

Alliance to Cure Cavernous Malformation  
Family Conference 2023

July 7-8, 2023

**Friday, July 7th**

7:00 am Breakfast, Registration, Visit Posters, Check-in kids & teens.

With Scientists:

8:00 am Welcome, Alliance News, and Keynote Introduction – Connie Lee, PsyD

8:10 am Keynote: Chris Gibson, MD, PhD, Recursion Pharmaceuticals. *Revolutionizing Drug Development: Insights from the Past, Visions for the Future*

9:10 am *Adding Patient & Clinician Priorities to the Research Agenda: Meaningful Clinical Endpoints, Tolerable Adverse Effects, and Routes of Administration* – Connie Lee

9:30 am Panel Discussion: *Translating Patient/Clinician Priorities to a Research Agenda & Trials* – Patient representatives, Ed Smith, Kelly Flemming, Doug Marchuk, Connie Lee

10:00 am Break with Scientists and move to Patient Meeting Room (Trade Room)

With Patients:

10:30 am Introductions – Family conference attendees introduce themselves.

11:00 am *Dr. Gary Steinberg and Dr. Kelly Flemming Answer Your Questions.* Drs. Steinberg and Flemming will participate in a moderated Q&A, answering pre-submitted questions and questions from family conference attendees. Dr. Steinberg is widely acclaimed as a national CCM neurosurgery expert and is the Medical Director of the CCM Center of Excellence at Stanford University. Dr. Flemming is a stroke neurologist, Co-Director of the CCM Center of Excellence at Mayo Clinic in Rochester, MN, and Vice Chair of the Alliance to Cure Medical Advisory Board.

12:00 pm Lunch with Scientists

1:15 pm Dr. Ed Smith, Boston Children's Hospital – *What Good Care Looks Like: The Center of Excellence Model.* How do you know you're receiving good care and comprehensive services? Dr. Smith will share information and a behind-the-scenes look at the Boston Children's Hospital CCM Center of Excellence program to set the bar.

Research Reports

2:00 pm Delaney Fisher, UVA - *Focused Ultrasound Blood-Brain Barrier Opening Controls CCM Growth and Can Reduce De Novo Cavernoma Formation*

2:20 pm Miguel Lopez-Ramirez, UCSD – *Mechanisms of Neuro-Inflammation in CCM*

2:40 pm Anika Varma, CHET – *CCM-Health Index Longitudinal Study Results*

3:00 pm Break with Scientists

- 3:30 pm Dr. Helen Kim – *The Brain Vascular Malformations Consortium: 14 Years of CCM Research*
- 4:00 pm *Facilitated Small Group Discussions*: we will break into conversation groups of 8-10 to discuss the day's presentations and how they relate to us. For the last 15 minutes, we'll regroup, summarize our learnings, and Alliance to Cure staff will answer questions.
- 5:00 pm End of Day
- Dinner There are no formal dinner plans because patient conference attendees frequently do not have the stamina to continue beyond 5 pm, or they have alternate plans. However, Alliance to Cure staff will be available for a self-pay dinner at a nearby restaurant for those interested.

### **Saturday, July 8th**

- 7:30 am Breakfast and socializing. Check-in kids and teens.
- 9:00 am Welcome
- 9:10 am Dr. Jacques Morcos, Director of Skull Base and Cerebrovascular Surgery, University of Miami. President-elect of the American Association of Neurological Surgeons – *CCM Clinical Decision-Making*
- 10:10 am Break
- 10:30 am Panel: Connie Lee, Jianbo Hu, Holly Blei – *Getting to a Cure, Part 1: A discussion of research initiatives inside and outside of the Alliance to Cure.*
- 11:30 am Darla Clayton – *Coping and Cognitive Rehabilitation*
- 12:00 pm Lunch
- 1:15 pm Panel: Lindsay Ramirez, Linda Fuchser, Darla Clayton, Jessica Biggs, Jeia Rutland Simpson, Michelle Crook, Connie Lee – *Getting to a Cure, Part 2: A discussion of patient care and involvement.*
- 2:00 pm Small Group Discussion – our final facilitated small group discussion breakouts will allow you to choose between Nutrition, Rehabilitation, Parenting, Treatment Decision-Making, and Advocacy. You will have a chance to participate in more than one group.
- 3:30 pm Closing Words and End of Meeting