Prevail Therapeutics Announces FDA Orphan Drug Designation Granted to PR006 for the Treatment of Patients with Frontotemporal Dementia with a GRN Mutation

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NEW YORK, Dec. 16, 2019 (GLOBE NEWSWIRE) -- Prevail Therapeutics Inc. (Nasdaq: PRVL), 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 (ODD) for the company's gene therapy candidate, PR006, for the treatment of patients with frontotemporal dementia (FTD) with a *GRN* mutation (FTD-GRN). PR006 is designed to increase progranulin levels in FTD-GRN patients by delivering a healthy *GRN* gene using an AAV9 vector.

Orphan Drug designation is granted by the U.S. Food and Drug Administration to drugs or biologics intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals 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.

"FTD with a *GRN* mutation is characterized by progressive difficulties in decision-making, behavior and language," said Asa Abeliovich, M.D., Ph.D., founder and chief executive officer of Prevail. "With no approved treatments for FTD, there is an urgent unmet need for therapies that slow or stop this disease. Orphan drug designation is an important milestone as we prepare to bring PR006 into the clinic in the first half of 2020."

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 and the ability to derive benefits from Orphan Drug designation. 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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