



2019 INTERNATIONAL TSC RESEARCH CONFERENCE AGENDA

JUNE 20 DAY ONE, THURSDAY

2:00-6:00 pm	Welcome and Plenary Session 1 (<i>Churchill Ballroom</i>) Session Chairs: Steven L. Roberds, PhD; Elizabeth P. Henske, MD
2:00-2:15 pm	Opening Remarks and TSC Community Speaker – Karen Shulist, TS Canada ST, and introducing Katie Duffy
2:15-2:45 pm	Arnold Kristof, MDCM, FRCPC, McGill University – Research priorities for lymphangioleiomyomatosis
2:45-3:15 pm	Thomas N. Darling, MD, PhD, Uniformed Services University of the Health Sciences – What the skin is telling us about TSC
3:15-3:45 pm	Carmen Priolo, MD, PhD, Brigham and Women's Hospital – Understanding TSC metabolic reprogramming through molecular imaging
3:45-4:00 pm	Aristotelis Astreinidis, PhD, University of Tennessee – EMT as a driver of rapalog non-responsiveness in TSC
4:00-4:30 pm	BREAK (<i>Churchill Court</i>)
4:30-5:00 pm	Sergiusz Józwiak, MD, PhD, Children's Memorial Health Institute – Epileptogenesis in TSC – lessons learnt from the EPISTOP study
5:00-5:30 pm	Kevin C. Ess, MD, PhD, Vanderbilt University – Cell autonomous and non-cell autonomous abnormalities from TSC patient derived stem cells
5:30-5:45 pm	Peter Davis, MD, Boston Children's Hospital – Clinical features and functional connectivity measures associated with epilepsy risk in infants with tuberous sclerosis
5:45-6:00 pm	John Blair, MSc, University California, Berkeley – Single cell transcriptomics of a human brain organoid model of tuberous sclerosis
6:00-8:45 pm	Reception and Dinner (<i>Mountbatten Lane & Salon</i>) Emcee – Kari Luther Rosbeck, TS Alliance Presentation of the Vicky H. Whittemore Travel Awards – Elizabeth P. Henske, MD, Brigham and Women's Hospital and Michael Wong, MD, PhD, Washington University Presentation of Manuel R. Gomez Award – Doug Loftus Keynote Address – David N. Franz, MD, Cincinnati Children's Hospital – Tuberous sclerosis complex: treating an "untreatable" disease

JUNE 21 DAY TWO, FRIDAY

8:00-11:45 am	Plenary Session 2 (<i>Churchill Ballroom</i>) Session Chairs: Michael Wong, MD, PhD; Carmen Priolo, MD, PhD
8:00-8:15 am	Community Speaker – Karina Stormfeldt Hjarsen, introduced by Ary Agami, TS Alliance of Mexico
8:15-8:45 am	Elizabeth P. Henske, MD, Brigham and Women's Hospital – Tumor microenvironment of TSC and LAM
8:45-9:15 am	Michael Borchers, PhD, University of Cincinnati – Natural killer cell phenotype and function in LAM
9:15-9:30 am	Rebecca Ihrle, PhD, Vanderbilt University – Single-cell snapshot proteomics reveal an intrinsic, differential susceptibility to TSC tumor development within a persistent stem cell niche
9:30-9:45 am	You Feng, PhD, Brigham and Women's Hospital – Therapeutic targeting of the autotaxin pathway in TSC
9:45-10:15 am	BREAK (<i>Churchill Court</i>)



JUNE 21

DAY TWO, FRIDAY (CONT.)

