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

August 1, 2023

~ Announced encouraging interim analysis data from ongoing Phase I/II clinical trial of AMT-130 in Huntington's disease showing preservation of function compared to baseline and clinical benefits relative to natural history; both dose cohorts of AMT-130 continue to be generally well-tolerated ~

~ Preparations underway to initiate two Phase I/II clinical trials in refractory temporal lobe epilepsy and SOD1-ALS later this year; IND for Fabry disease expected to be submitted in the second half of 2023 ~

~ Strong cash position of $628.6 million as of June 30, 2023, which excludes a $100 million milestone related to first commercial sale of HEMGENIX® in the U.S. received in July 2023 ~

~ Announced sale of royalty interest in HEMGENIX® for up to $400 million ~

~ Appointed Walid Abi-Saab, M.D., as Chief Medical Officer; Jeannette Potts as Chief Legal Officer ~

LEXINGTON, Mass. and AMSTERDAM, Aug. 01, 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 second quarter of 2023 and highlighted recent progress across its business.

"In the second quarter of the year, we continued to make strong progress advancing our programs, including announcing encouraging data from the interim analysis of our Phase I/II study of AMT-130 in Huntington's disease," stated Matt Kapusta, chief executive officer of uniQure. "These data show that patients treated with AMT-130 appear to have generally preserved clinical and motor function and neurofilament light chain (NfL) levels suggesting a stable to improving neurodegenerative profile. We believe that, collectively, the data suggest early and promising evidence of clinical benefit in patients treated with AMT-130 compared to a patient-matched natural history data set. We look forward to providing another update in the fourth quarter of 2023 that will include data from our European study, as well as preparing for regulatory interactions in the first quarter of 2024 to discuss the data and potential late-stage development of AMT-130."

"We are also working towards the initiation of clinical trials for our gene therapy candidates in refractory temporal lobe epilepsy and SOD1-ALS later this year, as well as the submission of an IND for our Fabry disease gene therapy," he added. "With a strong cash position following achievement of the $100 million milestone for first U.S. commercial sale of HEMGENIX® and completion of a royalty financing transaction, we are focused on rapidly moving programs into clinical development and creating near-term value to shareholders."

Recent Updates

- *Advancing AMT-130 for the treatment of Huntington's disease*
  - In June 2023, interim clinical data were announced on the high-dose and low-dose cohorts of the double-blinded and randomized U.S. Phase I/II study of AMT-130 for the treatment of early-stage Huntington's disease. After 12- and 24-months of follow-up, AMT-130 was generally well-tolerated with a manageable safety profile at both doses. For patients in both dose cohorts, clinical function was generally preserved, and compared to natural history, demonstrated benefits at up to 24 months in each of Total Motor Score, Total Functional Capacity and the composite Unified Huntington's Disease Rating Scale. CSF NfL for the low-dose cohort were below baseline at 24 months and were declining towards baseline for the high-dose cohort at 12 months. Suppression of CSF mHTT in the low-dose cohort during 24 months of follow-up support target engagement, with greater variability observed in the high-dose cohort during 12 months of follow-up.
  - Four control patients in the high-dose cohort of the U.S. study have now crossed over to treatment with AMT-130. Three of these four patients received the high dose of AMT-130, with one receiving the low dose. The remaining two control patients in the high-dose cohort did not meet the eligibility criteria for crossover. All crossover patients received a short course of immunosuppression therapy concurrent with the administration of AMT-130.
  - All six patients have been enrolled in the low-dose cohort of the European, open-label Phase I/II clinical trial of AMT-130. The Company continues to make progress enrolling the second, high-dose cohort of nine patients and expects to complete enrollment in the third quarter of 2023.
  - In the second half of 2023, uniQure expects to initiate a third cohort in the ongoing U.S. clinical trial to further investigate both doses in combination with perioperative immunosuppression with a focus on evaluating near-term safety and tolerability. The third cohort will enroll up to 10 patients, all of whom will receive AMT-130 using the current, established stereotactic neurosurgical delivery procedure.
  - In the fourth quarter of 2023, the Company plans to provide a clinical update from the U.S. and European Phase I/II studies of AMT-130, including 18- and 30-month follow-up data from the treated patients in the high- and low-dose U.S. cohorts, respectively. The data update will include safety and tolerability, biomarker and functional data across

