



CHILD NEUROLOGY SOCIETY

52nd ANNUAL MEETING

October 4-7, 2023 • Vancouver, BC

ANNUAL MEETING PROGRAM



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	 PERF Pediatric Epilepsy Research Foundation

Manage your schedule on your mobile device

We are excited to announce the launch of our mobile app for the CNS 52nd Annual Meeting! The app serves as your all-in-one event guide by putting everything you need to know right onto your mobile device.

- Customize your conference experience by creating your schedule in advance.
- Communicate with other attendees during the event.
- Browse and search sessions, speakers, and exhibitors.



The EventPilot Conference App is currently available for free in the Apple Store and Google Play. Download yours today by scanning the QR codes below!

- After downloading the app, use the unique code **CNS2023**.
- Log in using the four-digit ID found in your registration confirmation email and on your badge.



Apple Store



Google Play



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Exhibitors



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Learning Objectives

The 2023 CNS Scientific Program

The CNS Scientific Program is designed by and is primarily intended for child neurologists and professionals in other fields of study related to neurologic and developmental disorders in children and adolescents.

As a result of attending this meeting, the physician will be better able to care for children with neurological disease through an understanding of recent advances in neuroscience, neurol-diagnostics and therapeutics relevant to child neurology.

Accreditation Statement

This activity has been planned and implemented in accordance with the accreditation requirements and policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint providership of the Minnesota Medical Association (MMA) is accredited by the Accreditation Council for Continuing Medical Education to provide continuing medical education for physicians.

The Minnesota Medical Association designates this live activity for a maximum of 22.5 AMA PRA Category 1 Credit(s)[™]. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

Welcome to Vancouver!

BRUCE H. COHEN, MD, CNS PRESIDENT



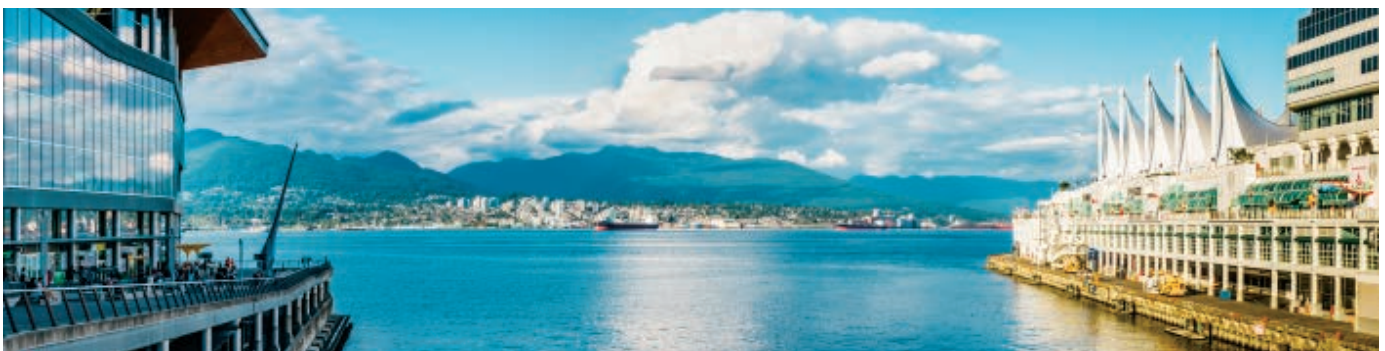
I was thrilled to learn we were returning to Vancouver, as the venue was perfect in 2017. I love our Canadian meetings, not having missed a meeting in Canada since I attended our annual meeting in Halifax. The Scientific Selection and Program Planning Committee, chaired by Yasmin Khakoo, MD, FAAN, FAAP and Associate Chair Bhooma Aravamuthan, MD, DPhil, have planned this year's scientific program. I give the team my deepest thanks for their hard work. This year we will introduce overlapping programming for more presentations, and the scientific program will be recorded for viewing after the meeting. Please look at all the programming, including the pre-program events, the Child Neurology

Foundation Symposium, the Lifetime Achievement Award ceremony on Wednesday morning, and the Professors and Educators of Child Neurology meeting that afternoon. There will be programming through Saturday afternoon, so please consider staying later before grabbing a late afternoon or red-eye flight home.

I am also excited to reunite with our national office staff, including familiar and new faces. This will mark Monique Terrell's first anniversary as CEO of the CNS, whose tenure has brought numerous wonderful changes to our society's operations. In addition to our long-time staff – Sue Hussman (Associate Director and orchestrator of the annual

meeting), our new staff includes William Stanton (Membership Manager) and Katelyn Geiger (Education Coordinator). Please help me welcome them and our staff back at the office, Kathy Pavel (Office Administrator) and Julianne Bruce (Administrative Coordinator).

Of course, the scientific program is only one reason to attend, but spending time with old friends is the highlight that creates the spirit and memories we cherish throughout the years. This year's hallway conversations will add to your enjoyment of this scientific and social gathering. I look forward to seeing you in Vancouver!



Schedule at Glance

All meetings/sessions at the Vancouver Convention Centre West - Halls A-B1

Dates/Times/Rooms Subject to Change

Tuesday, October 3

7:00 AM - 7:00 PM	Nursing Room	West Nursing Room
12:00 PM - 7:00 PM	CNS Registration	West Ballroom Foyer
12:00 PM - 7:00 PM	Speaker Ready Room	101-102
2:00 PM - 6:00 PM	Poster Drop Off/Pick Up	103
6:00 PM - 9:00 PM	CNS John M. "Jack" Pellock Resident Seminar on Epilepsy Reception & Dinner (Pre-registration required/SOLD OUT)	121-122

Wednesday, October 4

7:00 AM - 7:00 PM	CNS Registration	West Ballroom Foyer
7:00 AM - 7:00 PM	Speaker Ready Room	101-102
7:00 AM - 7:00 PM	Poster Drop Off/Pick Up	103
7:00 AM - 7:00 PM	Nursing Room	West Nursing Room
7:00 AM - 8:00 AM	Junior Early Career Forum - Lobby Talks	2nd floor - City Foyer
7:30 AM - 5:00 PM	CNS John M. "Jack" Pellock Resident Seminar on Epilepsy (Pre-registration required/SOLD OUT)	118-120
8:00 AM - 8:30 AM	Association of Child Neurology Nurses (ACNN)	215-216
8:00 AM - 11:00 AM	Symposium I: CNF Symposium: Clinical Trials in Pediatric Neurology: Dilemmas After the Trial	Ballroom A
8:30 AM - 1:00 PM	Program Coordinators of Child Neurology (PCCN)	209
11:30 AM - 1:30 PM	Kenneth F. Swaiman CNS Legacy Luncheon	109-110
11:45 AM - 12:30 PM	Junior Early Career Forum - Lobby Talks	2nd floor - City Foyer
2:00 PM - 3:30 PM	Professors and Educators of Child Neurology (PECN) Business Meeting (PECN Members Only)	211
2:00 PM - 4:30 PM	Navigating the Landscape of Publication and Leadership in Child Neurology	208
2:00 PM - 7:30 PM	Exhibit and Poster Review	Exhibit Hall A-B1
3:30 PM - 5:30 PM	Professors and Educators of Child Neurology (PECN) Meeting (CME)	211
5:00 PM - 6:00 PM	Pediatric Epilepsy Surgery SIG	210
5:00 PM - 6:00 PM	Ethics SIG	213
5:00 PM - 6:00 PM	Neuropalliative Care SIG	214
5:00 PM - 6:00 PM	Neuromuscular SIG	217
5:00 PM - 6:00 PM	Neurocritical Care SIG	218
5:00 PM - 6:00 PM	Neurorehabilitation SIG	219
5:00 PM - 6:00 PM	Neurodevelopment Disorders SIG	220
5:00 PM - 6:00 PM	Traumatic Brain Injury SIG	222
5:00 PM - 6:00 PM	Demyelinating Disease SIG	224
6:00 PM - 7:30 PM	Welcome Reception	Exhibit Hall A-B1
8:00 PM - 10:00 PM	Movement Disorders Video Rounds	Ballroom A

Thursday, October 5

7:00 AM - 7:30 PM	CNS Registration	West Ballroom Foyer
7:00 AM - 7:30 PM	Speaker Ready Room	101-102
7:00 AM - 7:30 PM	Nursing Room	West Nursing Room
7:00 AM - 8:00 AM	Junior Early Career Forum - Lobby Talks	2nd floor - City Foyer
7:00 AM - 8:00 AM	Annals of the Child Neurology Society (ACNS) Editorial Board Meeting	201
8:00 AM - 9:00 PM	Poster Drop Off/Pick Up	103
8:00 AM - 8:15 AM	Association of Child Neurology Nurses (ACNN)	215-216
8:00 AM - 10:15 AM	Symposium II: Presidential Symposium: Mitochondrial Disease: 10 Years of Advances & Progress	Ballroom BC
9:00 AM - 4:00 PM	Program Coordinators of Child Neurology (PCCN)	209
10:45 AM - 12:00 PM	Seminar 1: Neuromodulation in the Clinic: Current and Emerging Applications	Ballroom BC
10:45 AM - 12:00 PM	Seminar 2: Biomarkers & Endpoints for Trials in Neurodevelopmental Disorders	Ballroom A
10:45 AM - 12:00 PM	Seminar 3: Sleep in Children with Neurodevelopmental Disorders	109-110
11:30 AM - 12:30 PM	Junior Early Career Forum - Lobby Talks	2nd floor - City Foyer
11:30 AM - 7:00 PM	Exhibit and Poster Review	Exhibit Hall A-B1
12:00 PM - 1:30 PM	Dedicated Exhibit Time	Exhibit Hall A-B1
12:30 PM - 1:30 PM	Lunch	Exhibit Hall A-B1
12:30 PM - 2:00 PM	Poster Review and Guided Poster Tour #1	Exhibit Hall A-B1
12:30 PM - 2:30 PM	Industry Sponsored Satellite Symposium: Recognizing Rett Syndrome Early to Improve Long-term Management Outcomes	211
1:00 PM - 2:00 PM	Product Theater: Adolescent Migraine: From Burden to Treatment	Exhibit Hall A-B1
1:30 PM - 2:30 PM	Neonatal Neurology SIG	210
1:30 PM - 2:30 PM	Cerebral Palsy SIG	212
1:30 PM - 2:30 PM	Neurohospitalist SIG	213
1:30 PM - 2:30 PM	Neuromodulation SIG	214
1:30 PM - 2:30 PM	International Affairs Committee SIG	217-219
1:30 PM - 2:30 PM	Functional Neurological Disorders (FND) SIG	220
1:30 PM - 2:30 PM	Quality SIG	222
1:30 PM - 2:30 PM	Headache SIG	223
1:30 PM - 2:30 PM	Neuro-Oncology SIG	224
2:30 PM - 3:00 PM	Martha Bridge Denckla Award Lecture, William Davis Gaillard, MD	Ballroom BC
3:00 PM - 5:15 PM	Symposium III: Sexual and Gender Minority Healthcare in Child Neurology	Ballroom BC
4:30 PM - 5:00 PM	Junior Early Career Forum - Lobby Talks	2nd floor - City Foyer
5:30 PM - 7:00 PM	Poster Review (Wine and Cheese) and Guided Poster Tour #2	Exhibit Hall A-B1

SESSIONS highlighted in maroon are designated for CME credit. Agenda and amount of CME credits available are subject to change.

