uniQure Announces 2022 Financial Results and Highlights Recent Company Progress

February 27, 2023

~ Achieved U.S. and European Commission marketing approvals of first and only gene therapy for adults with hemophilia B ~

~ Advancing development of AMT-130 for patients with Huntington's disease – clinical update from U.S. Phase I/II trial expected in second quarter 2023 ~

~Hosted virtual investor event featuring AMT-260 in refractory temporal lobe epilepsy - expected to enter clinical development in second half of 2023 ~

~Expanded pipeline with in-licensing of AMT-162 for SOD1 ALS - plans to initiate a Phase I/II clinical trial in the second half of 2023 ~

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Feb. 27, 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 2022 and highlighted recent progress across its business.

"We had a strong ending to 2022 with the historic approval of the world's first gene therapy for hemophilia B for which we led the multi-year research and clinical development, and through our partnership with CSL Behring, HEMGENIX® will now be available to patients in the United States and European Union," stated Matt Kapusta, chief executive officer of uniQure. "We are immensely proud of our efforts that led to this product approval, and of the innovation and leadership in genomic medicine that it represents. At the same time, we are working with urgency to advance AMT-130, the first AAV gene therapy being clinically investigated in Huntington's disease, and are on track to provide a clinical update from our U.S. Phase I/II study in the second quarter of 2023. We are also preparing for the initiation of two new Phase I/II studies in refractory temporal lobe epilepsy and SOD1-ALS in the second half of this year, and readying for the submission of an investigational new drug application in Fabry disease in 2023."

"Throughout this progress, we have established cutting-edge capabilities in the field of genetic medicines – from how we discover and develop our gene therapies, to how we successfully navigate complex clinical and regulatory pathways, to how we manufacture these novel gene therapies globally for clinical and commercial usage," said Mr. Kapusta. "We believe these capabilities position us well for continued growth and success as we look forward to developing more life-altering gene therapy products for patients in need."

Recent Updates

- *Preparing global commercial supply of HEMGENIX® (etranacogene dezaparvovec-drbl) for the treatment of hemophilia B in the United States and European Union*
    - In February 2023, the European Commission granted conditional marketing approval for HEMGENIX®, the first and only one-time gene therapy for the treatment of adults 18 years of age and older living with severe and moderately severe hemophilia B. This follows the November 2022 U.S. marketing approval of HEMGENIX® in adults living with hemophilia B.
    - CSL Behring licensed the exclusive global rights to etranacogene dezaparvovec from uniQure in May 2021 and is now solely responsible for the further development, registration, and commercialization of the therapy. uniQure is responsible for the global commercial supply of etranacogene dezaparvovec and manufactures the product at its cGMP facility in Lexington, MA.
    - uniQure is eligible to receive a $100.0 million payment from CSL Behring following the first sale of HEMGENIX® in the United States, as well as a $75.0 million payment from CSL Behring following the first product sale in one of five major European countries if prior to July 2, 2023.
- *Advancing AMT-130 for the treatment of Huntington's disease*

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). The 6 control patients in the higher-dose cohort will have the option to cross over to treatment if they meet the study's eligibility criteria. In the first quarter of 2023, a second control patient was crossed over to treatment and received the higher dose of AMT-130.

- The Company 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. The data update is expected to include safety and tolerability, biomarker, functional and imaging data across both dose cohorts.

- Patient enrollment in the European, open-label Phase I/II clinical trial of AMT-130 is expected to be complete in the first half of 2023. Fifteen patients across the two dose cohorts will be treated with AMT-130, including six patients receiving the lower dose and nine patients receiving the higher dose. Clinical data from the lower-dose cohort is expected to be presented in the second half of 2023.

- A third cohort of patients is expected to begin enrollment in the U.S. in the second half of 2023 to explore the feasibility of certain surgical adaptations aimed at enhancing procedure efficiency.

- *Expanding the Pipeline and Progress Towards Investigational New Drug (IND) Applications*

    - *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) amyotrophic lateral sclerosis (ALS), a rare, genetic form of ALS. The licensing of AMT-162 enhances the Company's CNS pipeline and is highly complementary to AMT-161, a gene therapy candidate for ALS caused by mutations in the c9orf72 gene, allowing the Company to potentially address most inherited forms of ALS. AMT-162 has been granted both Orphan Drug and Fast Track designations and has a cleared IND. The Company expects to initiate a Phase I/II clinical study of AMT-162 in the second half of 2023.

