



## Focus on Rare Disease: Prime time—gene therapy for rare disorders

**Wednesday, March 3**

**2:00 – 3:00 pm**

*Open to the Broad community and the general public.*

- 2:00 pm      **Welcome**  
*Anna Greka, Broad Institute, Brigham and Women's Hospital, Harvard Medical School*
- 2:05 pm      **Polr3a Neonatal Progeroid Syndrome: A Medical Mystery**  
*Leslie Macari, parents of rare disease patient*
- 2:15 pm      **Keynote Lecture**  
**Base Editing and Prime Editing: Genome Editing Without Double-Strand Breaks**  
*David Liu, Broad Institute, Harvard University, HHMI*
- 2:45 pm      **Q & A panel (all speakers)**  
Moderator: *Anna Greka, chair of Focus on Rare Disease*