

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, RARE-X; Co-Founder, TXBP1 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.