



NEWS RELEASE

Prilenia: FDA Grants Orphan Drug Designation for pridopidine for the Treatment of Amyotrophic Lateral Sclerosis (ALS)

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NAARDEN, the Netherlands--(**BUSINESS WIRE**)--FDA Grants Orphan Drug Designation for pridopidine for the Treatment of Amyotrophic Lateral Sclerosis (ALS) **Prilenia Therapeutics B.V.**, a clinical stage biotech company focused on developing novel treatments for neurodegenerative and neurodevelopmental disorders, is pleased to announce that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation for pridopidine for the treatment of **ALS**.

"We are pleased to have been granted Orphan Drug Designation by the FDA for pridopidine for the treatment of ALS"

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Prilenia recently received a **positive opinion from the Committee of Orphan Medicinal Products (COMP)** in the EU for Orphan Drug Designation for treatment of ALS and is expecting to receive the European Commission decision soon.

Pridopidine acts as a highly selective and potent Sigma-1 receptor (S1R) agonist. Extensive safety data demonstrate pridopidine has a favorable safety and tolerability profile. Pridopidine is currently being assessed in the HEALEY ALS Platform Trial in the U.S., the first for ALS. The pridopidine regimen **enrolled its first participant** in January 2021 and is on track to generate results in H2 2022. Pridopidine is also being studied for the treatment of **Huntington's**

Disease (HD) in a global Phase 3 clinical trial that just met the **50% enrollment milestone** ahead of schedule.

“Pridopidine is an exciting compound that offers potential disease-modifying treatment for patients with ALS”, said Dr. Jeremy Shefner, MD, PhD, Kemper and Ethel Marley Professor and Chair of Neurology of Barrow Neurological Institute and Regimen Lead Investigator for the Pridopidine Regimen. “Orphan Drug Designation will speed development and simplify the approval pathway should efficacy be demonstrated in ongoing trials, which will be good news for patients with ALS.”

“We are pleased to have been granted Orphan Drug Designation by the FDA for pridopidine for the treatment of ALS”, said Dr. Michael R. Hayden, CEO and Founder of Prilenia. “This marks significant progress towards our goal of advancing pridopidine to address the unmet medical need for ALS.”

The Orphan Drug Designation qualifies Prilenia for various development and commercial incentives in the U.S., including a waiver of the new drug application fee and seven years of market exclusivity following approval.

Notes to Editors

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.

About Pridopidine

Prilenia's lead asset is pridopidine, a first-in-class, highly selective S1R agonist investigational drug.

Pridopidine has an established safety profile and therapeutic potential in several neurodegenerative diseases affecting adults and children. Prilenia acquired pridopidine from Teva in 2018.

Pridopidine is currently in late-stage clinical development for ALS and HD.

Background on ALS

Amyotrophic lateral sclerosis, ALS (also known as motor neuron disease), is the most prevalent adult-onset progressive motor neuron disease, affecting approximately 20,000 people in the U.S. and an estimated 50,000 people worldwide. ALS causes the degeneration of motor neurons, resulting in progressive muscle weakness and atrophy and eventually death. There are currently three FDA therapies approved specifically for treating ALS—riluzole, Nuedexta and edaravone.

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 reduces toxic protein aggregates and ameliorates muscle fiber wasting. Previous clinical data also suggests 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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