

## Foundation Fighting Blindness Usher 1B Virtual Workshop

Monday, September 13, 2021, 9:00 am – 5:00 pm CST

### Agenda

#### Schedule:

- 9:00 am: Welcome (Ben Yerxa, José-Alain Sahel, Shannon Boye)
- 9:10 am: Survey Outcome and Patient Perspectives
  - Todd Durham *10 mins*
  - Patient Advocate *20 mins*
  - Q&A *10 mins*
- 9:50 am: Basic Science (genetics, pathology of disease, disease models), **Moderator: Isabelle Audo**
  - Claire Gelfman, “USH1B: MYO7A Gene Description and Genetics” *15 mins*
  - Hannah Nonarath, “Investigating the pathophysiology of USH1B in a zebrafish model” *15 mins*
  - Martha Neuringer, “Toward a nonhuman primate model of USH1B.” *15 mins*
  - Aziz El Amraoui, “Modeling Usher 1B pathogenesis: Which readouts are pertinent for therapeutic beneficial outcomes?” *15 mins*
  - Q&A *15 mins*

*11:05 am Break: 20 minutes*
- 11:25 am: Translational Science, **Moderator: Claire Gelfman**
  - David Williams, “Modeling USH1B” *15 mins*
  - David Gamm, “Using human pluripotent stem cells as tools and treatments for USH1B” *15 mins*
  - Q&A *10 mins*
- 12:05 pm Clinical Course & Outcome Measure Development, **Moderator: José-Alain Sahel**

- Isabelle Audo, “Phenotypic characteristics associated with MYO7A mutations, a cohort study” *15 mins*
- Mark Pennesi, “Development of Endpoints for Clinical Trials” *15 mins*
- Todd Durham, “Insights from Published Natural History Studies of Usher Syndrome Type 1B.” *15 mins*
- Jacque Duncan, “High resolution retinal imaging in USH1B related retinal degeneration” *15 mins*  
Q&A *15 mins*

*1:20 pm Break/Lunch: 40 minutes*

- 2:00 pm Therapeutic Approaches, **Moderator: David Gamm**
  - Alberto Auricchio, “Expanding AAV transfer capacity in the retina” *15 mins*
  - Shannon Boye, “Development of a dual AAV vector- based gene therapy for MYO7A associated USH1B” *15 mins*
  - Serge Picaud, “Light toxicity and mechanosensitivity of photoreceptors for assessing therapies.” *15 mins*  
Q&A *10 mins*

*2:55 pm Break: 10 minutes*

- 3:05 pm Emerging Therapies Roundtable, **Moderator: Rusty Kelley**
  - Atsena (Patrick Ritschel) *10 mins*
  - Nacuity (Halden Conner/Jami Kern) *10 mins*
  - Eloxx (Sumit Aggarwal/Vijay Modur) *10 mins*
  - ProQR (Aniz Girach) *10 mins*  
Q&A *20 mins*
- 4:05 pm Summary and Next Steps
  - Shannon Boye *15 mins*
  - Patient Perspective Recap *20 mins*
- 4:45 pm Final Comments
- 5:00 pm Workshop Concludes