Usher 1F Workshop

Friday May 12, 2017
W Hotel on the Boston Common

7:30 – 8:10 AM	**Breakfast**
8:10 AM	**Introduction (Elliot Chaikof, MD, PhD)**
8:15 – 10:00 AM	**Session 1: Progress on Usher Syndrome type 1F**
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	**Session 2: New Tools for the Treatment of Genetic Diseases**
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**