Prevail Therapeutics' PR001 Receives Orphan Drug Designation and Rare Pediatric Disease Designation from FDA

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Orphan Drug Designation Granted for Treatment of Gaucher Disease

Rare Pediatric Disease Designation Granted for Treatment of Neuronopathic Gaucher Disease

NEW YORK, Feb. 12, 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, announced today that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation for the Company's investigational gene therapy, PR001, for the treatment of patients with Gaucher disease. The Company also announced that the FDA has granted Rare Pediatric Disease Designation for PR001 for the treatment of neuronopathic Gaucher disease (nGD), the most severe form of the condition.

"We are pleased to receive these important designations from the FDA, which underscore the critical nature of our work," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "These designations support our conviction that new gene therapies for Gaucher disease are urgently needed — especially for the severe, neuronopathic form of the disease, for which there are no FDA-approved therapies."

PR001 is also being developed as a potentially disease-modifying, single-dose gene therapy for Parkinson's disease with *GBA1* mutation (PD-GBA).

Orphan Drug Designation is granted by the FDA to drugs or biologics intended to treat a rare disease or condition, defined as one that affects fewer than 200,000 people in the United States. Programs with Orphan Drug status receive partial tax credit for clinical trial expenditures, waived user fees and eligibility for seven years of marketing exclusivity.

Rare Pediatric Disease Designation is granted by the FDA in the case of serious or life-threatening diseases affecting fewer than 200,000 people in the United States, primarily those 18 years of age and younger. The sponsor of a drug with Rare Pediatric Disease Designation may, upon marketing approval, qualify for receipt of a priority review voucher applicable to a subsequent marketing application, which voucher is fully transferable.

Prevail announced in December 2019 that its Investigational New Drug (IND) application for PR001 for the treatment of nGD is active. Prevail 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.

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 seizures and gaze and motor abnormalities. 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, the timing and progress of clinical trials, and the potential advantages of Orphan Drug Designation and Rare Pediatric Disease Designation by the FDA. 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; despite receiving Rare Pediatric Disease designation, the Company may not be successful in obtaining a priority review voucher; 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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