



LGS FOUNDATION  
**MEETING** OF THE  
**MINDS**



**ADVANCING LGS  
CLINICAL RESEARCH**

 **Sept. 14-16, 2023**

 **Hilton Baltimore Inner Harbor**

**LGS FOUNDATION<sup>®</sup>**  
LENNOX-GASTAUT SYNDROME



# A Huge Thank You to Our Conference Sponsors!

## PLATINUM



## GOLD



## BRONZE



# Meeting Goals and Objectives

## Meeting Goals:

- Convene caregivers, health care providers, clinical trialists, scientists, advocacy groups, and industry partners to discuss methods to measure seizure and non-seizure outcomes (communications, behavior, sleep, and other key areas) in LGS clinical trials.
- Guide the LGS Foundation's funding strategy in 2024 and beyond through information learned from this meeting.

## Learning Objectives:

At the end of the meeting, the attendees should:

- Understand various stakeholder perspectives on measuring seizures, LGS associated comorbidities, and other similar trials.
- Evaluate the current state of the art for measuring seizures and the comorbidities in LGS and other similar trials.
- Learn about the current state of the art for measuring comorbidities in relevant trials outside of the epilepsy field (e.g., sleep trials, rare disease trials, etc.).
- Analyze ongoing research efforts to create new measures of secondary endpoints in epilepsy trials.
- Discuss future paths toward designing better LGS clinical trials.

## Special Thanks to Our Organizers:



**Dr. Scott Perry, Cook Children's Hospital, Fort Worth, TX**



**Dr. Elaine Wirrell, Mayo Clinic, Rochester, MN**



**Dr. Peter Crino, University of Maryland, School of Medicine, MD**



**Dr. Jim Wheless, Le Bonheur Children's Hospital, TN**

# Meet the Speakers



Tracy Dixon-Salazar, PhD -  
LGS Foundation



Scott Perry, MD -  
Cook Children's Hospital



Jennifer Griffin -  
LGS Foundation



Elaine Wirrell, MD -  
Mayo Clinic



Dennis Dlugos, MD -  
Children's Hospital of  
Philadelphia



Randall Kaye, MD -  
Longboard Pharmaceuticals



Scott Andrews, PharmD -  
Takeda



Anup Patel, MD -  
Nationwide Children's  
Hospital



Tricia Proefrock, LGS Expert -  
Palm Beach Gardens, FL



Jackie Villatoro, LGS Expert -  
West Babylon, NY



Andee Cooper, LGS Expert -  
Jenks, OK



Crystal Ferguson, LGS Expert -  
Concord, NC



Rob Stowe, MD -  
Boston Children's Hospital



E. Martina Bebin, MD, MPA -  
Professor of Neurology &  
Pediatrics UAB Epilepsy Center



Eric Marsh, MD, PhD -  
Children's Hospital of  
Philadelphia



Ingo Helbig, MD, PhD -  
Children's Hospital of  
Philadelphia



Anne Berg, PhD -  
Research Professor,  
Northwestern Feinberg  
School of Medicine