Schedule at Glance CONTINUED

Friday, October 6

7:00 AM - 6:00 PM	CNS Registration	West Ballroom Foyer
7:00 AM - 6:00 PM	Speaker Ready Room	101-102
7:00 AM - 5:00 PM	Poster Drop Off/Pick Up	103
7:00 AM - 6:00 PM	Nursing Room	West Nursing Room
7:00 AM - 8:00 AM	Junior Early Career Forum - Lobby Talks	2nd floor - City Foyer
8:00 AM - 8:15 AM	CNS/CNF/ACNN Awards	Ballroom BC
8:15 AM - 8:45 AM	Philip R. Dodge Young Investigator Awards Lecture, Siddharth Srivastava, MD	Ballroom BC
8:30 AM - 11:45 AM	Association of Child Neurology Nurses (ACNN)	215-216
8:45 AM - 9:30 AM	Bernard Sachs Award Lecture, Amy Brooks-Kayal, MD	Ballroom BC
9:00 AM - 4:00 PM	Program Coordinators of Child Neurology (PCCN)	209
10:00 AM - 11:15 AM	Seminar 4: Mitochondrial Biomarkers: State of the Art, Future of the Science	Ballroom BC
10:00 AM - 11:15 AM	Seminar 5: Carpe Noctem! Unraveling the Epilepsy - Sleep Interface	Ballroom A
10:00 AM - 11:15 AM	Seminar 6: From Lorenzo's Oil to Gene Therapy - Changing X-ALD Therapeutics	109-110
11:45 AM - 1:45 PM	Industry Sponsored Satellite Symposium: Lennox-Gastaut Syndrome in the Real World: A Comprehensive Approach to Diagnosis and Management	211
12:00 PM - 1:30 PM	Lunch	Ballroom A
12:30 PM - 1:45 PM	Seminar 7: Early Life Epilepsy: Closing the Gaps with Collaborative Research	Ballroom BC
12:30 PM - 1:45 PM	Seminar 8: Heads Up: What's Next for Pediatric Migraine Treatment	Ballroom A
12:30 PM - 1:45 PM	Seminar 9: Prognostic Dilemmas in Fetal Neurology: What Would You Do?	109-110
2:15 PM - 4:00 PM	Platform I	109-110
2:15 PM - 4:00 PM	Platform II	Ballroom A
2:15 PM - 4:00 PM	Platform III	Ballroom BC
4:30 PM - 5:00 PM	CNS Business Meeting	Ballroom BC
4:30 PM - 5:00 PM	Junior Member Forum	118-120
5:15 PM - 6:00 PM	Junior Member Seminar 1: Medical Students: Finding a residency	118-120
5:15 PM - 6:00 PM	Junior Member Seminar 2: Residents: Finding a fellowship	121
5:15 PM - 6:00 PM	Junior Member Seminar 3: Residents and Fellows: Getting your first job	122
5:30 PM - 7:00 PM	Scientific Program and Planning Committee Meeting	201
6:00 PM - 7:00 PM	Neurogenetics SIG	210
6:00 PM - 7:00 PM	Neuroimmune Disorders SIG	212
6:00 PM - 7:00 PM	Fetal Neurology SIG	214
7:00 PM - 9:00 PM	Closing Gala	Summit Foyer (3rd Level)

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Saturday, October 7

7:00 AM - 5:00 PM	Nursing Room	West Nursing Room
7:00 AM - 12:00 PM	CNS Registration	West Ballroom Foyer
7:00 AM - 12:00 PM	Speaker Ready Room	101-102
8:00 AM - 8:45 AM	Howar Award Lecture, Phillip L. Pearl, MD	Ballroom BC
9:00 AM - 11:45 AM	Symposium IV: Year in Review	Ballroom BC
12:15 PM - 4:15 PM	CNS Clinical Research Workshop: Pediatric Neurology Clinical Trials - Outcome Measures	118
12:15 PM - 4:15 PM	Biomedical Writing Workshop	122

Exhibits and Poster Reviews

Wednesday

2:00 PM - 7:30 PM	Exhibit and Poster Review	Exhibit Hall A-B1
6:00 PM - 7:30 PM	Welcome Reception	Exhibit Hall A-B1

Thursday

11:30 AM - 7:00 PM	Exhibit and Poster Review	Exhibit Hall A-B1
12:30 PM - 2:00 PM	Poster Review and Guided Poster Tour #1	Exhibit Hall A-B1
5:30 PM - 7:00 PM	Poster Review (Wine and Cheese) and Guided Poster Tour #2	Exhibit Hall A-B1

CME Credit

All credits earned must be claimed/requested once at the end of the meeting.

- Please complete the survey form on Survey Monkey after attending all sessions for which you are requesting credit.
- Complete the online survey to claim CME credit by **December 15** (11:59 pm EST).

CME certificate (pdf) will be sent to the email address you used when registering following completion, beginning December 1.

No CME credit for 2023 will be issued for surveys completed after January 5, 2024.

[HTTPS://WWW.SURVEYMONKEY.COM/R/CNSCME23](https://www.surveymonkey.com/r/CNSCME23)

52nd Annual Meeting of CNS Scientific Program

Tuesday, October 3

6:00 PM – 9:00 PM

CNS John M. “Jack” Pellock Resident Seminar on Epilepsy Reception & Dinner

(Pre-registration required/SOLD OUT)

DINNER SPEAKER

Renée Shellhaas MD, MS,
Washington University in St Louis, St Louis, MO

Wednesday, October 4

7:30 AM – 5:00 PM

CNS John M. “Jack” Pellock Resident Seminar on Epilepsy

(Pre-registration required/SOLD OUT)

8:00 AM – 11:00 AM

SYMPOSIUM I: CNF Symposium: Clinical Trials in Pediatric Neurology: Dilemmas After the Trial

Supported by the Child Neurology Foundation

DESCRIPTION

This three-hour interactive symposium is designed to raise participant awareness of the importance of clinical trials within the child neurology sector, and to identify strategies to manage dilemmas that emerge when the clinical trial finishes.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- identify strategies to overcome existing barriers to supporting children with neurologic conditions when a clinical trial ends.

- utilize best practices for engaging and supporting patients during and after their clinical trial journey.

IMPACT STATEMENT

This educational session helped me to identify changes I could make in my practice related to:

- identifying the ways in which I can support my patients after they participate in a clinical trial.
- effectively discussing the impacts of clinical trials and the FDA approval process with my patients

ORGANIZERS:

Erika Fullwood Augustine, MD, MS
Kennedy Krieger Institute, Baltimore, MD

Anup Patel, MD, FAAN, FAES
Nationwide Children’s Hospital,
The Ohio State University, Columbus, OH

Welcome

Anup Patel, MD, FAAN, FAES
Nationwide Children’s Hospital,
The Ohio State University, Columbus, OH

The Family’s Perspective

Nicole Salazar

The PI’s Perspective

M. Scott Perry, MD
Jane and John Justin Institute for Mind Health,
Cook Children’s Medical Center, Fort Worth, TX

After FDA Approval

Michael Storey, PharmD, MS
Nationwide Children’s Hospital, Columbus, OH

Winding Down

Erika Fullwood Augustine, MD, MS
Kennedy Krieger Institute, Baltimore, MD

11:30 AM – 1:30 PM

Kenneth F. Swaiman CNS Legacy Luncheon

Awards Presented:

Bhuwan Garg High School Neuroscience Award
Rania Lateef

CNS/PECN Training Director Award
Rana R. Said, MD

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**Bernard D'Souza International Fellowship Awards**

Prem Chand, MBBS, FCPS
Priyanka Madaan, MD

Arnold P. Gold Foundation Humanism in Medicine Award

Yasmin Khakoo, MD, FAAN, FAAP

Roger and Mary Brumback Lifetime Achievement Award

Radha Giridharan, MD
Robert S. Greenwood MD
James John Riviello, Jr., MD

2:00 PM – 3:30 PM

Professors and Educators of Child Neurology (PECN) Business Meeting

ORGANIZER

Soe Mar, MD
Washington University School of Medicine,
St. Louis, MO

Introduction and Agenda

Soe Mar, MD
Washington University School of Medicine,
St. Louis, MO

Match report, Preference Signaling and the Interview Season Prep

Margie Ream, MD, PhD
Nationwide Children's Hospital, Columbus, OH

Subcommittees of PECN and education reform

Kathryn Xixis, MD
University of Virginia, Charlottesville, VA

Adam Wallace, MD
University of Wisconsin

Genomics Education for Child Neurology Trainees – Needs Assessment and Curriculum Development

Kuntal Sen, MD
The George Washington University, Washington DC

Merger between PECN and CNS: What to Expect

Donald Gilbert, MD
University of Cincinnati, Cincinnati, OH

Nancy Bass, MD
Medical College of Wisconsin, Milwaukee, Wisconsin

Soe Mar, MD
Washington University School of Medicine,
St. Louis, MO

CNCDP-K12 Report

Bradley L. Schlaggar, MD, PhD
Kennedy Krieger Institute, Baltimore, MD

Minority Research Scholars Program

Erika Fullwood Augustine, MD, MS
Kennedy Krieger Institute, Baltimore, MD

Updates AAP Section of Pediatric Neurology

Tim Lotze, MD
Baylor College of Medicine, Houston, TX

Updates AAN Section of Child Neurology

Donald Gilbert, MD
University of Cincinnati, Cincinnati, OH

Future of NDD

Miya Asato, MD
Kennedy Krieger Institute, Baltimore, MD

The ACGME, the RC, and Child Neurology Training

Louise Castile
RC Executive Director at ACGME, Chicago, IL

Howard Goodkin, MD, PhD
University of Virginia, Charlottesville, VA

2:00 PM – 4:30 PM

Navigating the Landscape of Publication and Leadership in Child Neurology

ORGANIZERS

Alexander Cohen, MD, PhD
Boston Children's Hospital, Harvard Medical School,
Boston, MA

Ariel Maia Lyons-Warren, MD, PhD
Texas Children's Hospital, Baylor College of Medicine,
Houston, TX

Rujuta Wilson, MD, MS
UCLA David Geffen School of Medicine, UCLA Center
for Autism Research and Treatment, Los Angeles, CA

2:00 PM – 7:30 PM

Exhibit and Poster Review

SESSIONS highlighted in maroon are designated for CME credit. Agenda and amount of CME credits available are subject to change.

Wednesday, October 4 CONTINUED

3:30 PM – 5:30 PM

Professors and Educators of Child Neurology (PECN) Meeting (CME): Artificial Intelligence: What's Coming for Child Neurology Education

COURSE DESCRIPTION

Artificial intelligence (AI) is transforming the practice of medicine, from clinical practice to research to medical education. The recent launch of ChatGPT, a publicly accessible language model software tool developed by OpenAI, has increased the accessibility of AI. With increased public use of AI, opportunities and controversy related to this technology have been brought to the forefront, including how AI may affect creativity, originality, and critical thinking; limitations in the accuracy of AI; and the ethics of using AI in replacing certain human tasks. This course will provide the audience with tools to navigate and leverage AI in the educational space, from applications to digital tools to trainee recruitment, and engage participants in a discussion of the controversies surrounding the application of AI to neurology education.

LEARNING OBJECTIVES

As a result of attending this educational session, participants will be able to:

- define artificial intelligence and describe current applications to neurology education
- explain the need for increased curricula about the use of AI across undergraduate, graduate, and continuing medical education spaces
- identify opportunities to use AI as a medical educator, including creation of digital tools and curricular planning

- understand applications of AI to trainee recruitment from the applicant and educator perspectives
- participate in discussion of AI controversies related to AI use in educational assignments and trainee recruitment and selection

IMPACT STATEMENT

Attendees will understand the growing impact of AI and the opportunities for its use in neurology education and recruitment of neurology trainees, while considering the controversies related to its use in these capacities.

You Can Run, But You Can't Hide: Artificial Intelligence Is Here

Andrew Knox, MD, MS
University of Wisconsin School of Medicine,
Madison, WI

AI in Recruitment

Rachel Gottlieb-Smith, MD, MHPE
University of Michigan, Ann Arbor, MI

Kathryn Xixis, MD

University of Virginia, Charlottesville, VA

Justin Rosati, MD

University of Rochester, Rochester, NY

AI in Medical Education

Jaclyn Martindale, DO
Wake Forest University School of Medicine,
Winston-Salem, NC

Jessica Goldstein, MD

University of Minnesota, Minneapolis, MN

6:00 PM – 7:30 PM

Welcome Reception

8:00 PM – 10:00 PM

Movement Disorder Video Rounds



Thursday, October 5

8:00 AM – 10:15 AM

Symposium II: Presidential Symposium: Mitochondrial Disease: 10 Years of Advances & Progress

DESCRIPTION

Mitochondrial disease diagnosis and care has advanced notably over the past 10 years. Improvements in genomics have allowed for accurate diagnoses. National and international consortiums have allowed for the creation of diagnosis and care guidelines. Further understanding of mitochondrial pathophysiology has led to the distinction of primary genetic mitochondrial diseases from secondary mitochondrial dysfunction seen in other genetic disorders. All of this has culminated in the discovery of small molecules to improve mitochondrial function and facilitated therapeutic clinical trials. The field is on the precipice of treatments to stabilize and reverse genetic and non-genetic mitochondrial dysfunction.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- recognize key red flags of genetic mitochondrial diseases as well as the unique aspects of mitochondrial epilepsy
- understand the need for genomic testing to exclude etiologies leading to secondary mitochondrial dysfunction on biochemical testing
- review standards of care for diagnosis and management of mitochondrial disease
- become aware of the US Mitochondrial Care Network and its goals and resources
- explore secondary mitochondrial dysfunction in other genetic diseases, Autism and cancer
- comprehend the impact of the ketogenic diet on mitochondrial metabolism

IMPACT STATEMENT

This educational session helped me to identify changes I could make in my practice related to:

- recognition, diagnosis and management of mitochondrial disease

- understanding the etiologies of secondary mitochondrial disease
- role of the ketogenic diet in impacting mitochondrial function

ORGANIZER

Sumit Parikh, MD
Mitochondrial Medicine Center, Cleveland Clinic,
Cleveland, OH

Mitochondrial Disease Basics (nDNA/mtDNA, Review of Red Flag Symptoms, Approach to Testing, Improvement in Genomics, Limitations of Muscle Biopsies)

Mary Kay Koenig, MD
University of Texas, Houston, Houston, TX

Development of National and International Consensus Guidelines for Diagnosis, Care and the US Mitochondrial Care Network

Sumit Parikh, MD
Mitochondrial Medicine Center,
Cleveland Clinic, Cleveland, OH

Advances in Mitochondrial Disease Clinical Trials

Amel Karaa, MD
Massachusetts General Hospital, Boston, MA

Secondary Mitochondrial Dysfunction in Other Genetic Disorders

Amy Goldstein, MD
Children's Hospital of Philadelphia, Philadelphia, PA

Epilepsy in Mitochondrial Disease

Russ Saneto, MD, PhD
Seattle Children's Hospital, Seattle, WA

The Ketogenic Diet and Mitochondrial Function

Jong Rho, MD
University of California, San Diego, San Diego, CA

Autism and Mitochondrial Dysfunction

Robert Naviaux, MD, PhD
University of California, San Diego School of Medicine,
San Diego, CA

