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

May 2, 2022

~ Partner CSL Behring submitted Marketing Authorization Application (MAA) in Europe for etranacogene dezaparvovec in hemophilia B; MAA validated by the European Medicines Agency (EMA) and granted accelerated assessment ~

~ Received $55 million in milestone payments associated with global submissions of marketing and license applications ~

~ Completed patient enrollment of higher dose cohort of U.S. Phase I/II study of AMT-130 and lower dose cohort of European Phase Ib/II study; total of 32 patient procedures completed to date ~

~ One year safety and biomarker data from all 10 patients in lower dose cohort of U.S. Phase I/II study of AMT-130 in Huntington's disease, including mHTT and NfL biomarkers, expected in second quarter 2022 ~

~ Multiple preclinical data presentations to be featured at American Society of Gene and Cell Therapy (ASGCT) Hybrid Congress ~

~ Appointed Erin Boyer as Chief People and Culture Officer and Richard Porter, previously founder and CEO of Corlieve as Chief Business Officer ~

LEXINGTON, Mass. and AMSTERDAM, May 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 first quarter of 2022 and highlighted recent progress across its business.

"uniQure is off to a strong start in 2022, with important advancements across our key programs and earlier-stage research activities, as well as the strengthening of our executive leadership team," stated Matt Kapusta, chief executive officer at uniQure. "In the first quarter of the year, our partner CSL Behring made important progress with global regulatory submissions for etranacogene dezaparvovec, including the granting of accelerated assessment by the EMA in Europe."

"We also made significant advancements enrolling patients in our Phase I/II studies for AMT-130 in Huntington's disease," he added. "To date, we have completed 32 patient procedures across both studies and look forward to presenting in the second quarter of 2022 one-year safety and biomarker data from the 10-patient lower dose cohort of the U.S. randomized, controlled Phase I/II trial. We continue to advance our pipeline of earlier-stage gene therapy candidates and are pleased to have recently initiated a dose-ranging IND-enabling study for AMT-260 in refractory temporal lobe epilepsy. I am very proud of the uniQure team's unwavering commitment to progressing our ambitious goals and achieving our mission of delivering the promise of gene therapies to patients."

Recent Key Accomplishments

- *Advancing the late-stage development of etranacogene dezaparvovec (AMT-061) for the treatment of hemophilia B*
    - In March 2022, the Company's global commercialization partner, CSL Behring, announced that the MAA for etranacogene dezaparvovec was validated by EMA and granted accelerated assessment. 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 either etranacogene dezaparvovec or any required AAV5 neutralizing antibody companion diagnostic.
    - In March and April 2022, the Company received $55 million in milestone payments related to CSL Behring's submissions of marketing and license applications for etranacogene dezaparvovec.
    - In December 2021, the Company and CSL Behring announced the achievement of primary and secondary endpoints from the HOPE-B pivotal trial of etranacogene dezaparvovec in severe and moderately severe hemophilia B patients. The primary endpoint of non-inferiority in annualized bleeding rate (ABR) 18-months following administration compared to baseline Factor IX (FIX) prophylactic therapy was achieved, as was a secondary superiority endpoint on ABR.
- *Advancing the clinical development of AMT-130 for the treatment of Huntington's disease*
    - In March 2022, the Company announced the completion of enrollment of all 26 patients in the first two cohorts of its randomized, double-blinded, Phase I/II clinical trial of AMT-130 for the treatment of early-stage Huntington's disease. The ongoing Phase I/II clinical trial of AMT-130 is a randomized, sham controlled, double-blinded study to

explore the safety, tolerability, and proof of concept of AMT-130 in patients with early manifest Huntington's disease. A third cohort exploring the use of an alternative stereotactic navigation system to simplify placement of infusion catheters in up to 18 additional randomized patients is expected to commence enrollment in the second half of 2022.

- Also in March 2022, the Company completed enrollment of the lower-dose cohort of six patients in the European Phase Ib/II study of AMT-130. The open-label study will enroll 15 patients with early manifest Huntington's disease across the same doses being explored in the U.S study. Together with the ongoing U.S. study, the European study is intended to establish safety, proof of concept, and the optimal dose of AMT-130 to take forward into Phase III development, or into an adaptive confirmatory study should an accelerated registration pathway be feasible. In total, 59 patients are expected to be enrolled in the U.S. and European Phase I/II trials of AMT-130.

