

LGS Research
at AES
Roundtable



2021

LGS Foundation Research Roundtable

at



AMERICAN EPILEPSY SOCIETY

LGS FOUNDATION
LENNOX GASTAUT SYNDROME



www.LGSFoundation.org



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Our Why

In Everything We Do, We Believe Every Child And Adult Deserves A Chance To Live Life To The Fullest.



Tracy Dixon-Salazar, PhD
Executive Director, LGS Foundation

“While there is no cure for LGS, there is a hopeful path forward,” said Dr. Tracy Dixon-Salazar, the LGS Foundation’s executive director. “Current treatment methods for LGS have remained roughly the same for the last 30 years. Our goal is to change that.”

With only a fraction of research funds going towards LGS advances, the one-million people with LGS worldwide worry that lifesaving care may not come into fruition in their lifetimes. The LGS Foundation’s grant program and roundtable discussions are combating these fears. Since 2013, the LGS Foundation has awarded more than half a million dollars in funding for 13 projects around the world and this number is poised to increase dramatically in the coming year. In addition to critical funds, the LGS Foundation has also created a network of researchers and families invested in learning from one another.

“We are excited to gather the brightest minds tackling LGS across the globe. And, we believe that now more than ever, the future is bright for those with LGS.”



Agenda

Friday, December 3

- | | |
|-------------|---|
| 11 AM CT | Opening Remarks
Tracy Dixon-Salazar, PhD
<i>Executive Director, LGS Foundation</i> |
| 11:10 AM CT | Moderator/Speaker Intros
Scott Baraban, PhD
<i>University of California, San Francisco</i> |
| 11:15 AM CT | LGS Clinical State of the Field
Anup Patel, MD
<i>Nationwide Children's Hospital</i> |
| 11:35 AM CT | EEG, Human Studies in LGS
John Archer, MD, PhD
<i>University of Melbourne</i> |
| 11:55 AM CT | Break |
| 12:10 PM CT | Chd2 Organoids/iPS Neurons
Jenny Hsieh, PhD
<i>University of Texas, San Antonio</i> |
| 12:30 PM CT | Chd2 Mouse Model
Robert Hunt, PhD
<i>University of California</i> |
| 12:50 PM CT | Dnm1 Mouse Model
Matt Weston, PhD
<i>University of Vermont</i> |
| 1:10 PM CT | Zebrafish Models
Colleen Carpenter, PhD
<i>University of Richmond</i> |
| 1:30 PM CT | Wrap Up
Scott Baraban, PhD
<i>University of California, San Francisco</i> |



About the Speakers



Tracy Dixon-Salazar, PhD **Executive Director, LGS Foundation**

Dr. Tracy Dixon-Salazar is a neuroscientist, geneticist, and patient advocate. Her desire to get her Ph.D. was inspired by her daughter, Savannah, who developed intractable seizures at the age of 2, which evolved into LGS by the age of 5. She did her Ph.D. and postdoctoral work at UC, San Diego, and during her research tenure, and after 16 years of watching daily, unrelenting seizures in her child, she uncovered the driver of her daughter's illness and identified a novel precision therapy that improved her child's life.

Scott Baraban, PhD **University of California, San Francisco**

Scott C. Baraban, Ph.D. is a Professor of Neurological Surgery and William K. Bowes Jr. Endowed Chair in Neuroscience Research at the University of California, San Francisco (UCSF). He obtained his Bachelor's degree from Johns Hopkins University (1983-87) and a Ph.D. in Pharmacology from the University of Virginia (1990-94). He trained as a postdoctoral fellow (1994-1997) with Phil Schwartzkroin at the University of Washington. A faculty member at UCSF since 1999, he directs the Epilepsy Research Laboratory in the Department of Neurological Surgery; a research program continuously funded by NIH for over 20 years.



Anup Patel, MD **Nationwide Children's Hospital**

Anup Patel, M.D. is board certified in neurology with special qualifications in child neurology. He is the President-Elect for the Child Neurology Foundation. He is an associate medical director for quality improvement at Nationwide Children's Hospital and the director of quality improvement for neurology. He is an associate professor for neurology and pediatrics at Nationwide Children's Hospital and The Ohio State University Medical Center in Columbus, Ohio. He has publications and clinical research interests in the field of pediatric epilepsy, health care utilization, learning healthcare systems, and quality improvement.



