

# Unlocking Treatments for TSC: 2015 Strategic Plan

March 10 – 12, 2015  
Bethesda, Maryland

## AGENDA

**Tuesday, March 10, 2015**

*Bethesda Marriott Suites, 6711 Democracy Blvd., Bethesda MD*

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| 5:00 – 5:30 p.m. | <b>Registration</b>   |
| 5:30 – 5:45 p.m. | <b>Welcome and Opening Remarks</b><br><i>Walter Koroshetz, Acting Director, NINDS/NIH</i>   |
|                  | <i>Break followed by Working Dinner</i>   |
| 6:15 – 6:30 p.m. | <b>Workshop Goals</b><br><i>Laura Mamounas, Neurogenetics Cluster, NINDS/NIH</i>  |
| 6:30 – 6:45 p.m. | <b>TSC Portfolio Analysis</b><br><i>Cara Long, Office of Science Policy &amp; Planning, NINDS/NIH</i>   |
| 6:45 – 7:15 p.m. | <b>Overview of TSC Clinical Manifestations &amp; Patient-Centered Perspectives:</b><br><i>Steve Roberds, Kari Luther Rosbeck, TS Alliance</i> |
| 7:15 – 7:30 p.m. | <b>The Family Perspective</b><br><i>Debora Moritz, TS Alliance</i>  |

## Wednesday, March 11, 2015

*Neuroscience Center, 6001 Executive Blvd., Bethesda MD*

8:30 – 9:00 a.m.	<b>Registration</b>
9:00 – 10:40 a.m.	<b>Session I: Summary of Working Group Deliberations and Recommendations</b>  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.	<b>Molecular Pathways &amp; Therapeutics:</b> <i>Brendan Manning, Harvard University</i>
9:25 – 9:50 a.m.	<b>Growth/Tumor Biology:</b> <i>Elizabeth Henske, Harvard University</i>
9:50 – 10:15 a.m.	<b>Neurocognition:</b> <i>Mustafa Sahin, Children's Hospital of Boston</i>
10:15 – 10:40 a.m.	<b>Epilepsy in TSC:</b> <i>Kevin Ess, Vanderbilt University</i>
10:40 – 11:00 a.m.	Break
11:00 – 12:45 p.m.	<b>Breakout Session 1</b>  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. <ul style="list-style-type: none"><li>• <b>Understanding phenotypic heterogeneity in TSC</b> (Chairs: <i>Hope Northrup, University of Texas Health Science Center; Alcino Silva, University of California Los Angeles</i>)</li><li>• <b>Gaining a deeper knowledge of TSC signaling pathways</b> (Chairs: <i>Eric Klann, New York University; Reuben Shaw, Salk Institute</i>)</li><li>• <b>Improving TSC disease models</b> (Chairs: <i>Cheryl Walker, Texas A&amp;M Health Center; Michael Wong, Washington University</i>)</li><li>• <b>Developing clinical biomarkers for TSC</b> (Chairs: <i>Charles Nelson, Children's Hospital of Boston; Lisa Young, Vanderbilt University</i>)</li><li>• <b>Facilitating therapeutics &amp; clinical trials research</b> (Chairs: <i>John Bissler, Le Bonheur and University of Tennessee Health Science Center; Elizabeth Thiele, Massachusetts General Hospital</i>)</li></ul>
12:45 – 2:00 p.m.	<b>Working Lunch and Summary of Breakout Session 1 outcomes</b>  <i>Discussion Leaders: Breakout Session co-Chairs</i>

## Session II: Short talks addressing the shared Focus Areas

2:00 – 2:20 p.m.	<b>Phenotypic Heterogeneity in TSC:</b> David Kwiatkowski, Brigham and Women's Hospital
2:20 – 2:40 p.m.	<b>TSC Signaling Pathways:</b> John Blenis, Weill Cornell Medical College
2:40 – 3:00 p.m.	<b>Preclinical Disease Models:</b> Karen Cichowski, Brigham and Women's Hospital
3:00 – 3:20 p.m.	<b>Preclinical Disease Models:</b> Mark Zervas, Amgen
3:20 – 3:40 p.m.	Break
3:40 – 4:00 p.m.	<b>Clinical Biomarkers:</b> Diane Chugani, Children's Hospital of Michigan
4:00 – 4:20 p.m.	<b>Clinical Biomarkers:</b> Martina Bebin, University of Alabama at Birmingham
4:20 – 4:40 p.m.	<b>Therapeutics/Clinical Trials:</b> Frank McCormack, University of Cincinnati
4:40 – 5:00 p.m.	<b>Therapeutics/Clinical Trials:</b> Darcy Krueger, Cincinnati Children's Hospital Center

