



Where the Next
50 Years Begins

Wednesday, October 12 - Saturday, October 15, 2022

Duke Energy Convention Center • Cincinnati, Ohio



Sponsors

The Child Neurology Society thanks the following partners for their generous financial support.

SILVER SPONSORS



BRONZE SPONSORS



LOCAL HOSTS



Thanks to the following for their support of CNS Award Lectures (Hower and Denckla) and PERF for the Pellock Seminar



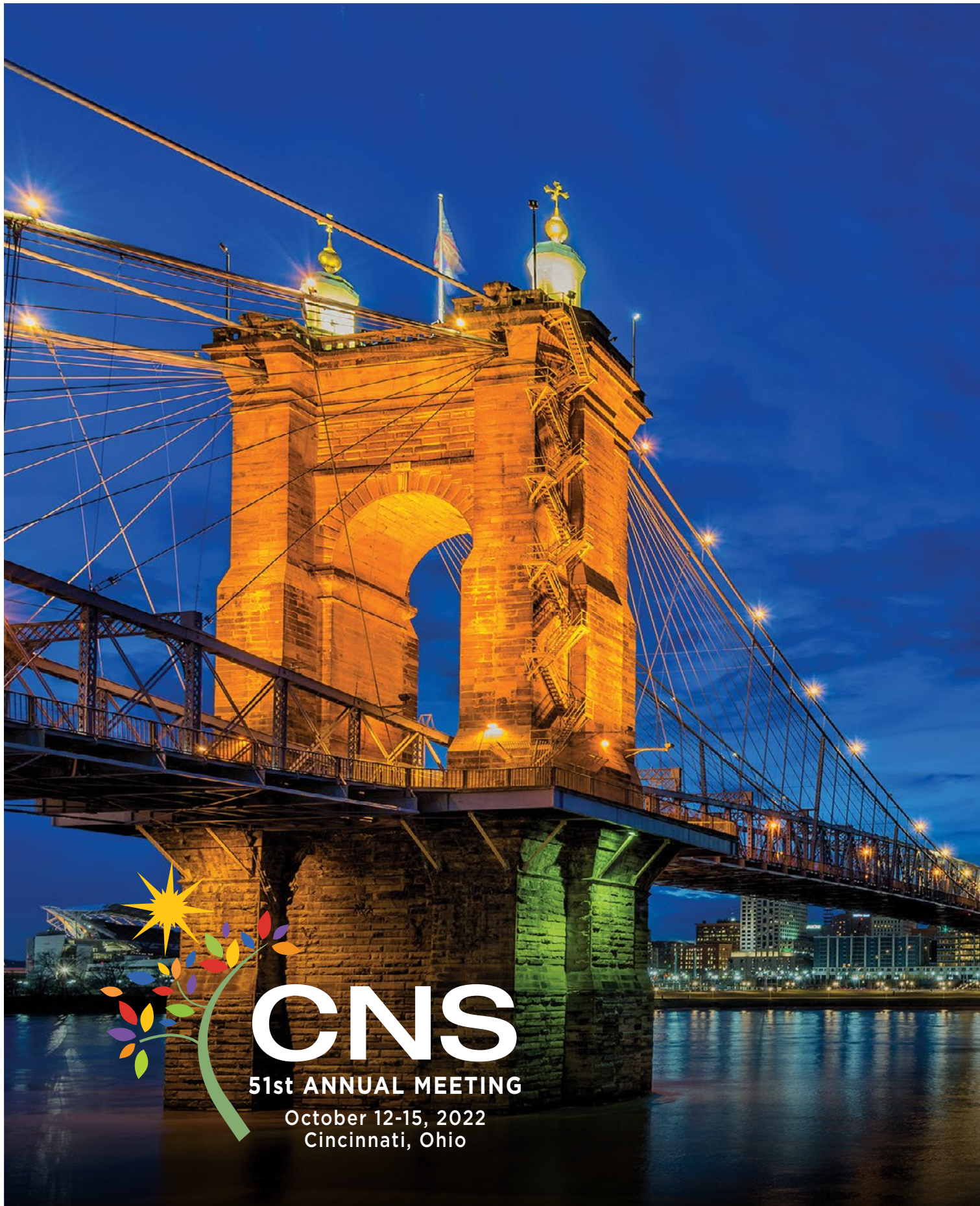


51st CNS Annual Meeting

- 4** Welcome to Cincinnati
- 6** Arnold P. Gold Foundation Humanism in Medicine Award
- 8** Roger and Mary Brumback Lifetime Achievement Awards
- 14** CNS-PECN Training Director Award
- 16** Martha Bridge Denckla Award
- 18** Philip R. Dodge Young Investigator Award
- 20** Bernard Sachs Award
- 22** Hower Award
- 24** Junior Member Awards
- 26** Bernard D'Souza International
- 28** Kenneth F. Swaiman CNS Legacy Luncheon
- 33** Learning Objectives
- 34** Schedule at a Glance
- 38** 51st Annual Meeting of the CNS Scientific Program
- 54** Industry-Sponsored Satellite Sessions
- 59** The CNS Junior and Early Career Forum
- 60** Duke Energy Convention Center Exhibit Hall A
- 62** Exhibitors



Child Neurology Society
 1000 West City Rd. E, Suite 290
 St. Paul, MN 55126
 Tel: 651/486-9447
 Fax: 651/486-9436
nationaloffice@childneurologysociety.org
www.childneurologysociety.org



Welcome to Cincinnati

51st Annual Meeting of the Child Neurology Society *Where the Next 50 Years Begins*

Fifty years ago over 100 child neurologists made their way to Ann Arbor, Michigan for what would become the first meeting of the CNS. Last year in Boston, 11 of those child neurologists meeting in Ann Arbor also attended the 50th Annual Meeting of our organization, two of them virtually, nine of them in person. Three of those members spent their careers in Northeast Ohio. I was lucky enough to spend time with them as colleagues: Gerald Erenberg, MD was a partner for most of my tenure at the Cleveland Clinic; Morris Levinsohn, MD was in practice just east of Cleveland and I enjoyed covering his patients during his rare vacations; and G. Dean Timmons, MD was the first child neurologist to “plant the flag” in Northeast Ohio, starting the program at Akron Children’s Hospital, my current employer. Ohio is home to many child neurologists and probably represents the highest concentration of child neurologists per capita. It is altogether fitting, then, that four of the prestigious Ohio programs have joined together to serve as local co-hosts for this year’s meeting in Cincinnati, “Where the Next 50 Years Begins”.

Major thanks and kudos to the CNS Scientific Selection and Program Planning Committee led by Program Chair, Yasmin Khakoo, MD and Associate

Chair, Bhooma Aravamuthan, MD, DPhil for putting together a dynamic program rich in educational opportunities. Thanks as well to Alex Cohen, MD, PhD and Ariel Lyons-Warren, MD, PhD for jump-starting this year’s junior member and early career programming with an innovative line-up of quasi-“Ted Talks” and Friday afternoon forums. As always, the rich science fare will be matched and counter-balanced by another area of excellence in the CNS: networking, collaboration, and community building. None of this could happen without our terrific National Office staff including Kathy Pavel, Emily McConnell, and Sue Hussman, who orchestrates every aspect of this meeting.

Finally, this year’s meeting marks a turning point in CNS history as we offer thanks and say farewell to retiring Executive Director, Roger Larson and welcome our new Executive Director/CEO, Monique Terrell. I hope you all take the opportunity to meet Monique in Cincinnati and share with her your vision of the Society’s past, present and future.

Bruce H. Cohen, MD
President

Where the Next
50 Years Begins

2022 Arnold P. Gold Foundation Humanism in Medicine Award

Jorge Vidaurre, MD

BY JOHN MYTINGER, MD



Jorge Vidaurre MD is the recipient of the 2022 Arnold P. Gold Foundation Humanism in Medicine Award. Jorge is the Director of the Pediatric Clinical Neurophysiology program and EEG Laboratory at Nationwide Children's Hospital, The Ohio State University. He was born in El Salvador, where he

received his Medical Doctorate degree at the Universidad Evangélica de El Salvador. He completed his Pediatric Neurology residency at The State University of New York (SUNY) Health Science Center in Brooklyn and his Clinical Neurophysiology fellowship at the Albert Einstein College of Medicine. During his training and early career, Jorge was influenced by wonderful advisors and mentors such as Nico Moshé, Shlomo Shinnar, E. Steve Roach, Joseph Marcus and many others.

After finishing his fellowship, Jorge returned to El Salvador and practiced medicine there for three years before accepting his current position at Nationwide Children's Hospital. Those early years shaped what would become a career of advocacy for medically underserved children and child neurologists working in low resource regions.

Tirelessly striving to improve access to care for underserved children, Jorge has spearheaded multiple infrastructure and educational endeavors. He has worked to improve access to EEG by helping to create EEG laboratories and coordinate the training of EEG technicians in Latin America and Africa. During

the COVID-19 pandemic, he organized multiple international educational programs to support training in low-medium income countries, using virtual technology to overcome travel restrictions.

As Chair of the Child Neurology Society (CNS) International Affairs Committee since 2015, Jorge has planned, developed, and organized numerous educational and training programs directed to improve neurological care in low resource regions around the world, including Africa, Latin America, the Caribbean, and Asia. These programs have included regional symposia and training workshops for pediatric neurologists, EEG technicians, and primary care clinicians. With support from the CNS and members of the international community, he has worked to build infrastructure in Africa, the Caribbean, and Latin America. He has also fostered partnerships between a growing list of professional associations, including the CNS, International Child Neurology Association (ICNA), and International League Against Epilepsy (ILAE), developing multiple short and long-term outreach projects focused on improving pediatric epilepsy training in low-middle income countries. In recognition of his work on international educational programs, he was elected chair of the Education Task Force (2022-2026) of the ILAE, with the goal of creating a standard, sustainable, and multilingual EEG curriculum to be used by clinicians all over the world. In his role as the ICNA Educational Advisor, he has worked closely with the president and executive board on issues related to global educational projects.

Jorge has passionately worked to raise national and international awareness about the challenges faced by child neurologists practicing in low-middle income countries and has organized multiple global health symposia at



the American Epilepsy Society (AES) and CNS annual meetings. He is the current chair of the Global Health and International Special Interest Group of AES, promoting involvement of neurologists practicing in low resource settings to discuss important topics in child neurology and foster future collaborations. He has also given more than 100 international lectures (as invited speaker in different regions, including Europe, Africa, Asia, Latin America, and the Caribbean), often with a focus on the care of children in low-middle income countries.

In 2017, Jorge became part of the Ibero-American Child Neurology Society Executive Board and Education Committee. He serves as an editor for the organization's practice guidelines for the diagnosis and treatment of pediatric neurological disorders. These guidelines are published in Spanish with the goal of establishing more standardized practice in these regions. Recently, he was appointed ILAE's Regional Coordinator for Latin America. Within this latter role, Jorge is tasked with fostering collaborations between the ILAE and leaders in Latin America to develop projects leading to improved epilepsy care for children.

Jorge has positively influenced numerous national and international medical students, residents, fellows and younger colleagues, often serving as a mentor. He is a fellow of both the AES and the American Clinical Neurophysiology Society (ACNS), actively participating in the Professional Development Mentorship Program of the ACNS and the Mentor-Fellows Program of the AES.

From personal experience, I know Jorge to be a joyful, spiritual, and humble friend who shies away from awards or other forms of personal recognition. Yet, it remains important to recognize a career spent caring for the most vulnerable of populations. It is likewise important to recognize that Jorge's success was only achieved with the support from his wife Patricia and sons Danny (16) and Diego (18).



Where the Next
50 Years Begins

2022 Roger and Mary Brumback Lifetime Achievement Award

Jeffrey Buchhalter, MD, PhD

BY DAVID URION, MD



It would be typical in biographical statements written for the occasion of one of our colleagues being given an august award for some manner of achievement that we march through a linear recounting of their career trajectory, assuming this will somehow explain to us how it is they won

the glittering prize we, as a collective body, are about to bestow upon them. When we give awards for some scientific achievement, or some particular teaching prowess, or some singular accomplishment, that perhaps makes sense. We are headed in the direction of a singular event, or a particular part of a career.