Natasha Ludwig, PhD -  
Kennedy Krieger Institute



Mary Wojnaroski, PhD -  
Nationwide Children's  
Hospital



Jeannie Schnur, LGS Expert -  
Leesburg, VA



Jenny Downs, MSc, PhD -  
Telethon KIDS Institute



Dale Todd -  
LGS Foundation



Renee Shellhaas, MD, MS -  
Washington University



Althea Robinson Shelton, MD, MPH -  
Vanderbilt University Medical Center



Gita Gupta, MD, MS -  
University of Michigan

# Research Conference Agenda

## Day 1: Thursday, September 14

7 am	<b>BREAKFAST</b>	<u>Location</u> Johnson
<b>SESSION 1: MEASURING WHAT IS MOST IMPORTANT TO FAMILIES IN LGS RESEARCH</b>		
8 am	<b>Welcome &amp; Opening Remarks</b> <i>Peter Crino, MD, PhD - University of Maryland</i> <i>Tracy Dixon-Salazar, PhD - LGS Foundation</i>	Peale (Level 1)
8:15 am	<b>Introduction to Session 1: Setting the Stage - Perspectives on Measuring What is Most Important to Families in LGS</b> <i>Scott Perry, MD - Cook Children's Hospital, TX</i>	
8:30 am	<b>Voices of the LGS Community: What Do Those with LGS Deem Most Important</b> <i>Tracy Dixon-Salazar, PhD - LGS Foundation</i>	
8:40 am	<b>Family Speaker: Seizures &amp; Safety in LGS</b> <i>Jennifer Griffin, Mom to Theo - LGS Foundation</i>	
8:45 am	<b>How we Choose What to Measure in LGS Research</b> <i>Elaine Wirrell, MD - Mayo Clinic, Rochester</i>	
9:15 am	<b>How we Currently Conduct Clinical Trials in LGS</b> <i>Dennis Dlugos, MD - Children's Hospital of Philadelphia</i>	
9:30 am	<b>Measuring Seizures and LGS Associated Comorbidities in Clinical Trials: How Industry Thinks About This</b> <i>Randall Kaye, MD - Longboard Pharmaceutical</i>	
9:45 am	<b>Measuring Seizures &amp; LGS Associated Comorbidities in Clinical Trials for LGS and DEE: What did we do? How do we count?</b> <i>Scott Andrews, PharmD - Takeda</i>	
10 am	<b>Hearing Directly From Families About How we Measure LGS Associated Comorbidities: Lesson from the PCORI SOM-L Study</b> <i>Anup Patel, MD - Nationwide Children's Hospital, Ohio</i>	
10:15 am	<b>Panel Discussion:</b> <i>Moderator: Scott Perry, MD - Cook Children's Hospital, TX</i>	



# Research Conference Agenda

## Day 1: Thursday, September 14

11:30 am	<b>LUNCH</b>	<u>Location</u> Johnson
<b>SESSION 2: TAKING A CLOSER LOOK AT HOW WE MEASURE WHAT MATTERS TO FAMILIES</b>		Peale
12:30 pm	<b>Welcome Back</b> Tracy Dixon-Salazar, PhD - LGS Foundation	
12:35 pm	<b>Session 2: Family Speakers</b> Tricia Proefrock, Mom to Mason Crystal Ferguson, Mom to Geordan Jackie Villatoro, Mom to Devin Andee Cooper, Mom to Kannon	
12:55 pm	<b>Taking a Closer Look at Measuring: What's Possible</b> Elaine Wirrell, MD - Mayo Clinic, Rochester	
1:15 pm	<b>TSC Clinical Research Consortium: Lessons Learned &amp; Impact on Future Clinical Research</b> E. Martina Bebin M.D., M.P.A. - UAB Epilepsy Center	
1:30 pm	<b>CDKL5 Deficiency Disorder (CDD) Severity Scale</b> Jenny Downs, MSc, PhD - Telethon KIDS Institute	
1:45 pm	<b>STXBP1 Disorders Trial Readiness</b> Ingo Helbig, MD - Children's Hospital of Philadelphia	
2 pm	<b>Inchstone Project: Measuring Communication and Behavior</b> Anne Berg, PhD - Northwestern Medicine, IL	
2:15 pm	<b>Inchstone Project: Measuring Fine/Gross Motor and Gait</b> Jenny Downs, MSc, PhD - Telethon KIDS Institute	
2:30 pm	<b>Inchstone Project: Measuring Awareness and Responsiveness</b> Dr. Natasha Ludwig, MD - Kennedy Krieger Institute Dr. Mary Wojnaroski, MD - Nationwide Children's Hospital	
2:45 pm	<b>Panel Discussion:</b> Moderator: Elaine Wirrell, MD - Mayo Clinic, Rochester	
4 pm	<b>Closing Remarks</b> Tracy Dixon-Salazar, PhD - LGS Foundation	
6 pm	<b>WELCOME RECEPTION</b>	Johnson

