Prevail Therapeutics Granted Composition of Matter Patent for Experimental Gene Therapy Program PR006

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Gene Therapy is Being Developed for the Treatment of Frontotemporal Dementia patients with GRN Mutations

NEW YORK, July 27, 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 June 23, 2020 issued a composition of matter patent, U.S. Patent No. 10,689,625, with claims directed to the AAV vector used in PR006, Prevail's experimental gene therapy program for the treatment of frontotemporal dementia patients with *GRN* mutations (FTD-GRN). The base patent term extends until October 2038, excluding patent term extensions or coverage in additional related patent filings.

"At Prevail, we are working continuously to bring innovative treatments to patients with neurodegenerative diseases," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "FTD-GRN is a devastating condition, with no disease-modifying therapeutic options available. We are excited about the possibility of making a significant impact with PR006."

In March of this year, Prevail announced an active IND for the Phase 1/2 clinical trial of PR006 for the treatment of FTD-GRN. The U.S. Food and Drug Administration (FDA) has granted the therapy Fast Track Designation.

PR006 is being developed as a potential one-time gene therapy for FTD-GRN, a progressive neurodegenerative disease caused by mutations in the *GRN* gene that reduce production of progranulin, a protein critical for lysosomal function, neuronal survival and normal microglial activities. The progranulin deficiency leads to lysosomal dysfunction, ineffective protein degradation and recycling, neuroinflammation, and ultimately neurodegeneration and death, typically within three to ten years of diagnosis.

PR006 is designed to increase progranulin levels in the brains of FTD-GRN patients by delivering a healthy *GRN* gene using an AAV9 vector.

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. In addition to PR006 for patients with frontotemporal dementia with *GRN* mutations (FTD-GRN), the company is developing PR001 for patients with Parkinson's disease with *GBA1* mutations (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. 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 March 31, 2020, filed with the SEC on May 14, 2020, 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.

Media Contact:
Mary Carmichael
Ten Bridge Communications
mary@tenbridgecommunications.com
617-413-3543

Investor Contact:

investors@prevailtherapeutics.com

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