Prevail Therapeutics Receives U.S. FDA Fast Track Designation for PR001 for the Treatment of Neuronopathic Gaucher Disease

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NEW YORK, Oct. 27, 2020 (GLOBE NEWSWIRE) -- Prevail Therapeutics Inc. (Nasdaq: PRVL), a biotechnology company developing potentially disease-modifying AAV-based gene therapies for patients with neurodegenerative diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for the Company's experimental gene therapy program, PR001, for the treatment of neuronopathic Gaucher disease (nGD). The Company expects to initiate enrollment of the PROVIDE Phase 1/2 clinical trial of PR001 for Type 2 neuronopathic Gaucher disease patients in the second half of 2020.

"FDA Fast Track designation for PR001 for the treatment of neuronopathic Gaucher disease underscores the significant unmet medical need for this devastating condition, for which there are no currently approved therapies," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "Type 2 Gaucher disease involves rapidly progressive neurodegeneration leading to death in infancy or early childhood, and Type 3 Gaucher disease is associated with significant neurological manifestations including seizures and motor abnormalities. We believe PR001 has the potential to serve as a much-needed therapeutic option for these patients as enzyme replacement therapies approved for Type 1 Gaucher disease cannot cross the blood brain barrier to address neurological symptoms."

The FDA previously granted PR001 Rare Pediatric Disease Designation for the treatment of nGD, and Orphan Drug Designation for the treatment of patients with Gaucher disease. In addition, the FDA has granted Fast Track designation for PR001 for the treatment of Parkinson's disease with *GBA1* mutations.

About Fast Track Designation
The FDA's Fast Track designation is a process designed to expedite or facilitate 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 may also allow for priority or rolling review of a company's Biologics License Application (BLA).

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 *GBA1* mutations (PD-GBA) and neuronopathic Gaucher disease (nGD); PR006 for patients with frontotemporal dementia with *GRN* mutations (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 the potential advantages of Fast Track designation and the potential for PR001 to serve as a potentially disease-modifying therapeutic option for patients with neuronopathic Gaucher disease. 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; the fact that gene therapies are novel, complex and difficult to manufacture; and risks relating to the impact on our business of the COVID-19 pandemic or similar public health crises. 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 June 30, 2020, filed with the SEC on August 11, 2020, 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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