

A Study to Investigate the Efficacy, Safety and Tolerability of Votoplam in Participants With Huntington's Disease

Status: RECRUITING

Eligibility Criteria

Age: 21 years to 70 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

* Signed informed consents must be obtained prior to participation in the study * Ambulatory male or female participants between 21 to 70 years of age, inclusive, on the day of Informed Consent signature * Genetically confirmed HD diagnosis with a cytosine-adenine-guanine (CAG) repeat length of 40 or above. Participants must have prior genetic confirmation and known CAG repeat length obtained prior to screening. * Meets all of the following criteria: * UHDRS IS score ≥ 90 * UHDRS TFC score = 13 * UHDRS TMS score = 7-25, inclusive * CAP100 ≥ 70 Calculation: CAP = Age at study entry $\times$ (CAG length

$\div 30$) / 6.49

Exclusion Criteria:

* History of gene therapy or cell transplantation or any other experimental brain surgery for the treatment of HD * Serologic evidence for active viral hepatitis as indicated by: * positive anti-HBc IgM * positive anti-HBc IgG confirmed by positive HBsAg and/or HBV DNA * positive HCV ab test confirmed by positive HCV RNA * Immunodeficiency diseases, including a positive human immunodeficiency virus (HIV) test result * History or current diagnosis of ECG or cardiac abnormalities indicating significant risk of safety for participants such as: * Concomitant clinically significant cardiac arrhythmias, e.g., sustained ventricular tachycardia, and clinically significant second- or third-degree AV block without a pacemaker * History of familial long QT syndrome or known family history of Torsade de Pointes * Women of childbearing potential, defined as all women physiologically capable of becoming pregnant from menarche until becoming post-menopausal, unless they have had surgical bilateral oophorectomy (with or without hysterectomy), total hysterectomy or bilateral salpingectomy at least six weeks before taking study treatment. In the case of oophorectomy alone, the reproductive status of the woman needs to have been confirmed by follow-up hormone level assessment. o WOCBP are excluded unless they are using highly effective methods of contraception (failure rate $\leq 1\%$ per year) while taking study treatment and for 8 months after stopping study treatment. * Pregnant or nursing (breastfeeding) women Other protocol defined inclusion/exclusion criteria may apply

Conditions & Interventions

Interventions:

DRUG: Votoplam (blinded), DRUG: Placebo

Conditions:

Huntington Disease

Keywords:

HD, INVEST-HD, HTT227, VOTOPLAM, HUNTINGTON DISEASE, UHDRS, mHTT, NfL

More Information

Contact(s): Novartis Pharmaceuticals - novartis.email@novartis.com

Principal Investigator:

IRB

Number:

System ID: NCT07326709

Thank you for choosing StudyFinder. Please visit <http://studyfinder.cctr.vcu.edu> to find a Study which is right for you and contact ctrrecruit@vcu.edu if you have questions or need assistance.