uniQure

Dear Huntington's Disease Patient Organization Leaders,

This morning, uniQure released a press statement presenting interim data from patients participating in the ongoing Phase I/II clinical trials of AMT-130, [HD Gene TRX-1 and HD Gene TRX-2], for Huntington's disease treatment in the United States and Europe. The analysis includes follow-up periods of up to 24 months and also introduces an external control group. The major updates are as follows:

- A significant, dose-dependent, slowing in disease progression measured by cUHDRS was observed through 24 months in patients receiving the high dose of AMT-130.
- Trends in measurements of motor and cognitive function showed near-baseline stability throughout the 24 months of follow-up in patients receiving the high dose of AMT-130.
- A statistically significant reduction of NfL in cerebral spinal fluid (CSF) was observed in patients treated with AMT-130.
- Based on data observed to date, AMT-130 remains generally well-tolerated, with a manageable safety profile at both doses. There were no new AMT-130-related serious adverse events reported.

In the United States, the Phase I/II clinical trial of AMT-130 is evaluating the safety, tolerability, and efficacy signals. In Europe, the Phase IB/II clinical trial of AMT-130 is similarly assessing these factors in an open-label study. For more information, please visit http://www.clinicaltrials.gov for HD Gene TRX-1 (NCT04120493) and HD Gene TRX-2 (NCT05243017).

Please note that uniQure management will host a conference call and webcast today, Tuesday, July 9, 2024 at 8:30 a.m. ET. Details for the conference call and webcast can be found in the press release that is posted to the investor page of uniQure's website, www.uniqure.com.

This morning's webcast is aimed primarily at the investor audience and will be hosted with uniQure's Chief Medical Officer, Dr. Walid Abi-Saab and Dr. Victor Sung, Professor of Neurology at University of Alabama.

We are profoundly grateful for the unwavering support from the HD community and the trailblazing individuals who have volunteered for our study. It is through these vital collaborations that we can advance our mission to develop treatments aimed at altering the course of Huntington's disease. We eagerly anticipate continuing our partnership with you and the patient community as the AMT-130 program progresses.

Sincerely,

Daniel Leonard

Senior Director of Global Patient Advocacy