Sean M. Healey & AMG Center for ALS Awarded NIH U01 Grant to Support Expanded Access to Pridopidine in Collaboration with Prilenia Therapeutics

The Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital has been awarded a grant from the National Institutes of Health (NIH) - Neurological Disorders and Stroke (NINDS) to conduct an intermediate size Expanded Access Protocol (EAP) in Amyotrophic Lateral Sclerosis (ALS) in collaboration with Prilenia Therapeutics.

The grant is supported by the ACT for ALS (Accelerating Access to Critical Therapies for ALS Act). This EAP will evaluate the benefits of the investigational drug pridopidine in individuals with ALS and will be led by Healey & AMG Center faculty, Drs. Suma Babu, James Berry, and Sabrina Paganoni. Prilenia will be the Regulatory sponsor of the EAP.

Expanded Access, also referred to as Compassionate Use, is an FDA-regulated pathway that allows individuals with a serious and life-threatening disease to access an investigational drug that is not yet approved by the FDA.

Pridopidine is a highly selective sigma-1 receptor (S1R) agonist in development by Prilenia. The S1R is highly expressed in the brainstem and spinal cord, areas implicated in ALS and important for bulbar, speech, and limb function. The S1R positively regulates key cellular pathways that are commonly impaired in ALS and other neurodegenerative diseases. In ALS cell culture and mouse models, pridopidine has been shown to be neuroprotective, specifically mediated via the activation of S1R.

The safety and efficacy of pridopidine were tested in the HEALEY ALS Platform Trial where positive effects of pridopidine on overall function (ALSFRS-R), speech, bulbar function, and breathing in people who are fast progressors and earlier in disease course were seen. Prilenia is actively planning a Phase 3 clinical trial evaluating pridopidine in people with ALS.

The EAP made possible by this award will allow ALS individuals who are otherwise not eligible for participation in clinical trials of pridopidine to access this investigational drug. Additionally, this EAP will provide real world safety, biomarker, and clinical data in two hundred (200) ALS individuals treated with pridopidine for up to two (2) years, thus contributing to our understanding of the effects of pridopidine in a broad population. The pridopidine EAP will be conducted at up to 45 enrolling centers across the U.S.

The researchers at the Healey & AMG Center for ALS and Prilenia are hopeful that this study will continue to pave the way for patient-centric and scientifically relevant EAPs to be conducted across the US. Such EAPs run in parallel to late phase randomized controlled clinical trials and

contribute research data to supplement clinical development programs of investigational products in ALS.

"We are grateful to the NIH for this award, and the opportunity to launch the pridopidine EAP" said the study PIs, Drs. Babu, Berry and Paganoni. "Programs like this help advance research and innovation in ALS."

"Prilenia is proud to work together with The Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital to make this important EAP possible for the many people living with more advanced stages of ALS. We share the great appreciation for the NIH and NINDS for supporting this work through the Act for ALS," said Dr. Michael R. Hayden, CEO and Founder of Prilenia. "We also want to express our gratitude to the participants and their families as well as the site healthcare providers and staff who will make this EAP possible, further advancing our understanding of pridopidine as a potential therapy for ALS."

About Pridopidine

Pridopidine (45 mg twice daily) is an oral, highly selective, and potent investigational S1R agonist that has exhibited a safety and tolerability profile similar to placebo in clinical studies to date. The S1R protein is highly expressed in the brain and spinal cord, where it regulates several key processes that are commonly impaired in various neurodegenerative diseases. Activation of the S1R by pridopidine stimulates multiple cellular protective pathways, including autophagy, axonal transport, mitochondrial energy production and calcium homeostasis, which are essential to neuronal function and survival, and may lead to neuroprotective effects.

Prilenia holds Orphan Drug designation for pridopidine in ALS and Huntington's Disease (HD) in the U.S. and EU. In addition, pridopidine has received Fast Track designation by the U.S. Food and Drug Administration (FDA) for the treatment of HD.

Background on ALS

Amyotrophic lateral sclerosis, 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 few FDA therapies approved for treating ALS—riluzole, edaravone (IV and oral formulation), sodium phenylbutyrate/taurursodiol, and tofersen. Dextromethorphan/quinidine is also used for the symptomatic treatment of pseudobulbar affect (PBA) in people with ALS.

About the Sean M. Healey & AMG Center for ALS at Mass General

At the Sean M. Healey & AMG Center for ALS at Mass General, we are on a quest to discover life-saving therapies for all individuals affected by ALS. Launched in November 2018, the Healey Center leverages a global network of scientists, physicians, nurses, caregivers, people with ALS and families working together to accelerate the pace of ALS therapy discovery and development.

Under the leadership of Merit Cudkowicz, MD and a Science Advisory Council of international experts, we are reimagining how to develop and test the most effective therapies to treat the disease, identify cures and, ultimately, prevent it.

The key to our success is our tightly integrated research and clinical efforts, encouraging opportunities to bring the challenges our patients face every day into our laboratories, focusing investigations on finding solutions that will make a meaningful difference to our patients without delay. Our collaborative efforts are designing more efficient and effective clinical trials while broadening access to these trials for people with ALS.

About Prilenia

Prilenia is a clinical stage biotechnology company founded in 2018 focused on the urgent mission to develop novel therapeutics to slow the progression of neurodegenerative diseases and neurodevelopmental disorders. The initial focus of the company has been on HD and ALS.

Prilenia is backed by a group of well-respected investors including: Forbion, Morningside, Sands Capital, SV Health Investors, Sectoral Asset Management, Talisman, Amplitude Ventures and the ALS Investment Fund. The Company is based in Naarden, the Netherlands, Herzliya, Israel and Waltham, Massachusetts in the U.S.

For more information visit www.prilenia.com and follow us on LinkedIn and Twitter.

Prilenia
Kristina Coppola
Head of Corporate Communications
info@prilenia.com