NEWS RELEASE

Prilenia to Present Pridopidine Data at the Huntington Study Group (HSG) 2024 Congress

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NAARDEN, Netherlands & WALTHAM, Mass.--(BUSINESS WIRE)-- **Prilenia Therapeutics B.V.**, a biopharmaceutical company focused on developing novel therapeutics to treat neurodegenerative and neurodevelopmental diseases, today announced the presentation of five scientific abstracts and an oral presentation focused on pridopidine, the company's investigational medicine for the treatment of Huntington's disease (HD), at the upcoming 31 st annual meeting of the Huntington Study Group to be held in Cincinnati, Ohio, November 7-9, 2024.

"To provide truly meaningful change for people living with HD, we need to shift the paradigm from treating symptoms to addressing progression. The data we will present at HSG reinforce pridopidine's benefits across function, cognition and fine motor skills measured by validated assessments and sustained for up to two years," said Dr. Michael R. Hayden, Chief Executive Officer of Prilenia. "The European Medicines Agency has accepted our MAA submission for the treatment of HD, and it is currently under review. This is the first submission seeking approval for a potentially first-in-class investigational new treatment that can impact these measures of clinical disease progression in HD."

Dr. Hayden will present clinical data on pridopidine during the Contemporary Trials and Next Steps (Clinical Trial Roundup) session (10:45–12:15 ET, Friday, November 8), additional to

presentation of the five abstracts. The data demonstrate pridopidine's effect and implications for HD management and clinical trial design:

- The Phase 3 PROOF-HD Trial Demonstrates Significant Benefits of Pridopidine on Progression, Cognition, and Motor Function in Huntington's Disease
    - Overview: Pridopidine shows consistent, sustained, and clinically meaningful benefits across multiple endpoints, including function, clinical disease progression (as measured by cUHDRS), cognition, motor and quality of life in patients not receiving antidopaminergics (ADMs) and on low doses of ADMs.
- Integrated Efficacy Analysis of Four Randomized Placebo-Controlled Trials Supports Pridopidine's Treatment Benefits Across Key Clinical Measures of Huntington's Disease
    - Overview: The integrated efficacy analysis from four clinical trials supports PROOF-HD findings, showing consistent, sustained and clinically meaningful benefits of pridopidine in HD in patients not receiving ADMs.
- Pridopidine Demonstrates Consistent Improvements in Q-Motor Measures, and Early Benefits in Q-Motor Predict Long-Term Changes in Function and cUHDRS in PROOF-HD
    - Overview: Pridopidine demonstrated improvements in Q-Motor assessments at all timepoints irrespective of ADM use, with strongest and most significant effects in patients not receiving ADMs. Early improvements in Q-Motor were predictive of long-term benefits on key clinical outcome measures of disease progression.
- Low Dose Antidopaminergic Medications do not Mask the Beneficial Effects of Pridopidine in Huntington's Disease
    - Overview: Participants on low dose ADMs maintain the positive and clinically meaningful benefits of pridopidine in HD. These observations may guide the use of ADMs for the treatment of chorea and behavioral disorders together with pridopidine.
- The Effect of Antidopaminergic Medications on Huntington's Disease
    - Overview: Analyses of data from the ENROLL-HD database demonstrate that use of ADM's was associated with worse outcomes in clinical outcome measures typically used in HD for function, cognition, cUHDRS and motor performance. These observations have important implications for the conduct and interpretation of investigational studies of disease-modifying agents in HD and for medical practice.

About Pridopidine

Pridopidine (45 mg twice daily) is a potent and highly selective, orally administered, sigma-1 receptor (S1R) agonist, that has the potential to alter treatment paradigms in neurodegenerative diseases. S1R regulates several cellular neuroprotective mechanisms commonly impaired in neurodegenerative diseases, such as HD and ALS.

Pridopidine has an extensive clinical development program encompassing efficacy and safety data. In the PROOF-HD Phase 3 clinical trial, pre-specified analyses that excluded patients receiving antidopaminergics (ADMs) showed benefits in people taking pridopidine across multiple measures, including clinical disease progression as measured by cUHDRS, cognition (Stroop Word Reading Test, SWR) and motor (Q-Motor). The primary and secondary endpoints in the full population were not met. Importantly, an integrated efficacy analysis from four Phase 3 and Phase 2 studies supports and validates the PROOF-HD findings, showing consistent, sustained and clinically meaningful benefits of pridopidine in HD in patients not receiving ADMs as measured by cUHDRS (excluding SWR as PRIDE-HD did not measure SWR), Q-Motor Finger Tapping (FT) and Pronation/Supination (PS).

In clinical studies to date, pridopidine (45 mg twice daily) has been well-tolerated with a safety and tolerability profile similar to placebo.

The European Medicines Agency has accepted our MAA submission for the treatment of HD, and it is currently under review. In parallel, Prilenia is also in discussions with the U.S. Food and Drug Administration (FDA) to determine next steps for HD in the United States. The Company will also consider regulatory submissions elsewhere globally following the regulatory review process in Europe.

Prilenia holds Orphan Drug designation for pridopidine in HD and ALS in the U.S. and EU. In addition, pridopidine has received Fast Track designation by the U.S. Food and Drug Administration (FDA) for the treatment of HD.

About HD

Huntington’s disease (HD) is a rare, inherited, autosomal dominant, neurodegenerative disease that results in functional, motor, cognitive and behavioral symptoms. HD is caused by a mutation in the huntingtin gene, and each child of a parent with HD has a 50 percent chance of developing the disease.

HD affects about 100,000 people around the world with an additional 300,000 people at risk

of developing HD. 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 disease progression.

About Prilenia

Prilenia Therapeutics is a biopharmaceutical company focused on developing novel therapeutics to treat neurodegenerative and neurodevelopmental diseases like Huntington's disease (HD) and amyotrophic lateral sclerosis (ALS). Our team has an unwavering dedication to scientific excellence, and we are driven by our passion and commitment to patients and their families, caregivers, and communities around the world affected by these diseases.

Based on the collective data from pridopidine's extensive development program, Prilenia is pursuing regulatory approval for its investigational medicine, pridopidine, for the treatment of HD and planning to initiate a single pivotal Phase 3 study in ALS. Pridopidine is a potent and selective, orally administered S1R agonist.

Prilenia is a private, Netherlands-headquartered company backed by leading life sciences investors.

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

For a copy of this release, visit Prilenia's website at **www.prilenia.com**.

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