



Dear Huntington's Disease Patient Organization Leaders,

We are writing to let you know that this morning uniQure issued a press release announcing interim data from 26 patients enrolled in the ongoing HD Gene TRX-1 trial, a phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease being conducted in the United States. The analysis includes up to 24 months of follow-up. The key takeaways are as follows:

- AMT-130 continues to be generally well-tolerated across both dose cohorts
- Patients treated with AMT-130 show preserved function compared to baseline and clinical benefits relative to natural history of the disease
- Neurofilament Light Chain (NfL) in cerebrospinal fluid (CSF) was below baseline at 24 months in patients treated with the low-dose of AMT-130 and declining towards baseline at 12 months in patients treated with the high-dose of AMT-130
- Suppression of CSF mHTT in low-dose cohort supports AMT-130 target engagement; Greater variability observed in high-dose cohort
- Data support continuing clinical development of AMT-130 and pursuing regulatory interactions to discuss late-stage development

The HD Gene TRX-1 trial is exploring the safety, tolerability, and efficacy signals in 26 total patients with early-manifest Huntington's disease. Additional details are available on <http://www.clinicaltrials.gov> (NCT04120493).

Please note that uniQure management will host a conference call and webcast today, Wednesday, June 21, 2023 at 8:30 a.m. ET. Details for the conference call and webcast can be found in the press release.

This morning's webinar is aimed primarily at an investor audience. However, we are also planning to partner with the Huntington's Disease Society of America to do a webinar aimed primarily at the HD patient community in the coming weeks. As soon as we have details, we will share them with you to share with your respective communities.

We are deeply appreciative of the support that we've received from the community and for the pioneering individuals who have volunteered to be a part of the study. Only through such collaborations can we develop safe and effective treatments to modify the course of HD. We look forward to continuing the work with you and the HD community as the AMT-130 program moves forward.

Sincerely,

Daniel Leonard  
Senior Director of Global Patient Advocacy