uniQure Announces 2021 Financial Results and Highlights Recent Company Progress

February 25, 2022

~ Data from largest gene therapy study in hemophilia B showed sustained therapeutic effect at 18-months and statistical superiority in reducing annualized bleeding rate compared to baseline FIX prophylactic therapy; Submissions of marketing applications on track for the first half of 2022 ~

~ Data from first four patients in ongoing U.S. Phase I/II clinical trial in Huntington's Disease showed AMT-130 was well tolerated, with no significant safety concerns observed ~

~ Additional clinical data from all patients in lower dose cohort of U.S. Phase I/II study of AMT-130, including mHTT and NfL biomarkers, expected to be presented in second quarter of 2022 ~

~ Initiated enrollment of European Phase Ib/II study of AMT-130 with first two patients dosed ~

~ Refractory temporal lobe epilepsy and Fabry programs advancing into IND-enabling studies in 2022 ~

~ Ended 2021 in strong financial position with $556 million in cash ~

LEXINGTON, Mass. and AMSTERDAM, Feb. 25, 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 2021 and highlighted recent progress across its business.

"We made tremendous progress in 2021 across all our strategic imperatives, including advancing our clinical-stage programs, expanding our research pipeline, and preparing for the submission of marketing applications planned for *etranacogene dezaparvovec* during the first half of 2022," stated Matt Kapusta, chief executive officer at uniQure. "With positive data from the HOPE-B pivotal study we look forward to working closely with our partner, CSL Behring to bring this potentially life-changing gene therapy to patients living with hemophilia B."

"During 2022, we are also keenly focused on maintaining our strong momentum in enrolling two ongoing Phase I/II studies in Huntington's disease. Importantly, we look forward to sharing additional safety and biomarker data from all ten patients in the lower dose cohort, including mutant HTT protein (mHTT) and neurofilament light chain (NfL), during the second quarter of 2022," he added. "We also are poised to advance our gene therapy product candidates for Fabry disease and refractory temporal lobe epilepsy into IND-enabling toxicology studies, and we expect to initiate at least two new gene therapy programs targeting the liver and CNS during the year. As we continue to advance and grow our pipeline, we are also expanding our manufacturing footprint with a second cGMP facility in Amsterdam, which we expect will come online in 2022."

Recent Key Accomplishments

- *Advancing the late-stage development of etranacogene dezaparvovec (AMT-061) for the treatment of hemophilia B*
  - 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.
  - Data from the HOPE-B trial demonstrated that *etranacogene dezaparvovec* produced mean FIX activity of 39.0 percent of normal at six months and 36.9 percent of normal at 18 months post infusion. After the six-month lead-in period post-infusion, the adjusted annualized bleeding rate (ABR) (1.51) for all bleeds was reduced by 64 percent ($p=0.0002$) and all FIX-treated bleeds was reduced by 77 percent (3.65 to 0.83; $p<0.0001$) over months seven to 18. In addition, 98 percent of subjects treated with a full dose of *etranacogene dezaparvovec* discontinued use of prophylaxis, with an overall 97 percent reduction in mean unadjusted annualized FIX consumption of 257,338.8 IU/yr/participant to 8,486.6 IU/yr/participant (from lead-in period to months 13-18). ABR for all bleeds after stable FIX expression, assessed at 18 months, was 1.51 compared with the ABR of 4.19 for the lead-in period of at least six months ($p=0.0002$). ABR for investigator-adjudicated FIX-treated bleeds was 0.83 compared with lead-in ABR of 3.65 ($p<0.0001$).
  - The Company and CSL Behring are making significant progress on the preparation of marketing applications for *etranacogene dezaparvovec,* which are expected to be submitted by CSL Behring in the U.S. and the European Union during the first half of 2022. As part of these efforts, the Company successfully competed validation of its commercial manufacturing process in December 2021.
- *Advancing the clinical development of AMT-130 for the treatment of Huntington's disease*
  - 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. AMT-130 was generally well tolerated with no serious adverse events related to AMT-130. 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 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.
    - Patient enrollment in a third cohort is expected to begin in the second half of 2022 to explore the use of alternative stereotactic navigation systems to simplify placement of catheters for infusions of AMT-130. This cohort is planned to include up to 18 additional randomized patients, approximately 12 of whom will receive the higher dose of 6x1013 vg.
    - In February 2022, the first two patients were enrolled in a European open-label Phase Ib/II study of AMT-130. This 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 a 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 1/II trials of AMT-130.
- *Advancing our research pipeline into the clinic*
    - *Refractory Temporal Lobe Epilepsy (TLE)* - The Company expects to initiate a GLP toxicology study in non-human primates in the second half of 2022, which is expected to support an IND submission in 2023. AMT-260 employs a miRNA silencing technology to suppress aberrantly expressed kainite receptors in the hippocampus of patients with TLE. TLE represents a large unmet clinical need affecting approximately 1.3 million people in the U.S. and Europe alone, of which approximately 800,000 patients are unable to adequately control acute seizures with currently approved anti-epileptic therapies.
    - *Fabry disease* – The Company expects to initiate a GLP toxicology study of AMT-191 by the middle of 2022, which is expected to support an IND submission in 2023. AMT-191 is a one-time administered AAV5 gene therapy incorporating an α-galactosidase A (GLA) transgene. In preclinical studies, AMT-191 has shown the potential for cross correction in the kidney and heart, and potentially into the brain. It also offers the possibility for re-dosing as shown in a study of non-human primates.
    - With the goal of prioritizing resources and accelerating the advancement of our programs in refractory TLE and Fabry disease into the clinic, the Company has deprioritized the preclinical development of AMT-150 in spinocerebellar ataxia type 3.
- *Expanding our manufacturing capabilities*
    - The Company recently completed the expansion of its Amsterdam facility providing additional laboratories for new research and development activities, as well as the construction of a cleanroom designed for the manufacturing of cGMP materials at a 500-liter scale. This expansion now allows for increased speed and agility across product development.
- *Strong cash position to advance the Company's programs*
    - As of December 31, 2021, the Company had cash and cash equivalents of $556.3 million. The Company expects that its cash and cash equivalents will be sufficient to fund operations into the first half of 2025 assuming achievement of the BLA and MAA submission as well as first commercial sales milestones under the CSL Behring Agreement.

