Unlocking Treatments for TSC: 2015 Strategic Plan

March 10 – 12, 2015
Bethesda, Maryland

AGENDA

Tuesday, March 10, 2015
Marriott Suites, Democracy Blvd, Bethesda

5:00 – 5:30 p.m. Registration

5:30 – 5:40 p.m. Welcome and Opening Remarks
Walter Koroshetz, Acting Director, NINDS/NIH

5:40 – 5:50 p.m. Workshop Goals
Laura Mamounas, Neurogenetics Cluster, NINDS/NIH

5:50 – 6:20 p.m. Overview of TSC Clinical Manifestations & Patient-Centered Perspectives:
Steve Roberds, Kari Luther Rosbeck, TS Alliance

6:20 – 6:35 p.m. TSC Portfolio Analysis
Cara Long, Office of Science Policy & Planning, NINDS/NIH

6:35 – 7:00 p.m. The Family Perspective
Debora Moritz, TS Alliance
Wednesday, March 11, 2015
Neuroscience Center, Executive Blvd, Bethesda

8:30 – 9:00 a.m.  Registration

9:00 – 10:40 a.m.  Session I: Summary of Working Group Deliberations and Recommendations
Chairs of Working Groups will provide an overview of their topic areas including: i) major research advances over past 5-10 years; ii) gaps/needs, challenges and opportunities for the field; and iii) Working Group preliminary Research Recommendations and Priorities.

9:00 – 9:25 a.m.  Molecular Pathways & Therapeutics: Brendan Manning, Harvard University

9:25 – 9:50 a.m.  Growth/Tumor Biology: Elizabeth Henske, Harvard University

9:50 – 10:15 a.m.  Neurocognition: Mustafa Sahin, Children’s Hospital of Boston

10:15 – 10:40 a.m.  Epilepsy in TSC: Kevin Ess, Vanderbilt University

10:40 – 11:00 a.m.  Break

11:00 – 12:45 p.m.  Breakout Session 1
Discussion topics include the following Focus Areas, based on the shared/overlapping priorities identified from the Working Group (WG) draft recommendations; each Breakout group will include representatives from the four WGs.

- Understanding phenotypic heterogeneity in TSC (Chairs: Hope Northrup, University of Texas Health Science Center; Alcino Silva, University of California Los Angeles)

- Gaining a deeper knowledge of TSC signaling pathways (Chairs: Eric Klann, New York University; Reuben Shaw, Salk Institute)

- Improving TSC disease models (Chairs: Cheryl Walker, Texas A&M Health Center; Michael Wong, Washington University)

- Developing clinical biomarkers for TSC (Chairs: Charles Nelson, Children’s Hospital of Boston; Lisa Young, Vanderbilt University)

- Facilitating therapeutics & clinical trials research (Chairs: John Bissler, Le Bonheur and University of Tennessee Health Science Center; Elizabeth Thiele, Massachusetts General Hospital)

12:45 – 2:00 p.m.  Working Lunch and Summary of Breakout Session 1 outcomes
Discussion Leaders: Breakout Session co-Chairs
Session II: Short talks addressing the shared Focus Areas

2:00 – 2:20 p.m.  
**Phenotypic Heterogeneity in TSC:** David Kwiatkowski, Brigham and Women’s Hospital

2:20 – 2:40 p.m.  
**TSC Signaling Pathways:** John Blenis, Weill Cornell Medical College

2:40 – 3:00 p.m.  
**Preclinical Disease Models:** Karen Cichowski, Brigham and Women’s Hospital

3:00 – 3:20 p.m.  
**Preclinical Disease Models:** Mark Zervas, Amgen

3:20 – 3:40 p.m.  
Break

3:40 – 4:00 p.m.  
**Clinical Biomarkers:** Diane Chugani, Children’s Hospital of Michigan

4:00 – 4:20 p.m.  
**Clinical Biomarkers:** Martina Bebin, University of Alabama at Birmingham

4:20 – 4:40 p.m.  
**Therapeutics/Clinical Trials:** Frank McCormack, University of Cincinnati

4:40 – 5:00 p.m.  
**Therapeutics/Clinical Trials:** Darcy Krueger, Cincinnati Children’s Hospital Center

Thursday, March 12, 2015
Neuroscience Center, Executive Blvd, Bethesda

8:30 – 10:15 a.m.  
**Breakout Session 2**
Focus Areas in Breakout 2 are the same as in Breakout 1, but comprise a different mix of participants to continue to explore the topic.

- **Understanding phenotypic heterogeneity in TSC** (*Chairs: Hope Northrup, University of Texas Health Science Center; Alcino Silva, University of California Los Angeles*)

- **Gaining a deeper knowledge of TSC signaling pathways** (*Chairs: Eric Klann, New York University; Reuben Shaw, Salk Institute*)

- **Improving TSC disease models** (*Chairs: Cheryl Walker, Texas A&M Health Center; Michael Wong, Washington University*)

- **Developing clinical biomarkers for TSC** (*Chairs: Charles Nelson, Children’s Hospital of Boston; Lisa Young, Vanderbilt University*)

- **Facilitating therapeutics & clinical trials research** (*Chairs: John Bissler, Le Bonheur and University of Tennessee Health Science Center; Elizabeth Thiele, Massachusetts General Hospital*)
10:15 – 10:30 a.m.  Break

10:30 – 11:30 a.m.  **Focus Group Summaries & Panel Discussion:**
Co-Chairs from each Focus Area breakout will briefly (5 min each) recap their session’s key recommendations followed by panel discussion.

*Discussion Leaders: Breakout Session co-Chairs*

11:30 – 1:15 p.m.  **Working Lunch and Breakout Session 3**
Original four Working Groups will reconvene to discuss & finalize their group’s Research Recommendations and Priorities based on workshop discussions.

**Molecular Pathways & Therapeutics** *(Chairs: Channing Der, University of North Carolina; Brendan Manning, Harvard University)*

**Growth/Tumor Biology** *(Chairs: Elahna Paul, Massachusetts General Hospital; Elizabeth Henske, Harvard University)*

**Neurocognition** *(Chairs: Gabriella D'Arcangelo, Rutgers University; Mustafa Sahin, Children’s Hospital of Boston)*

**Epilepsy in TSC** *(Chairs: Frances Jensen, University of Pennsylvania; Kevin Ess, Vanderbilt University)*

1:15 – 2:30 p.m.  **Summary of Key Research Recommendations and Next Steps**

*Panel Members: Brendan Manning, Elizabeth Henske, Mustafa Sahin, Kevin Ess, Brandy Fureman, Laura Mamounas, Steve Roberds, Kari Luther Rosbeck, Min Song, Coryse St. Hillaire-Clarke*

2:30 p.m.  Adjourn