



March 2, 2026

Dear Huntington's Disease Community,

We are writing to share an update following our recent Type A meeting with the U.S. Food and Drug Administration (FDA.) On March 2, 2026, uniQure issued a press release reporting the receipt of final meeting minutes from the FDA. The purpose of the meeting was to discuss the Biologics License Application (BLA) data package to support accelerated approval of AMT-130, uniQure's investigational gene therapy in Huntington's disease.

The FDA stated that it cannot agree that data from the Phase I/II studies, compared to an external control, are sufficient to provide the primary evidence of effectiveness required to support a marketing application for AMT-130. The FDA strongly recommended uniQure conduct a prospective, randomized, double-blind, sham surgery-controlled Phase III study. uniQure intends to continue engaging with the FDA regarding Phase III development considerations and plans to request a Type B meeting in the second quarter of 2026 to further discuss potential study design approaches.

In addition, we also informed the FDA of our intention to update the Phase I/II statistical analysis plan to include a four-year analysis, which we expect to complete in the third quarter of 2026. We believe the extended follow-up will provide additional insight into both the durability and the magnitude of the effects of AMT-130 observed so far.

The FDA's minutes acknowledge the significant unmet need for disease-modifying therapies in Huntington's disease and affirm the Agency's commitment to applying appropriate regulatory flexibility to help advance safe and effective treatment options. We believe our emerging Phase I/II data further supports the need for this flexibility to be applied. We also recognize how essential it is that patient voices continue to be heard as regulatory flexibility is considered, and we remain committed to ensuring your perspectives stay central to the path forward.

We remain deeply committed to the HD community and are profoundly grateful for the steadfast support you have shown over the months and years. The urgency the community feels is both real and understandable, and we share your determination to advance meaningful therapies. We believe AMT-130 holds strong potential to offer disease-modifying benefit.

Our team is fully focused on defining the most direct and responsible regulatory path forward so we can bring this therapy to patients as quickly as possible. We look forward to sharing additional updates as our regulatory discussions continue to progress. If you have any questions, please email medinfo@uniquire.com or call 1-866-520-1257.

Sincerely,
Daniel Leonard
Executive Director of Global Patient Advocacy

uniQure biopharma B.V.

uniQure Inc.
Paasheuvelweg 25A
1105 BP, Amsterdam
The Netherlands

uniQure
1 Hartwell Place
Lexington MA 02421
United States

Switzerland GmbH
Peter Merian-weg 4
4052 Basel
Switzerland

AMT-130 is an investigational therapy that has not been approved by the FDA. Its safety and efficacy have not been established.