Prevail Therapeutics Granted Composition of Matter Patent for Experimental Gene Therapy Program PR001

November 19, 2020

NEW YORK, Nov. 18, 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 United States Patent and Trademark Office (USPTO) on November 17, 2020 issued a composition of matter patent, U.S. Patent No. 10,837,028, with claims directed to the AAV vector used in PR001, Prevail's experimental gene therapy program for the treatment of Parkinson's disease with *GBA1* mutations (PD-GBA) and neuronopathic Gaucher disease (nGD). The base patent term extends until October 3, 2038, excluding patent term extensions or coverage in additional related patent filings.

"We are excited to make important progress this year with PR001, which is being evaluated in the Phase 1/2 PROPEL trial for patients with Parkinson's disease with *GBA1* mutations and in the Phase 1/2 PROVIDE trial for patients with Type 2 Gaucher disease," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "We are advancing clinical development of PR001 to make a potentially transformative difference for these patients who currently have no approved treatment options."

The Company recently announced that patient dosing has continued in the Phase 1/2 PROPEL clinical trial of PR001 for PD-GBA patients, and it expects to provide the next biomarker and safety analysis on a subset of patients in the PROPEL trial by mid-2021. The Company expects to initiate enrollment of the Phase 1/2 PROVIDE clinical trial of PR001 for Type 2 Gaucher disease patients in the fourth quarter of 2020 and currently anticipates it will provide the next update on PR001 biomarker and safety data for nGD in 2021.

The U.S. Food and Drug Administration has granted Fast Track designations for PR001 for the treatment of PD-GBA and nGD. In addition, the FDA granted PR001 Rare Pediatric Disease designation for the treatment of nGD, and Orphan Drug designation for the treatment of patients with Gaucher disease.

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 Prevail's gene therapy candidates to make a transformative difference for patients with neurodegenerative diseases; the expected timing of reporting additional interim data on a subset of patients from the PROPEL trial; and the anticipated timing of enrollment of and the next update on data from the PROVIDE 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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