uniQure Announces 2025 Financial Results and Provides Recent Company Updates

March 2, 2026

~ Held Type A meeting with FDA to discuss AMT-130 for Huntington's disease; Company evaluating Phase III development considerations and plans to request follow-up Type B meeting in the second quarter of 2026 ~

~ Completed enrollment of the first cohort in the Phase I/IIa study of AMT-260 in refractory mesial temporal lobe epilepsy, with additional clinical data expected in the first half of 2026 ~

~ Presented updated Phase I/II data from AMT-191 in Fabry disease showing durable, dose-dependent increases in α-Gal A enzyme activity ~

~ Cash, cash equivalents and current investment securities of approximately $622.5 million as of December 31, 2025 expected to fund operations into the second half of 2029 ~

~ uniQure to host earnings call at 8:00 a.m. ET ~

LEXINGTON, Mass. and AMSTERDAM, March 02, 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 fourth quarter and full year of 2025 and highlighted recent progress across its business.

"In 2025, we presented compelling 36-month clinical data from AMT-130 that we believe meaningfully demonstrate its potential to become a first disease-modifying therapy for people living with Huntington's disease," said Matt Kapusta, chief executive officer at uniQure. "While we have not reached alignment with the FDA on an approval pathway, we remain confident in the strength and durability reflected in our dataset. We are committed to engaging with the FDA to define a clear and efficient path to bring this potentially transformative therapy to Huntington's disease patients in urgent need for treatments."

"Beyond Huntington's disease, we have also made meaningful progress across our broader clinical portfolio and look forward to additional data readouts later this year," continued Mr. Kapusta. "Entering 2026 with a strong balance sheet, we remain financially disciplined and well-positioned to continue advancing our programs strategically and responsibly."

Recent Company Developments and Updates

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

- In September 2025, the Company announced positive topline data from the pivotal Phase I/II study for AMT-130 for the treatment of Huntington's disease. Topline 36-month efficacy results for 12 patients receiving high-dose AMT-130 included the following (data cutoff as of June 30, 2025):
    - A statistically significant 75% slowing in disease progression measured by the composite Unified Huntington's Disease Rating Scale (cUHDRS) was observed which met the primary endpoint compared to a propensity score-matched external control (p=0.003).
    - A key secondary endpoint of Total Functional Capacity (TFC) demonstrated a statistically significant 60% slowing of disease progression compared to a propensity score-matched external control (p=0.033).
    - A mean reduction from baseline in cerebrospinal neurofilament light protein (NfL) of -8.2% was observed at 36 months in the high-dose of AMT-130 of the Phase I/II studies.
    - AMT-130 was generally well-tolerated across both doses, with a manageable safety profile and no new drug-related serious adverse events observed since December 2022. The most common adverse events in the treatment groups were related to the administration procedure.
- In the fourth quarter of 2025, dosing was completed in a fourth cohort of six patients receiving high-dose AMT-130 to evaluate safety and efficacy in patients with lower baseline striatal volumes compared to previous cohorts in the U.S. Phase I/II study.
- On February 23, 2026, the Company presented new analyses at the 21st Annual Huntington's Disease Therapeutics Conference in Palm Springs, California showing that propensity score methodology using clinical covariates with TRACK-HD/TRACK-ON and PREDICT-HD datasets effectively substitutes for baseline striatal volume in predicting Huntington's disease progression. These covariates were the same as those used in the 3-year data analysis to match AMT-130-treated patients to the external comparator cohort derived from Enroll-HD.
- The Company held a pre-BLA meeting with the U.S. Food and Drug Administration (FDA) in October 2025 and a Type A meeting in January 2026 to discuss the regulatory path forward. 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 FDA strongly recommended the Company conduct a prospective, randomized, double-blind,

sham surgery-controlled study. The Company intends to continue engaging with the FDA regarding Phase III development considerations and plans to request a Type B meeting in the second quarter of 2026 to further discuss potential study design approaches.

Continued clinical progress in pipeline programs

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

- In May 2025, the Company presented initial safety and exploratory efficacy data from the first treated patient showing a 92% reduction in seizure frequency observed through the first five months of follow up, with no serious safety events.
- In 2025, the Company completed enrollment in the first dose cohort of six patients in the Phase I/IIa study. Enrollment has been initiated in a second dose cohort expected to include an additional six patients.
- The Company expects to provide updated data from the Phase I/IIa study in the second quarter of 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 312.52-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.
    - Six of 11 dosed patients were withdrawn from enzyme replacement therapy (ERT).
    - Plasma lyso-Gb3 levels were stable post-dose across all cohorts, regardless of ERT status
- Based on data observed to date, AMT-191 showed 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)

- Enrollment remains on voluntary pause following the Independent Data Monitoring Committee recommendation after review of preliminary safety and efficacy data in September 2025, which included one serious adverse event of dose-limiting toxicity determined to be related to AMT-162. The Company continues to collect and evaluate data from the five patients treated in the Phase I/II EPISOD1 study.

