



## uniQure Announces First Quarter 2025 Financial Results and Highlights of Recent Company Progress

May 9, 2025

~ AMT-130 granted Breakthrough Therapy designation by FDA ~

~ Initial safety data from third cohort of Phase I/II study show AMT-130 continues to be generally well-tolerated, with no treatment-related serious adverse events ~

~ Held Type B FDA meetings in the first and second quarters of 2025 to advance BLA preparations for AMT-130; regulatory update expected in the second quarter of 2025 ~

~ AMT-260 clinical data from first patient to be presented at Epilepsy Therapies & Diagnostics Development Symposium on May 29, 2025 ~

~ Cash, cash equivalents and current investment securities of approximately \$409.0 million as of March 31, 2025 expected to fund operations into second half of 2027 ~

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

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, May 09, 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 first quarter of 2025 and highlighted recent progress across its business.

"We continued to have constructive interactions with the U.S. Food and Drug Administration (FDA) in the first and second quarters of 2025, enabling us to further advance AMT-130 towards a planned Biologics License Application (BLA) submission," said [Matt Kapusta, chief executive officer of uniQure](#). "The recently granted Breakthrough Therapy designation is a significant milestone and underscores the FDA's continued engagement with AMT-130 as a potential disease-modifying treatment for Huntington's disease. We expect to provide a regulatory update in the second quarter, including guidance on the potential timing of our BLA submission for AMT-130, and anticipate sharing new clinical data from our AMT-130 Phase I/II trials in the third quarter. As we prepare for potential commercialization, we remain committed to making financially disciplined investments."

Mr. Kapusta continued, "In addition to AMT-130, it's important to highlight the continued progress of our broader pipeline of investigational gene therapies. We look forward to sharing initial data from the first patient treated with AMT-260 for refractory mesial temporal lobe epilepsy later this quarter, and we remain on track to present initial data from our AMT-191 study in Fabry disease in the second half of the year. With our strong financial position, we believe that we are well-resourced to execute across our pipeline and deliver meaningful value to patients and shareholders alike."

### Recent Company Developments and Updates

- *Advancing AMT-130 Toward Accelerated Approval for Huntington's disease*
  - In April 2025, the FDA granted Breakthrough Therapy designation to AMT-130 based on clinical evidence from Phase I/II trials showing the potential for slowing progression of Huntington's disease. The designation followed a November 2024 multidisciplinary meeting with the FDA during which key elements of an Accelerated Approval pathway for AMT-130 were discussed and aligned.
  - In March 2025, the Company held a Type B meeting with the FDA to discuss CMC requirements in support of a planned BLA submission. An additional Type B meeting focused on the pivotal statistical analysis plan was held in the second quarter of 2025. The Company expects to provide a regulatory update, including the potential timing of a BLA submission, after the receipt of official meeting minutes.
  - In February 2025, the Company completed enrollment of all 12 patients in the third cohort of the Phase I/II study. Patients were blinded and randomized to receive either the high (n=6) or low dose (n=6) of AMT-130 in conjunction with an immunosuppression regimen.

Key observations include:

- AMT-130 was generally well-tolerated at both dose levels;
- No treatment-related serious adverse events were reported;
- Three serious adverse events related to immunosuppression (mania, infection, and fever) were observed; all resolved fully with supportive care and;
- Perioperative changes in CSF NfL were consistent with previously reported observations, reinforcing their association with the surgical administration.

The Company plans to review these data with outside advisors and does not anticipate any impact on the potential timing of a BLA submission.

