

Usher1B Workshop, September 12-13, 2021

Grand Hyatt DFW at the Dallas/Fort Worth International Airport

Introduction

The urgent mission of the Foundation Fighting Blindness is to support the development of treatments and cures for inherited retinal dystrophies, including Usher syndrome, which is the most common genetic cause of combined deafness and blindness. There are three different types of Usher syndrome, defined by its severity, each having subtypes based on the genetic cause. Although individuals with Usher type 1 demonstrate similar symptoms, the genetic causes differ. Usher 1B is the most common form of Usher type 1 and accounts for 40% of all cases. There are currently no treatments for USH1B.

This Workshop is designed to bring together leading experts from academia and industry, to discuss openly, and in detail, what is known about Usher 1B disease pathology, disease models, clinical characteristics and therapeutic approaches. The agenda will also include a roundtable on emerging therapies with representatives from industry. Importantly, we will also hear from affected individuals and their families in order to ensure their perspectives for care, treatment and therapies inform both research and clinical studies.

Workshop Format

The Workshop will be held on September 12-13, 2021 at the **Grand Hyatt DFW at the Dallas/Fort Worth International Airport**— conveniently located inside of Terminal D. This day and a half meeting will be co-chaired by Dr. Shannon Boye and Professor José-Alain Sahel, leaders in the USH1B field.

Given the current travel and gathering size restrictions due to COVID-19, we have decided to hold the Workshop in a **hybrid in-person and virtual format**. The current gathering size is limited for the number of in-person attendees, but the cap may lift in the coming months. If the cap lifts,

additional registrants may have the option of attending in person (up to a certain number, depending on current restrictions) or virtually.

Due to potential size restrictions, In-person attendees will be invited by Foundation staff and contacted by email about hotel arrangements. If you would like to opt for in-person attendance, please let us know and we will reach out as space is available.

If you have any questions about the workshop registration, please email lkenney@fightingblindness.org .

Workshop Goals

The goals of the Workshop are to review the Usher 1B landscape, energize the clinical and research community through shared learnings, and identify knowledge gaps for future work. Importantly, the Foundation seeks to understand how we can facilitate next steps. We also look forward to hearing from affected individuals and patient advocates.

Virtual Workshop Registration

Register in advance for this webinar:

https://fightingblindness-org.zoom.us/webinar/register/WN_EKR_iaT9SAur6Y1LVKMDKA

In-person attendance is invite-only due to restrictions on gathering size. We are encouraging those unable to attend in-person to register for the webinar and take part virtually. After registering, you will receive a confirmation email containing information about joining the webinar.

Workshop Agenda

Use this [link](#) to access the current agenda.

Workshop Presenters

- Shannon Boye, PhD
- José-Alain Sahel, MD
- David Williams, PhD
- Martha Neuringer, PhD
- Aziz El-Amraoui, PhD
- Jacque Duncan, MD
- Joe Carroll, PhD
- David Gamm, MD, PhD
- Mark Pennesi, MD, PhD
- Isabelle Audo, MS, PhD
- Alberto Auricchio, MD
- Serge Picaud, PhD
- Hannah Nonarath, BS, MS
- Martha Neuringer, PhD
- Atsena (Patrick Ritschel, MBA)
- Nacuity (Halden Conner, MBA/Jami Kern, MBA, PhD)
- Eloxx (Sumit Aggarwal, MBA/Vijay Modur, PhD)
- ProQR (Aniz Girach, MD)

FFB Staff

- Ben Yerxa, PhD
- Claire Gelfman, PhD
- Rusty Kelley, MBA, PhD
- Todd Durham, PhD
- Amy Laster, PhD
- Chad Jackson, PhD
- Ben Shaberman
- Leilla Kenney