uniQure Announces First Quarter 2026 Financial Results and Provides Recent Company Updates

May 5, 2026

~ Advancing FDA interactions on AMT-130 for Huntington's disease; Type B meeting scheduled for the second quarter of 2026 ~

~ Progressing AMT-130 toward expected UK regulatory submission; MAA on track for third quarter of 2026 following successful pre-submission meeting with UK MHRA ~

~ Enrollment in AMT-260 temporal lobe epilepsy program on track; clinical update from first cohort in Phase I/IIa study to be presented at the Epilepsy Foundation Pipeline Conference ~

~ Presented updated data from AMT-191 Phase I/IIa in Fabry disease study showed sustained increases in α-Gal A Enzyme Activity and stable Lyso-Gb3 levels; subsequently all 11 dosed patients have discontinued enzyme replacement therapy ~

~ Strong balance sheet with $586.6 million in cash, cash equivalents and current investment securities as of March 31, 2026 and runway into the second half of 2029 ~

~ uniQure to host earnings call at 8:30 a.m. ET ~

LEXINGTON, Mass. and AMSTERDAM, May 05, 2026 (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 2026 and highlighted recent progress across its business.

"During the first quarter of 2026, we remained focused on advancing AMT-130 to patients globally as rapidly as possible while executing across our broader pipeline," said Matthew Kapusta, chief executive officer at uniQure. "We believe our data continue to support the potential for AMT-130 to fundamentally change the treatment landscape for Huntington's disease, and we look forward to continued engagement with the FDA. In parallel, following a constructive interaction with the MHRA, we are preparing to submit an MAA in the third quarter and evaluating additional international opportunities."

"We expect to deliver key clinical updates throughout 2026, including data from our AMT-260 program in refractory mesial temporal lobe epilepsy later in the second quarter and four-year AMT-130 data analysis in the third quarter," Mr. Kapusta continued. "With these important milestones ahead, we remain committed to advancing our programs with urgency while maintaining disciplined capital allocation to drive long-term shareholder value."

Recent Company Developments and Updates

Advancing AMT-130 for the treatment of Huntington's disease

- The Company held a Type A meeting with the U.S. Food and Drug Administration (FDA) in January 2026 to discuss the regulatory path forward following an October 2025 pre-Biologics License Application (BLA) meeting. Following receipt of final meeting minutes from the Type A meeting, the Company announced that the FDA stated it cannot agree that data from the Phase I/II studies, compared to an external control, are sufficient to provide the primary evidence of effectiveness required to support a marketing application for AMT-130.
- The Company has been granted a Type B meeting with the FDA and plans to discuss key elements of a new clinical trial design and to solicit feedback on the proposed statistical analysis plan for the four-year data expected in the third quarter of 2026.
- Following a constructive pre-submission meeting with the United Kingdom's (UK) Medicines and Healthcare products Regulatory Agency (MHRA), the Company expects to submit a Marketing Authorization Application (MAA) for AMT-130 based on three-year data in the third quarter of 2026.

Continued clinical progress in pipeline programs

AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (MTLE)

- In 2025, the Company completed enrollment in the first dose cohort of six patients in the Phase I/IIa study. Enrollment is ongoing in a second dose cohort, expected to include an additional six patients. Enrollment of the second cohort is expected to be completed in mid-2026.
- The Company expects to provide data from the first cohort of six patients in the Phase I/IIa study with up to six months of follow-up at the Epilepsy Foundation Pipeline Conference, June 18 -19, 2026.

AMT-191 for the treatment of Fabry disease

- In February 2026, the Company presented updated safety and exploratory efficacy data from the Phase I/II study of AMT-191 in Fabry disease (data cutoff as of January 8, 2026):

- Dose-dependent elevations were observed across 11 patients in three dose levels with α-Gal A activity ranging from 0.34- to 82.2-fold above mean normal range[1] at the lowest dose, 1.6- to 312.5-fold at the mid dose, and 27.7- to 223.7-fold at the highest dose. These increases were durable across follow-up periods ranging from four months to more than one year.
- Plasma lyso-Gb3 levels were stable post-dose across all cohorts, regardless of enzyme replacement therapy (ERT) status.

- As of February 18, 2026, all 11 dosed patients were withdrawn from ERT.
- AMT-191 continued to show a manageable safety profile at all dose levels. No SAEs related to AMT-191 were observed at the $4x10^{13}$ gc/kg and $2x10^{13}$ gc/kg doses. No additional SAEs were observed at the $6x10^{13}$ gc/kg dose beyond the five previously reported in September 2025 in two patients.
- Per protocol, additional dosing in the mid- and high-dose cohorts has been paused pending further evaluation of asymptomatic Grade 3 liver enzyme elevations reported in two patients from the mid-dose cohort, which were confirmed as dose-limiting toxicities.

