uniQure Announces Third Quarter 2023 Financial Results and Highlights Recent Company Progress

November 7, 2023

~ Announced FDA clearance of Investigational New Drug (IND) application for AMT-260 in refractory mesial temporal lobe epilepsy ~

~ On track to provide clinical update from U.S. and European Phase I/II trials of AMT-130 in Huntington's disease later in the fourth quarter of 2023 ~

~ uniQure and CSL win 2023 Prix Galien USA award for HEMGENIX® ~

~ Announced strategic reorganization to focus on advancing multiple clinical-stage programs and expect to deliver $180 million of cost savings over the next three years ~

~ Strong cash position of approximately $660 million as of September 30, 2023 expected to fund operations into the second quarter of 2027 ~

LEXINGTON, Mass. and AMSTERDAM, Nov. 07, 2023 (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 2023 and highlighted recent progress across its business.

"In the third quarter we made significant progress towards our goal of advancing multiple clinical-stage programs and focusing resources to generate value from these important investments," stated Matt Kapusta, chief executive officer of uniQure. "We are very pleased to have achieved FDA clearance of the IND for AMT-260 in refractory mesial temporal lobe epilepsy and look forward to initiating patient enrollment in the Phase I/IIa study as soon as possible. In addition, we are making good progress toward initiating patient screening for a Phase I/II trial of AMT-162 in SOD1-ALS and the submission of an IND for AMT-191 in Fabry disease. We also look forward to providing another clinical update later this quarter from our ongoing Phase I/II studies of AMT-130 in Huntington's disease, which will include up to 2.5 years of patient follow-up."

"As we prepare for 2024, we remain laser focused on execution across our portfolio of gene therapy product candidates with the goal of achieving clinical proof-of-concept as rapidly as possible. We also remain committed to carefully managing costs, prudently allocating capital, and thoughtfully evaluating strategies that can enhance value for shareholders in an increasingly challenging market environment."

Recent Updates

- *Advancing AMT-130 for the treatment of Huntington's disease*
  - Later in the fourth quarter of 2023, the Company plans to provide a clinical update from the U.S. and European Phase I/II studies of AMT-130, including 18- and 30-month follow-up data from the treated patients in the high- and low-dose U.S. cohorts, respectively. The data update will include safety and tolerability, biomarker, imaging and functional data across both high and low-dose cohorts.
  - A total of 33 patients have now been treated with AMT-130 across two dose cohorts in the U.S. and EU clinical trials. The Company recently initiated patient screening for a third cohort in up to 12 patients to further investigate both doses in combination with perioperative immunosuppression, with a focus on evaluating near-term safety and tolerability.
  - In the first quarter of 2024, the Company plans to request regulatory interactions with the Food and Drug Administration (FDA) to discuss the U.S. and EU data and potential late-stage development pathways for AMT-130.
- *Advancing additional programs into the clinic*
  - *AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (rMTLE)* – In the third quarter of 2023, the Company announced the clearance of an investigational new drug (IND) for the Phase I/IIa clinical study of AMT-260. Screening and patient enrollment is expected to begin in the fourth quarter of 2023 with first patient dosing planned to occur in the first quarter of 2024.
  - *AMT-162 for the treatment of SOD1 amyotrophic lateral sclerosis (ALS)* – In January 2023, the Company entered into a global licensing agreement with Apic Bio for ABP-102, now AMT-162 for the treatment of superoxide dismutase 1 (SOD1) ALS, a rare, genetic form of ALS. The Company expects to initiate patient screening in the fourth quarter of 2023 with first patient dosing planned to occur in the first quarter of 2024.
  - *AMT-191 for the treatment of Fabry disease* – The Company expects to submit an IND in the fourth quarter of 2023 and to begin patient dosing in 2024.
- *Supporting the commercial launch of HEMGENIX® in the United States and European Union*
  - In October 2023, uniQure and CSL Behring LLC ("CSL") announced that the companies received the 2023 Prix Galien USA Award in the category of Best Product for Rare/Orphan Diseases for HEMGENIX®. HEMGENIX® was one of several product nominations in this category. Created in 1970, the Prix Galien Awards recognizes outstanding innovation and scientific advancement and is regarded as the equivalent of the Nobel Prize for the life science industry.
  - In July 2023, uniQure received a $100 million milestone payment from CSL associated with the first commercial

sale of HEMGENIX® in the United States.

