uniQure Announces Second Quarter 2024 Financial Results and Provides Company Update

August 1, 2024

~ Announced RMAT designation for AMT-130 in Huntington's disease and positive interim Phase I/II data demonstrating the slowing of disease progression and reductions in a key biomarker of neurodegeneration; Meeting with FDA expected in the second half of 2024 to discuss potential for expedited clinical development ~

~ Initiated patient screening for three additional Phase I/II studies in mesial temporal lobe epilepsy, SOD1 ALS, and Fabry disease ~

~ Today, announced an organizational restructuring intended to streamline operations; Together with the recent sale of the Lexington manufacturing facility, these changes are expected to reduce headcount by 65% and lower recurring cash burn by $75 million per year including savings from the retirement of $50 million of debt ~

~ Strong cash position of approximately $524 million as of June 30, 2024, expected to fund operations through the end of 2027 and multiple value-generating inflection points ~

LEXINGTON, Mass. and AMSTERDAM, Aug. 01, 2024 (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 2024 and highlighted recent progress across its business.

"We have made significant progress over the past few months across several key business objectives, including advancing our clinical pipeline and taking important actions to considerably reduce our capital requirements," stated Matt Kapusta, chief executive officer of uniQure. "With the recent Regenerative Medicine Advanced Therapy (RMAT) designation and the latest interim data supporting dose-dependent slowing of Huntington's disease progression, we are eager to engage further with regulators to pursue an expedited clinical development pathway for AMT-130. We've also made meaningful progress across our three other clinical programs, with patient screenings underway and enrollment expected to begin shortly. At the same time, we've taken targeted measures to substantially reduce operating expenses, streamline operations, and extend cash runway. These actions are designed to ensure we have the funding required to achieve key milestones and drive shareholder value, as we endeavor to deliver transformative medicines to patients in need."

Recent Company Updates

- *Advancing AMT-130 for the treatment of Huntington's disease*
    - In June 2024, the U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) designation for investigational gene therapy AMT-130, becoming the first therapeutic candidate to receive such a designation for Huntington's disease. RMAT designation allows sponsor companies to have early, close and frequent interactions with the FDA.
    - In July 2024, uniQure announced positive interim data from the ongoing U.S. and European Phase I/II studies of AMT-130 for the treatment of early-stage Huntington's disease. [1]
        - A statistically significant, dose-dependent slowing in disease progression measured by the composite Unified Huntington's Disease Rating Scale (cUHDRS) was observed through 24 months in patients receiving the high dose of AMT-130 compared to a propensity score-weighted external control (p=0.007).
        - A statistically significant reduction of neurofilament light chain (NfL) in cerebrospinal fluid (CSF) was observed at 24 months in patients treated with AMT-130 compared to baseline (p=0.02).
        - AMT-130 continued to be generally well-tolerated with a manageable safety profile across both doses.
    - The Company expects to hold a Type B, multi-disciplinary RMAT meeting with the FDA in the second half of 2024 with the goal of defining the future clinical and regulatory pathway for AMT-130.
    - Patient dosing is ongoing in a third cohort of up to 12 patients to further evaluate both doses of AMT-130 in combination with perioperative immunosuppression regimen, with a focus on evaluating near-term safety and tolerability. Enrollment in this third cohort is expected to be completed in the second half of 2024.
    - The Company expects to provide an additional interim update from its ongoing Phase I/II clinical trials of AMT-130 in mid-2025. The update will include follow-up data on all patients treated with AMT-130 in the first two cohorts,

including three years of follow-up on 21 treated patients.

- *Initiating new Phase I/II clinical studies*

  - *AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (mTLE)* – Patient screening in a Phase I/II clinical study has been initiated and enrollment is expected to begin in the third quarter of 2024. The first part of the U.S., multi-center, open-label trial Phase I/II is expected to include up to 12 patients across two dose cohorts.

  - *AMT-191 for the treatment of Fabry disease* – Patient screening in a Phase I/II clinical study has been initiated and enrollment is expected to begin in the third quarter of 2024. The U.S., multi-center, open-label trial is expected to include up to 12 adult male patients across two dose cohorts.

  - *AMT-162 for the treatment of SOD1 amyotrophic lateral sclerosis (ALS)* – Patient screening in a Phase I/II clinical study has been initiated and enrollment is expected to begin in the third quarter of 2024. The U.S., multi-center, open-label trial is expected to include up to 12 patients across three dose cohorts.

Capital Preservation Initiatives

uniQure conducted and recently concluded a detailed review of its operating expenses with the goals of conserving capital, streamlining operations, and ensuring sufficient cash resources to achieve multiple potentially meaningful value creating milestones. As a result of this review, uniQure has or will be taking the following steps:

- The sale of the Lexington, Massachusetts manufacturing facility to Genezen announced on July 1, 2024.

- Global workforce reductions aimed at organizational rightsizing, delayering and outsourcing sub- and non-critical activities.

