



NEWS RELEASE

Prilenia Closes Oversubscribed \$43 Million in Series B Financing Round

11/3/2021

-- Financing led by Sands Capital alongside Forbion and Morningside, with participation from additional new and all existing investors

--Proceeds to support advancing the Company's Huntington's Disease (HD) and Amyotrophic Lateral Sclerosis (ALS) programs to regulatory submissions and preparing for commercialization, as well as planning for additional indications

NAARDEN, Netherlands--**(BUSINESS WIRE)**--**Prilenia Therapeutics B.V.**, a clinical stage biotech company focused on developing novel treatments for neurodegenerative and neurodevelopmental disorders, today announced that it has raised \$43M in an oversubscribed Series B financing round. The proceeds will be used to prepare for potential registration and commercialization of its lead drug candidate, pridopidine, for patients with **Huntington's Disease (HD)** and **Amyotrophic Lateral Sclerosis (ALS)**, as well as to accelerate timelines and expand the Company's executive team and operations.

The funding round was upsized and led by new investor Sands Capital, alongside Forbion and Morningside. Amplitude Ventures also joined the round as a new investor, in addition to existing investors Sectoral Asset Management, Talisman and the ALS Investment Fund. The Series B financing brings the total capital invested in Prilenia Therapeutics since its founding in September 2018 to \$133.5M.

"With the support of this group of leading investors, we are well-capitalized and resourced to continue advancing

our programs towards potential registration and commercialization,” said Dr. Michael R. Hayden, CEO and Founder of Prilenia. “The recent completion of patient enrollment in our PROOF-HD Phase 3 clinical trial ahead of schedule and above target enrollment numbers is a significant milestone. We look forward to advancing our programs in both HD and ALS.”

“Prilenia has made tremendous progress since its founding through outstanding execution on all fronts,” said Geert-Jan Mulder, MD, Prilenia Chairman and Managing Partner at Forbion. “We are excited to continue to support the Company on its journey to develop pridopidine as a potential treatment for HD and ALS patients.”

“We are honored and pleased to join this group of outstanding investors in supporting Prilenia on its next stage of progress and growth,” said Michael Ginder, Research Analyst at Sands Capital, who will be joining Prilenia’s Board of Directors.

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. Currently, pridopidine is the only Phase 3 clinical stage drug candidate assessing HD disease progression as measured by TFC and is also being assessed for treatment of ALS in the **HEALEY ALS Phase 2/3 platform trial**.

About Pridopidine

Prilenia’s lead asset, **Pridopidine** is a highly selective and potent Sigma-1 Receptor (S1R) agonist with an established safety and tolerability profile. Pridopidine is orally administered with therapeutic potential in HD, ALS and other neurodegenerative diseases and neurodevelopmental disorders such as Rett and Fragile-X syndromes.

Pridopidine is currently in late-stage clinical development for HD and ALS. Both trials, the global phase 3 clinical trial in HD (PROOF-HD) and the Healey platform trial in ALS are currently active.

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 including: Forbion, Morningside, Sands Capital, Sectoral Asset Management, Talisman, Amplitude Ventures 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](https://twitter.com/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 declines, 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.

Pridopidine demonstrates neuroprotective properties mediated by the S1R in several in vivo and in vitro HD models. In all these models, when the S1R is deleted or an S1R antagonist is used, these neuroprotective properties are eliminated completely. These are comprised of a robust, neuroprotective effect against mutant huntingtin-(mHTT)-induced cell death in human HD induced pluripotent stem cells (iPSCs), and mouse HD cortical neurons. In HD cortico-striatal cultures, pridopidine increases spine density and rescues the aberrant calcium signaling, enhances mitochondrial function and mitigates mHTT-induced ER and oxidative stress, all known features of HD.

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

Pridopidine for 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. ALS causes the degeneration of motor neurons, resulting in progressive muscle weakness and atrophy and eventually death. There are currently two FDA therapies approved specifically for treating ALS—riluzole and edaravone.

Compelling preclinical data supports the potential use of pridopidine as a therapeutic for ALS. Pridopidine exerts its 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, in ALS models 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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