



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

July 29, 2025

~ Achieved alignment with the FDA on the AMT-130 statistical analysis plan and CMC requirements to support a planned BLA submission in the first quarter of 2026;

~ Company expects to present AMT-130 topline three-year data in September 2025 ~

~ Presented case study from first participant treated with AMT-260 for refractory mesial temporal lobe epilepsy showing 92% seizure reduction with no serious adverse events through first five months of follow up ~

~ Appointment of Kylie O'Keefe as Chief Customer and Strategy Officer ~

~ Cash, cash equivalents and current investment securities of approximately \$377.0 million as of June 30, 2025 are expected to fund operations into second half of 2027 ~

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

LEXINGTON, Mass. and AMSTERDAM, July 29, 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 second quarter of 2025 and highlighted recent progress across its business.

"We delivered tremendous progress across our business in the first half of 2025, setting the stage for a transformative period ahead for uniQure," said [Matt Kapusta, chief executive officer of uniQure](#). "With alignment from the FDA on a biological license application (BLA) pathway and pivotal topline data expected in September, AMT-130 is well-positioned to potentially become the first disease-modifying therapy for people living with Huntington's disease."

"At the same time, we're encouraged by early clinical signals from the first patient treated with AMT-260 in refractory mesial temporal lobe epilepsy and the continued advancement of our Fabry and ALS programs," continued Mr. Kapusta. "Backed by a strong balance sheet and an exceptional team, we believe that we are well prepared to execute on the opportunities ahead, including the planned commercialization of AMT-130, and delivering lasting value to patients and shareholders."

Recent Company Developments and Updates

- *Alignment with FDA continues to support Accelerated Approval pathway for AMT-130*
 - In June 2025, the Company announced its alignment with the FDA on the statistical analysis plan (SAP) and Chemistry, Manufacturing and Controls (CMC) requirements to support a planned Biologics License Application (BLA) submission for AMT-130 in the first quarter of 2026. The primary efficacy analysis will evaluate the three-year change in the composite Unified Huntington's Disease Rating Scale (cUHDRS) in patients receiving the high dose of AMT-130 compared to patients in a propensity score-matched external control arm derived from the ENROLL-HD data set. Certain sensitivity analyses, including one using a propensity score-weighted methodology, will also be provided. The FDA also agreed to uniQure's approach of leveraging prior knowledge and manufacturing experience from the HEMGENIX[®] process, supplemented with additional full-scale Good Manufacturing Practices (GMP) batches and a single Process Performance Qualification (PPQ) run.
 - In July 2025, the Company submitted the final SAP to the FDA. Additionally, the Company initiated its PPQ campaign to support the planned BLA submission.
 - In May 2025, the Company announced key safety observations from the third cohort of the Phase I/II study evaluating the impact of prophylactic immunosuppression in 12 patients receiving AMT-130. AMT-130 was generally well-tolerated at both doses with no treatment-related serious adverse events. Three serious adverse events related to immunosuppression were observed and resolved with supportive care. The Company expects a short-term steroid regimen will be incorporated as a prophylactic immunosuppression strategy.
 - 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 Company continues to prepare for a BLA submission of AMT-130 in the treatment of Huntington's disease. Certain next steps and expected timing include:
 - September 2025: Present topline, three-year data from the ongoing Phase I/II trials.
 - Q3 2025: Initiate a fourth cohort evaluating high-dose AMT-130 in six patients with lower striatal volumes

compared to those of patients enrolled in previous cohorts.

- Q4 2025: Hold pre-BLA meeting with FDA and complete PPQ campaign.
- Q1 2026: Submit BLA with a request for priority review designation.

- *Advancing additional clinical programs to proof-of-concept*

- *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. The 14 clinical sites in the U.S. continue to screen patients for this study.
- *AMT-191 for the treatment of Fabry disease* – The Company continues to enroll patients in the Phase I/IIa study and expects to present initial safety and exploratory efficacy data at the 15th ICIEM 2025 in Kyoto, Japan on Friday, September 5, 2025.
- *AMT-162 for the treatment of SOD1 amyotrophic lateral sclerosis (ALS)* – The Company continues to enroll patients in the Phase I/II EPISOD1 study and expects to present initial data in the first half of 2026.

- *Strengthening the leadership team*

- In June 2025, Kylie O’Keefe was appointed as Chief Customer and Strategy Officer, responsible for leading all commercial and medical affairs at uniQure. Ms. O’Keefe brings decades of commercial experience to the company having most recently served as Chief Commercial Officer at PTC Therapeutics, where she led global commercial strategy, operations and portfolio management for multiple rare neurology and metabolic commercial products across more than 50 countries.

- *Strong financial position*

- As of June 30, 2025, the Company had cash, cash equivalents and investment securities of \$377.0 million. The Company expects that cash, cash equivalents and investment securities will be sufficient to fund operations into the second half of 2027, including the launch of AMT-130.