About the Speakers



John Archer, MD, PhD **University of Melbourne**

Dr. John Archer obtained his medical degree through the University of New South Wales in 1990. He trained in General Neurology in Sydney and Oxford and subsequently completed a Ph.D. in Epilepsy and Neuroimaging through Austin Hospital and The University of Melbourne in 2002. From 2002-7 he initiated and managed the neurology service at Cairns Base Hospital, whilst helping establish the medical program of James Cook University. In 2007 he was appointed Senior Lecturer in Medicine at the University of Melbourne, Neurologist and Head of EEG laboratory Austin Health, and Research Fellow The Florey Institute of Neuroscience and Mental Health.

Jenny Hsieh, PhD **University of Texas, San Antonio**

Dr. Jenny Hsieh is Professor of Biology and the Semmes Foundation Distinguished Endowed Chair in Cell Biology and Director of the UTSA Brain Health Consortium. The UTSA Brain Health Consortium is a campus-wide transdisciplinary research initiative which spans stem cells/precision medicine, neuroscience, biomedical engineering, psychology and behavior with over 40 participating full-time faculty members.



Robert Hunt, PhD **University of California**

Dr. Robert Hunt is an Associate Professor of Anatomy & Neurobiology at the UC Irvine School of Medicine, and Director of the Epilepsy Research Center. He obtained a PhD in Physiology from the University of Kentucky (2010) under the supervision of Dr. Bret Smith and completed a postdoctoral fellowship at University of California San Francisco in the laboratory of Dr. Scott Baraban. In 2015, he joined the faculty at UC Irvine where his lab studies the principles of nervous system development, epilepsy and traumatic brain injury. Within this context, his lab is developing, and applying, new technologies to repair the brain. Publications from his research have appeared in the leading scientific journals Nature Neuroscience, Neuron, Nature Communications and Journal of Neuroscience. Since 2018, he has served as a Contributing Editor for Epilepsy Currents. Dr. Hunt is the recipient of a prestigious K99/R00 Pathway to Independence Award from NINDS, and a LGS Foundation Research Award as a new investigator inspired him to begin working on basic mechanisms of LGS-related genetic disorders. He regularly serves on grant review panels for NIH, DoD, NSF, and starting in 2022, he will be a regular member of CNNT study section at NIH. Visit his lab website and learn about his research: www.roberthuntlab.org



About the Speakers



Matt Weston, PhD **University of Vermont**

University of Vermont and State Agricultural College
Matthew Weston was a Postdoctoral Research Fellow at Baylor College of Medicine. His research aimed to provide novel insight into the regulation of

synaptic and circuit function by the mTOR signaling network, and identify potential targets for treatment of mTOR-related diseases and establish a rigorous framework to test the effects of other neurological disease-causing genes on neuronal function. He earned his Ph.D. in neuroscience from Baylor College of Medicine and earned his B.A. from the University of Virginia. Currently, Dr. Weston is an Assistant Professor in the Department of Neurological Sciences, University of Vermont College of Medicine

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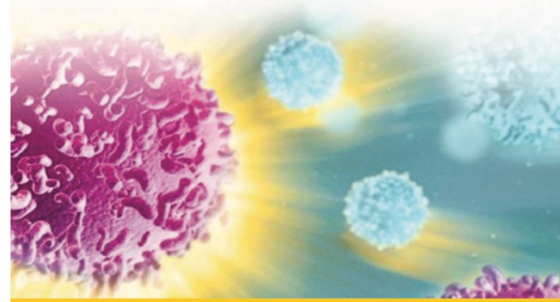
Colleen Carpenter, PhD **University of Richmond**

Dr. Colleen Carpenter is an Assistant Professor of Biology and a Coston Family Fellow in Molecular Biology at the University of Richmond, Virginia. She received her BA in Chemistry from Wesleyan University and a PhD in Pharmacology from the University of

Michigan. She did her postdoctoral training at the University of California, San Francisco in the laboratory of Dr. Scott Baraban. The low cost and high-throughput scalability of zebrafish make it an attractive, cost-effective organism for drug discovery and Dr. Carpenter's scientific interests surround modeling neurological diseases such as epilepsy in zebrafish in hopes of finding new, effective treatments for these disorders. Her current work focuses on severe pediatric genetic epilepsies, particularly Lennox-Gastaut Syndrome. She is also passionate about science communication and education, especially with regards to making science accessible to the general public. Dr. Carpenter is a member of numerous scientific societies including the Society for Neuroscience and the International Zebrafish Society and has presented her work at multiple national and international conferences.



