

LCA Family Conference

Saturday, October 6, 9 a.m. to 3 p.m.

Mystic Marriott Hotel & Spa

9:00 – 9:45 a.m. *Welcome and Opening Remarks*

9:45 – 10:15 a.m. *IRD Milestones: Reasons to Be Excited*

At no time in history has there been more promising research applied to genetically inherited eye disease. Hear from our partners at the Foundation Fighting Blindness about how much easier it is today to access genetic testing and genetic counseling and the exciting research and trials that are underway.

10:15 – 10:30 a.m. BREAK

10:30 – 11:15 a.m. *The Road to Treatment: Understanding How Therapies Get Developed*

From research to FDA approval, what does it take to develop and approve a new treatment for rare disease? We'll explore the regulatory, clinical and industry aspects so you have a deeper understanding of what is involved in developing treatments, including how rare disease is different.

11:15 a.m. – 12: 15 p.m. *What Can You Do? Patient Role in Rare Disease Advocacy*

The very nature of rare diseases puts patients at the center of research, awareness and clinical development. Those who develop therapies face significant challenges to find patients, conduct clinical trials, and develop financially viable therapies. Patient advocacy programs are the essential bridge between research and patients. We'll speak with the top patient advocacy professionals in the industry. We'll explore how patients, caregivers, and the rare disease community can support research and the role they play in more effectively bringing treatments to market.

12:15 – 1:30 p.m. *Lunch*

1:30 – 2:30 p.m. *Living with LCA*

Hear first-hand from parents who have raised children with LCA and from those living with e LCA. This is a guided group discussion session will be your opportunity to speak directly with those who have been living with LCA for years. You'll get important tips and understanding of how to manage a growing LCA family.

2:30 – 2:45 p.m. *Closing Remarks*