This award, the CNS Roger and Mary Brumback Award for Lifetime Achievement in Child Neurology, is quite different. It acknowledges that the person we honor has spent the entirety of their professional life devoted to making the lives of children with neurologic disorders better, more manageable, more understandable, perhaps even more comfortable. It acknowledges a singular devotion to our craft and developing it in multi-faceted ways. It acknowledges the contributions that are manifestly public, as well as the hidden ones that are also necessary to move things along. It is a recognition that the individual does everything in their power to solve some challenge or problem that our patients and their families face day in and day out. It embraces the humble and the grand, the pedestrian but crucial things that stand in the way as well as the broad strokes of great insights and discoveries. It speaks to the totality of a professional life.

As such, this award speaks more to a person's character and temperament than it does to individual achievements and accomplishments. It speaks less to papers or books written and more to a way of living into a life. It speaks more to the "why" of a life than it does to the "what and where" of a life.

So, I will acknowledge the extraordinary places where Jeff was formed – UCLA, Harbor Hospital, Boston Children's Hospital. And the places he stopped along the way – CHOP, Mayo/Rochester, Phoenix Children's Hospital, Alberta Children's Hospital. These are all important and wonderful places, noble in their attributes and full of remarkable colleagues. But many live their professional lives in great places with wonderful colleagues, and they are not recognized for their lifetime achievement.

We should speak instead to the virtues he has embodied. An indefatigable drive to make the lives of children with neurologic disease, particularly epilepsy, better. A remarkable ability to connect with frightened parents and make them feel safer, and in good hands. As one who inherited quite a number of his patients when he left Boston Children's Hospital decades ago, I can testify to parents and children speaking of his kindness, his calm, and his manifest concern for their well-being.

We should speak of his unerring sense of what matters to us as a profession, even if some of the issues to which he applied his cogent thinking seemed nearly penitential for many of us. His efforts at rationalizing our coding and billing and establishing standards for what we do and how we should do it. He recognized this was important for us as a profession, but it was even more important for our transparent conduct towards the public.

We should speak of his efforts to advocate for all of us in addressing weighty matters such as SUDEP with families early in the course of our treatment of their children, as difficult



as that may prove to be. He never shied away from difficult conversations, with families or with colleagues. Consistently honest, and invariably kind. He is a model for us all.

We should speak of his early understanding of the importance of big data, and how it could be used to improve the care and lives of children across entire jurisdictions. When many of us thought that this was either a pernicious tool of those who would commodify our work even further, reduce it to measuring outcomes by winkle-picking data, he recognized that like any tool, it could be used for good or ill purpose. He invited us all to join him in using it to improve the health of all children, especially those who had not always had access to excellent care.

We should speak of his balance between rigorous professional demands and his family life, his devotion as a husband and father. Having been present when he and his wife first met, and knowing his two daughters, and the delight he so clearly feels about them, I can affirm that we can all marvel at the good fortune of this family, and how it was always in the forefront of his mind and heart.

We should speak of his good fellowship. Some of us have been fortunate enough in “the before time” to have embarked on splendid culinary adventures, either at CNS meetings or when being invited to speak in places he was serving. His ability to make disparate members of our tribe feel comfortable and welcomed at the table speaks to a vision of what we could all be as a group.

We should speak of his extraordinary wit and sharp sense of humor. Two scenes come readily to mind:

The first finds Jeff walking into a chaotic ED setting with a child in status epilepticus, EKG paper flying everywhere, much shouting, random Brownian motion on the part of residents. Realizing he had to assert some order on all this

chaos, Jeff raises his voice clearly above the din: “No wait! Everybody mambo!” Then, as everybody pauses, puzzled by what they just heard, he quietly walks to the head of the stretcher and takes charge.

A second scene finds him being bedeviled by a neonatologist for some precise estimation of what an extreme premie’s cognitive outcome might be. Jeff patiently explains the data we then had and its limitations, and being asked one more time for an estimate, sighs and says “Do I look like a burning bush?”

And so, on the occasion of Jeff being given this richly deserved honor, we can paraphrase Boswell’s remark on Samuel Johnson: Whenever we find ourselves going down an unfamiliar road, we meet him on his way coming back.



Where the Next
50 Years Begins

2022 Roger and Mary Brumback Lifetime Achievement Award

Roger B. Larson, CAE

BY BRUCE H. COHEN, MD AND PHILLIP L. PEARL, MD



Most deserving of a CNS Lifetime Achievement Award, the first ever bestowed on a non-physician, is Roger Larson: historian, editor, literary critic, author and, as of this conference, recently retired Executive Director of the Child Neurology Society. Ultimately embodying the CNS, Roger witnessed and contributed to our Society's growth and maturation from its early childhood through our 50th anniversary year.

The Midwestern roots of both Roger and the CNS are closely intertwined. The fourth of six children born to quiet, sweet-souled parents raised on neighboring farms in southern Minnesota, Roger grew up in Rochester, MN where his father worked for IBM for over 30 years – he comes by loyalty and longevity naturally. Roger graduated from Mayo High School in 1972, the same year the CNS was founded (and a year after 2018 CNS Sachs Awardee, Bill Dobyns, MD graduated from Mayo).

Roger began his freshman year majoring in American Studies at the University of Minnesota taking a seat in his first class in late September less than ¼ mile away from the academic offices where Ken Swaiman, MD, Larry Lockman, MD, and Mary Currey were busily composing, photocopying and sending out material for the 1st CNS meeting in Ann Arbor, MI. Roger would have graduated with a double-major in History and American Studies in 1976, but for reasons he characterizes as “too complicated, or cock-eyed, to explain,” opted not to, choosing instead to be a perennial student taking grad level courses that interested him, spending hours

drinking coffee and discoursing on books, film and culture with friends in the American Studies graduate program, and even more hours writing columns, serial fiction, and book & film reviews for the *Minnesota Daily* (the University's award-winning newspaper) and *City Pages* (Minneapolis/St. Paul's leading alternative weekly). He wrote for and alongside Michael Phillips, the *Chicago Tribune* film critic who succeeded the deceased Gene Siskel (of Siskel & Ebert, “At the Movies” fame; there's a story there that Roger may share at the Legacy Luncheon).

In 1981 Roger got a part-time job working for Larry Lockman, MD on a multi-clinical drug study for the treatment of Lennox-Gastaut, funded by Burroughs Wellcome. Mark Scher, MD was Chief Resident at the time. Close readers will see Roger acknowledged in the 2nd edition of Swaiman & Wright's *Pediatric Neurology: Principles and Practice*, the starting point for a four-decade labor of love “dotting i's, crossing t's, correcting spelling and inserting commas for child neurologists from morning 'til night.”

Little by little, Roger started helping Mary Currey out on CNS business, first by processing abstracts (this being the snail mail, pre-fax era) and later membership applications. It is most likely Roger processed both of our membership applications, as we joined the Society in 1985 and 1986. He attended his first CNS Meeting in Halifax in 1988 (Bruce's 2nd, Phil's 1st) and recruited the first exhibitor to a CNS Annual Meeting the following year in San Antonio, setting the stage for what would later become a fixture at conferences bringing financial success. That and other parts of what became progressively bigger and better annual meetings is largely owing, he insists, to the quiet, steady excellence of Sue Hussman; Sue first joined the CNS as an independent contractor for the Joint CNS-ICNA Meeting in 1994 before later becoming a full-time staffer and for the past 10 years, Associate Director.

In 1989, when Marvin Fishman, MD was CNS President, Mary and Roger moved the CNS National Office out of the University of Minnesota's Pediatric Neurology Division and set up an independent office in a charming old brownstone building less than ½ mile from where Roger now lives. The office later moved to Shoreview, a suburb 10 miles north.



Roger passed on leaving the CNS for another job twice in the intervening years, both times because of the enormous loyalty and regard he had for the Presidents at the time: Joe Volpe, MD and Ann Tilton, MD. The rest is history.

Roger took the 4-page stapled “newsletter” in the early 90s and turned it into successively larger and more sophisticated iterations of what has become *CNS Connections*. He oversaw the development of the first CNS website in 1999 and has “survived” four subsequent website redevelopment projects. When Mary Currey retired in early 2012, the then CNS President, E. Steve Roach, MD asked Roger to become the 2nd CNS Executive Director (ED). His first meeting as ED was Huntington Beach, a trial-by-fire (or water) event when nearly 1/3 of the registered member had their flights delayed or cancelled by Hurricane Sandy working its way up the East Coast. It was at this meeting that he met Dr. Yasmin Khakoo’s 8-year-old daughter, Aliya, who he vividly recalls earnestly handing him a \$5 bill, thus becoming the youngest contributor to the Philip R. Dodge Young Investigator Award. Ten years later, at this year’s Legacy Luncheon in Cincinnati, Aliya will become the first Dodge YIA contributor to receive the CNS Bhuwan Garg High School Neuroscience Award. Roger’s life with the CNS is filled with many of these full-circle moments.

Roger has known or worked with all but one CNS President and all but six CNS Councillors (some, years after they first served). Many of us first met Roger at the annual meeting, usually somewhere near the registration desk, podium or posters. Developing relationships with young members is Roger’s nature; for him, this is not “work.” Those relationships often developed into fellowship that, over time, became friendships.

Among our most cherished hours each week are those spent Zooming with Roger. After a personal check-in, we would get down to business. There have been about 100 of those meetings by which we have developed a friendship. Roger has been a source of information on “the hows and whys and history of our society.” We seemingly tapped Roger’s memory almost daily searching for a piece of CNS history that is undocumented, yet important. The last question I (BHC) asked Roger a day before his retirement was “why do we spell *Councillor* (our elected regional board members) in such an odd manner?” It may have been the only time Roger was not sure of the answer. He presumes it may have something to do with Mary Currey’s Canadian upbringing in Alberta.

Roger has never lost sight of the importance of our

discipline and the Society’s role in it: the commitment and value of the annual meeting allowing members at all ages and stages to present their “best stuff”, and our collective role in ensuring the sustainability of the Society, our profession, and the children and families we serve. For those fortunate enough to serve on the Board, having the opportunity to get to know Roger better was a career highlight. A consistent highlight of BOD meetings was his always erudite introductory remarks, replete with poems, historical quotations and illustrative examples articulated beautifully.

The day will come when a new CNS President and 6 CNS Councillors will be elected that he has not known or worked with, but that’s likely a decade or more off in the future. In the run-up to last year’s 50th Anniversary meeting in Boston, Roger began sending an acrostic of daily *eConnections* messages (“Countdown to Boston”), with themes labelled alphabetically ascending from A to Z then back to A again. The collected entries (available on the CNS website) became a veritable history of the Society, with loads of heartwarming and illuminating anecdotes and photographs. It became increasingly obvious as the meeting drew closer – to him, and painfully, to us – that these were actually love letters written in the throes of saying goodbye.

We are so fortunate that Roger has been a part of us and we of him, and that we will have this opportunity to celebrate his legacy at this year’s meeting. He will be joined by his wife, Buffy, a professional musician (trumpet) and music teacher at two bilingual inner city Minneapolis schools. Buffy and Roger first married a few months after he began working at the U of M (and the CNS). He will tell you that they flunked “Empty Nest Syndrome 101”, separating and divorcing 7 years ago when he found himself devoting all of his “extra” non-parenting time to the CNS. Like many parts and stories told about his life, this one comes full circle. We are happy to report that Roger and Buffy found their way back together and remarried this past July, a few months before he retired from the CNS. They have two adult children: Soren, who lives in Minneapolis and travels around the country timing races, will be “stopping by” the Cincinnati meeting in between timing the Chicago Marathon on the 9th and the Columbus Marathon on the 16th; and Mekea, a New York-based graphic designer and photographer well known to CNS members from past annual meetings where she worked alongside CNS photographer, Suzanne Shaff, and coordinated videotaped CNS Conversations/Podcast sessions with the late Theresa Trapilo, a mainstay at CNS meetings going back to Dr. Volpe’s presidency in 1994.

2022 Roger and Mary Brumback Lifetime Achievement Award

Michael J. Noetzel, MD

BY CHRISTINA A. GURNETT, MD, PHD, BRADLEY L. SCHLAGGAR MD, PHD, AND JONATHAN W. MINK, MD, PHD



Michael Justin Noetzel, MD was born on April 3, 1951, and raised in Cleveland, Ohio. He died on February 20, 2022, just six weeks before his 71st birthday, and just over four months from his intended date of retirement from Washington University School of Medicine which would have also marked

the 45th anniversary of his arrival in St. Louis. Over those 70+ years, Michael lived a remarkable life of kindness, generosity, and service to others.

