



## uniQure Announces 2024 Financial Results and Highlights Recent Company Progress

February 27, 2025

~ Announced alignment with the U.S. Food and Drug Administration (FDA) on key elements of the Accelerated Approval pathway for AMT-130 in Huntington's disease; Initiated preparations for a potential Biologics License Application (BLA) submission ~

~ Completed patient enrollment in the third cohort of the Phase I/II study of AMT-130 ~

~ Initiated dosing of the Phase I/II study of AMT-260 in mesial temporal lobe epilepsy (mTLE); Implementing protocol changes to expand study inclusion criteria ~

~ Completed enrollment of the first cohorts in the Phase I/II studies of AMT-191 in Fabry disease and AMT-162 in SOD1-ALS; Received favorable recommendations from the respective Independent Data Monitoring Committees (IDMC) to proceed with dosing the second cohorts ~

~ Cash and cash equivalents of approximately \$367.5 million as of December 31, 2024, combined with \$80.7 million in net proceeds from the recently completed financing, are expected to fund operations into the second half of 2027 ~

LEXINGTON, Mass. and AMSTERDAM, Feb. 27, 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 fourth quarter and full year of 2024 and highlighted recent progress across its business.

"This past year was transformative for uniQure, marked by significant clinical and operational progress," stated [Matt Kapusta, chief executive officer of uniQure](#). "On the clinical front, we made great strides advancing AMT-130 for Huntington's disease, including securing alignment with the FDA on key elements of the Accelerated Approval pathway, a major milestone that brings us closer to delivering the first potentially disease-modifying treatment for this devastating condition. With this regulatory clarity, we have initiated BLA-readiness activities and look forward to further engagement with the FDA throughout the first half of 2025. Our RMAT designation has enabled a productive and expedited dialogue with the FDA, which we will continue leveraging to advance AMT-130 through the regulatory process as rapidly as possible. This is welcome news for patients awaiting further development of our groundbreaking therapies."

Mr. Kapusta continued, "Beyond AMT-130, we continue to advance our broader pipeline of investigational gene therapies, with patient enrollment progressing in the Phase I/II studies of AMT-191 for Fabry disease and AMT-162 for SOD1-ALS. Additionally, we are implementing FDA-approved protocol changes to the Phase I/II study of AMT-260 in mTLE, including broadening the inclusion criteria for certain patients in the first cohort – a step we anticipate will help accelerate trial enrollment."

"Operationally, we took decisive steps in 2024 to streamline our organization, including the sale of our Lexington manufacturing facility and a company-wide restructuring that significantly reduced our cash burn and strengthened our financial position," Mr. Kapusta continued. "These strategic actions enable us to prioritize investments and achieve multiple value-creating milestones, including the potential approval and commercial launch of AMT-130."

### Recent Company Developments and Updates

- *Pursuing Accelerated Approval of AMT-130 for the treatment of Huntington's disease*
  - In May 2024, the FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation for AMT-130, stating that preliminary clinical evidence indicates that AMT-130 has the potential to address unmet medical needs for treatment of Huntington's disease.
  - In December 2024, uniQure reached agreement with the FDA on key elements of an Accelerated Approval pathway for AMT-130 in Huntington's disease. As part of the RMAT Type B meeting, the FDA agreed that data from the ongoing Phase I/II studies, compared to a natural history external control, may serve as the primary basis for a BLA submission, eliminating the need for an additional pre-submission study. The FDA also agreed that the composite Unified Huntington's Disease Rating Scale (cUHDRS) may be used as an intermediate clinical endpoint and reductions in neurofilament light chain (NfL) in the cerebrospinal fluid (CSF) may serve as supportive evidence of therapeutic benefit.
  - The Company has scheduled a Type B meeting with the FDA in the first quarter of 2025 to discuss chemistry, manufacturing and control (CMC) requirements to support its planned BLA submission. A separate Type B meeting to discuss the pivotal statistical analysis plan for the BLA is anticipated to take place in the second quarter of 2025. After the completion of these interactions, the Company expects to provide a regulatory update in the second quarter of 2025, including the expected timing of a potential BLA submission.
  - In February 2025, the Company completed enrollment of all 12 patients in the third cohort investigating an

optimized immunosuppression regimen. The Company expects to provide an initial safety update on the third cohort in the second quarter of 2025.