## Thursday, March 12, 2015

Neuroscience Center, 6001 Executive Blvd., Bethesda MD

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.	<p><b>Focus Group Summaries &amp; Panel Discussion:</b></p> <p>Co-Chairs from each Focus Area breakout will briefly (5 min each) recap their session’s key recommendations followed by panel discussion.</p> <p><i>Discussion Leaders: Breakout Session co-Chairs</i></p>
11:30 – 1:15 p.m.	<p><b>Working Lunch and Breakout Session 3</b></p> <p>Original four Working Groups will reconvene to discuss &amp; finalize their group’s Research Recommendations and Priorities based on workshop discussions.</p> <p><b>Molecular Pathways &amp; Therapeutics</b> (<i>Chairs: Channing Der, University of North Carolina; Brendan Manning, Harvard University</i>)</p> <p><b>Growth/Tumor Biology</b> (<i>Chairs: Elahna Paul, Massachusetts General Hospital; Elizabeth Henske, Harvard University</i>)</p> <p><b>Neurocognition</b> (<i>Chairs: Gabriella D’Arcangelo, Rutgers University; Mustafa Sahin, Children’s Hospital of Boston</i>)</p> <p><b>Epilepsy in TSC</b> (<i>Chairs: Frances Jensen, University of Pennsylvania; Kevin Ess, Vanderbilt University</i>)</p>
1:15 – 2:30 p.m.	<p><b>Summary of Key Research Recommendations and Next Steps</b></p> <p><i>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</i></p>
2:30 p.m.	<b>Adjourn</b>

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### Final Participant List

**Meropi Athanasiou, PhD**

Congressionally Directed Medical Research  
Programs

Fort Detrick, Frederick, MD

Telephone: (301) 619-7319

E-mail: meropi.athanasiou.civ@mail.mil

**Martina Bebin, MD, MPA**

University of Alabama at Birmingham  
Birmingham, AL

Telephone: (205) 975-5587

E-mail: ebebin@uab.edu

**Noah Berkowitz, MD, PhD**

Novartis Pharmaceuticals  
East Hanover, NJ

Telephone: (862) 778-8880

E-mail: noah.berkowitz@novartis.com

**John Bissler, MD**

LeBonheur/St. Jude Children's Hospital  
Memphis, TN

Telephone: (513) 290-3459

E-mail: jbissler@uthsc.edu

**John Blenis, PhD**

Weill Cornell Medical College  
New York, NY

E-mail: job2064@med.cornell.edu

**Naba Bora, PhD**

US Army  
Frederick, MD

Telephone: (301) 619-6881

E-mail: nabajyoti.bora.civ@mail.mil

**Anna Byars**

Cincinnati Children's Hospital Medical Center  
Cincinnati, OH

E-mail: anna.byars@cchmc.org

**Robert Carson, MD, PhD**

Vanderbilt University  
Nashville, TN

Telephone: (615) 936-5536

E-mail: robert.carson@vanderbilt.edu

**Diane Chugani**

Wayne State University/Children's Hospital of  
Michigan

Detroit, MI

Telephone: (313) 993-3847

E-mail: dchugani@med.wayne.edu

**Karen Cichowski**

Brigham and Women's Hospital, Harvard  
Medical School

Boston, MA

Telephone: (617) 525-4722

E-mail: kcichowski@rics.bwh.harvard.edu

**Peter Crino, MD, PhD**

Temple University  
Moorestown, NJ

Telephone: (215) 908-9288

E-mail: peter.crino@temple.edu

**Gabriella D'Arcangelo, PhD**

Rutgers, the State University of New Jersey  
Piscataway, NJ

Telephone: (848) 445-2839

E-mail: darcangelo@dls.rutgers.edu

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**Thomas Darling, MD, PhD**

Uniformed Services University  
Bethesda, MD  
Telephone: (301) 295-3528  
E-mail: [tdarling2@gmail.com](mailto:tdarling2@gmail.com)

