



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

May 9, 2023

~ Clinical update from U.S. Phase I/II trial in Huntington's disease expected in second quarter 2023 ~

~ Continued progress towards initiating two new Phase I/II clinical studies for AMT-260 in refractory temporal lobe epilepsy and AMT-162 in SOD1-ALS ~

LEXINGTON, Mass and AMSTERDAM, the Netherlands, May 09, 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 first quarter of 2023 and highlighted recent progress across its business.

"uniQure continued its momentum from a historic 2022 into the first quarter of 2023 as we make progress across all of our corporate objectives," stated [Matt Kapusta, chief executive officer of uniQure](#). "Our priorities for 2023 include supplying CSL's launch of HEMGENIX[®], advancing the development of AMT-130 in Huntington's disease, and preparing for the initiation of two new clinical studies in SOD1-ALS and refractory temporal lobe epilepsy. Later in this second quarter of 2023, we look forward to providing a clinical update from our ongoing U.S. Phase I/II study of AMT-130, the first investigational AAV gene therapy in clinical development for Huntington's disease. This update will help to further guide our clinical development of AMT-130 as we work diligently to bring a disease-modifying treatment option to patients with this devastating neurodegenerative disease."

Recent Updates

- *Commercial launch of HEMGENIX[®] (etranacogene dezaparvovec-drlb) 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 HEMGENIX[®] from uniQure in May 2021 and is solely responsible for its commercialization. Following the first commercial sale of HEMGENIX[®] in the United States, uniQure is entitled to receive a \$100.0 million payment from CSL Behring. The Company is also eligible to receive a \$75.0 million payment from CSL Behring following the first product sale in one of five major European countries if achieved prior to July 2, 2023.
- *Advancing AMT-130 for the treatment of Huntington's disease*
 - The Company plans to announce one to two-years of follow up data from its 26 patient, randomized, controlled and double-blinded U.S. Phase I/II clinical study in the second quarter of 2023. The data update is expected to include safety and tolerability, biomarker, imaging and functional data across both high and low dose cohorts.
 - Three of the six control patients in the higher-dose cohort have now crossed over to treatment. Two of these three patients have received the higher dose of AMT-130. Control patients in the higher-dose cohort have the option to cross over to treatment if they meet the study's eligibility criteria after unblinding. The company expects to complete any additional crossover procedures by the end of the second quarter of 2023.
 - All six patients have been enrolled in the lower-dose cohort of the European, open-label Phase I/II clinical trial of AMT-130. The Company continues to enroll the second, higher-dose cohort of nine patients. Patient enrollment in the higher-dose cohort is expected to complete in mid- 2023 with one-year of clinical data from the lower-dose cohort expected 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*
 - *American Society of Gene and Cell Therapy (ASGCT) 2023* – The company will have a major presence at the

annual ASGCT meeting being held in Los Angeles, CA from May 16-20, 2023. Twelve abstracts have been accepted for presentation, including two oral presentations, that will feature innovation in expanding the Company's research pipeline and advancements in AAV gene-therapy manufacturing.

- *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 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 the third quarter of 2022, the Company initiated a GLP toxicology study of AMT-260 in non-human primates. The Company expects to submit an investigational new drug (IND) 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 the third quarter of 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 March 31, 2023, the Company had cash and cash equivalents and investment securities of \$315.3 million. The Company expects cash and cash equivalents and investment securities will fund operations into 2025 assuming the achievement of \$100.0 million of first commercial sale milestone in the United States, 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.

Financial Highlights

Cash position: As of March 31, 2023, the Company held cash and cash equivalents and investment securities of \$315.3 million, compared to \$392.8 million as of December 31, 2022.

Revenues: Revenue for the three months ended March 31, 2023 was \$5.3 million, compared to \$1.8 million in the same period in 2022. The increase is primarily a result of contract manufacturing revenues of \$4.9 million recognized in the current period related to contract manufacturing HEMGENIX® for CSL Behring partially offset by a decrease in collaboration revenues of \$1.4 million.

