



Prevail Therapeutics Announces IND Active for Phase 1/2 Trial of its Gene Therapy PR001 to Treat Parkinson's Disease Patients with GBA1 Mutations

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NEW YORK--([BUSINESS WIRE](#))--Prevail Therapeutics Inc. (Prevail), a gene therapy company developing AAV-based gene therapies for patients with neurodegenerative diseases, today announced the U.S. Food and Drug Administration (FDA) has accepted the Company's Investigational New Drug (IND) application for its lead gene therapy program, PR001. PR001 is a potentially disease-modifying, single-dose, AAV9-based gene therapy being developed for the treatment of Parkinson's disease patients with a *GBA1* mutation (PD-GBA).

"We are pleased that the FDA has accepted the IND for our first program, which we believe has the potential to transform the lives of patients with Parkinson's disease with a *GBA1* mutation," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "At Prevail, our goal is to halt the progression of serious neurodegenerative diseases by applying precision medicine to the development of gene therapies. Our active IND brings us a step closer to achieving that goal, and we look forward to entering this new phase as a clinical-stage company."

The active IND allows Prevail to initiate a Phase 1/2 clinical trial that will investigate the safety and tolerability of PR001, and will also measure key biomarkers and exploratory efficacy endpoints, in patients with PD-GBA. The Company plans to begin dosing patients in the trial this year.

About PD-GBA

Parkinson's disease is a chronic, progressive neurodegenerative disorder that affects up to one million people in the United States and more than seven million people worldwide. PD-GBA affects 7% to 10% of the total Parkinson's disease population worldwide and an estimated 90,000 individuals in the United States alone. *GBA1* encodes the lysosomal enzyme, beta-glucocerebrosidase, or GCCase. Mutations in the *GBA1* gene lead to a deficiency of GCCase, resulting in lysosomal dysfunction in CNS cells, which we believe leads to the inflammation and neurodegeneration present in PD-GBA. *GBA1* mutations impact the risk of developing Parkinson's disease as well as many other aspects of the disease course, including the severity, age of onset and rate of progression of disease and the likelihood of dementia. There are no treatments available that modify the progressive course or the underlying disease process of Parkinson's disease.

About Prevail Therapeutics

Prevail Therapeutics 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. Prevail was founded by Dr. 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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