
Goodwill	518	496
Restricted cash	2,714	2,933

Total non-current assets	**63,127**	**64,424**
Total assets	**$ 350,507**	**$ 448,630**
Current liabilities		
Accounts payable	$ 2,348	$ 5,681
Accrued expenses and other current liabilities	17,784	12,457
Current portion of operating lease liabilities	5,552	5,865
Current portion of deferred revenue	7,666	7,627
Total current liabilities	**33,350**	**31,630**
Non-current liabilities		
Long-term debt, net of current portion	35,494	36,062
Operating lease liabilities, net of current portion	30,333	31,133
Deferred revenue, net of current portion	22,413	23,138
Derivative financial instruments related party	547	3,075
Other non-current liabilities	477	534
Total non-current liabilities	**89,264**	**93,942**
Total liabilities	**$ 122,614**	**$ 125,572**
Shareholders' equity		
Total shareholders' equity	**227,893**	**323,058**
Total liabilities and shareholders' equity	**$ 350,507**	**$ 448,630**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended September 30,	
	2020	**2019**
	(in thousands, except share and per share amounts)	
Total revenues	**$ 1,789**	**$ 1,046**
Operating expenses:		
Research and development expenses	(36,302)	(23,554)
Selling, general and administrative expenses	(10,789)	(8,929)
Total operating expenses	**(47,091)**	**(32,483)**
Other income	1,032	453
Other expense	(220)	(342)
Loss from operations	**(44,490)**	**(31,326)**
Non operating items, net	(9,285)	7,722
Net loss	**$ (53,775)**	**$ (23,604)**
Basic and diluted net loss per ordinary share	$ (1.21)	$ (0.58)
Weighted average shares used in computing basic and diluted net loss per ordinary share	44,471,844	40,738,938