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PROCEED trial evaluating PR001 for GD1 announced

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At Prevail, our team strives to develop disease-modifying gene therapies for patients with neurodegenerative diseases, including Parkinson's disease (PD), frontotemporal dementia (FTD) and Gaucher disease (GD). Our vision is to leverage transformative advances in biology and technology to deliver therapies for disorders that cause neurodegeneration. Traditional therapeutic approaches have repeatedly failed to successfully halt or reverse neurodegeneration and genomic medicine has opened new doors for innovative treatment options by targeting critical genes and pathways that cause these disorders. This precision medicine is exciting, and we are proud to be advancing this class of therapeutic development.

Over the past three years, we have initiated three clinical trials: PROCLAIM, for the treatment of FTD patients with *GRN* gene mutations (FTD-GRN), PROPEL, for the treatment of PD patients with at least one *GBA1* mutation (PD-GBA) and PROVIDE, for the treatment of Type 2 GD (GD2) patients. Each trial is actively recruiting patients in the United States, as well as several global sites including the United Kingdom, Spain, Israel and Australia.

Today, we are excited to announce that the FDA has accepted our third Investigational New Drug (IND) application under our PR001 experimental gene therapy program. In addition to evaluating PR001 in the PD-GBA and GD2 indications, this acceptance allows us to begin to study PR001 for Type 1 Gaucher disease (GD1) in our Phase 1/2 clinical trial PROCEED.

PROCEED is a Phase 1/2 study designed to evaluate the safety and tolerability of a single intravenous administration of PR001 to GD1 patients who have had a suboptimal response when treated with enzyme or substrate replacement therapy. The study will first initiate in the United States with planned global expansion.

GD1 has a wide variety of symptoms, such as spleen and liver enlargement, low blood counts, issues with bleeding and bone pain and damage. Diagnosis is suspected by clinical symptoms

and confirmed by measuring GBA enzyme activity or genetic testing. People living with GD1 are at a higher risk of developing Parkinson's disease because of the genetic connection between both diseases. At Prevail, we are conducting research in Gaucher disease, which has led to our previously initiated PROVIDE trial for patients with GD2. We have also recently initiated our PROCEED trial for patients with GD1 with the goal of expanding our treatment landscape for a wider range of people living with this disease around the globe.

"There are few clinical trials in progress for patients diagnosed with Type 1 Gaucher disease, and I am happy to see increased therapeutic options for those who will benefit from them most," said Dr. Goker-Alpan, principal investigator at the Lysosomal and Rare Disease Research and Treatment Center, a PROCEED trial site. "I look forward to evaluating the potential therapeutic impact of PR001 for Type 1 Gaucher disease and helping facilitate new treatment approaches which may decrease the burden of managing Gaucher disease for patients."

Our mission is to serve patient communities by understanding the challenges they face and treating these most difficult diseases. The Prevail team is pleased to work with the Gaucher Community Alliance, the International Gaucher Alliance and other advocacy groups to connect with patient communities and learn the needs at the forefront of people living with GD. Our team is thankful to the patients, caregivers, advocates and clinicians who provide us with the collaboration needed to advance new treatments for those most affected by neurodegenerative disorders.