Upcoming Investor Events (each to be conducted virtually)

- Cowen 42nd Annual Health Care Conference, March 7-9, 2022
- Stifel 2022 CNS Days, March 28-29, 2022
- Guggenheim Genomic Medicines and Rare Disease Day, March 31 & April 1, 2022

Financial Highlights

Cash Position: As of December 31, 2021, the Company held cash and cash equivalents of $556.3 million, compared to $244.9 million as of December 31, 2020. Upon the CSL Behring Agreement becoming fully effective on May 6, 2021, the Company received $462.4 million of payments. In January and December 2021, the Company and Hercules amended the debt facility agreement, under which the Company drew down an additional $35.0 million in January 2021 and a further $30.0 million for a total of $100.0 million outstanding under the facility as of December 31, 2021. The Company also extended the maturity from June 2023 to December 2025. In March and April 2021, the Company sold 921,730 ordinary shares for gross proceeds of approximately $29.6 million under an Open Market Sale Agreement with SVB Leerink LLC. In July 2021, the Company paid a net EUR 42.1 million ($49.9 million) related to its acquisition of Corlieve.

Revenues: Revenue for the year ended December 31, 2021, was $524.0 million, compared to $37.5 million during the same period in 2020. The increase is a result of $462.4 million of license revenue recognized upon closing of the CSL Behring transaction in May 2021 as well as $55.0 million of license revenue related to milestone payments the Company expects to collect following the submissions of a biologics license application ("BLA") and market authorization application ("MAA") by CSL Behring in the first half of 2022. In 2020, the Company had recognized $27.8 million in non-cash license revenue as of the December 1, 2020, effective date of the amended Bristol-Myers Squibb collaboration and license agreement as well as revenue of $4.4 million in December 2020 following achievement of a research milestone for one of the four Collaboration Targets.

R&D Expenses: Research and development expenses were $143.5 million for the year ended December 31, 2021, compared to $122.4 million during the same period in 2020. 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 as well as increased activities associated with preclinical product candidates, and an increase from fair value changes related to liability recorded for contingent consideration owed in relation to the acquisition of Corlieve.

SG&A Expenses: Selling, general and administrative expenses were $56.3 million for the year ended December 31, 2021, compared to $42.6 million during the same period in 2020. The change was primarily related to recruitment of personnel, increased share-based compensation expenses, and as a result of financial advisory payments made in relation to our licensing transaction with CSL Behring.

Other Income, net: Other income, net was $11.4 million for the year ended December 31, 2021, compared to other income, net of $2.0 million during the same period in 2020. The increase in other income, net was primarily related to the receipts of employee retention credits under the U.S. CARES Act, income related to payments received from European authorities to subsidize research and development efforts in the Netherlands and income related to a settlement agreement that the Company and VectorY B.V. entered into in April 2021.

