

# Assessing Genetic Heterogeneity in the Context of Genome Editing Off-Targets in Gene Therapy Products Workshop

December 16, 2022

## *Tentative Agenda*

- 9:00 AM ET Introduction by FDA Moderator **-Dr. Jessica Chery**
- 9:05 AM ET Opening Remarks **-Dr. Peter Marks**
- 9:15 AM ET Progress in Genome Editing Technologies **-Dr. Matthew Porteus**
- 9:45 AM ET Human Genetic Variation **-Dr. Gilean McVean**
- 10:15 AM ET Implications of human genetic variation for the design of genomic medicines **-Dr. David Scott**
- 10:45 AM ET Break
- 11:00 AM ET In-silico and experimental analyses of off-target gene editing by engineered nucleases **-Dr. Gang Bao**
- 11:30 AM ET Defining impact of genetic variation on off-target activity of genome editors with sensitive and unbiased biochemical and cellular assays **-Dr. Shengdar Tsai**
- 12:00 PM ET Lunch break
- 1:00 PM ET Development of CRISPR Therapies for In Vivo and Ex Vivo Applications: gRNA specificity, off target identification and validation, and genotoxicity assessment Validation of Off-Target Events **-Dr. Laura Sepp-Lorenzino**
- 1:30 PM ET Leveraging iPSCORE – Understanding the Impact of Inherited Variants and Somatic Mutations on Human iPSC Cellular Traits **-Dr. Kelly Frazer**
- 2:00 PM ET Human genetic diversity alters off-target outcomes of therapeutic gene editing **-Dr. Luca Pinello**
- 2:30 PM ET Break
- 2:50 PM ET Panel Discussion and Q&A: Genetic Variation
- 3:50 PM ET Closing Remarks