Michael graduated in 1969 from St. Ignatius High School, in the Ohio City neighborhood of Cleveland. While at St. Ignatius, he was a standout scholar athlete lettering in football and baseball. He went on to attend Yale University where he was also a standout scholar athlete, lettering in football and baseball, and excelling in the former. As a freshman safety, he helped the 1969 Yale Bulldogs tie for first place in the Ivy League; he was named a member of the All-Ivy 1st Team in 1972. He was awarded the Woody Knapp Memorial Trophy, which is “given to that outstanding member of the football team who best typifies the cheerful dispositional, leadership qualities and unselfish devotion to others” – qualities that would characterize Michael throughout his life.

After graduating *cum laude* from Yale, Michael attended the University of Virginia School of Medicine, graduating in 1977, whereupon he moved to St. Louis for residency training in Pediatrics and Pediatric Neurology, joining the likes of Blaise Bourgeois, Joan Conry, Ed Kovnar, Tom Langan, and Bill Turk. Upon completing his training, he was appointed to the faculty of the Washington University School of Medicine in 1982, joining a Department of Pediatrics chaired by Philip Dodge and a Division of Pediatric Neurology directed by Arthur Prensky. Other faculty members in the Division at that time included Ed Dodson, Ruthmary Deuel, Steve Rothman, and Joe Volpe. Michael would go on to spend his entire 45-year career

at Washington University and St Louis Children’s Hospital. He was a pediatric neurologist who was a true “quintuple threat” – clinician, researcher, teacher, administrator, and role model. He had unparalleled personal qualities that allowed him to excel in all of those roles, while also maintaining his persona of a down-to-earth, “regular guy”. Utilizing all of his talents, the Ivy League All-Star led St Louis Children’s Hospital flag football and baseball teams to victories in the 1980s, perhaps the first and only time a neurologist filled this role at our hospital.

He was the Director of the Division of Pediatric and Developmental Neurology from 2007-2014 and the founder and Medical Director of the Neurorehabilitation Program and Therapy Services at St Louis Children’s Hospital from 1990-2020. Although he planned to officially retire in July 2022, he was unwilling to retire his reflex hammer and was already on the schedule to teach as an Emeritus Professor in residents’ clinic in the fall.

Michael’s scientific accomplishments were as vast as the variety of conditions managed by child neurologists, and reflected his recognition of the power of collaborative, multicenter research. He received a Clinical Investigator Development Award from the NIH in 1984. He went on to play an important role in several major NIH-funded trials including the Diabetes Control and Complication Trial and Silent Cerebral Infarct Multi-Center Clinical Trial focusing on sickle cell disease. Both studies resulted in landmark publications in the *New England Journal of Medicine* that continue to guide the management of these diseases today. Recently chosen to serve a 5-year term on the International Pediatric Stroke Study Publications Committee, Michael was looking forward to influencing the field even during retirement.

Michael was an active participant in the Child Neurology Society, serving as Councillor for the Midwest from 2001-2003. He rarely missed a meeting, where former colleagues and trainees looked forward to seeing him every year.

Michael received numerous awards for his research and teaching, including the 2013 Distinguished Clinician Award from Washington University and the 2020 John Doronzo



Memorial Award for Clinical Excellence from the Brain Injury Association of Missouri.

Throughout his career, he became known as an outstanding educator to hundreds of medical students, residents, and fellows. His generosity of spirit and time was extraordinary as he exemplified the mentor whose door was always open. He was deeply invested in building the careers and success of all those around him.

Michael had immense respect for the expertise of those on his team. An excellent case in point is the Neurorehabilitation Program at St. Louis Children's Hospital, which he founded and built. The team was quite large, including physical therapists, occupational therapists, speech language pathologists, pharmacists, care coordinators, nurses, nurse practitioners, psychologists, social workers, school teachers, art and music therapists, nutritionists, and child life specialists. Michael led that team by making sure that for everyone around that table, their voice and expertise was heard, their concerns heeded, and that their contributions to the care of the patient and family were recognized, respected, and integrated into the plan.

For those of us who worked with him for many years, he was the quintessential servant leader. Michael embodied this concept of leadership, which came naturally to him. He was an exceptional and empathic listener, who modeled ownership. His humble demeanor belied the wisdom he delivered, often with a chuckle and a twinkle in his eye. He experienced immense joy from the success of his trainees and junior faculty. By contrast, he seemingly never demonstrated elation over his own success. His humility, yoked to a strong sense of purpose, was ever present. Michael had an understated way of making us all better physicians and, frankly, better human beings.

From playing softball in Forest Park men's league, ski trips with family to coaching his daughter's grade school basketball teams where he was known to wear the same "lucky" vest every game (even though they rarely won), and planting trees in St Louis as part of Forest ReLeaf of Missouri, Michael enjoyed a life rich with love, service, and purpose. Known as "Dutch" by his grandkids, he led them in games

of whiffleball, funny photo contests, and was even filmed playing slip n' slide in the backyard. Michael is survived by his wife (Mary), children (Evan, Justin, Katie, and Anna), and 8 grandchildren.

Late in 2021, Michael's colleague and former trainee, Laura Jansen, had the honor of telling him that he had been selected to receive the 2022 Roger and Mary Brumback Lifetime Achievement Award from the Child Neurology Society. In response to the congratulatory notes sent by colleagues and former trainees, Michael replied "...when a lifetime achievement award is given to an individual physician, it reflects to a significant degree the environment in which that physician practices medicine. In my case, for nearly 45 years, I have been blessed to pursue a career in Child Neurology surrounded by truly great leaders and mentors, and kind and dedicated healthcare providers...I cherish my association with each and every one of you."

Michael's impact on the discipline of child neurology, Washington University, St. Louis Children's Hospital, his students and trainees, and the lives of his countless patients will be measured in generations. His generous spirit and kindness will be sorely missed by all who had the opportunity to know him.



Where the Next
50 Years Begins

2022 CNS-PECN Training Director Award

Timothy Lotze, MD

BY GARY CLARK, MD



Dr. Timothy Lotze grew up in Houston, Texas. While completing his physics degree at Texas A&M University (Class of 1991), Tim felt the pull of medicine. This was in part because of his father's distinguished career in obstetrics and gynecology along with a desire to directly help

people in need. While attending the University of Texas Health Science Center in San Antonio, Tim found an early love for neuroscience in medical school and initially considered pursuing adult neurology. However, his love for working with children steered him into a categorical pediatrics residency in 1995 at Nationwide Children's Hospital (then known as Columbus Children's Hospital). Through the course of this residency, Tim found child neurology to be an ideal career for his interests and set his sights on training at Baylor College of Medicine to achieve that goal. Prior to coming to Baylor, Tim spent an extra year in Columbus serving as the Chief Resident in pediatrics from 1998-1999. This year was highly influential in developing his interest for clinical education and established a foundation upon which he continued to develop his skills in this area.

Upon completion of his residency training in child neurology in 2002, Tim joined the faculty at Baylor College of Medicine and Texas Children's Hospital. While early in his career, he started to develop his clinical interest and expertise in neuromuscular disorders and pediatric multiple sclerosis. He remained active in training the child neurology residents at Baylor, however, to include ongoing development of residency program activities. Back in those days of paper charts and limited computer technology, he created an early electronic medical record system that utilized Microsoft Access to document resident encounters as well as to track various diagnoses and capture faculty billing on inpatient rounds. Tim received the ACGME Marvin Dunn Award in 2008, when he presented this work at the ACGME annual meeting.

When Tim was offered the opportunity to serve as the Child Neurology Residency Program Director in 2005, he jumped at the chance. Over the subsequent 17 years, Tim has continued to build and refine the residency program into one of the best in the country. Tim has led the program through a variety of enhancements to include expanding from two to five hospital-funded residency slots per year (two of which are reserved for basic neuroscience pathway residents) and the incorporation of the Neurodevelopmental Disabilities residency into the curriculum. With the onset of the Covid pandemic, Tim was instrumental in helping the program to quickly pivot resident education and clinical activities into an online format.

Tim has continually worked with colleagues in pediatrics and adult neurology to assure the residents are receiving the best education while making sure to meet ACGME and ABPN requirements. Leveraging the resources available at Texas Children's Hospital, Tim continually refined the education programs to help assure that residents in training would be ready for modern practice. Examples of this include increasing resident exposure to genetics and gene therapy, fetal neurology, neurocritical care, neuroimmunology, and palliative care.

Tim has also promoted scholarship amongst the residents through teaching critical evaluation of the literature, research methodology, and presentation and writing skills. Nearly all graduates have presented at national meetings as well as published in peer-reviewed journals; most of these publications are collaborative with other residents as well as faculty within the Division.

Tim has rare qualities that make him invaluable as a program director. Tim is a leader, is well-organized, utilizes technology optimally to leverage complicated systems in medicine, is an outstanding physician, a great teacher, a visionary, has a wonderful sense of humor and is the ultimate professional. He inspires trainees to love to learn and to be the very best child neurologist and neurodevelopmental disabilities specialist. Tim engenders a love of learning and a professional approach to the practice of medicine. He finds his greatest satisfaction in helping others to be successful, and this is especially true when it comes to the residents that he has helped to train. He blends clinical teaching with humor and



establishes an environment that promotes a growth mindset. The American Neurological Association (ANA) recognizes institutions that get the most students to go into neurology, and Baylor College of Medicine has been recognized for many years as top in the US, largely due to Tim's efforts in student teaching. Through his modeling, residents aspire to likewise become clinical and educational leaders in the field, and many of the graduates have achieved that goal.

Tim is consistently ranked as one of our top teachers at Texas Children's Hospital and Baylor College of Medicine. In 2009, he simultaneously received two Rose Fulbright Awards from the College in recognition of Education Leadership as well as Evaluation and Teaching. That same year, he was inducted into the Baylor Academy of Distinguished Educators. His ongoing hard work and success in educational leadership led to him receiving the American Academy of Neurology Program Director Recognition Award in 2016. A letter of support from a former trainee at that time stated:

"Dr. Lotze's professionalism, dependence on evidence-based medicine, and patient compassion and advocacy has strongly influenced me during my training. He has been a model not only in his role as a teacher but more as an example. ... He was also key in teaching me how to care for a complex patient with an organized systematic approach, while not losing sight of the family and the child."

As part of his nomination for the PECN Program Director Award, his former chief residents made this key observation about a key quality in Tim's leadership:

"It has always been clear to us that Dr. Lotze has a primary interest: the wellbeing of his patients and the residents in the child neurology and neurodevelopmental disabilities programs. He takes an active role in overcoming obstacles – allowing residents to outline the changes requested and following it with effective execution, prioritizing the resident's success and wellness over all other administrative interests and clinical service obligations. When residents propose programmatic changes to Dr. Lotze, he always responds with "why not?" in place of "why?" This simple act of allowing residents to advocate for themselves and entrusting

them with their own education epitomizes the care and guidance he provides trainees on their journey of growth to become the best child neurologists they can be."

This statement speaks to Tim's recognition and support for his residents and the faculty to be active participants in the ongoing construction and development of the program. This shared responsibility provides everyone with an opportunity for improving the quality of the program as well as a sense of accomplishment when effective change is made.

Another former chief resident made this observation, which speaks to Tim's open and giving nature:

"He has always had an "open door" policy, and wholeheartedly assists trainees in overcoming personal and professional challenges. Certainly, being a program director has its fair share of crises and stumbling blocks, but Dr. Lotze manages to approach any issues with a professional, level-headed mindset, and optimistic attitude. I found this to be of utmost importance and I have tried to emulate his example while I co-managed such issues as a Chief Resident. All of this to say, that it is abundantly clear that Dr. Lotze truly cares about each individual in his training program and sees to it that their educational as well as personal needs are met."

Tim has done all of the above while developing his nationally recognized expertise in neuromuscular and neuroinflammatory disorders. He is one of our busiest clinicians but is always ready to volunteer to take more service time, especially when this involves teaching, mentoring and being an outstanding example to our trainees. His dedication to the success of our large Child Neurology training program has no doubt been the driving force of its evolution and continued growth. I believe his innovative, holistic-minded, and supportive approach to the trainees and program make him one of the best residency program directors of Child Neurology in the country, and thus fully deserving of this award.

