



2025 International TSC Research Conference Agenda

June 26 Day One, Thursday — Grand Ballroom E	
2:00 pm - 2:15 pm	Welcome and Opening Remarks: Kari Luther Rosbeck, President & CEO, TSC Alliance TSC Community Speaker: Guinevere Pandolfi
Plenary Session 1: Translational Research in LAM, Renal Disease, and Topics Relevant to Adult Care in TSC Moderators: Kate Richardson, MS, and Carmen Priolo, MD, PhD	
2:15 pm - 2:45 pm	William Stanford, PhD, Ottawa Hospital Research Institute – Organotypic Disease Modeling and Therapeutic Development for Renal TSC and LAM
2:45 pm - 3:00 pm	Yan Tang, PhD, Brigham and Women's Hospital – Gene Replacement Therapy for Pulmonary Lymphangioleiomyomatosis
3:00 pm - 3:15 pm	Flaviane Silva, PhD, University of Massachusetts Chan Medical School – The Nutrient Sensing mTOR Pathway in Renal Cystogenesis
3:15 - 3:45 pm	Simon Johnson, DM FCRP FERS, University of Nottingham – mTOR Driven Alveolar Type 2 Cell Senescence Increases Lung Damage in LAM
3:45 pm - 4:00 pm	Manoocher Soleimani, MD, University of New Mexico – The Critical Role of the Proto-Oncogene c-KIT in Kidney Cystogenesis in Tuberous Sclerosis Complex (TSC)
4:00 pm - 4:30 pm	Break – Foyer E-H
4:30 pm - 5:00 pm	Lara Friel, MD, PhD, University of Texas Health at Houston – Considerations for the Management of Pregnant Persons with Tuberous Sclerosis Complex and Sporadic Lymphangioleiomyomatosis: An Update from the Reproductive Health Initiative Taskforce and A Call to Action
5:00 pm - 5:15 pm	David Ritter, MD, PhD, Cincinnati Children's Hospital – Patient Reported Pregnancy Outcomes in Women with Tuberous Sclerosis Complex Shows High Rates of Preeclampsia
5:15 pm - 5:30 pm	Diego Maciel-Lima, MSc, Federal University of Parana – Career Guidance Program for People with TSC: Single Case Experimental Design
5:30 pm - 5:45 pm	Laura Gorecki, MS, University of Texas Health, Houston – Influential Factors for Disclosing a Tuberous Sclerosis Complex Diagnosis to Romantic Partners
5:45 pm - 6:00 pm	Amber Goedken, PharmD, PhD, University of Iowa – Women's Health Population-Based Assessment
Close of Plenary Sessions for Day 1	
6:00 pm - 7:00 pm	Welcome Reception – Foyer E-H
7:00 pm – 9:00 pm	Dinner and Keynote Presentation – White Oak Emcee: Kari Rosbeck, President & CEO, TSC Alliance Presentation of the Vicky H. Whittemore Travel Awards: Steven L. Roberds, PhD, TSC Alliance Presentation of the Manuel R. Gomez Award: Shafali Spurling Jeste, MD, Children's Hospital, Los Angeles Keynote Presentation: Jeffrey Karp, BEng PhD, Brigham and Women's Hospital <i>Introduced by Carmen Priolo, MD, PhD</i>



June 27 Day Two, Friday – Grand Ballroom E

7:30 am - 8:30 am Breakfast – White Oak

Plenary Session 2: Research to Address Challenges of Controlling Epilepsy in TSC

Moderators: Howard Weiner, MD, and Kimberly Raab-Graham, PhD

8:30 am - 8:45 am **Welcome and Introduction:** Steven L. Roberds, PhD, Chief Scientific Officer, TSC Alliance
TSC Community Speaker: Jessica and Orlando Crooks

8:45 am - 9:15 am Lisa Julian, PhD, Simon Fraser University – **Human Stem Cell Models Reveal Proteostasis, Cell Fate and Structural Alterations in the Early TSC2-/- Neuroepithelium**

9:15 am - 9:45 am Darcy Krueger, MD, PhD, Cincinnati Children's Hospital – **Towards More Effective Use of Everolimus and Sirolimus in the Treatment of Epilepsy in Tuberous Sclerosis Complex and Smith-Kingsmore Syndrome**

9:45 am - 10:00 am Zin-Juan Klaft, MD, Tufts University – **Tuberin Replacement Therapy in Cultured, Patient-Derived Neural Progenitor Cells and a Cerebral in Vivo Mouse Model of Tuberous Sclerosis Complex Type 2**

10:00 am - 10:30 am **Break – Foyer E-H**

10:30 am - 11:00 am Eleonora Aronica, MD, PhD, University of Amsterdam – **Epileptogenic Networks in Tuberous Sclerosis Complex: From Tissue to Molecular and Network Alterations**

11:00 am - 11:15 am Nina Gruetzmacher, PhD, Université Paris-Cité – **Inhibition of the Potassium Channel TREK1 by mTOR: A Mechanism for Aberrant Neuronal Excitability in TSC**