- In December 2021, initial observations were announced on the first four patients in the low-dose cohort of the double-blinded and randomized U.S. Phase I/II study of AMT-130. The investigational gene therapy was generally well tolerated with no serious adverse events related to treatment. NfL increased as expected immediately following the AMT-130 surgical procedure and returned to baseline in the two treated patients. NfL remained relatively constant in the two untreated control patients. Structural magnetic resonance imaging did not reveal any clinically meaningful safety findings in either treated or control patients at one year of follow-up.

    - During the second quarter of 2022, the Company expects to provide a clinical update on all 10 patients followed for twelve months in the lower dose cohort, including safety, mHTT and NfL biomarker data. Volumetric MRI and functional data is expected to be available during the first half of 2023, after all patients in the two dose cohorts are unblinded.

- *Advancing our research pipeline into the clinic and the Company's proprietary technologies*

    - *Progress towards 2023 INDs:*

        - *Refractory Temporal Lobe Epilepsy (rTLE)* – In March 2022, the Company initiated an IND-enabling dose-ranging study of AMT-260 and plans to initiate a GLP toxicity study in non-human primates in the second half of 2022 to support an IND submission in 2023.

        - *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.

    - The Company today announced its significant scientific presence at the upcoming American Society of Gene and Cell Therapy (ASGCT) Hybrid Congress, to be held May 16-19, 2022 in Washington, DC. In total, six data presentations will be delivered by the Company's researchers and scientists, including five posters, and one oral presentation. These presentations will highlight the Company's research and technology capabilities and feature some new preclinical findings on the company's gene therapy candidates for Huntington's disease, refractory temporal lobe epilepsy, Fabry disease, Parkinson's disease, Amyotrophic lateral sclerosis (ALS) and Alzheimer's disease.

- *Enhancements to the Leadership Team*

    - In March 2022, Richard Porter was appointed Chief Business Officer, responsible for global business development, corporate strategic planning, market access and commercial planning. Dr. Porter joined the Company following the acquisition of Corlieve Therapeutics in July 2021. Dr. Porter brings over 25 years of R&D experience to the Company, having previously served as the COO of Therachon, until its acquisition by Pfizer. Previously, he was at Roche in positions of increasing responsibility, most recently as the Global Head of Scientific Business Strategy and Operations for Neuroscience Ophthalmology and Rare Diseases. Dr. Porter holds a Ph.D. in neuropharmacology from Southampton University (UK) and conducted his postdoctoral training at the Strong Memorial Hospital, University of Rochester, NY (USA) and the University of Oxford (UK).

    - In April 2022, Erin Boyer joined the leadership team as Chief People and Culture Officer, responsible for leading the global Human Resources team. Ms. Boyer brings more than 20 years of Human Resources experience, including extensive leadership experience at biotech companies. She joins from Epizyme in Cambridge, Mass., where she served as Chief People and Culture Officer since 2020. Prior to Epizyme, she served as VP of Human Resources at Obsidian Therapeutics, and before that spent five years at Alnylam Pharmaceuticals in roles of increasing responsibility, including Global Head of Talent Strategy and Planning.

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

    - As of March 31, 2022, the Company had cash and cash equivalents of $524.9 million, which included the collection of $20 million of the $55 million in milestone payments owed by CSL Behring. The remaining $35 million in milestone payments were collected by the Company in April 2022. The Company expects that its cash and cash equivalents will fund operations into the first half of 2025 assuming the achievement of $175.0 million of first commercial sales milestones under the CSL Behring Agreement.

Upcoming Investor Events

- RBC Global Healthcare Conference, May 17-18, 2022
- uniQure Annual General Meeting, June 14, 2022

Financial Highlights

Cash Position: As of March 31, 2022, the Company held cash and cash equivalents of $524.9 million, compared to $556.3 million as of December 31, 2021.

Revenues: Revenue for the three months ended March 31, 2022, was $1.8 million, compared to $0.5 million during the same period in 2021. The increase is a result of collaboration revenue recognized as a result of closing the CSL Behring Agreement in May 2021.

