uniQure Announces Third Quarter 2025 Financial Results and Provides Corporate Update

November 10, 2025

~ Announced pivotal topline data from Phase I/II study of AMT-130 in Huntington's disease met its primary and key secondary endpoints, demonstrating statistically significant slowing of disease progression at 36 months and supportive trends across key clinical and biomarker endpoints ~

~ Preliminary feedback from FDA at a recent pre-Biologics License Application (BLA) meeting for AMT-130 indicated a key shift from prior regulatory communications; uniQure plans to urgently interact with the FDA to define next steps ~

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

~ Presented initial data from Phase I/IIa study of AMT-191 in Fabry disease showing sustained increases in α-gal enzyme activity in patients with Fabry disease; additional clinical data expected in the first half of 2026 ~

~ Raised approximately $323.7 million in net proceeds in an upsized public follow-on offering, resulting in cash, cash equivalents and current investment securities of $694.2 million as of September 30, 2025 ~

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

LEXINGTON, Mass. and AMSTERDAM, Nov. 10, 2025 (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 third quarter of 2025 and highlighted recent progress across its business.

"The third quarter of 2025 marked a defining moment for uniQure as we presented our topline three-year data of AMT-130, an investigational gene therapy for Huntington's disease, that demonstrated statistically significant slowing of disease progression," said Matt Kapusta, chief executive officer of uniQure. "While the recent FDA feedback was unexpected and has introduced uncertainty in the timing of our BLA submission, we strongly believe that AMT-130 has the potential to bring substantial benefit for patients with Huntington's disease. We are fully committed to working with the FDA to determine the most expeditious path forward to rapidly bring AMT-130 to patients and their families in the U.S."

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 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=.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 in the Phase I/II studies with a manageable safety profile across both doses with 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.
- Data from the Phase I/II studies were presented at the 2025 Huntington's Disease Clinical Research Congress, which took place from October 10-13, 2025, in Nashville, Tennessee.
- In October 2025, the Company initiated and fully recruited a fourth cohort evaluating high-dose AMT-130 in six patients with lower striatal volumes compared to patients in previous cohorts. Patient dosing is expected to complete before year-end 2025.

- In October 2025, the Company held a pre-BLA meeting with the U.S. Food and Drug Administration (FDA) to discuss the planned BLA submission for AMT-130. Though final meeting minutes have not yet been received, based on discussions at the meeting, the Company believes that the FDA currently no longer agrees that data from the Phase I/II studies of AMT-130 in comparison to an external control, as per the prespecified protocols and statistical analysis plans shared with the FDA in advance of the analyses, may be adequate to provide the primary evidence in support of a BLA submission. Timing of the BLA submission remains unclear. Final meeting minutes are expected within 30 days of the meeting, and the Company plans to urgently interact with the FDA to find a path forward for the timely accelerated approval of AMT-130.

Advancing additional clinical programs towards proof-of-concept

- *AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (MTLE)* – In September 2025, the Company completed enrollment of the first three patients in the first cohort, which, following a positive Independent Data Monitoring Committee (IDMC) review, allowed for the expansion of the first cohort into mesial temporal lobe epilepsy in the dominant hemisphere and the initiation of the second cohort. The Company expects to provide updated data from the study in the first half of 2026.

- *AMT-191 for the treatment of Fabry disease* – In September 2025, the Company presented initial safety and exploratory efficacy data of the first four treated patients that showed between 27- to 208-fold increase in α-Gal A activity relative to mean normal range (1.38-8.66 nmol; mean normal of 3.57 nmol). All four patients were withdrawn from enzyme replacement therapy and maintained stable plasma lyso-Gb3 levels through the data cutoff date of July 24, 2025. Based on data observed to date, AMT-191 showed a manageable safety profile. A second, lower dose cohort of three patients completed enrollment and a third cohort of three patients is currently enrolling. The Company expects to present updated results from the Phase I/IIa clinical trial in the first half of 2026.

