



Prilenia Therapeutics Raises \$62.5m to Fund Late Stage Trials in HD and ALS

- *Forbion leads Series A financing round, accompanied by Morningside, Sectoral and Talisman*
- *Funding to support two late-stage trials of pridopidine in Huntington Disease (HD) and Amyotrophic Lateral Sclerosis (ALS)*
- *Former Chairman, Michael R. Hayden, MD PhD, appointed CEO of Prilenia*

[Naarden, NL and Herzliya, IL, 03 June 2020] - Prilenia Therapeutics B.V., a clinical stage biotech company focused on developing novel treatments for neurodegenerative and neurodevelopmental disorders, announces that it has raised \$62.5M in a Series A financing round to launch the Company's planned HD and ALS late-stage clinical trials.

The funding round was led by Forbion and included new investors, Morningside Venture Investments and Sectoral Asset Management. Existing investors Talisman Capital Partners and Genworks 2 also participated in the round. The Series A financing brings the total capital invested in Prilenia Therapeutics since its foundation in September 2018 to \$84.5M.

The proceeds will fund two late-stage trials, which could lead to the registration of pridopidine for the treatment of HD and ALS. Pridopidine is a highly selective sigma-1 receptor (σ_1R) agonist. It is shown to maintain functional capacity in early HD patients, as measured by the Total Functional Capacity (TFC) score. Furthermore, it was recently selected from an international competition of over 30 potential therapeutics for inclusion in the first ever ALS platform trial, led by the Healey Center for ALS at Massachusetts General Hospital. The trials – Phase 3 trial in HD and the platform trial in ALS - are expected to commence in H2 2020.

Alongside the closing of the financing round, Michael R. Hayden, MD, PhD, has been appointed as CEO of Prilenia. He has been serving as Executive Chairman of Prilenia since the Company was founded. Michael is a world-renowned scientist in Huntington's Disease research. He is the former President of Global R&D and Chief Scientific Officer at Teva Pharmaceuticals, where he led the development of 35 new products towards approval in several major markets, predominantly in CNS. Michael co-founded five biotechnology companies of which two realized successful exits and three became public, most recently 89bio (ETNB).

Commenting on the Series A financing round and the upcoming trials, Michael R. Hayden said: *"Having such renowned investors Forbion, Morningside and Sectoral join us on this important journey is important validation of the team and our development program. With this funding, we will be able to complete two critical clinical trials in disease areas with significant unmet need. We believe we have the*

opportunity to bring real hope to HD and ALS patients and their families and we are keen to accelerate our progress.”

Geert Jan Mulder, MD, Managing Partner and co-founder at Forbion, added: *“We are convinced that pridopidine has the potential to seriously impact these intractable diseases. The more we have scrutinized the program, the more excited we have become. We look forward to continuing to work with Prilenia’s outstanding management team, as well as the new and existing investors to help drive it to success.”*

As part of this Series A round, a new Dutch Company, Prilenia Therapeutics BV has been formed. The new company will hold Israeli subsidiary company Prilenia Neurotherapeutics Ltd. Geert-Jan Mulder will join the Board of Directors for the new Company, together with Jason Dinges from Morningside and Stefan Larson from Sectoral.

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 is based in Naarden, the Netherlands and Herzliya, Israel.

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 for Huntington 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 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 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 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 multiple deletion and antagonist models.

Prilenia has an orphan drug designation for pridopidine for the treatment of HD in both the US and Europe.

Pridopidine for ALS

ALS is the most prevalent adult-onset progressive motor neuron disease, affecting approximately 30,000 people in the U.S. and an estimated 500,000 people worldwide. ALS causes the progressive degeneration of motor neurons, resulting in progressive muscle weakness and atrophy. There are currently three FDA therapies approved specifically for treating ALS: riluzole, nuedexta and edaravone.

Compelling preclinical data supports the potential use of pridopidine as a therapeutic for ALS. In ALS SOD1^{G93A} 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 SOD1^{G93A} 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.

In addition to the HD and ALS programs, Pridopidine is also being studied for the treatment of Parkinson's Disease Levodopa Induced Dyskinesia (PD-LID) and other conditions.

Contacts:

Prilenia contact:

Michael Hayden, CEO
Limor Ben Har, COO
E: info@prilenia.com

Media contact:

Melanie Toyne-Sewell / Agnes Stephens
T: +44 (0) 7890 022814 / +44 (0) 79644 79544
E: prilenia@instinctif.com