ForPatients
by Roche

Huntington Disease (HD)

A Study to Investigate the Pharmacokinetics and Pharmacodynamics of RO7234292 (RG6042) in CSF and Plasma, and Safety and Tolerability Following Intrathecal Administration in Patients With Huntington's Disease

Trial Status	Trial Runs In	Trial Identifier
Completed	2 Countries	NCT04000594 GEN-PEAK BP40410

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:

An Open-Label Adaptive Multiple-Dose Study to Investigate the Pharmacokinetics and Pharmacodynamics of RO7234292 in CSF and Plasma, and Safety and Tolerability Following Intrathecal Administration in Patients With Huntington's Disease

Trial Summary:

Study BP40410 is an open-label, adaptive multiple-dose clinical study designed to characterize the PK of RO7234292 (RG6042) in plasma and CSF as well as the acute time course and recovery profile of CSF mHTT lowering in response to RO7234292 (RG6042) treatment after intrathecal (IT) administration of RO7234292 (RG6042) to patients with manifest Hungtington's disease (HD).

Hoffmann-La Roche	Phase 1
Sponsor	Phase

NCT04000594 GEN-PEAK BP40410
Trial Identifiers

Eligibility Criteria:

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

Inclusion Criteria:

- Manifest HD diagnosis
- Independence Scale score of >=70.
- Genetically confirmed disease by direct deoxyribonucleic acid testing with a cytosine, adenine, and guanine base sequence found in DNA which is translated into glutamine (CAG) age product (CAP) score > 400.
- Ability to read the words "red," "blue," and "green" in the patient's native language.
- Ability to walk unassisted without a cane or walker and move about without a wheelchair on a daily basis as reviewed at screening and baseline visit.
- Ability to undergo and tolerate MRI scans.

Exclusion Criteria:

- 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.
- Current active psychosis, confusional state, or violent behavior.
- Any serious medical condition or clinically significant laboratory, vital signs, or ECG abnormalities at screening that, in the Investigator's judgment, precludes the patient's safe participation in and completion of the study.
- Clinical diagnosis of chronic migraines or history of low pressure headache after lumbar puncture requiring hospitalization or blood patch.
- Treatment with investigational therapy within 4 weeks prior to screening or 5 drug elimination half-lives of investigational therapy, whichever is longer.
- Concurrent or planned concurrent participation in any interventional clinical study, including explicit pharmacological and non-pharmacological interventions. Observational studies are acceptable.
- Unable or unsafe to perform lumbar puncture on the patient.
- Previous lumbar surgery that is likely, in the opinion of the Investigator or surgical team, to make IT catheter insertion or IT injection unduly difficult or hazardous.
- Poor peripheral venous access.
- Scoliosis or spinal deformity making IT injection not feasible in the outpatient setting.
- Preexisting intra-axial or extra-axial lesions as assessed by a centrally read MRI scan during the screening period.