NEWS RELEASE

Prilenia and Ferrer Announce Initiation of the Confirmatory PRECISE-HD Study of Pridopidine in Huntington's Disease

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PRECISE-HD (Pridopidine Phase 3 Study to Establish Clinical Impact and Safety in Huntington's Disease (HD)) is designed to further evaluate the efficacy and safety of pridopidine, an oral investigational drug, on disease progression, functional capacity, motor function, cognition, speech and Quality of life

Focused on generating the data to further support regulatory evaluation, PRECISE-HD will be undertaken in up to 75 sites globally, including the US, EU, UK and Canada. Recruitment has now commenced in the US, with other countries expected to follow later in the year

NAARDEN, Netherlands & WALTHAM, Mass. & BARCELONA, Spain--(BUSINESS WIRE)-- **Prilenia Therapeutics B.V.** and **Ferrer**[i] today announced initiation of the confirmatory PRECISE-HD study (Pridopidine Phase 3 Study to Establish Clinical Impact and Safety in Huntington's Disease (HD) (**NCT07609108**)). Recruitment has now commenced in the US, with other countries expected to follow on a rolling basis later in the year.

PRECISE-HD Study

Focused on further evaluating the efficacy and safety of pridopidine, an investigational medicinal product (taken as an oral capsule twice a day), and the generation of data to support registration, the confirmatory 400-participant randomized, double-blind, placebo-controlled PRECISE-HD study will assess the clinical impact of pridopidine on disease progression, functional capacity, motor function,

cognition, speech and quality of life (QoL).

Incorporating learnings from prior research and advice and input from the HD patient and research community and regulatory authorities, the study will focus on people living with HD, from early to mid-stage disease (defined as Total Functional Capacity (TFC) score of 7-13), a Total Motor Score (TMS) ≥20 and Independence Scale score ≤ 90%, meaning people who have experienced some motor changes or impact on independent functioning. This will allow for appropriate assessment of any potential effect of the therapy on disease progression in comparison to placebo. PRECISE-HD will be undertaken in up to 75 sites globally, including the US, EU, UK and Canada.

Katie Jackson, President/CEO of Help 4 HD International, remarked: "For families affected by Huntington's disease, each day is a race against time. There are currently no available treatments to slow the progression of HD, which gradually diminishes every bit of quality of life. Despite these challenges, the therapeutic needs of the HD community remain significantly underserved. Every study aimed at changing this reality is critically important. It is essential that this study be completed promptly so its results—and their potential impact on individuals with HD and their families, who have already waited far too long for progress—can be fully evaluated."

Victor Sung, M.D. Director of the Division of Movement Disorders in the University of Alabama, Director of the UAB Huntington's Disease Clinic, Director of the HDSA Center of Excellence and PRECISE-HD Steering Committee Member said: "Across prior studies, pridopidine has shown meaningful clinical effects in specific circumstances[ii], and this study has been carefully designed to confirm those effects, and uniquely incorporates prior learnings and novel elements specifically designed to provide treatment effect clarity."

PRECISE-HD is comprised of two consecutive stages: A 52-week placebo-controlled stage with participants randomized to receive either pridopidine or placebo on a 1:1 basis. This will be followed by a 104-week open-label extension stage, in which all eligible participants will receive pridopidine, allowing researchers to assess any potential effect on disease progression for up to three years in total, compared to matched external control cohorts from longitudinal, multinational observational studies[iii].

PRECISE-HD 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.

Further details on the PRECISE-HD study can be found at: **www.clinicaltrials.gov NCT07609108**. A PRECISE-HD study website (**www.precisehdtrial.com**) will also be made available shortly.

ENDS

[This press release is intended for informational purposes only. Pridopidine is an investigational medicine and is not approved for commercial use by any regulatory authority. Its safety and efficacy have not been established. Information contained herein does not constitute medical advice. Patients should consult their healthcare provider for guidance regarding diagnosis or treatment options. Local regulations may vary; this release is not intended to promote or advertise any product.]

About pridopidine

Pridopidine (taken as an oral capsule twice a day) is an investigational highly selective, oral sigma-1 receptor (S1R) agonist. S1R has been shown to play a role in stimulating multiple neuroprotective pathways impaired in neurodegenerative diseases, such as Huntington's disease (HD) and amyotrophic lateral sclerosis (ALS)[iv].

In clinical studies to date, pridopidine has shown a generally favorable safety and tolerability profile, with data from more than 1,600 people (mostly from HD studies), some of whom have received active treatment for up to seven years[v].

In addition to HD, pridopidine is in late-stage clinical development for ALS. The currently recruiting pivotal Phase 3 PREVAiLS trial in ALS is guided by findings from the Phase 2 HEALEY ALS Platform trial in patients with rapidly progressing disease early in their disease (defined as definite or probable ALS by El Escorial Criteria (EEC) and <18 months since symptom onset)[vi].