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LGS FOUNDATION'S 2021 RESEARCH ROUNDTABLE



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LGS Foundation Research Grant Program

In the past, the LGS Foundation's seed grant program has awarded one-year research grants up to \$30,000 and two-year grants up to \$50,000 to young investigators, physician residents, and clinicians who were interested in studying Lennox-Gastaut Syndrome.

Starting in 2022, the LGS Foundation will open a new seed grant program. Applications ranging from \$25,000 - \$75,000 may be submitted at any time during the year for rapid review. Our seed grants are intended to help researchers explore novel ideas related to the clinical aspects, therapies, causes, and evolution of LGS. Watch for our new Request for Applications (RFA) in early 2022.

Together, we can bring hope and support to families impacted by rare diseases.

At Zogenix, patients and their families are central to everything we do. We strive to rapidly deliver treatments that address unmet medical needs, in order to change the lives of patients and their communities.

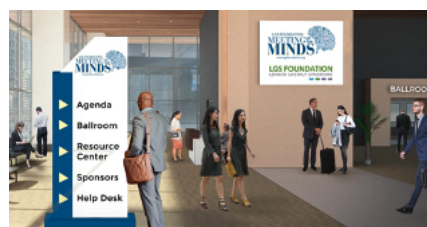
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Rare Disease Therapies



LGS Foundation Meeting of the Minds

IN SEPTEMBER 2021, THE LGS FOUNDATION HOSTED THE FIRST-EVER LGS RESEARCH MEETING OF THE MINDS.



ABOUT THE CONFERENCE

This 2-day discussion by LGS Experts focused on finding Disease-Modifying Therapies in LGS and consisted of 19 presentations and 4 panel discussions.

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Nearly 250 researchers and family members from 7 different countries attended this virtual research conference.



WATCH THE TALKS

Scientific Talks & Conference Program can be found at:

lgsmeetingoftheminds.org

LGS Foundation Meeting of the Minds

KEY LEARNINGS

LEARNING 1:

- **PATIENT FAMILIES WANT A TRANSFORMATIVE TREATMENT. THERE IS AN UNDERLYING, UNIFYING LGS EPILEPTIC NETWORK WHICH IS AN ANATOMICAL STRUCTURE MADE OF GROUPS OF CELLS, CHANNELS, AND MOLECULES. WE NEED TO UNDERSTAND AND TREAT THE LGS NETWORK.**

LEARNING 2:

- **PATIENT FAMILIES COMPARE CURRENT LGS TREATMENT TO THROWING DARTS AT A DARTBOARD, BUT WE ARE NOT LEARNING FROM THE DART PATTERNS. WE NEED TO LEARN FROM EVERY PATIENT AND UNDERSTAND HOW TO TREAT LGS TODAY.**

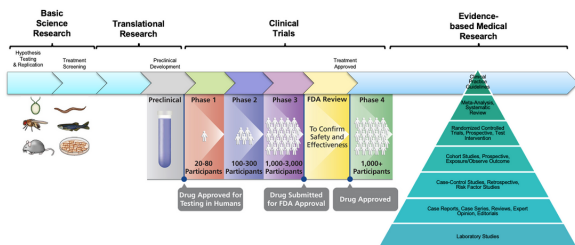
NEXT STEPS:

- **LGS FOUNDATION WILL CONTINUE THE CONVERSATION AS PART OF THE MEETING OF THE MINDS SEMINAR SERIES.**
- **LGS FOUNDATION WILL RELEASE A NEW REQUEST FOR APPLICATIONS (RFA) IN 2022 TO ADDRESS KEY LEARNINGS FROM THE MEETING.**

"I HAVE LEARNED A LOT ABOUT LGS. I HAD NEVER REALLY APPRECIATED HOW UNIQUE IT IS. THE DEVELOPMENTAL COMPONENT IS FASCINATING AND MOTIVATES ME TO LOOK AT OUR MICE OVER TIME TO SEE HOW THE PHENOTYPES EVOLVE."

- Basic Scientist

LGS Research Landscape



"We appreciate the opportunity to learn more about LGS therapies and research today and to share our family's voice!"
- Mom of an adult daughter with LGS



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