

BROAD INSTITUTE OF MIT AND HARVARD IN COLLABORATION WITH  
THE TERMEER FOUNDATION PRESENT THE RARE DISEASE DAY EVENT

# Accelerating Rare Disease Research: Patients as Partners

MONDAY, FEBRUARY 27, 2023 FROM 1:30 - 5:30 PM ET  
STREAMED LIVE FROM THE BROAD INSTITUTE AUDITORIUM

## EVENT PROGRAM

- 1:30 PM**      **Invitation to the Broad Discovery Center Museum**
- 2:00 PM**      **Welcome Remarks**  
*Anna Greka* (Associate Professor, Harvard Medical School, Brigham and Women's Hospital;  
Institute Member, Director, Kidney Disease Initiative, Broad Institute)  
*Belinda Termeer* (President, Co-Founder, and Board Member of The Termeer Foundation)
- 2:15 PM**      **Spotlight on Mitochondrial Diseases**  
*Vamsi Mootha* (Professor, Harvard Medical School, HHMI Investigator; Institute Member, Broad Institute)
- 2:45 PM**      **Working Hand in Hand with Patient Foundations to Develop Therapies for Ultra-Rare Genetic  
Neurodevelopmental Disorders**  
*Yael Weiss* (CEO and Founder of Mahzi Therapeutics; Termeer Fellow, Class of 2022)
- 3:00 PM**      **From Rare Disease Parent to CEO: Gene Therapy for Dravet Syndrome**  
*Daniel Fischer* (CEO, President, and Co-Founder of Tevard Biosciences; Termeer Fellow, Class of 2022)
- 3:15 PM**      **Rare as One: Leveraging the Power of Patients to Accelerate Disease Research**  
*Tania Simoncelli* (Vice-President of Science and Society, Chan Zuckerberg Initiative)
- 3:25 PM**      **Inherited Retinal Degeneration: Therapeutic Landscape**  
*Eric Pierce* (Director, Harvard Ophthalmology Ocular Genomics Institute)
- 3:40 PM**      **A Panel Discussion: Partnering with Patients to Empower Progress**  
**Moderator:** *Samantha Baxter* (Associate Director, Genetic and Genomic Data Sharing, Genetic  
Counselor in the Program in Medical and Population Genetics, Broad Institute)  
**Panelists:** *Charlene Son Rigby* (CEO, Global Genes / RARE-X; Co-Founder, STXBP1 Foundation)  
*Eric Pierce* (Director, Harvard Ophthalmology Ocular Genomics Institute)  
*Daniel Fischer* (CEO, President, and Co-Founder of Tevard Biosciences; Termeer Fellow, Class of 2022)  
*Yael Weiss* (CEO and Founder, Mahzi Therapeutics; Termeer Fellow, Class of 2022)  
*Vamsi Mootha* (Professor, Harvard Medical School, HHMI Investigator; Institute Member, Broad Institute)  
*Tania Simoncelli* (Vice-President of Science and Society, Chan Zuckerberg Initiative)
- 4:15 PM**      **Plans for a Rare Disease Initiative at Broad | *Anna Greka***
- 4:30 PM**      **Networking in the Broad Discovery Center**

We are grateful to the Broad Institute of MIT and Harvard and The Termeer Foundation for hosting this event.