Oncology and Mitochondrial Dysfunction

Bruce Cohen, MD, FAAN
Northeast Ohio Medical University, Akron Children's
Hospital, Akron, OH

Q & A

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Thursday, October 5 CONTINUED

10:45 AM – 12:00 PM

Seminar 1: Neuromodulation in the Clinic: Current and Emerging Applications

DESCRIPTION

The role of neuromodulation in treating children with neurologic disease is rapidly expanding. In the United States, neuromodulation devices are FDA cleared in children for the treatment of epilepsy, movement disorders, pain, headache, and depression and under investigation for many other indications such as pain, ADHD, autism, rehabilitation, depression and sleep. Current methods include, but are not limited to, Deep Brain Stimulation (DBS), Transcranial Magnetic Stimulation (TMS), Transcranial Alternating/ Direct Current Stimulation (tACS/tDCS), Responsive NeuroStimulation (RNS), Chronic Subthreshold Cortical Stimulation (CSCS), and Vagal Nerve Stimulation (VNS). Neuromodulation is increasingly becoming the standard of care for refractory conditions or for individuals who cannot tolerate medication. It can be used to target specific nerves or brain structures, or even influence neural networks. Therefore, clinicians must be familiar with these therapies so that they can identify patients who will benefit. In this session, we will review the basic principles of neuromodulation therapy. Experts from the clinical specialties of 1) headache, 2) epilepsy, and 3) movement disorders will then discuss currently used applications in their field, including advantages and disadvantages, as well as emerging treatments. We will conclude with a panel discussion that addresses potential challenges in using neuromodulation therapies in the pediatric population.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- identify patients that would be appropriate for referral and consideration of neuromodulatory therapies.
- discuss advantages and disadvantages of neuromodulation modalities used in the specialties of headache, epilepsy, and movement disorders.

IMPACT STATEMENT

This educational session helped me to identify changes I could make in my practice related to

- identifying new therapies that my headache, epilepsy, or movement patients might benefit from.

- discussing advantages and disadvantages of a neuromodulatory therapy with patients and their families.

ORGANIZER

Angela Hewitt, MD, PhD
University of Rochester Medical Center,
Rochester, NY

MODERATOR

Angela Hewitt, MD, PhD
University of Rochester Medical Center, Rochester, NY

Steve Wu, MD
Cincinnati Children's Hospital Medical Center,
Cincinnati, OH

Neuromodulation for Headache: Zap the Pain Away

Daniel N. Lax, MD
Albert Einstein College of Medicine,
Montefiore Medical Center, Bronx, NY

Stopping Seizures with Stimulation

Keith Starnes, MD
Mayo Clinic, Rochester, MN

Zap the Wiggles Away

Amy Robichaux Viehoveer, MD, PhD
Washington University in St. Louis, St. Louis, MO

10:45 AM – 12:00 PM

Seminar 2: Biomarkers & Endpoints for Trials in Neurodevelopmental Disorders

DESCRIPTION

Advances in genetic testing, diagnostic screening, and prospective studies of infants at high risk for neurodevelopmental disorders has considerably improved our ability to make early diagnoses of Neurodevelopmental Disorders (NDDs). However, evidence-based treatments have lagged behind these diagnostic advances (PMID 29461424). Even when potentially effective therapeutics are developed, often in the pre-clinical space, clinical trials to test these treatments are hampered by the lack of objective, quantitative measures of drug target engagement, meaningful change, or patient stratification and selection (PMID: 35044968). Biomarkers, reflecting both quantitative measures of behavior as well as brain function, can improve clinical trial design by providing measures of each of the gap areas listed

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above. In fact, the field of NDDs has earmarked considerable funds and resources to develop and test biomarkers for clinical trials. This symposium will discuss the current state and future directions of clinical trials and biomarker development in NDDs across a lifespan. Speakers will present data on (1) quantitative measures of motor function in infancy and early childhood that can aid in prediction of NDDs and elucidate a pathway to language and cognitive impairment, (PMID: 33477359), (2) resting state and sleep electroencephalography as potential measures of drug target engagement and clinical response in genetic syndromes associated with NDDs (PMID: 34344470; PMID: 32791992), and (3) the content, reliability, validity, and sensitivity of the National Institutes of Health (NIH) toolbox cognition battery as a clinical outcome measure in children and adults with NDDs. The symposium will focus on what the child neurologist needs to know about the research and clinical utility of biomarker and clinical trial development in NDDs.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- provide new insights on the structure of biological heterogeneity in ASD and related Neurodevelopmental Disorders (NDDs) and show early data on how such differences may de-risk clinical trial development in NDDs.
- understand how functional imaging biomarkers, such as electroencephalography, and quantitative measures of behavior (motor and cognition), can improve clinical trial readiness, particularly as measures of diagnosis, drug target engagement and patient selection.

IMPACT STATEMENT

This educational session helped me to:

- identify changes I could make in the design of trials and biology studies in NDD and identify changes I could make in my practice to understand when trans-diagnostic approaches are appropriate.



Inspired by **patients.**
Driven by **science.**

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Did you know that **mitochondrial disorders** are among the **most prevalent** group of inherited **neurological disorders**?¹



To learn more about
mitochondrial disease and TK2d,
visit **MaybeMito.com/#tk2d**

Visit us at booth 207 or contact us to learn more at **ucb.com/UCBCares**.

TK2d: thymidine kinase 2 deficiency.

1. Gorman SG, Schaefer AM, Ng Y, et al. Prevalence of nuclear and mitochondrial DNA mutations related to adult mitochondrial disease. *Ann Neurol*. 2015;77(5):753-9.

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Thursday, October 5 CONTINUED

- counsel patients on the implications of biomarker testing in clinical trials and how they might improve our understanding of whether a treatment may be effective.

ORGANIZER

Rujuta Wilson, MD, MS
Semel Institute for Neuroscience and Human Behavior,
UCLA Center for Autism Research and Treatment,
Los Angeles, CA

Current State and Future Directions of Clinical Trials and Biomarker Development in NDDs Across a Lifespan

Evdokia Anagnostou, MD
Bloorview Research Institute, University of Toronto,
Toronto, ON, CAN

Measures of Motor Function as Biomarkers in Neurodevelopmental Disorders

Rujuta Wilson, MD, MS
Semel Institute for Neuroscience and Human Behavior,
UCLA Center for Autism Research and Treatment,
Los Angeles, CA

The National Institutes of Health (NIH) Toolbox Cognition Battery as an Outcome Measure for Neurodevelopmental Disorders

David Randal Hessel, PhD
University of California-Davis, Division of Clinical
Psychiatry, Sacramento, CA

Electrophysiological Biomarkers for Clinical Trials in Genetic Neurodevelopmental Disorders

Shafali Jeste, MD
Division of Neurology, Neurological Institute,
Children's Hospital, Los Angeles, Los Angeles, CA

10:45 AM – 12:00 PM

Seminar 3: Sleep in Children with Neurodevelopmental Disorders

DESCRIPTION

We will discuss sleep in children with neurodevelopmental disorders, including children with genetic syndromes and with undefined causes. First, Dr. Marcos Frank will review sleep in normal and abnormal brain development, including mechanistic insights obtained from his own research. He will explore the regulation and function of sleep in developing animals, the role of sleep in brain plasticity, and the cellular basis for sleep homeostasis. Next, Dr. Amy Licitis will present the

mechanisms and treatment of insomnia in children with neurodevelopmental disorders. She will discuss research on the neurobiology of sleep disorders in children with particular genetic syndromes. Then, Dr. Robert Rudock will review the evaluation of nocturnal spells in children with developmental disorders. He will explore features that help distinguish between seizures and parasomnias in challenging cases and will discuss other sleep-related movements. Finally, Dr. Anne Morse will examine additional sleep issues commonly found in children with neurodevelopmental disorders, including obstructive sleep apnea and restless leg syndrome. She will present her approach to providing comprehensive sleep care to children with neurodevelopmental disorders, with both genetic syndromes and with undefined causes of neurodevelopmental disorders.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- review causes of insomnia and characterize nocturnal spells in children with neurodevelopmental disorders.
- discuss treatments for sleep issues in children with neurodevelopmental disorders.

IMPACT STATEMENT

This educational session helped me to:

- identify changes I could make in my practice related to the treatment of insomnia in children with neurodevelopmental disorders.
- identify changes I could make in my practice related to my ability to distinguish between nocturnal seizures and parasomnias.

ORGANIZER

Amy Licitis, MD, MSCI, FAASM
Washington University Department of Neurology,
St. Louis, MO

Sleep in Normal and Abnormal Development: Insights from Pre-clinical Models

Marcos Frank, PhD
Elson S. Floyd College of Medicine, Washington State
University, Spokane, WA

Insomnia in Children with Neurodevelopmental Disorders: Considering the Causes and Optimizing Treatment

Amy Licitis, MD, MSCI, FAASM
Washington University Department of Neurology,
St. Louis, MO

SESSIONS highlighted in maroon are designated for CME credit. Agenda and amount of CME credits available are subject to change.

Nocturnal Spells in Children with Neurodevelopmental Disorders: Is It a Seizure, a Parasomnia, or Something Else?

Robert Rudock, MD, MBA

Washington University School of Medicine in St. Louis, St. Louis, MO

Providing Patient-Centered Care: Addressing Sleep Issues Comprehensively in Children with Neurodevelopmental Disorders

Anne Morse, DO, FAASM

Geisinger Commonwealth School of Medicine, Geisinger Medical Center, Janet Weis Children's Hospital, Scranton, PA

11:30 AM – 7:30 PM

Exhibit and Poster Review

12:30 PM – 2:00 PM

Poster Review and Guided Poster Tour #1

2:30 PM – 3:00 PM

Martha Bridge Denckla Award Lecture

William Davis Gaillard, MD

Imaging insights into cognitive system development and plasticity

3:00 PM – 5:15 PM

Symposium III: Sexual and Gender Minority Healthcare in Child Neurology

DESCRIPTION

Sexual and gender minority (SGM) is an inclusive term describing people who identify outside of heteronormative binary standards of gender and sexuality. Nearly 21% of Generation Z self-identifies as an SGM person, and 2% self-identify as transgender alone (Jones 2022). Despite this, child neurologists receive minimal dedicated training on the neurological needs of SGM people (Obedin-Maliver 2011). Many are unaware of the direct impact SGM identity has on a variety of neurological conditions, including headache, epilepsy, and functional neurological disorder (FND), all of which have been observed at higher rates in the

SGM community (Rosendale 2019; Rosendale 2021; Hranilovich 2021; Johnson 2019; Konrad and Kostev 2020). Further, gender-affirming hormone therapy can interact with medications commonly used to treat neurological conditions (Rosendale et al. 2019; Hranilovich et al. 2021) and may increase the risk for idiopathic intracranial hypertension (News 2022) and stroke (Streed 2021).

Minority stress and resilience theory describe the interplay of proximal (intrapersonal) and distal (interpersonal, institutional, and systemic) factors resulting in the increased risk for adverse biopsychosocial outcomes experienced by many SGM community members (Toomey 2021). These factors define the social determinants of health for SGM people (Fallin-Bennett 2015; Hughto 2015), describe the impact of bias and stigma, and can result in risk behaviors as harmful coping mechanisms (Roberts 2012; Johnson 2019; Doyle 2021; Hughto 2021).

Drs. Jaclyn Martindale and Alison Christy will moderate this course. A diverse group of experts will introduce SGM terminology, the application of minority stress and resilience theory to neuropsychiatric outcomes, and the importance of accessible affirming healthcare within child neurology. They will then discuss connections to pediatric FND and headaches. We will end with a panel discussion, a question-and-answer session, and clear, simple action items to improve clinical care for SGM people.

LEARNING OBJECTIVES

As a result of this educational session, participants will:

- be able to describe how the gender minority stress and resilience theory applies to neuropsychiatric outcomes in the sexual and gender minority communities
- be able to describe the impact sexual and gender minority identity has on headaches and functional neurological disorders.
- will learn simple tools to approach and interact with patients and families, conveying support for gender diversity and increasing patients' comfort and trust in their neurological care.

IMPACT STATEMENT

This educational session:

- helped me better understand the importance of accessible, affirming healthcare in developing and treating neuropsychiatric conditions in sexual and gender minority communities.

SESSIONS highlighted in maroon are designated for CME credit. Agenda and amount of CME credits available are subject to change.

Thursday, October 5 CONTINUED

- helped me understand how sexual and gender minority identity, and gender-affirming medical therapies, affect the management of neurological disorders in childhood.
- gave me simple, easy action items to improve the care of sexual and gender minority patients and their families in my practice.