Strengthened financial position

- In 2025, the Company received aggregate net proceeds of $404.2 million, after deducting underwriting discounts and commissions and other offering expenses, through the completion of follow-on public offerings in which a total of 11.8 million ordinary shares were issued, and in lieu of ordinary shares to certain investors, pre-funded warrants to purchase 0.5 million of the Company's ordinary shares.
- In the third quarter of 2025, the Company further announced the refinancing of its existing $50 million debt outstanding to extend the term to October 2030 and reduce its cost of capital. An additional term loan tranche of $100 million could be drawn down at the Company's option subject to the achievement of a pre-defined regulatory milestone for AMT-130. A third tranche of $25 million is available subject to the lender's approval.
- As of December 31, 2025, the Company had cash, cash equivalents and current investment securities of $622.5 million. The Company expects that cash, cash equivalents and investment securities will be sufficient to fund operations into the second half of 2029.

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

Financial Highlights

Cash Position: As of December 31, 2025, the Company held $622.5 million in cash, cash equivalents and current investment securities, compared to $367.5 million as of December 31, 2024. The net increase was primarily attributable to proceeds of approximately $404.2 million raised through public offerings of ordinary shares and pre-funded warrants.

Revenues: Revenue for the year ended December 31, 2025 was $16.1 million, compared to $27.1 million in 2024. The decrease of $11.0 million was primarily driven by a $10.7 million decrease in collaboration revenue and a $6.1 million decrease in contract manufacturing revenues, offset by a $5.8 million increase in license revenues.

Cost of Contract Manufacturing Revenues: Cost of contract manufacturing revenues was nil for the year ended December 31, 2025, compared to $17.1 million in 2024. Following the divestment of the Lexington facility in 2024, cost of contract manufacturing revenues are recorded net of the associated revenue within other expenses.

R&D Expenses: Research and development expenses were $140.7 million for the year ended December 31, 2025, compared to $143.8 million in 2024. The decrease of $3.1 million was primarily driven by a $26.0 million decrease in total other research and development expenses, $25.0 million of which related to decreases in employee, contractor-related and severance cost as well as facility cost resulting from the 2024 divestiture of the Company's Lexington manufacturing operation and organizational restructuring in the same year. This was offset by a $22.9 million increase in total direct research and development expenses of which $19.4 million related to the preparation of a potential Biologics License Application submission for AMT-130.

SG&A Expenses: Selling, general and administrative expenses were $65.5 million for the year ended December 31, 2025, compared to $52.7 million in 2024. The $12.8 million increase was primarily driven by a $9.4 million increase in professional fees, including $6.5 million incurred to support the preparation of the planned commercialization of AMT-130 in the United States, as well as a $3.6 million increase in employee and contractor-related expenses and a $2.8 million increase in other expenses. This was offset by a $1.8 million decrease in share-based compensation expenses and a $1.2 million decrease in severance costs.

Other Income: Other income was $14.4 million for the year ended December 31, 2025, compared to $7.9 million during the same period in 2024. The $6.5 million increase in 2025 was primarily related to a $6.0 million one-time sale of critical reagents to Genezen.

Other Expense: Other expense was $8.0 million for the year ended December 31, 2025, compared to $4.6 million in 2024. The $3.4 million increase primarily relates to a $3.0 million increase of expenses related to the supply of Hemgenix® to CSL Behring.

Non-Operating Items, Net: Total non-operating items, net were an expense of $8.0 million for the year ended December 31, 2025, compared to $52.8 million in 2024. The $44.8 million decrease in total non-operating expenses, net was primarily driven by a $36.6 million increase in net foreign currency result and a $10.9 million gain from changes in the fair value of the liability related to pre-funded warrants, net of issuance costs, offset by a $2.7 million increase in interest expense net of interest income.

Income Tax Expense: Income tax expense was $5.6 million for 2025, compared to $2.4 million in 2024. The $3.2 million increase relates to current tax expense incurred in relation to the tax treatment of the $375.0 million upfront payment (net of directly attributable expenses) from the 2023 royalty financing transaction.

