HD COMMUNITY UPDATE

News for the Huntington's Disease Patient & Advocacy Community

New Confirmatory "PRECISE-HD" study of Pridopidine in Huntington's Disease Ready to Start Recruitment in June

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Dear HD Community,

We would like to share an update on the confirmatory **PRECISE-HD** study (Pridopidine Phase 3 Study to Establish Clinical Impact and Safety in Huntington's Disease (HD)), a global clinical study evaluating the investigational medicine **pridopidine** in people living with Huntington's disease. The study has now been listed on **www.clinicaltrials.gov** (NCT07609108).

Incorporating past study learnings, extensive input from the patient and research communities, and advice from regulatory authorities, PRECISE-HD is intended to collect important data on the efficacy and safety of pridopidine and is designed to generate robust data to further support regulatory evaluation. Site initiation is underway, and **recruitment is expected to commence at the first sites in the US in June 2026,** followed by sites in other countries on a rolling basis later in the year.

What is the PRECISE-HD study?

Currently, there are no approved treatments to slow or stop HD from progressing.

PRECISE-HD is a confirmatory Phase 3 clinical study evaluating the efficacy and safety of the investigational medicine pridopidine (taken as an oral capsule twice a day) in people living with HD. The study design was informed by previous research, input from the HD community, and discussions with regulatory authorities.

The study plans to enroll **400 people** living with HD, from early to mid-stage disease (defined as Total Functional Capacity (TFC) score of 7-13), and a Total Motor Score (TMS) of ≥20, meaning people who have experienced motor changes or impact on independent functioning. This population is intended to enable the appropriate assessment of any potential effect of the therapy on disease progression in comparison to placebo.

PRECISE-HD will consist of two sequential stages:

Stage 1 — Placebo-controlled (52 weeks): Participants receive either pridopidine or placebo (half receiving pridopidine and half placebo). Neither participants nor their doctors will know which they are receiving.

Stage 2 — Open-label extension (104 weeks): All (eligible) participants, regardless of the treatment received during Stage 1, will be assigned to receive pridopidine for two years, allowing researchers to assess longer-term effects over up to three years in total.

The study will evaluate a range of HD-related outcomes, with the primary endpoint being the change from baseline to Week 52 in the combined Unified Huntington's Disease Rating Scale (cUHDRS) score. Several other endpoints, including function, motor, cognition, speech and quality of life, will be also measured, along with the safety and tolerability of pridopidine.

The study is planned to be conducted at up to 75 study sites globally, including in the US, EU, UK and Canada. Recruitment is expected to commence at the first sites in the US in June 2026, followed by sites in other countries on a rolling basis later in the year.

People who would like more information about PRECISE-HD can speak with their neurologist or HD specialist. They may also contact their local HD patient advocacy organization for additional guidance and support. Eligibility, study procedures, and any potential risks and benefits will be explained by the study team at the investigational sites during the informed consent process.

What is pridopidine?

Pridopidine is an investigational medicine, and its safety and efficacy have not been established, participants may or may not benefit from taking part in this study.

It exerts its effect by activating a protein in the brain called the sigma-1 receptor (S1R) and is often referred to as an S1R agonist. S1R has been shown to play a role in stimulating multiple neuroprotective pathways impaired in neurodegenerative diseases, such as HD. The clinical effects of pridopidine on HD progression will be evaluated in this study.

To date, more than 1,600 people have received pridopidine in clinical studies — the majority from HD trials — with some participants on active treatment for up to seven years. Across these studies, pridopidine has shown a generally favorable safety and tolerability profile[i].

Who is sponsoring the PRECISE-HD study

PRECISE-HD is being undertaken by **Prilenia** and **Ferrer**, who are co-developing pridopidine in partnership. **Prilenia** is a private biopharmaceutical company driven by an unwavering commitment to scientific excellence and accelerating progress for people affected by Huntington's disease (HD), amyotrophic lateral sclerosis (ALS) and other neurodegenerative disorders. **Ferrer** is a B Corp-certified international pharmaceutical company that uses business to fight for social justice and offers transformative solutions for life-threatening diseases in more than one hundred countries with a growing focus on rare neurological disorders.

Prilenia and Ferrer are also studying pridopidine in a Phase 3 study in ALS.

For more information on the companies, please visit www.prilenia.com or www.ferrer.com.

For more information on PRECISE-HD, visit www.clinicaltrials.gov (NCT07609108) and a PRECISE-HD study website (www.precisehdtrial.com) will be launched soon.

PRECISE-HD
PRIDOPIDINE PHASE III STUDY TO ESTABLISH CLINICAL IMPACT AND SAFETY IN HUNTINGTON'S DISEASE

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⚠ Important Note

Pridopidine is an investigational medicine and is not approved for use by any regulatory authority. Its safety and efficacy have not yet been established. This update is for informational purposes only and does not constitute medical advice. Please speak with your healthcare provider before making any decisions about treatment or participation in a clinical study.

PRECISE-HD CLINICAL STUDY

Questions & Answers for the HD Community

This document answers common questions about the PRECISE-HD study, the drug being tested, who may be eligible, and what participation involves. It is intended for people living with HD, their families, carers, and advocacy groups. It is not medical advice — please speak with your healthcare provider for personal guidance.

About Pridopidine

Q: What is pridopidine?

