uniQure Announces Third Quarter 2022 Financial Results and Highlights Recent Company Progress

November 2, 2022

~ Patient enrollment at the higher dose of AMT-130 to resume in European Phase Ib/II study following Data Safety Monitoring Board recommendation ~

~ U.S. and European pre-approval inspections of Company's Lexington, MA manufacturing site completed with EMA issuing Good Manufacturing Certificate ~

~ Virtual investor event focused on next pipeline program, AMT-260 investigational gene therapy for refractory temporal lobe epilepsy, to be held on Tuesday, November 29, 2022 ~

LEXINGTON, Mass. and AMSTERDAM, Nov. 02, 2022 (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 2022 and highlighted recent progress across its business.

"We are pleased that following a comprehensive review of all available safety, biomarker and imaging data, the Data Safety Monitoring Board (DSMB) has recommended that we resume patient enrollment at the higher dose in the ongoing Phase Ib/II study evaluating AMT-130 in Huntington's disease," stated Matt Kapusta, chief executive officer of uniQure. "Patient safety will always be our utmost priority, and we are grateful for the close collaboration between our clinical team, study investigators and most importantly, the patients, all of whom were instrumental in completing our comprehensive investigations. We believe that AMT-130 has the potential to provide a positive impact for patients with this devastating disease for which there is no currently approved treatment. We look forward to finishing patient enrollment in the higher-dose cohort of the European study in the first half of 2023 and remain on track to announce data from the U.S. Phase I/II study in the second quarter of 2023."

"In the third quarter, the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) completed site inspections of our Lexington, Massachusetts gene therapy manufacturing facility, and we have since received good manufacturing practice (GMP) certification from the European authorities. This is an important milestone as we seek to establish industry-leading commercial gene therapy manufacturing capabilities. Our partner, CSL Behring, continues to interact with U.S. and European health authorities as they complete their review of the marketing applications for etranacogene dezaparvovec and, assuming approval, we look forward to partnering with our colleagues at CSL Behring to bring this potentially transformational treatment option to people living with hemophilia B."

Recent Updates

- *Advancing etranacogene dezaparvovec for the treatment of hemophilia B*
  - In May 2022, the Company's global commercialization partner, CSL Behring, announced that the biologic license application (BLA) for etranacogene dezaparvovec (AMT-061) was accepted by the FDA for priority review. This followed the EMA validation of the marketing application authorization (MAA) in March 2022. In accordance with the Company's commercialization and license agreement, CSL Behring is solely responsible for all regulatory activities, including any filings and agency interactions associated with etranacogene dezaparvovec and the companion diagnostic test for neutralizing antibodies to AAV5.
  - In the United States, the BLA remains under Priority Review with etranacogene dezaparvovec having Breakthrough Therapy Designation. In July 2022, CSL Behring was notified by the Committee for Advanced Therapies (CAT) in Europe that they will be unable to complete their review in accordance with the accelerated assessment timetable and will switch to a standard review procedure.
  - In July and August 2022, respectively, the EMA and the FDA conducted comprehensive, multi-day, pre-approval inspections of the Company's Lexington manufacturing site. Following the inspection, EMA issued a GMP certificate related to the production of commercial supply of etranacogene dezaparvovec.
- *Advancing AMT-130 for the treatment of Huntington's disease*
  - In October 2022, the DSMB reviewed all available safety, biomarker and imaging data from the ongoing Phase I/II clinical trials of AMT-130 and recommended resuming treatment at the higher dose. As previously announced in August 2022, patient enrollment at the higher dose had been voluntarily paused following the reporting of suspected unexpected significant adverse reactions (SUSARs) in three patients shortly after they received the higher dose of AMT-130. The SUSAR events in all three patients have since fully resolved.
    - The DSMB recommended implementing additional risk mitigation procedures during the first two weeks after the surgical administration of AMT-130, including a seven-day, in-person post-surgical visit. The DSMB also made no change to the treatment protocol regarding the use of immunosuppression that will continue to be at the discretion of the treating physician.
    - The Company plans to resume patient dosing in the open-label European clinical study as soon as possible and complete enrollment in the first half of 2023. To date, 10 patients have been treated in the European study, including all six patients in the lower-dose cohort and four of nine patients in the higher-dose cohort.
  - The Company still plans to announce one to two-years of follow up data from the U.S. Phase I/II clinical study in

