Prevail Therapeutics Provides PR001 Program Update

January 9, 2020

PR001 Administered to International Type 2 Gaucher Disease Patient Via Compassionate Use Pathway

Company Advancing its Phase 1/2 Clinical Trial for the Treatment of Neuronopathic Gaucher Disease and Expects to Initiate Patient Dosing in First Half of 2020

NEW YORK, Jan. 09, 2020 (GLOBE NEWSWIRE) -- Prevail Therapeutics Inc. (Nasdaq: PRVL) ("Prevail" or the "Company"), a biotechnology company developing potentially disease-modifying AAV-based gene therapies for patients with neurodegenerative diseases, today provided an update on its investigational program, PR001, an AAV9-based gene therapy delivering the *GBA1* gene, for the neuronopathic Gaucher disease (nGD) indication.

Prevail has granted a compassionate use request for the administration of PR001 to a single patient with Type 2 Gaucher disease via a compassionate use pathway, following approval by an international regulatory authority, and the patient was recently dosed. Type 2 Gaucher disease is the more severe form of nGD, which presents in infancy and involves rapidly progressing neurodegeneration leading to death in infancy or early childhood.

Separately, as announced on December 26, 2019, Prevail's Investigational New Drug (IND) application for PR001 for the treatment of nGD is now active. The Company is proceeding with its Phase 1/2 clinical trial for Type 2 Gaucher disease patients and expects to initiate patient dosing during the first half of 2020. The Company also plans to initiate a Phase 1/2 clinical trial for Type 3 Gaucher disease patients in the second half of 2020 under the same IND. Prevail intends for the Company's clinical studies to serve as the primary option for patients to receive access to any of Prevail's investigational drugs.

"Our decision to grant a compassionate use request for this patient and our continued progress toward initiating clinical trials underscore our deep dedication to our mission of treating all eligible patients with neuronopathic Gaucher disease, the most severe form of Gaucher disease," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "We continue to believe that PR001 has the potential to be a disease-modifying therapy for the treatment of Parkinson's disease with *GBA1* mutation and neuronopathic Gaucher disease, which share the same underlying genetic mechanism."

Prevail is also developing PR001 for Parkinson's disease patients with a *GBA1* mutation (PD-GBA). The Company has an active IND for PR001 for the treatment of PD-GBA and the PROPEL Phase 1/2 clinical trial for PD-GBA patients is now recruiting.

About Neuronopathic Gaucher Disease
Gaucher disease is a lysosomal storage disorder caused by mutations in the glucocerebrosidase gene *GBA1*, leading to multi-organ pathology. Patients with severe mutations in the *GBA1* gene can present with neuronopathic Gaucher disease, also termed Type 2 or Type 3 Gaucher disease. Type 2 Gaucher disease presents in infancy and involves rapidly progressive neurodegeneration leading to death in infancy or early childhood. Type 3 Gaucher disease typically presents in childhood and can involve neurological manifestations such as gaze and motor abnormalities and seizures. There are no therapies approved by the FDA for the treatment of neuronopathic Gaucher disease.

About Prevail Therapeutics
Prevail is a gene therapy company leveraging breakthroughs in human genetics with the goal of developing and commercializing disease-modifying AAV-based gene therapies for patients with neurodegenerative diseases. The company is developing PR001 for patients with Parkinson's disease with a GBA1 mutation (PD-GBA) and neuronopathic Gaucher disease; PR006 for patients with frontotemporal dementia with GRN mutation (FTD-GRN); and PR004 for patients with certain synucleinopathies.

Prevail was founded by Dr. Asa Abeliovich in 2017, through a collaborative effort with The Silverstein Foundation for Parkinson's with GBA and OrbiMed, and is headquartered in New York, NY.

Forward-Looking Statements Related to Prevail
Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Examples of these forward-looking statements include statements concerning Prevail's ability to develop meaningful therapeutic advances for patients with neurodegenerative diseases and the timing and progress of clinical trials. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. These risks and uncertainties include, among others: Prevail's novel approach to gene therapy makes it difficult to predict the time, cost and potential success of product candidate development or regulatory approval; Prevail's gene therapy programs may not meet safety and efficacy levels needed to support ongoing clinical development or regulatory approval; the regulatory landscape for gene therapy is rigorous, complex, uncertain and subject to change; and the fact that gene therapies are novel, complex and difficult to manufacture. These and other risks are described more fully in Prevail's filings with the Securities and Exchange Commission (SEC), including the "Risk Factors" section of the Company's Quarterly Report on Form 10-Q for the period ended September 30, 2019, filed with the SEC on November 12, 2019, and its other documents subsequently filed with or furnished to the SEC. All forward-looking statements contained in this press release speak only as of the date on which they were made. Except to the extent required by law, Prevail undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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