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