- 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 on 24 treated patients.
- *Advancing additional clinical programs to proof-of-concept*
  - *AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (mTLE)* – In November 2024, the Company announced the first patient dosed in the Phase I/II clinical trial of AMT-260 for the treatment of mTLE. The FDA recently approved a protocol amendment expanding the inclusion criteria for certain patients in the first cohort to include patients with non-lesional mesial temporal lobe epilepsy in the non-dominant hemisphere. This broader inclusion criteria may assist in accelerated enrollment. The Company expects to present initial data from the study in the first half of 2026.
  - *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 28-day safety data from the first study cohort in the Phase I/II EPISODE1 study. The Company initiated enrollment in the second dose cohort in the first quarter of 2025 and expects to present initial data from the study in the first half of 2026.
  - *AMT-191 for the treatment of Fabry disease* – In February 2025, the Company announced completion of enrollment in the first cohort in the Phase I/IIa clinical trial of AMT-191 and a favorable recommendation from the IDMC having reviewed safety data from the initial two patients. The Company expects to initiate enrollment in the second dose cohort in the second quarter of 2025 and to present initial data from the study in the second half of 2025.
- *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 price of \$17.00 per share. Net proceeds from the offering are expected to fund operations into the second half of 2027, including the potential BLA submission and U.S. commercial launch of AMT-130.
  - In the second quarter of 2024, the Company completed the sale of its Lexington, MA manufacturing facility to Genezen and retired \$50 million of its outstanding debt with Hercules Capital.
  - In the third quarter of 2024, the Company announced an organization restructuring which, combined with the Lexington facility sale, eliminated approximately 65% of the global workforce and reduced recurring cash burn by approximately \$70 million per year.

#### Upcoming Investor Events

- TD Cowen 45<sup>th</sup> Annual Healthcare Conference, March 3<sup>rd</sup> – Boston, MA
- Leerink's Global Healthcare Conference 2025, March 10<sup>th</sup> – Miami, FL
- Kempen Life Sciences Conference, April 3<sup>rd</sup> – Amsterdam, NL

#### Financial Highlights

**Cash position:** As of December 31, 2024, the Company held cash, cash equivalents and investment securities of \$367.5 million, compared to \$617.9 million as of December 31, 2023. Including the net proceeds of \$80.7 million from the recently completed follow-on offering, the Company's proforma cash, cash equivalents and investment securities was approximately \$448 million. The reduction in cash was in part driven by non-recurring payments, including \$53 million related to the retirement of debt, \$31.5 million related to milestone payments, \$12.0 million of one-time payments related to the divestment of the Lexington facility transaction, and \$4.7 million of severance payments related to the Company's restructuring. Based on the Company's current operating plan, including the planned U.S. launch of AMT-130, the Company expects cash, cash equivalents and investment securities will be sufficient to fund operations through the second half of 2027.

**Revenues:** Revenue for the year ended December 31, 2024 was \$27.1 million, compared to \$15.8 million in the same period in 2023. The increase of \$11.3 million in revenue resulted from a \$7.4 million increase in license revenue, an increase of \$8.6 million from collaboration revenue, and a decrease of \$4.7 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 \$17.1 million for the year ended December 31, 2024,

compared to \$13.6 million for the same period in 2023. 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 \$143.8 million for the year ended December 31, 2024, compared to \$214.9 million during the same period in 2023. The \$71.1 million decrease was related to a decrease of \$30.5 million in employee-related expenses, \$17.7 million lower expenses related to changes in the fair value of contingent consideration, a net decrease of \$8.3 million in external program spend and an \$8.1 million decrease in costs related to preclinical supplies.

**SG&A expenses:** Selling, general and administrative expenses were \$52.7 million for the year ended December 31, 2024, compared to \$74.6 million during the same period in 2023. The \$21.9 million decrease was primarily related to a \$7.6 million decrease in employee-related expenses, a decrease of \$5.4 million in information technology costs, a \$4.4 million decrease in professional fees and a \$2.1 million decrease in intellectual property fees and compared to the prior year period.

**Other income:** Other income was \$7.9 million for the year ended December 31, 2024, compared to \$6.1 million during the same period in 2023. The increase was primarily related to the \$1.2 million gain recorded on divesting the Lexington manufacturing facility.

**Other expense:** Other expense was \$4.6 million for the year ended December 31, 2024, compared to \$1.7 million during the same period in 2023. The increase was primarily related to \$2.5 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 expense of \$52.8 million for the year ended December 31, 2024, compared to \$23.7 million for the same period in 2023. The \$29.1 million increase in other non-operating items, net was primarily related to an increase in non-cash interest expense of \$23.9 million related to the royalty agreement that the Company entered into in May 2023 and an increase in net foreign currency losses of \$8.8 million.

**Net loss:** The net loss for the year ended December 31, 2024, was \$239.6 million, or \$4.92 basic and diluted loss per ordinary share, compared to \$308.5 million net loss for the same period in 2023, or \$6.47 basic and diluted loss per ordinary share.