AMT-162 for the treatment of SOD1 amyotrophic lateral sclerosis (ALS)

- In 2025, the Company voluntarily paused enrollment in the Phase I/II EPISOD1 multi-center, open-label U.S. trial of AMT-162 for the treatment of SOD1-ALS, following an Independent Data Monitoring Committee review of available preliminary safety and efficacy data, including a SAE determined to be a dose-limiting toxicity observed in one patient in the second cohort. Following review of the preliminary efficacy and safety data generated from EPISOD1, the decision was made to discontinue development of AMT-162. Safety data will continue to be collected from the five patients dosed in EPISOD1, consistent with applicable safety and regulatory requirements.

Focused execution and strong financial position

- In April 2026, uniQure, CSL Behring and Genezen entered into agreements under which uniQure's remaining HEMGENIX® supply and any minimum purchase commitments will terminate after delivery of contractually specified batches to CSL Behring, which uniQure expects to occur in mid-2026. Concurrently, CSL Behring entered into a direct relationship with Genezen as the supplier of HEMGENIX®. These agreements have no impact on future royalties or milestones to uniQure under the license agreement with CSL Behring.
- As of March 31, 2026, the Company had cash, cash equivalents and current investment securities of $586.6 million. The Company expects that cash, cash equivalents and investment securities will be sufficient to fund operations into the second half of 2029.

Financial Highlights

Cash Position: As of March 31, 2026, the Company held $586.6 million in cash, cash equivalents and current investment securities, compared to $622.5 million as of December 31, 2025. The Company expects that cash, cash equivalents and investment securities will be sufficient to fund operations into the second half of 2029.

Revenues: Revenue for the three months ended March 31, 2026 was $3.6 million, compared to $1.6 million in the same period in 2025. The increase of $2.0 million is due to an increase in license revenue, compared to the prior period.

R&D Expenses: Research and development expenses were $29.2 million for the three months ended March 31, 2026, compared to $36.1 million during the same period in 2025. The $6.9 million decrease was driven by a $2.6 million decrease in the fair value of contingent consideration, a $1.2 million decrease in costs related to external program spend, a $1.6 million decrease in employee and contractor-related expenses, including share-based compensation, and a $1.6 million decrease in facilities and other expenses, compared to the prior period.

SG&A Expenses: Selling, general and administrative expenses were $20.1 million for the three months ended March 31, 2026, compared to $10.9 million during the same period in 2025. The $9.2 million increase was primarily related to a $5.5 million increase in employee and contractor-related expenses, including share-based compensation, mainly as a result of employees recruited in 2025 to support commercial planning of AMT-130, a $1.8 million increase in professional fees, a $0.6 million increase in intellectual property fees, and a $1.3 million increase in information technology costs and other expenses, compared to the prior period.

Other Income: Other income was $1.6 million for the three months ended March 31, 2026, compared to $8.3 million during the comparative period in 2025. The $6.7 million decrease was primarily related to a prior period one-time gain of $6.0 million related to the sale of critical reagents and a $0.8 million decrease in research and development grants, compared to the prior period.

Other Expense: Other expense was $1.5 million for the three months ended March 31, 2026, compared to $2.0 million during the same period in 2025. The decrease was primarily due to a $0.3 million decrease in costs associated with the supply of Hemgenix® to CSL Behring and a $0.2 million decrease in sublease expenses in the three months ended March 31, 2026, compared to the prior period.

Other non-Operating Items, net: Other non-operating items, net was an expense of $7.3 million for the three months ended March 31, 2026, compared to an expense of $3.8 million for the same period in 2025. The $3.5 million increase was primarily related to an increase in net foreign currency losses of $9.5 million, offset by a $1.1 million increase in interest income, a $1.1 million decrease in interest expense, and a $3.8 million gain resulting from changes in the fair value of the liability related to pre-funded warrants, compared to the prior period.

Net loss: The net loss for the three months ending March 31, 2026, was $53.5 million, or $0.85 basic and diluted loss per ordinary share, compared to a $43.6 million net loss for the comparative period in 2025, or $0.82 basic and diluted loss per ordinary share.

Upcoming investor events:

- 2026 RBC Capital Markets Global Healthcare Conference, May 19th – New York, NY

Investor Conference Call and Webcast Information

uniQure management will host an investor conference call and webcast today, Tuesday, May 5th at 8:30 a.m. ET. The event will be webcast under the Events & Presentations section of uniQure's website at https://www.uniqure.com/investors-media/events-presentations, and following the event a replay will be archived for 90 days. Analysts wishing to participate in the question and answer session should access the live call by dialing (646) 307-1963 or toll-free (800) 715-9871 and entering conference ID 4607289. If you are joining the conference call, please join 15 minutes before the start time.

