

AGENDA

FRIDAY, OCTOBER 5

6:00 PM–8:00 PM

Welcome Reception

Dinner on the Patio

Location: Outside Patio, Octagon Restaurant, Mystic Marriott

Dinner, drinks, and an opportunity to meet the Sofia Sees Hope team, sponsors, and conference participants.

SATURDAY, OCTOBER 6 LCA FAMILY CONFERENCE

Location: Mystic Ballroom

8:30 AM

Mystic Aquarium

The bus leaves and returns from the north entrance of the hotel.

9:00 AM–9:15 AM

Welcome and Opening Remarks

- **LAURA MANFRE**,
Board Chair & Co-Founder, Sofia Sees Hope
- **ANNETTE TONTI**,
Executive Director, Sofia Sees Hope

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9:15 AM–10:00 AM

IRD Milestones: Reasons to Be Excited

Dr. Brian Mansfield, Senior Vice President of Research, Foundation Fighting Blindness

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.

10:00 AM–11:00 AM

The Road to Treatment: Understanding How Therapies Are Developed

MODERATOR:

JEFFREY FINMAN, *Jupiter Point Pharma Consulting, LLC, Board Member, Sofia Sees Hope*

PANELISTS:

- **DR. WILEY CHAMBERS**, *Supervisory Medical Officer in the Office of New Drugs at the FDA*
- **JENNIFER HUNT**, *Vice President of Clinical Operations, Editas Medicine*
- **TAMI MOREHOUSE**, *Phase 1 RPE65 Trial Subject RPE65 genetic therapy trial*

From research to federal 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.

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11:00 AM–11:15 AM • COFFEE BREAK

11:15 AM–12:15 PM

The Role of the Patient Voice in Developing Treatments for Rare Disease

MODERATOR:

ANNETTE TONTI,

Executive Director, Sofia Sees Hope

PANELISTS:

- **JAMIE RING,**
Head of Patient Advocacy, Spark Therapeutics
- **JILL DOLGIN,**
Head of Patient Advocacy, AGTC
- **KRISTEN ANGELL,**
Associate Director, Advocacy, National Organization for Rare Disorders (NORD)

The very nature of rare disease puts patients at the center of research, disease awareness, and clinical development. Patient advocacy organizations and rare disease families are often the essential bridge between research and patients. This panel will explore the role of patient advocacy from multiple perspectives including two industry experts and NORD, the largest rare disease alliance organization in the country. We'll explore how patients, caregivers, and the rare disease community can support research, the role they play in more effectively bringing treatments to those who may benefit from them, and involvement in policy and patient-engagement programs.

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12:15 PM–1:15 PM

LUNCH BREAK

Take this time to visit our sponsors, meet new people & enjoy lunch!

1:15 PM–1:30 PM

Conference Sessions Wrap-Up

1:30 PM–2:30 PM

Living with an IRD

(Session open to IRD Families only)

Location: Conference Room 2

DISCUSSION LEADERS:

- **BETH BORYSEWICZ,**
TVI for State of Connecticut and Sofia Sees Hope Board Director
- **MICHAEL & SELA CORNELL,**
Father and daughter from Chicago

Hear first-hand from a Teacher of the Visually Impaired (TVI), caregivers, and individuals living with LCA and other rare inherited retinal diseases. This is a group discussion and will be your opportunity to ask questions and discuss issues impacting those who live and care for others with retinal disease.

2:45 PM–3:00 PM

Bus returns from Aquarium