uniQure Announces Updated Preliminary AMT-191 Phase I/IIa Data Showing Sustained Increases in α-Gal A Enzyme Activity in Patients with Fabry Disease

February 6, 2026

~ Supraphysiological expression of α-Gal A activity maintained for over a year in longest treated patient as of data cutoff date ~

~ Stable Lyso-Gb3 levels maintained post-dosing, regardless of enzyme replacement therapy status across all cohorts ~

~ Six of 11 patients have discontinued enzyme replacement therapy as of data cutoff date ~

LEXINGTON, Mass. and AMSTERDAM, Feb. 06, 2026 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced updated preliminary safety and exploratory efficacy data from 11 patients in its Phase I/IIa trial of AMT-191, an investigational AAV gene therapy for the treatment of Fabry disease. The updated data was presented at the WORLD*Symposium* in San Diego, California.

As of the January 8, 2026 study data cutoff date, all 11 patients in the three dose cohorts ($6x10^{13}$ genome copies/kilogram (gc/kg), $4x10^{13}$ gc/kg and $2x10^{13}$ gc/kg) had elevated α-galactosidase A (α-Gal A) activity.

Importantly, dose-dependent elevations were observed across the three dose levels with α-Gal A activity ranging from 0.34- to 82.2-fold above mean normal level[1] at the lowest dose, 1.6- to 312.52-fold at the mid dose, and 27.7- to 223.7-fold at the highest dose. These increases were durable for the measured time period ranging from the longest follow-up period of more than a year in a treated patient (high-dose cohort) to the shortest follow-up period of four months in a treated patient (mid-dose cohort).

Six of 11 dosed patients were withdrawn from enzyme replacement therapy (ERT) having met a pre-specified criteria including elevated α-Gal A activity. Stable plasma lyso-Gb3 levels were maintained post-dose across all dose cohorts, regardless of ERT status through the cutoff date.

"These updated preliminary data reinforce our confidence in the biological activity of AMT-191, including sustained and dose-dependent increases in α-Gal A activity across all dose cohorts of the treated patients," stated Walid Abi-Saab, M.D., chief medical officer of uniQure. "While the study remains ongoing, we believe the preliminary data collected are supportive of the potential for AMT-191 as a one-time administered gene therapy for people living with Fabry disease, and we look forward to providing additional updates on the program."

AMT-191 continued to show a manageable safety profile. No Serious Adverse Events (SAEs) related to AMT-191 have been observed at the $4x10^{13}$ gc/kg and $2x10^{13}$ gc/kg doses. Two patients at the $4x10^{13}$ gc/kg dose experienced asymptomatic Grade 3 liver enzyme elevations. Both were confirmed as dose-limiting toxicity following an Independent Data Monitoring Committee review, and per protocol, the company has paused additional dosing in the mid- and high-dose cohorts pending further evaluation. Both patients have responded to corticosteroid therapy and remain in follow up.

No additional SAEs have been observed at the $6x10^{13}$ gc/kg dose beyond the five previously reported in two patients: two SAEs unrelated to AMT-191 (stroke, diplopia), two related SAEs (chest pain, increased troponin), and one possibly related SAE (leptomeningeal enhancement). As previously reported, one patient at the $6x10^{13}$ gc/kg dose experienced an asymptomatic, Grade 3 liver enzyme elevation that fully resolved with a limited course of corticosteroid therapy.

These updated data were presented at the 22nd Annual WORLD*Symposium* during a poster presentation (Poster Ref: LB-07) on February 3, 2026. An oral presentation will take place on Friday, February 6, 2026 in the session from 10:30-11:30 a.m. Pacific Time. The presentation will also be available on uniQure's website on the Events & Presentations page after the oral presentation.

About the Phase I/IIa Clinical Program of AMT-191

The Phase I/IIa clinical trial of AMT-191 is a multi-center, open-label trial being conducted in the United States consisting of three dosing cohorts of three or more adult male patients each receiving an intravenous infusion of AMT-191. Patients were not excluded from the trial based on pre-existing neutralizing anti-bodies to AAV5. Patients continue to receive their regular enzyme replacement therapy until meeting withdrawal criteria and will be followed for a period of 24 months. The trial will explore the safety, tolerability, and early signs of efficacy by measuring the expression of lysosomal enzyme α-Gal A. Additional details are available on www.clinicaltrials.gov (NCT06270316).

AMT-191 has been granted both Orphan Drug and Fast Track designation by the U.S. Food and Drug Administration.

About Fabry Disease

Fabry disease is an X-linked genetic lysosomal storage disorder caused by a deficiency of the α-galactosidase A (α-Gal A) enzyme, leading to toxic accumulation of globotriaosylsphingosine (lyso-Gb3) that can damage the kidneys, heart, nervous system, eyes, gut and skin. It is estimated that type 1 classic Fabry disease affects at least one in 40,000 males and approximately one in 20,000 females, and type 2 Fabry disease may occur in some populations as frequently as 1 in 1,500 to 4,000 males. The current standard of care for Fabry disease is bi-weekly infusions of enzyme replacement therapy, a treatment with limited effectiveness in many patients due to poor cross-correction, with inefficient clearance of substrates in the target organs, in particular the kidney and the heart.

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 regarding: the potential of AMT-191 to be a one-time administered gene therapy for people living with Fabry disease; and plans to provide additional AMT-191 program updates. uniQure's actual results could differ materially from those anticipated in these forward-looking statements for many reasons. These risks and uncertainties include, without limitation: risks associated with the clinical results and the development and timing of uniQure's programs; the risk that more patient data become available that results in different findings than that presented in preliminary or interim data; uniQure's interactions with regulatory authorities, which may affect the initiation, timing and progress of clinical trials and pathways and timing for regulatory approval; uniQure's ability to continue to build and maintain the company infrastructure and personnel needed to achieve its goals; uniQure's effectiveness in managing current and future clinical trials and regulatory processes; the continued development and acceptance of gene therapies; uniQure's ability to demonstrate the therapeutic benefits of its gene therapy candidates in clinical trials; uniQure's ability to obtain, maintain and protect intellectual property; and uniQure'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 uniQure'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, July 29, 2025 and November 10, 2025, and in other filings that uniQure 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 uniQure assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

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[1] Normal range (1.38 – 8.66 nmol); mean normal of 3.57 nmol