- Inclusive of the sale of the manufacturing facility, the elimination of approximately 65% or 300 roles across the organization. Certain intended organizational changes are subject to review and advice from the Company's Amsterdam-based works council, which is ongoing and expected to be completed in the third quarter of 2024. The Company expects to substantially complete the restructuring in the fourth quarter of 2024.

- A reduction in annual recurring cash burn of approximately 40% or $75 million, which includes savings in interest expense from the retirement of $50 million in outstanding debt.

- Current balance of cash, cash equivalents and investment securities of $524 million as of June 30, 2024 are expected to fund operations through the end 2027.

As a result of the sale of the Lexington manufacturing facility, Pierre Caloz, chief operating officer of uniQure will depart the company. Amin Abujoub, Ph.D. who previously served as chief quality officer, has been appointed to the new role of chief technical operations officer in which he will be responsible for global oversight of contract manufacturers as well as internal operations, facilities, process and analytical development, and quality.

"After a comprehensive review motivated by our patient-driven mission, we are making important changes to align uniQure with our objectives of delivering sustainable value creation and ensuring we are optimally positioned for the future," added Matt Kapusta. "We have taken great care to ensure those impacted by these changes are supported, and greatly appreciate the contributions they have made to the company. I particularly want to thank Pierre for his leadership, dedication and compassion, all of which were critical in achieving multiple HEMGENIX® approvals and establishing world-class commercial manufacturing capabilities. Through our relationship with Genezen, we hope to leverage these capabilities for years to come."

"These decisions will enable us to prioritize investments in our Huntington's disease, temporal lobe epilepsy and other gene therapy programs, as well as innovating and broadening the long-term applicability of AAV-delivered gene therapy."

Upcoming Investor Events

- Wells Fargo 2024 Healthcare Conference, September 5 – Boston, MA
- 2024 Cantor Global Healthcare Conference, September 18 – New York, NY

Financial Highlights

Cash position: As of June 30, 2024, the Company held cash and cash equivalents and investment securities of $524.4 million, compared to $617.9 million as of December 31, 2023. Based on the Company's current operating plan and pending successful completion of the capital preservation measures described above, the Company expects cash, cash equivalents and investment securities will be sufficient to fund operations through the end of 2027.

Revenues: Revenue for the three months ended June 30, 2024, was $11.1 million, compared to $2.4 million in the same period in 2023. The increase of $8.7 million in revenue resulted from an increase of $6.8 million in collaboration revenue, an increase of $1.1 million in license revenue and an increase of $0.8 million in revenue from contract manufacturing of HEMGENIX® for CSL.

Cost of license revenue: Costs of license revenues were $0.2 million for the three months ended June 30, 2024, compared to $0.0 million for the

same period in 2023. The increase primarily relates to not incurring such costs in 2023.

Cost of contract manufacturing revenues: Costs of contract manufacturing revenues were $7.2 million for the three months ended June 30, 2024, compared to $1.4 million for the same period in 2023. The increase primarily relates to expensing costs previously capitalized as inventory.

R&D expenses: Research and development expenses were $33.7 million for the three months ended June 30, 2024, compared to $46.0 million during the same period in 2023. The $12.3 million decrease was primarily related to a decrease of $6.6 million in employee-related expenses partially offset by an increase of $1.1 million severance costs related to the sale of the Lexington facility, a $4.5 million decrease in costs incurred related to preclinical supplies, and a decrease of $2.0 million related to the changes in fair value of contingent consideration.

SG&A expenses: Selling, general and administrative expenses were $15.8 million for the three months ended June 30, 2024, compared to $21.2 million during the same period in 2023. The $5.4 million decrease was primarily related to a $3.8 million decrease in financial advisory fees, a $1.6 million decrease in intellectual property fees and information technology expenses, and a $1.1 million decrease in employee-related expenses compared to the prior year period. These decreases were partially offset by a $1.6 million increase in professional fees primarily related to the sale of the Lexington facility.

Other non-operating items, net: Other non-operating items, net was an expense of $11.3 million for the three months ended June 30, 2024, compared to $3.2 million for the same period in 2023. The $8.1 million increase in other non-operating items, net was primarily related to an increase in non-cash interest expense of $9.3 million related to the royalty agreement that the Company entered into in May 2023 and an increase in net foreign currency losses of $1.4 million, which were partially offset by an increase of $2.6 million in interest income earned on investment securities and cash on hand.

Net loss: The net loss for the three months ended June 30, 2024, was $56.3 million, or $1.16 basic and diluted loss per ordinary share, compared to $68.5 million net loss for the same period in 2023, or $1.44 basic and diluted loss per ordinary share.

