Prevail Therapeutics Receives U.S. FDA Fast Track Designation for PR006 to Slow the Progression of Frontotemporal Dementia with a GRN Mutation

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NEW YORK, March 24, 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, PR006, to slow the progression of frontotemporal dementia with a *GRN* mutation (FTD-GRN). Prevail announced an active IND for the Phase 1/2 clinical trial of PR006 for the treatment of FTD-GRN earlier this month.

"The FDA's decision to grant Fast Track Designation for PR006 is an important step forward in our mission to deliver a potentially disease-modifying gene therapy to FTD-GRN patients as quickly as possible," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "FTD-GRN progresses rapidly and there are currently no therapeutic options available. We believe PR006 has the potential to fill this unmet medical need and make a significant impact for patients."

About Fast Track Designation
The FDA's Fast Track designation is a process designed to facilitate the development and expedite 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 allows for accelerated approval or rolling review of a company's Biologics License Application (BLA). In addition, such a product candidate could be eligible for Priority Review if supported by clinical data at the time of BLA submission.

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. FTD affects 50,000 to 60,000 people in the U.S. and 80,000 to 110,000 individuals in the European Union. 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 PR006 for patients with frontotemporal dementia with a *GRN* mutation (FTD-GRN); PR001 for patients with Parkinson's disease with a *GBA1* mutation (PD-GBA) and neuronopathic Gaucher disease; 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 and slow the progression of FTD-GRN. 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 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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