

# Inaugural O'Donnell Brain Institute Symposium: Autism Spectrum Disorders

Thursday-Friday, March 25-26, 2021

<https://cme.utsouthwestern.edu/rp2103x>

## PURPOSE AND CONTENT

Autism spectrum disorder (ASD) is one of the most common neurodevelopmental disorders, affecting one in fifty-five children in the United States. This symposium aims to update the community on new discoveries in the pathophysiology and causes of ASD as well as emerging novel treatments. The symposium will: 1) review novel ways to assess and characterize clinical features of patients with ASD, 2) review neural circuit mechanisms in both humans and animal models of ASD, 3) review insights gained from studying syndromic ASD, and 4) review novel treatments for ASD. Attendees will gain an understanding of the current state of research in the underlying pathophysiology of ASD as well as novel treatments to target those mechanisms.

## EDUCATIONAL OBJECTIVES

Upon completion of this activity, participants should be able to:

- *Evaluate clinical phenotyping, genotyping, and biomarker development as critical tools for assessing ASD (Session 1)*
- *Identify the critical neural circuits that contribute to neurodevelopmental disabilities and behavior in ASD (Session 2)*
- *Analyze clinical presentation, disease mechanisms and latest research and treatment strategies for syndromic autisms (Session 3)*
- *Describe novel treatments being developed for ASD such as gene replacement therapy, cerebellar circuit modulation, and neuropsychopharmacology (Session 4).*

## TARGET AUDIENCE

This symposium is designed for and open to physicians and others involved in autism research and clinical care of Autism Spectrum Disorders.

## SPONSORED BY

UT Southwestern Peter O'Donnell Jr. Brain Institute and the UT Southwestern Office of Continuing Medical Education.

## ACCREDITATION AND DESIGNATION STATEMENT

The University of Texas Southwestern Medical Center is accredited by the Accreditation Council for Continuing Medical Education (ACCME) to provide continuing medical education for physicians.

The University of Texas Southwestern Medical Center designates this live educational activity for a maximum of 14.5 AMA *PRA Category 1 Credit™*. Physicians should only claim credit commensurate with the extent of their participation in the activity.

The University of Texas Southwestern Medical Center certifies that non-physicians will receive an attendance certificate stating that they participated in the activity that was designated for 14.5 AMA *PRA Category 1 Credit™*.

## EVALUATIONS & CERTIFICATES

You will receive an email with a link and instructions for accessing and completing the online course evaluation. Once the evaluation is completed, you will be able to claim credit and view/download/print your CME Certificate or Certificate of Attendance.

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## Day One – Thursday, March 25, 2021

TIME	TOPIC	SPEAKER
<b>Session 1</b>	<b>Advancing Assessment and Characterization of Autism</b>	
8:15 a.m. – 8:30 a.m.	Welcome – Opening Remarks	William Dauer, M.D.
8:30 a.m. – 9 a.m.	<b>The Science Behind Interpreting a Genome Sequence in Thousands of Families with Autism</b>	<b>Stephen Scherer, Ph.D.</b>
9 a.m. – 9:30 a.m.	<b>Deciphering Autism: From Genetics to Mechanisms</b>	<b>Maria Chahrour, Ph.D.</b>
9:30 a.m. – 10 a.m.	<b>Toward Strategic Prevention in Autism Spectrum Disorder</b>	<b>Jed Elison, Ph.D.</b>
10 a.m. – 10:30 a.m.	<b>EEG Biomarkers for Improving Clinical Trials in Autism</b>	<b>Sara Webb, Ph.D.</b>
10:30 a.m. – 11 a.m.	<b>Mechanisms of Sensorimotor Impairment and Their Implications for Understanding Pathophysiology in ASD</b>	<b>Matthew Mosconi, Ph.D.</b>
11 a.m. – 11:30 a.m.	<b>Minority Underrepresentation in Autism Diagnoses</b>	<b>Patricia Evans, M.D., Ph.D.</b>
11:30 a.m. – 12:30 p.m.	Session 1 Panel Discussion	Stephen Scherer, Ph.D. Maria Chahrour, Ph.D. Jed Elison, Ph.D. Sara Webb, Ph.D. Matthew Mosconi, Ph.D. Patricia Evans, M.D., Ph.D.
12:30 p.m. – 1:30 p.m.	Poster Session (Not for CME Credit) See page 8 for special agenda and instructions	Lunch Break – Poster Session

