



NEWS RELEASE

Prilenia receives positive EMA opinion on Orphan Drug Designation for pridopidine in ALS

6/29/2021

NAARDEN, Netherlands--(BUSINESS WIRE)--**Prilenia Therapeutics B.V.**, a clinical stage biotech company focused on developing novel treatments for neurodegenerative and neurodevelopmental disorders, received a positive opinion on European Orphan Drug Designation for pridopidine in **Amyotrophic lateral sclerosis (ALS)**. The orphan designation is expected to be granted within 30 days, following the positive opinion issued by the EMA Committee for Orphan Medicinal Products (COMP).

"This positive opinion from the EMA further validates the potential for pridopidine to impact the devastating course seen in patients with ALS. We are encouraged by pridopidine's diverse effects favorably impacting neurodegeneration in ALS models and look forward to the trial readout later next year"

Tweet this

ALS (also known as motor neuron disease and Lou Gehrig disease) is a life-threatening and chronically debilitating disease due to progressive degeneration of motor neurons, ultimately leading to paralysis and respiratory failure.

Pridopidine is a potent and selective Sigma-1 receptor (S1R) agonist, currently being assessed in the HEALEY ALS Platform trial in the US; a multi-center, multi-regimen clinical study evaluating the safety and efficacy of investigational products for the treatment of ALS. The ongoing trial enrolled its first participant for the pridopidine regimen in January 2021 and is on track to generate results in Q3 2022.

The S1R is a promising therapeutic target for the treatment of ALS, based on human genetic data as well as

compelling preclinical evidence. Pridopidine demonstrates beneficial effects in key processes relevant to ALS and shows neuroprotective properties in numerous ALS preclinical models, via activation of the S1R.

The COMP granted Orphan Drug Designation for pridopidine in ALS based on supportive preclinical data indicating potential preservation of motor function, which is considered by the committee as an unmet need and a clinically relevant advantage over available treatment.

Under orphan designation in the European Union (EU), Prilenia would benefit from this designation with protocol assistance, reduced regulatory fees and market exclusivity. Orphan Drug Designation in Europe is granted for a serious or life-threatening disease affecting not more than five in 10,000 people.

Dr. Michael R. Hayden, CEO and Founder of Prilenia, commented: “This positive opinion from the EMA further validates the potential for pridopidine to impact the devastating course seen in patients with ALS. We are encouraged by pridopidine’s diverse effects favorably impacting neurodegeneration in ALS models and look forward to the trial readout later next year”.

Prilenia holds Orphan Drug Designation for pridopidine in **Huntington’s Disease (HD)** in both the US and EU. In addition to the ALS trial, Pridopidine is being studied in a global phase 3 trial for HD (**PROOF-HD**).

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 raised \$ 90.5 million thus far and is backed by a group of well-respected investors: Talisman, Forbion, Morningside and Sectoral and 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. Pridopidine was acquired from Teva in 2018.

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

About Pridopidine for ALS

Amyotrophic lateral sclerosis, ALS (also known as motor neuron disease) and Lou Gehrig disease, is the most prevalent adult-onset progressive motor neuron disease, affecting approximately 350,000 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.

The Sigma-1 Receptor is a promising therapeutic target for the treatment of ALS based on human genetic data and compelling preclinical evidence.

Mutations in the S1R gene are causative of ALS where complete loss-of-function (LOF) mutations cause a severe juvenile form of ALS while partial LOF mutations cause late onset ALS, indicating a correlation between S1R function and the severity of ALS. 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. Preclinical data highlight the role of S1R in ALS. Mice lacking the S1R show degeneration of motor neurons and motor impairment. Furthermore, in ALS mice that also lack S1R expression, disease progression is accelerated.

CONTACTS

For further information, please contact:

Contacts:

Prilenia Therapeutics

Limor Ben Har, COO

E: info@prilenia.com

Instinctif Partners

Melanie Toyne-Sewell / Agnes Stephens

T: +44 (0) 207 457 2020

E: prilenia@instinctif.com