2022 Martha Bridge Denckla Award

Michael Shevell, MDCM, FRCPC, FCAHS

BY STEVEN PAUL MILLER, MDCM, MAS, FRCPC



Michael Shevell was born in Côte Saint-Luc, Quebec, Canada in 1958. He received a DEC in Health Sciences from Marianopolis College in 1977, followed by an undergraduate degree in physiology (1980) and medicine (1984) at McGill University. He was inspired to pursue a career in

Neurology through the integration of basic science, anatomy, and clinical cases in the Med I Central Nervous System course. He pursued residency in pediatrics and pediatric neurology at the Montreal Children's Hospital (MCH) and McGill with Drs. Gordon Watters, Bernard Rosenblatt, Kenneth Silver and Frederick Andermann. He acknowledges their engaged mentorship, dedication, and thoughtful teaching efforts for driving a passion to do even better for each child in his care.

Michael gravitated over time to the neurology of newborns and its relation to later neurodevelopmental disabilities. When Michael was training in neurology in the 1980's, the real excitement was molecular genetics, so he pursued a fellowship in Dr. David Rosenblatt's lab, focused on rare inborn errors of metabolism, learning the vocabulary of genetics. Through this training, he recognized the importance of phenotyping as central to understanding genotypes. Michael's first paper in this area was on a deeply phenotyped cohort of children with benign familial neonatal seizures. When he obtained his first faculty position at MCH, he immediately developed a database to catalogue all the patients he encountered in his Pediatric Neurology practice. This database proved fundamental in his subsequent studies on the outcomes of neonates with encephalopathy. His contributions to defining the phenotypes of common neurological disorders of the young child is foundational to his contributions to the care of children with neurodevelopmental disabilities. Michael's trainees will all recall his encouragement to listen to and observe the child

and family in their care.

Training in pediatric neurology in the 1980's Michael was impressed by how doors were "not just opening up – they were flung wide open" with the introduction of MRI and molecular diagnostics. He recalls seeing the first MRI that he had ordered for a clinical indication, and "seeing the answer right there." Impressed by the MRI window to the developing brain, he considered molecular genetics to be a comparable cellular window. As his interests developed, Michael reflects the transition of his interests from the protein to the "bigger picture" of the child in their family.

Michael's contributions to child health were synergistic with Dr. Annette Majnemer, his life partner. Michael and Annette started working together in the 1990's, early in their respective careers. Interweaving their individual perspectives, they focused on the relationship between the neonatal neurologic and OT examinations with neurodevelopmental outcomes and on the etiology of global developmental delay. With both Michael and Annette grounding their work in the real-world questions being asked by clinicians and families in the Pediatric Neurology clinic, their collaboration grew over the next two decades with combined and independent research endeavors. Working with Annette, Michael "put the 'neuro' into neuro-disability" research and clinical care. His seminal studies influenced the early identification of disability, identifying children at risk for neurodevelopmental sequelae and possible predictive factors, and characterizing novel childhood disability sub-types. Michael's academic activities consistently linked intrinsic (biologic, functional) and extrinsic (family, environmental) determinants of developmental disability, including the first observations of neuro-behavioral abnormalities in neonates with congenital heart disease even prior to surgery. Concurrently, Michael became increasingly engaged in addressing ethical and historical issues related to care provision. Together, his work provided a scientific rationale for the evaluation of childhood disability, and broadly influenced the practice of child neurology.

Complementing his impactful program of research is Michael's passion for mentoring young people and trainees.



He has supervised close to 50 pediatric neurology trainees, and numerous research trainees. He lectures internationally, across dozens of countries, as an invited expert and visiting professor, raising awareness of neurodevelopmental disabilities and reminding us all of the importance of attending to the whole child in our care and their family. Michael's success as an educator and mentor is best reflected in the leadership roles his trainees and mentees have assumed and in crossing child-health disciplines; he has instilled a commitment to mentorship in each of his mentees, amplifying his impact as an educator.

Michael is also a passionate advocate for child health locally, nationally and internationally. For example, his leadership role in NeuroDevNet emphasizes the imperative to collaborate with various professional disciplines, methodologies, and perspectives to address and solve problems. Michael served in leadership roles for the Child Neurology Society including as Councillor for the Northeast (2017-2019), Awards Committee Member (2016-2019), and Chair of the Ethics Committee (1997-2009). He received the Child Neurology Society's Hower Award in 2014, one of our Society's highest recognitions, given annually to a person whom the Child Neurology Society deems has contributed greatly to the further understanding of neurological problems of childhood through research, teaching, clinical application, and leadership.

Michael has been similarly active in the International Child Neurology Association, including a long stint on its Executive Board, and Chairing the Scientific Programming Committee for the Xth (Montreal, 2006) and XIIIth (Iguazu Falls, 2014) World Congresses. Most recently, he has been appointed as a Fellow of the Canadian Academy of Health Sciences, elected on the basis of his demonstrated leadership,

creativity, distinctive commitment to advance academic health sciences.

With this breadth of perspective and recognition by his university and peer communities, Michael became Chairman of the Department of Pediatrics at McGill and Pediatrician-in-Chief of the Montreal Children's Hospital in 2011. He served in this impactful role until 2021, overseeing the move of the department into a new hospital and robustly expanding scholarship and training activities throughout all sectors of departmental activities. Through this role he took particular pride in the success and diversity of the faculty that were recruited and supported. In our discussions, Michael also reflects how this leadership experience afforded him a broader view of the world that relates to how he sees his research contributions: striving to ensure his work is continuously meaningful and relevant in terms of outcomes that are relevant to the child and family.

Michael's chief interests in his life outside of child neurology are his wife Annette, and their daughters, Allison and Meaghan. Allison is now a pediatric intensive care physician and is currently pursuing a Master's in Epidemiology at McGill University, with a focus on investigating long-term outcomes after pediatric critical illness. Meaghan, after graduate studies in Human Rights, first coordinated the Global Child McGill research group and is now a consultant in the Equity, Diversity & Inclusion practice of an international firm that evaluates the effectiveness of programs established by NGOs throughout the world.



Where the Next
50 Years Begins

2022 Philip R. Dodge Young Investigator Award

Bhooma Aravamuthan, MD, DPhil

BY CHRISTINA A GURNETT, MD, PHD



Bhooma was born to Lakshmi and Rajagopalan Aravamuthan, just in time to be acknowledged in her father's PhD thesis. Her mother is a talented painter and musician, with her artistic talents displayed in national forums in India. Her father, after growing up without running water, spent 25+ years as an

endowed chemical engineering professor before retiring this year. She is a product of their love, grit, and determination to succeed.

Bhooma was broadly interested in neuroscience from a young age, but that passion crystallized as an undergraduate when she learned that her uncle had been diagnosed with Parkinson's disease. She began looking for labs doing translational research in Parkinson's disease and found one at Oxford. While applying for MD/PhD programs she, on a lark, applied for a prestigious George C. Marshall scholarship to fund a research degree at Oxford. When she was awarded the Marshall Scholarship, both the NIH and Washington University in St. Louis (WashU) graciously funded a somewhat unorthodox training program: Bhooma did her DPhil (Oxford's terminology for PhD) in the labs of Tipu Aziz at Oxford and Judie Walters at NIH. She then did her MD in St. Louis as a part of WashU's Medical Scientist Training Program. To this day, she continues to take a deep breath before beginning to explain her training pathway to others.

During her DPhil, Bhooma began combining human subjects and animal model-based research to answer clinically relevant questions about neurologic disease – an approach that is evident in her current work. Using diffusion tensor imaging under the guidance of Heidi Johansen-Berg at Oxford, she demonstrated the ability to trace anatomically relevant connections from the subthalamic and pedunculopontine nuclei in the human brain. She subsequently studied the spike-timing relationships between these nuclei and the

motor cortex in the parkinsonian rat brain under Dr. Walters' guidance at the NIH. She combined these data in her thesis to hypothesize ideal deep brain stimulation targets and stimulation frequencies for people with Parkinson's disease.

She continued her study of pedunculopontine nucleus electrophysiology while obtaining her MD at WashU, recording from awake macaques during vestibular perturbations in Dora Angelaki's lab. She used this experience to consider the role of the pedunculopontine nucleus in falls in people with Parkinson's disease, bask in the advice and guidance of yet another strong woman mentor, and definitively learn that she never wanted to work with macaques again. Entering the clinical portion of her medical school training, she remained resolute in pursuing a career as an adult movement disorders physician scientist.

However, formative experiences with pediatric neurology changed her mind. Her floor attendings were Doug Larsen and Anne Connolly, both expert clinicians and renowned educators. Doug suggested that she shadow Jan Brunstrom in the Cerebral Palsy Center and Anne suggested she meet Mike Noetzel at Pediatric Rehabilitation Rounds. After working with kids with cerebral palsy (CP) and their families repeatedly during her intern year, Bhooma committed herself to a career focused on understanding movement disorders in people with CP. Eager to learn about the effect on basal ganglia circuitry following hypoxic-ischemic injury to the developing brain, she joined the lab of Mish Shoykhet as a second year pediatrics resident. She recorded single units in the basal ganglia of young rats following cardiac arrest, intercalating these overnight recording shifts with night shifts in the hospital.

She carried this focus with her to Boston Children's Hospital where she began her child neurology training. She quickly developed ideas and methods to quantify dystonia in CP. As an NINDS R25 recipient under the mentorship of Seward Rutkove, she optimized electromyography and nerve conduction study analogues of spasticity and dystonia in rodents following neonatal hypoxic-ischemic injury. She used this data to write for the newly re-configured CNCDP-K12 award, then in its second year, which she was awarded. After neurology training and a movement disorders fellowship in



Boston, she returned to WashU as faculty in 2018 to join the CP Center that first piqued her interest in the field.

Following this already promising trajectory, Bhooma's star has meteorically risen. She is now widely regarded as a national expert in dystonia in CP. Her work has been featured in flagship journals including *Annals of Neurology*, *Neurology*, and *Pediatrics* and continues to span human subjects and animal-model based approaches. She methodically elucidates the features clinicians use to diagnose dystonia in people with CP and then uses these features to characterize dystonia in her newly developed mouse models of neonatal brain injury. She uses diverse techniques including machine learning-based pose estimation, chemogenetics, and calcium imaging to dissect the pathophysiology of dystonia in these animal models. In addition to the 2022 Dodge Young Investigator Award, her work garnered her the 2022 American Academy of Neurology Jon Stolk Award in Movement Disorders, the first time this award has been given to a pediatric neurologist. She has presented her work in diverse and illustrious forums including at the recent NINDS/NICHHD Workshop on Cerebral Palsy and at a briefing to the United States Congressional Neuroscience Caucus.

Bhooma is also a respected thought leader in the CP field. Her work on CP diagnosis and increased need for neurologist involvement in the care of people with CP has helped ignite renewed interest in CP at CNS. Her scientific leadership is evidenced by her co-chairing the Scientific Planning Committees for CNS and the American Academy of Cerebral Palsy and Developmental Medicine (AAPDM) in 2023, perhaps the first time these positions have been held by the same person across these two societies. As an advocate and member of the AAN Inclusion, Diversity, Equity, Anti-racism, and Social Justice (IDEAS) subcommittee, she champions disability rights on behalf of her colleagues and the families she cares for in clinic. Much of her recent work has focused on partnering with people with CP and their caregivers to define the research agenda and to give them a voice as partners in "community driven research."

In addition to her scientific accolades, Bhooma has already become a supportive mentor for an unprecedented number of undergraduate students, medical students, and clinical fellows, who flock to her burgeoning research group to capture some of her positive energy. With a never-ending list of ideas for new innovative research projects, she sets high expectations for her trainees who have uniformly had highly productive and rewarding research experiences. Her trainees describe her as being "fully dedicated to their success" and "always making time for them, even if it means meeting late at night on Zoom with her adorable kids playing around her." Clinical trainees also find humor in her obsession with the Queen square reflex hammer, often quoting her famous Twitter rant about it being the little black dress of reflex hammers that never goes out of style.

Bhooma repeatedly begins her talks by stating that dystonia in CP is her "professional love", but a bio about her would be incomplete without recognition of her personal loves. She often claims that her work-life balance is achieved only because they are, by nature, both intertwined. She is happily married to Dan Weber, an adult epileptologist, and is proud mom to twin sons and a daughter, Singam, Neel, and Andal. Though her children were not born in time to be acknowledged in her doctoral thesis, Bhooma is surely proud to acknowledge them here.