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## Day One – Thursday, March 25, 2021

TIME	TOPIC	SPEAKER
<b>Session 2</b>	<b>Neural Circuit Mechanisms in Autism: Humans and Animal Models</b>	
1:30 p.m. – 2 p.m.	<b>Clinical Trial Readiness for Neurodevelopmental Disorders: On the Road to Precision</b>	<b>Shafali Jeste, M.D.</b>
2 p.m. – 2:30 p.m.	<b>Which Circuits? And How Often?</b>	<b>Jason Lerch, Ph.D.</b>
2:30 p.m. – 3 p.m.	<b>Cell Type-Specific Transcriptional Networks Related to Autism</b>	<b>Genevieve Konopka, Ph.D.</b>
3 p.m. – 3:30 p.m.	<b>Mechanisms of Sensory Cortical Dysfunction in Mouse Autism Models</b>	<b>Kimberly Huber, Ph.D.</b>
3:30 p.m. – 4 p.m.	<b>Arousal Modulation of Cortical Network Dynamics in Neurodevelopmental Disorders</b>	<b>Michela Fagiolini, Ph.D.</b>
4 p.m. – 4:30 p.m.	<b>Peripheral Sensory Neuron Dysfunction: Emerging Roles in Autism Spectrum Disorders</b>	<b>Lauren Orefice, Ph.D.</b>
4:30 p.m. – 5:30 p.m.	Session 2 Panel Discussion	Shafali Jeste, M.D. Jason Lerch, Ph.D. Lauren Orefice, Ph.D. Kimberly Huber, Ph.D. Michela Fagiolini, Ph.D. Genevieve Konopka, Ph.D.
5:30 p.m.	Day One Adjourn	

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## Day Two – Friday, March 26, 2021

TIME	TOPIC	SPEAKER
<b>Session 3</b>	<b>Insights from Syndromic Autism; Humans and Animal Models</b>	
8:30 a.m. – 9 a.m.	<b>Targeting mTOR: Therapies for Neurodevelopmental Disorders</b>	<b>Mustafa Sahin, M.D., Ph.D.</b>
9 a.m. – 9:30 a.m.	<b>Fragile X Pathophysiology and Treatment: Obstacles and New Opportunities</b>	<b>Mark Bear, Ph.D.</b>
9:30 a.m. – 10 a.m.	<b>Angelman Syndrome Biomarkers and Treatment Opportunities</b>	<b>Ben Philpot, Ph.D.</b>
10 a.m. – 10:30 a.m.	<b>Mechanisms and Treatment Opportunities for Angelman Syndrome</b>	<b>Ype Elgersma, Ph.D.</b>
10:30 a.m. – 11 a.m.	<b>Learning from Animal Models of Shank3 Mutations</b>	<b>Guoping Feng, Ph.D.</b>
11 a.m. – 12:15 p.m.	Session 3 Panel Discussion	Mustafa Sahin, M.D., Ph.D. Mark Bear, Ph.D. Ben Philpot, Ph.D. Ype Elgersma, Ph.D. Guoping Feng, Ph.D.
12:15 p.m. – 1:15 p.m.	Poster Session (Not for CME Credit) See page 9 for special agenda and instructions	Lunch Break – Poster Session



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## Day Two – Friday, March 26, 2021

