Usher 1F Workshop
Friday May 12, 2017
W Hotel on the Boston Common

7:30 – 8:10 AM

8:10 AM

Breakfast

8:15 – 10:00 AM

8:15 – 8:30 AM (15 min)
Monte Westerfield, PhD (Univ. of Oregon)
How a zebrafish model of Usher Syndrome type 1F can help develop therapies for vision loss

8:30 – 8:45 AM (15 min)
Elliott Sohn, MD (Univ. of Iowa)
A comprehensive strategy for treating rare inherited retinal diseases like Usher 1F

8:45 – 9:00 AM (15 min)
Andrew Emili, PhD (Univ. of Toronto)
Identifying critical PCDH15 mRNA isoforms

9:00 – 9:15 AM (15 min)
Zubair Ahmed, PhD (Univ. of Maryland)
Development and initial phenotyping of PCDH15 knockin mice, a mammalian model to validate therapies for hearing and vision loss

9:15 – 9:45 AM

Roundtable
Moderator: Stephen Rose, PhD (Foundation Fighting Blindness)

9:45 – 10:00 AM

Break

10:00 – 10:30 AM (30 min)
Philip Reilly, MD, JD (Author, Orphan)
Bringing therapies to the clinic for rare genetic diseases

10:45 – 12:30 AM

10:45 – 11:05 AM (20 min)
Chad Cowan, PhD (Harvard, HSCI)
Challenges in the clinical translation of stem cell technology

11:15 – 11:35 AM (20 min)
Derrick Rossi, PhD (Harvard, BCH)
New opportunities in RNA technology for disease treatment

11:45 – 12:05 AM (20 min)
David Liu, PhD (Broad Institute)
Overcoming translational hurdles related to CRISPR technology

12:05 – 12:45 AM

Roundtable
Moderator: Andrew Emili, Ph.D.

12:45 – 2:00 PM

Lunch