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

August 8, 2022

~ Announced 12-month data on the lower-dose cohort of AMT-130 in Huntington's disease showed the investigative gene therapy was generally well tolerated at this dose with a mean reduction of 53.8% of mutant Huntingtin protein (mHTT) observed in cerebral spinal fluid (CSF) ~

~ Announced postponement of AMT-130 higher-dose procedures due to recent suspected unexpected severe adverse reactions at this dose; Lower-dose procedures are not affected and no impact is expected on anticipated data readouts in 2023 ~

~ Advancing regulatory reviews of the U.S. and European marketing applications for etranacogene dezaparvovec in hemophilia B ~

~ Investor conference call and webcast today at 8:30 a.m. ET ~

LEXINGTON, Mass. and AMSTERDAM, Aug. 08, 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 second quarter of 2022 and highlighted recent progress across its business.

"We continued to make strong progress across all of our programs highlighted by encouraging data from the lower-dose cohort in our Phase I/II study of AMT-130 and the advancement of the U.S. and European regulatory reviews for the marketing applications for etranacogene dezaparvovec in hemophilia B," stated Matt Kapusta, chief executive officer at uniQure. "Our manufacturing, CMC and Quality teams are working tirelessly to produce commercial launch supplies and also to ensure successful pre-approval site inspections, which are expected to be completed in the third quarter of this year. We also are very pleased to have recently received notification that GMP certification will be provided to our Lexington manufacturing facility by the European authorities."

"In July, we reported to the health authorities suspected unexpected severe adverse reactions in three of the 14 patients treated with the higher dose of AMT-130," he continued. "While these patients have fully or substantially recovered, and no clear root cause has yet been identified, we believe it's prudent to temporarily delay additional higher-dose procedures until we put additional monitoring and treatment plans in place and complete our safety review early in the fourth quarter of 2022. The delay is not expected to impact any future lower-dose procedures, as no serious adverse events related to AMT-130 have been reported in any of the additional 12 patients treated at the lower dose, and no impact is expected on the timing of previously guided data read-outs in 2023, including the presentation of one to two-year follow-up on both low and high-dose cohorts in the second quarter of 2023."

Recent Updates

- *AMT-061 for the treatment of hemophilia B*
    - In May 2022, the Company's global commercialization partner, CSL Behring, announced that the BLA for etranacogene dezaparvovec was accepted by the Food and Drug Administration (FDA) for priority review. This follows the MAA submission by CSL Behring which was validated by the EMA in March 2022.
    - 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 now switch to a standard review procedure. In the United States, the BLA remains under Priority Review at this time. In accordance with the Company's commercialization and license agreement, CSL Behring is solely responsible for all regulatory activities, including filings and agency interactions associated with etranacogene dezaparvovec.
    - In July 2022, following a comprehensive multi-day facility inspection, the European Medicines Agency notified the Company that Good Manufacturing Practice (GMP) certification can be issued for the Company's Lexington manufacturing site to produce commercial supply of etranacogene dezaparvovec.
- *AMT-130 for the treatment of Huntington's disease*
    - In June 2022, initial 12-month observations were announced on the lower-dose cohort of the double-blinded and randomized U.S. Phase I/II study of AMT-130 for the treatment of early-stage Huntington's disease.
        - At 12 months of follow-up on these patients, 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 three control patients with

evaluable data, mean levels of mHTT showed an increase compared to baseline at one, three, six and nine months of follow-up, and decreased by 16.8% 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. At 12 months, mean NfL showed an 8% increase compared to baseline. Two of the six treated patients were at or below baseline at 12 months of follow-up, with an additional patient below baseline at 15 and 18 months of follow-up.

- In mid-July, the Company reported to the appropriate regulatory agencies suspected unexpected severe adverse reactions (SUSARs) in two patients that were treated with the higher-dose of AMT-130 at a single clinical site in the European Phase Ib/II study. Both patients presented with localized inflammatory responses and other related symptoms approximately one to two weeks after their procedures. A third patient, who had previously been treated with the higher-dose of AMT-130 in the U.S. during March 2022, experienced severe headache and other related symptoms soon after AMT-130 administration that was initially deemed by the investigator as not related to AMT-130 but related to the procedure. Upon further review and discussion with the clinical trial's independent Data Safety Monitoring Board (DSMB) following the events observed in the European study, the Company has reclassified and reported the patient reaction in the U.S. trial as a SUSAR. All three patients have fully or substantially recovered after treatment and have been released from the hospital.