both high and low dose cohorts.
    - In the first quarter of 2024, the Company plans on having regulatory interactions to discuss the U.S. and EU data and potential late-stage development pathways for AMT-130.
- *Expanding the Clinical Pipeline & Advancing Innovation*
    - *AMT-260 for the treatment of refractory temporal lobe epilepsy (rTLE)* – In the first half of 2023, the Company completed its GLP toxicology study of AMT-260 in non-human primates. The Company continues to expect submission of an investigational new drug (IND) application and initiation of a Phase I/II clinical study of AMT-260 in the second half of 2023.
    - *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-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 the second half of 2023.
    - *American Society of Gene and Cell Therapy (ASGCT) 2023* – The company had a major presence at the annual ASGCT meeting held in Los Angeles, CA from May 16-20, 2023. Twelve abstracts were accepted for presentation, including two oral presentations, that featured innovation in expanding the Company's research pipeline and advancements in AAV gene-therapy manufacturing.
- *Commercial launch of HEMGENIX® (etranacogene dezaparvovec-drbl) for the treatment of hemophilia B in the United States and European Union*
    - In June 2023, uniQure announced the achievement of a $100 million milestone payment from CSL Behring associated with the first commercial sale of HEMGENIX® in the United States.
    - In May 2023, uniQure announced the sale of a royalty interest in HEMGENIX® for up to $400 million. The company received a $375 million upfront cash payment in exchange for the lowest royalty tier on CSL Behring's worldwide net sales of HEMGENIX® up to 1.85 times the purchase price until June 30, 2032 or, if such cap is not met by June 30, 2032, up to 2.25x the purchase price through December 31, 2038. uniQure will retain the rights to all other royalties under its existing Commercialization and License Agreement with CSL Behring, as well as contractual milestones totaling up to $1.4 billion.
- *Strengthening of Leadership Team*
    - In June 2023, Walid Abi-Saab, M.D., was appointed as Chief Medical Officer, responsible for leading all clinical research and development, regulatory affairs, medical affairs, and program management at uniQure. Dr. Abi-Saab brings more than 20 years of experience to the company having previously served as Chief Medical Officer of Galapagos. Prior to joining Galapagos, he served as Group Vice President, Global Clinical Development at Shire, where he was responsible for the clinical development plans of all programs in the therapeutic area and oversaw the development of more than 10 marketed products and other programs in rare diseases and specialty pharma. He also has previously held leadership positions at Novartis, Abbott Laboratories, and Pfizer.
    - In May 2023, Jeannette Potts, Ph.D. was appointed as Chief Legal Officer, following the planned departure of David Cerveny. Ms. Potts has more than 25 years of global legal experience in the biopharmaceutical industry. Most recently, she served as Senior Vice President, General Counsel & Corporate Secretary at Forma Therapeutics, a clinical-stage biopharmaceutical company focused on rare hematologic diseases and cancer, which was acquired by Novo Nordisk in 2022. Prior to joining Forma, Jeannette served as Head Counsel, Research and Development, at Takeda Pharmaceuticals, and had previously been Vice President, Associate General Counsel at Millennium Pharmaceuticals before it was acquired by Takeda.
- *Strong cash position to advance the Company's programs*
    - As of June 30, 2023, the Company had cash, cash equivalents and investment securities of $628.6 million, which excludes the $100 million milestone payment received from CSL Behring in July. The Company expects cash, cash equivalents and investment securities will fund operations into the second quarter of 2026.

Upcoming Investor Events

- Goldman Sachs Boston Bus Tour, August 9, 2023 – Boston, MA
- Wells Fargo 2023 Healthcare Conference, September 6, 2023 – Boston, MA
- Citi's 2023 18 th Annual BioPharma Conference, September 7, 2023 – Boston, MA
- 2023 Cantor Global Healthcare Conference, September 28, 2023 – New York, NY

Financial Highlights

Cash position: As of June 30, 2023, the Company held cash and cash equivalents and investment securities of $628.6 million, compared to $392.8 million as of December 31, 2022. The Company entered into a royalty agreement in May 2023 and received an upfront payment of $375.0 million.

Revenues: Revenue for the three months ended June 30, 2023 was $2.4 million, compared to $0.5 million in the same period in 2022. The increase is primarily a result of contract manufacturing revenues of $1.3 million recognized in the current period related to contract manufacturing HEMGENIX® for CSL Behring and an increase in license revenues of $0.8 million.