- *Implementing strategic reorganization focused on advancing multiple clinical-stage programs, achieving cost savings, and extending cash runway*
  - In October 2023, the Company announced a strategic reorganization plan which included a reduction of 28% of the workforce not related to manufacturing HEMGENIX®, the discontinuation of more than half of the research and technology projects, and a focus on executing across four clinical-stage programs. The Company expects a total cost savings of approximately $180 million over the next three years, which is expected to extend cash runway into the second quarter of 2027.
  - As part of the strategic reorganization and the significant reduction in research activities, Ricardo Dolmetsch, Ph.D., the Company's former chief scientific officer, departed the Company and will remain as a scientific consultant through the end of the year. Richard Porter, Ph.D., assumed responsibilities for research, as well as non-clinical and vector development in his new role as Chief Business and Scientific Officer. Dr. Porter has more than 25 years of neuroscience leadership in the biopharma industry and joined uniQure in June 2021 through the acquisition of uniQure France SAS (formerly, Corlieve Therapeutics), where he was founder and chief executive officer.

Upcoming Investor Events

- Stifel 2023 Healthcare Conference, November 14, 2023 – New York, NY

Financial Highlights

Cash position: As of September 30, 2023, the Company held cash and cash equivalents and investment securities of $658.9 million, compared to $392.8 million as of December 31, 2022. The Company entered into a royalty agreement in May 2023 and received an upfront payment of $375.0 million, and collected $100.0 million in July 2023 for a milestone due from CSL following the first sale of HEMGENIX® in the U.S.

Revenues: Revenues for the three months ended September 30, 2023 were $1.4 million, compared to $1.4 million in the same period in 2022. The current period included an increase in license revenues of $0.5 million and contract manufacturing revenues of $0.4 million related to contract manufacturing HEMGENIX® for CSL, and a decrease of $0.9 million in collaboration revenues.

R&D expenses: Research and development expenses were $65.4 million for the three months ended September 30, 2023, compared to $48.1 million in the same period in 2022. The change was primarily related to a non-cash, $8.8 million increase in the fair value of contingent consideration associated with the Company's acquisition of Corlieve Therapeutics in 2021.

SG&A expenses: Selling, general and administrative expenses were $18.1 million for the three months ended September 30, 2023, compared to $13.3 million in the same period in 2022. The increase was primarily related to an increase in personnel and contractor-related expenses, and in legal fees associated with various corporate initiatives.

Other non-operating items, net:
Other non-operating items, net was an expense of $7.8 million for the three months ended September 30, 2023, compared to net income of $11.3 million for the same period in 2022. The decrease in other non-operating items, net was primarily related to a decrease in foreign currency gains, net of $14.2 million and an increase in interest expense of $12.4 million primarily related to the royalty agreement that the Company entered into in May 2023, which partially was offset by an increase of $7.5 million in interest income earned on investment securities and cash on hand.

Net loss:
The net loss for the three months ended September 30, 2023, was $89.6 million, or $1.88 basic and diluted loss per ordinary share, compared to $47.9 million net loss for the same period in 2022, or $1.02 basic and diluted loss per ordinary share.

