



Prevail Therapeutics to Highlight Multiple CNS-Focused Gene Therapy Programs at 2020 American Society of Gene & Cell Therapy Annual Meeting

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

Company Provides Design Overview for Planned Phase 1/2 PR001 PROVIDE and PR006 PROCLAIM Clinical Trials

NEW YORK, April 29, 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 two upcoming oral presentations and two poster presentations at the virtual 2020 American Society of Gene & Cell Therapy (ASGCT) Annual Meeting. 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 multiple ongoing or planned clinical trials.

"Patients living with neurodegenerative diseases are in urgent need of novel therapies with the potential to slow or stop the course of their disease," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "Our gene therapy candidates have the potential to improve outcomes for patients with genetically defined forms of Parkinson's disease, frontotemporal dementia and neuronopathic Gaucher disease."

PR001

Prevail will highlight the design of the planned PROVIDE Phase 1/2 trial for the treatment of Type 2 neuronopathic Gaucher disease (nGD) patients and preclinical data demonstrating the impact of PR001 treatment on key disease biomarkers and functional parameters. These data provide the basis for the company's clinical programs in nGD and Parkinson's disease with *GBA1* mutations (PD-GBA).

Poster title: Design of a Phase 1/2 Study to Evaluate the Safety and Efficacy of an AAV9-Based Gene Therapy in Infants with Type 2 Gaucher Disease (PROVIDE Trial)

Session title: AAV Vectors - Clinical Studies

Session date and time: Wednesday, May 13th, 5:30 p.m.-6:30 p.m. ET

Presentation title: PR001 Gene Therapy Increased GCase Activity and Ameliorated *GBA1*-Associated Disease Phenotypes

Session title: AAV Vectors Preclinical and Proof-of-Concept Studies in Systemic Diseases

Presentation date and time: Thursday, May 14th 3:45 p.m.-4:00 p.m. ET

Presenter: Patty Sheehan, Ph.D., Senior Scientist, Prevail Therapeutics

PR006

Prevail will present the design of the PROCLAIM Phase 1/2 clinical trial for the treatment of frontotemporal dementia patients with *GRN* mutations (FTD-GRN) and preclinical data demonstrating the impact of PR006 treatment on progranulin expression, lysosomal dysfunction and inflammation in the CNS.

Poster title: Proposed Patient Population and Outcome Measures for a First in Human Study of PR006, an AAV9-Based Gene Therapy, for Frontotemporal Dementia Patients with Pathogenic *GRN* Mutations

Session title: AAV Vectors - Clinical Studies

Session date and time: Wednesday, May 13th, 5:30 p.m.-6:30 p.m. ET

Presentation title: PR006, an AAV Gene Therapy Vector Expressing Progranulin, Improved FTD-GRN Phenotypes *In Vitro* and *In Vivo*

Session title: AAV Vectors Preclinical and Proof-of-Concept Studies in CNS Disorders

Presentation date and time: Friday, May 15th 10:15 a.m.-10:30 a.m. ET

Presenter: Laura D. Heckman, Ph.D., Director, Preclinical Development, Prevail Therapeutics

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.

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