    - *AMT-260 for the treatment of refractory temporal lobe epilepsy (rTLE)* – In November, 2022, the Company hosted an investor research and development event that explained the unmet medical need in rTLE and the impact of disease on patients, along with preclinical data on AMT-260 and plans for its early stage clinical development. The Company expects to submit an investigational new drug application and initiate a Phase I/II clinical study of AMT-260 in the second half of 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.

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

    - As of December 31, 2022, the Company had cash and cash equivalents and investment securities of $392.8 million. The Company expects cash and cash equivalents and investment securities will fund operations into 2025 assuming the achievement of $100.0 million of a first commercial sale milestone for HEMGENIX® in the U.S. and into the first half of 2025 if the $75.0 million first commercial sale milestone for HEMGENIX® in any of the five contractually defined European countries is achieved prior to July 2, 2023.

Upcoming Investor Events

- Cowen 43rd Annual Health Care Conference, Monday, March 6, 2023, Boston
- Stifel Virtual 2023 CNS Days, Tuesday, March 28, 2023
- Guggenheim Virtual Healthcare Talks: Genomic Medicines and Rare Disease, Tuesday, April 4, 2023

Financial Highlights

Cash position: As of December 31, 2022, the Company held cash and cash equivalents and investment securities of $392.8 million, compared to $556.3 million as of December 31, 2021. As of December 31, 2022, the Company held investments in debt securities of $164.8 million, compared to nil as of December 31, 2021. The investments in debt securities have remaining maturities ranging from three to 14 months.

Revenues: Revenue for the year ended December 31, 2022 was $106.5 million, compared to $524.0 million in the same period in 2021. In 2022, the Company recognized $100.0 million of license revenue related to a milestone payment the Company expects to receive following the first sale of HEMGENIX in the U.S. In 2021, the Company recognized $462.4 million of license revenue upon closing of the CSL Behring transaction in May 2021 as well as $55.0 million of license revenue for milestone payments related to CSL Behring's BLA and MAA submissions in the first months of 2022.

R&D expenses: Research and development expenses were $197.6 million for the year ended December 31, 2022, compared to $143.5 million during the same period in 2021. The increase was primarily related to advancing the clinical development of the Company's Huntington's disease gene therapy program, the preclinical development of the temporal lobe epilepsy program (AMT-260), an increase in personnel and contractor related expenses to support the growth of the Company, and contractual payments owed to licensors upon FDA approval of HEMGENIX and a valid patent claim granted within the EU.

SG&A expenses: Selling, general and administrative expenses were $55.1 million for the year ended December 31, 2022, compared to $56.3 million during the same period in 2021. The increase in personnel and contractor related expenses to support the growth of the Company was offset by a reduction in professional and financial advisory fees we had incurred in 2021 related to the closing of the CSL Behring transaction.

Other non-operating items, net:
Other non-operating income, net was income of $14.9 million for the year ended December 31, 2022, compared to other non-operating income, net of $22.2 million for the same period in 2021. The decrease in other non-operating income, net was primarily related to a decrease in net foreign currency gains of $6.4 million, an increase of $3.8 million in interest expense related to the long-term debt with Hercules Capital, Inc. and a $2.9 million increase

in other non-operating gains as a result of releasing a financial liability.

Net loss:
The net loss for the year ended December 31, 2022, was $126.8 million, or $2.71 basic and diluted loss per ordinary share, compared to $329.6 million net income for the same period in 2021, or $7.17 basic net income per ordinary share and $7.04 diluted net income per ordinary share.

About HEMGENIX®

HEMGENIX® is a gene therapy that reduces the rate of abnormal bleeding in eligible people with hemophilia B by enabling the body to continuously produce factor IX, the deficient protein in hemophilia B. It uses AAV5, a non-infectious viral vector, called an adeno-associated virus (AAV). The AAV5 vector carries the Padua gene variant of Factor IX (FIX-Padua) to the target cells in the liver, generating factor IX proteins that are 5x-8x more active than normal. These genetic instructions remain in the target cells, but generally do not become a part of a person's own DNA. Once delivered, the new genetic instructions allow the cellular machinery to produce stable levels of factor IX.

