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Reflecting on our commitment to the communities we serve

When Prevail isn't hard at work developing novel gene therapies for neurodegenerative and rare diseases, we are supporting our patient communities and raising disease awareness. We are grateful for the patient advocacy groups who host events and create initiatives that uplift the voices of patients and their caregivers. As our team reflects on these important partnerships heading into the new year, we want to take a moment to highlight a few initiatives that Prevail is proud to have supported.

Parkinson's Disease

Parkinson's Foundation ClinGen Sponsorship
As we kick off 2023, we are thrilled to continue our work with the Parkinson's Foundation-led Parkinson's Disease Gene Curation Expert Panel (ClinGen). ClinGen convenes an international multidisciplinary expert panel of molecular geneticists, clinicians with genetic research focus, and Parkinson's disease-specific genetic counselors to assess gene-disease validity for 21 Parkinson's disease genes.

Parkinson's Awareness Month Lunch & Learn
In April 2022, to mark Parkinson's Awareness Month, Prevail hosted a Lunch & Learn event for our team members. Speakers included Dr. Thomas Tropea, a doctor of neurology and movement disorders at University of Pennsylvania Medicine, as well as Pola Sussman, an advocate and person living with Parkinson's disease with *GBA1* mutations (PD-GBA), and her husband and fellow advocate, Rich Sussman.

Dr. Tropea's team at UPenn is studying the genetic characterization of Parkinson's disease and shared how patients respond to their participation in genetic characterization and the associated results. Pola and Rich Sussman shared their journey with Parkinson's disease, highlighting their optimistic viewpoint for scientific progress and future treatment.

The Michael J. Fox Foundation for Parkinson's Research Awareness and Education
In 2022, Prevail was honored to support the Michael J. Fox Foundation's Spotlight Campaign, which offers a full suite of educational resources for patients. Our support included online resources about Parkinson's genetics and genetic testing, including an "AskTheMD" video on genetic testing for Parkinson's disease. A blog post, Filling Out the Roster of Parkinson's Disease Therapies, also detailed different therapeutic modalities in development for Parkinson's, including gene therapy and Prevail's investigational treatment for Parkinson's, PR001.

Additionally, several members of the Prevail team participated in a virtual walk to support the Michael J. Fox Foundation for Parkinson's Research. The Fox Trot Walk aims to raise funds for Parkinson's disease research and Prevail was proud to walk as a sponsor. Our team members walked with family and friends from across many different locations to contribute to this important cause. We had a fantastic time connecting as a group with a shared mission to bring about change and increased resources from afar.

Gaucher Disease

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Gaucher Disease Awareness Month Lunch & Learn

In honor of Gaucher Awareness Month in October, Prevail welcomed Dr. Robin Ely, co-founder, and clinical director of the National Gaucher Foundation, for an educational Lunch & Learn about the need for Gaucher disease treatments and the Foundation's commitment to the Gaucher disease community. Dr. Ely shared her compelling experience fighting for and supporting the development of a treatment for her son, Brian, when he was diagnosed with Gaucher disease Type 1 (GD1) at age 4 years, when there was no available treatment. Now, Dr. Ely focuses on research that may help expand the treatment landscape for all patients living with Gaucher disease.

The International Gaucher Alliance Annual Conference and GARDIAN Registry

In May 2022, Prevail supported and attended the first International Working Group on Gaucher Disease (IWGGD)'s Symposium biennial meeting, convened by the International Gaucher Alliance (IGA). We had the opportunity to meet with physicians, researchers, advocates, and people with Gaucher disease and share our progress in both Gaucher disease Types 1 and 2. We look forward to continuing to build on these connections annually.

Over the course of the past year, we were also honored to help sponsor the Alliance's GARDIAN registry for patients with neuronopathic Gaucher disease (nGD). GARDIAN is a global registry that aims to increase understanding of the history of the disease and its impact on patients and caregivers. With this registry, those with nGD are encouraged to share their experience, either through themselves or a loved one, including details ranging from symptoms experienced to the steps that were taken when first approaching a diagnosis. With this information, the IGA will seek to improve current paths in research and drug development, while supporting clinical trial development and inform regulatory decisions.