ORGANIZER

Jaclyn Martindale, DO
Wake Forest University School of Medicine,
Winston Salem, NC

MODERATORS

Jaclyn Martindale, DO
Wake Forest University School of Medicine,
Winston Salem, NC

Alison Christy, MD, PhD
Providence Health and Services, Portland, OR

Practicing the Basics: Inclusive Neurology Care for Sex, Sexuality, and Gender Diverse People

Casey Orozco-Poore, MD
University of California - Los Angeles, Los Angeles, CA

Functional Neurological Disorder and Cerebrovascular Disease in LGBTQ+ Pediatric Patients

Z L'Erario, MD
Fordham Graduate School of Social Service,
New York, NY

Headache in Transgender Patients

Jennifer Hranilovich, MD
Children's Hospital of Colorado, Aurora, CO

5:30 PM - 7:00 PM

Poster Review (Wine and Cheese) and Guided Poster Tour #2

Recognizing **RETT SYNDROME EARLY** to Improve Long-Term Management Outcomes

HYBRID INTERACTIVE CME SESSION

Join us on
October 5th
at 12:30 - 2:30 PM PT



Scan the code or visit
the link below to register

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LEARNING OBJECTIVES

- Implement guideline recommendations for the early diagnosis of RTT based on patient age and evaluation of the clinical presentation of characteristic symptoms during early growth and development
- Summarize current guideline recommended treatment strategies for the optimal management of RTT from childhood through adulthood
- Evaluate clinical trial efficacy and safety data for new and emerging therapies used to treat RTT
- Develop a personalized, multidisciplinary team coordinated management plan for individuals with RTT that incorporates approved treatment options and ongoing management from childhood through adulthood

Accreditation/Credit Designation:

Physicians' Education Resource®, LLC, designates this live activity for a maximum of 1.5 *AMA PRA Category 1 Credits*™. Physicians should claim only the credit commensurate with the extent of their participation in the activity. Physicians' Education Resource®, LLC, is approved by the California Board of Registered Nursing, Provider #16669, for 1.5 Contact Hours.

Acknowledgment of Educational Grant Support:

This activity is supported by educational grants from Acadia Pharmaceuticals Inc

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Friday, October 6

8:00 AM – 8:15 AM

CNS/CNF/ACNN Awards

Tauen Chang Outstanding Junior Member Awards

Claudia Gambragh-Lyles, MD

Mary Rolfes, MD

Alexander J. Sandweiss, MD, PhD

Alexandra Santana Almansa, MD

Tauen Chang Outstanding Junior Member Post Graduate Award

Milena Andzelm, MD PhD

Jennifer Harmon, MD, PhD

M. Richard Koenigsburger Scholarship

Hui Li, MD, PhD

AAP Section of Neurology Travel Grant

Miranda Creasey, MD

8:15 AM – 8:45 AM

Philip R. Dodge Young Investigator Award Lecture

Siddharth Srivastava, MD

Cerebral Palsy in the Modern Genomics Era

8:45 AM – 9:30 AM

Bernard Sachs Award Lecture

Amy Brooks-Kayal, MD

Molecules, Medicines & Mentorship: Seeking Precision Therapy for Epilepsy

10:00 AM – 11:15 AM

Seminar 4: Mitochondrial Biomarkers: State of the Art, Future of the Science

DESCRIPTION

Much has changed – and much has not – since Drs. Munnich and Saudubray famously wrote in 1992 “a mitochondrial disorder can account for any presenting symptom in any organ and tissue at any age.” While next generation sequencing has revolutionized genetic diagnosis in mitochondrial disorders, genotype-phenotype correlations remain notoriously unpredictable, a critical problem that is exacerbated by a dearth of diagnostic, monitoring, and prognostic biomarkers. Limited natural history

data suggests these diseases are more complex than widely appreciated. In a handful of mitochondrial disorders, diagnostic biomarkers are available and can be critical to guiding management, including rare diseases amenable to targeted therapies. In the first session, standard of care diagnostics will be reviewed with an emphasis on disorders with disease modifying treatments as well as current knowledge gaps. In the second session, the role of established and emerging medical and neurometabolic disease monitoring methods in critical care will be discussed and existing challenges highlighted. Finally, the third session will report the results of cutting-edge science, including single cell mitochondrial DNA sequencing enabled by a novel methodology and metabolomic analysis, to identify mechanistic monitoring and prognostic mitochondrial disease biomarkers. Single cell genetic studies reveal the first cell type-specific patterns of mitochondrial DNA mutations that likely confound conventional diagnostic sequencing and nominate solutions. Metabolomics, meanwhile, can distinguish patients with the most severe neurologic phenotypes (stroke-like episodes) from those without and outline multiple metabolic defects in a disease previously thought to be isolated to oxidative phosphorylation. Attendees will be able therefore to identify standard of care biomarkers with the ability to direct management in suspected mitochondrial disorders prior to the receipt of definitive genetic diagnoses, to understand parameters to guide management in the acute setting, and to appreciate novel approaches with great promise to produce new diagnostic, prognostic, and monitoring biomarkers for these diseases.

LEARNING OBJECTIVES

As a result of attending this educational session participants will be able to:

- identify key diagnostic, non-genetic biomarkers for intervenable mitochondrial diagnoses at baseline and for clinical monitoring in acute crises
- describe challenges to be met and advances made in the development of new metabolic and single cell sequencing biomarkers in mitochondrial DNA-related disease

IMPACT STATEMENT

This educational session will help attendees to identify changes they could make in their practice related to:

- provide earlier diagnoses in mitochondrial disorders amenable to specific interventions
- monitoring and collaborative neurologic and intensive care for mitochondrial disorder patients during acute illness

Friday, October 6 CONTINUED

ORGANIZER

Melissa A. Walker, MD, PhD
Child Neurology Division, Massachusetts General
Hospital, Harvard Medical School, Boston, MA

Diagnostic and Prognostic Biomarkers in Primary Mitochondrial Disease

Amy Goldstein, MD
Mitochondrial Medicine Frontier Program, Children's
Hospital of Philadelphia, University of Pennsylvania
Perelman School of Medicine, Philadelphia, PA

Metabolic Crisis: Using Biomarkers in the Critical Care Setting

Divakar S. Mithal, MD
Section of Neurology, Ann and Robert H. Lurie
Children's Hospital of Chicago
Department of Pediatrics, Northwestern University
Feinberg School of Medicine, Chicago, IL

From Full Scan Metabolomics to Single Cell (Dual) Genomics: New Horizons in Mito Biomarkers

Melissa A. Walker, MD, PhD
Child Neurology Division, Massachusetts General
Hospital, Harvard Medical School, Boston, MA

10:00 AM – 11:15 AM

Seminar 5: *Carpe Noctem!* Unraveling the Epilepsy – Sleep Interface

DESCRIPTION

The link between sleep and epilepsy is inextricable yet not well understood. There are numerous pediatric epilepsy syndromes with well-established links between seizures and sleep, from a host of self-limited epilepsies of childhood to more life-long conditions such as Lennox-Gastaut syndrome. Surveys of families with children with epilepsy describe sleep as a source of great consternation, from the concern about how insufficient or disrupted sleep may impact seizure control, behavior, and development, to the concern of sudden unexpected death in epilepsy to their child while they sleep. As pediatric neurologists, it is paramount to become familiar with the effective evaluation of any patient's sleep to identify potential areas of concern (e.g., sleep-disordered breathing, inappropriate sleep associations) and target them with appropriate diagnostics and interventions. This talk will highlight the nuances and considerations when taking into account a child's epilepsy diagnosis and

treatment. As the treating provider for their epilepsy, we also have an opportunity to conceptualize the impact of our medications on not only seizure control but sleep. We will provide keen insights into the science of chronopharmacology and circadian (and infra- and ultradian) rhythms of seizures and show how this knowledge can improve patients' seizure control and improve patient quality of life. Finally, we will review the International League Against Epilepsy's updated terminology on developmental/epileptic encephalopathy with spike-wave activation in sleep (D/EE-SWAS), which was recommended to subsume the terms of electrical status epilepticus of sleep, continuous spike-waves of sleep, and Landau-Kleffner syndrome. We will discuss the pros and cons of this nomenclature adjustment, and the clinical guidance including diagnosis and management, amongst the numerous pitfalls of this complex and heterogeneous condition.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- identify the current evidence regarding chronopharmacology principles in the treatment of sleep-related and nocturnal seizures.
- appropriately evaluate for concurrent sleep disorders in pediatric epilepsy patients and apply appropriate diagnostic and treatment plans
- define the new International League Against Epilepsy (ILAE) recommended terminology of developmental/epileptic encephalopathy with spike-wave activation in sleep and describe the clinical challenges in its diagnosis and treatment.

IMPACT STATEMENT

This educational session helped me to identify changes I could make in my practice related to:

- improving seizure control and mitigating antiseizure medication side effects when managing children with nocturnal or sleep-related seizure phenotypes.
- assessing for sources of sleep disruption in children with epilepsy and how to appropriately manage these challenges.
- the appropriate diagnosis and treatment of developmental/epileptic encephalopathy with spike-wave activation in sleep.

SESSIONS highlighted in maroon are designated for CME credit. Agenda and amount of CME credits available are subject to change.

ORGANIZER

Robert C. Stowe, MD
Boston Children's Hospital, Harvard Medical School,
Boston, MA

Interconnections Between Sleep and Epilepsy

Temitayo Oyegbile-Chidi, MD, PhD, FAAN
University of California, Davis, Sacramento, CA

Dissecting the Effects of Sleep Versus Circadian Rhythms on Seizure Risk

Vishnu Cuddapah, MD, PhD
Children's Hospital of Philadelphia, Philadelphia, PA

Developmental/Epileptic Encephalopathy with Spike-Wave Activation in Sleep: New Name, Same Problems

Robert C. Stowe, MD
Boston Children's Hospital, Harvard Medical School,
Boston, MA

10:00 AM – 11:15 AM

Seminar 6: From Lorenzo's Oil to Gene Therapy – Changing X-ALD Therapeutics

DESCRIPTION

X-linked Adrenoleukodystrophy (X-ALD) is a complex progressive neurometabolic genetic disorder with various clinical presentations throughout the lifespan from infancy to early childhood to young adulthood. It is caused by pathogenic variants in the ABCD1 gene, encoding a peroxisomal membrane protein essential for the β -oxidation of very long chain fatty acids (VLCFAs). The genetic defect in ABCD1 leads to VLCFA accumulation in brain, spinal cord, adrenal glands and testes; plasma levels serve as a diagnostic marker detectable by newborn screening. There are 3 main clinical ALD phenotypes: adrenal insufficiency in males beginning from mid-infancy (Addison disease); cerebral ALD (CALD), a rapidly progressive inflammatory demyelinating leukodystrophy affecting males in childhood; and myeloneuropathy in young adult men and women. ALD was added to the Recommended Uniform Screening Panel (RUSP) and many states in the US and other countries now screen for this disorder. Hematopoietic stem cell transplant (HSCT) is the current standard of care therapy for early active CALD. Recently, in 2022, elivaldogene autotemcel (SKYSONA), an ex-vivo autologous lentiviral-based gene therapy, was FDA approved to

slow active progression of neurologic dysfunction in CALD boys. In this symposium, Dr. Carlson will review clinical X-ALD phenotypes and neuroimaging characteristics. Dr. Sen will discuss newborn screening inclusion with caveats of testing family members, ethics and care co-ordination. Dr. Eichler will focus on the two current therapies (HSCT and gene therapy). Dr. Hisama's presentation will delve into the adult adrenomyeloneuropathy (AMN) phenotype including outcomes in men who received HSCT. X-ALD has been a frontrunner in advocacy for stakeholders in orphan diseases, from the 1990s when the movie Lorenzo's Oil was released, up until 2022 when ex vivo gene therapy was FDA approved. This symposium will be a timely, important and relevant discussion on advances in X-ALD diagnostics and therapeutics, enabling broader understanding of precision medicine in neurogenetics.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- identify the diverse clinical phenotypes and neuroimaging characteristics of X-ALD.
- review the new disorders included in newborn screening in recent years and focus on various aspects of screening for X-ALD.
- describe the therapeutic advances in X-ALD including HSCT and the newly approved lentiviral based autologous ex-vivo gene therapy.
- evaluate the outcomes in adults with X-ALD who received HSCT and discuss the AMN phenotype. Learn the differences in clinical presentations in ALD between adults and children. Understand the screening recommendations for asymptomatic and symptomatic adults diagnosed with ALD, and discuss the components and challenges in an interdisciplinary model of medical care for adults with ALD.

IMPACT STATEMENT

This educational session helped me to identify changes I could make in my practice related to

- identifying three main diverse clinical phenotypes of X-ALD spanning the lifespan from infancy to early childhood to young adulthood (Addison disease, Cerebral ALD and Myeloneuropathy). The neuroimaging characteristics for X-ALD (with pattern recognition) were reviewed. This will expand medical practice to include a comprehensive

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Friday, October 6 CONTINUED

knowledge base of this diverse neurogenetic condition and its detection by newborn screening, with a better understanding of the diagnostic and therapeutic advances and treatments available.

- enabling a better understanding of the role of HSCT and Gene Therapy in this (Cerebral ALD) condition and the value of NBS in implementing timely implementation of these essential therapeutics. It also included the young adult phenotype of myeloneuropathy to bridge the age span.

