



Dear Huntington's Disease Patient Organization Leaders,

This morning, uniQure issued a press release announcing interim data from 39 patients enrolled in the ongoing Phase I/II clinical trials of AMT-130, [HD Gene TRX-1 and HD Gene TRX-2], for the treatment of Huntington's disease being conducted in the United States and Europe. The analysis includes up to 30 months of follow-up. The key takeaways are as follows:

- AMT-130 continues to be generally well-tolerated across both dose cohorts
- Patients administered AMT-130 continue to show evidence of preserved neurological function relative to the natural history of the disease
- Neurofilament Light Chain (NfL) in the cerebrospinal fluid (CSF) continues to show a favorable trend with low dose patients below baseline 30 months
- NfL continues to show a favorable trend with high dose patients near baseline at 18 months
- Data support uniQure's continuing clinical development of AMT-130 and pursuing regulatory interactions for ongoing development

In the US, the Phase I/II clinical trial of AMT-130 is exploring the safety, tolerability, and efficacy signals in 26 patients. In Europe, the Phase IB/II clinical trial of AMT-130 is exploring the safety, tolerability, and efficacy signals in an open label clinical trial with 13 patients. Additional details are available on <http://www.clinicaltrials.gov> for HD Gene TRX-1 (NCT04120493) and for HD Gene TRX-2 (NCT05243017).

Please note that uniQure management will host a conference call and webcast today, Tuesday, December 19, 2023 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](http://www.uniqure.com).

This morning's webcast is aimed primarily at the investor audience. However, we are also planning to host a webinar with the Huntington's Disease Society of America intended for the HD community early next year.

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 continue our mission to develop treatments with the aim of modifying the course of Huntington's Disease. We look forward to continuing to work with you and the patient community as the AMT-130 program advances.

Sincerely,

Daniel Leonard  
Senior Director of Global Patient Advocacy