Prevail Therapeutics Provides Clinical Advancement Update on PR001 for the Treatment of Parkinson's Disease with GBA1 mutations

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Company Will Present at Cowen Healthcare Conference Today

NEW YORK, March 03, 2020 (GLOBE NEWSWIRE) -- Prevail Therapeutics Inc. (Nasdaq: PRVL), a biotechnology company developing potentially disease-modifying AAV-based gene therapies for patients with neurodegenerative diseases, provided an update today on the clinical advancement of its gene therapy program PR001 for patients with Parkinson's disease with *GBA1* mutations (PD-GBA). Enrollment in the PR001 Phase 1/2 PROPEL clinical trial is progressing, patient dosing continues, and the Company is on track to report interim data on a subset of patients in the second half of 2020.

The Company will present on its clinical progress at the Cowen & Co. Annual Healthcare Conference in Boston today.

"We believe the PROPEL trial makes PR001 the first potentially disease-modifying gene therapy for PD-GBA patients to enter clinical trials. Its ongoing progress brings us a step closer to new treatment options for patients living with PD-GBA," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "We are excited about the potential of PR001 to slow or stop disease progression for PD-GBA patients."

The PROPEL trial is a randomized, double-blind Phase 1/2 clinical trial evaluating the safety and tolerability of two escalating dose levels of PR001 in up to 16 patients with moderate-to-severe PD-GBA. The trial also evaluates the effect of PR001 on biomarkers of disease activity and on Parkinson's disease clinical efficacy measures. Full trial details are available at clinicaltrials.gov.

PR001 utilizes an AAV9 viral vector to deliver the *GBA1* gene to a patient's cells, correcting the lysosomal enzyme deficiency caused by PD-GBA patients' *GBA1* mutations. *GBA1* encodes the lysosomal enzyme, beta-glucocerebrosidase, or GCase, which is required for the disposal and recycling of glycolipids. PD-GBA patients have a mutation in at least one chromosomal copy of *GBA1*.

In addition to the PROPEL clinical trial for patients with PD-GBA, PR001 is also being developed for neuronopathic Gaucher disease, a devastating disorder that shares the same underlying genetic mechanism. In December 2019, the Company announced that its IND for PR001 for the treatment of neuronopathic Gaucher disease is active.

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. 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, the continued progression of our PROPEL Phase 1/2 clinical trial, the continued dosing of patients and the expected timing for the delivery of interim data from a subset of patients from our PROPEL Phase 1/2 clinical trial. 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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