ALN-HTT02 Overview

An Investigational RNAi Therapeutic

Disease State(s) Under Investigation	Huntington's disease[1]
MoA	Designed to target production of all huntingtin protein (HTT) species in the CNS[2]
Route of Administration	Intrathecal[1]

Clinical Trials Overview

Phase 1	Treatment Arms	Primary Endpoint
Active, enrolling		
Safety, Tolerability, PK, and PD of **ALN-HTT02** Adult Patients (25–70 years) with Huntington's Disease (Stage 2 or early 3)[1,2]	ALN-HTT02; placebo	Safety and tolerability (frequency of AEs)

The ALN-HTT02 clinical program is being conducted in partnership with Regeneron.

The safety and efficacy of ALN-HTT02 has not been established by any health authority.

AE, adverse event; CNS, central nervous system; HTT, huntingtin protein; MoA, mechanism of action; PD, pharmacodynamics; PK, pharmacokinetics; RNA, ribonucleic acid; RNAi, RNA interference. 1. ClinicalTrials.gov. 2025. https://clinicaltrials.gov/study/NCT06585449 (Accessed February 17, 2026). 2. Sloan K et al. European Huntington's Disease Network and Enroll-HD Congress, September 12–14, 2024, Strasbourg, France. Oral presentation.

Alnylam