Where the Next
50 Years Begins

2022 Bernard Sachs Award

Steven Paul Miller, MDCM, MAS, FRCPC

BY MICHAEL ISRAEL SHEVELL, MDCM, FRCPC, FAAN, FANA, FCAHS



Steven Miller was born and raised in Côte Saint-Luc, Quebec, a demographically unique proximate suburb of Montreal. After achieving expedited entry into medical school, Steven received his medical degree from McGill University in 1995 and completed his residency in pediatric

neurology at the Montreal Children's Hospital in 2000, where he gratefully acknowledged the educational influences of Gordon Watters, Frederick Andermann, Bernard Rosenblatt, Michael Shevell and Chantal Poulin. Steven's future promise and impact was evident by his being awarded the Holmes Gold Medal for his graduating class, indicating his rank as the top student over the entire 4 year medical school curriculum. Quite a feat as McGill is Canada's top-ranked Medical-Doctoral university. He further excelled in his residency by publishing, remarkably, 15 peer-reviewed papers during his pediatric neurology training.

Following residency training, Steven went to UCSF to follow his passion in neonatal neurology where he undertook a combined clinical and research fellowship with former Sachs Awardee, Donna Ferriero. This was followed by Faculty appointments first at UCSF, then at the University of British Columbia in Vancouver, and from 2012 till 2022 at the Hospital for Sick Children (Toronto). While in Vancouver, Steven held a prestigious Canada Research Chair in Neonatal Neuroscience and a Canadian Institute for Health Research (CIHR) Clinician-Scientist Award. The move to Toronto saw Steven being awarded the Bloorview Children's Hospital Foundation Chair in Pediatric Neuroscience and promotion to Full Professor in the Department of Pediatrics at the University of Toronto. He also assumed the Directorship of the Division of Pediatric Neurology at the Hospital for Sick Children (HSC), replacing former Sachs Awardee, O. Carter Snead III (2005), as well as the Headship of the Centre for Brain & Mental Health at HSC.

The Sachs Award is meant to recognize an individual of "international stature" for "leading research in neuroscience with relevance to the care of children with neurological disorders". Steven's body of research has been driven by his passion to improve the care and outcome of the vulnerable at-risk infants who populate our NICUs and bear a disproportionate burden of morbidity encountered in clinical pediatric neurology practice. Steven has utilized cutting edge, and continually evolving, imaging techniques and modalities to further our understanding of disturbed brain development in not one, but three discrete populations of interest that collectively form the majority of at-risk newborns; preterm infants, infants with congenital heart disease, and term asphyxiated newborns. Through carefully designed protocols coupled with well-defined longitudinal clinically relevant outcomes, Steven has demonstrated in each of these groups the influence of modifiable environmental factors encountered in clinical care on brain structure and function. These environmental factors have included infections, cardiorespiratory parameters, and the frequency of painful procedures experienced by a critically ill newborn. Most recently, his research draws attention to the importance of social disparities to understanding why neonates with similar brain injuries often have widely divergent neurological outcomes. For each of these factors identified, clinical applicability is evident that, when applied to the NICU care setting, will improve outcomes for a significant subset of children who eventually experience neurodevelopmental disabilities. Taken together, his research has established the "importance of the everyday" experience of critically ill newborns on brain development and subsequent neurodevelopmental outcomes.

Standard metrics testify to Steven's research impact. Over 220 peer reviewed publications, over 25 chapters/invited papers (including the chapter on hypoxic-ischemic brain injury in the term newborn in the 6th Edition of Swaiman's definitive textbook, and the chapter on white matter injury in *Avery's Textbook of Neonatology*), and one co-edited reference book on acquired brain injury in the fetus and newborn. His h index is currently 80, his papers have been cited over 20,000 times, and he currently has 67 papers with more than 100



citations. Steven has received numerous operating grants from both North American national granting agencies (NIH/NIHNS, CIHR) as well as varied Foundations (March of Dimes, Gerber, American Heart Association). Of particular note is his role as one of three co-Principal Investigators of the Child-BRIGHT Strategy for Patient Oriented Research (SPOR) network. This is a \$25 million dollar 5-year grant that represents the largest dollar value child health research effort in Canadian history. This national network features as its *raison d'être* family engagement to drive a research program in brain-based disabilities targeting challenges relevant to the lives of affected families. He has also been appointed by his peer community to the Institute Advisory Board of the Canadian Institute of Health Research (CIHR), Institute of Human Development and Child and Youth Health (IHDCYH), and now serves as its Vice-Chair. This is the highest level of pediatric research governance nationally in Canada. He is presently an Associate Editor of *Pediatric Research*, one of child health's leading research-focused journals.

The secret for Steven's extraordinary success as a researcher is his ability to leverage his intelligence and passion to work collaboratively. He is skilled at bringing together and creating complementary teams that involve families, clinicians, basic scientists, imaging experts (acquisition & analysis), and rehabilitation professionals. He is **always thinking** about the next question to be asked, extrapolating both from the acquired science and the observations of an astute clinician. These attributes are also evidenced in his leadership activities. For example, the Centre for Brain & Mental Health at HSC that he led for a decade of stupendous growth now brings together over 1500 faculty, staff and trainees to collaboratively improve the brain health and mental health children and youth.

Steven's excellence and leadership has been recognized by numerous awards including the Physician Researcher Award for Scientific Accomplishment HSC (2021), Newburger-Bellinger Award (2016), the Prichard Award from the International Child Neurology Association (2014), the Young Alumni Award from the Faculty of Medicine of McGill University (2011), and the Medal in Medicine from the Royal

College of Physicians & Surgeons of Canada (2010). Most recently, he was awarded the 2021 Children's Healthcare Canada Award for Individual Leadership for his efforts to improve the health of children nationally.

The Sachs Awardee must also be an "outstanding teacher and scholar". A testament to Steven's teaching ability that is self-evident is his frequent national and international speaking invitations, which number over 80 in the past 5 years alone. He is a sought out mentor and graduate studies and fellowship supervisor. He has also contributed enormously to his scholarly community, having served as President of the prestigious Society for Pediatric Research (2013-2014) and Chair of the CNS Scientific Program Committee (2009-2011). Steven is passionate about fostering the success of academic Child Neurologists in North America and beyond. He has trained over 35 neonatal neurology fellows; his graduates are presently thriving in diverse academic faculty roles. His colleagues have recognized his education contributions with the 2019 CanMEDS Excellence award. As his long-time mentor, I have been especially proud of his thorough commitment to mentorship through continually fostering the career development of his trainees across multiple levels of experience and a number of disciplines.

Recently Steve has begun a new phase in his personal and professional trajectory, having assumed in April 2022 the Chair of the Department of Pediatrics at the University of British Columbia and Pediatrician-in-Chief at the British Columbia Children's Hospital. Given his intelligence, passion and energy, one can only anticipate an impactful and transformative mandate at this institution.

His professional world aside, Steve is a devoted family man. He has an extraordinary life partnership with Mina Matsuda-Abedini, a highly respected pediatric nephrologist. Together they have raised two children, Hana and Sam. Hana is starting university studies at McGill University and Sam is a high school student. Steve can without fail be found in attendance in support of their extra-curricular activities.

2022 Hower Award

Leon G. Epstein, MD

BY MATTHEW KIRSCHEN, MD, PHD



The Child Neurology Society has chosen Leon G. Epstein, MD as the 2022 Hower Award recipient for his contributions to the CNS and his achievements as an accomplished academic neuroscientist, gifted educator, master clinician, and forward-thinking neuroethicist. Dr. Epstein is the ideal

choice for this award as he serves as a role model for child neurologists and trainees worldwide.

Dr. Epstein was born in the Bronx and grew up in a working-class neighborhood before moving to Levittown on Long Island, New York. After graduating high school, he traveled to Michigan where he intended to major in aerospace engineering at the University of Michigan. However, due to his insatiable curiosity, his studies took him on a journey through philosophy, politics, and eventually to medicine. He graduated from Detroit's Wayne State University School of Medicine in 1973 and subsequently completed an internship in pediatrics at St. Joseph's Mercy Hospital, University of Michigan. He ventured to the University of Arizona for his residency in Neurology and then back to New York in 1976 for his fellowship in Child Neurology at Columbia Presbyterian Medical Center under the mentorship of Sidney Carter.

Dr. Epstein's first faculty appointment was as an assistant professor of Pediatrics and Neurology at the State University of New York at Stony Brook in 1978. Two years later, he transitioned to the University of Medicine and Dentistry of New Jersey (UMDNJ) to join Richard Koenigsberger and became an Associate Professor and Director of the Neurology Clinic at Children's Hospital of New Jersey. During this time,

Dr. Epstein cultivated his interest in neurovirology with a particular interest in the neurologic manifestations of HIV in children. He reported the first series of children with AIDS in the United States. These children were born to HIV-infected mothers and had neurologic manifestations including a progressive encephalopathy.

To further his skill set and address the pathogenesis of HIV in the nervous system, Dr. Epstein spent time in the 1980s as a visiting scientist at the National Institute of Neurological Disorders and Stroke (NINDS) in Carleton Gajdusek's Laboratory for Central Nervous System Studies, and in the University of Amsterdam's Human Retrovirus Laboratory. Dr. Epstein's ensuing work included seminal papers on the pathophysiology of HIV infection in the brain. He conducted groundbreaking studies that identified HIV-1 infection of macrophages/microglia in the brain and demonstrated that brain inflammatory pathways were important in causing HIV-induced brain injury. He also served as a consultant to the CDC, surgeon general, and WHO regarding HIV in children.

In 1989, Dr. Epstein left New Jersey for the University of Rochester School of Medicine where he continued his HIV-focused neurovirology work as the Director of the Laboratory of Molecular Neurovirology. His work has been NIH-funded since 1986. He was the Principal Investigator of several multi-center studies evaluating cognitive impairment in HIV infected individuals, including the Northeastern AIDS Dementia study and a project using quantitative MRI to develop neuroimaging biomarkers of HIV-related cognitive decline. Dr. Epstein has led several clinical trials aimed at treating HIV-associated cognitive decline. To further these efforts, he received a Fulbright scholarship and spent a year in Chester Beatty's laboratory at the Institute for Cancer Research at the University of London.

Dr. Epstein's research has also demonstrated the impact of viral infections, and Human Herpesvirus-6 (HHV-6) in particular, for their role in causing febrile seizures in young



children. He was a co-investigator in the large prospective Consequences of Prolonged Febrile Seizures in Childhood (FEBSTAT) study, which determined that infections with either HHV-6 or -7 account for one-third of cases of febrile status epilepticus.

In 1999, Dr. Epstein left the University of Rochester to become the Division Head of Neurology at the Ann and Robert H. Lurie Children's Hospital of Chicago. He is currently the Derry A. and Donald L. Shoemaker Professor of Pediatrics and Neurology at the Northwestern University Feinberg School of Medicine. He has served in a variety of leadership roles, including Associated Program Director for the Northwestern University General Clinical Research Center and Medical Director for the Clinical Research Unit, a part of the Northwestern University Clinical and Translational Science Institute. He is beloved by his faculty and scores of trainees. His trainees claim that he is the most approachable department chair in the country and appreciate his participation in educational conferences, sharing countless teaching pearls (even if his discussion points are long-winded).

Dr. Epstein has a longstanding interest in biomedical ethics. He chaired the Child Neurology Society Ethics Committee and has been a member, for more than a decade, of the Ethics, Law, and Humanities Committee (ELHC), a combined committee of the American Academy of Neurology (AAN), the American Neurological Association (ANA) and the CNS, serving as Chair since 2019. In this role, he has co-authored AAN position statements concerning ethical issues including neuroenhancement, brain death, clinical research, physician-assisted death, drug pricing, social media, health disparities, and racial justice. During the sometimes tense deliberations to construct these position

statements, he is a keen listener, sensitive to the perspectives of all stakeholders, and a masterful facilitator of often opposing opinions. He leads by example and creates an environment where people respect one another. He is kind and patient, always willing to seek out the good in people and give them the benefit of the doubt.

On a personal level, he has been married for over 40 years to Jane Holl, MD MPH, a pediatrician and health services researcher; they have three adult sons. Dr. Epstein has endless curiosity. He loves to ask questions and generate excitement and curiosity in others. His curiosity extends beyond his clinical work and is evidenced by how he enriches his life by hiking in and climbing alpine mountains and building and playing acoustic guitars.

Dr. Epstein is devoted to his patients, colleagues, students, and our larger community. He has a strong social conscience and works tirelessly as an advocate for neurologic health, especially for children with disabilities and chronic neurologic illness. His academic accomplishments, leadership skills, teaching, and clinical skills set the bar high for those of us who follow in his path.