- *AMT-162 for the treatment of SOD1 amyotrophic lateral sclerosis (ALS)* – Following an IDMC recommendation after a September review of preliminary safety and efficacy data, the Company voluntarily paused enrollment in the study as a dose limiting toxicity, which resulted in a serious adverse event determined to be related to AMT-162, was observed in one patient in the second cohort. The Company will continue to collect and evaluate data from the five patients treated in the Phase I/II EPISOD1 study.

Strengthened financial position

- In September 2025, the Company completed multiple financing transactions designed to enhance financial flexibility.
    - The Company closed an upsized underwritten public offering raising net proceeds of approximately $323.7 million, including the full exercise of the underwriters' option to purchase additional shares as well as pre-funded warrants.
    - The Company also 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 September 30, 2025, the Company had cash, cash equivalents and investment securities of $694.2 million. The Company expects that cash, cash equivalents and investment securities will be sufficient to fund operations into 2029.

Financial Highlights

Cash position: As of September 30, 2025, the Company held cash, cash equivalents and current investment securities of $694.2 million, compared to $367.5 million as of December 31, 2024. The net increase was primarily attributable to proceeds of $404.2 million raised through public offerings of ordinary shares and pre-funded warrants.

Revenues: Revenue for the three months ended September 30, 2025 was $3.7 million, compared to $2.3 million in the same period in 2024. The increase of $1.4 million in revenue resulted from a $1.5 million increase in license revenues and a decrease of $0.1 million from collaboration revenues.

Cost of contract manufacturing revenues: Cost of contract manufacturing revenues were nil for the three months ended September 30, 2025, compared to $0.8 million for the same period in 2024. Following the divestment of the Lexington facility in July 2024, cost of contract manufacturing revenues are recorded net of revenue within other expenses.

R&D expenses: Research and development expenses were $34.4 million for the three months ended September 30, 2025, compared to $30.6 million during the same period in 2024. The $3.8 million increase was driven by an increase of $10.1 million in direct research and development expenses, of

which $6.6 million related to the preparation for the Biologics License Application submission for AMT-130, offset by a decrease of $3.4 million in severance costs and a $3.0 million decrease in costs related to disposables, facilities and other expenses.

SG&A expenses: Selling, general and administrative expenses were $19.4 million for the three months ended September 30, 2025, compared to $11.6 million during the same period in 2024. The $7.8 million increase was primarily related to a $2.4 million increase in employee-related expenses and a $4.9 million increase in professional fees, including $3.0 million incurred to support the preparation of a potential commercialization of AMT-130 in the United States.

Other income: Other income was $1.5 million for the three months ended September 30, 2025, compared to $2.6 million during the same period in 2024. The decrease was primarily related to a $1.2 million gain recorded on the divestment of the Lexington manufacturing facility in the prior period.

Other expense: Other expense was $2.0 million for the three months ended September 30, 2025, compared to $1.9 million during the same period in 2024.

Non-operating items, net: Non-operating items, net was an expense of $20.9 million for the three months ended September 30, 2025, compared to an expense of $4.2 million for the same period in 2024. The $16.7 million increase was primarily related to unfavorable foreign currency movements of $8.0 million, a decrease in interest income of $1.4 million, a $5.7 million loss driven by changes in the fair value of the liability related to the pre-funded warrants, and $1.5 million in issuance expenses related to the pre-funded warrants.

Income tax (expense) / benefit: Income tax expense was $8.6 million for the three months ended September 30, 2025, compared to an income tax benefit of $0.0 million recorded during the same period in 2024. The increase relates to current tax expense expected to be incurred in relation to recording in full the $375.0 million upfront payment from the 2023 royalty financing transaction as taxable income in 2023.

Net loss: The net loss for the three months ending September 30, 2025, was $80.5 million, or $1.38 basic and diluted loss per ordinary share, compared to a $44.4 million net loss for the same period in 2024, or $0.91 basic and diluted loss per ordinary share.