HEMGENIX® is a registered trademark of CSL Behring.

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. The recent approvals of our gene therapy for hemophilia B – an historic achievement based on more than a decade of research and clinical development – represents 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 platform to advance a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory temporal lobe epilepsy, 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, statements about whether a third cohort of patients in our AMT-130 clinical trial for Huntington's Disease will begin enrollment in the U.S. in the second half of 2023, whether the Company will announce one to two-years of follow up data from the U.S. Phase I/II clinical study of AMT-130 in the second quarter of 2023, whether clinical data from the lower-dose cohort for AMT-130 is expected to be presented in the second half of 2023, whether the Company will submit an investigational new drug application for Fabry disease in 2023, whether the company will initiate a Phase I/II clinical study of AMT-162 for the treatment of ALS in the second half of 2023, and whether the Company will submit an investigational new drug application or initiate a Phase I/II clinical study of AMT-260 for rTLE in the second half of 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 regulatory approval and commercial launch of HEMGENIX®, 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 27, 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.

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UNAUDITED CONSOLIDATED BALANCE SHEETS

	December 31, 2022	December 31, 2021
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 228,012	$ 556,256
Current investment securities	124,831	—
Accounts receivable and contract asset	102,376	58,768
Inventories	6,924	—
Prepaid expenses	11,817	10,540
Other current assets and receivables	2,814	2,675
Total current assets	**476,774**	**628,239**
Non-current assets		
Property, plant and equipment, net	50,532	43,505

Non-current investment securities		39,984		—
Operating lease right-of-use assets		32,726		25,573
Intangible assets, net		58,778		62,686
Goodwill		25,581		27,633
Deferred tax assets, net		14,528		15,647
Other non-current assets		6,061		5,897
Total non-current assets		**228,190**		**180,941**
Total assets	**$**	**704,964**	**$**	**809,180**
Current liabilities				
Accounts payable	$	10,984	$	2,502
Accrued expenses and other current liabilities	$	30,571	$	28,487
Current portion of contingent consideration		25,982		—
Current portion of operating lease liabilities		8,382		5,774
Total current liabilities		**75,919**		**36,763**
Non-current liabilities				
Long-term debt		102,791		100,963
Operating lease liabilities, net of current portion		31,719		28,987
Contingent consideration, net of current portion		9,334		29,542
Deferred tax liability, net		8,257		12,913
Other non-current liabilities		935		4,236
Total non-current liabilities		**153,036**		**176,641**
Total liabilities		**228,955**		**213,404**
Shareholders' equity				
Total shareholders' equity		**476,009**		**595,776**
Total liabilities and shareholders' equity	**$**	**704,964**	**$**	**809,180**

Balancing check	-	-

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Years ended December 31,		
	2022	**2021**	**2020**
	(in thousands, except share and per share amounts)		
Total revenues	**$ 106,483**	**$ 524,002**	**$ 37,514**
Operating expenses:			
Cost of license revenues	(1,254)	(24,976)	-
Cost of contract manufacturing revenues	(2,089)	-	-
Research and development expenses	(197,591)	(143,548)	(122,400)
Selling, general and administrative expenses	(55,059)	(56,290)	(42,580)
Total operating expenses	**(255,993)**	**(224,814)**	**(164,980)**
Other income	7,171	12,306	3,342
Other expense	(820)	(876)	(1,302)
(Loss) / income from operations	**(143,159)**	**310,618**	**(125,426)**
Non-operating items, net	14,900	22,188	(16,017)
(Loss) / income before income tax benefit / (expense)	**$ (128,259)**	**$ 332,806**	**$ (141,443)**
Income tax benefit / (expense)	1,470	(3,217)	16,419
Net (loss) / income	**$ (126,789)**	**$ 329,589**	**$ (125,024)**
Earnings per ordinary share - basic			
Basic net (loss) / income per ordinary share	$ (2.71)	$ 7.17	$ (2.81)
Earnings per ordinary share - diluted			
Diluted net (loss) / income per ordinary share	$ (2.71)	$ 7.04	$ (2.81)
Weighted average shares - basic	46,735,045	45,986,467	44,466,365
Weighted average shares - diluted	46,735,045	46,840,972	44,466,365

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