  - At this time, the DSMB does not view these findings as a dose-limiting toxicity. The DSMB has recommended temporarily delaying higher-dose enrollment pending a safety review, which is expected to take place early in the fourth quarter of 2022. The DSMB is permitting continued enrollment at the lower-dose of AMT-130, and the Company expects to begin crossing over control patients from the U.S. study at this dose in the third quarter of 2022.

  - No impact is expected on the timing of previously guided data read-outs in 2023. In the U.S. Phase I/II study, all 26 patients in the first two dose cohorts have been enrolled, and the Company continues to expect to present one to two-year follow up data in the second quarter of 2023. In the European Phase Ib/II study, the six-patient lower-dose cohort is fully enrolled and the Company continues to expect to present one-year follow-up data in 2023. Four of nine patients in the European study have been enrolled in the higher-dose cohort. To date, a total of 36 patients have been enrolled across the two clinical trials including 10 control patients and 26 patients treated with AMT-130, of which 14 patients received the higher-dose and 12 patients received the lower-dose.

- *Progress towards 2023 INDs*

  - *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* – The Company expects to initiate a GLP toxicology study of AMT-191 in non-human primates in the second half of 2022, which is expected to support an IND submission in 2023.

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

  - As of June 30, 2022, the Company had cash and cash equivalents of $500.5 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

- Citi's 17 th Annual BioPharma Conference, September 7 – 8, 2022
- Wells Fargo 2022 Healthcare Conference, September 7 – 9, 2022
- Wainwright 24th Global Investment Conference, September 12 – 14th, 2022 (virtual attendance)
- Cantor Fitzgerald Cell & Gene Therapy Conference, September 15, 2022
- Jefferies Cell and Genetic Medicines Summit, September 29 - 30, 2022
- Chardan 6th Annual Genetic Medicines Conference, October 3 - 4, 2022

Financial Highlights

Cash position: As of June 30, 2022, the Company held cash and cash equivalents of $500.5 million, compared to $556.3 million as of December 31, 2021.

Revenues: Collaboration revenue for the three months ended June 30, 2022, was $0.5 million, compared to collaboration revenue of $1.5 million for

the same period in 2021. Revenue for 2021 during this period also included license revenue of $462.4 million from selling the exclusive global rights to etranacogene dezaparvovec to CSL Behring and nil in the same period 2022.

Cost of contract revenues: Cost of contract revenues for the three months ended June 30, 2022 was nil compared to $23.2 million for the same period in 2021. Costs incurred in 2021 are associated with recognized license revenue.

Cost of contract manufacturing: Cost of contract manufacturing for the three months ended June 30, 2022, was $0.8 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 $46.2 million for the three months ended June 30, 2022, compared to $32.8 million during the same period in 2021. The increase was related to the preclinical development of temporal lobe epilepsy (AMT-260) advancing the clinical development of the Company's Huntington's disease gene therapy program, and recruitment of personnel to support the development of product candidates.

SG&A expenses: Selling, general and administrative expenses were $12.5 million for the three months ended June 30, 2022, compared to $17.3 million during the same period in 2021. The reduction was primarily related to incurring financial advisory fees to close the CSL Behring transaction in 2021 with no such fees in 2022.

Other non-operating Items, net:
Other non-operating income, net was income of $16.7 million for the three months ended June 30, 2022, compared to other non-operating income, net of $4.7 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 $12.8 million ($19.4 million recorded in the current period compared to $6.6 million for the same period in 2021).

Net (loss) / income:
The net loss for the three months ended June 30, 2022, was $39.1 million, or $0.84 basic and diluted loss per ordinary share, compared to $399.5 million net income for the same period in 2021, or $8.68 basic net income per ordinary share and $8.51 diluted net income per ordinary share.