**Channing Der, PhD**

University of North Carolina at Chapel Hill  
Chapel Hill, NC  
Telephone: (919) 966-5634  
E-mail: [cjder@med.unc.edu](mailto:cjder@med.unc.edu)

**Darius Ebrahimi-Fakhari, MD**

Boston Children's Hospital, Harvard Medical  
School  
Boston, MA  
E-mail: [darius.ebrahimi-fakhari@childrens.harvard.edu](mailto:darius.ebrahimi-fakhari@childrens.harvard.edu)

**David Eckstein, PhD**

National Center for Advancing Translational  
Sciences  
Bethesda, MD  
Telephone: (301) 496-0141  
E-mail: [david.eckstein@nih.gov](mailto:david.eckstein@nih.gov)

**Kevin Ess, MD, PhD**

Vanderbilt University Medical Center  
Nashville, TN  
E-mail: [kevin.ess@vanderbilt.edu](mailto:kevin.ess@vanderbilt.edu)

**Robert Finkelstein, PhD**

NINDS, NIH  
Rockville, MD  
E-mail: [bobf@nih.gov](mailto:bobf@nih.gov)

**Leigh Finnegan**

NHGRI  
Washington, DC  
E-mail: [leigh.finnegan@nih.gov](mailto:leigh.finnegan@nih.gov)

**David Franz, MD**

Cincinnati Children's Hospital Medical Center  
Cincinnati, OH  
Telephone: (513) 636-4222  
E-mail: [Franz@cchmc.org](mailto:Franz@cchmc.org)

**Brandy Fureman, PhD**

NINDS, NIH  
Rockville, MD  
E-mail: [furemanb@ninds.nih.gov](mailto:furemanb@ninds.nih.gov)

**Michael Gambello, MD, PhD**

Emory School of Medicine  
Atlanta, GA  
Telephone: (404) 727-6483  
E-mail: [mgambel@emory.edu](mailto:mgambel@emory.edu)

**Marjorie Garvey**

NIMH  
Rockville, MD  
E-mail: [garvey@mail.nih.gov](mailto:garvey@mail.nih.gov)

**Rashmi Gopal-Srivastava**

ORDR, NCATS, NIH  
Bethesda, MD  
Telephone: (301) 402-4336  
E-mail: [garvey@mail.nih.gov](mailto:garvey@mail.nih.gov)

**Ron Heffron, MS**

TSA Science & Medical Committee  
Irvine, CA  
E-mail: [rheffron@moffattnichol.com](mailto:rheffron@moffattnichol.com)

**Lisa Henske, MD**

Brigham and Women's Hospital  
Boston, MA  
Telephone: (617) 775-5722  
E-mail: [ehenske@partners.org](mailto:ehenske@partners.org)

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**Deborah Hirtz, MD**

NINDS, NIH  
Bethesda, MD  
E-mail: hirtzd@ninds.nih.gov

**Gerta Hoxhaj, PhD**

Harvard School of Public Health  
Boston, MA  
Telephone: (617) 432-5615  
E-mail: ghoxhaj@hsph.harvard.edu

**Frances Jensen, MD**

University of Pennsylvania  
Philadelphia, PA  
Telephone: (215) 662-3360  
E-mail: frances.jensen@uphs.upenn.edu

**Alice Kau, PhD**

NICHD, NIH  
Bethesda, MD  
Telephone: (301) 496-1385  
E-mail: kaua@mail.nih.gov

