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

November 5, 2024

*~ Type B meeting scheduled with the FDA in the fourth quarter of 2024 to initiate discussions regarding a potential expedited clinical development pathway for AMT-130 in Huntington's disease ~*

*~ Presented positive interim data from Phase I/II trial of AMT-130 demonstrating slowing of Huntington's disease progression and reductions in key biomarker of neurodegeneration ~*

*~ Initiated patient dosing in new Phase I/II studies of AMT-162 in SOD1-ALS and AMT-191 in Fabry disease; First patient enrolled in observational stage of Phase I/II study of AMT-260 in mTLE ~*

*~ Completed sale of Lexington manufacturing facility and announced organizational restructuring expected to significantly reduce operating expenses and annual cash burn ~*

*~ Strong cash position of approximately $435 million as of September 30, 2024, following retirement of $50 million of debt, with cash runway through the end of 2027 ~*

LEXINGTON, Mass. and AMSTERDAM, Nov. 05, 2024 (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 2024 and highlighted recent progress across its business.

"uniQure has made significant strides during the third quarter both clinically and operationally. We advanced our pipeline of clinical gene therapy programs, including the presentation of positive long-term follow-up data on AMT-130 supporting significant, dose-dependent slowing of Huntington's disease progression," stated Matt Kapusta, chief executive officer of uniQure. "We have scheduled a Type B meeting with the FDA for late November and welcome the opportunity, as part of the Regenerative Medicine Advanced Therapy (RMAT) designation, to discuss the potential for an accelerated development pathway for AMT-130. We believe the recently announced compelling clinical data, combined with AMT-130's manageable safety profile and the lack of therapeutic options for patients in need, present a strong case for accelerated development. In addition, dosing has begun across two new Phase I/II studies in SOD1-ALS and Fabry disease, and we are making substantial progress towards the initiation of a third clinical trial in mesial temporal lobe epilepsy, with the first patient recently enrolling into the observational phase of the study."

"We also delivered on one of our key corporate goals, which was to take meaningful actions to streamline operations and preserve capital. Following the sale of our Lexington manufacturing facility, we announced a strategic reorganization expected to further reduce our cash burn and operating expenses," he continued. "These decisions, which are delivering an immediate favorable impact, have extended our cash runway through the end of 2027 and multiple clinical and regulatory milestones with the potential to generate shareholder value."

Mr. Kapusta further commented, "Given the positive interim data on AMT-130, the upcoming Type B meeting, the sale of our manufacturing facility, commencement of three clinical trials and the rightsizing of our organization, uniQure has executed on its key short-term goals. Going forward we are turning our near-term focus toward working with the FDA in an effort to obtain an accelerated pathway for ATM-130 and further advancing our clinical pipeline. We look forward to providing updates on all our progress."

Recent Company Updates

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

  - Based on the granting of the RMAT designation, the Company has scheduled a Type B, multi-disciplinary meeting with the U.S. Food and Drug Administration (FDA) in late November at which the Company plans to present the most recent clinical data and initiate discussions regarding the potential for an expedited development pathway for AMT-130. The Company will also discuss with the FDA a future communication plan that is expected to include additional sub-disciplinary meetings to take place in the first half of 2025. Once the Company and the FDA define the registrational pathway for AMT-130, the Company expects to issue a public announcement.

  - In July 2024, uniQure announced positive interim data from the ongoing U.S. and European Phase I/II studies of AMT-130 for the treatment of early-stage Huntington's disease [1]. At 24 months, the data demonstrated a statistically significant, dose-dependent slowing in disease progression measured by the composite Unified Huntington's Disease Rating Scale (cUHDRS) in patients receiving the high dose of AMT-130 compared to a propensity score-weighted external control (p=0.007), as well as a statistically significant reduction of neurofilament light chain (NfL) in cerebrospinal fluid (CSF) in patients dosed with AMT-130 compared to baseline (p=0.02). AMT-130 continued to be generally well-tolerated with a manageable safety profile across both doses.

  - Patient dosing is ongoing in a third cohort of up to 12 patients to further evaluate both doses of AMT-130 together with an immunosuppression regimen, with a focus on evaluating near-term safety and tolerability. Enrollment in this

third cohort is expected to be completed in the fourth quarter of 2024.

- The Company expects to provide an additional interim update from its ongoing Phase I/II clinical trials of AMT-130 in mid-2025. 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 21 treated patients.

- *Initiating new Phase I/II clinical studies*

  - *AMT-191 for the treatment of Fabry disease* – In August 2024, the Company announced that the first patient had been dosed in the Phase I/II clinical trial of AMT-191 for the treatment of Fabry disease. AMT-191 was granted Orphan Drug and Fast Track designations in September and October 2024, respectively. The U.S., multi-center, open-label trial is expected to include up to 12 adult male patients across two dose cohorts.

