PROOF-HD Phase 3 Pridopidine Trial Reaches 25% Enrollment

huntingtonsdiseasenews.com/2021/05/25/proof-hd-trial-testing-pridopidine-huntingtons-disease-reaches-25-percent-enrollment/

May 25, 2021

Prilenia Therapeutics has enrolled more than 120 patients in PROOF-HD, a Phase 3 clinical trial investigating the efficacy and safety of oral pridopidine in early stage Huntington's disease.

That amounts to 25% of the target enrollment of up to 480 patients, the company announced. The trial is ongoing at 60 sites in the U.S., Canada, and Europe.

With those enrollment numbers, PROOF-HD (NCT04556656), conducted in collaboration with the Huntington Study Group, remains on track to complete recruitment by the last quarter of 2021, according to Prilenia.

To enter the study, participants must be age 25 or older and have a clinical diagnosis of early stage, adult-onset Huntington's disease.

More information on who is eligible to participate in the study can be found here. Trial information and contacts are detailed here.

Pridopidine is a small molecule currently in development by Prilenia for the treatment of Huntington's and other neurodegenerative diseases, such as amyotrophic lateral sclerosis (ALS). The therapy is designed to enter the brain and spinal cord, where it activates the sigma-1 receptor or S1R, a protein expressed at high levels in brain cells. There, S1R regulates various cellular mechanisms that are important for the maintenance of neuronal health and function.

By activating S1R, pridopidine increases the production of brain-derived neurotrophic factor (BDNF), a protein with neuroprotective effects, whose levels are reduced in people with Huntington's.

Studies in animal models and human cells have shown that pridopidine may prevent neuronal cell death while strengthening the connections between neurons.

A previous Phase 2 trial (NCT02006472), called PRIDE-HD, showed that one year of treatment with pridopidine — in a dose of 45 mg given twice a day — significantly prevented a decline in function in people with early stage Huntington's. Results from a Phase 2 long-term extension study, called OPEN-HART (NCT01306929), also supported these benefits.

Building on these findings, the PROOF-HD study was designed to evaluate the effect of the same dosage of pridopidine versus a placebo on functional capacity over 65 weeks (about 16 months).

Functional capacity will be measured using the Unified Huntington's Disease Rating Scale–Total Functional Capacity (UHDRS-TCF) — a tool used to assess Huntington's stage and the level of a patient's functionality. Motor and behavioral assessments also will be done.

To date, pridopidine has raised no safety issues, and no participants have dropped out from the study. This is consistent with the previously established favorable safety and tolerability profile of the medication.

To help further broaden the scientific strategy for pridopidine, Prilenia has appointed Y Paul Goldberg, PhD, as chief scientific officer and head of early clinical development. Goldberg will set the early clinical development plan for pridopidine in new indications and evaluate opportunities for expanding the company's pipeline.

"Joining Prilenia presents a fantastic opportunity to help advance a new and promising oral therapy offering relief for severely undertreated neurological diseases. I look forward to working with the team to accelerate the development of pridopidine, to potentially provide a much needed treatment option for patients and their families," said Goldberg.

Pridopidine has received orphan drug designation in both the U.S. and Europe for the treatment of Huntington's disease.