ORGANIZER

Martha Carlson, MD, PhD
University of Michigan, Ann Arbor, MI

X-ALD Clinical Diversity and Neuroimaging

Martha Carlson, MD, PhD
University of Michigan, Ann Arbor, MI

The Evolving Landscape of Newborn Screening: X-ALD and More

Kuntal Sen, MD, FACMG
Center for Neuroscience and Behavioral Medicine,
Children's National Hospital, GWU School of Medicine
and Health Sciences, Washington DC

How to Implement HSCT Versus Gene Therapy for X-ALD

Florian Eichler, MD
Massachusetts General Hospital, Harvard Medical
School, Boston, MA

ALD in Adults: What Child Neurologists Should Know

Fuki Marie Hisama, MD, FACMG, FAAN
University of Washington School of Medicine,
Seattle, WA

12:30 PM – 1:45 PM

Seminar 7: Early Life Epilepsy: Closing the Gaps with Collaborative Research

DESCRIPTION

The incidence of epilepsy is highest in the first years of life. Drug-resistant epilepsy (DRE) occurs in one-third of all people with epilepsy. DRE is more common in children less than three years of age (35-65%), likely due to specific etiologies that present in early childhood, such as structural brain abnormalities, early-onset neurogenetic and metabolic disorders, and perinatal brain injury. Each is associated with a high risk of intractable epilepsy. Early recognition and resolution of DRE are essential to optimize

developmental outcomes, as uncontrolled seizures at this critical period of brain development can be associated with developmental stagnation or decline.

Clinical practice in the diagnosis and management of infantile epilepsies varies widely, with limited well-designed research to guide decision-making. A recent Agency for Healthcare Research and Quality (AHRQ) report "Management of Infantile Epilepsy" highlighted significant knowledge gaps related to surgical therapy, medication management, and outcomes beyond seizure freedom. Most available data derive from studies that are single-center, retrospective, or inadequately powered, limiting confidence in their conclusions.

Over the last decade, several teams of Child Neurology Society members have developed practice-changing research networks to define and deliver the best possible care to children with early life epilepsies. This seminar will highlight the results of collaborative clinical research initiatives related to early life epilepsies. Four engaging speakers will provide a deeper understanding of the diagnostic and treatment practices surrounding these difficult epilepsies. This seminar will intertwine operational knowledge gained over a decade of work by the Neonatal Seizure Registry and the Pediatric Epilepsy Research Consortium, among others, from projects that address knowledge gaps in early life epilepsy. Throughout, the speakers will champion the benefits of working together to inform, engage, and inspire attendees to embrace multidisciplinary collaborative research.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- list knowledge gaps in the evaluation and treatment of early life epilepsy and discuss practice-changing findings from multiple collaborative research programs addressing this topic.
- describe methods of collaborative research that can be used to address knowledge gaps in early life epilepsy.

IMPACT STATEMENT

This educational session helped me to identify changes I could make in my practice related to:

- the diagnosis, evaluation, and treatment of early life epilepsy.
- design and implementation of collaborative multi-center research.

SESSIONS highlighted in maroon are designated for CME credit. Agenda and amount of CME credits available are subject to change.

ORGANIZERS

Renée A. Shellhaas, MD MS
Washington University School of Medicine,
St. Louis, MO

M. Scott Perry, MD
Jane and John Justin Institute for Mind Health,
Cook Children's Medical Center, Fort Worth, TX

Introduction

M. Scott Perry, MD
Jane and John Justin Institute for Mind Health,
Cook Children's Medical Center, Fort Worth, TX

Lessons Learned from Collaboration on Neonates with Seizures

Renée A. Shellhaas, MD, MS
Washington University School of Medicine,
St. Louis, MO

Care Inequities for Children with Epilepsy, What We Learned, and Can We Fix It?

Zachary Grinspan, MD, MS
Weill Cornell Medicine, New York, NY

How Quickly Should We Move to Epilepsy Surgery?

Nathan Cohen, MD
The George Washington University School of
Medicine, Children's National Hospital,
Washington DC

Measuring Inch Stones of Success for Those with Profound Intellectual and Multiple Disabilities: From FDA Guidances to Community Initiatives

Anne T. Berg, PhD
Decoding Developmental Epilepsies,
Washington, DC

12:30 PM – 1:45 PM

Seminar 8: Heads Up: What's Next for Pediatric Migraine Treatment

DESCRIPTION

Pediatric migraine is one of the leading neurologic causes of disability among children and adolescents. Over the last decade, there has been major advancement in migraine treatment with the FDA approval of CGRP targeted therapies in adults, and increasing use in adolescents based on expert consensus; the FDA approval of neuromodulatory devices for adolescents and adults; increasing use of procedures to treat migraine in adolescents; as

A CLINICAL RESEARCH STUDY FOR DYSKINESIA DUE TO CEREBRAL PALSY (DCP)



WHAT OPPORTUNITIES COULD BE CREATED THROUGH CEREBRAL PALSY RESEARCH?

Neurocrine Biosciences is looking for patients 6 to 70 years old with dyskinesia due to cerebral palsy (DCP) for the Kinect-DCP Study. The purpose of the study is to determine if a study drug, called valbenazine, is safe to use and if it helps to improve dyskinesia due to cerebral palsy. To learn more or refer a patient, visit the study website at KinectDCPTrial.com.



NBI-98854-DCP3018_KinectDCPStudy_HalfPageAd_V1_01November2022

Friday, October 6 CONTINUED

well as new applications of technology to cognitive behavioral therapy. This seminar will cover recent updates in the acute and preventative treatment of pediatric migraine, including new pharmacologic treatments, updates in cognitive behavioral therapy, neuromodulation, and procedural treatments.

The symposium will begin with an overview of current pharmacologic treatments, with an emphasis on the newer anti-CGRP medications currently FDA approved in adults and undergoing trials in pediatrics, with increasing use based on expert consensus guidelines. The next speaker will discuss updates in cognitive behavioral therapy in the management of pediatric migraine, focusing on the application of technological advances to improve care as well as how to reduce healthcare disparity related to access to therapy. Speakers will later present other non-pharmacologic treatment options, including neuromodulation devices (non-invasive vagus nerve stimulation, transcranial magnetic stimulation, and trigeminal nerve stimulation) and procedural treatments (onabotulinumtoxinA and peripheral nerve block injections). Discussion will include mechanism of action and current studies to support the use of these treatments in children and adolescents.

Our hope is to equip clinicians taking care of children and adolescents with pediatric migraine with novel tools for treatment that may lead to reduced pain and ultimately reduced disability among these patients.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- initiate appropriate pharmacologic and non-pharmacologic therapy for acute and preventative treatment of children and adolescents with migraine based on a patient's individual preferences and characteristics.
- describe the new technological applications to cognitive behavioral therapy in children and adolescents.
- explain when to consider anti-CGRP medications, neuromodulation devices, and procedural treatment for pediatric headache management.
- understand disparities in care for children with migraine and identify steps to mitigate these disparities.

IMPACT STATEMENT

This educational session helped me to identify changes I could make in my practice related to:

- prescribing new therapies in the management of pediatric migraine, including anti-CGRP medications and neuromodulation devices
- explaining to patients expectations for cognitive behavioral therapy
- deciding when to refer for procedural management for pediatric migraine
- reducing healthcare disparities in children with migraine

ORGANIZER

Marielle Kabbouche Samaha, MD, FAHS, FAAN
Cincinnati Children's Hospital, Cincinnati, OH

Neuromodulators: "It's Electric!"

Joanne Kacperski, MD, FAHS
Cincinnati Children's Hospital Medical Center,
University of Cincinnati, Cincinnati, OH

Proceeding with Procedures:

The When, The Why and The How

Rachel Gottlieb-Smith, MD, MHPE
University of Michigan, Ann Arbor, MI

CGRP Therapies for Children and Adolescents?

Daniel N. Lax, MD
Montefiore Medical Center, Albert Einstein
College of Medicine, Bronx, NY

Behavioral Therapies for Migraine:

Applications, Impact and Future

Shalonda Slater, PhD
Cincinnati Children's Hospital Medical Center,
Cincinnati, OH

12:30 PM – 1:45 PM

Seminar 9: Prognostic Dilemmas in Fetal Neurology: What Would You Do?

DESCRIPTION

Fetal neurology is a rapidly evolving field with advancements in neuroimaging and genetic testing leading to more accurate prenatal diagnosis. Fetal neurological consultations are commonly initiated based on brain malformations or destructive lesions documented by fetal surveillance with sonography

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followed by fetal MRI. Diagnostic challenges contribute to uncertainties in prenatal counseling and relate to resolution limitations of present prenatal genetic testing and neuroimaging. This seminar presents commonly encountered fetal neurologic cases. Focused presentations followed by moderated panel discussions highlight clinical decision-making in fetal-neonatal neurology that influence diagnostic considerations and outcome predictions.

This seminar represents ongoing efforts of the Fetal Neurology Consortium, founded in 2020, to address current clinical challenges in fetal neurology. The course is designed for fetal-neonatal neurologists, child neurologists and trainees with special interests in developmental neurology.

LEARNING OBJECTIVES

- As a result of attending this educational session, participants will be able to learn about the diagnostic and prognostic challenges in fetal neurology that influence diagnostic accuracy across developmental time.
- As a result of this educational session, participants will be able to understand the inherent variability in prognostic counseling, complex perinatal management, decision-making, and outcome-prediction for fetal neurologic disorders.

IMPACT STATEMENT

This education session will help attendees identify changes they can make in their pediatric neurology practice related to:

- This educational session will help attendees identify changes they can make in their pediatric neurology practice related to diagnostic evaluation and prognostic counseling process in the context of fetal neurologic disorders.
- This educational session will help attendees identify changes they could make in their pediatric neurology practice related to perinatal management and interdisciplinary care for fetal neurologic disorders.

ORGANIZER

Sonika Agarwal, MBBS, MD
Children's Hospital of Philadelphia,
University of Pennsylvania, Philadelphia, PA

Introduction

Sonika Agarwal, MBBS, MD
Children's Hospital of Philadelphia, University of Pennsylvania, Philadelphia, PA.

Focused case discussions with learning objectives that are moderated by panel discussion and Q&A at the end.

PANELISTS

Dawn Gano, Vann Chau, Sarah Mulkey, Sonika Agarwal, Mark Scher

Case 1: Sarah Mulkey

Sarah Mulkey, MD, PhD
Prenatal Pediatrics Institute, Children's National Hospital, Washington, DC

Case 2: Vann Chau

Vann Chau, MD, FRCPC
The Hospital for Sick Children, University of Toronto, Toronto, Canada

Case 3: Dawn Gano

Dawn Gano, MD, MAS
UCSF Weill Institute for Neurosciences,
San Francisco, CA

Mark Scher (panelist)

Mark Scher, MD
University Hospitals of Cleveland, Rainbow Babies and Children, Cleveland, OH

2:15 PM – 4:00 PM

Platform I

PL1-1 Anne Anderson, MD

Drug resistant epilepsy in a mouse model of TSC: in vivo and ex vivo analysis

PL1-2 Juliet Knowles, MD, PhD

Myelin Plasticity Promotes Thalamocortical Hypersynchrony and Generalized Epilepsy Progression

PL1-3 Kirsty McWalter

Exome-based testing for patients with seizures identifies genetic diagnoses missed by panel based testing

PL1-4 Alexandra Santana Almansa, MD

Retrospective, multicenter study of lacosamide to treat neonatal seizures

PL1-5 Hui Li, MD, PhD

Neurocognitive outcomes of neonates with HIE and normal MRIs

PL1-6 Jaclyn Martindale, DO

Gender Representation in Leadership Roles and Awards in the Child Neurology Society

SESSIONS highlighted in maroon are designated for CME credit. Agenda and amount of CME credits available are subject to change.

Friday, October 6 CONTINUED

PL1-7 Aleksandra Zakharova, MD

Hyperkinetic dystonia in dyskinetic cerebral palsy responds to combined DBS stimulation in thalamus and pallidum.

