uniQure Announces First Quarter 2024 Financial Results and Highlights Recent Company Progress

May 7, 2024

~ On track to initiate FDA interaction regarding AMT-130 in second quarter of 2024 and provide a clinical update from the Phase I/II trials in mid-2024 ~

~ Clinical trial initiation for Fabry disease on track to begin in second quarter of 2024, followed by refractory mesial temporal lobe epilepsy and SOD1-ALS in third quarter of 2024 ~

~ Comprehensive review of operations and options to reduce expenses underway and expected to be completed in mid-2024 ~

LEXINGTON, Mass. and AMSTERDAM, May 07, 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 first quarter of 2024 and highlighted recent progress across its business.

"In the first quarter, we made solid progress across three key business priorities – clarifying the road ahead for AMT-130 in Huntington's disease, initiating three new Phase I/II clinical trials, and prudently conserving our capital," stated Matt Kapusta, chief executive officer of uniQure. "We are on track to initiate FDA interactions on AMT-130 this quarter and look forward to presenting longer-term follow-up data from our Phase I/II trials mid-year, which will include up to three years of patient follow-up on 29 treated patients. We have also made significant progress towards the initiation of new Phase I/II studies. We have obtained IRB approvals for initial sites, which are poised for imminent activation, and have also identified prospective patients. Enrollment for the Fabry disease study is on track to begin in the second quarter of 2024, with the SOD1-ALS and MTLE studies expected to begin in third quarter."

"In addition, we are actively engaged in a comprehensive review of uniQure's operations and options to reduce expenses and increase shareholder value," he added. "We believe deeply in the significant value of our assets, as well as the need to achieve a focused and sustainable level of investment in order to maximize this potential. We expect to complete this evaluation mid-2024."

Recent Updates

- *Advancing AMT-130 for the treatment of Huntington's disease*
    - The Company is on track to initiate interactions with the U.S. Food and Drug Administration in the second quarter of 2024 with the goal of defining the future clinical and regulatory pathway for AMT-130. These interactions, which are expected to continue throughout the year, are expected to include discussions of the interim data from the ongoing Phase I/II clinical trials and the potential to leverage natural history comparators alongside our long-term Phase I/II clinical data. The Company expects to provide an update on its regulatory plans as part of the next clinical update on AMT-130 mid-year and have greater clarity regarding a potential approval pathway for AMT-130 before the end of the year.
    - In mid-2024, the Company expects to provide an update from its ongoing Phase I/II clinical trials of AMT-130. The data will include up to three years of follow-up data on 29 treated patients in both the low- and higher-dose cohorts, of which 21 patients will have a minimum of two years of follow-up.
    - 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, 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.
- *Initiating new Phase I/II clinical studies*
    - *AMT-191 for the treatment of Fabry disease* - Patient enrollment in a Phase I/IIa clinical trial is expected to begin in the second quarter of 2024. The Phase I/IIa clinical trial will be a U.S.-based, multi-center, open-label trial consisting of two cohorts enrolling up to six adult male patients each. The study is designed to evaluate safety, tolerability, and early signs of efficacy.
    - *AMT-162 for the treatment of SOD1 amyotrophic lateral sclerosis (ALS)* – Patient enrollment in a Phase I/II clinical trial is expected to begin in the third quarter of 2024. The Phase I/II clinical trial will be a U.S.-based, multi-center, open-label trial consisting of three cohorts with up to four patients each receiving a one-time intrathecal infusion with immunosuppression. The study is designed to evaluate safety, tolerability, and early signs of efficacy.
    - *AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (MTLE)* – Patient enrollment in a Phase I/IIa

clinical trial is expected to begin in third quarter of 2024. The Phase I/II will be a U.S.-based consisting of two parts. The first part is a multicenter, open-label trial with two dosing cohorts of six patients each to assess safety, tolerability, and first signs for efficacy of AMT-260 in patients with refractory MTLE. The second part is expected to be a randomized, controlled trial to generate proof of concept (POC) data.

Upcoming Investor Events

- RBC Capital Markets 2024 Global Healthcare Conference, May 15th – New York, NY

Financial Highlights

Cash position: As of March 31, 2024, the Company held cash and cash equivalents and investment securities of $555.7 million, compared to $617.9 million as of December 31, 2023. The Company expects cash, cash equivalents and investment securities will fund operations into the second quarter of 2027.

Revenues: Revenue for the three months ended March 31, 2024 was $8.5 million, compared to $5.3 million in the same period in 2023. The net increase of $3.2 million in revenue resulted from an increase of $2.9 million from collaboration revenue and an increase of $1.2 million in license revenue partially offset by a decrease of $0.9 million from contract manufacturing of HEMGENIX® for CSL.

Cost of contract manufacturing revenues: Cost of contract manufacturing revenues were $9.1 million for the three months ended March 31, 2024, compared to $2.4 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 $40.7 million for the three months ended March 31, 2024, compared to $60.8 million during the same period in 2023. The $20.1 million decrease was primarily related to an $8.1 million decrease in external spend for the ALS SOD1 program, as prior year costs included a $10.0 million one-time cost for the acquisition of the program from Apic Bio. Additionally, there was a $7.0 million decrease in preclinical and other program spend, a $2.0 million decrease in costs incurred related to preclinical supplies and a $2.7 million decrease in employee related expenses.

