Prevail THERAPEUTICS

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Prevail partners with the International Gaucher Alliance to sponsor the GARDIAN Registry for Gaucher disease

GARDIAN

THE NEURONOPATHIC GAUCHER REGISTRY

OWNED AND GOVERNED BY THE GLOBAL PATIENT COMMUNITY

At Prevail Therapeutics, the patient communities that we aim to help stay consistently at the top of our minds. We are committed to ensuring that people living with neurodegenerative disease and rare conditions remain at the heart of our work, and we are always looking for opportunities to get involved. As a result, we are proud to share our sponsorship of the GARDIAN Registry, a global registry launched by the International Gaucher Alliance (IGA) for people and families affected by Gaucher disease Type 2 (GD2) and Type 3 (GD3), which together are known as neuronopathic Gaucher disease (nGD).

GARDIAN stands for the **Ga**ucher **R**egistry for **D**evelopment, **I**nnovation and **A**nalysis of **N**euronopathic disease. Its goal is to increase understanding of GD2 and GD3 by asking patients and their families to share their personal experiences in a database created for physicians and researchers working to find new treatment options.

The registry invites participants to submit their experiences with GD2 or GD3, including severity of symptoms, fatigue levels, treatment options recommended to them and more, to study the natural history of both diseases. It can also identify patient populations that may be eligible for clinical trials. GARDIAN is available in 8 languages: UK English, US English, Spanish, Arabic, Japanese, Chinese, German and French.

People living with Gaucher disease, and their families, know the daily implications of these diagnoses and have the power to impact how we in industry view and treat this disease. Through the GARDIAN Registry, we can gather data based on real experiences to build a better picture of Gaucher disease to help advance disease management, create improved treatments and ultimately impact lives.

We are eager to understand the day-to-day experiences of people living with Gaucher disease and are committed to expanding treatment options for people living with neurodegenerative and rare disease. The PROCEED trial, which will assess our PR001 experimental gene therapy for Gaucher disease Type 1 (GD1), has been activated at the first clinical trial site location at the Lysosomal Rare Disorders Research and Treatment Center in Fairfax, Virginia. The trial is

expected to expand in the U.S. and globally. Our PROVIDE trial, which also studies PR001 for the treatment of children and infants diagnosed with GD2, is currently recruiting as well.

At Prevail, we recognize the critical need to include patient and caregiver voices throughout the drug development process, and because of this we seek to understand the unique needs of each disease community we serve to create more effective treatments that may lead to a better quality of life. By collaborating with the IGA to help support GARDIAN, we hope to develop a more authentic understanding of the patient experience that will improve current drug development efforts.

To learn more about the registry, visit gardianregistry.org or contact info@gardianregistry.org. Find out more about our commitment to Gaucher disease on our patients and families page.