R&D Expenses: Research and development expenses were $45.0 million for the three months ended March 31, 2022, compared to $32.7 million during the same period in 2021. The change was primarily related to recruitment of personnel to support the development of product candidates, advancing the clinical development of the Company's Huntington's disease gene therapy program, increased activities associated with preclinical product candidates, and an increase of the fair value of the liability recorded for contingent consideration owed in relation to the acquisition of Corlieve Therapeutics SAS.

SG&A Expenses: Selling, general and administrative expenses were $11.0 million for the three months ended March 31, 2022, compared to $12.4 million during the same period in 2021. The reduction was primarily related to incurring one-off professional 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 $6.8 million for the three months ended March 31, 2022, compared to other non-operating income, net of $3.1 million for the same period in 2021. The increase in other non-operating income was primarily related to an increase in net foreign currency gains of $8.6 million in the current period compared to $4.6 million the same period in 2021, partially offset by an increase in interest expense related to the loan facility with Hercules Capital, Inc. of $1.0 million as a result of the Company drawing down an additional $35.0 million in January 2021 and a further $30.0 million in December 2021.

Net Loss:
The net loss for the three months ended March 31, 2022, was $46.7 million, or $1.00 loss per ordinary share, compared to $41.6 million, or $0.91 loss per ordinary share, during the same period in 2021.

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 we are able to enroll up to 18 additional patients or otherwise commence enrollment at any time for our third cohort exploring the use of an alternative stereotactic navigation system to simplify placement of infusion catheters, whether we are able to enroll up to 59 patients in the U.S. and European Phase I/II trials of AMT-130, whether we are able to disclose volumetric MRI and functional data during the first half of 2023 or ever, and whether we are able to initiate a GLP toxicity study in non-human primates in the second half of 2022 or submit an associated investigational new drug application in 2023. 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 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

	March 31, 2022	December 31, 2021
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 524,886	$ 556,256
Accounts receivable and contract asset	38,644	58,768
Prepaid expenses	12,422	10,540
Other current assets and receivables	2,260	2,675
Total current assets	**578,212**	**628,239**
Non-current assets		
Property, plant and equipment, net	44,918	43,505
Operating lease right-of-use assets	24,941	25,573
Intangible assets, net	61,135	62,686
Goodwill	26,565	27,633
Deferred tax assets, net	15,442	15,647
Other non-current assets	5,937	5,897
Total non-current assets	**178,938**	**180,941**
Total assets	**$ 757,150**	**$ 809,180**
Current liabilities		
Accounts payable	$ 9,920	$ 2,502
Accrued expenses and other current liabilities	19,945	28,487
Current portion of contingent consideration	9,081	-
Current portion of operating lease liabilities	5,754	5,774
Total current liabilities	**44,700**	**36,763**
Non-current liabilities		
Long-term debt	101,421	100,963
Operating lease liabilities, net of current portion	28,232	28,987
Contingent consideration, net of current portion	22,059	29,542
Deferred tax liability, net	11,298	12,913
Other non-current liabilities	3,439	4,236
Total non-current liabilities	**166,449**	**176,641**
Total liabilities	**211,149**	**213,404**
Shareholders' equity		
Total shareholders' equity	**546,001**	**595,776**
Total liabilities and shareholders' equity	**$ 757,150**	**$ 809,180**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended March 31,	
	2022	2021
	(in thousands, except share and per share amounts)	
Total revenues	**$ 1,792**	**$ 454**
Operating expenses:		
Research and development expenses	(45,003)	(32,656)
Selling, general and administrative expenses	(10,987)	(12,375)
Total operating expenses	**(55,990)**	**(45,031)**

Other income	311	352
Other expense	(193)	(233)
Loss from operations	**(54,080)**	**(44,458)**
Non-operating items, net	6,786	3,115
Loss before income tax benefit / (expense)	**$ (47,294)**	**$ (41,343)**
Income tax benefit / (expense)	616	(213)
Net loss	**$ (46,678)**	**$ (41,556)**
Basic and diluted net loss per ordinary share	$ (1.00)	$ (0.91)
Weighted average shares used in computing basic and diluted net loss per ordinary share	46,599,114	45,468,485