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Update to the Huntington's Disease Community:

Today we shared encouraging results from our PIVOT-HD Phase 2 study of PTC518 in Huntington's disease (HD) patients who have completed 12 months of treatment. The results demonstrated a durable dose-dependent lowering of mutant huntingtin (mHTT) protein in the blood and cerebrospinal fluid (CSF). In addition, there were early signals of favorable CNS effect on key disease functional scales and, importantly, continued safety and tolerability at the 12-month timepoint. We are pleased to report that based on the PIVOT-HD data collected to date, the FDA has lifted the partial clinical hold on the program.

We are pleased with the outcomes achieved and believe the data supports the promise of PTC518 to address the need for an effective and safe disease-modifying therapy for patients living with Huntington's disease. Based on these promising results, we will begin work on the design of the Phase 3 efficacy trial of PTC518.

We share our deep gratitude to patients, families, advocates, researchers and the clinical sites around the world who participated in the trial. Without you, advancements in research and innovation would not be possible.

This encouraging data brings new hope that we can make a difference for HD families. We look forward to advancing PTC518 into late-stage clinical trials and continuing our collaboration with the HD community.

Our patient engagement team is available to speak with you and answer questions. They can be reached at 1-833-PTC-HOPE (+1-833-782-4673), or patientengagement@ptcbio.com.