  - *AMT-162 for the treatment of SOD1 amyotrophic lateral sclerosis (ALS)* – In October 2024, the Company announced that the first patient had been dosed in the Phase I/II clinical trial of AMT-162 for SOD1-ALS. The U.S., multi-center, open-label trial is expected to include up to 12 patients across three dose cohorts.

  - *AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (mTLE)* – The first patient has been enrolled into the observational phase of the Phase I/II clinical trial of AMT-260 for the treatment of mTLE. The FDA-approved study protocol provides that the first three patients to be enrolled in the study are required to have MRI-confirmed unilateral, hippocampal sclerosis. Due to the more restrictive inclusion criteria for these sentinel patients, enrollment has taken longer than expected. The Company is rapidly activating recruitment sites with 10 centers currently open and an additional two sites expected to be activated by the end of 2024.

- *Capital preservation initiatives*

  - In July 2024, the Company announced the closing of the sale of its Lexington, MA manufacturing facility to Genezen.

  - In August 2024, the Company announced an organizational restructuring which, combined with the Lexington manufacturing facility sale, is expected to eliminate approximately 65% or 300 roles across the organization and reduce recurring cash burn by $70 million per year.

  - In the third quarter of 2024, the Company made significant progress in reducing its operating expenses, with immediate benefit realized as a decrease in fixed costs from the sale of the Lexington facility and the reduction in personnel. The Company expects its expenses to further decline upon the completion of the restructuring, which is expected in the first half of 2025.

  - In July 2024, the Company retired $50 million of its outstanding debt with Hercules Capital, which is expected to reduce annual interest expense by approximately $5 million. As of September 30, 2024, the Company had $50 million of debt outstanding.

Upcoming Investor Events

- Guggenheim Healthcare Talks – Global Healthcare Conference, November 12$^{th}$ – Boston, MA

- Stifel 2024 Healthcare Conference, November 18$^{th}$ – New York, NY

**Financial Highlights**

**Cash position:** As of September 30, 2024, the Company held cash and cash equivalents and investment securities of $435.2 million, compared to $617.9 million as of December 31, 2023. The reduction in cash was in part driven by non-recurring payments made in the third quarter of 2024, including $53 million related to the retirement of debt, $12M of one-time payments related to the Lexington facility transaction, and $1M of severance payments related to the Company's corporate restructuring. Based on the Company's current operating plan, the Company expects cash, cash equivalents and investment securities will be sufficient to fund operations through the end of 2027.

**Revenues:** Revenue for the three months ended September 30, 2024 was $2.3 million, compared to $1.4 million in the same period in 2023. The increase of $0.8 million in revenue resulted from a $1.6 million increase in license revenue, a decrease of $0.4 million from collaboration revenue, and a decrease of $0.3 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 $0.8 million for the three months ended September 30, 2024, compared to $1.0 million for the same period in 2023. The decrease relates to the sale of the Lexington facility. Following the sale 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 $30.6 million for the three months ended September 30, 2024, compared to $65.4 million during the same period in 2023. The $34.8 million decrease was related to a decrease of $14.6 million related to changes in the fair value of contingent consideration, a $13.7 million decrease in employee-related expenses, partially offset by an increase of $3.4 million severance costs related to the reorganization announced in August 2024, a net decrease of $4.9 million in external program spend and a $3.7 million decrease in costs incurred related to preclinical supplies.

**SG&A expenses:** Selling, general and administrative expenses were $11.6 million for the three months ended September 30, 2024, compared to $18.1 million during the same period in 2023. The $6.5 million decrease was primarily related to a $4.0 million decrease in employee-related expenses, partially offset by an increase of $0.7 million severance costs related to the reorganization announced in August 2024, and a $1.3 million decrease in professional fees compared to the prior year period.

**Other income:** Other income was $2.6 million for the three months ended September 30, 2024, compared to $1.4 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 $1.9 million for the three months ended September 30, 2024, compared to $0.2 million during the same period in 2023. The increase was primarily related to $1.5 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 $4.2 million for the three months ended September 30, 2024, compared to $7.8 million for the same period in 2023. The $3.6 million decrease in other non-operating items, net was primarily related to an increase in net foreign currency gains of $7.4 million, which was partially offset by a decrease of $2.6 million in interest income earned on investment securities and an increase in non-cash interest expense of $1.2 million related to the royalty agreement that the Company entered into in May 2023.