Where the Next
50 Years Begins

Outstanding Junior Member Award Renamed to Honor Tae Chang

Presented at Kenneth F. Swaiman CNS Legacy Luncheon

Reservation/Ticket required



Taeun Chang, MD (1971-2022)

The CNS Executive Committee unanimously approved renaming the Outstanding Junior Member Awards in honor of Tauen Chang, MD following her untimely passing last spring.

Since its inception in 1996, four awards have been presented to residents at each CNS Annual Meeting for work submitted to the Scientific Selection Committee. Tae was one of the first and one of the few residents to win the award twice (2002 and 2003). This year's first Tae Chang Junior Member Awards will be presented at the October 12 Legacy Luncheon, with all proceeds from luncheon ticket sales going to the newly renamed Tauen Chang Outstanding Junior Member Award. Below is the announcement written by Drs. William Gaillard and Phillip Pearl for the June 19 "eConnections".

Dear Fellow CNS Members:

It is with a heavy heart that we sadly announce the death of Taeun Chang, MD on June 18, 2022 from complications of a diagnosis of cancer that she received in late 2020. Tae was a beloved faculty member at Children's National, where she spent her entire career after starting her child neurology

residency in 2000. She attended MIT in Cambridge, MA for her undergraduate degree, the George Washington University School of Medicine, and pediatric residency at the Children's Hospital in Pittsburgh. Tae held the rank of Professor of Neurology and Pediatrics at GW, and received the 2022 Children's National Hospital Clinical Research Mentoring Award. An exceptional neonatal neurologist, she was selfless in fostering and supporting the careers of those around her. The many, many accolades from mentees, describing her effects on their careers and lives, are heartwarming, and can be summarized by the words in the nomination packet from Roger Packer MD, Senior Vice President, Center for Neuroscience Research, and her longtime chair and mentor at Children's National:

"...in both patient care and research, Dr. Chang has acted as a role model for both her colleagues in neurology and for two generations of pediatric neurology fellows, many of whom not only learned from Dr. Chang, but followed her path as a clinical/translational researcher in child neurology, especially neonatal neurology. She championed their professional development, involving them in ongoing studies and has always been available to trainees and peers, both within neurology and neonatology...These activities and her personal guidance, as well as encouragement with a focus on true academic productivity, has resulted in many trainees entering the neonatal neurology discipline and pursuing academic careers."

Most striking was the anecdote that Tae shared with us at the Saturday

morning symposium on Fetal Neurology during the 2019 Child Neurology Society in Charlotte (photo above), when she explained that a couple decided to continue their pregnancy following Dr. Chang's fetal consultation, and named their baby after her. What a legacy!

We mourn the early loss of this colleague and friend.

William Davis Gaillard, MD
Chief, Child Neurology,
Children's National Hospital
Associate Director, Center for
Neuroscience, Children's National
Research Institute

Phillip L. Pearl, MD
Immediate Past President,
Child Neurology Society
Children's National 1997-2013
Boston Children's Hospital 2014-present



(L-R): Children's National Hospital colleagues, Tauen Chang, MD (2002 & 2003 Outstanding Junior Member Award); Adeline Vanderver, MD (2003 CNS Outstanding Junior Member Award); William D. Gaillard, MD (Chief, Child Neurology); and Andrea Gropman (1996 & 1998 CNS Outstanding Junior Member Award)

Tauen Chang Outstanding Junior Member Award



Mekka Garcia
NYU Langone Health



Laura Gilbert
Washington University



Riley Kessler
Children's Hospital of
Philadelphia



Ezgi Saylam
Nationwide Children's
Hospital

Tauen Chang Outstanding Junior Member Post Graduate Award



Travis Larsh
Cincinnati Children's Hospital
Medical Center



Avantika Singh
Boston Children's Hospital

M. Richard Koenigsberger Scholarship



Stephen Chrzanowski
Boston Children's Hospital

AAP Section on Neurology Trainee Travel Award



Alexis Karlin
Children's Hospital of
Philadelphia

Bhuwan Garg High School Student Neuroscience Prize



Aliya Fisher
New York, NY

Bernard D'Souza International Fellowship Award

The Child Neurology Society is pleased to present the 2022 Bernard D'Souza International Fellowship Awards to Dr. Robert K. Sebunya, from Uganda, and Dr. Paulina C. Tejada, from Chile. Drs. Sebunya and Tejada were actually selected among a strong field of applicants in 2021, but COVID-related travel restrictions precluded their attending the 50th CNS Annual Meeting in Boston. Both D'Souza Fellows will present their work on posters displayed in the Exhibit Hall, and both will give talks as part of the Thursday afternoon Global Neurology symposium organized by CNS International Affairs Committee Chair, Jorge Vidaurre, MD. As part of the fellowship award, funded by years of accumulated (and wisely invested) \$10 check-off contributions included as part of CNS members' annual dues, Drs. Sebunya and Tejada will spend a week completing clerkships at Nationwide Children's Hospital under the direction of Dr. Vidaurre before returning to their research and patients in Uganda and Chile.



Robert K. Sebunya
Kampala, Uganda



Paulina C. Tejada
Santiago, Chile



CNF Young Researcher Grants and
Medical Student Scholarship profiles
appear on pages 85-87.



A Note of Thanks to the ABPN

On behalf of the CNS, we would like to thank the American Board of Psychiatry and Neurology (ABPN) for a generous \$100,000 grant in support of non-CNS member educational access in 2022 to the CNS Lifelong Learning website and annual meeting. Recognizing the disruptive impact COVID-19 has had and continues to have on continuing education and recertification plans for child and adult neurologists, the ABPN grant will enable all ABPN diplomates (whether or not they are CNS members) to access CNS remote self-assessment exams and CNS annual meeting CME content at member rates.

Bruce Cohen, MD
President

Monique Terrell
Executive Director



Offering no-charge genetic testing to aid in a differential diagnosis

Not knowing the underlying cause of a child's signs and symptoms can be challenging for families. The PTC Pinpoint™ testing program is designed to help by offering:



No-charge testing for neurotransmitter disorders and cerebral palsy (CP) of unknown etiology



Genetic counseling post testing



Family follow-up testing

PTC Therapeutics and Invitae have partnered to provide no-charge genetic testing, genetic counseling, and family screening programs for individuals with a suspected neurotransmitter disorder, such as Aromatic L-amino Acid Decarboxylase (AADC) deficiency or CP of unknown etiology.

**For more information, please visit
PTCPinpoint.com.**



Kenneth F. Swaiman CNS Legacy Luncheon

Presentation of 2022 Awards

Wednesday, October 12, 11:30 AM – 1:30 PM

Reservation/Ticket required

Arnold P. Gold Foundation Humanism in Medicine Award

Presented to Jorge Vidaurre, MD

Introduced by E. Steve Roach, MD

Bernard D'Souza International Fellows Award

Robert K. Sebunya, MD, M.Phil

Kampala, Uganda

Paulina C. Tejada, MD

Chile, Santiago, Chile

Introduced by Jorge Vidaurre, MD; Chair,
CNS International Affairs Committee

Roger & Mary Brumback Lifetime Achievement Awards

Presented to Jeffrey Buchhalter, MD, PhD

Introduced by William Trescher, MD

Presented to Roger Larson, CAE

Introduced by Barry Kosofsky, MD, PhD and
Nina Schor, MD, PhD

Presented to Michael Noetzel, MD

(accepted by his daughter, Anna Noetzel)

Introduced by Christina Gurnett, MD, PhD

CNS-PECN Training Director Award

Presented to Tim Lotze, MD

Introduced by Michael Lopez, MD

Presentation of 2022 Junior Member Awards



Where the Next
50 Years Begins

Join Us for a **Biogen-Sponsored** Product Theater

The Latest NURTURE Trial Data on Pediatric Patients: Potential Benefits of Early Treatment With SPINRAZA® (nusinersen)

Spinal muscular atrophy (SMA) is a progressive, genetic neuromuscular disease with a broad spectrum of severity in children and adults. Individuals with SMA have an urgent need for diagnosis and treatment.

Come listen to our SMA experts, Dr. Diana Castro and Dr. Gyula Acsadi, to learn about the potential benefits of early therapeutic intervention and the efficacy and safety of SPINRAZA in presymptomatic infants.^{1,2}

We will then explore the latest data from the NURTURE study in presymptomatic infants, including its study design, baseline characteristics, and primary and secondary outcomes.³

FEATURING:



Diana Castro, MD
Neurologist and
Neuromuscular Physician
Founder of Neurology
Rare Disease Center
Fort Worth, Texas



Gyula Acsadi, MD, PhD
Pediatric Neurologist
Chief of Pediatric Neurology
Connecticut Children's
Medical Center
Hartford, Connecticut

October 12, 2022 | **3:00 PM - 4:00 PM EDT**

Duke Energy Center, Exhibit Floor, Level 1

Please visit us at booth 1913

to learn more about the recent data on SPINRAZA
in presymptomatic infants with SMA.^{3,4}

INDICATION

SPINRAZA® (nusinersen) is indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

IMPORTANT SAFETY INFORMATION

Coagulation abnormalities and thrombocytopenia, including acute severe thrombocytopenia, have been observed after administration of some antisense oligonucleotides. Patients may be at increased risk of bleeding complications.

In the sham-controlled studies for patients with infantile-onset and later-onset SMA, 24 of 146 SPINRAZA-treated patients (16%) with high, normal, or unknown platelet count at baseline developed a platelet level below the lower limit of normal, compared to 10 of 72 sham-controlled patients (14%). Two SPINRAZA-treated patients developed platelet counts <50,000 cells per microliter, with the lowest level of 10,000 cells per microliter recorded on study day 28.

Renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. SPINRAZA is present in and excreted by the kidney. In the sham-controlled studies for patients with infantile-onset and later-onset SMA, 71 of 123 SPINRAZA-treated patients (58%) had elevated urine protein, compared to 22 of 65 sham-controlled patients (34%).

Laboratory testing and monitoring to assess safety should be conducted. Perform a platelet count, coagulation laboratory testing, and quantitative spot urine protein testing at baseline and prior to each dose of SPINRAZA and as clinically needed.

Severe hyponatremia was reported in an infant treated with SPINRAZA requiring salt supplementation for 14 months.

Cases of rash were reported in patients treated with SPINRAZA.

SPINRAZA may cause a reduction in growth as measured by height when administered to infants, as suggested by observations from the controlled study. It is unknown whether any effect of SPINRAZA on growth would be reversible with cessation of treatment.

The most common adverse reactions (≥20% of SPINRAZA-treated patients and ≥5% more frequently than in control patients) that occurred in the infantile-onset controlled study were lower respiratory infection and constipation. Serious adverse reactions of atelectasis were more frequent in SPINRAZA-treated patients (18%) than in control patients (10%). Because patients in this controlled study were infants, adverse reactions that are verbally reported could not be assessed. The most common adverse reactions that occurred in the later-onset controlled study were pyrexia, headache, vomiting, and back pain. Post-lumbar puncture syndrome has also been observed after the administration of SPINRAZA.

Please see the Brief Summary of Prescribing Information on the following pages.

References

1. Govoni A, et al. *Mol Neurobiol*. 2018;55(8):6307-6318.
2. Finkel RS, et al. *N Engl J Med*. 2017;377(18):1724.
3. Crawford TO, et al. Oral presentation at: Muscular Dystrophy Association, 4th Clinical and Scientific Conference; March 13-16, 2022. Accessed April 19, 2022.
4. SPINRAZA Prescribing Information. Cambridge, MA: Biogen.



© 2022 Biogen. All rights reserved. SPZ-US-5327 09/22
225 Binney Street, Cambridge, MA 02142



SPINRAZA® (nusinersen) injection, for intrathecal use

Brief Summary of Full Prescribing Information

1 INDICATIONS AND USAGE

SPINRAZA is indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

2 DOSAGE AND ADMINISTRATION

2.1 Dosing Information

SPINRAZA is administered intrathecally by, or under the direction of, healthcare professionals experienced in performing lumbar punctures.

Recommended Dosage

The recommended dosage is 12 mg (5 mL) per administration.

Initiate SPINRAZA treatment with 4 loading doses. The first three loading doses should be administered at 14-day intervals. The 4th loading dose should be administered 30 days after the 3rd dose. A maintenance dose should be administered once every 4 months thereafter.

Missed Dose

If a loading dose is delayed or missed, administer SPINRAZA as soon as possible, with at least 14-days between doses and continue dosing as prescribed.

If a maintenance dose is delayed or missed, administer SPINRAZA as soon as possible and continue dosing every 4 months.