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. The approvals of uniQure's 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. uniQure is now advancing 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. Examples of these forward-looking statements include, but are not limited to, statements concerning the Company's cash runway and its ability to fund its operations through the end of 2027 and achieve multiple value-generating inflection points; the Company's expectations regarding planned organizational changes, including reductions in headcount and lower annual cash burn resulting from such changes; the ability of such organizational changes to yield the funding required to achieve key clinical and regulatory milestones, maximize shareholder value, and deliver transformative medicines to patients; the Company's planned workforce reductions, including the extent and timing thereof and the costs associated with such workforce reductions; the Company's plans to announce additional interim updates from its ongoing U.S. and European Phase I/II clinical studies of AMT-130; the Company's plans to meet the FDA regarding potential expedited clinical development pathways for AMT-130, the timing of such regulatory interactions and expectations regarding potential regulatory clarity from such interactions; the Company's plans regarding the third cohort in its AMT-130 clinical trial and the timing of enrollment for such cohort; and the Company's plans to initiate patient enrollment for AMT-191, AMT-260 and AMT-162 and the design of trials for the Company's additional clinical programs. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons. These risks and uncertainties include, among others: risks associated with the implementation of the Company's restructuring plans; risks associated with the clinical results and the development and timing of the Company's programs; the Company's interactions with regulatory authorities, which may affect the initiation, timing and progress of clinical trials and pathways to approval; the Company's ability to continue to build and maintain the company infrastructure and personnel needed to achieve its goals following planned workforce reductions; the Company's effectiveness in managing current and future clinical trials and regulatory processes; the continued development and acceptance of gene therapies; the Company's ability to demonstrate the therapeutic benefits of its gene therapy candidates in clinical trials; the Company's ability to obtain, maintain and protect intellectual property; the Company's ability to fund its operations and to raise additional capital as needed; and the impact of global economic uncertainty, rising inflation, rising interest rates or market disruptions on its business. These risks and uncertainties are more fully described under the heading "Risk Factors" in the Company's periodic filings with the U.S. Securities & Exchange Commission ("SEC"), including its Annual Report on Form 10-K filed February 28, 2024 and in other filings that the Company makes with the SEC from time to time. 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, 2024	December 31, 2023
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 287,877	$ 241,360
Current investment securities	236,553	376,532
Inventories, net	-	12,024
Accounts receivable	7,850	4,193
Prepaid expenses	18,278	15,089
Assets held for sale	37,964	-
Other current assets	3,446	2,655
Total current assets	**591,968**	**651,853**
Non-current assets		
Property, plant and equipment, net	$ 26,186	$ 46,548
Operating lease right-of-use assets	14,925	28,789
Intangible assets, net	58,659	60,481
Goodwill	23,112	26,379
Deferred tax assets, net	10,718	12,276
Other non-current assets	5,278	5,363
Total non-current assets	**138,878**	**179,836**
Total assets	**$ 730,846**	**$ 831,689**
Current liabilities		
Accounts payable	$ 4,407	$ 6,586
Accrued expenses and other current liabilities	26,491	30,534
Current portion of contingent consideration	28,060	28,211
Current portion of operating lease liabilities	3,625	8,344
Liabilities held for sale	17,885	-
Total current liabilities	**80,468**	**73,675**
Non-current liabilities		
Long-term debt	102,507	101,749
Liability from royalty financing agreement	415,940	394,241
Operating lease liabilities, net of current portion	12,369	28,316
Contingent consideration, net of current portion	12,078	14,795
Deferred tax liability, net	7,323	7,543
Other non-current liabilities	3,054	3,700
Total non-current liabilities	**553,271**	**550,344**
Total liabilities	**633,739**	**624,019**
Shareholders' equity		
Total shareholders' equity	**97,107**	**207,670**
Total liabilities and shareholders' equity	**$ 730,846**	**$ 831,689**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended June 30,	
	2024	2023
	(in thousands, except share and per share amounts)	
Total revenues	**$ 11,126**	**$ 2,422**
Operating expenses:		
Cost of license revenues	(234)	—

Cost of contract manufacturing revenues		(7,227)		(1,352)
Research and development expenses		(33,655)		(46,036)
Selling, general and administrative expenses		(15,767)		(21,181)
Total operating expenses		**(56,883)**		**(68,569)**
Other income		1,983		1,302
Other expense		(236)		(229)
Loss from operations		**(44,010)**		**(65,074)**
Non-operating items, net		(11,341)		(3,237)
Loss before income tax (expense) / benefit	**$**	**(55,351)**	**$**	**(68,311)**
Income tax (expense) / benefit		(948)		(163)
Net loss	**$**	**(56,299)**	**$**	**(68,474)**
Basic and diluted net loss per ordinary share	$	(1.16)	$	(1.44)
Weighted average shares used in computing basic and diluted net loss per ordinary share		48,622,440		47,649,520

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[1] All p-values are nominal and unadjusted. Statistical comparisons of patients treated with AMT-130 to the propensity score-weighted external control were conducted on a post-hoc basis.

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