Prevail Therapeutics Receives U.S. FDA Fast Track Designation for PR001 for the Treatment of Parkinson's Disease Patients with a GBA1 Mutation

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Company Plans to Initiate Phase 1/2 Clinical Trial in the Second Half of 2019

NEW YORK, July 08, 2019 (GLOBE NEWSWIRE) -- Prevail Therapeutics Inc. (Nasdaq: PRVL) (Prevail), a biotechnology company developing potentially disease-modifying AAV-based gene therapies for patients with neurodegenerative disorders, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation for the Company's lead gene therapy program, PR001, for the treatment of Parkinson's disease patients with a *GBA1* mutation (PD-GBA). PR001 is a potentially disease-modifying, single-dose, AAV9-based gene therapy being developed for the treatment of PD-GBA and neuronopathic Gaucher disease.

"We are pleased that the FDA has granted Fast Track Designation for PR001, which underscores the unmet need of patients with Parkinson's disease with a *GBA1* mutation, a chronic and progressive neurodegenerative disorder that comprises 7% to 10% of the total Parkinson's disease population worldwide," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "With no treatments available that modify the progressive course or the underlying disease process of Parkinson's disease, a potential disease-modifying therapy like PR001 could significantly transform the lives of patients with this disease."

Following the FDA's acceptance of Prevail's Investigational New Drug (IND) application in June, the Company is on track to begin dosing patients in a Phase 1/2 clinical trial in the second half of 2019. The trial will investigate the safety and tolerability of PR001, and will also measure key biomarkers and exploratory efficacy endpoints, in patients with PD-GBA.

About Fast Track Designation
The FDA's Fast Track designation is a process designed to facilitate the development and expedite the review of product candidates to treat serious conditions and fill an unmet medical need. Fast Track designation allows for early and frequent communication with the FDA throughout the entire drug development and review process. It allows for accelerated approval or rolling review of a company's Biologics License Application (BLA). In addition, such a product candidate could be eligible for Priority Review if supported by clinical data at the time of BLA submission.

About PD-GBA
Parkinson's disease is a chronic, progressive neurodegenerative disorder that affects up to one million people in the United States and more than seven million people worldwide. PD-GBA affects 7% to 10% of the total Parkinson's disease population worldwide and an estimated 90,000 individuals in the United States alone. *GBA1* encodes the lysosomal enzyme, beta-glucocerebrosidase, or GCase. Mutations in the *GBA1* gene lead to a deficiency of GCase, resulting in lysosomal dysfunction in CNS cells, which we believe leads to the inflammation and neurodegeneration present in PD-GBA. *GBA1* mutations impact the risk of developing Parkinson's disease as well as many other aspects of the disease course, including the severity, age of onset and rate of progression of disease and the likelihood of dementia. There are no treatments available that modify the progressive course or the underlying disease process of Parkinson's 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. 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

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: the potential for PR001 to transform the lives of patients with Parkinson's disease; the timing of initiation of Prevail's Phase 1/2 clinical trial of PR001; and the potential advantages of FDA's Fast track designation. 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: our novel approach to gene therapy makes it difficult to predict the time, cost and potential success of product candidate development or regulatory approval; PR001 may not meet safety and efficacy levels needed to support ongoing clinical development or regulatory approval; and the regulatory landscape for gene therapy is rigorous, complex, uncertain and subject to change. These and other risks are described more fully in Prevail's filings with the Securities and Exchange Commission. 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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