2.2 Important Preparation and Administration Instructions

SPINRAZA is for intrathecal use only.

Prepare and use SPINRAZA according to the following steps using aseptic technique. Each vial is intended for single dose only.

Preparation

- Store SPINRAZA in the carton in a refrigerator until time of use.
- Allow the SPINRAZA vial to warm to room temperature (25°C/77°F) prior to administration. Do not use external heat sources.
- Inspect the SPINRAZA vial for particulate matter and discoloration prior to administration. Do not administer SPINRAZA if visible particulates are observed or if the liquid in the vial is discolored. The use of external filters is not required.
- Withdraw 12 mg (5 mL) of SPINRAZA from the single-dose vial into a syringe and discard unused contents of the vial.
- Administer SPINRAZA within 4 hours of removal from vial.

Administration

- Consider sedation as indicated by the clinical condition of the patient.
- Consider ultrasound or other imaging techniques to guide intrathecal administration of SPINRAZA, particularly in younger patients.
- Prior to administration, remove 5 mL of cerebrospinal fluid.
- Administer SPINRAZA as an intrathecal bolus injection over 1 to 3 minutes using a spinal anesthesia needle [see *Dosage and Administration* (2.1)]. Do not administer SPINRAZA in areas of the skin where there are signs of infection or inflammation [see *Adverse Reactions* (6.3)].

2.3 Laboratory Testing and Monitoring to Assess Safety

Conduct the following laboratory tests at baseline and prior to each dose of SPINRAZA and as clinically needed [see *Warnings and Precautions* (5.1, 5.2)]:

- Platelet count
- Prothrombin time; activated partial thromboplastin time
- Quantitative spot urine protein testing

3 DOSAGE FORMS AND STRENGTHS

Injection: 12 mg/5 mL (2.4 mg/mL) nusinersen as a clear and colorless solution in a single-dose vial.

4 CONTRAINDICATIONS

None.

5 WARNINGS AND PRECAUTIONS

5.1 Thrombocytopenia and Coagulation Abnormalities

Coagulation abnormalities and thrombocytopenia, including acute severe thrombocytopenia, have been observed after administration of some antisense oligonucleotides.

In the sham-controlled studies for patients with infantile-onset and later-onset SMA, 24 of 146 (16%) SPINRAZA-treated patients with high, normal, or unknown platelet count at baseline developed a platelet level below the lower limit of normal, compared to 10 of 72 (14%) sham-controlled patients.

In the sham-controlled study in patients with later-onset SMA (Study 2), two SPINRAZA-treated patients developed platelet counts less than 50,000 cells per microliter, with a lowest level of 10,000 cells per microliter recorded on study day 28.

Because of the risk of thrombocytopenia and coagulation abnormalities from SPINRAZA, patients may be at increased risk of bleeding complications.

Perform a platelet count and coagulation laboratory testing at baseline and prior to each administration of SPINRAZA and as clinically needed.

5.2 Renal Toxicity

Renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. SPINRAZA is present in and excreted by the kidney [see *Clinical Pharmacology* (12.3)]. In the sham-controlled studies for patients with infantile-onset and later-onset SMA, 71 of 123 (58%) of SPINRAZA-treated patients had elevated urine protein, compared to 22 of 65 (34%) sham-controlled patients. Conduct quantitative spot urine protein testing (preferably using a first morning urine specimen) at baseline and prior to each dose of SPINRAZA. For urinary protein concentration greater than 0.2 g/L, consider repeat testing and further evaluation.

6 ADVERSE REACTIONS

The following serious adverse reactions are described in detail in other sections of the labeling:

- Thrombocytopenia and Coagulation Abnormalities [see *Warnings and Precautions* (5.1)]
- Renal Toxicity [see *Warnings and Precautions* (5.2)]

6.1 Clinical Trials Experience

Because clinical trials are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of SPINRAZA cannot be directly compared to rates in clinical trials of other drugs and may not reflect the rates observed in practice.

In clinical studies, 346 patients (47% male, 76% Caucasian) were treated with SPINRAZA, including 314 exposed for at least 6 months, 258 exposed for at least 1 year, and 138 exposed for at least 2 years. The safety of SPINRAZA was studied in presymptomatic infants with SMA; pediatric patients (approximately 3 days to 16 years of age at first dose) with symptomatic SMA; in a sham-controlled trial in infants with symptomatic SMA (Study 1; n=80 for SPINRAZA, n=41 for control); in a sham-controlled trial in children with symptomatic SMA (Study 2; n=84 for SPINRAZA, n=42 for control); an open-label study in presymptomatic infants (Study 3, n=25) and other studies in symptomatic infants (n=54) and later-onset patients (n=103). In Study 1, 58 patients were exposed for at least 6 months and 28 patients were exposed for at least 12 months. In Study 2, 84 patients were exposed for at least 6 months and 82 patients were exposed for at least 12 months.

Clinical Trial in Infantile-Onset SMA (Study 1)

In Study 1, baseline disease characteristics were largely similar in the SPINRAZA-treated patients and sham-control patients except that SPINRAZA-treated patients at baseline had a higher percentage compared to sham-control patients of paradoxical breathing (89% vs 66%), pneumonia or respiratory symptoms (35% vs 22%), swallowing or feeding difficulties (51% vs 29%), and requirement for respiratory support (26% vs 15%).

The most common adverse reactions that occurred in at least 20% of SPINRAZA-treated patients and occurred at least 5% more frequently than in control patients were lower respiratory infection and constipation. Serious adverse reactions of atelectasis were more frequent in SPINRAZA-treated patients (18%) than in control patients (10%). Because patients in Study 1 were infants, adverse reactions that are verbally reported could not be assessed in this study.

Table 1. Adverse Reactions that Occurred in at Least 5% of SPINRAZA Patients and Occurred at Least 5% More Frequently or At Least 2 Times as Frequently Than in Control Patients with Infantile-Onset SMA (Study 1)

Adverse Reactions	SPINRAZA 12 mg ¹ N=80 %	Sham-Procedure Control N=41 %
Lower respiratory infection ²	55	37
Constipation	35	22
Teething	18	7
Urinary tract infection	9	0
Upper respiratory tract congestion	8	2
Ear infection	6	2
Flatulence	5	2
Decreased weight	5	2

¹ Loading doses followed by 12 mg (5 mL) once every 4 months

² Includes adenovirus infection, bronchiolitis, bronchitis, bronchitis viral, corona virus infection, Influenza, lower respiratory tract infection, lower respiratory tract infection viral, lung infection, parainfluenzae virus infection, pneumonia, pneumonia bacterial, pneumonia influenzal, pneumonia moraxella, pneumonia parainfluenzae viral, pneumonia pneumococcal, pneumonia pseudomonal, pneumonia respiratory syncytial viral, pneumonia viral, and respiratory syncytial virus bronchiolitis.

SPINRAZA® (nusinersen) injection, for intrathecal use

Brief Summary of Full Prescribing Information (cont'd)

Clinical Trial in Infantile-Onset SMA (Study 1) (cont'd)

In an open-label clinical study in infants with symptomatic SMA, severe hyponatremia was reported in a patient treated with SPINRAZA requiring salt supplementation for 14 months.

Cases of rash were reported in patients treated with SPINRAZA. One patient, 8 months after starting SPINRAZA treatment, developed painless red macular lesions on the forearm, leg, and foot over an 8-week period. The lesions ulcerated and scabbed over within 4 weeks, and resolved over several months. A second patient developed red macular skin lesions on the cheek and hand ten months after the start of SPINRAZA treatment, which resolved over 3 months. Both cases continued to receive SPINRAZA and had spontaneous resolution of the rash.

SPINRAZA may cause a reduction in growth as measured by height when administered to infants, as suggested by observations from the controlled study. It is unknown whether any effect of SPINRAZA on growth would be reversible with cessation of treatment.

Clinical Trial in Later-Onset SMA (Study 2)

In Study 2, baseline disease characteristics were largely similar in the SPINRAZA-treated patients and sham-control patients except for the proportion of SPINRAZA-treated patients who had ever achieved the ability to stand without support (13% vs 29%) or walk with support (24% vs 33%).

The most common adverse reactions that occurred in at least 20% of SPINRAZA-treated patients and occurred at least 5% more frequently than in control patients were pyrexia, headache, vomiting, and back pain.

Table 2. Adverse Reactions that Occurred in at Least 5% of SPINRAZA Patients and Occurred at Least 5% More Frequently or At Least 2 Times as Frequently Than in Control Patients with Later-Onset SMA (Study 2)

Adverse Reactions	SPINRAZA 12 mg ¹ N=84 %	Sham-Procedure Control N=42 %
Pyrexia	43	36
Headache	29	7
Vomiting	29	12
Back pain	25	0
Epistaxis	7	0
Fall	5	0
Respiratory tract congestion	5	2
Seasonal allergy	5	2

¹ Loading doses followed by 12 mg (5 mL) once every 6 months

Post-lumbar puncture syndrome has also been observed after administration of SPINRAZA.

6.2 Immunogenicity

As with all oligonucleotides, there is potential for immunogenicity. The detection of antibody formation is highly dependent on the sensitivity and specificity of the assay. Additionally, the observed incidence of antibody (including neutralizing antibody) positivity in an assay may be influenced by several factors, including assay methodology, sample handling, timing of sample collection, concomitant medications, and underlying disease. For these reasons, comparison of the incidence of antibodies to nusinersen in the studies described below with the incidence of antibodies in other studies or to other products may be misleading.

The immunogenic response to nusinersen was determined in 294 patients with post-baseline plasma samples evaluated for anti-drug antibodies (ADAs). Seventeen patients (6%) developed treatment-emergent ADAs, of which 5 were transient, 12 were considered to be persistent. Persistent was defined as having one positive test followed by another one more than 100 days after the first positive test. In addition, "persistent" is also defined as having one or more positive samples and no sample more than 100 days after the first positive sample. Transient was defined as having one or more positive results and not confirmed to be persistent. There are insufficient data to evaluate an effect of ADAs on clinical response, adverse events, or the pharmacokinetic profile of nusinersen.

6.3 Postmarketing Experience

The following adverse reactions have been identified during post-approval use of SPINRAZA. Because these reactions are reported voluntarily from a population of uncertain size, it is not always possible to reliably estimate their frequency or establish a causal relationship to drug exposure.

Serious infections associated with lumbar puncture, such as meningitis, have been observed. Hydrocephalus, aseptic meningitis, and hypersensitivity reactions (e.g. angioedema, urticaria, rash) have also been reported.

8 USE IN SPECIFIC POPULATIONS

8.1 Pregnancy

Risk Summary

There are no adequate data on the developmental risk associated with the use of SPINRAZA in pregnant women. When nusinersen was administered by subcutaneous injection to mice throughout pregnancy and lactation, developmental toxicity (long-term neurobehavioral impairment) was observed at all doses tested (*see Data*). In the U.S. general population, the estimated background risk of major birth defects and miscarriage in clinically recognized pregnancies is 2 to 4% and 15 to 20%, respectively. The background risk of major birth defects and miscarriage for the indicated population is unknown.

Data

Animal Data

When nusinersen (0, 3, 10, or 25 mg/kg) was administered subcutaneously to male and female mice every other day prior to and during mating and continuing in females throughout organogenesis, no adverse effects on embryofetal development were observed. Subcutaneous administration of nusinersen (0, 6, 12.6, or 25 mg/kg) to pregnant rabbits every other day throughout organogenesis produced no evidence of embryofetal developmental toxicity.

When nusinersen (1.4, 5.8, or 17.2 mg/kg) was administered to pregnant female mice by subcutaneous injection every other day throughout organogenesis and continuing once every six days throughout the lactation period, adverse neurobehavioral effects (alterations in locomotor activity, learning and memory deficits) were observed when offspring were tested after weaning or as adults. A no-effect level for neurobehavioral impairment was not established.

8.2 Lactation

Risk Summary

There are no data on the presence of nusinersen in human milk, the effects on the breastfed infant, or the effects of the drug on milk production. Nusinersen was detected in the milk of lactating mice when administered by subcutaneous injection. The developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for SPINRAZA and any potential adverse effects on the breastfed infant from SPINRAZA or from the underlying maternal condition.

8.4 Pediatric Use

The safety and effectiveness of SPINRAZA in pediatric patients from newborn to 17 years have been established [*see Clinical Studies (14.1)*].