Rare Disease Day Trivia with the Gaucher Community Alliance and Patient and Family Conference

Rare Disease Day falls on the last day of February each year, and in 2022 we hosted a virtual rare disease trivia session and Lunch & Learn event for Prevail team members, led by Cyndi Frank and Aviva Rosenberg, co-founders of the Gaucher Community Alliance (GCA). It was a fantastic opportunity for our team to join and learn more about the rare disease community and how best to support patients and their families. In addition, we were proud to have supported, shared our commitment with and learned from the GCA and the Gaucher community at the Gaucher Patient & Family Conference in October and look forward to continuing to build our relationship in 2023.

The American Society of Gene and Cell Therapy's Gaucher Disease Video

Prevail is eager for opportunities to increase education on rare diseases, so when the American Society of Gene and Cell Therapy (ASGCT) began developing an informational video on the facts and science behind Gaucher disease, our team was ready and excited to support. The video takes viewers through the connection of mutations in the *GBA* gene and Gaucher disease, the science of gene therapies used to combat the disease, and steps patients may want to take when considering entering a clinical trial. Prevail, alongside AVROBIO, contributed educational grants to aid in the video's development.

In December 2022 the PROCEED trial, which will assess our PR001 experimental gene therapy for Gaucher disease Type 1, was activated in at the first clinical trial site location at the Lysosomal Rare Disorders Research and Treatment Center in Fairfax, Virginia. With plans to expand in the U.S. and globally, we look forward to continuing to build connections with and learn more from the Gaucher community. Learn more about our commitment to Gaucher disease here.

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Frontotemporal Dementia

International Society for Frontotemporal Dementias Annual Conference
In November, the Prevail team traveled to France to support and participate in the annual International Society for Frontotemporal Dementias (ISFTD) Congress. We welcomed physicians, researchers, caregivers, and patients to learn more about Prevail in our booth, presented at the Caregiver's Day and shared data updates on our investigational treatment for *FTD-GRN*, PR006. We are already looking ahead to next year's event and hope to have lots more to share!

Frontotemporal Dementia Awareness Week and Association for Frontotemporal Degeneration Support
In honor of Frontotemporal Dementia (FTD) Week in September, our team put their chef hats on as part of The Association for Frontotemporal Degeneration's (AFTD) 10th annual Food for Thought 2022 campaign. Team members cooked recipes filled with ingredients to support brain health while learning about and bringing awareness to FTD education.

Our team also welcomed AFTD's Director of Research Engagement, Shana Dodge, Ph.D., for an insightful Lunch & Learn event focused on FTD community perspectives, gathered from the AFTD's "Frontotemporal Degeneration: A Voice of the Patient Report." Finally, in 2022, Prevail supported and participate in the AFTD's Annual Education Conference, which always invigorates our commitment to deliver potential therapies to the more than 1,750 people living with FTD.

Digital Spotlight with Rare Revolution Magazine
Last fall, Rare Revolution Magazine, a publication focused on highlighting rare diseases that lack proper public awareness and education, helped us shed light on FTD through a sponsored digital spotlight. The spotlight provided the opportunity to illuminate perspectives from caregivers, neurologists, genetic counselors, and advocates for expanded FTD awareness. Through seven in-depth articles that cover different experiences with FTD—such as the choice to receive a genetic test, an overview of different genetic variants and a deep dive into gene therapy—this partnership gave Prevail the platform to help spread awareness of FTD.

It is our honor to contribute to collaborations that foster important conversations as we work together toward new and improved treatments for rare disease. With the help of those who are passionate about supporting people with neurodegenerative diseases, the Prevail team has developed new perspectives that allow it to better address the needs of patients. Prevail extends its gratitude to all who shared their insights with our team, as the bravery of these communities to stand up and share their stories is monumental in helping biotech companies, like Prevail, better understand a patient's perspective, and create therapies that may one day lead to a cure.