ForPatients
by Roche

Huntington Disease (HD)

A Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of RG6496 in Huntington's Disease

Trial Status	Trial Runs In	Trial Identifier
Recruiting	1 Country	NCT07246941 2025-520631-17-00 BP45378

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

A Phase I, 2-Part Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Single-ascending Doses of Intrathecally Administered RG6496 in a Randomized, Placebo-controlled, Investigator/Participant-blind Study With an Open-label Extension in Huntington's Disease Gene Expansion Carriers

Trial Summary:

This is a first-in-human (FIH) study of RG6496 that will assess the safety and tolerability of single-ascending doses of RG6496 administered to huntington's disease gene expansion carriers (HDGECs). The study consists of two parts: Part 1 [single-ascending dose] followed by Part 2 [open-label extension (OLE)].

Hoffmann-La Roche
Sponsor

Phase 1
Phase

NCT07246941 2025-520631-17-00 BP45378
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	#25 Years & # 65 Years	No

Inclusion Criteria:

Part 1

- Confirmation of HDGEC status with cytosine-adenine-guanine (CAG) expansion > 39.

- Confirmation of SNP carrier status of the target SNP
- Independence Scale (IS) score of #70, total functional capacity (TFC) #10, total motor score (TMS) >6.
- Ability to read the words "red," "blue," and "green" and be fluent in the language of the informed consent form (ICF) and the tests used at the study site.
- Ability to walk unassisted.
- Total body weight > 40 kilogram (kg) and body mass index (BMI) within the range 18-32 kilogram per square meter (kg/m^2) (inclusive) at baseline.
- Ability to undergo and tolerate MRI scans.

Part 2

- Completed the post-dose safety follow-up period in the Part 1 of the study.
- In the opinion of the Investigator, the participant has not experienced a worsening in health that precludes their safe continued participation in the study.

Exclusion Criteria:

Part 1

- Concurrent or planned participation in any interventional clinical study, including current use of an ASO or any HTT-lowering therapy or treatment with investigational therapy within 90 days or 5 drug-elimination half-lives, whichever is longer prior to screening
- Pregnant or breastfeeding, or with the intention of becoming pregnant during the study or within the timeframe in which contraception is required
- Malignancy within 5 years prior to screening
- Planned brain surgery during the study
- Positive HIV test, hepatitis B surface antigen and hepatitis B surface antigen at screening
- Active psychosis, confusional state, or violent behavior, including aggression that could cause harm to self or others, over the 12 weeks prior to screening.
- Current or previous history of a primary independent psychotic disorder.
- Scoliosis or spinal deformity or surgery making IT injection not feasible in an outpatient setting
- History of attempted suicide or suicidal ideation with plan (i.e., active suicidal ideation) that required hospital visit and/or change in level of care within 12 months prior to screening.

Part 2

- Prematurely discontinued from Part 1 for any reason (i.e., before the completion of the postdose safety follow-up period of Part 1).
- Pregnant or breastfeeding, or with the intention of becoming pregnant during the study or within the timeframe in which contraception is required.
- Concurrent or planned participation in any interventional clinical study, including current use of an ASO or any HTT-lowering therapy
- Received any active investigational treatment other than RG6496 during or since completion of Part 1 of the study.
- Had confirmed Dose-Limiting Adverse Event (DLAE)(s) in Part 1 of the study.