The investigational drug pridopidine has Orphan Drug designation in HD and ALS in the US and EU, and FDA Fast Track designation for the treatment of HD. These designations do not imply that pridopidine has been approved or that safety and efficacy have been established.

About Huntington's Disease

Huntington's disease (HD) is a rare, inherited, autosomal dominant, neurodegenerative disease that results in functional, motor, cognitive and behavioral symptoms, and ultimately leads to death. HD is caused by a mutation in the huntingtin (HTT) gene[vii], and each child of a parent with HD has a 50 percent chance of developing the disease.[viii]

Across the world an estimated 100,000 people have HD[ix,x], with an additional 300,000 people at risk of developing HD[xi,xii]. It is usually diagnosed between the ages of 30 and 50, although HD can occur at any age, including in children and young adults (known as juvenile onset HD or JHD). The disease progresses slowly over 15 to 20 years, with patients slowly losing their ability to work, communicate, manage day-to-day life and take care of themselves. This increasing disability leads to full reliance on a caregiver and, ultimately, death.

The only currently available treatments for HD focus on symptomatic relief and palliative care, with no approved therapies shown to impact measures of overall disease progression.

About Prilenia

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. Our mission is simple but urgent: to develop and provide sustainable access to transformative medicines for people affected by devastating neurodegenerative diseases.

Prilenia is partnered with Ferrer for the commercialization and co-development of pridopidine.

The company is incorporated in the Netherlands and backed by leading life sciences investors.

For more information, please visit **www.prilenia.com**, and connect with us on **LinkedIn** or **X (Twitter)**.

About Ferrer

At Ferrer, we use business to fight for social justice. We have long been a company that wants to do things differently; instead of maximizing shareholder returns, we reinvest much of our profit in initiatives that give back to society. Back where it belongs. We go beyond compliance and are guided by the highest standards of sustainability, ethics and integrity. As such, since 2022, we are a B Corp.

Founded in Barcelona in 1959, Ferrer offers transformative solutions for life-threatening diseases in more than one hundred countries. In line with our purpose, we have an increasing focus on pulmonary vascular and interstitial lung diseases and rare neurological disorders. Our 1,800-strong team is driven by a clear conviction: our business is not an end in itself, but a way to change lives.

We are Ferrer. Ferrer for good. **www.ferrer.com**

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[i] In April 2025, Prilenia and Ferrer signed a commercialization and co-development agreement for pridopidine. **https://news.prilenia.com/press-releases/press-release-details/2025/Prilenia-Enters-into-a-Collaboration-and-License-Agreement-with-Ferrer-for-the-Commercialization-and-Co-Development-of-Pridopidine-in-Europe-and-Other-Select-Markets/default.aspx**

[ii] Reilmann, R., Feigin, A., Rosser, A.E. et al. Pridopidine in early-stage manifest Huntington's disease: a phase 3 trial. Nat Med 31, 3780–3789 (2025). **https://doi.org/10.1038/s41591-025-03920-3**

[iii] ENROLL-HD and TRACK-HD

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[vi] Writing Committee for the HEALEY ALS Platform Trial, HEALEY ALS Platform Trial Study Group. Pridopidine in Amyotrophic Lateral Sclerosis: The HEALEY ALS Platform Trial. JAMA. 2025;333(13):1128–1137. doi:10.1001/jama.2024.26429

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[viii] Myers RH. Huntington's disease genetics. NeuroRx. 2004 Apr;1(2):255-62. doi: 10.1602/neurorx.1.2.255. PMID: 15717026; PMCID: PMC534940.)

[ix] **https://medically.roche.com/global/en/medical-material/HSG-2019-poster-yohrling-prevalence-of-huntington-s-disease-in-the-US-pdf.html**

[x] Pringsheim T, Wiltshire K, Day L, Dykeman J, Steeves T, Jette N. The incidence and prevalence of Huntington's disease: a systematic review and meta-analysis. Mov Disord. 2012 Aug;27(9):1083-91. doi: 10.1002/mds.25075. Epub 2012 Jun 12. PMID: 22692795.

[xi] Medina et al., Prevalence and Incidence of Huntington's Disease: An Updated Systematic Review and Meta-Analysis. Mov Disord. 2022 Dec;37(12):2327-2335.

[xii] Jiang, A., Handley, R. R., Lehnert, K., & Snell, R. G. (2023). From Pathogenesis to Therapeutics: A Review of 150 Years of Huntington’s Disease Research. International Journal of Molecular Sciences, 24(16), 13021. **https://doi.org/10.3390/ijms241613021**

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