- In the third quarter of 2025, the Company expects to present data from its ongoing Phase I/II studies of AMT-130 in support of a potential BLA submission. 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 data on 24 treated patients.
- *Advancing additional clinical programs to proof-of-concept*
  - *AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (mTLE)* – The Company continues to screen patients across 12 clinical sites, with plans to activate additional centers in the second half of the year. The Company expects to present initial safety and exploratory efficacy data from the first treated patient at the Epilepsy Therapies & Diagnostics Development Symposium on Thursday, May 29, 2025.
  - *AMT-191 for the treatment of Fabry disease* – In February 2025, the Company announced a favorable recommendation from the independent data monitoring committee (IDMC) following a review of safety data from the first two patients treated in the Phase I/IIa study. The Company has treated a total of four patients in the first dose cohort. The Company plans to present initial data from the study in the second half of 2025.
  - *AMT-162 for the treatment of SOD1 amyotrophic lateral sclerosis (ALS)* – In January 2025, the Company announced a favorable recommendation from the IDMC based on the review of initial safety data from the first study cohort in the Phase I/II EPISOD1 study. The Company has completed enrollment in a second dose cohort and expects to initiate enrollment in the third dose cohort in the third quarter of 2025. The Company expects to present initial data from the study in the first half of 2026.
- *Strong financial position*
  - In the first quarter of 2025, the Company completed a public offering of 5.1 million ordinary shares, including the full exercise of the underwriters' overallotment option, at a public offering price of \$17.00 per share for net proceeds of approximately \$80.5 million after deducting underwriting discounts and commissions and offering expenses.

## Financial Highlights

**Cash position:** As of March 31, 2025, the Company held cash, cash equivalents and current investment securities of \$409.0 million, compared to \$367.5 million as of December 31, 2024. The increase in cash was primarily related to net proceeds of \$80.5 million from the follow-on offering in January and February 2025. Based on the Company's current operating plan, including the planned U.S. launch of AMT-130, the Company expects cash, cash equivalents and current investment securities will be sufficient to fund operations into the second half of 2027.

**Revenues:** Revenue for the three months ended March 31, 2025 was \$1.6 million, compared to \$8.5 million in the same period in 2024. The decrease of \$6.9 million in revenue resulted from a \$0.4 million increase in license revenue, a decrease of \$3.3 million from collaboration revenue, and a decrease of \$4.0 million from contract manufacturing of HEMGENIX<sup>®</sup> for CSL Behring. Following the divestment of the Lexington facility in July 2024, revenue from contract manufacturing is recorded net of cost within other expenses.

**Cost of contract manufacturing revenues:** Cost of contract manufacturing revenues were nil for the three months ended March 31, 2025, compared to \$9.1 million for the same period in 2024. Following the divestment of the Lexington facility in July 2024, cost of contract manufacturing is recorded net of revenue within other expenses.

**R&D expenses:** Research and development expenses were \$36.1 million for the three months ended March 31, 2025, compared to \$40.7 million during the same period in 2024. The \$4.6 million decrease was related to a decrease of \$7.7 million in employee-related expenses, a decrease of \$2.5 million in facility expenses and a \$1.1 million decrease in costs related to preclinical supplies. This was offset by a net increase of \$7.1 million in external program spend and \$1.1 million higher expenses related to an increase in the fair value of contingent consideration.

**SG&A expenses:** Selling, general and administrative expenses were \$10.9 million for the three months ended March 31, 2025, compared to \$13.9 million during the same period in 2024. The \$3.0 million decrease was primarily related to a \$2.2 million decrease in employee-related expenses and a \$0.3 million decrease in intellectual property fees compared to the prior year period.

**Other income:** Other income was \$8.3 million for the three months ended March 31, 2025, compared to \$1.4 million during the same period in 2024. The increase was primarily related to a one-time gain of \$6.0 million related to the sale of critical reagents.

**Other expense:** Other expense was \$2.0 million for the three months ended March 31, 2025, compared to \$0.2 million during the same period in 2024. The increase was primarily related to \$1.4 million of non-cash expense recognized to amortize the right to purchase HEMGENIX<sup>®</sup> from Genezen on favorable terms.

**Other non-operating items, net:** Other non-operating items, net was an expense of \$3.8 million for the three months ended March 31, 2025, compared to an expense of \$10.7 million for the same period in 2024. The \$6.9 million decrease in other non-operating items, net was primarily related to a decrease in interest income of \$2.4 million, a reduction of interest expense of \$1.0 million due to the \$50.0 million repayment of Hercules debt in July 2024, and an increase in net foreign currency gains of \$8.3 million.

**Net loss:** The net loss for the three months ending March 31, 2025, was \$43.6 million, or \$0.82 basic and diluted loss per ordinary share, compared to

a \$65.6 million net loss for the same period in 2024, or \$1.36 basic and diluted loss per ordinary share.

