



Prevail Therapeutics to Highlight Multiple Gene Therapy Programs for Neurodegenerative Diseases at the Virtual 2020 Alzheimer's Association International Conference

July 22, 2020

Preclinical Data Demonstrate Potential of Prevail's AAV Gene Therapy Approach to Slow or Halt Progression in Multiple Neurodegenerative Diseases

Company Provides Overview of Planned Phase 1/2 PR006 PROCLAIM Clinical Trial for FTD-GRN Patients

*Company to Host a Panel Discussion and Q&A Session on FTD-GRN
with Jonathan Rohrer, Ph.D., MRCP, an Expert in Frontotemporal Dementia (FTD) and its Genetic Causes*

NEW YORK, July 22, 2020 (GLOBE NEWSWIRE) -- [Prevail Therapeutics Inc.](https://www.prevailtherapeutics.com) (Nasdaq: PRVL), a biotechnology company developing potentially disease-modifying AAV-based gene therapies for patients with neurodegenerative diseases, today announced three upcoming poster presentations at the Alzheimer's Association International Conference (AAIC) 2020. These data underscore the robust preclinical evidence in support of Prevail's AAV-based gene therapy approach, and highlight the Company's strategy to validate these data in the planned PROCLAIM clinical trial evaluating PR006 for the treatment of frontotemporal dementia patients with *GRN* mutations (FTD-GRN). The conference will be held virtually July 27-31, 2020.

"Our novel gene therapy candidates have the potential to transform the treatment of patients with FTD-GRN and other devastating neurodegenerative diseases," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "An increased understanding of genetically defined forms of neurodegenerative diseases, including the role that *GRN* mutations can play in frontotemporal dementia, has opened exciting possibilities to stop or slow disease progression using a gene therapy approach."

PR001

Prevail will highlight preclinical data demonstrating the effect of PR001 treatment on key disease biomarkers and functional parameters. These data provide the basis for the Company's clinical trials for Type 2 neuronopathic Gaucher disease (nGD) patients and Parkinson's disease with *GBA1* mutations (PD-GBA) patients.

Poster title: PR001 Gene Therapy Improved Phenotypes in Models of Parkinson's Disease with *GBA1* Mutation

Session date and time: Monday, July 27, 12:00 a.m. - 11:59 p.m. CDT

PR006

Prevail will present the design of the PROCLAIM Phase 1/2 clinical trial for the treatment of FTD-GRN patients and preclinical data demonstrating the effect of PR006 treatment on progranulin expression, lysosomal dysfunction and inflammation in the CNS.

Poster title: Preclinical Development of PR006, a Gene Therapy for the Treatment of Frontotemporal Dementia with Progranulin Mutations

Session date and time: Monday, July 27, 12:00 a.m. - 11:59 p.m. CDT

Poster title: Design of a Phase 1/2 Study of an AAV9-Based Gene Therapy for Fronto-Temporal Dementia Patients with Pathogenic *GRN* Mutations (PROCLAIM Trial)

Session date and time: Wednesday, July 29, 12:00 a.m. - 11:59 p.m. CDT

FTD-GRN Panel Discussion and Q&A Session

In addition to its presentations at AAIC, on Wednesday, July 29 at 2:30 p.m. EDT, management will be hosting a panel discussion and Q&A session on FTD-GRN by Jonathan Rohrer, Ph.D., MRCP, an expert in frontotemporal dementia (FTD) and its genetic causes. A live webcast of the event and replay following its conclusion will be available on the Events and Presentations section of the Company's website at <https://ir.prevailtherapeutics.com/events>.

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; 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 transform the treatment of patients with, and slow or halt the progression of, FTD-GRN and other 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.

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