

ALLIANCE TO CURE

CAVERNOUS MALFORMATION



Agenda

19th Annual International CCM Scientific Meeting

Intercontinental Hotel
Miami, Florida
July 6-7, 2023



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Recursion

2023 International CCM Scientific Meeting Agenda

Privacy Policy

We would like to reiterate to everyone the “workshop” nature of this very special gathering and the unique multidisciplinary opportunity to cross-germinate ideas and form collaborations. A community of trust and respect for our privacy policy is essential to the success of the CCM Scientific Meeting. To maintain privacy and encourage the presentation and discussion of unpublished data, the Alliance to Cure Cavernous Malformation does not publicize written abstracts, and it is not permissible to copy or disseminate unpublished data or results presented in presentation videos or at the live meeting.

Violations to the privacy policy will be reviewed by the organizing committee and will result in denied Meeting attendance for at least one year.

Thursday, July 6th

7:00 Registration - Sevilla
Breakfast – Brickell



PLENARY – Sevilla

8:00 Welcome – Connie Lee, Alliance to Cure Cavernous Malformation CEO

Session I – Genetic Therapy and Genetic Modifiers

8:10 Introduction to Session – Doug Marchuk, Duke University

8:15 Keynote: Gene Therapy – Aravind Asokan, Duke University

9:15 Study design for gene-replacement therapy using a novel AAV vector in a mouse model of CCM3 – Doug Marchuk

9:30 Whole gene and sup-tRNA gene therapy for CCM – Angela Glading, University of Rochester

9:45 Panel & Audience Discussion – Aravind Asokan, Doug Marchuk, Angela Glading, Ellie Chilcott (University College London)

10:00 Break

Session II – Surgery and Patient-Reported Outcomes

10:25 Outcomes of Brainstem Cavernous Malformation Resection, with and without use of flexible omnidirectional carbon dioxide laser: A single surgeon series of 279 surgeries – Gary Steinberg, Stanford University

10:45 Predicting Long-Term Functional and Cranial Nerve Outcomes in Surgically Treated Brainstem Cavernous Malformations: A Single Surgeon’s Experience – Eva Wu

11:00 Association of Quality-of-Life Domains and Clinical Symptoms in Pediatric Cerebral Cavernous Malformation Patients – Shweta Chawla, UCSF

11:15 A 12-Month Longitudinal Study of Disease Progression in Cerebral Cavernous Malformation – Anika Varma, University of Rochester

11:35 Panel & Audience Discussion: Gary Steinberg, Eva Wu, Shweta Chawla, Anika Varma, Kelly Flemming (Mayo Clinic)

11:50 LUNCH – Brickell

Session III – Disease Models

1:00 Introduction – Brent Derry, Sick kids Hospital, Toronto

1:10 Keynote: Developing New Models of Disease – Josef Penninger, University of British Columbia

2:10 Model and target cerebral cavernous malformation in organoids – Ziyuan Guo, Cincinnati Children's Hospital and Medical Center

2:30 A novel pathogenic microdeletion in exon 11 of the mouse Krit1 gene – Petr Tvrdik, University of Virginia

2:50 Panel and Audience Discussion – Doug Marchuk, Brent Derry, Ziyuan Guo, Petr Tvrdik, (Josef Penninger)

3:20 Group Photo – Location TBA

4:00 POSTER SESSION AND RECEPTION – Bayfront

Research Posters

- *Minimally invasive resection of deep-seated cavernomas using tubular retractors* – Joshua Burks, University of Miami
- *Integration of CCM Signaling Pathways into a Logic-Based Computational Model of Brain Endothelial Cells* – Tanya Cruz, University of Virginia
- *Cerebral Cavernous Malformation Surgeries at the University of California San Francisco, 2018-Present* – Mateo Ortiz Ambrosia, UCSF
- *Prognostic and Etiological Biomarkers for familial Cerebral Cavernous Malformations (fCCM)* – Jacob Croft, TTUHSC El Paso
- *Modeling Human Blood-Brain Barrier Formation and Cerebral Cavernous Malformations in Blood-Brain Barrier Assembloids* – Lan Dao, Cincinnati Children's Hospital Medical Center
- *Tozuleristide has an affinity to mouse cerebral cavernous malformations* – Khadijeh Sharifi, University of Virginia

Patient Posters

Patient Posters share stories and images of the patients who will be attending the family conference.

Allison; Ben & Mary Ellen; Billie; Darla, Trent & Ama; David; Elan; Hanna; Julia; Kandie; Keira; Linda; Linnea; Rachel; Ross; Shannon; Vanessa; Walter;

7:00 DINNER - Olé Restaurant at the Intercontinental

Friday, July 7th

7:00 Breakfast – Brickell

PLENARY – Sevilla

8:00 Welcome – Connie Lee, Alliance to Cure Cavernous Malformation CEO

8:10 Keynote Address to Patients, Families, and Investigators: Revolutionizing Drug Development: Insights from the Past, Visions for the Future – Christopher Gibson, Recursion Pharmaceuticals

Session IV Patient/Clinician Priorities and the Research Roadmap

9:10 Patient & Clinician Priorities: Meaningful Clinical Endpoints, Tolerable Adverse Effects, and Routes of Administration – Connie Lee, Alliance to Cure Cavernous Malformation

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

10:00 Break with Family Conference attendees

Session V Mechanisms and Novel Treatment

10:30 Focused Ultrasound Blood-Brain Barrier Opening Controls Cerebral Cavernous Malformation Growth and Can Reduce De Novo Cavernoma Formation – Delaney Fisher, University of Virginia

10:50 Mechanisms of neuroinflammation in cerebral cavernous malformation disease – Miguel Lopez-Ramirez, UCSD

11:10 Genetic variation in mTOR pathway genes and risk of earlier seizure onset in familial Cerebral Cavernous Malformation – Mateo Ortiz Ambrosio, UCSF

11:30 Panel and Audience Discussion: Angela Glading, Richard Price, Delaney Fisher, Miguel Lopez Ramirez, Mateo Ortiz Ambrosio

12:00 LUNCH with Family Conference Attendees

Session VI Pathogenesis and Biomarkers

1:30 A proximity-labeling approach to identify functional interactors of KRIT1 – Andrea Perrelli, University of Rochester

1:50 Somatic variants of MAP3K3 are sufficient to cause cerebral and spinal cord cavernous malformations – Jian Ren, Xuanwu Hospital, Beijing

2:10 Identification of circulating epigenetic biomarkers for lesion burden by whole-genome methylome profiling in familial CCM – Shantel Weinsheimer, UCSF

2:30 Panel and Audience Discussion: Angeliki Louvi, Andrea Perrelli, Jian Ren, Shantel Weinsheimer

2:50 Closing Remarks – Doug Marchuk, Brent Derry, Ed Smith, Kelly Flemming

3:00 Break with Family Conference attendees

3:15 End of meeting



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