November 2, 2026

Dear Huntington's Disease Community,

On November 2, 2026, uniQure issued a press release announcing that the U.S. Food and Drug Administration (FDA) has accepted its Biologics License Application (BLA) for ifezuntirgene inilparvovec (eye-feh-ZUN teer-JEEN in-il-par-VOE-vek) (also referred to as AMT-130), an investigational gene therapy candidate for the treatment of Huntington's disease, for review.

Reaching this step in the regulatory process reflects the contributions of so many. First and foremost, we are grateful to the people who gave their time, effort, and trust as participants in the HD- GeneTRX 1 and 2 studies. In addition, we must also recognize the efforts of all of the families and care partners who supported them, and express our appreciation for the investigators, advocacy organizations, and healthcare professionals who have given so much to this program. Each of your contributions to this research matter deeply, and we thank you.

What this means: Acceptance of a BLA means that the FDA has determined that the application is sufficiently complete to begin a formal review. The application has been granted Priority Review with a Prescription Drug User Fee Act (PDUFA) target action date of May 1, 2027. A PDUFA date is the FDA's target date for completing its review of an application. It is a goal, not a guarantee of a decision, approval, or availability, and the date can change.

What this does not mean: Acceptance for review is not an approval decision, and ifezuntirgene inilparvovec remains investigational until any approval decision is final. The FDA will evaluate the data contained in the application and may request additional information during the review. The outcome of the review cannot be predicted.

What happens next: We will respond promptly to any FDA requests for additional information. Ifezuntirgene inilparvovec remains investigational and has not been approved. The FDA has also requested a tentative Advisory Committee (AdCom). Patients, families, and advocates may have an opportunity to share their experiences and perspectives directly with the committee. The committee looks at the available evidence and gives the FDA scientific and medical advice about the safety, effectiveness, and appropriate use of a potential treatment. This advice is just that - advice. The committee provides recommendations to help inform the FDA's review, but the FDA makes the final decision about a treatment being approved.

Why is uniQure conducting a Phase 3 confirmatory study? Under the FDA's accelerated approval program, approval may be based on an early measure (often called a surrogate or intermediate endpoint) that is reasonably likely to predict clinical benefit. To confirm that the treatment provides the anticipated clinical benefit, the FDA requires an additional study, called a confirmatory trial, after approval. The FDA and uniQure have aligned on the key elements of the confirmatory study design. The study is designed as a randomized, standard-of-care trial enrolling approximately 200 patients in a 2:1 ratio of ifezuntirgene inilparvovec to standard of care, with change in Total Functional Capacity (TFC) from baseline at 36

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

months as the primary endpoint. uniQure plans to begin screening participants in the U.S. before the end of 2026. Clinical trial sites within the United States are still being confirmed and activated. The first sites will be in the United States, and we are committed to opening sites in other countries as well. As sites in specific countries are confirmed, we expect to share information about international locations in the first half of 2027. Once the study details are posted on ClinicalTrials.gov, we will share them with the community. Because new sites may be added over time, please use ClinicalTrials.gov as your main source for the most up-to-date information about trial locations and site status.

Thank you for your ongoing support. This community has been an essential part of the journey, and we are deeply grateful to everyone who has shared their experiences, participated in research, and advocated for progress on behalf of individuals and families affected by Huntington's disease.

We look forward to sharing additional updates where possible as the regulatory process continues. 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

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