



Prevail Therapeutics Announces Investigational New Drug Application Active for PR006 for the Treatment of Frontotemporal Dementia with GRN Mutation

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NEW YORK, March 02, 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 accepted the Company's Investigational New Drug (IND) application for its experimental gene therapy program, PR006, for the treatment of frontotemporal dementia patients with *GRN* mutation (FTD-GRN), and that the Company may proceed with the initiation of its Phase 1/2 clinical trial.

PR006 is being developed as a potential one-time gene therapy for FTD-GRN, a progressive neurodegenerative disease caused by mutations in the *GRN* gene that reduce production of progranulin, a protein critical for lysosomal function, neuronal survival, and normal microglial activities. The progranulin deficiency leads to lysosomal dysfunction, ineffective protein degradation and recycling, neuroinflammation, and ultimately neurodegeneration and death, typically within three to ten years of diagnosis.

PR006 is designed to increase progranulin levels in the brains of FTD-GRN patients by delivering a healthy *GRN* gene using an AAV9 vector.

"FTD-GRN is a serious and progressive neurodegenerative disease for which there are no approved treatments," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "Now that the PR006 IND is active, we look forward to advancing clinical development of PR006, which has the potential to positively impact the lives of patients by slowing or stopping the progression of this devastating disease."

The PROCLAIM Phase 1/2 clinical trial will investigate the safety and tolerability of PR006, and will also measure key biomarkers and exploratory efficacy endpoints in patients with FTD-GRN. The Company plans to begin dosing for PROCLAIM this year. We believe this makes PR006 the first gene therapy for FTD-GRN to enter clinical trials.

The FDA has granted Orphan Drug Designation for PR006 for the treatment of patients with FTD.

About Frontotemporal Dementia with a GRN Mutation

Frontotemporal dementia (FTD) is the second most common cause of dementia in people under the age of 65, after Alzheimer's disease. 50,000 to 60,000 people in the U.S. and 80,000 to 110,000 individuals in the European Union are affected by FTD. Frontotemporal dementia with a *GRN* mutation (FTD-GRN) represents 5-10% of all patients with FTD. FTD results from the progressive degeneration of the frontal and temporal lobes of the brain, which control decision-making, behavior, emotion and language. In FTD-GRN patients, reduced levels of progranulin lead to age-dependent lysosomal dysfunction, neuroinflammation, and neurodegeneration. There are no approved treatments for FTD or FTD-GRN.

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 ability of PR006 to increase progranulin levels in the brains of FTD-GRN patients, and timing of enrollment and dosing of patients in the PROCLAIM 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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