

July 15th 2025

Community Update:

Novartis development in Huntington Disease (HD)

Dear Huntington's disease Community,

We are pleased to share additional information on our plans following PTC Therapeutics' communication of the results of the PIVOT-HD Phase 2 study of voptlam (PTC518).

Novartis has entered into a global license and collaboration agreement with PTC Therapeutics for voptlam. Novartis will take over the sponsorship of the Long-Term Extension study and any future Phase 3 studies.

We know that there is an urgent need for therapies that could delay the onset or slow the progression of HD.

Voptlam is an HTT mRNA splice modulator with the potential to become the first oral disease-modifying therapy for HD. The PIVOT-HD (sponsored by PTC therapeutics) study evaluated the safety profile of voptlam and its ability to reduce HTT protein levels in people with HDD-IS Stage 2 and mild Stage 3 HD. The topline data from the PIVOT-HD study are encouraging and support moving ahead with a Phase 3 study to gather more data supporting the efficacy and safety of voptlam.

We are currently completing the analysis of the Phase 2 study and engaging with HD experts, health authorities, and patient groups. The Phase 3 protocol will incorporate insights from the HD community and patient advocacy groups. 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 to the people who continue to participate in the long-term extension study. These contributions are essential for advancing research and innovation.

Neuroscience is a core area for the company, focused on making a significant impact for people with severe neurological conditions. While Novartis only recently licensed voptlam, we have experience in HD from a past program that was discontinued due to safety concerns. We are thrilled now to be able to use our expertise in HD and passion for science to advance the development of voptlam for people with HD.

Kind regards,

The Novartis voptlam Clinical Development Team

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