Investor Conference Call and Webcast Information

uniQure management will host an investor conference call and webcast today, Monday, August 8, 2022, at 8:30 a.m. ET. The conference call may be accessed via pre-registering here. Participants will then receive a dial-in number and personal PIN. Research analysts who wish to ask a question should do so through the pre-registered conference call number. If you are joining the conference call, please dial-in 15 minutes before the start time. The webcast of the conference call also may be accessed here, or through the Investors & Newsroom section of the uniQure website. Following the live webcast, a replay of the call will be available on our website for several weeks.

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," "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, whether our announced delay of the AMT-130 high-dose procedures will be temporary, whether the delay will impact any future low-dose procedures, whether any control patients in the low-dose cohort of the AMT-130 clinical trial study will receive treatment in the third quarter of 2022 or ever, whether we will announce any data readouts for our AMT-130 clinical trial in 2023, whether our pre-approval site inspections for AMT-061 will be completed in the third quarter of this year or will be successful, whether we will ultimately receive GMP certification for our Lexington manufacturing facility by the European authorities or other regulatory authorities, whether we will submit an IND in for AMT-260 for the treatment of refractory temporal lobe epilepsy (rTLE) in 2023 or ever, whether we will initiate a GLP toxicology study of AMT-191 for Fabry disease in non-human primates in the second half of 2022 or submit an IND in 2023, and whether our cash and cash equivalents will fund operations into the first half of 2025. 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 and the pending safety review, 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 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.

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UNAUDITED CONSOLIDATED BALANCE SHEETS

	June 30, 2022	December 31, 2021
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 500,524	$ 556,256
Accounts receivable and contract asset	3,119	58,768
Inventories	2,949	-
Prepaid expenses	15,126	10,540
Other current assets and receivables	1,581	2,675
Total current assets	**523,299**	**628,239**
Non-current assets		
Property, plant and equipment, net	45,984	43,505
Operating lease right-of-use assets	28,482	25,573
Intangible assets, net	57,450	62,686
Goodwill	24,976	27,633
Deferred tax assets, net	15,046	15,647
Other non-current assets	5,974	5,897
Total non-current assets	**177,912**	**180,941**
Total assets	**$ 701,211**	**$ 809,180**
Current liabilities		
Accounts payable	$ 10,028	$ 2,502
Accrued expenses and other current liabilities	23,047	28,487
Current portion of contingent consideration	8,681	-
Current portion of operating lease liabilities	6,505	5,774
Total current liabilities	**48,261**	**36,763**
Non-current liabilities		
Long-term debt	101,890	100,963
Contingent consideration, net of current portion	20,405	29,542
Operating lease liabilities, net of current portion	30,721	28,987
Deferred tax liability, net	9,953	12,913
Other non-current liabilities	3,493	4,236
Total non-current liabilities	**166,462**	**176,641**
Total liabilities	**214,723**	**213,404**
Shareholders' equity		
Total shareholders' equity	**486,488**	**595,776**
Total liabilities and shareholders' equity	**$ 701,211**	**$ 809,180**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended June 30,	
	2022	2021
	(in thousands, except share and per share amounts)	
Total revenues	**$ 497**	**$ 463,868**
Operating expenses:		
Cost of contract revenues	**-**	(23,178)
Cost of contract manufacturing	(832)	-
Research and development expenses	(46,192)	(32,747)
Selling, general and administrative expenses	(12,491)	(17,299)
Total operating expenses	**(59,515)**	**(73,224)**
Other income	3,186	7,590
Other expense	(229)	(226)
(Loss) / income from operations	**(56,061)**	**398,008**

Non-operating items, net	16,682	4,718
(Loss) / income before income tax benefit / (expense)	**$ (39,379)**	**$ 402,726**
Income tax benefit / (expense)	318	(3,258)
Net (loss) / income	**$ (39,061)**	**$ 399,468**
Earnings per ordinary share - basic		
Basic net (loss) / income per ordinary share	$ (0.84)	$ 8.68
Earnings per ordinary share - diluted		
Diluted net (loss) / income per ordinary share	$ (0.84)	$ 8.51
Weighted average shares - basic	46,668,554	46,037,900
Weighted average shares - diluted	46,668,554	46,929,870

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