SG&A expenses: Selling, general and administrative expenses were $13.9 million for the three months ended March 31, 2024, compared to $17.8 million during the same period in 2023. The $3.9 million decrease was primarily related to a $1.7 million decrease in professional and intellectual property fees, as well as a reduction of expenses for information technology.

Other non-operating items, net:
Other non-operating items, net was an expense of $10.7 million for the three months ended March 31, 2024, compared to $4.3 million for the same period in 2023. The $6.5 million increase in other non-operating items, net was primarily related to an increase in non-cash interest expense of $12.5 million related to the royalty agreement that the Company entered into in May 2023, which partially was offset by an increase of $4.8 million in interest income earned on investment securities and cash on hand.

Net loss:
The net loss for the three months ended March 31, 2024, was $65.6 million, or $1.36 basic and diluted loss per ordinary share, compared to $77.2 million net loss for the same period in 2023, or $1.63 basic and diluted loss per ordinary share.

About uniQure

uniQure's mission is to reimagine the future of medicine by delivering innovative cures that transform lives. The recent approvals of our gene therapy for hemophilia B – a 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 and manufacturing platform to advance a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory mesial temporal lobe epilepsy, amyotrophic lateral sclerosis (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 into the second quarter of 2027; the Company's ongoing review of its operations and options to reduce expenses, the Company's expectations that such review will lead to a strategy that, if effectively executed, will reduce operating expenses and increase shareholder value, and the expected timing of the completion of such review; the Company's plans to announce additional follow-up data from its ongoing U.S. and European Phase I/II clinical studies of AMT-130; the Company's plans to initiate interactions with the FDA regarding the further development of AMT-130, the timing of such interactions and expectations regarding 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 in the second quarter of 2024, and to initiate patient enrollment for AMT-260 and AMT-162 in the third quarter of 2024. 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 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; 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.

uniQure Contacts:

FOR INVESTORS:

Maria E. Cantor
Direct: 339-970-7536
Mobile: 617-680-9452
m.cantor@uniQure.com

Chiara Russo
Direct: 617-306-9137
Mobile: 617-306-9137
c.russo@uniQure.com

FOR MEDIA:

Tom Malone
Direct: 339-970-7558
Mobile:339-223-8541
t.malone@uniQure.com

uniQure N.V.

UNAUDITED CONSOLIDATED BALANCE SHEETS

	March 31, 2024	December 31, 2023
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 243,062	$ 241,360
Current investment securities	312,621	376,532
Accounts receivable and contract asset	10,717	4,193
Inventories	7,672	12,024
Prepaid expenses	18,839	15,089
Other current assets and receivables	3,092	2,655
Total current assets	**596,003**	**651,853**
Non-current assets		
Property, plant and equipment, net	44,554	46,548
Operating lease right-of-use assets	27,695	28,789
Intangible assets, net	59,111	60,481
Goodwill	25,795	26,379
Deferred tax assets, net	11,594	12,276
Other non-current assets	5,298	5,363
Total non-current assets	**174,047**	**179,836**
Total assets	**$ 770,050**	**$ 831,689**
Current liabilities		
Accounts payable	$ 5,231	$ 6,586
Accrued expenses and other current liabilities	22,658	30,534
Current portion of contingent consideration	27,587	28,211
Current portion of operating lease liabilities	7,997	8,344
Total current liabilities	**63,473**	**73,675**
Non-current liabilities		
Long-term debt	102,120	101,749
Liability from royalty financing agreement	405,398	394,241
Operating lease liabilities, net of current portion	26,983	28,316
Contingent consideration, net of current portion	14,625	14,795
Deferred tax liability, net	7,376	7,543
Other non-current liabilities	3,321	3,700
Total non-current liabilities	**559,823**	**550,344**
Total liabilities	**623,296**	**624,019**
Shareholders' equity		
Total shareholders' equity	**146,754**	**207,670**
Total liabilities and shareholders' equity	**$ 770,050**	**$ 831,689**

UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended March 31,	
	2024	**2023**
	(in thousands, except share and per share amounts)	
Total revenues	**$ 8,485**	**$ 5,325**
Operating expenses:		
Cost of license revenues	(150)	—
Cost of contract manufacturing revenues	(9,076)	(2,435)
Research and development expenses	(40,692)	(60,809)
Selling, general and administrative expenses	(13,937)	(17,848)
Total operating expenses	**(63,855)**	**(81,092)**
Other income	1,376	1,811
Other expense	(234)	(216)
Loss from operations	**(54,228)**	**(74,172)**
Non-operating items, net	(10,734)	(4,262)
Loss before income tax (expense) / benefit	**$ (64,962)**	**$ (78,434)**
Income tax (expense) / benefit	(656)	1,207
Net loss	**$ (65,618)**	**$ (77,227)**
Basic and diluted net loss per ordinary share	$ (1.36)	$ (1.63)
Weighted average shares used in computing basic and diluted net loss per ordinary share	48,384,510	47,436,335

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