FOR IMMEDIATE RELEASE

HDSA Statement on uniQure's AMT-130 Regulatory Update

NEW YORK, NY — June 17, 2026 — The Huntington's Disease Society of America (HDSA) today issued the following statement from Amy Gray, Chief Executive Officer, regarding uniQure's announcement that the U.S. Food and Drug Administration (FDA) indicated the three-year analysis from the Phase I/II AMT-130 study may serve as the primary basis for a Biologics License Application (BLA) for accelerated approval in Huntington's disease.

"Today's announcement from uniQure represents an encouraging and meaningful step forward for the Huntington's disease community," said Amy Gray, Chief Executive Officer of HDSA. "I applaud the leadership at the FDA for allowing uniQure to take this important step forward in the development of its investigational treatment for Huntington's disease.

"For families facing Huntington's disease, time is everything. Every day without an approved treatment capable of slowing disease progression is another day that patients and families continue to bear the devastating impact of this fatal neurodegenerative disease. The FDA's indication that three-year data from the Phase I/II AMT-130 study may serve as the primary basis for a BLA for accelerated approval offers renewed hope to thousands of families who have been waiting far too long for treatment options.

"This progress did not happen in isolation. Following regulatory hurdles late last year, the Huntington's disease community united like never before. HDSA joined forces with partner organizations and advocates across the country to deliver a powerful community petition with more than 47,000 signatures to the FDA. Building on this collective momentum, the HDSA network mobilized by sending more than 11,000 messages to Congress, participating in dozens of legislative meetings, and sharing deeply personal stories about the urgent need for treatments.

"Clinicians from HDSA Centers of Excellence also traveled to Washington, D.C., to educate policymakers and share the promise of innovative therapies, while HDSA hosted a congressional briefing to advocate for the appropriate use of natural history data in rare disease research.

"I am deeply grateful to the Members of Congress who stood with Huntington's disease patients and families during this effort. Their willingness to engage with the FDA, ask important questions, and advocate for a regulatory framework that reflects the realities of rare disease research helped ensure that the voices of our community were heard.

"While today is a milestone worth celebrating, there is important work ahead. This announcement opens the door for a future submission, but it is also the beginning of a regulatory process that includes designing and executing a necessary confirmatory study. HDSA remains fully committed to collaborating with regulators, uniQure, HD sister organizations, and the broader healthcare community to help navigate these next steps safely and as expeditiously as possible.

"Today, we celebrate an important milestone and the hope it brings to families across the country. We are encouraged by this momentum and stand ready for the work to come."

About Huntington's Disease

Huntington's disease (HD) is an inherited neurological disease that causes the progressive breakdown of movement, thinking, and behavior. Symptoms worsen over time and can affect a person's ability to work, communicate, and care for themselves. Today, approximately 41,000 Americans are living with symptoms, and more than 200,000 are at risk of developing the disease. Each child of a parent with HD has a 50 percent chance of inheriting the gene. There is currently no cure, but research is accelerating the development of better treatments and improved care.

About the Huntington's Disease Society of America

The Huntington's Disease Society of America (HDSA) is the leading national nonprofit dedicated to improving the lives of everyone impacted by Huntington's disease. Through community events, education, advocacy, specialized care, and research advancement, HDSA connects individuals and families to trusted resources and works to ensure no one faces Huntington's disease alone.

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