Net loss: The net loss for the year ending December 31, 2025, was $199.0 million, or $3.46 basic and diluted loss per ordinary share, compared to a $239.6 million net loss for the same period in 2024, or $4.92 basic and diluted loss per ordinary share.

Upcoming investor events:

- TD Cowen 46th Annual Health Care Conference, March 2nd – Boston, MA
- Leerink Global Partners Global Healthcare Conference, March 11th – Miami, FL
- Barclays 28th Annual Global Healthcare Conference, March 12th – Miami, FL
- Kempen Life Science Conference, April 15th – Amsterdam, Netherlands

Investor Conference Call and Webcast Information

uniQure management will host an investor conference call and webcast today, Monday, March 2nd at 8:00 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, 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 half of 2029; AMT-130 as a potentially first disease-modifying therapy for people living with Huntington's disease; 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 regarding Phase III development considerations and request a Type B meeting with the FDA in the second quarter of 2026; the Company's ability and plans to strategically advance its programs; the Company's plans to enroll an additional six patients in a second cohort in the

Phase I/IIa study for AMT-260; the Company's plans for further clinical updates, including plans to announce additional data from the Company's AMT-260 program in the first half of 2026; the Company's plans to continue to collect data from patients in the EPISOD1 study; 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, 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 Phase III or confirmatory 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

	December 31, 2025	December 31, 2024
	(in thousands)	
Current assets		
Cash and cash equivalents	$ 80,240	$ 158,930
Current investment securities	542,301	208,591
Accounts receivable	5,863	5,881
Prepaid expenses	20,506	9,281
Other current assets and receivables	7,076	7,606
Total current assets	**655,986**	**390,289**
Non-current assets		
Property, plant and equipment, net	13,800	20,424
Other investments	30,237	27,464
Operating lease right-of-use assets	12,525	13,647
Intangible assets, net	72,790	71,043
Goodwill	25,355	22,414
Deferred tax assets, net	8,654	9,856
Other non-current assets	5,561	1,399
Total non-current assets	**168,922**	**166,247**
Total assets	**$ 824,908**	**$ 556,536**
Current liabilities		
Accounts payable	$ 5,170	$ 7,227
Accrued expenses and other current liabilities	41,292	29,225
Liability related to pre-funded warrants	12,595	—
Current portion of operating lease liabilities	3,862	3,601
Total current liabilities	**62,919**	**40,053**
Non-current liabilities		
Long-term debt	49,699	51,324
Liability from royalty financing agreement	473,199	434,930
Operating lease liabilities, net of current portion	9,832	11,136
Contingent consideration	18,736	10,860

Deferred tax liability, net	7,967	7,043
Other non-current liabilities, net of current portion	3,655	7,942
Total non-current liabilities	**563,088**	**523,235**
Total liabilities	**626,007**	**563,288**
Shareholders' equity / (deficit)		
Total shareholders' equity / (deficit)	**198,901**	**(6,752)**
Total liabilities and shareholders' equity / (deficit)	**$ 824,908**	**$ 556,536**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Years ended December 31,		
	2025	**2024**	**2023**
	(in thousands, except share and per share amounts)		
License revenues	$ 15,934	$ 10,133	**$** 2,758
Contract manufacturing revenues	—	6,114	10,835
Collaboration revenues	164	10,872	2,250
Total revenues	**16,098**	**27,119**	**15,843**
Operating expenses:			
Cost of license revenues	(1,686)	(1,267)	(65)
Cost of contract manufacturing revenues	—	(17,060)	(13,563)
Research and development expenses	(140,673)	(143,782)	(214,864)
Selling, general and administrative expenses	(65,456)	(52,657)	(74,591)
Total operating expenses	**(207,815)**	**(214,766)**	**(303,083)**
Other income	14,410	7,926	6,059
Other expense	(8,042)	(4,573)	(1,690)
Loss from operations	**(185,349)**	**(184,294)**	**(282,871)**
Non-Operating items, net	(7,991)	(52,833)	(23,686)
Loss before income tax expense	**$ (193,340)**	**$ (237,127)**	**$ (306,557)**
Income tax expense	(5,631)	(2,429)	(1,921)
Net loss	**$ (198,971)**	**$ (239,556)**	**$ (308,478)**
Earnings per ordinary share - diluted			
Diluted net loss per ordinary share	$ (3.46)	$ (4.92)	$ (6.47)
Weighted average shares - basic and diluted	57,502,068	48,649,129	47,670,986

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