

UNIQUE AMT-130 UPDATES

Nov. 3, 2025 – uniQure released an update on discussions with the FDA about AMT-130, an investigational one-time gene therapy designed to slow Huntington disease (HD) progression. The process for AMT-130 approval may need to be modified, but it has not been halted.

What this means is the timing for uniQure's FDA submission for AMT-130 is now unclear. uniQure remains fully committed to working with the FDA to determine the best path forward.

[Click here to read uniQure's letter to the community.](#)

Sept. 24, 2025 – uniQure has an ongoing clinical trial in the United States and the United Kingdom testing gene therapy AMT-130, which is not currently available in Canada. They have released topline results from their phase I/II trial showing for the first time that a drug can slow the progression of Huntington disease (HD) in people. This update is very encouraging, but it is still early on in the process. These results are from fewer than 30 early manifest participants who have received the treatment so far, so they need to be taken with caution.

uniQure will meet the FDA later in 2025, aiming to file for approval in early 2026, while scaling up manufacturing and surgical capacity. The findings further support huntingtin-lowering as a therapeutic strategy, and boost prospects for other treatments in development, all made possible by the extraordinary commitment of trial participants and the broader HD community.

The Huntington Society of Canada is following the research closely, sharing updates with the community, and talking with researchers and companies about bringing trials and new treatments to Canada. While we cannot speed up approvals, we are committed to keeping Canadian families informed and advocating for access.

[Click here to read uniQure's press release.](#)

[Click here to read the HDBuzz article.](#)

If you would like to connect with a member of the Family Services team, please reach out to your RCD or, if you're new to HSC, please fill out our online form:

<https://contactme.cloud/form/huntingtonsociety>