

BROAD INSTITUTE OF MIT AND HARVARD IN COLLABORATION WITH THE TERMEER FOUNDATION PRESENT  
THE 9TH ANNUAL RARE DISEASE DAY EVENT

# Climbing Ladders to Cures in Rare Disease Research

WEDNESDAY, FEBRUARY 28, 2024 | 1:00 - 6:30 PM ET | Streamed Live 1:45 - 5:30 PM ET  
MERKIN AUDITORIUM | BROAD INSTITUTE OF MIT AND HARVARD | 415 MAIN STREET, CAMBRIDGE, MA 02142

## EVENT PROGRAM

- 1:00 PM**      **Invitation to the Broad Discovery Center Museum**
- 1:45 PM**      **Welcome Remarks**  
**Anna Greka** Harvard Medical School, Mass General Brigham;  
Kidney Disease Initiative, Ladders to Cures Accelerator, Broad Institute  
**Belinda Termeer** Termeer Foundation
- 2:00 PM**      **KEYNOTE | Scaling the Diagnosis of Rare Genetic Diseases and Readiness for Treatments**  
**Wendy Chung** Harvard Medical School; Department of Pediatrics, Boston Children's Hospital
- 2:30 PM**      **The Dawn of Interventional Genetics: Developing the Sustainable Practice of Treating Ultra Rare Genetic Diseases**  
**Winston Yan** N=1 Collaborative; Termeer Scholar 2023
- 2:50 PM**      **Lift Every Valley: A Nascent Journey in Duchenne Muscular Dystrophy**  
**Michelle Rengarajan** Harvard Medical School, Mass General Brigham
- 3:10 PM**      **Niche to NewCo: Lessons and Pearls for Building a Rare Disease Biotech**  
**Natalie Galant** Paradox Immunotherapeutics; Termeer Fellow 2023
- 3:30 PM**      **Inside the Orphan Drug Revolution**  
**Jim Geraghty** Biotech Entrepreneur and Board Chair
- 3:50 PM**      **Break**
- 4:00 PM**      **Using Population Data to Improve Variant Interpretation**  
**Kaitlin Samocha** Center for Genomic Medicine, Mass General Brigham; Broad Institute
- 4:20 PM**      **Finding Undiagnosed Patients and Launching Treatments: Probably Genetic's Platform**  
**Lukas Lange** Probably Genetic; Termeer Fellow 2022
- 4:40 PM**      **Exploring and Exploiting Human Virus-like Proteins for Genome Engineering**  
**Michael Segel** Zhang Lab, Broad Institute
- 5:00 PM**      **Buffalo Initiative: Enabling Lived Experience to Drive the Research Agenda**  
**Sunitha Malepati** CACNA1A Foundation; Buffalo Initiative
- 5:20 PM**      **Closing Remarks**
- 5:30 PM**      **Networking in the Broad Discovery Center**