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