TIME	TOPIC	SPEAKER
<b>Session 4</b>	<b>Therapeutic Opportunities for Autism</b>	
1:30 p.m. – 2 p.m.	<b>Refining Translational Medicine in Neurodevelopmental Disorders</b>	<b>Craig Erickson, M.D.</b>
2 p.m. – 2:30 p.m.	<b>Rethinking Approach to Clinical Trials in ASD</b>	<b>Evdokia Anagnostou, M.D.</b>
2:30 p.m. – 3 p.m.	<b>Clinical Trial Readiness for Neurodevelopmental Disorders</b>	<b>Kimberly Goodspeed, M.D.</b>
3 p.m. – 3:30 p.m.	<b>Prospects and Considerations for Gene Therapy for Neurodevelopmental Disorders</b>	<b>Steven Gray, Ph.D.</b>
3:30 p.m. – 4:30 p.m.	<b>Cerebellar Modulation of ASD-Relevant Behavior</b>	<b>Peter Tsai, M.D., Ph.D.</b> <b>Catherine Stoodley, D.Phil.</b>
4:30 p.m. – 5:30 p.m.	Session 4 Panel Discussion	Craig Erickson, M.D. Evdokia Anagnostou, M.D. Kimberly Goodspeed, M.D. Steven Gray, Ph.D. Peter Tsai, M.D., Ph.D. Catherine Stoodley, D.Phil.
5:30 p.m.	Day Two Adjourn	

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## SYMPOSIUM FACULTY

### UT Southwestern Faculty

**Maria Chahrour, Ph.D.**

Assistant Professor, Neuroscience, and Psychiatry

**William Dauer, M.D.**

Professor, Neurology, and Neuroscience  
Director, Peter O'Donnell Jr. Brain Institute

**Patricia Evans, M.D., Ph.D.**

Professor, Pediatrics, Neurology, and Psychiatry

**Kimberly Goodspeed, M.D.**

Assistant Professor, Pediatrics, Neurology,  
and Psychiatry

**Steven Gray, Ph.D.**

Associate Professor, Pediatrics, Molecular Biology,  
and Neurology

**Kimberly Huber, Ph.D.**

Professor, Neuroscience

**Genevieve Konopka, Ph.D.**

Associate Professor, Neuroscience

**Peter Tsai, M.D., Ph.D.**

Assistant Professor, Neurology, Neuroscience,  
Pediatrics, and Psychiatry

### Guest Faculty

**Evdokia Anagnostou, M.D.**

Holland Bloorview Kids Rehabilitation Hospital,  
University of Toronto

**Mark Bear, Ph.D.**

Massachusetts Institute of Technology;  
Picower Institute for Learning and Memory

**Ype Elgersma, Ph.D.**

Erasmus Medical Center, Netherlands

**Jed Elison, Ph.D.**

University of Minnesota, Institute of Child Development

**Craig Erickson, M.D.**

Cincinnati Children's Hospital; University of Cincinnati

**Michela Fagiolini, Ph.D.**

Harvard Medical School; Boston Children's Hospital

**Guoping Feng, Ph.D.**

Massachusetts Institute of Technology; McGovern Institute

**Shafai Jeste, M.D.**

UCLA School of Medicine and Center for  
Autism Research and Treatment

**Jason Lerch, Ph.D.**

Oxford University; Wellcome Centre for Integrative Neuroimaging

**Matt Mosconi, Ph.D.**

University of Kansas Center for Autism Research and Training

**Lauren Orefice, Ph.D.**

Harvard Medical School; Massachusetts General Hospital

**Ben Philpot, Ph.D.**

University of North Carolina, Chapel Hill

**Mustafa Sahin, M.D., Ph.D.**

Harvard Medical School; Boston Children's Hospital

**Stephen Scherer, Ph.D.**

The Hospital for Sick Children, University of Toronto

**Catherine Stoodley, D.Phil.**

American University

**Sara Webb, Ph.D.**

University of Washington, Seattle Children's Research Institute

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## DISCLOSURE OF FINANCIAL RELATONSHIPS

As an organization accredited by the Accreditation Council for Continuing Medical Education (ACCME), The University of Texas Southwestern Medical Center, Office of Continuing Medical Education (UTSW CME) requires that the content of CME activities and related materials provide balance, independence, objectivity, and scientific rigor. Planning must be free of the influence or control of a commercial entity and promote improvements or quality in healthcare. All persons in the position to control the content of an education activity are required to disclose all relevant financial relationships in any amount occurring within the past 12 months with any entity producing, marketing, re-selling, or distributing health care goods or services consumed by, or used on patients.