R&D expenses: Research and development expenses were $46.0 million for the three months ended June 30, 2023, compared to $46.2 million in the same period in 2022. The decrease in external program expense was offset by an increase in various other expenses, including employee and contractor-related expenses, facility expenses and research and development disposable costs.

SG&A expenses: Selling, general and administrative expenses were $21.2 million for the three months ended June 30, 2023, compared to $12.5 million in the same period in 2022. The increase was primarily related to an increase in financial advisory fees, an increase in various other professional fees and an increase in personnel and contractor-related expenses.

Other non-operating items, net:
Other non-operating items net was an expense of $3.2 million for the three months ended June 30, 2023, compared to net income of $16.7 million for the same period in 2022. The decrease in other non-operating items, net was primarily related to a decrease in foreign currency gains of $19.0 million and an increase in interest expense.

Net loss:
The net loss for the three months ended June 30, 2023, was $68.5 million, or $1.44 basic and diluted loss per ordinary share, compared to $39.1 million net loss for the same period in 2022, or $0.84 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 – represent 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, the Company's ability to fund operations into 2026, whether there will be continued progress on the commercialization of HEMGENIX® and the Company's ability to meet certain milestones, statements about the clinical benefits for patients treated with AMT-130, the timing of patient enrollment in our AMT-130 clinical trial for Huntington's Disease, whether the Company will announce follow up data from the U.S. Phase I/II clinical study of AMT-130 and whether such announcement will include data from the Company's European study, whether that data will help to further guide our ongoing clinical development of AMT-130, 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, 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, and whether the Company will begin interactions with regulatory agencies in the first quarter of 2024. 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®, material changes to our interim or preliminary data, 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 and ongoing litigation, the 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 August 1, 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

	June 30, 2023	December 31, 2022
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 513,598	$ 228,012
Current investment securities	114,989	124,831
Accounts receivable and contract asset	102,559	102,376
Inventories	10,212	6,924
Prepaid expenses	12,351	11,817
Other current assets and receivables	3,250	2,814
Total current assets	**756,959**	**476,774**
Non-current assets		
Property, plant and equipment, net	48,567	50,532
Non-current investment securities	—	39,984
Operating lease right-of-use assets	31,395	32,726
Intangible assets, net	59,713	58,778
Goodwill	26,016	25,581
Deferred tax assets, net	13,995	14,528
Other non-current assets	6,124	6,061
Total non-current assets	**185,810**	**228,190**
Total assets	**$ 942,769**	**$ 704,964**
Current liabilities		
Accounts payable	$ 9,373	$ 10,984
Accrued expenses and other current liabilities	23,537	30,571
Current portion of contingent consideration	27,666	25,982
Current portion of operating lease liabilities	7,780	8,382
Total current liabilities	**68,356**	**75,919**
Non-current liabilities		
Long-term debt	101,110	102,791
Liability from royalty financing agreement	372,445	—
Operating lease liabilities, net of current portion	30,195	31,719
Contingent consideration, net of current portion	9,581	9,334
Deferred tax liability, net	6,802	8,257
Other non-current liabilities	960	935
Total non-current liabilities	**521,093**	**153,036**
Total liabilities	**589,449**	**228,955**
Shareholders' equity		
Total shareholders' equity	**353,320**	**476,009**
Total liabilities and shareholders' equity	**$ 942,769**	**$ 704,964**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended June 30,	
	2023	2022
	(in thousands, except share and per share amounts)	
Total revenues	**$ 2,422**	**$ 497**
Operating expenses:		
Cost of contract manufacturing revenues	(1,352)	(832)
Research and development expenses	(46,036)	(46,192)
Selling, general and administrative expenses	(21,181)	(12,491)
Total operating expenses	**(68,569)**	**(59,515)**
Other income	1,302	3,186
Other expense	(229)	(229)

Loss from operations		**(65,074)**		**(56,061)**
Non-operating items, net		(3,237)		16,682
Loss before income tax (expense) / benefit	**$**	**(68,311)**	**$**	**(39,379)**
Income tax (expense) / benefit		(163)		318
Net loss	**$**	**(68,474)**	**$**	**(39,061)**
Basic and diluted net loss per ordinary share	$	(1.44)	$	(0.84)
Weighted average shares used in computing basic and diluted net loss per ordinary share		47,649,520		46,668,554

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