2:15 PM – 4:00 PM

Platform II

PL2-1 Mary Rolfes, MD

Host transcriptomics identifies unique host gene expression patterns in childhood arterial ischemic stroke

PL2-2 Claudia Gambrah-Lyles, MD

Assessing needs and perceptions of research participation in pediatric onset multiple sclerosis: a multi-stakeholder survey

PL2-3 Alexander Sandweiss, MD, PhD

Infectious profiling reveals potential triggers of pediatric NMDAR encephalitis: a large case control study

PL2-4 Milena Andzelm, MD, PhD

Human Genetic Modulation of the Neuronal Response to Interferons

PL2-5 Sheffali Gulati, FRCPCH (UK), FAMS, FIAP, FIMSA

Sleep related breathing disorder in 5-18 years children with dystrophinopathy: a cross sectional study

PL2-6 Nausheen Hasan, PharmD

Interim Analysis of EVOLVE: A Long-term Observational Study Evaluating Eteplirsen, Golodirsen, or Casimersen in Routine Clinical Practice

PL2-7 Miranda Creasey, MD

Representation of Non-White Participants in Interventional Duchenne Muscular Dystrophy (DMD) Clinical Trial Publications

2:15 PM – 4:00 PM

Platform III

PL3-1 Jonathan Santoro, MD

Immunotherapy Responsiveness and Risk of Relapse in Down Syndrome Regression Disorder

PL3-2 Rose Gelineau-Morel, MD

Investigation of the biotransformation of trihexyphenidyl and application to dystonic cerebral palsy

PL3-3 Patricia Musolino, MD, PhD

Interim Results from the NEXUS Open-Label Phase 2 Study on the Safety and Efficacy of Leriglitzone in the Treatment of Childhood Cerebral Adrenoleukodystrophy

PL3-4 Man Amanat, MD, MPH

A Comprehensive Approach for Designing Effective Antisense Oligonucleotides in the Treatment of Inherited White Matter Disorders

PL3-5 Joshua Bonkowsky, MD, PhD

Astrocyte-targeted gene therapy demonstrates safety and efficacy in two murine models of Vanishing White Matter disease

PL3-6 Jennifer Harmon, MD, PhD

Development of a Clinical-Translational Model of Leukoencephalopathy with Calcifications and Cysts

PL3-7 Marc Patterson, MD

Evaluation of the Long-Term Effect of Arimocloamol in NPC – 48 Months Data from CT-ORZY-NPC-002

4:30 PM – 5:00 PM

CNS Business Meeting

4:30 PM – 5:00 PM

Junior Member Forum

5:15 PM – 6:00 PM

Junior Member Forum Breakout Sessions

5:30 PM – 7:00 PM

Scientific Program and Planning Committee Meeting

7:00 PM – 9:00 PM

Closing Gala

SESSIONS highlighted in maroon are designated for CME credit. Agenda and amount of CME credits available are subject to change.

Saturday, October 7

8:00 AM – 8:45 AM

Howe Award Lecture

Phillip L. Pearl, MD

The Neurology of Creativity

9:00 AM – 11:45 AM

Symposium IV: Year in Review

ORGANIZERS

Grace Gombolay, MD
Emory University/Children's Healthcare of Atlanta,
Atlanta, GA

Dave Clarke, MD
University of Texas Dell Medical School, Austin, Texas

COURSE DESCRIPTION

With new exciting advancements in child neurology, in areas of clinical care, research, new medications, gene therapies, and new technologies, this session provides an overview within different pediatric neurology subspecialties. Recent advancements in the past 1-2 years will be covered in this symposium by experts in the field, with a focus on studies that are relevant to the general child neurologist. The topics will include epilepsy, neonatal neurology, stroke/neurocritical care, neuroimmunology, neuromuscular, neuro-oncology, diversity/equity/inclusion, movement disorders, headaches, and neurogenetics. Studies will include multi-center consortia and clinical trials. This symposium will provide an update on the most relevant and high-impact studies within child neurology.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- Identify high impact studies that have been published in child neurology in the past 1-2 years
- Discuss novel therapies and treatments available for pediatric neurological disorders

IMPACT STATEMENT

This educational session will help attendees to identify changes they can make in their practices related to:

- Evidence based practices for management of pediatric neurological diseases
- Discussing important studies in pediatric neurology

Neonatal Neurology

Dawn Gano, MD, MAS
UCSF Weill Institute for Neurosciences,
San Francisco, CA

Genetic Epilepsies

Annapurna Poduri, MD
Boston Children's Hospital, Boston, MA

General Epilepsy

Sucheta Joshi, MD
C.S. Mott Children's Hospital, University of Michigan,
Ann Arbor, MI

Diversity, Equity and Inclusion

Diana Cejas, MD, MPH
Carolina Institute for Developmental Disabilities (CIDD)
University of North Carolina at Chapel Hill,
Chapel Hill, NC

Neuroimmunology

Jennifer Yang, MD
UC San Diego and Rady Children's Hospital San Diego,
San Diego, CA

Neuromuscular

Michael Lopez, MD, PhD
University of Alabama at Birmingham,
School of Medicine, Birmingham, AL

Movement Disorders

Michael Kruer, MD
University of Arizona College of Medicine – Phoenix,
Phoenix, AZ

Pediatric Stroke and Neurocritical Care

Lisa Sun, MD
The Johns Hopkins Hospital, Baltimore, MD



CNF Young Researcher Grants and Medical Student Scholarship profiles appear in the summer issue of the *CNS Connections* magazine.

Saturday, October 7 CONTINUED

Neuro-oncology

Fatema Malbari, MD
Neurology Brain Tumor Program Epilepsy Center
Neurofibromatosis Clinic, Texas Medical Center,
Houston, TX

Headache

Shawn Aylward, MD
Nationwide Children's Hospital, The Ohio State
University College of Medicine, Columbus, OH

Neurogenetics

Andrea Gropman, MD
George Washington School of Medicine and Health
Sciences, Children's National Hospital, Washington D.C.

12:15 PM – 4:15 PM

CNS Clinical Research Annual Workshop: 2023 – Pediatric Neurology Clinical Trials – Outcome Measures

DESCRIPTION

This course is a 4-hour clinical research workshop providing interactive training on specific research methodology topics to support clinical research engagement by all CNS members regardless of prior clinical research experience.

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- Have an understanding of different types of outcome measures including what constitutes an outcome measure, how to design them and how to track them.
- Appropriately develop and validate outcome measures for their specific area of research.
- Support their clinical research by incorporating patient perspectives into outcome measure development.

IMPACT STATEMENT

This educational session helped me to identify changes I could make in my practice related to:

- Initiate new clinical research projects.
- Meaningful engage in existing clinical research projects.

ORGANIZER

Ariel Maia Lyons-Warren, MD, PhD; Baylor College of Medicine, Houston, TX

CO-ORGANIZER

Rose Gelineau-Morel, MD
Children's Mercy Hospital, Kansas City, KS

Josh Bonkowsky, MD, PhD
University of Utah School of Medicine, Primary
Children's Hospital, Salt Lake City, UT

Janet Soul, MDCM, FRCPC
Boston Children's Hospital, Harvard Medical School,
Boston Mass, Boston, MA

Angela Hewitt, MD, PhD
University of Rochester Medical Center, Rochester, NY

Daniel Calame, MD, PhD
Baylor College of Medicine, Houston, TX

Mustafa Sahin, MD PhD
Boston Children's Hospital, Harvard Medical School,
Boston Mass, Boston, MA

Welcome

Ariel Maia Lyons-Warren, MD, PhD
Baylor College of Medicine, Houston, TX

Introduction to Outcomes Measures

Kristi Hardy, PhD
National Institute of Neurological Disorders & Stroke,
Rockville, MD

Coffee Break & Networking

Case Report Forms and Database Management

Janet Soul, MDCM, FRCPC
Boston Children's Hospital, Harvard Medical School,
Boston Mass, Boston, MA

Breakout Sessions

Finding and Adapting Fit-for-purpose Clinical Outcome Measures: FDA-Patient-Focused Drug Development Guidance

Anne Berg, PhD
Northwestern-Feinberg School of Medicine,
Department of Neurology and Decoding
Developmental Epilepsies

Panel Discussion

Q&A

SESSIONS highlighted in maroon are designated for CME credit. Agenda and amount of CME credits available are subject to change.

Join us for a Satellite CME Lunch Symposium

Lennox-Gastaut Syndrome in the Real World

A Comprehensive Approach to Diagnosis and Management

**Friday,
October 6, 2023**

11:45 AM – 1:45 PM

Vancouver Convention Centre
West Building
Meeting Room 211, Level 2

Program Chair

Elaine C. Wirrell, MD, FRCPC

*Professor and Chair of Child Neurology
Consultant Child Neurologist
Mayo Clinic
Rochester, Minnesota, USA*



Pre-Register at

www.millermeded.com/LGS23 or scan QR code

Pre-registration does not guarantee seating. On-site registration may be available, space permitting.



This activity has been approved for AMA PRA Category 1 Credit™.

Jointly provided by Partners for Advancing Clinical Education (PACE) and Miller Medical Communications, LLC.



This activity is supported by an independent educational grant from UCB, Inc.

Saturday, October 7 CONTINUED

12:15 PM – 4:15 PM

Biomedical Writing Workshop

DESCRIPTION

This four-hour small group interactive workshop is designed to help novice medical writers overcome common barriers to successful publication. We review common correctable reasons for manuscript rejection and teach effective techniques for overcoming writer's block. Using text examples, we illustrate techniques and pointers for improving manuscript quality. We teach effective methods to respond to revision requests and interact with journal editors. The session ends with a casual meet-the-editors question-and-answer period, featuring several individuals who are current or former medical editors. Major topics include:

- (1) Introduction: Why Manuscripts are Rejected and Simple Ways to Improve a Manuscript
- (2) Techniques for Outwitting Writer's Block
- (3) Responding to Reviews and Revising Your Manuscript
- (4) Rules of the Road: Permissions, Consents, and Other Potholes
- (5) Meet the Editors Q & A

LEARNING OBJECTIVES

As a result of this educational session, participants will be able to:

- recognize barriers to successful publication
- develop strategies for overcoming writer's block

- be able to more effectively revise manuscripts and respond to reviewers and editors
- understand the requirements for republication, use of patient materials, and privacy concerns.

IMPACT STATEMENT

This educational session helped me to identify changes I could make that will:

- allow me to improve the quality of the manuscripts submitted for publication
- allow me to more effectively revise manuscripts following journal peer reviews

ORGANIZER

E. Steve Roach, MD
Editor-in-Chief of the
Annals of the Child Neurology Society
University of Texas Dell Medical School, Austin, TX

PRESENTERS

Grace Gombolay, MD
Associate Editor of the
Annals of the Child Neurology Society
Emory University/Children's Healthcare of Atlanta,
Atlanta, GA

Yasmin Khakoo, MD, FAAN, FAAP
Editor-in-Chief, *Pediatric Neurology*
Sloan-Kettering Cancer Institute, New York, NY

Dave Clarke, MD
Associate Editor, *ACNS*
University of Texas Dell Medical School, Austin, Texas



SESSIONS highlighted in maroon are designated for CME credit. Agenda and amount of CME credits available are subject to change.

The CNS Junior and Early Career Forum

BY ALEXANDER COHEN, MD, PHD AND ARIEL LYONS-WARREN, MD, PHD

The Junior and Early Career Forum (now in its second year) was born out of an “International Collaboration of the Young Members” spanning the international child neurology organizations including ICNA, AOCNA, ACNA, EPNS, and the CNS. Representatives from each of these international/regional child neurology societies meet quarterly to share ideas and discuss new programs that could benefit Junior Child Neurologists worldwide. One feature of many of the international child neurology associations is a separate organization or forum to give a voice to junior and early career members, leading to our creation of the JECF.

The CNS has long supported a number of programs highlighting and benefitting our trainees and early career members, including:

1. Free Medical Student/Resident Trainee Membership and reduced rate meeting attendance.
2. The CNS Bhuwan Garg High School Neuroscience Prize and multiple Outstanding Junior Member awards at the Annual Meeting.
3. The Child Neurologist Career Development Program (CNCDP-K12) and Minority Research Scholars Programs.
4. The John M. “Jack” Pellock Resident Seminar on Epilepsy for CN and NDD residents in their final year of training.
5. Early Career Research Awards including CNF and PERF partner program grants and the Philip R. Dodge Young Investigator Award.

In addition to these awards and programs that highlight and support early research and academic achievement, the Child Neurology Society is expanding its junior member and early career programming with a Wednesday seminar on Navigating the Landscape of Publication and Leadership in Child Neurology as well as brief career-focused “lobby talks” interwoven throughout the meeting that will be given by early/mid-career members with the theme of “Things I knew last year.” The lobby talks will be held in a dedicated “open” meeting space (the 2nd floor in the City Foyer) to allow attendees to listen in between scientific sessions while they grab coffee, tea, and snacks. Topics will include practical issues such as: what to look for in a first job offer, how to network at a meeting, how to find a mentor, and when and how to ask for a raise.

Finally, we will host a junior member and early career open forum on Friday night, before the closing reception, to discuss topics and needs particular to our trainee and junior faculty members well as leadership opportunities. This open forum will conclude with breakout sessions for our trainee members on:

1. Choosing your Residency Program.
2. Finding a Fellowship Position.
3. Finding Your First Job.

All of these talks and opportunities are open to all who consider themselves in training, early career... or just “young at heart.”