The ACCME defines “relevant financial relationships” as financial relationships in any amount occurring within the past 12 months that create a conflict of interest. The UTSW Office of CME has implemented a mechanism to identify and resolve all conflicts of interest prior to the activity. The intent of this policy is to identify potential conflicts of interest so participants can form their own judgments with full disclosure of the facts. Participants will be asked to evaluate whether the speaker's outside interests reflect a possible bias in the planning or presentation of the activity.

### FACULTY AND PLANNER DISCLOSURES

Name	Role	Financial Relationship Disclosed
Mark Bear, PhD	Speaker	Business Ownership: Allos Pharma (Fragile X Therapies) Stock: Q-State Biosciences (CNS Disorders)
Ype Elgersma, PhD	Speaker	Professional Services: Hoffman LaRoche (Angelman Syndrome); Biogen (Rare Disorders)
Craig Erickson, MD	Speaker	Independent Contractor: Stalica (autism treatment consulting) Patent: Cincinnati Children's Hospital (Fragile X Treatment IP) Intellectual Property: Confluence Pharmaceuticals (Neurodevelopmental treatments)
Guoping Feng, PhD	Speaker	Business Ownership: Rugen Therapeutics (start-up); NeuroGalaxy (start-up); KeyBio (start-up)
Steven Gray, PhD	Speaker	Intellectual Property: Taysa Gene Therapies (AAV and gene therapy); Asklepios Biopharmaceuticals (AAV and gene therapy) Patent: Neurogene (AAV and gene therapy), Abeona Therapeutics (AAV and gene therapy) Independent Contractor: Lysogene (SAB); Sarepta (SAB); Alector (Consulting); Amicus (Consulting); Opsin Biotherapeutics (Consulting) Speaker: Roche
Shafali Jeste, MD	Speaker	Professional Services: Roche Pharmaceuticals (Consultant)
Lauren Orefice, PhD	Speaker	Professional Services: Deerfield (Consultant)
Ben Philpot, PhD	Speaker	Intellectual Property: AskBio (Angelman Syndrome); Deerfield/Pinnacle Hill (Angelman Syndrome)
Mustafa Sahin, MD, PhD	Speaker	Grant/Contract: Novartis (PTEN/TSC); Biogen (TSC); Bridgebio (TSC); Astellas (spastic paraplegia); Acuta (TSC) Independent Contractor: Novartis (SAB); Roche (SAB); Regenxbio (SAB); Akermes (SAB)
Stephen Scherer, PhD	Speaker	Scientific Advisory Committee: Deep Genomics and Population Diagnostics Research: Athena Diagnostics and Lineagen
Evdokia Anagnostou, MD	Speaker	Grant/Contract: Roche (Clinical trial in ASD) Patent: Anxiety Meter (real time measurement of anxiety)
Maria Chahrour, PhD	Speaker	
Jed Elison, PhD		
Michela Fagiolini, PhD		
Jason Lerch, PhD		No relationships to report
Genevieve Konopka, PhD		
Matthew Mosconi, PhD		
Catherine Stoodley, DPhil		
Sara Webb, PhD		
Patricia Evans, MD, PhD	Speaker, Planner	
Kimberly Goodspeed, MD	Speaker, Planner	No relationships to report
Kimberly Huber, PhD	Speaker, Planner, Course Director	
Peter Tsai, MD, PhD	Speaker, Planner	
Mark Vinciguerra (CME Staff)	Planner	

### Disclosure of Unlabeled Uses

This educational activity may contain discussion of published and/or investigational uses of agents that are not approved by the U.S. Food and Drug Administration. For additional information about approved uses, including approved indications, contraindications, and warnings, please refer to the prescribing information for each product, or consult the Physicians' Desk Reference.

