NOVARTIS

Community Update: Votoplam data from long-term extension study (Phase 2 LTE)

April 28th, 2026

Dear Huntington's Disease Community,

We would like to share an update on our recent interim analysis of the Long-Term Extension (LTE) study of votoplam (HTT227, formerly PTC518).

As previously informed, Novartis entered into a global license and collaboration agreement with PTC Therapeutics for votoplam in December 2024. Novartis has assumed the sponsorship of the Phase 2 Long-Term Extension study (initiated by PTC therapeutics), and is the sponsor of the votoplam Phase 3 study - INVEST-HD. Participants who completed the Phase 2 PIVOT-HD parent study (PTC518-CNS-002-HD / NCT05358717 sponsored by PTC therapeutics) were eligible to enroll in a separate long-term extension (LTE) study "**An Extension Study to Evaluate the Long-Term Safety and Efficacy of Votoplam in Participants With Huntington's Disease (HD)"** NCT number NCT06254482) for up to 54 months.

The topline data from the Long-term extension (LTE) study of votoplam (12 months from PIVOT-HD + 12 months from LTE) supports our confidence in our Phase 3 (INVEST-HD) study which began enrolling people in March 2026. Novartis plans to present 24 months LTE data at a future scientific meeting in 2026. It is important to recognize that all participants in the LTE study are on active treatment, so data must be compared to an external natural history arm rather than an internal placebo arm. We will share more about this in the coming months.

As shared previously, you can view the Phase 3 INVEST-HD study (NCT7326709) information here: Study Details | NCT07326709 | A Study to Investigate the Efficacy, Safety and Tolerability of Votoplam in Participants With Huntington's Disease | ClinicalTrials.gov. Novartis remains committed to transparency, collaboration with the HD community, and sharing updates as the development continues. We encourage anyone who is interested to review the study information posted on ClinicalTrials.gov and to speak to their healthcare provider.

We extend our gratitude to the people living with HD, families, advocates, researchers, and clinical sites worldwide who participated in the PIVOT HD trial and are in the long-term extension study. These contributions are essential for advancing research and innovation.

Kind regards,
Votoplam Clinical Development Team at Novartis
Novartis Pharmaceuticals
novartis.email@novartis.com

FA- 11666427