Other Non-operating Items, net:
Other non-operating income, net was income of $22.2 million for the year ended December 31, 2021, compared to other non-operating loss of $16.0 million for the same period in 2020. The increase in other non-operating income was primarily related to net foreign currency gains in the current period compared to net foreign currency losses in the same period in 2020.

Net Income:
The net income for the year ended December 31, 2021, was $329.6 million, or $7.17 basic net income per ordinary share and $7.04 diluted net income per ordinary share, compared to a loss of $125.0 million, or $2.81 basic and diluted loss per ordinary share during the same period in 2020.

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 re not limited to, whether we will bring etranacogene dezaparvovec to patients, whether we will share mutant HTT protein (mHTT) and neurofilament light chain (NfL) or other safety and biomarker data from any patients in our AMT-130 clinical trials, during the second quarter of 2022, the first half of 2023 or ever, whether we will be able to advance our gene therapy product candidates for Fabry disease or refractory temporal lobe epilepsy into IND-enabling toxicology studies, whether we will initiate any new gene therapy programs targeting the liver or CNS during the year, whether our second cGMP facility in Amsterdam will come online in 2022 or ever, whether marketing applications for etranacogene dezaparvovec, will be submitted in the U.S. or the European Union during the first half of 2022 or ever.. 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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uniQure N.V.
UNAUDITED CONSOLIDATED BALANCE SHEETS

	December 31, 2021	**December 31, 2020**
	(in thousands, except share and per share amounts)	
Current assets		

Cash and cash equivalents	$	556,256	$	244,932
Accounts receivable and contract asset		58,768		6,618
Prepaid expenses		10,540		4,337
Other current assets and receivables		2,675		3,024
Total current assets		**628,239**		**258,911**
Non-current assets				
Property, plant and equipment, net		43,505		32,328
Operating lease right-of-use assets		25,573		26,086
Intangible assets, net		62,686		3,361
Goodwill		27,633		542
Deferred tax assets, net		15,647		16,419
Other non-current assets		5,897		2,748
Total non-current assets		**180,941**		**81,484**
Total assets	**$**	**809,180**	**$**	**340,395**
Current liabilities				
Accounts payable	$	2,502	$	3,772
Accrued expenses and other current liabilities		28,487		18,038
Current portion of operating lease liabilities		5,774		5,524
Total current liabilities		**36,763**		**27,334**
Non-current liabilities				
Long-term debt		100,963		35,617
Operating lease liabilities, net of current portion		28,987		30,403
Contingent consideration		29,542		-
Deferred tax liability, net		12,913		-
Other non-current liabilities		4,236		3,136
Total non-current liabilities		**176,641**		**69,156**
Total liabilities		**213,404**		**96,490**
Shareholders' equity				
Total shareholders' equity		**595,776**		**243,905**
Total liabilities and shareholders' equity	**$**	**809,180**	**$**	**340,395**

uniQure N.V.
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Years ended December 31,		
	2021	**2020**	**2019**
	(in thousands, except share and per share amounts)		
Total revenues	**$ 524,002**	**$ 37,514**	**$ 7,281**
Operating expenses:			
Cost of contract revenues	(24,976)	-	-
Research and development expenses	(143,548)	(122,400)	(94,737)
Selling, general and administrative expenses	(56,290)	(42,580)	(33,544)
Total operating expenses	**(224,814)**	**(164,980)**	**(128,281)**
Other income	12,306	3,342	1,888
Other expense	(876)	(1,302)	(2,028)
Income / (loss) from operations	**310,618**	**(125,426)**	**(121,140)**
Non-operating items, net	22,188	(16,017)	(3,061)
Income / (loss) before income tax (expense) / income	**$ 332,806**	**$ (141,443)**	**$ (124,201)**
Income tax (expense) / income	(3,217)	16,419	-
Net income / (loss)	**$ 329,589**	**$ (125,024)**	**$ (124,201)**
Earnings per ordinary share - basic			
Basic net income / (loss) per ordinary share	$ 7.17	$ (2.81)	$ (3.11)
Earnings per ordinary share - diluted			

Diluted net income / (loss) per ordinary share	$ 7.04	$ (2.81)	$ (3.11)
Weighted average shares - basic	45,986,467	44,466,365	39,999,450
Weighted average shares - diluted	46,840,972	44,466,365	39,999,450