Financial Highlights

Cash position: As of June 30, 2025, the Company held cash, cash equivalents and current investment securities of \$377.0 million, compared to \$367.5 million as of December 31, 2024. The net 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 June 30, 2025 was \$5.3 million, compared to \$11.1 million in the same period in 2024. The decrease of \$5.8 million in revenue resulted from a \$3.4 million increase in license revenue, a decrease of \$7.1 million from collaboration revenue, and a decrease of \$2.1 million from contract manufacturing of HEMGENIX[®] 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 June 30, 2025, compared to \$7.2 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 \$35.4 million for the three months ended June 30, 2025, compared to \$33.7 million during the same period in 2024. The \$1.7 million increase was related to a net increase of \$6.3 million in external program spend and \$4.0 million higher expenses related to an increase in the fair value of contingent consideration. This was offset by a decrease of \$4.7 million in employee-related expenses, a decrease of \$2.1 million in facility expenses and a \$1.8 million decrease in costs related to preclinical supplies and other expenses.

SG&A expenses: Selling, general and administrative expenses were \$13.5 million for the three months ended June 30, 2025, compared to \$15.8 million during the same period in 2024. The \$2.3 million decrease was primarily related to a \$1.6 million decrease in employee-related expenses and a \$0.6 million decrease in professional fees compared to the prior year period.

Other expense: Other expense was \$2.2 million for the three months ended June 30, 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[®] from Genezen on favorable terms.

Other non-operating items, net: Other non-operating items, net was an income of \$6.6 million for the three months ended June 30, 2025, compared to an expense of \$11.3 million for the same period in 2024. The \$17.9 million increase in other non-operating items, net was primarily related to an increase in net foreign currency gains of \$19.6 million, a decrease in interest income of \$2.3 million and a reduction of interest expense of \$0.6 million due to the \$50.0 million repayment of Hercules debt in July 2024.

Net loss: The net loss for the three months ending June 30, 2025, was \$37.7 million, or \$0.69 basic and diluted loss per ordinary share, compared to a

\$56.3 million net loss for the same period in 2024, or \$1.16 basic and diluted loss per ordinary share.

Investor Conference Call and Webcast Information

uniQure management will host an investor conference call and webcast today, Tuesday, July 29 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

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 timing and 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 the timing of its planned BLA submission and anticipated request for priority review designation; the Company's plans to incorporate a course of steroids as an immunosuppression strategy for patients receiving AMT-130; the Company's plans to announce AMT-130 topline three-year data in September 2025; the initiation of a fourth cohort evaluating high-dose AMT-130; the Company's commercialization plans, including with respect to AMT-130; and the Company's plans for further clinical updates and plans to announce initial data in its 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 and timing for 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, its Quarterly Report on Form 10-Q filed with the SEC on May 9, 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.

uniQure Contacts:

FOR INVESTORS:

Chiara Russo

Direct: 781-491-4371

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

	June 30, 2025	December 31, 2024
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	\$ 253,778	\$ 158,930
Current investment securities	123,196	208,591
Accounts receivable	5,606	5,881
Prepaid expenses	18,538	9,281
Other current assets and receivables	7,455	7,606
Total current assets	408,573	390,289
Non-current assets		
Property, plant and equipment, net	17,492	20,424

Other investments	29,735	27,464
Operating lease right-of-use assets	13,635	13,647
Intangible assets, net	75,604	71,043
Goodwill	25,340	22,414
Deferred tax assets, net	9,017	9,856
Other non-current assets	5,494	1,399
Total non-current assets	176,317	166,247
Total assets	\$ 584,890	\$ 556,536
Current liabilities		
Accounts payable	\$ 5,541	\$ 7,227
Accrued expenses and other current liabilities	31,479	29,225
Current portion of operating lease liabilities	3,909	3,601
Total current liabilities	40,929	40,053
Non-current liabilities		
Long-term debt	51,842	51,324
Liability from royalty financing agreement	455,259	434,930
Operating lease liabilities, net of current portion	10,946	11,136
Contingent consideration, net of current portion	15,931	10,860
Deferred tax liability, net	7,963	7,043
Other non-current liabilities	6,023	7,942
Total non-current liabilities	547,964	523,235
Total liabilities	588,893	563,288
Shareholders' (deficit) / equity		
Total shareholders' (deficit) / equity	(4,003)	(6,752)
Total liabilities and shareholders' (deficit) / equity	\$ 584,890	\$ 556,536

uniQure N.V.
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended June 30,	
	2025	2024
	(in thousands, except share and per share amounts)	
Total revenues	\$ 5,262	\$ 11,126
Operating expenses:		
Cost of license revenues	(656)	(234)
Cost of contract manufacturing revenues	-	(7,227)
Research and development expenses	(35,383)	(33,655)
Selling, general and administrative expenses	(13,500)	(15,767)
Total operating expenses	(49,539)	(56,883)
Other income	2,597	1,983
Other expense	(2,185)	(236)
Loss from operations	(43,865)	(44,010)
Non-operating items, net	6,571	(11,341)
Loss before income tax (expense) / benefit	\$ (37,294)	\$ (55,351)
Income tax (expense) / benefit	(425)	(948)
Net loss	\$ (37,719)	\$ (56,299)
Basic and diluted net loss per ordinary share	\$ (0.69)	\$ (1.16)
Weighted average shares used in computing basic and diluted net loss per ordinary share	54,807,967	48,622,440

uniQure