



NEWS RELEASE

Prilenia Announces First Patient Enrolled for Pridopidine Phase 2/3 Platform Trial for ALS

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- Pridopidine was chosen in global competition as one of the top drug candidates to participate in the first ever ALS Platform Trial led by the Healey Center at Massachusetts General Hospital
- This trial is the second late-stage clinical trial in neurodegenerative disorders with pridopidine, in addition to the global Phase 3 trial in Huntington's disease
- Positive data from this clinical study could lead to registration of pridopidine for the treatment of ALS

NAARDEN, The Netherlands--(**BUSINESS WIRE**)--Prilenia Therapeutics B.V., a clinical stage biotech company focused on developing novel treatments for neurodegenerative and neurodevelopmental disorders, today announces the enrollment of the first participant for treatment with pridopidine in the pivotal HEALEY ALS Platform Trial.

The HEALEY ALS Platform trial ("HEALEY trial"; **NCT04297683**) is a multi-center, multi-regimen clinical trial evaluating the safety and efficacy of investigational products for the treatment of amyotrophic lateral sclerosis (ALS). It is the first platform trial for ALS, designed to accelerate development of promising new treatments by enabling investigators to simultaneously assess multiple therapeutic candidates.

The HEALEY ALS Platform Trial - Regimen D Pridopidine (**NCT04615923**) evaluates the safety and efficacy of pridopidine as a single agent for ALS patients. Pridopidine is a highly potent and selective Sigma-1 receptor (S1R) agonist, which was selected as a therapeutic candidate out of approximately 30 competing investigational treatments. It was chosen by an independent expert review committee based on compelling human genetic data, efficacy in preclinical models, favorable safety profile and readiness of drug supply. The trial treatment dose (45 mg

bid) has an extensive safety record (based on >1,300 patient years), confirming placebo-like safety and tolerability profile.

Michael R. Hayden, MD, PhD, CEO of Prilenia, said: “Enrolling the first patient in the HEALEY ALS platform trial for treatment with pridopidine is great news for both the ALS patient community and Prilenia. Previous studies with pridopidine demonstrate it is a highly selective S1R agonist with an extensive safety record and a great therapeutic potential in multiple neurodegenerative disorders including HD and ALS. We are excited to be part of this platform trial which is designed to hasten development of new treatments to patients living with ALS.”

The HEALEY trial is being led by ALS specialists at the Sean M. Healey & AMG Center for ALS at Mass General Hospital. The study is recruiting adults with sporadic or familial ALS at 54 sites, all of which are part of the Northeast ALS (NEALS) Consortium, across the United States.

Merit Cudkowicz, MD, MSc, Director of the Sean M. Healey & AMG Center for ALS at Mass General and Principal Investigator for the trial commented: “ALS is a serious disease, with no treatments that halt or reverse disease progression currently available. Pridopidine is one of the top drugs selected for our ALS platform trial due to the strength of its preclinical efficacy data and safety profile, which demonstrate its potential as a breakthrough treatment. We look forward to continuing to work with Prilenia and the clinical team as we advance the pridopidine arm of our study.”

The HEALEY Trial - Regimen D is the second trial that Prilenia has initiated this year, following enrollment of the first patients in the **Phase 3 PROOF-HD clinical trial** for pridopidine in Huntington’s Disease (HD) in October 2020.

More information on the HEALEY Trial - Regimen D Pridopidine can be found here:

<https://clinicaltrials.gov/ct2/show/NCT04615923>

Notes to Editors

About Prilenia (**www.prilenia.com**)

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 has raised \$ 88.5 million thus far and is backed by a group of well-respected investors: Talisman, Forbion, Morningside, Sectoral and ALS Investment Fund. The Company is based in Naarden, the Netherlands, Herzliya, Israel and Boston, MA in the U.S.

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. The highly selective S1R agonist was acquired from Teva in 2018.

Pridopidine's favorable safety profile has been established in clinical trials in >1300 study participants, exposed to various doses for a total of ~1300 patient years.

Pridopidine for ALS

Compelling preclinical data supports the potential use of pridopidine as a therapeutic for ALS. In ALS SOD1G93A motor neurons (MNs), pridopidine exerts neuroprotective effects via activation of the S1R. Specifically, pridopidine improves BDNF (brain-derived neurotrophic factor) and GDNF (glial cell line-derived neurotrophic factor) axonal transport, restores synaptic activity and neuro-muscular junction (NMJ) function, and increases neuronal survival. In vivo, pridopidine treatment of SOD1G93A mice reduces toxic protein aggregates and ameliorates muscle fiber wasting.

Previous clinical data also suggest that S1R is a promising target for ALS therapy, indicating that S1R activation may enhance bulbar and speech function in ALS patients. The sigma 1 receptor has been genetically validated for ALS, as patients with mutations in this gene develop ALS.

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