# Research Conference Agenda

## Day 2: Friday, September 15

7 am	BREAKFAST	<u>Location</u> Johnson
<b>SESSION 3: UNDERSTANDING &amp; MEASURING SLEEP ISSUES IN LGS</b>		
8 am	<b>Welcome Back</b> <i>Tracy Dixon-Salazar, PhD - LGS Foundation</i>	Peale
8:10 am	<b>Session 3: Family Speakers</b> <i>Jeannie Schnur, Mom to Jackson</i> <i>Dale Todd, Dad to Adam</i>	
8:20 am	<b>Introduction - Why are we talking about sleep?</b> <i>Renee Shellhaas, MD, MS - Washington University</i>	
8:25 am	<b>How can we measure sleep for clinical trials?</b> <i>Gita Gupta, MD, MS - University of Michigan</i>	
8:40 am	<b>Lessons learned from multicenter sleep research</b> <i>Renee Shellhaas, MD MS, - Washington University</i>	
8:55 am	<b>What can we learn from research on sleep disorders in children with autism and other developmental disorders?</b> <i>Althea Robinson Shelton, MD, MPH - Vanderbilt University Medical Center</i>	
9:10 am	<b>Sleep disorders in people with DEEs – is better sleep a primary outcome or a mechanism to improve other endpoints?</b> <i>Rob Stowe, MD - Boston Children's Hospital</i>	
9:25 am	<b>Panel Discussion: How Do We Advance Sleep Research in LGS</b> <i>Moderators: Renee Shellhaas, MD, MS - Washington University</i> <i>Gita Gupta, MD, MS - University of Michigan</i>	
10:25 am	<b>BREAK</b>	

### Did You Know?

#### New Study Comparing Two Treatments for Children with Lennox-Gastaut Syndrome

This project will study the comparative effectiveness of palliative surgery versus additional anti-seizure medications for LGS. The common palliative surgeries are vagus nerve stimulation and corpus callosotomy; while the study is not powered for analysis of RNS, DBS, and other surgeries, data will be collected and descriptives provided.



**SOM-L**  
SURGERY OR MEDS FOR LGS



# Research Conference Agenda

## Day 2: Friday, September 15

### SESSION 4: DISCUSSION - WHERE DO WE GO FROM HERE?

Location

- 10:40 am **Where Do We Go From Here: What do we know? Where are the gaps? What can researchers, clinicians, families do to fill those gaps?**  
*Moderator: Scott Perry, MD - Cook Children's Hospital, TX*
- 10:55 am **Recap: What we have heard over the last few days?**  
*Moderator: Pete Crino, MD, PhD - University of Maryland*
- 11:10 am **Breakout Sessions**
- |   |  |   |
|---|--|---|
| <b>Breakout 1:</b><br><b>How can we create better trials to accurately measure seizures in LGS?</b><br><i>Moderators: Dennis Dlugos, MD<br/>Auvin Stephane, MD, PhD</i> | <b>Breakout 2:</b><br><b>How can we create better studies to accurately measure LGS Associated Commodities?</b><br><i>Moderator: James Wheless, MD</i> | <b>Breakout 3:</b><br><b>How can we address the unique needs of adults with LGS?</b><br><i>Moderator: Pete Crino, MD, PhD</i> |
|---|--|---|
- 12:15 pm **Group Sharing of Ideas from Breakouts**  
*Moderators: Pete Crino, MD, PhD - University of Maryland  
Scott Perry, MD - Cook Children's Hospital, TX*
- 1:15 pm **Closing Remarks**  
*Tracy Dixon-Salazar, PhD - LGS Foundation*

Peale

Bridging the gap between patient families and researchers.

## LGS FOUNDATION COLLABORATIVE RESEARCH NETWORK



Join Us

There is an urgent need to advance research and improve patient care for those living with LGS, especially adults. We need LGS families and world-class researchers to tackle this major challenge. Our plan is to identify the most promising patient-centered research questions and recruit world-class researchers to answer them. Join us today to learn more about how we can advance patient-partnered research.



# Finding the Cures, Together

The whole LGS Community must lead the fight to find the cures. We want to do our part! To that end, the LGS Foundation has launched its Finding the Cures, Together Research Program. Here we have laid out our steps to find the cures for LGS.