10:15-10:45 am	William L. Stanford, PhD, Ottawa Hospital Research Institute – Stem cell models identify proteasome inhibition as a potential therapeutic for tuberous sclerosis neural but not mesenchymal tumors
10:45-11:15 am	Patrick Bolton, PhD, FRCPsych, King's College London – Early neurocognitive predictors of neurodevelopmental outcome in TSC
11:15-11:30 am	Elizabeth A. Thiele, MD, PhD, Massachusetts General Hospital – A randomized controlled trial of cannabidiol (GWP42003-P, CBD) for seizures in tuberous sclerosis complex (GWPCARE6)
11:30-11:45 am	Angela Peron, MD, University of Milan – Sleep disorders in children and adolescents with tuberous sclerosis
11:45 am-12:45 pm	Lunch Provided (Mountbatten Salon)
12:45-1:45 pm	Plenary Session 3 (Churchill Ballroom) Session Chairs: Arnold Kristof, MDCM, FRCPC; Angélique Bordey, PhD
12:45-1:15 pm	Jonathan Lipton, MD, PhD, Boston Children's Hospital – The circadian clock as a capacitor for pathogenic mechanisms in tuberous sclerosis complex?
1:15-1:30 pm	Pranetha Baskaran, PhD, Kings College London – Unkempt is a novel downstream regulator of mTOR signaling in mammalian neurogenesis
1:30-1:45 pm	Nicola Alesi, MD, Brigham and Women's Hospital – A model for the pathogenesis of bone lesions in tuberous sclerosis complex
1:45-2:00 pm	MOVE TO BREAKOUT WORKING GROUPS
2:00-3:30 pm	Breakout Working Groups Induced pluripotent stem cell applications for TSC (Seymour) Leader: Peter Crino, MD, PhD, University of Maryland Genetic heterogeneity and modifiers (Carlyle) Leader: David J. Kwiatkowski, MD, PhD, Brigham and Women's Hospital Clinical trial design for neurological aspects of TSC (Scott A & B) Leader: Darcy A. Krueger, MD, PhD, Cincinnati Children's Hospital
3:30-4:00 pm	BREAK (Churchill Court)
4:00-6:00 pm	Plenary Session 4 (Churchill Ballroom) Session Chairs: Shafali S. Jeste, MD; Andrew Tee, PhD
4:00-4:30 pm	Mustafa Sahin, MD, PhD, Boston Children's Hospital – Mechanism based biomarker and treatment trials in the neurology of TSC
4:30-5:00 pm	John J. Bissler, MD, FAAP, FASN, Le Bonheur Children's Hospital – Insights into TSC renal disease and treatment
5:00-5:15 pm	Polina Kosillo, PhD, University of California, Berkeley – Tsc1-mTOR signaling controls the structure and function of midbrain dopamine neurons
5:15-5:45 pm	Report on Breakout Working Groups
5:45-6:00 pm	One-Minute Poster Previews
6:00-7:30 pm	Poster Session and Reception (Mountbatten Lane and Salon)



JUNE 22

DAY THREE, SATURDAY

- 8:00-11:45 am **Plenary Session 5 (Churchill Ballroom)**
Session Chairs: Hilaire C. Lam, PhD; Kevin C. Ess, MD, PhD
- 8:00-8:15 am **Community Speaker** – Lisa Moss, introduced by Kari Rosbeck, TS Alliance
- 8:15-8:45 am Angélique Bordey, PhD, Yale University –
Silencing tuber neurons reduces seizure activity
- 8:45-9:15 am Wei Shi, MD, PhD, Children's Hospital Los Angeles –
Lung mesenchyme-specific deletion of Tsc2 results in spontaneous development of pulmonary nodular lesions
- 9:15-9:30 am Michael Wong, MD, PhD, Washington University –
The role of hypothalamic orexin and mTORC1 activation in a sleep disorder phenotype of a mouse model of TSC
- 9:30-9:45 am Krinio Giannikou, PhD, Brigham and Women's Hospital –
Genomic landscape of SEGAs: unique gene expression profile with few somatic events
- 9:45-10:15 am **BREAK (Churchill Court)**
- 10:15-10:45 am Shafali S. Jeste, MD, University of California, Los Angeles –
Early prediction and intervention for neurodevelopmental disorders in tuberous sclerosis complex (TSC)
- 10:45-11:15 am Jeff MacKeigan, PhD, Michigan State University –
Exploring vulnerabilities in signaling networks to find new molecular targets
- 11:15-11:30 am Heng Du, MD, Brigham and Women's Hospital –
Targeting mTOR/JUN/AXL pathway in TSC-related tumors
- 11:30-11:45 am Helen Bateup, PhD, University of California, Berkeley –
Synaptic alterations in the striatum drive TAND-related behaviors in mice
- 11:45 am-12:45 pm **Lunch Provided (Mountbatten Salon)**
- 12:45-2:15 pm **Plenary Session 6 (Churchill Ballroom)**
Session Chairs: Joao Garcia Martínez, MD; Jeff MacKeigan, PhD
- 12:45-1:15 pm David J. Kwiatkowski, MD, PhD, Brigham and Women's Hospital –
Targeting transcriptional addiction in TSC: CDK7 inhibition leads to selective death of TSC mutant cells and tumors through NFE2L2 and glutathione depletion
- 1:15-1:45 pm Andrew Tee, PhD, Cardiff University –
Dissection of the anti-tumor activity of cannabidiol in tuberous sclerosis complex models
- 1:45-2:00 pm Ji Wang, PhD, Brigham and Women's Hospital –
Interleukin-6, a pro-inflammatory cytokine, promotes tumorigenesis in TSC
- 2:00-2:15 pm Jamie Capal, MD, Cincinnati Children's Hospital –
Language predictors of ASD in tuberous sclerosis
- 2:15-2:45 pm **BREAK (Churchill Court)**
- 2:45-4:00 pm **Joint Session: The future of clinical trials in TSC (Churchill Ballroom)**
Session Chairs: Elizabeth P. Henske, MD; Michael Wong, MD, PhD
Panelists: Cathy Evanochko, TS Canada ST; Raymond Kim, MD, PhD, FRCPC, FCCMG, FACMG, University Health Network; Chris Kingswood, MD, Brighton & Sussex University Hospitals Trust; Eva Schoeters, be-TSC; Maria Zak, NP, Hospital for Sick Children; Zacil Vilchis-Zapata, MD, MSc, CIR Biomédicas Autonomous University of Yucata.