Prilenia and Huntington Study Group Announce Completion of Patient Enrollment Ahead of Schedule in Global Phase 3 PROOF-HD Huntington's Disease Clinical Trial

-- Pridopidine is the most advanced drug candidate in clinical development for maintenance of functional capacity in Huntington's Disease --

NAARDEN, NL, 20 October 2021 --- Prilenia Therapeutics B.V., a clinical stage biotech company focused on developing novel treatments for neurodegenerative and neurodevelopmental disorders, today announced the completion of patient enrollment in the Pridopidine Outcome On Function in Huntington's Disease (PROOF-HD) global Phase 3 clinical trial.

PROOF-HD is a randomized, double-blind, placebo-controlled study evaluating the safety and efficacy of pridopidine in individuals with early stage Huntington's Disease (HD). The trial is being conducted in the U.S., Canada and nine countries in Europe. This study is being undertaken in collaboration with the Huntington Study Group (HSG), a world leader in clinical research for HD.

The aim of the study is to evaluate pridopidine's impact on Total Functional Capacity (TFC) score as the primary endpoint. TFC is a validated, clinically meaningful and regulatory accepted endpoint that measures the patient's ability to maintain function in different domains. Pridopidine acts as a highly selective and potent Sigma-1 receptor (S1R) agonist and has been shown to maintain functional capacity in early HD patients in a prior trial. Currently, pridopidine is the only Phase 3 clinical stage drug candidate assessing progression as measured by TFC in HD.

"We are proud to reach this significant milestone for pridopidine as we work diligently to provide a much-needed treatment option for the patients and families faced with this challenging disease," said Dr. Michael R. Hayden, CEO and Founder of Prilenia. "Thanks to the support of HSG, the Clinical Trials Coordination Center (CTCC) and TFS, we have been able to reach this milestone well ahead of schedule and with numbers over our enrollment target of 480 participants, despite the global pandemic. We are also deeply grateful to the clinical site teams and the HD community globally for supporting this trial. Our lead asset, pridopidine, has previously demonstrated a favorable long-term safety and tolerability profile and we are currently on track to obtain PROOF-HD's top-line results in Q1 2023."

"Completing the enrollment of the PROOF-HD clinical trial brings us closer to finding more effective treatments for Huntington's Disease and reducing the burden for families affected by the disease," said Dr. Andrew Feigin, MD, Chair at The Huntington Study Group. "If successful, data from the Phase 3 PROOF-HD trial has the potential to make a large positive impact on the Huntington's Disease community, and we look forward to continuing this clinical trial in partnership with Prilenia."

About Pridopidine

Prilenia's lead asset is pridopidine, a first-in-class drug candidate with an established safety profile and therapeutic potential in several neurodegenerative diseases affecting adults and children. Pridopidine is being developed for the treatment of Huntington's Disease (HD) in a global Phase 3 clinical study and as a potential treatment for Amyotrophic Lateral Sclerosis (ALS) in a Phase 2/3 clinical study. Additional neurodegenerative and neurodevelopmental indications will be evaluated in future pridopidine clinical studies. The highly selective S1R agonist was acquired from Teva in 2018.

About Prilenia

Prilenia is a clinical stage biotech startup founded in 2018 with the purpose of improving the lives of patients and their families by developing treatments for neurodegenerative and neurodevelopmental disorders. Prilenia is backed by a group of well-respected investors: Forbion, Morningside, Sectoral, Talisman and the ALS Investment Fund. The Company is based in Naarden, the Netherlands, Herzliya, Israel and Boston, MA in the U.S. For more information visit www.prilenia.com and follow us on Twitter @prileniaTx.

Pridopidine for Huntington's Disease

Huntington's Disease (HD) is a fatal, inherited, neurodegenerative disorder. Every offspring of an HD patient has a 50% chance of inheriting the gene. Usually starting at around 40 years of age, HD patients suffer from a movement disorder, progressive functional and cognitive decline, psychiatric disturbances and behavioral symptoms. Following diagnosis, functional, motor and cognitive functions decline steadily, ultimately leading to immobility, dementia and premature death.

Pridopidine has demonstrated maintenance of functional capacity in HD patients, as measured by Total Functional Capacity (TFC), in a post hoc analysis of a phase 2 clinical trial. This effect was most prominent in early-stage HD patients (HD1 and HD2), who showed functional benefit from pridopidine 45 mg, taken twice a day.

There is extensive in-vitro and in-vivo preclinical evidence that further supports pridopidine's potential beneficial effect in HD. The therapeutic effect has been shown to be mediated exquisitely by the Sigma-1 Receptor (S1R) using systems that either delete or antagonize S1R activity.

Prilenia has been granted orphan drug designation for pridopidine for the treatment of HD in both the U.S. and Europe.

About Huntington Study Group Clinical Research

Founded in 1993 in Rochester, NY, the Huntington Study Group (HSG) is a not-for-profit organization comprised of the world's first collaborative network of experts in Huntington's Disease. HSG Clinical Research, Inc. is a wholly-owned for-profit subsidiary of the HSG, conducting clinical trials to benefit the HSG and its mission of seeking treatments that make a difference for those affected by HD. There are 800 credentialed HD experts at more than 130 HSG credentialed research sites worldwide. The HSG also

offers educational services like CME4HD™ for healthcare professionals and care providers on treating patients with HD. For more information, visit www.huntingtonstudygroup.org.

About TFS International

TFS was founded in 1996 and has grown to become the leading global mid-size clinical CRO focusing on small and mid-size Biotech customers. TFS employs nearly 700 professionals throughout 21 countries and currently delivers clinical research services in more than 40 countries. TFS provides end-to-end solutions including full clinical development services, strategic resourcing, and flexible single services. TFS partnering approach with customers is based on our four business principles – commitment, flexibility, value creation and global reach. Our core therapeutic specialties are Dermatology, Hematology and Oncology, Ophthalmology as well as Internal Medicine and Neurology. Detailed information about TFS, and its business offerings can be obtained through www.tfscro.com.

About the CTCC

The Clinical Trials Coordination Center (CTCC) is part of the University of Rochester Medical Center's Center for Health + Technology (CHeT). The CTCC specializes in the development, management, and conduct of clinical research studies. Over the past 25 years, the CTCC has managed the conduct of over 100 clinical research studies with 45 sponsors (government, industry, and private) that enrolled over 38,000 research participants in US, Canada, Europe, New Zealand, and Australia. Visit the CTCC website https://www.urmc.rochester.edu/health-technology/our-expertise/clinical-trials-coordination.aspx.

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