R&D expenses: Research and development expenses were \$60.8 million for the three months ended March 31, 2023, compared to \$45.0 million in the same period in 2022. The increase was primarily related to the \$10.0 million payment made to Apic Bio to acquire ABP-102, now AMT-162, 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 a licensor upon EMA approval of HEMGENIX®.

SG&A expenses: Selling, general and administrative expenses were \$17.8 million for the three months ended March 31, 2023, compared to \$11.0 million in the same period in 2022. The increase was primarily related to an increase in personnel and contractor related expenses to support the growth of the Company and an increase in professional fees incurred in the current period, primarily driven by an increase in professional fees related to the Apic Bio transaction.

Other non-operating items, net:

Other non-operating items net was an expense of \$4.3 million for the three months ended March 31, 2023, compared to net income of \$6.8 million for the same period in 2022. The decrease in other non-operating items, net was primarily related to recognizing foreign currency losses, net of \$2.4 million in the current period compared to recognizing foreign currency gains, net of \$8.6 million in the prior period.

Net loss:

The net loss for the three months ended March 31, 2023, was \$77.2 million, or \$1.63 basic and diluted loss per ordinary share, compared to \$46.7 million net loss for the same period in 2022, or \$1.00 basic and diluted loss 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, whether there will be continued progress on the commercialization of HEMGENIX®, 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 that data will help to further guide our ongoing clinical development of AMT-130, 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 and the Quarterly Report on Form 10-Q filed May 9, 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

	March 31, 2023	December 31, 2022
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	\$ 153,851	\$ 228,012
Current investment securities	161,487	124,831
Accounts receivable and contract asset	104,793	102,376
Inventories	7,477	6,924
Prepaid expenses	12,567	11,817
Other current assets and receivables	2,468	2,814
Total current assets	442,643	476,774
Non-current assets		
Property, plant and equipment, net	50,072	50,532
Non-current investment securities	-	39,984
Operating lease right-of-use assets	32,135	32,726
Intangible assets, net	59,704	58,778
Goodwill	25,998	25,581
Deferred tax assets, net	14,331	14,528
Other non-current assets	6,121	6,061
Total non-current assets	188,361	228,190
Total assets	\$ 631,004	\$ 704,964
Current liabilities		
Accounts payable	\$ 8,885	\$ 10,984
Accrued expenses and other current liabilities	\$ 22,529	\$ 30,571
Current portion of contingent consideration	27,253	25,982
Current portion of operating lease liabilities	7,669	8,382
Total current liabilities	66,336	75,919

Non-current liabilities

Long-term debt	103,253	102,791
Operating lease liabilities, net of current portion	31,075	31,719
Contingent consideration, net of current portion	9,641	9,334
Deferred tax liability, net	6,970	8,257
Other non-current liabilities	958	935
Total non-current liabilities	151,897	153,036
Total liabilities	218,233	228,955
Shareholders' equity		
Total shareholders' equity	412,771	476,009
Total liabilities and shareholders' equity	\$ 631,004	\$ 704,964

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended March 31,	
	2023	2022
	(in thousands, except share and per share amounts)	
Total revenues	\$ 5,325	\$ 1,792
Operating expenses:		
Cost of contract manufacturing revenues	(2,435)	-
Research and development expenses	(60,809)	(45,003)
Selling, general and administrative expenses	(17,848)	(10,987)
Total operating expenses	(81,092)	(55,990)
Other income	1,811	311
Other expense	(216)	(193)
Loss from operations	(74,172)	(54,080)
Non-operating items, net	(4,262)	6,786
Loss before income tax benefit / (expense)	\$ (78,434)	\$ (47,294)
Income tax benefit / (expense)	1,207	616
Net loss	\$ (77,227)	\$ (46,678)
Basic and diluted net loss per ordinary share	\$ (1.63)	\$ (1.00)
Weighted average shares used in computing basic and diluted net loss per ordinary share	47,436,335	46,599,114

