



Prevail Therapeutics Announces IND Active for PR001 for Treatment of Neuronopathic Gaucher Disease

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NEW YORK, Dec. 26, 2019 (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 announced that the U.S. Food and Drug Administration (FDA) has notified Prevail that the Company's Investigational New Drug (IND) application for PR001 for the treatment of neuronopathic Gaucher disease (nGD) patients is now active and that Prevail may proceed with initiating its proposed clinical trial. As previously reported, Prevail's IND for PR001 for the treatment of nGD had been put on clinical hold by the FDA, and this clinical hold has now been removed.

The Company's planned Phase 1/2 clinical trial for nGD patients will commence at a dose higher than originally proposed. Prevail submitted nonclinical data in which no PR001-related safety events or adverse findings were observed, supporting the initiation of the Phase 1/2 clinical trial at this higher dose.

Prevail is activating a Phase 1/2 clinical trial for Type 2 Gaucher disease patients and expects to initiate patient dosing during the first half of 2020. 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. 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 nGD IND. Type 3 Gaucher disease is a form of nGD that typically presents in childhood and involves multiple neurological manifestations.

"We are pleased to now have an active IND for PR001 for the nGD indication and look forward to initiating a Phase 1/2 clinical trial in the first half of 2020," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "Patients with nGD have the most severe form of Gaucher disease and a significant unmet need for therapies to treat their neurological manifestations. We believe PR001 has tremendous potential to help patients suffering from this devastating disease."

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 timing of initiation of clinical trials for the treatment of nGD, the potential for PR001 to slow or stop disease progression in these patients and Prevail's ability to develop meaningful therapeutic advances for patients with neurodegenerative diseases. 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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