**John Kehne, PhD**

NINDS, NIH  
Bethesda, MD  
E-mail: john.kehne@nih.gov

**Eric Klann, PhD**

New York University  
New York, NY  
E-mail: ek65@nyu.edu

**Henrik Klitgaard**

UCB Pharma  
Braine l'Alleud, Belgium  
E-mail: henrik.klitgaard@ucb.com

**Walter Koroshetz, MD**

NIH  
Bethesda, MD  
Telephone: (301) 496-3167  
E-mail: koroshetzw@ninds.nih.gov

**Darcy Krueger, MD, PhD**

Cincinnati Children's Hospital Medical Center  
Cincinnati, OH  
E-mail: darcy.krueger@cchmc.org

**Vera Krymskaya, PhD, MBA**

University of Pennsylvania  
Philadelphia, PA  
E-mail: krymskay@mail.med.upenn.edu

**David Kwiatkowski, MD, PhD**

Brigham and Women's Hospital  
Boston, MA  
Telephone: (781) 775-7285  
E-mail: dk@rics.bwh.harvard.edu

**Cara Long**

NINDS, NIH  
Bethesda, MD  
E-mail: cara.long@nih.gov

**Ernie Lyons**

NINDS, NIH  
Rockville, MD  
E-mail: lyonse@ninds.nih.gov

**Wendy Macklin, PhD**

University of Colorado School of Medicine  
Aurora, CO  
E-mail: wendy.macklin@ucdenver.edu

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**Laura Mamounas, PhD**

NINDS, NIH  
North Bethesda, MD  
E-mail: mamounas@ninds.nih.gov

**Brendan Manning, PhD**

Harvard School of Public Health  
Boston, MA  
Telephone: (617) 432-5614  
E-mail: bmanning@hsph.harvard.edu

**Francis McCormack, MD**

University of Cincinnati  
Cincinnati, OH  
Telephone: (513) 558-4831  
E-mail: frank.mccormack@uc.edu

**Elizabeth McNeil, MD, MSc**

NINDS, NIH/OCR  
Bethesda, MD  
E-mail: mcneilde@ninds.nih.gov

**Debora Moritz**

Tuberous Sclerosis Alliance  
Scottsdale, AZ  
Telephone: (602) 292-4411  
E-mail: deboramoritz@hotmail.com

**Joel Moss, MD, PhD**

NHLBI  
Bethesda, MD  
Telephone: (301) 496-1597  
E-mail: mossj@nhlbi.nih.gov

**Leon Murphy, PhD**

Novartis Institutes for BioMedical Research,  
Inc.  
Cambridge, MA  
E-mail: leon.murphy@novartis.com

**Jo Anne Nakagawa**

Tuberous Sclerosis Alliance  
Silver Spring, MD  
Telephone: (240) 638-4654  
E-mail: jnakagawa@tsalliance.org

**Charles Nelson**

Boston Children's Hospital/Harvard Medical  
School  
Boston, MA  
Telephone: (617) 355-0401  
E-mail: charles\_nelson@harvard.edu

**Anh Quynh Nguyen**

NHGRI  
Bethesda, MD  
Telephone: (301) 451-8323  
E-mail: anhquynh.nguyen@nih.gov

**Hope Northrup, MD**

UTHealth  
Houston, TX  
Telephone: (713) 500-5760  
E-mail: suzanne.j.paramore@uth.tmc.edu

**Melissa Parisi, MD, PhD**

NICHD, NIH  
Bethesda, MD  
Telephone: (301) 435-6880  
E-mail: parisima@mail.nih.gov

**Elahna Paul, MD, PhD**

Massachusetts General Hospital  
Boston, MA  
Telephone: (617) 726-2908  
E-mail: epaul@mgh.harvard.edu

**Mario Pende, PhD**

Inserm  
Paris, France  
E-mail: mario.pende@inserm.fr



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**Norbert Perrimon, PhD**

Harvard Medical School  
Boston, MA  
E-mail: perrimon@receptor.med.harvard.edu

**Carmen Priolo, MD, PhD**

Brigham and Women's Hospital  
Boston, MA  
E-mail: cpriolo@partners.org

**Rebekah Rasooly, PhD**

NIDDK, NIH  
Bethesda, MD  
Telephone: (301) 594-6007  
E-mail: rebekah.rasooly@nih.gov