#### 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 and the planned use of proceeds from its first quarter 2025 public offering; the Company's plans for further interactions with the FDA to discuss the requirements for its planned BLA submission for AMT-130; the Company's ability to utilize an accelerated pathway to progress AMT-130 through regulatory approval; the Company's plans to announce additional interim data and regulatory updates from its ongoing Phase I/II clinical studies of AMT-130, along with an initial safety update on the third cohort of the AMT-130 study and other program updates; the effectiveness of planned protocol changes in accelerating enrollment in the AMT-260 study; and the Company's organizational restructuring and other actions designed to increase shareholder value and fund its pipeline of gene therapy candidates. 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 to be filed 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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**UNAUDITED CONSOLIDATED BALANCE SHEETS**

	<u>December 31,</u> <u>2024</u>	<u>December 31,</u> <u>2023</u>
	<u>(in thousands, except share and per share amounts)</u>	
<b>Current assets</b>		
Cash and cash equivalents	\$ 158.930	\$ 241.360
Current investment securities	208.591	376.532
Accounts receivable	5.881	4.193
Inventories, net	—	12.024
Prepaid expenses	9.281	15.089
Other current assets and receivables	7.606	2.655
<b>Total current assets</b>	<u><b>390.289</b></u>	<u><b>651.853</b></u>
<b>Non-current assets</b>		
Property, plant and equipment, net	\$ 20.424	\$ 46.548
Other investments	27.464	\$ 2.179
Operating lease right-of-use assets	13.647	28.789
Intangible assets, net	71.043	60.481
Goodwill	22.414	26.379
Deferred tax assets, net	9.856	12.276
Other non-current assets	1.399	3.184
<b>Total non-current assets</b>	<u><b>166.247</b></u>	<u><b>179.836</b></u>
<b>Total assets</b>	<u><b>\$ 556.536</b></u>	<u><b>\$ 831.689</b></u>
<b>Current liabilities</b>		
Accounts payable	\$ 7.227	\$ 6.586
Accrued expenses and other current liabilities	29.225	30.534
Current portion of contingent consideration	—	28.211
Current portion of operating lease liabilities	3.601	8.344
<b>Total current liabilities</b>	<u><b>40.053</b></u>	<u><b>73.675</b></u>
<b>Non-current liabilities</b>		
Long-term debt	51.324	101.749
Liability from royalty financing agreement	434.930	394.241
Operating lease liabilities, net of current portion	11.136	28.316
Contingent consideration, net of current portion	10.860	14.795
Deferred tax liability, net	7.043	7.543
Other non-current liabilities	7.942	3.700
<b>Total non-current liabilities</b>	<u><b>523.235</b></u>	<u><b>550.344</b></u>
<b>Total liabilities</b>	<u><b>563.288</b></u>	<u><b>624.019</b></u>
<b>Shareholders' equity</b>		
<b>Total shareholders' equity</b>	<u><b>(6.752)</b></u>	<u><b>207.670</b></u>
<b>Total liabilities and shareholders' equity</b>	<u><b>\$ 556.536</b></u>	<u><b>\$ 831.689</b></u>

**uniQure N.V.**

**UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS**

	<u>Year ended December 31,</u>		
	<u>2024</u>	<u>2023</u>	<u>2022</u>
	<u>(in thousands, except share and per share amounts)</u>		
License revenues	10.133	\$ 2.758	\$ 100.000
Contract manufacturing revenues	6.114	10.835	1.717
Collaboration revenues	10.872	2.250	4.766
<b>Total revenues</b>	<u><b>27.119</b></u>	<u><b>15.843</b></u>	<u><b>106.483</b></u>
<b>Operating expenses:</b>			
Cost of license revenues	(1.267)	(65)	(1.254)
Cost of contract manufacturing revenues	(17.060)	(13.563)	(2.089)
Research and development expenses	(143.782)	(214.864)	(197.591)
Selling, general and administrative expenses	(52.657)	(74.591)	(55.059)
<b>Total operating expenses</b>	<u><b>(214.766)</b></u>	<u><b>(303.083)</b></u>	<u><b>(255.993)</b></u>

Other income	7.926	6.059	7.171
Other expense	(4.573)	(1.690)	(820)
<b>Loss from operations</b>	<b>(184.294)</b>	<b>(282.871)</b>	<b>(143.159)</b>
Non-operating items, net	(52.833)	(23.686)	14.900
<b>Loss before income tax (expense) / benefit</b>	<b>\$ (237.127)</b>	<b>\$ (306.557)</b>	<b>\$ (128.259)</b>
Income tax (expense) / benefit	(2.429)	(1.921)	1.470
<b>Net loss</b>	<b>\$ (239.556)</b>	<b>\$ (308.478)</b>	<b>\$ (126.789)</b>
<b>Earnings per ordinary share - basic and diluted</b>			
Basic and diluted net loss per ordinary share	\$ (4,92)	\$ (6,47)	\$ (2,71)
Weighted average shares - basic and diluted	48.649.129	47.670.986	46.735.045

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