uniQure

June 17, 2026

Dear Huntington's Disease Community,

We are writing to share an important update from our recently held Type B meeting with the U.S. Food and Drug Administration (FDA) regarding AMT-130, uniQure's investigational gene therapy candidate in Huntington's disease (HD).

On June 17, 2026, uniQure issued a press release to announce that the FDA communicated that the 3-year analysis from the ongoing Phase I/II study would be acceptable as the primary basis of a Biologics License Application (BLA) for the accelerated approval for AMT-130 in Huntington's disease. The FDA seeks to align on the confirmatory study design prior to the BLA submission, including consideration of concurrent control on standard-of-care therapy instead of a sham procedure. The FDA communicated that they would work as expeditiously as possible with uniQure on this effort. uniQure is committed to conducting the confirmatory study without delay and expects to further align with the FDA on the details of such a study prior to BLA submission. uniQure intends to submit the BLA in the third quarter of 2026.

"Today's announcement reflects the outcome we have worked toward throughout our continued regulatory engagement with FDA, and we are deeply grateful for FDA's genuine commitment to addressing the unmet need of Americans living with HD," said Matt Kapusta, chief executive officer at uniQure.

Our deepest gratitude goes to the patients, families, and advocacy partners who shared their experiences throughout this part of the process. Your voices were vital in keeping the patient perspective at the forefront of our recent regulatory discussions. Drug development is a long journey and often the path is unclear; however, your fortitude continues to inspire us. We remain focused on bringing AMT-130 to patients and families as quickly and responsibly as possible in the United States and globally.

uniQure will continue to keep you informed as additional updates become available. Thank you for your ongoing support and being an essential part of this journey.

If you have any questions, please email medinfo@uniqure.com or call 1-866-520-1257.

Sincerely,

Daniel Leonard

Executive Director of Global Patient Advocacy

AMT-130 has not been approved by the US FDA, EMA, MHRA or any other regulatory body as safe and effective for any use.

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