uniQure

November 3, 2025

Dear Huntington's Disease Community,

We issued a press release today announcing that uniQure received feedback from the U.S. Food and Drug Administration (FDA) during a recent pre-Biologics License Application (BLA) meeting regarding AMT-130, an investigational gene therapy for Huntington's disease (HD). The FDA defines a BLA as "a comprehensive submission to the FDA requesting approval to distribute a biologic product commercially." A pre-BLA meeting is intended for the company and the FDA to align on the requirements and expectations for the application.

Though final meeting minutes have not been received, uniQure believes the FDA indicated that the data from the ongoing Phase I/II study of AMT-130 in comparison to an external control may no longer serve as the primary basis for a BLA submission. Although the timing of the BLA submission for AMT-130 is now unclear, uniQure remains fully committed to working with the FDA to determine the best path forward to bring AMT-130 to patients and their families.

"We are surprised by the FDA's preliminary feedback at the pre-BLA meeting, which is a drastic change from the guidance the FDA provided in November 2024 where it agreed that data from the ongoing Phase I/II studies, compared to a natural history external control, may serve as the primary basis for a BLA submission under the Accelerated Approval pathway," said Matt Kapusta, chief executive officer at uniQure. "This news is unexpected, and we are truly disappointed for people living with HD, who have no treatment options for this devastating disease. We strongly believe AMT-130 can provide substantial benefit to patients, and remain fully committed to working with the FDA to determine the best path forward to rapidly bring AMT-130 to patients and their families in the U.S."

We understand how difficult it will be for patients and families to hear this unexpected news, we assure you that we will work tirelessly to partner with the FDA to establish a path forward for AMT-130. We have partnered with the HD community throughout the entirety of this clinical development program, and we are deeply appreciative of that collaboration. We will continue to communicate transparently and partner with the community as we work through this challenging situation.

If there are additional questions, please feel free to email medinfo@uniqure.com or call 1-866-520-1257.

Sincerely,

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