the second quarter of 2023.
    - All 26 patients have been enrolled in the first two cohorts of the randomized, controlled and double-blinded U.S. Phase I/II study of AMT-130, including 10 patients in the lower-dose cohort (6 treated patients and 4 control patients) and 16 patients in the higher-dose cohort (10 treated patients and 6 control patients). One control patient from the higher-dose cohort was successfully crossed over to treatment in the third quarter of 2022 and received the lower dose of AMT-130.
  - In June 2022, the Company announced 12-month follow-up data from the lower-dose cohort of the U.S. Phase I/II study of AMT-130.
    - The lower dose was generally well-tolerated with no serious adverse events related to treatment.
    - In the four treated patients with evaluable data from this cohort, mean levels of mutant Huntingtin protein (mHTT) in the cerebral spinal fluid (CSF) declined at all timepoints compared to baseline and decreased by 53.8% at 12 months of follow-up.
    - In the six treated patients in the lower-dose cohort, measurements of neurofilament light chain (NfL) in the CSF, a biomarker of neuronal damage, initially increased as expected following the AMT-130 surgical procedure and declined thereafter, nearing baseline at 12 months of follow-up.
- *Progress towards 2023 Investigational New Drug (IND) Applications*
  - *AMT-260 for the treatment of refractory temporal lobe epilepsy (rTLE)* – In July 2022, the Company initiated an IND-enabling GLP toxicology study in non-human primates to support an IND submission expected in 2023.
  - *AMT-191 for the treatment of Fabry disease* – In August 2022, the Company initiated a GLP toxicology study of AMT-191 in non-human primates which is expected to support an IND submission in 2023.
  - The Company plans to host a virtual investor event on Tuesday, November 29, 2022 to highlight the unmet medical need of patients with refractory temporal lobe epilepsy and its gene therapy candidate AMT-260, as well as advancements in platform technology and AAV manufacturing capabilities.
- *Strong cash position to advance the Company's programs*
  - As of September 30, 2022, the Company had cash and cash equivalents of $440.3 million. The Company expects that its cash and cash equivalents will fund operations into the first half of 2025 assuming the achievement of the first commercial sales milestones under the CSL Behring Agreement.

Upcoming Investor Events

- Stifel 2022 Healthcare Conference, Wednesday, November 16, 2022, New York, NY
- uniQure virtual investor event, Monday, November 29, 2022

Financial Highlights

Cash position: As of September 30, 2022, the Company held cash and cash equivalents of $440.3 million, compared to $556.3 million as of December 31, 2021.

Revenues: Collaboration revenue for the three months ended September 30, 2022, was $1.4 million, compared to collaboration revenue of $2.0 million for the same period in 2021.

Cost of contract manufacturing: Cost of contract manufacturing for the three months ended September 30, 2022, was $0.9 million compared to nil for the same period in 2021. Costs incurred in 2022 related to the manufacture of etranacogene dezaparvovec for CSL Behring.

R&D expenses: Research and development expenses were $48.1 million for the three months ended September 30, 2022, compared to $36.4 million during the same period in 2021. The increase was primarily related to an increase in the fair value of contingent consideration associated with the acquisition of Corlieve Therapeutics SAS, the preclinical development of temporal lobe epilepsy (AMT-260), an increase in facility-related expenses and an increase in disposable costs.

SG&A expenses: Selling, general and administrative expenses were $13.3 million for the three months ended September 30, 2022, compared to $12.0 million during the same period in 2021. The increase was primarily related to the recruitment of personnel and contractor-related expenses.