11:15 am - 11:30 am S. Katie Ihnen, MD, PhD, Cincinnati Children's Hospital – **Electrical Stimulation for Seizure Induction in Children with Tuberous Sclerosis Complex (TSC) and Drug-Refractory Epilepsy (DRE)**

11:30 am - 12:00 pm Darcy Krueger, MD, PhD, Cincinnati Children's Hospital – **Efficacy and Safety of Vigabatrin: Updated Perspectives for Use in TSC**
Q&A alongside Elizabeth Thiele, MD, PhD & Priyamvada Tatachar, MD

12:00 pm - 1:00 pm **Lunch – White Oak**

Mini-Symposia and Discussion Sessions

1:00 pm - 2:45 pm **TSC-Associated Neuropsychiatric Disorders** – Grand Ballroom F
Session Co-Chairs: Jamie Capal, MD, University of Rochester Medical Center, and Anna Jansen, MD, PhD, Antwerp University Hospital

1:00 pm - 2:45 pm **TSC Disease Mechanisms and Genetics** – Glen Echo
Session Co-Chairs: Laura Farach, MD, UTHealth Houston, and Lisa Julian, PhD, Simon Fraser University

1:00 pm - 2:45 pm **Lymphangiomyomatosis (LAM) and Angiomyolipomas in TSC** – Forest Glen
Session Co-Chairs: Nishant Gupta, MD, Cincinnati Children's Hospital, and Lyndsay Harshman, MD, University of Iowa

2:45 pm - 3:15 pm **Break – Foyer E-H**

3:15 pm - 5:00 pm Mini-Symposia Resume

5:00 pm - 8:00 pm **Poster Session & Reception – Grand Ballrooms G & H**



June 28 Day Three, Saturday – Grand Ballroom E	
7:30 am - 8:30 am	Breakfast – White Oak
Plenary Session 3: Impacting TAND Through Basic, Translational, and Clinical Research – Grand Ballroom E Moderators: Rajsekar Rajaraman, MD, and Shafali Jeste, MD	
8:30 am - 8:45 am	Welcome and Introduction: Dean J. Aguiar, PHD, Vice President, Translational Research, TSC Alliance TSC Community Speaker: Henrik Bergström, TSC Sverige
8:45 am - 9:15 am	Mustafa Sahin, MD PhD, Boston Children's Hospital – Investigating TAND and Epilepsy Using Human Neurons
9:15 am - 9:30 am	Kirstin Risgaard, MS, University of Texas Health, Houston – The Relationship Between Sleep and Tuberous Sclerosis Complex in Adults
9:30 am - 10:00 am	Jamie Capal, MD, University of Rochester Medical Center – Regulating Together in Tuberous Sclerosis Complex: A Pilot Feasibility Study in Children and Adolescents with TSC-Associated Neuropsychiatric Disorder (TAND)
10:00 am - 10:30 am	Break – Foyer E-H
10:30 am - 10:45 am	Nola Chambers, PhD, University of Cape Town – Validation of TAND-SQ Cluster and Total Severity Scores
10:45 am - 11:00 am	Kate Fifield, BSc, King's College London – Developing Smartphone EMA for Young Adults with Tuberous Sclerosis Complex (TSC): A Qualitative Analysis and Reflections of an Advisory Group
11:00 am - 11:30 am	Elizabeth Thiele, MD PhD, Massachusetts General Hospital – Neurobehavioral Aspects of TSC: Herscot Center 20-Year Experience
11:30 am - 11:45 am	Nicole McDonald, PhD, University of California, Los Angeles – Parent-Mediated Intervention for Behavior Problems in TSC: Initial Findings from the RAINBOW Trial
12:00 pm - 1:00 pm	Lunch – White Oak
12:00 pm - 1:00 pm	TSC Preclinical Consortium Meeting (all welcome) – Glen Echo
Plenary Session 4: Insights into TSC Biology from Basic Science, Cell Biology, Immunology, and Neuroscience – Grand Ballroom E Moderators: Uchenna Unachukwu, PhD, and Nicola Alesi, MD, PhD	
1:00 pm - 1:15 pm	Rebecca Ihrie, PhD, University of Colorado – Epigenetic Alterations in TSC2-Mutant Models of Brain Development
1:15 pm - 1:45 pm	Elizabeth Henske, MD, Brigham and Women's Hospital – Targeting T Cell Dysfunction and the Immune Microenvironment in TSC and LAM
1:45 pm - 2:15 pm	Mini-Symposia Reports
2:15 pm - 2:45 pm	Break – Foyer E-H
2:45 pm - 3:15 pm	Brendan Manning, PhD, Harvard T.H. Chan School of Public Health – Metabolic Effects of mTOR in Tumors, the Tumor Microenvironment, and Host
3:15 pm - 3:30 pm	Weibo Niu, PhD, Emory University – Integrated Multi-omics Reveals mTOR-LPL-Driven Dysregulated Lipid Metabolism in Human Microglia of Tuberous Sclerosis Complex
3:30 pm - 4:00 pm	Peter Crino, MD PhD, University of Maryland School of Medicine – Understanding the Epileptic Network in TSC