Prevail Therapeutics Announces First Patient Dosed in Phase 1/2 PROCLAIM Clinical Trial Evaluating PR006 for the Treatment of Frontotemporal Dementia Patients with GRN Mutations

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NEW YORK, Dec. 11, 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 first patient has been dosed in the Phase 1/2 PROCLAIM clinical trial evaluating PR006, an investigational AAV9 gene therapy delivering the *GRN* gene, for the treatment of frontotemporal dementia patients with *GRN* mutations (FTD-GRN).

"Dosing the first patient in our PROCLAIM clinical trial marks an important milestone in our efforts to advance a potentially disease-modifying treatment for patients with frontotemporal dementia with *GRN* mutations," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "We are excited to progress clinical development of PR006 and to bring forward a much-needed therapy for this rapidly progressing neurodegenerative disease."

The PROCLAIM trial is a Phase 1/2 open-label trial investigating the safety and tolerability of PR006 as well as key biomarkers and exploratory efficacy endpoints. The Company expects to enroll up to 15 patients, and it currently anticipates it will provide a biomarker and safety analysis on a subset of patients enrolled in the PROCLAIM trial in 2021.

"Frontotemporal dementia is a devastating condition, with no disease-modifying therapeutic options available," said Dr. Jonathan Rohrer, principal research fellow at the University College London Queen Square Institute of Neurology. "PROCLAIM is an important clinical study which could further increase our understanding of frontotemporal dementia due to mutations in the progranulin gene, and help demonstrate the potential of gene therapy to correct the underlying genetic cause of this condition, potentially slowing or stopping disease progression."

PR006 has been granted Orphan Drug designation for the treatment of FTD and Fast Track designation for the treatment of FTD-GRN by the U.S. Food and Drug Administration, as well as orphan designation for the treatment of FTD by the European Commission.

About Frontotemporal Dementia with *GRN* Mutations
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 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 for PR006 to be a disease-modifying gene therapy to patients with FTD-GRN; the potential benefits of Fast Track and Orphan Drug designation by the FDA and orphan designation by the FDA and the European Commission; the anticipated timing of enrollment and of reporting of interim data on a subset of patients from the PROCLAIM trial; and the potential for the PROCLAIM trial to increase understanding of frontotemporal dementia and help demonstrate the potential of gene therapy to correct the underlying genetic cause of this condition, potentially slowing or stopping disease progression. 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" sections of the Company's most recent Annual Report on Form 10-K and Quarterly Report on Form 10-Q filed with the SEC, 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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