Prevail Therapeutics Receives European Commission Orphan Designation for PR006 for the Treatment of Frontotemporal Dementia

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NEW YORK, Nov. 30, 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 European Commission has granted orphan designation for PR006 for the treatment of frontotemporal dementia (FTD). PR006 is an investigational AAV9 gene therapy delivering the *GRN* gene and is being developed as a potential therapy for patients with frontotemporal dementia with *GRN* mutations (FTD-GRN).

"The European Commission's decision to grant orphan designation for PR006 is an important step in helping to advance this potential therapeutic option 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 as part of our mission to deliver a potentially disease-modifying gene therapy to these patients as quickly as possible."

The Company expects to initiate enrollment in the Phase 1/2 PROCLAIM trial of PR006 in the fourth quarter of 2020, and it currently anticipates it will provide a biomarker and safety analysis on a subset of patients in the PROCLAIM trial in 2021.

The U.S. Food and Drug Administration has also granted Orphan Drug designation for PR006 for the treatment of FTD and Fast Track designation for FTD-GRN.

About European Commission Orphan Designation
Orphan designation is granted by the European Commission to encourage development of medicines intended to treat a seriously debilitating or life-threatening condition that affects fewer than five in 10,000 people in the European Union. Orphan designation by the European Commission provides companies with certain benefits and incentives, including protocol assistance, reduced regulatory fees and 10 years of market exclusivity following regulatory approval.

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 Prevail's mission to deliver a potentially disease-modifying gene therapy to patients with FTD-GRN as quickly as possible; the potential benefits of orphan designation by the European Commission; and the anticipated timing of enrollment and of reporting of interim data on a subset of patients from 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; 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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