WEDNESDAY, OCTOBER 4

Looking for a job and job transitions

- 7:00 AM What to look for in a job offer: young academic perspective
- 7:15 AM What to look for in a job offer: young private practice perspective
- 7:30 AM Do I need a recruiter?
- 7:45 AM Effort, salary, and fractions
- 11:45 AM How to read a P&L statement
- 12:00 PM Searching for a job with dual physician households
- 12:15 PM Should I stay or should I go? Transitioning between academia, private practice, and industry
- 2:00 PM Navigating the Landscape of Publication and Leadership in Child Neurology

THURSDAY, OCTOBER 5

Growing in your career, day 1

- 7:00 AM How do you start a multi-disciplinary/sub-speciality clinic?
- 7:15 AM Making sense of the NIH “alphabet” soup
- 7:30 AM How to network at a meeting
- 7:45 AM Establishing a career as a clinician-education in academic departments
- 11:30 AM How to find mentors at your (existing/new) institution
- 11:45 AM How to become a good mentor
- 12:00 PM Virtual teaching in current era of medical education
- 12:15 PM Use of social media for professional development
- 4:30 PM Advertising your practice
- 4:45 PM Creating a business plan

FRIDAY, OCTOBER 6

Growing in your career, day 2

- 7:00 AM Reviewing a paper
- 7:15 AM Responding to reviews
- 7:30 AM When and how to ask for a raise in academia
- 7:45 AM When and how to ask for a raise in private practice
- 4:30 PM Junior Member Forum
- 5:15 PM Junior Member Seminar 1: Medical Students: Finding a residency
- 5:15 PM Junior Member Seminar 2: Residents: Finding a fellowship
- 5:15 PM Junior Member Seminar 3: Residents and Fellows: Getting your first job

Industry-Sponsored Satellite Sessions

Industry-sponsored satellite sessions are independently staged, accredited, or non-accredited educational or product theater events. Corporate partners pay a gateway fee to make these accessible to attendees.

Satellite Seminar

Thursday, October 5

Recognizing Rett Syndrome Early to Improve Long-term Management Outcomes

12:30 PM – 2:30 PM

Vancouver Convention Centre, Room 211

PRE-REGISTRATION

<https://event.gotoper.com/event/c2848314-2ecb-4af8-afac-389436bab47e/regProcessStep1:3b955580-e298-4008-9bde-f09067f3bfed>



Note: Pre-Registration does not guarantee seating. Onsite registration may be available, space permitting.

PROGRAM DESCRIPTION

This program on Rett syndrome aims to address several gaps in knowledge related to this rare neurodevelopmental disorder. The program will focus on improving knowledge and competence in diagnosing RTT by utilizing appropriate diagnostic criteria and genetic testing. The program will also highlight current recommended treatment strategies for optimal management of RTT to improve long-term patient outcomes and quality of life. In addition, the program will cover the development of comprehensive, individualized treatment plans to manage individuals with RTT from childhood through adulthood. By addressing these gaps in knowledge and competence, healthcare professionals can improve patient outcomes and provide better care for individuals with RTT throughout their journey.

LEARNING OBJECTIVES

- Implement guideline recommendations for the early diagnosis of RTT based on patient age and evaluation of the clinical presentation of characteristic symptoms during early growth and development
- Summarize current guideline recommended treatment strategies for the optimal management of RTT from childhood through adulthood

- Evaluate clinical trial efficacy and safety data for new and emerging therapies used to treat RTT
- Develop a personalized, multidisciplinary team coordinated management plan for individuals with RTT that incorporates approved treatment options and ongoing management from childhood through adulthood

SPEAKERS

TBA

CREDITS AVAILABLE

This activity will be designated *1.5 AMA PRA Category 1* credits for physicians and *1.5 contact hours* for nurses.

GRANT/FUNDING SOURCE

Acadia Pharmaceuticals Inc.

Product Theater

Thursday, October 5

Theranica USA

Adolescent Migraine: From Burden to Treatment

1:00 PM – 2:00 PM

Vancouver Convention Centre, Exhibit Hall

PROGRAM DESCRIPTION

Remote Electrical Neuromodulation (REN) provides an indicated, drug-free, dual-use product that lessens the burden of migraine for adolescent patients ages 12+. This session will focus on how to treat adolescent patients with effective, safe, and indicated treatments. The program will highlight how the adolescent migraine patient has been significantly underserved. Many of the treatments available add to the daily burden of migraine with unfavorable side effects. Discussion on the burden of migraine for adolescents, from school to home life, will lead to the goals of treatment and considerations of putting adolescents on drug therapy. Clinical data on all approved options for adolescents will be presented. The session will close with a highlight on Nerivio and the role that it plays in providing drug-free, dual-use treatment for adolescents ages 12 and over.

Speakers

Marielle Kabbouche Samaha, MD
Cincinnati Children's Hospital
Director, Acute and Inpatient Headache Program
Child Neurologist, Division of Neurology
Professor, UC Department of Pediatrics

Rashmi Rao, MD
Assistant Professor of Clinical Neurology at
LSU Health New Orleans
Pediatric Headache Specialist at
Children's Hospital New Orleans

Satellite Seminar

Friday, October 6

Lennox-Gastaut Syndrome in the Real World: A Comprehensive Approach to Diagnosis and Management

11:45 AM – 12:15 PM: On-site Check-in and Lunch

12:15 PM – 1:45 PM: Satellite CME

Vancouver Convention Centre, Room 211

Pre-Registration

www.millermeded.com/LGS23

Note: Pre-Registration does not
guarantee seating. Onsite registration
may be available, space permitting.



PROGRAM DESCRIPTION

The symposium will focus on the clinical diagnosis and management of patients with Lennox-Gastaut syndrome (LGS), with a review of primary and secondary outcomes from recent clinical trials and how those findings translate into clinical practice.

LEARNING OBJECTIVES

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

- Identify clinical features and diagnostic challenges associated with Lennox-Gastaut syndrome

- Discuss clinical applications of seizure end points from clinical trials of Lennox-Gastaut syndrome
- Summarize the impact of anti-seizure medications on comorbidities in patients with Lennox-Gastaut syndrome

FACULTY

Elaine C. Wirrell, MD, FRCPC (Program Chair)
Professor and Chair of Child Neurology
Consultant Child Neurologist
Mayo Clinic
Rochester, Minnesota, USA

Elizabeth A. Thiele, MD, PhD
Professor of Neurology
Harvard Medical School
Director, Pediatric Epilepsy Program
Director, Herscot Center for Tuberous Sclerosis
Complex
Massachusetts General Hospital
Boston, Massachusetts

Joseph E. Sullivan, MD
Murphy Parker Endowed Professor in
Pediatric Epilepsy
Professor of Neurology and Pediatrics
University of California, San Francisco (UCSF)
Director, UCSF Benioff Children's Hospital Pediatric
Epilepsy Center of Excellence
San Francisco, California

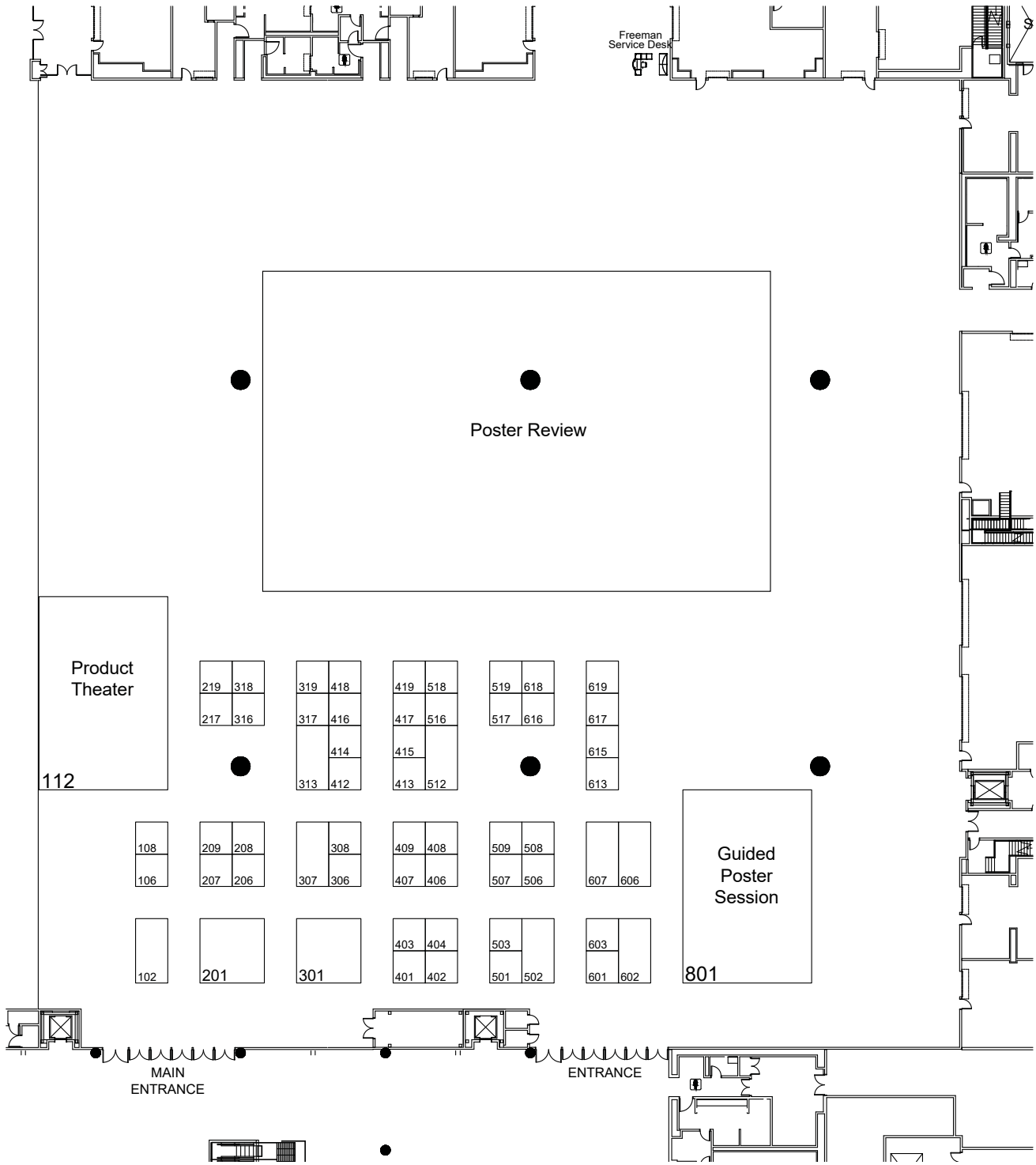
CREDITS AVAILABLE

This activity is approved for *AMA PRA Category 1
Credit™*

GRANT/FUNDING SOURCE

Jointly provided by Partners for Advancing Clinical
Education (PACE) and Miller Medical Communications,
LLC. This activity is supported by an independent
educational grant from UCB, Inc.

Vancouver Convention Centre West – Halls A-B1



Exhibitors

PREMIER SPONSOR

Acadia Pharmaceuticals Inc. (#301)

Acadia is trailblazing breakthroughs in neuroscience to elevate life through science. For more than 25 years, we have been working at the forefront of healthcare to bring vital solutions to people who need them most. Visit our Booth #301. For more information, visit us at www.acadia.com and follow us on LinkedIn and Twitter.



American Board of Psychiatry & Neurology, Inc. (ABPN) (#419)

The American Board of Psychiatry and Neurology serves the public interest and the professions of psychiatry and neurology by promoting and assessing the competence of psychiatrists and neurologists and by establishing standards for initial and continuing certification programs.

Association of Child Neurology Nurses (ACNN) (#508)

The Association of Child Neurology Nurses is an international nonprofit organization of nurses and other health care professionals caring for children with neurological conditions. ACNN provides educational opportunities at national and regional conferences, virtual learning through online webinars, nursing excellence awards, research support, newsletters, and opportunities for networking. Additional information can be found at www.neurologynurses.org.

Barrow Neurological Institute at Phoenix Children's (#319)

Barrow Neurological Institute at Phoenix Children's is a premier center of excellence and destination for managing and treating individuals with complex neurologically-related pediatric disorders by providing comprehensive, compassionate, state of the art patient and family-centered care. Come visit us at our booth #319 to learn more about the innovative education training programs we have to offer.

BioMarin Pharmaceutical Inc. (#502)

BioMarin is a world leader in developing and commercializing innovative therapies for rare diseases driven by genetic causes. With a 20-year history, BioMarin remains steadfast to its original mission – to bring new treatments to market that will make a big impact on small patient populations. Visit www.biomarin.com to learn more.

Bionano Laboratories (#316)

Bionano Laboratories provides access to genetic answers and support utilizing cutting-edge technologies to advance the way you see the genome. With a comprehensive clinical genetic testing offering that includes microarray, panels, whole exome sequencing, and optical genome mapping, the Bionano Laboratories team is dedicated to finding answers. Learn more: www.bionanolaboratories.com

Catalyst Pharmaceuticals, Inc. (#404 & 503)

Catalyst is committed to developing and commercializing innovative first-in-class medicines that address rare neurological and epileptic diseases.

Children's Healthcare of Atlanta (#413)

Children's Healthcare of Atlanta Neurosciences is a nationally ranked department and is a part of the #1 pediatric health system in the Southeast. With over 12 subspecialty programs and 40,000 patient encounters annually, CHOA Neurosciences is the largest provider of neurological health in the region.

Children's Mercy Kansas City (#507)

Children's Mercy Kansas City is a leading independent non-profit, 390-bed pediatric health system dedicated to holistic care, translational research, breakthrough innovation, and educating the next generation of caregivers. Together, we transform the health, well being and potential of children, with unwavering compassion for those most vulnerable.

Children's National Hospital (#517)

Children's National Hospital, based in Washington, D.C., was established in 1870 to help every child grow up stronger. Today, it is the No. 5 children's hospital in the nation, #3 for Neurology and ranked in all specialties evaluated by *U.S. News & World Report*, transforming pediatric medicine for all children.

