ForPatients
*by Roche*

Huntington Disease (HD)

# A Randomized Study of SPK-10001 Gene Therapy in Participants With Huntington's Disease

| Trial Status | Trial Runs In | Trial Identifier |
|---|---|---|
| Recruiting | 1 Country | NCT06826612 SPK-10001-101 |

*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 1/2, Randomized, Sequential, Dose-Escalation Study to Evaluate the Safety, Tolerability, and Efficacy of a One-Time, Bilateral, Intraparenchymal Infusion of SPK-10001 Into the Caudate and Putamen in Participants With Huntington's Disease

## *Trial Summary:*

The main goal of this study is to evaluate the safety, tolerability, and preliminary efficacy of SPK-10001 in participants with Huntington's Disease.

**Hoffmann-La Roche**
Sponsor

**Phase 1/Phase 2**
Phase

**NCT06826612 SPK-10001-101**
Trial Identifiers

## *Eligibility Criteria:*

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

## *Inclusion Criteria:*

- Have confirmed huntingtin (HTT) cytosine-adenine-guanine (CAG) repeat length #40 on genetic testing and confirmation diagnostic test by the central laboratory (CL) at screening.
- Have striatal atrophy demonstrated by caudate/intracranial volume less than the age-adjusted cutoff values associated with HDISS Stage 1.
- Have UHDRS Total Motor Score (TMS) equal to or greater than the age-adjusted cutoff value associated with HDISS Stage 2.
- Have UHDRS Total Functional Capacity (TFC) greater than or equal to 11.

- Use of cholinesterase inhibitors, memantine, amantadine, or riluzole must have been at stable dosing for at least 12 weeks before screening and baseline and anticipated to remain stable during the first 12 months after SPK-10001 administration.
- Antidepressant or benzodiazepine use must have been at stable dosing for at least 12 weeks before screening and baseline and anticipated to remain stable during the first 12 months after SPK-10001 administration.
- Antipsychotics for motor symptoms or mood stabilization (i.e., irritability or aggressive behavior) and/or tetrabenazine, valbenazine, or deutetrabenazine must have been at a stable dose for at least 12 weeks before screening and baseline and are anticipated to remain stable during the first 12 months after SPK-10001 administration.

## *Exclusion Criteria:*

- A safe trajectory is not able to be identified for targeting placement of the cannula into the caudate or putamen on both sides of the brain due to extent of atrophy or other anatomical features.
- Have received an antisense oligonucleotide therapy during the past year.
- History of deep brain stimulation.
- History of or intention to undergo gene therapy, cell transplantation, or brain surgery during the course of the study.
- Have participated in an investigational drug study with a systemic administration within 6 weeks or 5 half-lives of screening, whichever is longer.

Additional protocol-defined inclusion/exclusion criteria apply.