Other non-operating items, net:
Other non-operating income, net was income of $11.3 million for the three months ended September 30, 2022, compared to other non-operating income, net of $8.6 million for the same period in 2021. The increase in other non-operating income, net was primarily related to an increase in net foreign currency gains of $3.9 million partially offset by an increase in interest expense related to the long-term debt with Hercules Capital, Inc.

Net loss:
The net loss for the three months ended September 30, 2022, was $47.9 million, or $1.02 basic and diluted loss per ordinary share, compared to $36.5 million net loss for the same period in 2021, or $0.79 basic and diluted loss per ordinary share.

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, refractory temporal lobe epilepsy, Fabry disease, 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," "establish," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "seek," "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, whether we will announce data from the U.S. Phase I/II study in the second quarter of 2023, whether we are able to bring AMT-061 to people living with hemophilia B and whether the treatment will be transformational, whether we will resume patient dosing in the open-label European study [in the fourth quarter of 2022] or complete enrollment in the first half of 2023, whether we will announce one to two-years of follow up data from the U.S. Phase I/II study in the second quarter of 2023, whether we will file an IND in 2023 for our rTLE program or for our Fabry program. The Company'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 postponement in our clinical trial for Huntington's disease, the impact of financial and geopolitical events 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 the Company's periodic securities filings, including its Annual Report on Form 10-K filed February 25, 2022. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and the Company 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, 2022	December 31, 2021
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 440,313	$ 556,256
Accounts receivable and contract asset	3,603	58,768
Inventories	4,075	-
Prepaid expenses	13,692	10,540
Other current assets and receivables	2,894	2,675
Total current assets	**464,577**	**628,239**
Non-current assets		
Property, plant and equipment, net	47,886	43,505
Operating lease right-of-use assets	27,804	25,573
Intangible assets, net	53,837	62,686
Goodwill	23,418	27,633
Deferred tax assets, net	14,627	15,647
Other non-current assets	6,085	5,897
Total non-current assets	**173,657**	**180,941**
Total assets	**$ 638,234**	**$ 809,180**
Current liabilities		
Accounts payable	$ 7,566	$ 2,502
Accrued expenses and other current liabilities	26,437	28,487
Current portion of contingent consideration	23,537	-
Current portion of operating lease liabilities	6,434	5,774
Total current liabilities	**63,974**	**36,763**
Non-current liabilities		
Long-term debt	102,394	100,963
Operating lease liabilities, net of current portion	29,893	28,987
Contingent consideration, net of current portion	9,158	29,542
Deferred tax liability, net	8,592	12,913
Other non-current liabilities	3,053	4,236
Total non-current liabilities	**153,090**	**176,641**

Total liabilities	**217,064**	**213,404**
Shareholders' equity		
Total shareholders' equity	**421,170**	**595,776**
Total liabilities and shareholders' equity	**$ 638,234**	**$ 809,180**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended September 30,	
	2022	**2021**
	(in thousands, except share and per share amounts)	
Total revenues	**$ 1,449**	**$ 1,989**
Operating expenses:		
Cost of contract revenues	**-**	-
Cost of contract manufacturing	(861)	
Research and development expenses	(48,068)	(36,432)
Selling, general and administrative expenses	(13,324)	(12,023)
Total operating expenses	**(62,253)**	**(48,455)**
Other income	1,485	1,680
Other expense	(199)	(214)
Loss from operations	**(59,518)**	**(45,000)**
Non-operating items, net	11,332	8,558
Loss before income tax benefit / (expense)	**$ (48,186)**	**$ (36,442)**
Income tax benefit / (expense)	329	(89)
Net loss	**$ (47,857)**	**$ (36,531)**
Earnings per ordinary share - basic		
Basic net (loss) per ordinary share	$ (1.02)	$ (0.79)
Earnings per ordinary share - diluted		
Diluted net (loss) per ordinary share	$ (1.02)	$ (0.79)
Weighted average shares - basic	46,772,430	46,152,404
Weighted average shares - diluted	46,772,430	46,152,404

