uniQure

A Message to the Huntington's Disease Community from uniQure

Dear Huntington's Disease Community Members,

This morning, uniQure issued a press release which included an update on our clinical trial of AMT-130 (the name of the study drug) for Huntington's Disease (HD.) After a brief pause announced this past August in patient enrollment at the higher-dose level in the European Phase I/II clinical trial of AMT-130, the trial's Data Safety Monitoring Board (DSMB) has recommended that patient enrollment resume at this higher dose. This means that the independent group of expert HD physicians looked at all of the information available to them and deemed the risk-benefit of AMT-130 worthy of proceeding.

The European and U.S. portions of this clinical program are designed to evaluate the safety and tolerability of AMT-130, in addition to helping to determine the optimal dose (how much of the drug is needed) to advance into a late-stage clinical trial. The study also will serve as an early evaluation of AMT-130's ability to lower the huntingtin protein and any related effect this might have on the disease.

This past summer, uniQure was made aware of some unexpected severe reactions shortly after the administration of AMT-130 in the higher-dose group in the trial. At that time, the DSMB met and decided to pause enrollment in the higher-dose group to investigate the safety events and determine any mitigation steps. After a thorough investigation, the DSMB is lifting the pause as noted above. More details can be found in the uniQure press release issued this morning. It is important to note that all of the unexpected safety events in the three patients have fully resolved.

We would like to extend our deepest gratitude to the patients, families and clinical sites that have joined us on our journey to investigate AMT-130 as a possible treatment for HD. Without your participation, our mission to deliver transformative therapies would not be possible. We look forward to continuing the work with the HD community and will continue to share updates on the AMT-130 clinical program.

Sincerely,

Daniel Leonard
Senior Director of Global Patient Advocacy, uniQure