NEWS RELEASE

Prilenia and Ferrer to Share New Data and Analyses of Pridopidine's Impact on Huntington's Disease at the Huntington Study Group (HSG) Congress

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Prilenia and Ferrer will present five posters at the 2025 HSG Huntington's Disease (HD) Clinical Research Congress, including new analyses of 2-year data following patients not taking antidopaminergic medicines (ADMs) through both double-blind and open-label phases of PROOF-HD

Data demonstrates significant and clinically meaningful slowing of progression and significantly less decline across multiple endpoints of function, cognition and motor features in pridopidine-treated patients compared to both placebo and propensity-matched natural history cohorts including Track-HD and Enroll-HD[i]

Prilenia and Ferrer plan a global confirmatory clinical trial of pridopidine in early-stage HD patients to commence in 1H2026

NAARDEN, The Netherlands & WALTHAM, Mass. & BARCELONA, Spain--(BUSINESS WIRE)-- **Prilenia Therapeutics B.V.** and **Ferrer** today announced the presentation of five posters outlining slowing of clinical progression and additional pridopidine data at the 2025 HSG HD Clinical Research Congress, 10-13 October, 2025.

"The data show pridopidine's ability to deliver consistent and sustained slowing of disease progression and significantly less decline across multiple endpoints of function, cognition and motor features, measured by scales including cUHDRS, TFC, SWR and Q-Motor, and compared to both placebo and two natural history cohorts, in HD

patients not taking antidopaminergic medicines (ADMs)," said Michal Geva, Prilenia's Head of Research. "This is further evidenced by the Phase 3 data **published in Nature Medicine**[ii] showing a significant reduction in decline from baseline of 0.41 cUHDRS points at week 52 for pridopidine treated patients compared to placebo (p = 0.035), with clinically meaningful differences extending to week 65 (0.27) and beyond."

"It is clear that the HD community needs access to new therapies that improve multiple independent clinical endpoints that matter most to people with HD and their families, including function, disease progression, cognition and motor function," said Oscar Pérez, Chief Scientific Officer at Ferrer. "We have a carefully designed roadmap aimed at paving the way to availability of pridopidine as an oral and easy to administer disease-modifying therapy capable of significantly slowing down clinical progression of HD."

Prilenia and Ferrer plan to initiate a global confirmatory study of pridopidine in early-stage HD patients not taking ADMs - expected to commence in the first half of 2026. In addition to the primary population, a small, proof-of-concept arm of patients taking low-dose ADMs (per label guidance) is also planned to be enrolled.

Pridopidine is an investigational, orally administered, small molecule, sigma-1 receptor (S1R) agonist that crosses the BBB and reaches its receptor with high affinity all over the brain. It has demonstrated a favorable safety and tolerability profile from placebo-controlled studies and clinical experience involving more than 1600 people and data extending up to seven years.

Five posters will be presented at the Huntington Study Group (HSG) HD Clinical Research Congress:

1. The Open-Label Period of the PROOF-HD Trial Shows Persistent Benefits of Pridopidine in Progression, Cognition, and Motor Function in Huntington's Disease, long term data showed a significant and clinically meaningful slowing of progression compared to propensity matched natural history cohorts from ENROLL-HD and TRACK-HD. Data at 2 years shows slowing of progression by 65% and 59% and (for TFC and cUHDRS respectively, both p = <0.0001), 88% for cognition (assessed by SWR, p = <0.0001) and 78% for motor features(assessed by Q-Motor, p = <0.0001) for pridopidine-treated patients off ADMs. Similar results were seen compared to Track-HD.
2. Antidopaminergic Medications (ADMs) Are Associated With Faster Decline In Clinical Measures Of Disease Progression in the Placebo Arm of PROOF-HD, highlighting the importance of careful consideration, including balanced randomization and statistical considerations, concerning ADM use in the assessment of results in all HD trials.
3. Low-dose antidopaminergic medications (ADMs) do not mask the beneficial effects of pridopidine in Huntington's disease (HD), demonstrating the potential for continued low-dose ADM use together with pridopidine without potential loss of efficacy. Participants on both pridopidine and low-dose ADMs,

maintained treatment benefits versus placebo through 1 year in cUHDRS (similar to the benefits seen in patients not taking ADMs), while higher ADM doses resulted in faster decline in clinical measures of disease progression.

4. Baseline levels of CAG-Age-Product 100 (CAP100) and Q-Motor Finger Tapping Inter-Onset-Interval (FT-IOI) predict long-term progression in PROOF-HD. Baseline CAP100 and FT-IOI predict progression and functional decline in HD. Incorporating these measures as covariates in future trials may improve interpretation of treatment results.
5. Covariate Adjustments of Key Factors Related to Disease Progression (CAP100 and Q-Motor) Enhance the Meaningful Benefits of Pridopidine on Function, Cognition, And Motor in the Phase 3 PROOF-HD Trial, showing that adjusting for even small imbalances at randomization can improve model precision, with adjustment showing improved consistency and magnitude of pridopidine treatment effect size across endpoints.

About pridopidine

Pridopidine (45 mg twice daily) is a potent and selective, orally administered sigma-1 receptor (S1R) agonist that stimulates key neuroprotective mechanisms impaired in neurodegenerative diseases, such as HD and Amyotrophic Lateral Sclerosis (ALS)[iii].

Pridopidine's extensive clinical development program involved approximately 1,600 participants, demonstrating a favorable safety and tolerability profile.

In addition to HD, pridopidine is in late-stage clinical development for ALS, with Prilenia and Ferrer planning to initiate a single, pivotal Phase 3 trial in ALS early in 2026, building on the findings in the population with early and rapid progressing disease (defined as definite or probable ALS by EEC and <18 months since symptom onset) from the Phase 2 HEALEY ALS Platform Trial[iv].

Pridopidine has Orphan Drug Designation in HD and ALS in the US and the EU, and FDA Fast Track Designation for the treatment of HD[v].

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 gene[vi], and each child of a parent with HD has a 50 percent chance of developing the disease.[vii]

Across the world an estimated 100,000 people have HD[viii,ix], with an additional 300,000 people at risk of developing HD[x,xi]. 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 nothing impacting measures of overall 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 in adults and children. 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] Data on file

[ii] Reilmann, R., Feigin, A., Rosser, A.E. et al. Pridopidine in early-stage manifest Huntington's disease: a phase 3 trial.

Nat Med (2025). **https://doi.org/10.1038/s41591-025-03920-3**

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[iv] 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

[v] Cudkowicz, M. AAN Annual Meeting, April 6-9, 2025, San Diego, CA

[vi] Eddings CR, Arbez N, Akimov S, Geva M, Hayden MR, Ross CA. Pridopidine protects neurons from mutant-huntingtin toxicity via the sigma-1 receptor. Neurobiol Dis. 2019 Sep;129:118-129. doi: 10.1016/j.nbd.2019.05.009. Epub 2019 May 17. PMID: 31108174; PMCID: PMC6996243.

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

[ix] 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.

[x] 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.

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