*“Bringing together our collaborative voices, we can transform the way new treatments and therapies are developed and tested.”*



*Learn More About the 'Finding the Cures, Together' Research Program*

# LGS Foundation Grants

## Apply Now!



### About the Program

The LGS Foundation accepts unsolicited proposals year-round to seed new basic, translational, and clinical research projects on LGS. Projects may be on any novel topic in LGS but must be directly relevant to LGS, which is characterized by specific seizure types and hallmark EEG features. There are no deadlines for submission. Proposals will be reviewed quickly by our Medical Science Advisory Board, Patient Family Advisory Board, and Ad-Hoc Reviewers. Invitations to submit a full proposal will be extended. Meritorious proposals will be funded immediately upon contracting.



#### Level 1: \$25,000

Submit an LOI today for a \$25,000 LGS research seed grant. May be paid over one year or two. Indirect costs are not permitted. International applicants are welcome to apply.



#### Level 2: \$50,000

Submit an LOI today for a \$50,000 LGS research seed grant. May be paid over one year or two. Indirect costs are not permitted. International applicants are welcome to apply.



#### Level 3: \$75,000

Submit an LOI today for a \$75,000 LGS research seed grant. May be paid over one year or two. Indirect costs are not permitted. International applicants are welcome to apply.

### Grantee Approval Process

Program Open: Year Round

LOI Decision: 2-3 weeks from receipt

Full Proposal Due: Within 3 months

Scientific Review: Takes 4-5 weeks

Committee Review: Takes 2 weeks

Applicant Notified: 2-3 days after review



*To apply, please submit a short (up to two pages) letter of intent to [Research@LGSFoundation.org](mailto:Research@LGSFoundation.org) outlining your project, timeline, and a rough budget.*

# Family Research Day Agenda

## Friday, September 15

6 pm

**WELCOME RECEPTION**

presented by;



**Jazz Pharmaceuticals**

Location  
**Johnson**

## Saturday, September 16

7 am

**BREAKFAST**

Location  
**Johnson**

8 am

**Welcome Recap: What we learned from Researchers  
Day 1 & 2 - And Why Your Participation Matters**  
*Tracy Dixon-Salazar, PhD - LGS Foundation*

*Peale  
(Level 1)*

9:30 am

**What is important to you: A Deeper Discussion**  
*Group Discussion*

11:30 am

**LUNCH**

**Johnson**

12:30 pm

**Working together for the best solutions**  
*Group Discussion*

2:30 pm

**Where do we go from here?**  
*Tracy Dixon-Salazar, PhD - LGS Foundation*



The success of this Natural History Study relies on families sharing their records so that researchers can study it and identify patterns. In addition to helping advance science, the LGS Foundation will regularly share de-identified, aggregated data from the research studies, and these can be used by you to understand the LGS journey better.



Learn More About the LGS Foundation's 'Learn From Every Patient Database'

**AVAILABLE FOR  
DOWNLOAD  
ABOUT LGS  
TREATMENTS KITS**



Made possible by support from our partners at:

**ASSERTIO**



# LGS FOUNDATION<sup>®</sup>

## LENNOX-GASTAUT SYNDROME



### Our Vision:

To end the suffering and devastation caused by LGS.

### Our Mission:

The Lennox-Gastaut Syndrome (LGS) Foundation is a nonprofit organization dedicated to improving the lives of individuals impacted by LGS through advancing research, awareness, education, and family support.

### Our Pillars:

To accomplish our mission we must:

Support, empower, and educate our community

Accelerate research

Raise awareness and build community

Build and strengthen our organization

### Our Theory of Change:

If we create meaningful, impactful, patient-driven programs guided by our pillars, we will improve the lives of those impacted by LGS.

Get Involved: **Volunteer** **Fundraise** **Donate**

### Visit Our Website



E-mail: [Info@LGSFoundation.org](mailto:Info@LGSFoundation.org)

Phone: 718-374-3800

### Follow Us on Social Media!



[www.LGSFoundation.org](http://www.LGSFoundation.org)