

From Promise to Practice: Reflections on Our Commitment to Rare Disease Communities in 2023

2023 was a busy and exciting year for Prevail as we continued our mission to develop novel gene therapies for rare and neurodegenerative diseases and provide vital support for patient communities. Striving towards this mission would be impossible without the support of our exceptional patient advocacy partners. We deeply appreciate the invaluable work of patient advocacy groups that organize events and initiatives to amplify the voices of patients and their caregivers. As our team reflects on these meaningful partnerships and looks ahead to 2024, we would like to spotlight some of the initiatives, events, and resources that Prevail is honored to have been involved in over the last year.

[Parkinson's Disease](#)

Gene Therapy Podcast

In September, Dr Travis Lewis, MD, Executive Medical Director at Prevail, participated in 'The Parkinson's Experience' podcast hosted by 17 Branches to discuss the role of gene therapy in Parkinson's disease. Dr Lewis shared his expertise and insights, delving into what gene therapy is, how it sets itself apart from stem cell replacement, and its potential applications in the treatment of Parkinson's disease.

Parkinson's Foundation Research 101 Webinar

In November, Prevail sponsored a webinar as part of the Parkinson's Foundation 'Health @ Home' series, on the impact of clinical research on Parkinson's Disease and how it can enhance the lives of patients. The webinar outlined how patients can engage in clinical trials and contribute to groundbreaking research in the field. Prevail was honoured to support these critical conversations for advancing understanding of the role of clinical research in shaping outcomes for people living with Parkinson's Disease.

The Future of Parkinson's Disease 2023 Conference

Towards the end of 2023, Prevail attended the Future of Parkinson's Disease 2023 Conference in Austin, Texas. The conference was hosted by one of our advocacy partners, the Parkinson's Foundation, as well as the Parkinson Study Group, which provided a platform for leading investigators, coordinators, and advocates to exchange the latest advances in the treatment and management of Parkinson's disease.

[Gaucher Disease](#)

Behind the Mystery of Gaucher Disease

Prevail was fortunate to feature on an episode of Behind the Mystery, a recurring series on The Balancing Act devoted to advocating for rare and genetic diseases, to demystify Gaucher Disease. Teaming up with experts in patient advocacy, healthcare, and industry, this episode underscored the significance of early genetic testing and swift intervention. It delved into personalized treatment strategies alongside existing and upcoming therapies, including gene transfer treatments that have the potential to diminish the necessity for frequent interventions, ultimately enhancing the quality of life.

Lunch and Learn Gaucher Awareness Month

To commemorate Gaucher Awareness Month in November, Prevail welcomed Fan Ding to participate in a Lunch and Learn to share her remarkable story of living with Gaucher Disease. Fan's battle with Gaucher Disease began in China when she was diagnosed at the age of 2 and was told she wouldn't live past the age of 10. Through perseverance and endless support from her family, Fan became the first person in China to receive enzyme replacement therapy.

WORLDFair Patient and Healthcare Provider Meeting

In October, Prevail attended the WORLDFair Patient and Healthcare Provider Meeting held at the prestigious University of Minnesota. Dr. Sarah Neuhaus, Prevail's Senior Medical Director of Clinical Development, delivered a comprehensive presentation on the latest and groundbreaking treatments currently accessible for individuals living with Gaucher Disease. This meeting not only served as a platform for sharing insights into the most recent advancements in Gaucher Disease treatment, but also provided a valuable opportunity to discuss potential innovative treatments and technologies that can bring new hope to individuals affected by Gaucher Disease.

[Frontotemporal Dementia](#)

Association for Frontotemporal Dementia Conference

In May, Prevail actively participated in the Association for Frontotemporal Dementia Conference held in St. Louis, Missouri. This event provided a platform for Prevail to engage in collaborative discussions with peers, researchers, and advocates. It was a valuable opportunity for Prevail to demonstrate our dedication to advancing new therapies and enhancing quality of life for individuals living with frontotemporal dementia.

Frontotemporal Dementia Awareness Week

Prevail celebrated Frontotemporal Dementia Awareness Week in September which included a Lunch and Learn with Amanda Dawson, an advocate and frontotemporal dementia caregiver, who shared her experiences of caring for her father living with frontotemporal dementia. Prevail also participated in The Association for Frontotemporal Degeneration (AFTD)'s 11th Annual #FoodforThought campaign. This campaign encouraged team members to spend time with their loved ones by cooking recipes that support brain health and raise awareness of FTD.

Penn Frontotemporal Dementia Center Annual Caregiver Conference

In November, Prevail was honored to attend the Penn Frontotemporal Dementia (FTD) Center Annual Caregiver Conference in Philadelphia. The conference offered the remarkable opportunity to learn from leading experts in the fields of neuropsychology, clinical care, genetics, and cognitive neuroscience. The Penn FTD Center Annual Caregiver Conference provided Prevail with insights into the latest developments in FTD treatment innovation and caregiver strategies.

Prevail is privileged to facilitate conversations, collaboration, and co-creation with the key stakeholders who support our mission to produce new, innovative treatments across our pipeline. Such advancements would not be possible without the tireless work of the Prevail team, the selfless work of our advocacy partners, and the rare disease community whose perspectives and experiences illuminate our path towards finding a cure. Over the past year, we have deepened our understanding



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of patient perspectives, and we seek to continue our efforts to address the needs of patients, caregivers, and advocates in 2024.