About uniQure

uniQure's mission is to reimagine the future of medicine by delivering innovative cures that transform lives. The recent approvals of our gene therapy for hemophilia B – a historic achievement based on more than a decade of research and clinical development – represent a major milestone in the field of genomic medicine and ushers in a new treatment approach for patients living with hemophilia. We are now leveraging our modular and validated technology and manufacturing platform to advance a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory mesial temporal lobe epilepsy, amyotrophic lateral sclerosis (ALS), Fabry disease, and other severe 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, the Company's statements about its cash runway and its ability to fund operations into 2027, the Company's cost savings related to the recently-announced strategic organization, whether there will be continued progress on the commercialization of HEMGENIX®, the Company's plans to announce follow up data from the U.S. and European Phase I/II clinical study of AMT-130 in 2023, whether that data will help to further guide our ongoing clinical development of AMT-130, the Company's expectation to submit an investigational new drug application for AMT-191 in Fabry disease in 2023 and begin patient dosing in 2024, the Company's expectation to initiate patient screening in AMT-162 and AMT-260 in the fourth quarter of 2023 and begin patient dosing in each in the first quarter of 2024, and whether the Company will begin interactions with regulatory agencies in the first quarter of 2024 with respect to AMT-130. 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 regulatory approval and commercial launch of HEMGENIX®, material changes to our interim or preliminary data, 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, the Company's ability to raise additional capital to support late stage development of the Company's clinical program(s) if supported by future data, our Commercialization and License Agreement with CSL Behring, our clinical development activities, clinical results, collaboration arrangements, regulatory oversight, product commercialization and intellectual property claims and ongoing litigation, 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 27, 2023 and the Quarterly Report on Form 10-Q filed November 7, 2023. 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, 2023	December 31, 2022
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 229,484	$ 228,012
Current investment securities	429,428	124,831
Accounts receivable and contract asset	1,644	102,376
Inventories	15,654	6,924
Prepaid expenses	14,884	11,817
Other current assets and receivables	2,532	2,814
Total current assets	**693,626**	**476,774**
Non-current assets		
Property, plant and equipment, net	45,946	50,532
Non-current investment securities	—	39,984
Operating lease right-of-use assets	30,360	32,726
Intangible assets, net	57,976	58,778
Goodwill	25,273	25,581
Deferred tax assets, net	12,351	14,528
Other non-current assets	6,018	6,061
Total non-current assets	**177,924**	**228,190**
Total assets	**$ 871,550**	**$ 704,964**
Current liabilities		
Accounts payable	$ 5,584	$ 10,984
Accrued expenses and other current liabilities	28,427	30,571
Current portion of contingent consideration	26,708	25,982
Current portion of operating lease liabilities	7,888	8,382
Total current liabilities	**68,607**	**75,919**
Non-current liabilities		
Long-term debt	101,431	102,791
Liability from royalty financing agreement	383,711	—
Operating lease liabilities, net of current portion	28,977	31,719
Contingent consideration, net of current portion	14,030	9,334
Deferred tax liability, net	4,917	8,257
Other non-current liabilities	1,093	935
Total non-current liabilities	**534,159**	**153,036**
Total liabilities	**602,766**	**228,955**
Shareholders' equity		
Total shareholders' equity	**268,784**	**476,009**
Total liabilities and shareholders' equity	**$ 871,550**	**$ 704,964**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended September 30,	
	2023	**2022**
	(in thousands, except share and per share amounts)	
Total revenues	**$ 1,407**	**$ 1,449**
Operating expenses:		
Cost of contract manufacturing revenues	(1,006)	(861)
Research and development expenses	(65,400)	(48,068)
Selling, general and administrative expenses	(18,074)	(13,324)
Total operating expenses	**(84,480)**	**(62,253)**
Other income	1,424	1,485
Other expense	(228)	(199)
Loss from operations	**(81,877)**	**(59,518)**
Non-operating items, net	(7,763)	11,332
Loss before income tax (expense) / benefit	**$ (89,640)**	**$ (48,186)**
Income tax (expense) / benefit	69	329
Net loss	**$ (89,571)**	**$ (47,857)**
Basic and diluted net loss per ordinary share	$ (1.88)	$ (1.02)
Weighted average shares used in computing basic and diluted net loss per ordinary share	47,770,101	46,772,430