**Lora Reineck**

NHLBI, NIH  
Bethesda, MD  
Telephone: (301) 435-0222  
E-mail: reineck@nih.gov

**Robert Riddle**

NINDS, NIH  
Bethesda, MD  
E-mail: riddler@nih.gov

**Akua Roach**

Congressionally Directed Medical Research  
Programs  
Fort Detrick, Frederick, MD  
Telephone: (301) 619-7059  
E-mail: akua.n.roach.ctr@us.army.mil

**Steven Roberds, PhD**

Tuberous Sclerosis Alliance  
Silver Spring, MD  
Telephone: (240) 638-4645  
E-mail: sroberds@tsalliance.org

**Kari Rosbeck**

Tuberous Sclerosis Alliance  
Silver Spring, MD  
Telephone: (301) 562-9890  
E-mail: krosbeck@tsalliance.org

**Mustafa Sahin, MD, PhD**

Boston Children's Hospital  
Boston, MA  
Telephone: (617) 919-6258  
E-mail: Mustafa.Sahin@childrens.harvard.edu

**Paul Scott, PhD**

NINDS, NIH  
Bethesda, MD  
E-mail: scottp@ninds.nih.gov

**Reuben Shaw, PhD**

The Salk Institute for Biological Studies  
La Jolla, CA  
Telephone: (858) 453-4100 ext 1460  
E-mail: shaw@salk.edu

**Alcino Silva, PhD**

University of California, Los Angeles  
Los Angeles, CA  
Telephone: (310) 794-5981 ext. 45981  
E-mail: silvaa@mednet.ucla.edu

**Marie Celeste Simon, PhD**

University of Pennsylvania  
Philadelphia, PA  
Telephone: (215) 746-5532  
E-mail: celeste2@mail.med.upenn.edu

**Katie Smith**

Tuberous Sclerosis Alliance  
Silver Spring, MD  
Telephone: (301) 562-9890  
E-mail: ksmith@tsalliance.org

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**Minkyung (Min) Song, PhD**

National Cancer Institute, NIH  
Rockville, MD  
E-mail: songm@mail.nih.gov

**Coryse St. Hillaire-Clarke, PhD**

NINDS, NIH  
Bethesda, MD  
E-mail: sthillaireclacn@mail.nih.gov

**Delia Talos, MD**

University of Pennsylvania  
Philadelphia, PA  
E-mail: talosd@mail.med.upenn.edu

**Elizabeth Thiele, MD, PhD**

Massachusetts General Hospital  
Boston, MA  
E-mail: ethiele@mgh.harvard.edu

**Ashlee Van't Veer**

NINDS, NIH  
Bethesda, MD  
E-mail: Vantveerav@ninds.nih.gov

**Andrew Wagner, MD, PhD**

Dana-Farber Cancer Institute  
Boston, MA  
Telephone: (617) 632-5204  
E-mail: awagner@partners.org

**Cheryl Walker, PhD**

Texas A&M Health Science Center  
Houston, TX  
Telephone: (713) 677-7440  
E-mail: cwalker@ibt.tamhsc.edu

**Howard Weiner, MD**

New York University Langone Medical Center  
New York, NY  
Telephone: (212) 263-6419  
E-mail: howard.weiner@nyumc.org

**Vicky Whittemore, PhD**

NIH  
Bethesda, MD  
Telephone: (301) 496-1917  
E-mail: vicky.whittemore@nih.gov

**Alan Willard, PhD**

NINDS, NIH  
Bethesda, MD  
E-mail: alanw@ninds.nih.gov

**Michael Wong, MD, PhD**

Washington University School of Medicine  
Creve Coeur, MO  
Telephone: (314) 401-0736  
E-mail: wong\_m@wustl.edu

**Joyce Wu**

University of California, Los Angeles  
Los Angeles, CA  
E-mail: joycewu@mednet.ucla.edu

**Lisa Young, MD**

Vanderbilt University  
Nashville, TN  
E-mail: Lisa.Young@vanderbilt.edu

**Mark Zervas, PhD**

Amgen  
Cambridge, MA  
Telephone: (617) 444-5485  
E-mail: mzervas@amgen.com