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, 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 half of 2029; the Company's ability and plans to strategically advance its programs; the Company's plans and timing with respect to future interactions with regulatory authorities and regulatory updates related to AMT-130, including the Company's plans to continue engaging with the FDA and have a Type B meeting regarding a potential new clinical trial design and statistical analysis plan for the four-year analysis of AMT-130, and the Company's plans to submit a MAA to the MHRA in the third quarter of 2026; the potential for AMT-130 to change the treatment landscape for Huntington's disease; the Company's plans to enroll an additional six patients in a second cohort in the Phase I/IIa study for AMT-260 by mid-2026; the Company's plans to provide further clinical updates, including plans to announce additional data from the Company's AMT-260 program in June 2026 and four-year data from the Company's AMT-130 program in the third quarter of 2026; the Company's plans to continue to collect safety data from patients in the EPISOD1 trial of AMT-162; the timing of when the Company's HEMGENIX ® supply and minimum purchase commitments are expected to terminate pursuant to agreements entered into with CSL Behring and Genezen; and the Company's plans to attend upcoming investor events. 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, including the risk that clinical results will be unable to demonstrate data sufficient to support further clinical development or regulatory approval in any country where approval is pursued; the risk that more patient data become available that results in a different interpretation than the one derived from preliminary, interim or topline data; the Company's interactions with regulatory authorities, including the FDA and MHRA, which may affect the initiation, timing and progress of clinical trials and pathways and timing for regulatory approval; whether the measurements that the Company is evaluating are viewed as robust and sensitive measurements of disease progression suitable for regulatory approval; the Company's ability to conduct and fund a new study for AMT-130; the Company's ability to continue to build and maintain the 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; and the Company's ability to fund its operations. 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 the Company's Annual Reports on Form 10-K and Quarterly Reports on Form 10-Q, 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

	March 31,	**December 31,**

	2026	2025
	(in thousands, U.S. dollars)	
Current assets		
Cash and cash equivalents	$ 139,994	$ 80,240
Current investment securities	446,556	542,301
Accounts receivable	3,562	5,863
Prepaid expenses	16,589	20,506
Other current assets and receivables	9,043	7,076
Total current assets	**615,744**	**655,986**
Non-current assets		
Property, plant and equipment, net	$ 12,014	$ 13,800
Other investments	30,150	30,237
Operating lease right-of-use assets	12,261	12,525
Intangible assets, net	69,990	72,790
Goodwill	24,811	25,355
Deferred tax assets, net	8,194	8,654
Other non-current assets	5,542	5,561
Total non-current assets	**162,962**	**168,922**
Total assets	**$ 778,706**	**$ 824,908**
Current liabilities		
Accounts payable	$ 4,399	$ 5,170
Accrued expenses and other current liabilities	43,201	41,292
Liability related to pre-funded warrants	8,605	12,595
Current portion of operating lease liabilities	2,992	3,862
Total current liabilities	**59,197**	**62,919**
Non-current liabilities		
Long-term debt	49,942	49,699
Liability from royalty financing agreement	482,334	473,199
Operating lease liabilities, net of current portion	10,388	9,832
Contingent consideration	17,029	18,736
Deferred tax liability, net	7,796	7,967
Other non-current liabilities, net of current portion	2,677	3,655
Total non-current liabilities	**570,166**	**563,088**
Total liabilities	**629,363**	**626,007**
Shareholders' equity		
Total shareholders' equity	**149,343**	**198,901**
Total liabilities and shareholders' equity	**$ 778,706**	**$ 824,908**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended March 31,	
	2026	2025
	(in thousands, U.S dollars, except share and per share amounts)	
Total revenues	**$ 3,562**	**$ 1,567**
Operating expenses:		
Cost of license revenues	(219)	(197)
Research and development expenses	(29,176)	(36,140)
Selling, general and administrative expenses	(20,068)	(10,908)
Total operating expenses	**(49,463)**	**(47,245)**
Other income	1,632	8,306
Other expense	(1,451)	(1,959)
Loss from operations	**(45,720)**	**(39,331)**
Non-operating items, net	(7,327)	(3,810)
Loss before income tax expense	**$ (53,047)**	**$ (43,141)**
Income tax expense	(488)	(496)

Net loss	**$ (53,535)**	**$ (43,637)**
Basic and diluted net loss per ordinary share	$ (0.85)	$ (0.82)
Weighted average shares used in computing basic and diluted net loss per ordinary share	62,742,847	53,110,580

[1] Normal range (1.38 – 8.66 nmol); mean normal of 3.57 nmol