# Inaugural O'Donnell Brain Institute Symposium: Autism Spectrum Disorders | March 25-26, 2021

**POSTER SESSION - THURSDAY, MARCH 25, 2021 - 12:30-1:30pm**

## **Instructions:**

Use this link to access the Poster Session:

<https://zoom.us/j/93148550122?pwd=Sm5tbTZmbERTRVB4a3Q1eVIHZjNVUT09>

Review the list of posters/presenters/breakout rooms

Locate and click "Breakout Rooms" on your Zoom controls bar

Using the Breakout Rooms list that appears, click "JOIN" to enter the breakout room of your choice

Click "Leave Room" to exit the breakout room and enter into the main room

Repeat the above steps to enter additional breakout rooms

## **If you experience problems with assigning yourself into a breakout room:**

Rename yourself with the Room number you wish to go to in front of your name. We will know to place you in that room. When wishing to change rooms, again, place the new room number in front of your name. (example: 4 Will Smith)

<b><u>Room</u></b>	<b><u>Presenter</u></b>	<b><u>Poster Title</u></b>
1	Ana Ortiz*	INVESTIGATIONS INTO CELL TYPE SPECIFIC ROLES OF FOXP1
2	Megan Bone*	TREATING HYPERVENTILATION SPELLS IN GENETIC AUTISM
3	Lauretta Elhayek*	KDM5A MUTATIONS IDENTIFIED IN AUTISM
4	Zhe Zhang*	POSTSYNAPTIC FMRP REGULATES CALLOSAL SYNAPSES
5	Islam Oguz Tuncay*	RECESSIVE ASD IN A CONSANGUINEOUS COHORT
6	Andrew J. Holley*	CONDITIONAL EXPRESSION OF FMR1 IN THE INFERIOR COLLICULUS
7	Francisco Garcia-Oscos*	FOXP1 SELECTIVELY REGULATES VOCAL LEARNING
8	Sebastian Rademaher**	PTEN REGULATES MRNA SPLICING IN PRIMARY NEURONS

## **Institution:**

\* UT Southwestern Medical Center

\*\* Charite-Univeritatsmedizin Berlin



# Inaugural O'Donnell Brain Institute Symposium: Autism Spectrum Disorders | March 25-26, 2021

**POSTER SESSION - FRIDAY, MARCH 26, 2021 - 12:15-1:15pm**

## Instructions:

Use this link to access the Poster Session:

<https://zoom.us/j/93148550122?pwd=Sm5tbTZmbERTRVB4a3Q1eVIHZjNVUT09>

Review the list of posters/presenters/breakout rooms

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(example: 4 Will Smith)

<u>Room</u>	<u>Presenter</u>	<u>Poster Title</u>
1	Megan Bone*	A ZOO-BASED AUTISM SOCIAL SKILLS INTERVENTION
2	Emily Seon Hye Park*	THE CELL-TYPE SPECIFIC ROLE OF FOXP1 IN THE CORTEX
3	Kaitlyn Niederstadt**	AN EXAMINATION OF IMPLICIT TEACHER BIASES
4	Julia Wilkerson*	FMRP UBIQUITINATION AND DEGRADATION ARE REQUIRED FOR SYNAPSE ELIMINATION
5	Newaz Ahmed*	THE ROLE OF FOXP1 AND FOXP2 IN STRIATAL D1 SPINY PROJECTION NEURONS
6	Gemma Molinaro*	PTEN REGULATES EXCITABILITY IN FEMALES
7	Elyza Kelly*	REGULATION OF AUTISM RELEVANT BEHAVIORS BY DISTINCT CEREBELLAR-PREFRONTAL CORTICAL CIRCUITS

## Institution:

\* UT Southwestern Medical Center

\*\* UT Tyler