Upcoming investor events:

- Guggenheim 2nd Annual Healthcare Innovation Conference, November 12th – Boston, MA
- Stifel 2025 Healthcare Conference, November 13th – New York, NY

Investor Conference Call and Webcast Information

uniQure management will host an investor conference call and webcast today, Monday, November 10th 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 the passcode 2196195. 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 2029; the Company's plans and ability to progress AMT-130 in the U.S., including plans to interact with the FDA regarding AMT-130; the uncertainty in the timing of the Company's BLA submission for AMT-130; the timing and outcome of regulatory interactions with respect to the AMT-130 program, including the receipt of final minutes from the Company's pre-BLA meeting with the FDA; the Company's belief that the FDA no longer agrees that data from the Phase I/II studies of AMT-130 in comparison to an external control may be adequate to provide the primary evidence in support of a BLA submission; the Company's belief that AMT-130 has the potential to bring substantial benefit to patients; the completion of dosing in a fourth cohort evaluating high-dose AMT-130 before year-end 2025; the Company's anticipated growth; the expansion of the first cohort in the AMT-260 study into mesial temporal lobe epilepsy in the dominant hemisphere and plans to initiate a second cohort in the AMT-260 study; the enrollment of a third cohort in the Company's AMT-191 study ; the Company's plans for further clinical updates and plans to announce additional data in its AMT-191 and AMT-260 programs; 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 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; 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 its Annual Report on Form 10-K filed with the SEC on February 27, 2025, its Quarterly Reports on Form 10-Q filed with the SEC on May 9, 2025 and July 29, 2025, 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

	September 30, 2025	December 31, 2024
	(in thousands, U.S. dollars)	
Current assets		
Cash and cash equivalents	$ 597,056	$ 158,930
Current investment securities	97,189	208,591
Accounts receivable	3,883	5,881
Prepaid expenses	10,326	9,281
Other current assets and receivables	7,739	7,606
Total current assets	**716,193**	**390,289**
Non-current assets		
Property, plant and equipment, net	$ 15,402	$ 20,424
Other investments	29,972	27,464
Operating lease right-of-use assets	13,079	13,647
Intangible assets, net	74,144	71,043
Goodwill	25,327	22,414
Deferred tax assets, net	8,772	9,856
Other non-current assets	5,493	1,399
Total non-current assets	**172,189**	**166,247**
Total assets	**$ 888,382**	**$ 556,536**
Current liabilities		
Accounts payable	$ 6,228	$ 7,227
Accrued expenses and other current liabilities	51,947	28,932
Liability related to pre-funded warrants	30,722	-
Income taxes payable	7,821	293
Current portion of operating lease liabilities	3,914	3,601
Total current liabilities	**100,632**	**40,053**
Non-current liabilities		
Long-term debt	51,880	51,324
Liability from royalty financing agreement	465,507	434,930
Operating lease liabilities, net of current portion	10,361	11,136
Contingent consideration, net of current portion	17,754	10,860
Deferred tax liability, net	7,958	7,043
Other non-current liabilities, net of current portion	5,544	7,942
Total non-current liabilities	**559,004**	**523,235**
Total liabilities	**659,636**	**563,288**
Shareholders' equity / (deficit)		
Total shareholders' equity / (deficit)	**228,746**	**(6,752)**
Total liabilities and shareholders' equity / (deficit)	**$ 888,382**	**$ 556,536**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended September 30,	
	2025	**2024**
	(in thousands, U.S dollars, except share and per share amounts)	
Total revenues	**$ 3,701**	**$ 2,287**
Operating expenses:		
Cost of license revenues	(398)	(264)
Cost of contract manufacturing revenues	-	(757)
Research and development expenses	(34,366)	(30,595)
Selling, general and administrative expenses	(19,438)	(11,575)
Total operating expenses	**(54,202)**	**(43,191)**
Other income	1,510	2,591
Other expense	(2,044)	(1,915)
Loss from operations	**(51,035)**	**(40,228)**
Non-operating items, net	(20,868)	(4,181)
Loss before income tax expense	**$ (71,903)**	**$ (44,409)**
Income tax (expense) / benefit	(8,626)	31
Net loss	**$ (80,529)**	**$ (44,378)**
Basic and diluted net loss per ordinary share	$ (1.38)	$ (0.91)
Weighted average shares used in computing basic and diluted net loss per ordinary share	58,516,415	48,718,533