uniQure

January 20, 2026

Dear Huntington's Disease Community,

On January 9th, uniQure issued a press release announcing that we scheduled a Type A meeting with the U.S. Food and Drug Administration (FDA). The purpose of this meeting is 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.

We are encouraged that the FDA has accepted our request for a Type A meeting. In the interest of transparent communication, and to address questions we are receiving from the HD community, we would like to provide additional context regarding what a Type A meeting is, and to express our sincere appreciation for the community's efforts to raise awareness of the urgent unmet need in HD.

Type A meetings are intended to address urgent issues and help a sponsor, such as uniQure, resolve developmental roadblocks and obtain critical feedback from the FDA. Type A meetings are typically scheduled within 30 days of the FDA's receipt of a written meeting request. The FDA generally issues official meeting minutes within approximately 30 days following the meeting. uniQure expects to provide a regulatory update following the receipt of the FDA's official meeting minutes.

The extraordinary efforts of the HD community over the past two months have played an important role in raising awareness of the significant unmet need in HD. The two petitions currently circulating have gathered nearly 50,000 signatures, and there have been numerous meetings with, and letters sent to Congressional representatives.

uniQure is deeply appreciative of the Coalition for the unprecedented advocacy seen over the past two months. In addition, we recognize the incredible commitment of every patient, caregiver, family member, and friend who has made their voice heard. We encourage you to continue these efforts to help highlight the urgency of this unmet need. uniQure also plans to include a community representative at the Type A meeting so that the FDA may hear a patient voice directly.

uniQure remains committed to constructive dialogue with the FDA as we work towards a potential approval pathway for AMT-130. We remain deeply committed to the HD community and to advancing innovating treatments for this devastating disease.

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

Sincerely,

Daniel Leonard
Executive Director of Global Patient Advocacy

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

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