## Investor Conference Call and Webcast Information

uniQure management will host an investor conference call and webcast today, Friday, May 9 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. Interested parties participating by phone will need to register using [this online form](#). After registering for dial-in details, all phone participants will receive an auto-generated e-mail containing a link to the dial-in number along with a personal PIN number to use to access the event by phone. If you are joining the conference call, please dial in 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](http://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 2027; the outcome of regulatory interactions with respect to the AMT-130 program, FDA's support for the program and the Company's plans to provide further regulatory updates, including timing of its planned BLA submission; whether AMT-130 continues to be well tolerated when dosed with immunosuppression and the Company's review of data from the third cohort of AMT-130; the Company's commercialization plans; and the Company's plans for further clinical updates and plans to announce additional interim data in its AMT-130, AMT-260, AMT-191 and AMT-162 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 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 regulatory 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; and the Company's ability to fund its operations and to raise additional capital as needed. 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 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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## uniQure N.V.

### UNAUDITED CONSOLIDATED BALANCE SHEETS

	March 31, 2025	December 31, 2024
(in thousands, except share and per share amounts)		
<b>Current assets</b>		
Cash and cash equivalents	\$ 217,229	\$ 158,930
Current investment securities	191,782	208,591
Accounts receivable	2,522	5,881
Prepaid expenses	13,420	9,281
Other current assets and receivables	10,468	7,606
<b>Total current assets</b>	<b>435,421</b>	<b>390,289</b>
<b>Non-current assets</b>		
Property, plant and equipment, net	18,081	20,424
Other investments	28,273	27,464
Operating lease right-of-use assets	13,466	13,647
Intangible assets, net	71,992	71,043

Goodwill	23,351	22,414
Deferred tax assets, net	9,390	9,856
Other non-current assets	5,429	1,399
<b>Total non-current assets</b>	<b>169,982</b>	<b>166,247</b>
<b>Total assets</b>	<b>\$ 605,403</b>	<b>\$ 556,536</b>
<b>Current liabilities</b>		
Accounts payable	\$ 5,268	\$ 7,227
Accrued expenses and other current liabilities	27,344	29,225
Current portion of operating lease liabilities	3,698	3,601
<b>Total current liabilities</b>	<b>36,310</b>	<b>40,053</b>
<b>Non-current liabilities</b>		
Long-term debt	51,592	51,324
Liability from royalty financing agreement	446,660	434,930
Operating lease liabilities, net of current portion	10,906	11,136
Contingent consideration, net of current portion	12,560	10,860
Deferred tax liability, net	7,338	7,043
Other non-current liabilities	6,351	7,942
<b>Total non-current liabilities</b>	<b>535,407</b>	<b>523,235</b>
<b>Total liabilities</b>	<b>571,717</b>	<b>563,288</b>
<b>Shareholders' equity / (deficit)</b>		
<b>Total shareholders' equity / (deficit)</b>	<b>33,686</b>	<b>(6,752)</b>
<b>Total liabilities and shareholders' equity / (deficit)</b>	<b>\$ 605,403</b>	<b>\$ 556,536</b>

uniQure N.V.  
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended March 31,	
	2025	2024
	(in thousands, except share and per share amounts)	
<b>Total revenues</b>	<b>\$ 1,567</b>	<b>\$ 8,485</b>
<b>Operating expenses:</b>		
Cost of license revenues	(197)	(150)
Cost of contract manufacturing revenues	-	(9,076)
Research and development expenses	(36,140)	(40,692)
Selling, general and administrative expenses	(10,908)	(13,937)
<b>Total operating expenses</b>	<b>(47,245)</b>	<b>(63,855)</b>
Other income	8,306	1,376
Other expense	(1,959)	(234)
<b>Loss from operations</b>	<b>(39,331)</b>	<b>(54,228)</b>
Non-operating items, net	(3,810)	(10,734)
<b>Loss before income tax expense</b>	<b>\$ (43,141)</b>	<b>\$ (64,962)</b>
Income tax expense	(496)	(656)
<b>Net loss</b>	<b>\$ (43,637)</b>	<b>\$ (65,618)</b>
Basic and diluted net loss per ordinary share	\$ (0.82)	\$ (1.36)
Weighted average shares used in computing basic and diluted net loss per ordinary share	53,110,580	48,384,510

**uniQure**