**Net loss:** The net loss for the three months ending September 30, 2024, was $44.4 million, or $0.91 basic and diluted loss per ordinary share, compared to $89.6 million net loss for the same period in 2023, or $1.88 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

**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 through the end of 2027 and multiple milestones with the potential to generate shareholder value; the Company's expectations regarding its organizational restructuring, including reductions in headcount and reductions in annual cash burn resulting the restructuring; the Company's plans to announce additional interim updates from its ongoing U.S. and European Phase I/II clinical studies of AMT-130 in mid-2025 along with other future program updates; the Company's plans to meet the FDA regarding potential expedited clinical development pathways for AMT-130; the strength of the Company's case for accelerated development; the Company's future communication plan and additional meetings with FDA expected to take place in the first half of 2025; the Company's plans regarding the third cohort in its AMT-130 clinical trial and the timing of enrollment for such cohort; and the Company's site activation plans for its AMT-260 clinical trial and the design of trials for its AMT-191, AMT-260 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 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 February 28, 2024, its Quarterly Reports on Form 10-Q filed May 7, 2024, August 1, 2024 and November 5, 2024, 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: 617-306-9137
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**

| | September 30 2024 | December 31, 2023 |
|---|---|---|
| | (in thousands, except share and per share amounts) | |
| **Current assets** | | |
| Cash and cash equivalents | $ 251,626 | $ 241,360 |
| Current investment securities | 183,615 | 376,532 |
| Inventories, net | - | 12,024 |
| Accounts receivable | 5,322 | 4,193 |
| Prepaid expenses | 19,286 | 15,089 |
| Other current assets and receivables | 4,289 | 2,655 |
| **Total current assets** | **464,138** | **651,853** |
| **Non-current assets** | | |
| Property, plant and equipment, net | $ 25,566 | $ 46,548 |
| Other investments | 28,260 | $ 2,179 |
| Operating lease right-of-use assets | 14,833 | 28,789 |
| Intangible assets, net | 76,609 | 60,481 |
| Goodwill | 24,084 | 26,379 |
| Deferred tax assets, net | 10,863 | 12,276 |
| Other non-current assets | 1,453 | 3,184 |
| **Total non-current assets** | **181,668** | **179,836** |
| **Total assets** | **$ 645,806** | **$ 831,689** |
| **Current liabilities** | | |
| Accounts payable | $ 5,441 | $ 6,586 |
| Accrued expenses and other current liabilities | 32,301 | 30,534 |
| Current portion of contingent consideration | 29,233 | 28,211 |
| Current portion of operating lease liabilities | 4,298 | 8,344 |
| **Total current liabilities** | **71,273** | **73,675** |
| **Non-current liabilities** | | |
| Long-term debt | 51,113 | 101,749 |
| Liability from royalty financing agreement | 426,687 | 394,241 |
| Operating lease liabilities, net of current portion | 12,185 | 28,316 |
| Contingent consideration, net of current portion | 12,181 | 14,795 |
| Deferred tax liability, net | 7,627 | 7,543 |
| Other non-current liabilities | 8,919 | 3,700 |
| **Total non-current liabilities** | **518,712** | **550,344** |
| **Total liabilities** | **589,985** | **624,019** |
| **Shareholders' equity** | | |
| **Total shareholders' equity** | **55,821** | **207,670** |
| **Total liabilities and shareholders' equity** | **$ 645,806** | **$ 831,689** |

**uniQure N.V.**

**UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS**

| | Three months ended September 30, | |
|---|---|---|
| | 2024 | 2023 |
| | (in thousands, except share and per share amounts) | |
| **Total revenues** | **$ 2,287** | **$ 1,407** |
| **Operating expenses:** | | |
| Cost of license revenues | (264) | — |
| Cost of contract manufacturing revenues | (757) | (1,006) |
| Research and development expenses | (30,595) | (65,400) |
| Selling, general and administrative expenses | (11,575) | (18,074) |

| | | |
|---|---|---|
| **Total operating expenses** | **(43,191)** | **(84,480)** |
| Other income | 2,591 | 1,424 |
| Other expense | (1,915) | (228) |
| **Loss from operations** | **(40,228)** | **(81,877)** |
| Non-operating items, net | (4,181) | (7,763) |
| **Loss before income tax expense** | **$ (44,409)** | **$ (89,640)** |
| Income tax benefit | 31 | 69 |
| **Net loss** | **$ (44,378)** | **$ (89,571)** |
| | | |
| Basic and diluted net loss per ordinary share | $ (0.91) | $ (1.88) |
| Weighted average shares used in computing basic and diluted net loss per ordinary share | 48,718,533 | 44,770,101 |

[1] All p-values are nominal and unadjusted. Statistical comparisons of patients treated with AMT-130 to the propensity score-weighted external control were conducted on a post-hoc basis.

This press release was published by a CLEAR® Verified individual.

uniQure