2019 INTERNATIONAL TSC RESEARCH CONFERENCE AGENDA

JUNE 20  DAY ONE, THURSDAY

2:00-6:00 pm  Welcome and Plenary Session 1 (Churchill Ballroom)
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 (Churchill Court)

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 (Mountbatten Lane & Salon)
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 (Churchill Ballroom)
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 Ihrie, 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 (Churchill Court)
**2019 INTERNATIONAL TSC RESEARCH CONFERENCE**

<table>
<thead>
<tr>
<th>Time</th>
<th>Speaker/Institution</th>
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<tr>
<td>10:15-10:45 am</td>
<td>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</td>
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<td>10:45-11:15 am</td>
<td>Patrick Bolton, PhD, FRCPsych, King’s College London – Early neurocognitive predictors of neurodevelopmental outcome in TSC</td>
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<td>11:15-11:30 am</td>
<td>Elizabeth A. Thiele, MD, PhD, Massachusetts General Hospital – A randomized controlled trial of cannabidiol (GWP42003-P, CBD) for seizures in tuberous sclerosis complex (GWPCARE6)</td>
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<td>11:30-11:45 am</td>
<td>Angela Peron, MD, University of Milan – Sleep disorders in children and adolescents with tuberous sclerosis</td>
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<td>11:45 am-12:45 pm</td>
<td>Lunch Provided <em>(Mountbatten Salon)</em></td>
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<td>12:45-1:45 pm</td>
<td>Plenary Session 3 <em>(Churchill Ballroom)</em>  Session Chairs: Arnold Kristof, MDCM, FRCPC; Angélique Bordey, PhD</td>
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<td>12:45-1:15 pm</td>
<td>Jonathan Lipton, MD, PhD, Boston Children’s Hospital – The circadian clock as a capacitor for pathogenic mechanisms in tuberous sclerosis complex?</td>
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<td>1:15-1:30 pm</td>
<td>Pranetha Baskaran, PhD, Kings College London – Unkempt is a novel downstream regulator of mTOR signaling in mammalian neurogenesis</td>
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<td>1:30-1:45 pm</td>
<td>Nicola Alesi, MD, Brigham and Women’s Hospital – A model for the pathogenesis of bone lesions in tuberous sclerosis complex</td>
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<td>1:45-2:00 pm</td>
<td>MOVE TO BREAKOUT WORKING GROUPS</td>
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<td>2:00-3:30 pm</td>
<td>Breakout Working Groups  Induced pluripotent stem cell applications for TSC <em>(Seymour)</em>  Leader: Peter Crino, MD, PhD, University of Maryland  Genetic heterogeneity and modifiers <em>(Carlyle)</em>  Leader: David J. Kwiatkowski, MD, PhD, Brigham and Women’s Hospital  Clinical trial design for neurological aspects of TSC <em>(Scott A &amp; B)</em>  Leader: Darcy A. Krueger, MD, PhD, Cincinnati Children’s Hospital</td>
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<td>3:30-4:00 pm</td>
<td>BREAK <em>(Churchill Court)</em></td>
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<td>4:00-6:00 pm</td>
<td>Plenary Session 4 <em>(Churchill Ballroom)</em>  Session Chairs: Shafali S. Jeste, MD; Andrew Tee, PhD</td>
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<td>4:00-4:30 pm</td>
<td>Mustafa Sahin, MD, PhD, Boston Children's Hospital – Mechanism based biomarker and treatment trials in the neurology of TSC</td>
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<td>4:30-5:00 pm</td>
<td>John J. Bissler, MD, FAAP, FASN, Le Bonheur Children’s Hospital – Insights into TSC renal disease and treatment</td>
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<td>5:00-5:15 pm</td>
<td>Polina Kosillo, PhD, University of California, Berkeley – Tsc1-mTOR signaling controls the structure and function of midbrain dopamine neurons</td>
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<td>5:15-5:45 pm</td>
<td>Report on Breakout Working Groups</td>
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<td>5:45-6:00 pm</td>
<td>One-Minute Poster Previews</td>
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<td>6:00-7:30 pm</td>
<td>Poster Session and Reception <em>(Mountbatten Lane and Salon)</em></td>
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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 Martinez, 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.