Exhibitors

Dayton Children's Hospital (#617)

Dayton Children's is a freestanding children's hospital with more than 35 specialties serving a pediatric population of 510,000 from a 20-county region of central and southwestern Ohio and eastern Indiana. The hospital is a designated Level 1 pediatric trauma center. In 2022, Forbes named Dayton Children's a Best In-State Employer!

Egetis Therapeutics, US (#418)

At Egetis Therapeutics, we **"care for the rare"**, focusing our efforts on developing treatments for serious rare diseases where there are no current therapeutic options. Our aim is to ensure patients with the rarest of diseases can access the treatments they need to improve and extend their lives.

Emalex Biosciences Inc. (#516)

Emalex Biosciences, a portfolio company of Paragon Biosciences, is committed to improve the lives of people living with CNS disorders. Emalex is conducting a Phase 3 clinical study of a new class of drug for patients with Tourette syndrome who have limited treatment options.

First Choice Neurology (#519)

First Choice Neurology is Physician-Owned/Physician-Managed with Pediatric and Adult Neurologists. We are established in multiple Florida counties and provide services at over 45 major hospitals. We are actively growing. Our goal is to connect and build a better future together.

Fluffy Friends for Children with Chronic Conditions (#407)

Fluffy Friends for Children with Chronic Conditions is a trauma-informed program that enhances physician rapport. The goals are to ensure patients feel safe and secure in their physicians' care enough to ameliorate medically induced post-traumatic stress disorder and mitigate physician burnout by improving work-life fulfillment.

Fulgent Genetics (#414)

Fulgent Genetics is a full-service genomic testing company built around a foundational technology platform. Through our diverse testing menu, Fulgent is focused on transforming patient care in pediatric neurology, as well as in oncology, anatomic pathology, infectious and rare diseases, and reproductive health.

GeneDx (#602)

GeneDx delivers improved health outcomes through genomic and clinical insights. The company is at the forefront of transforming healthcare through its industry-leading exome and genome testing and interpretation, fueled by one of the world's largest rare disease data sets.

IntraNerve Neuroscience (#603)

IntraNerve Neuroscience (INN) is Joint Commission accredited in Ambulatory Care – Telehealth. We offer EEG, Intraoperative Neuromonitoring, and Remote Physician Interpretation. Our Neurologists/Epileptologists, Technologists, and IT support are dedicated to providing care and assistance 24/7/365. We partner with facilities like yours across the country to provide high quality, reliable neuroscience services.

Invitae (#106)

Leading medical genetics company trusted by patients and providers to deliver genetic information using digital technology. Provide accurate and actionable answers to strengthen medical decision-making for individuals and their families. Applying a rigorous approach to data and research to bring comprehensive genetic information into mainstream medicine. Visit www.invitae.com

Ionis Pharmaceuticals, Inc. (#509)

For more than 30 years, Ionis has been a leader in RNA-targeted therapy, pioneering new markets and changing standards of care. Knowing that sick people depend on us fuels our vision to become the leader in genetic medicine, utilizing a multi-platform approach to discover, develop, and deliver life-transforming therapies.

J2 Bio-Pharma (#217)

J2 Bio-Pharma is an organization with a focus on child friendly epilepsy products. Our company's primary focus is to provide more medical options to neurologists and epileptologists that can help improve the quality of life for children living with epilepsy. We invite you to visit our booth to gain more insights about our organization and other promising products we may have in the pipeline.

PARTNER SPONSOR

Jazz Pharmaceuticals (#307)

Jazz Pharmaceuticals is a global biopharmaceutical company whose purpose is to innovate to transform the lives of patients and their families. We are dedicated to developing life-changing medicines for people with serious diseases – often with limited or no therapeutic options. Please visit www.jazzpharmaceuticals.com for more information.



Jazz Pharmaceuticals Epilepsy Clinical Trials (#506)

Jazz Pharmaceuticals Epilepsy Clinical Trials Division is working on a diverse and exciting pipeline in neuroscience, including development in epilepsy and other neurological disorders. Jazz Pharmaceuticals, founded in 2003, is a global biopharmaceutical company dedicated to developing life-changing medicines for people with serious diseases. For more information on the company's epilepsy-focused clinical trials, please visit www.jazzepilepsyclinicaltrials.com

Johns Hopkins All Children's Hospital (#318)

Johns Hopkins All Children's Hospital started in 1926 in St. Petersburg, FL as a community effort to combat polio. Today, it is a 259-bed teaching hospital and regional referral center. It is the only one of three Johns Hopkins academic medical centers outside of the Baltimore/Washington, D.C. region.

Kennedy Krieger Institute (#408)

Located in the Baltimore-Washington, D.C., region, Kennedy Krieger Institute is internationally recognized for improving the lives of tens of thousands of children, adolescents and adults with neurological, rehabilitative or developmental needs through inpatient and day hospital programs, outpatient clinics, home and community services, education, and research.

LivaNova (#409 & 616)

The VNS Therapy™ System is indicated for use as an adjunctive therapy in reducing the frequency of seizures in patients 4 years of age and older with partial onset seizures that are refractory to antiepileptic medications.

Marinus Pharmaceuticals (#317 & 306)

Marinus is a commercial stage pharmaceutical company dedicated to the development of innovative therapeutics for seizure disorders. Marinus is investigating the potential of ganaxolone, a neuroactive steroid GABAA receptor modulator, in IV and oral formulations to maximize therapeutic reach in acute and chronic care settings. For more information visit www.marinuspharma.com.

National Institute of Neurological Disorders and Stroke (NINDS) (#406)

The National Institute of Neurological Disorders and Stroke (NINDS), part of the National Institutes of Health, provides research support, common data elements, clinical trials, a Migraine Trainer app, and free publications for patients and families on epilepsy, Batten disease, headache, brain injury, and other neurological disorders.

Nationwide Children's Hospital (#607)

Nationwide Children's is ranked among the 10 best children's hospitals for Neurology and Neurosurgery by US News. Unique areas of focus include movement disorders, stroke, spinal muscular atrophy and muscular dystrophy – including groundbreaking clinical and translational research. We are also top 10 in NIH funding among freestanding children's hospitals.

Neurelis, Inc. (#313 & 412)

Neurelis, Inc. is an innovation-driven neuroscience company focused on the development and commercialization of product candidates and innovative delivery technologies for the broader central nervous system (CNS), including epilepsy and psychiatry. In 2020, Neurelis reached a milestone in patient care with its first FDA-approved treatment. For information, please visit <http://www.neurelis.com>.

Neurogene (#619)

Neurogene is developing life-changing genetic medicines for patients and their families affected by rare, devastating neurological diseases. Come by and learn about Neurogene's EXACT technology and NGN-401, our latest gene therapy to enter the clinic for females ages 4 to 10 years old with typical Rett Syndrome.

Exhibitors

Nicklaus Children's Hospital (#403)

The Nicklaus Children's Hospital Brain Institute offers comprehensive evaluation and treatment for complex pediatric neurological disorders, including intractable epilepsies and brain tumors. It is 22 in the nation and best in Florida for pediatric neurology & neurosurgery in the 2023-24 U.S. News and World Report Best Children's Hospital rankings. nicklauschildrens.org/Brain

Norton Children's Medical Group, Norton Healthcare (#108)

Norton Children's Medical Group, affiliated with the UofL School of Medicine, is the pediatric provider division of Norton Medical Group. NCMG encompasses all pediatric care, including inpatient, outpatient, primary and specialty care and employs more than 400 medical providers who work in over 170 locations in Kentucky and Southern Indiana.

Novartis Gene Therapies (#606)

Novartis Gene Therapies is reimagining medicine to transform the lives of people living with rare genetic diseases. Utilizing cutting-edge technology, we are working to turn promising gene therapies into proven treatments.

OSF Healthcare/Children's Hospital of Illinois (#308)

The University of Illinois College of Medicine (Peoria) in conjunction with the Children's Hospital of Illinois is seeking a Child Neurologist to join our team. CHOI is a major medical facility with 124 beds and a 32-bed critical care unit and the only Level 1 trauma center in downstate Illinois.

Pediatric Epilepsy Surgery Alliance (#402)

The Pediatric Epilepsy Surgery Alliance (PESA) is the only non-profit organization whose sole focus is serving the community of children who need neurosurgery to treat their seizures. We empower families with research, support services, and impactful programs before, during, and after surgery. Formerly known as The Brain Recovery Project.

Pediatric Search Partners (#618)

Pediatric Search Partners is a boutique search firm specializing in physicians and leaders dedicated to children's healthcare, including pediatric neurology and epilepsy. Our seasoned team has over 75 years of collective experience in physician and executive recruitment within healthcare. Since 2009, we have completed over 650 successful searches connecting those who care for children to life-giving opportunities.

Pediatrix Medical Group (#613)

Our physician-led health care organization partners with hospitals, health systems and health care facilities to offer clinical services across numerous specialties and subspecialties supporting the continuum of care from birth through adulthood.

Pfizer (#615)

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development, and manufacture of health care products, including innovative medicines and vaccines.

PreventionGenetics (#208)

Located in Marshfield, WI, PreventionGenetics is a CLIA and ISO 15189:2012 accredited laboratory founded in 2004 and acquired in 2021 by Exact Sciences. PreventionGenetics provides tests for nearly all clinically relevant genes including our whole-genome sequencing test, PGnome®, and whole-exome sequencing test, PGxome®.

PTC Therapeutics, Inc. (#206)

PTC is an established global biopharmaceutical company that delivers transformative therapies for people living with rare diseases. For 25 years, we have been harnessing our scientific platforms to create new therapies that address the underlying cause of the disease and deliver on our promise to create more moments for patients.

Rady Childrens Hospital San Diego (#501)

Rady Children's Hospital-San Diego is a nonprofit, 511-bed pediatric-care facility dedicated to excellence in care, research and teaching in San Diego, California. The Rady Children's Division of Neurology ranks among the top pediatric programs in the nation and are dedicated to providing the highest quality care.

Religen Inc. (#417)

Religen Inc., established in 2017 is CLIA approved lab, with the focus to commercialize clinical diagnostic tests in pediatric neurology. **Folate Receptor Antibody Test (FRAT®)** is a serum-based test that measures the presence of autoantibodies that interact (either block or bind) with the activity of the Folate Receptor (FRA).

Sarepta Therapeutics (#512)

Sarepta Therapeutics is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. Our focus is on Duchenne and limb-girdle muscular dystrophies, and we have 40+ programs in development across 3 technologies - gene therapy, RNA and gene editing.

Sentynl Therapeutics, Inc. (#401)

Sentynl Therapeutics is a U.S.-based biopharmaceutical company focused on bringing innovative therapies to patients living with rare diseases by sourcing effective and well-differentiated products across therapeutic areas. Sentynl is committed to the highest ethical standards and compliance with all applicable laws, regulations, and industry guidelines. For more information, visit www.sentynl.com.

The Permanente Medical Group (#219)

The Permanente Medical Group (TPMG) is the largest medical group in the United States. Our 9,300 physicians and 40,000 nurses and staff lead the transformation of health care. Through nation-leading preeminent research and technology systems, we deliver superior clinical outcomes, and often life-changing impact our 4.4 million patients.

SUPPORTER SPONSOR

Theranica (#209)

Theranica, creator of Nerivio, is on a mission to improve the quality of life of patients living with migraine. Nerivio is a discreet, wearable, drug free Remote Electrical Neuromodulator (REN) that is safe and effective for acute and/or prevention of migraine in patients ages 12 and up.

theranica

PREMIER SPONSOR

UCB, Inc. (#201 & 207)

At UCB, we come together everyday laser-focused on a simple question: How will this create value for people living with severe diseases? We are a global biopharmaceutical company committed to innovation to improve the lives of people with neurological and immunological diseases, finding solutions to meet their unique needs.



United Mitochondrial Disease Foundation (#415)

United Mitochondrial Disease Foundation offers a number of clinician-facing resources including Mito University, an online library for the medical community to help further understanding of mitochondrial disease, regular clinician education webinars and the annual Mitochondrial Medicine Symposium, which will be held June 26 – June 29, 2024. Visit umdf.org for information.

Upsher-Smith Laboratories, LLC (#102)

Upsher-Smith Laboratories, LLC is a trusted U.S. pharmaceutical company striving to improve the health and lives of patients through an unwavering commitment to high-quality products and sustainable growth. For more information, visit www.upsher-smith.com.

UT Health Austin Pediatric Neurosciences at Dell Children's (#601)

UT Health Austin Pediatric Neurosciences at Dell Children's is a clinical partnership between Dell Children's Medical Center and UT Health Austin, the clinical practice of the Dell Medical School at The University of Texas at Austin. Our comprehensive care approach prioritizes family-centered treatment, collaboration among specialists, and inter-institutional partnerships.

Zevra Therapeutics (#518)

Zevra Therapeutics is a rare disease company driven by science, data and patients' unmet needs to create transformational therapies for diseases with limited or no treatment options. We specialize in a data-driven approach, carefully balancing patient needs, to advance therapies and find solutions to overcome complex clinical and regulatory challenges.



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