A: Pridopidine is an investigational medicine, which means it is not approved by any regulatory authority. It exerts its effect by activating a protein in the brain called the sigma-1 receptor (S1R) and is often referred to as an S1R agonist. In preclinical models of neurodegenerative diseases including HD, modulation of S1R enhances/activates key neuroprotective pathways that may have an impact on HD progression, pridopidine has been shown to support neuronal cell function and survival via activation of S1R. The clinical effects of pridopidine will be evaluated in this study.

Q: Has pridopidine been tested in people with HD before?

A: Yes. Pridopidine has been evaluated in previous clinical studies in people with Huntington's disease. To date, more than 1,600 people have received pridopidine in clinical studies — the majority from HD trials — with some participants on active treatment for up to seven years. Across these studies, pridopidine has shown a generally favorable safety and tolerability profile. Further data is being collected to better understand its safety and efficacy.

Findings from earlier studies, input from the patient and research communities, and discussions with regulatory authorities have informed the design of PRECISE-HD.

Q: Is pridopidine approved for use?

A: No. Pridopidine is an investigational medicine and has not been approved for use by any regulatory authority anywhere in the world. Its safety and efficacy have not yet been established.

About the Study

Q: What is PRECISE-HD?

A: PRECISE-HD, or Pridopidine Phase 3 Study to Establish Clinical Impact and Safety in Huntington's Disease (HD), is a global clinical study evaluating the investigational medicine pridopidine, taken as an oral capsule twice a day, in people living with Huntington's disease. The study is being conducted by Prilenia and Ferrer.

Q: How large is the study and where is it taking place?

A: The study plans to enroll approximately 400 participants across up to 75 study sites globally, including in the US, EU, UK and Canada. Recruitment is expected to commence at the first sites in the US in June 2026, followed by sites in other countries on a rolling basis later in the year.

Q: How is the study structured?

A: The study has two consecutive stages. The first stage lasts 52 weeks, during which participants are randomly assigned with the same chance to receive either pridopidine or a placebo. Neither participants nor their doctors know which treatment they are receiving. This is called a 'double-blind' design and is the gold standard for clinical research. After this first stage, all eligible participants may enter Stage 2, a 104-week open-label extension in which all participants will receive pridopidine.

Study Participation

Q: Who is PRECISE-HD for?

A: PRECISE-HD is intended for people living with HD, from early to mid-stage disease (defined as Total Functional Capacity (TFC) score of 7-13), meaning people who have experienced motor changes or impact on independent functioning. This population is intended to enable the appropriate assessment of any potential effect of the therapy on disease progression in comparison to placebo.

Q: How do I find out if there is a study site near me?

A: We are currently initiating study sites and expect PRECISE-HD to be undertaken at up to 75 study sites globally, including in the US, EU, UK and Canada. The full list of sites will be available on www.clinicaltrials.gov (NCT07609108) (the official registry of clinical studies) and a PRECISE-HD study website (www.precisehdtrial.com) will be launched soon. People seeking more information may also speak with their neurologist or HD specialist, or may contact their local HD patient organization, for general guidance and support.

Aims of the Study

Q: What is the study trying to show?

A: The study is trying to show if pridopidine can slow down the progression of HD in comparison to placebo. The primary end point of PRECISE-HD is to evaluate the change from baseline to Week 52 in the combined Unified Huntington's Disease Rating Scale (cUHDRS). The study will also assess additional endpoints, including measures of function, motor, cognition, speech, quality of life, safety, and tolerability.

Safety and tolerability of pridopidine also will be further evaluated.

Q: Why is speech being assessed in this study?

A: Speech difficulties are a common and distressing aspects of HD that significantly impacts quality of life and communication. Despite this, speech has historically been under-measured in HD clinical studies. Recognizing the unmet need in this area, speech is one of the outcomes also being assessed in PRECISE-HD as an endpoint, using technology-based, objective and quantitative methodologies to collect additional information on speech changes during the study.

Q: Who is running the study?

A: PRECISE-HD is being conducted by Ferrer and Prilenia, who are co-developing pridopidine in partnership. Ferrer is an international pharmaceutical company with a growing focus on rare neurological disorders. Prilenia is a biopharmaceutical company involved in the development of investigational therapies for neurodegenerative disorders.

General information about the sponsoring companies is also available on www.ferrer.com and www.prilenia.com.

Next Steps & Contact

Q: How can I find out more or express interest in the study?

A: People who would like more information about PRECISE-HD can speak with their neurologist or Huntington's disease specialist. Local HD patient organizations may also be able to provide general guidance and support. More information and the full list of study sites will be available on www.clinicaltrials.gov (NCT07609108) (the official registry of clinical studies).

The full eligibility, study procedures, and any potential risks and benefits will be explained by the study team during the informed consent process. Participation in any clinical study is completely voluntary.

We also expect to launch a PRECISE-HD website (www.precisehdtrial.com) soon, which will contain additional information.

Important Disclaimer

Pridopidine is an investigational medicine that has not been approved by any regulatory authority. Its safety and efficacy have not been established. This document is intended for informational purposes only and does not constitute medical advice. Patients and families should consult their healthcare provider regarding treatment decisions or clinical study participation. Local regulations may vary.

[i] Goldberg YP, Navon-Perry L, Cruz-Herranz A, Chen K, Hecker-Barth G, Spiegel K, Cohen Y, Niethammer M, Tan AM, Schuring H, Geva M, Hayden MR. The Safety Profile of Pridopidine, a Novel Sigma-1 Receptor Agonist for the Treatment of Huntington's Disease. CNS Drugs. 2025 May;39(5):485-498. doi: 10.1007/s40263-025-01171-x. Epub 2025 Mar 7. PMID: 40055280; PMCID: PMC11982116.