Juvenile Animal Toxicity Data

In intrathecal toxicity studies in juvenile monkeys, administration of nusinersen (0, 0.3, 1, or 3 mg/dose for 14 weeks and 0, 0.3, 1, or 4 mg/dose for 53 weeks) resulted in brain histopathology (neuronal vacuolation and necrosis/cellular debris in the hippocampus) at the mid and high doses and acute, transient deficits in lower spinal reflexes at the high dose in each study. In addition, possible neurobehavioral deficits were observed on a learning and memory test at the high dose in the 53-week monkey study. The no-effect dose for neurohistopathology in monkeys (0.3 mg/dose) is approximately equivalent to the human dose when calculated on a yearly basis and corrected for the species difference in CSF volume.

8.5 Geriatric Use

Clinical studies of SPINRAZA did not include sufficient numbers of subjects aged 65 and over to determine whether they respond differently from younger subjects.

17 PATIENT COUNSELING INFORMATION

Thrombocytopenia and Coagulation Abnormalities

Inform patients and caregivers that SPINRAZA could increase the risk of bleeding. Inform patients and caregivers of the importance of obtaining blood laboratory testing at baseline and prior to each dose to monitor for signs of increased potential for bleeding. Instruct patients and caregivers to seek medical attention if unexpected bleeding occurs [*see Warnings and Precautions (5.1)*].

Renal Toxicity

Inform patients and caregivers that SPINRAZA could cause renal toxicity. Inform patients and caregivers of the importance of obtaining urine testing at baseline and prior to each dose to monitor for signs of potential renal toxicity [*see Warnings and Precautions (5.2)*].

Manufactured for:

Biogen

Cambridge, MA 02142

SPINRAZA is a registered trademark of Biogen.

© Biogen 2016-2020

INTERACTIVE TAKEDA BRAIN

AUGMENTED
REALITY

**Experience the Takeda
Medical Affairs Booth in
Augmented Reality!**

#1919

Copyright © 2022 Takeda Pharmaceutical Company Limited. All rights reserved.
TAKEDA and the TAKEDA logo are registered trademarks of Takeda
Pharmaceutical Company Limited.

VV-MEDMAT-72657. 08/2022.



Learning Objectives

The 2022 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, neuro-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 and Child Neurology Society. 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 and enduring activity for a maximum of 30.75 AMA PRA Category 1 Credit(s)[™]. Physicians should claim only the credit commensurate with the extent of their participation in the activity.



CHILD NEUROLOGY SOCIETY



MINNESOTA
MEDICAL
ASSOCIATION

Schedule at a Glance

All meetings/sessions at The Duke Energy Center

SESSIONS highlighted in maroon are offered for CME credit as part of the CNS Scientific Program.

Live-streamed sessions:
Legacy Luncheon
SYMPOSIUM II - VI
Award Lectures

EXHIBITS & POSTER REVIEW

EXHIBIT HALL

WEDNESDAY
2:00 PM-7:30 PM

6:00 PM - 7:30 PM
Welcome Reception

Welcome Reception
Supported by local Ohio hosts: Akron Children's Hospital, Cincinnati Children's Hospital Medical Center, Nationwide Children's Hospital, and University Hospitals: Rainbow Babies & Children

THURSDAY
11:30 AM-7:00 PM
Lunch served
12:45 AM-2:00 PM
Poster Review

Wine & Cheese Reception
5:30 PM-7:00 PM
Poster Review

Tuesday, October 11

START	END	MEETING/SESSION	ROOM ASSIGNED
7:00 AM	6:00 PM	Nursing Room	210
12:00 PM	8:00 PM	Speaker Ready Room	250
12:00 PM	7:00 PM	CNS Registration	Main Foyer
12:30 PM	2:15 PM	CNS Pellock Resident Seminar on Epilepsy (Pre-registration required/SOLD OUT)	206
2:00 PM	6:00 PM	Poster Drop-Off/Pick-Up	Main Foyer
3:15 PM	5:15 PM	Pellock Resident Seminar on Epilepsy Breakout Sessions 1-7	TBA
6:00 PM	9:00 PM	Pellock Resident Seminar on Epilepsy Reception & Dinner	200&205

Wednesday, October 12

START	END	MEETING/SESSION	ROOM ASSIGNED
7:00 AM	7:30 PM	Speaker Ready Room	250
7:00 AM	7:30 PM	CNS Registration	Main Foyer
7:00 AM	7:00 PM	Poster Drop-Off/Pick-Up	Main Foyer
7:00 AM	6:00 PM	Nursing Room	210
8:00 AM	2:00 PM	Program Coordinators of Child Neurology	237
8:00 AM	5:00 PM	International Pediatric Stroke IPSS	236
8:00 AM	11:00 AM	Symposium I: CNF Symposium: Clinical Trials in Pediatric Neurology	Junior Ballroom AB
11:30 AM	1:30 PM	Kenneth F. Swaiman CNS Legacy Luncheon	Grand Ballroom
1:00 PM	2:30 PM	CNS Pellock Resident Seminar on Epilepsy (Pre-registration required/SOLD OUT)	206
1:30 PM	5:00 PM	Neuromodulation SIG – Meet & Greet/Sign-up	234
2:00 PM	3:30 PM	PECN Business Meeting (PECN Members only)	Junior Ballroom CD
2:00 PM	7:30 PM	EXHIBITS & POSTER REVIEW	Exhibit Hall A
3:00 PM	4:00 PM	Industry Sponsored Product Theater: Biogen	Exhibit Hall A
3:30 PM	5:30 PM	Pellock Resident Seminar on Epilepsy Breakout Sessions 1-7	TBA
3:30 PM	5:30 PM	PECN CME Program: Educational Tools	Junior Ballroom CD
6:00 PM	7:30 PM	Welcome Reception	Exhibit Hall A
7:30 PM	8:30 PM	Neurogenetics SIG – Networking Hour	264
7:30 PM	8:30 PM	TBI SIG – Networking Hour	251
8:00 PM	10:00 PM	Movement Disorders Video Rounds	Junior Ballroom CD

Thursday, October 13

START	END	MEETING/SESSION	ROOM ASSIGNED
6:00 AM	7:00 PM	Speaker Ready Room	250
7:00 AM	7:00 PM	CNS Registration	Main Foyer
7:00 AM	6:00 PM	Nursing Room	210
7:00 AM	8:00 AM	ACNS Editorial Board Meeting	207-208
7:00 AM	8:00 AM	Industry Sponsored Product Theater: Marinus	206
7:00 AM	9:00 AM	Platform Session I	Junior Ballroom AB
7:00 AM	9:00 AM	Platform Session II	Junior Ballroom CD
7:00 AM	9:00 AM	Platform Session III	230-233
8:00 AM	5:00 PM	Program Coordinators of Child Neurology	237
8:00 AM	4:00 PM	Association of Child Neurology Nurses (ACNN)	264
9:30 AM	12:15 PM	Symposium II Presidential Symposium: Quality and Capitated Care	Grand Ballroom
11:30 AM	7:00 PM	EXHIBITS & POSTER REVIEW	Exhibit Hall A
12:30 PM	2:30 PM	Industry Sponsored Satellite Symposium: Miller Medical Communications	200&205
12:30 PM	2:00 PM	Guided Poster Tour #1	Exhibit Hall A
12:30 PM	4:00 PM	Functional Neurological Disorders (FND) SIG – Meet & Greet/Sign-up	203
12:30 PM	4:00 PM	Neurogenetics SIG – Meet & Greet/Sign-up	201
12:30 PM	4:00 PM	Neuroimmune Disorders SIG – Meet Greet/Sign-up	211
12:30 PM	4:00 PM	TBI SIG – Meet & Greet/Sign-up	251
12:30 PM	2:00 PM	Lunch (Exhibits & Poster Review)	Exhibit Hall A
12:30 PM	1:30 PM	Industry Sponsored Product Theater: Genentech	Exhibit Hall A
2:30 PM	3:00 PM	Martha Bridge Denckla Award Lecture: Michael Shevell, MDCM, FRCP, FCAHS	Grand Ballroom
3:00 PM	5:15 PM	Symposium III: Global Neurology: Impact of COVID-19 Pandemic and Natural Disasters	Grand Ballroom
3:00 PM	7:00 PM	Poster Drop-Off/Pick-Up	Main Foyer
5:00 PM	7:00 PM	Guided Poster Tour #2	Exhibit Hall A
5:30 PM	7:00 PM	Poster Review: Authors present (Wine & Cheese Reception)	Exhibit Hall A
5:30 PM	7:00 PM	Industry Sponsored Satellite Symposium: PTC Therapeutics	200&205
6:00 PM	7:00 PM	Industry Sponsored Product Theater: Alexion	Exhibit Hall A
7:30 PM	8:30 PM	Demyelinating Disease SIG – Networking Hour	201
7:30 PM	8:30 PM	Functional Neurological Disorders (FND) SIG – Networking Hour	203
7:30 PM	8:30 PM	Headache SIG – Networking Hour	202
7:30 PM	8:30 PM	Neurodevelopment Disorders SIG – Networking Hour	212
7:30 PM	8:30 PM	Neuroimmune Disorders SIG – Networking Hour	211

Schedule at a Glance

SESSIONS highlighted in maroon are offered for CME credit as part of the CNS Scientific Program.

Industry Sponsored Sessions are accredited through independent CME providers and/or are Product Theaters offering no CME credit. See pages 54-56 for more information.

Friday, October 14

START	END	MEETING/SESSION	ROOM ASSIGNED
7:00 AM	6:00 PM	Speaker Ready Room	250
7:00 AM	6:00 PM	CNS Registration	Main Foyer
7:00 AM	4:00 PM	Poster Drop-Off/Pick-Up	Main Foyer
7:00 AM	6:00 PM	Nursing Room	210
7:00 AM	8:30 AM	Pediatric Neurology Journal – Editorial Board Meeting	207-208
7:30 AM	11:00 AM	Fetal Neurology SIG – Meet & Greet/Sign-up	251
8:00 AM	5:00 PM	Program Coordinators of Child Neurology	237
8:00 AM	8:15 AM	Child Neurology Foundation (CNF) Research Award/Grants Presentations	Grand Ballroom
8:15 AM	8:45 AM	Philip R. Dodge Young Investigator Award Lecture: Bhooma Aravamuthan, MD, DPhil	Grand Ballroom
8:30 AM	3:30 PM	Association of Child Neurology Nurses (ACNN)	264
8:45 AM	9:30 AM	Bernard Sachs Award Lecture: Steven Paul Miller, MDCM, MAS, FRCPC	Grand Ballroom
9:45 AM	12:00 PM	Symposium IV: Ethics: Neuropalliative Care Across the Age Spectrum	Grand Ballroom
12:00 PM	1:30 PM	Lunch	Junior Ballroom ABCD & Meeting Room 230-233
12:00 PM	2:00 PM	Industry Sponsored Satellite Symposium: Takeda	200&205
12:30 PM	1:45 PM	Seminar 1: Cerebral Palsy: What is CP? A Consensus-Based Approach	Junior Ballroom AB
12:30 PM	1:45 PM	Seminar 2: Neurodevelopmental Disorders: Challenges in Sickle Cell Disease	Junior Ballroom CD
12:30 PM	1:45 PM	Seminar 3: Neuro-Oncology: Acute Neuro-toxicities in Childhood Cancer Patients	230-233
12:30 PM	4:00 PM	Cerebral Palsy SIG – Meet & Greet/Sign-up	204
12:30 PM	4:00 PM	Demyelinating Disease SIG – Meet & Greet/Sign-up	201
12:30 PM	4:00 PM	Headache SIG – Meet & Greet/Sign-up	202
12:30 PM	4:00 PM	Neurodevelopment Disorders SIG – Meet & Greet/Sign-up	251
2:15 PM	4:30 PM	Symposium V: Neuroimmunology: Pediatric Neuroimmunological Diseases	Grand Ballroom
4:30 PM	5:00 PM	CNS Business Meeting	Grand Ballroom
5:00 PM	5:45 PM	Junior Member Forum	Junior Ballroom CD
5:30 PM	7:00 PM	Industry Sponsored Satellite Symposium: GeneDX	200&205
5:45 PM	6:30 PM	Junior Member Seminar 1: Medical Students: Finding a Residency	Junior Ballroom C/D
5:45 PM	6:30 PM	Junior Member Seminar 2: Residents: Finding a Fellowship	Junior Ballroom A/B
5:45 PM	6:30 PM	Junior Member Seminar 3: Residents & Fellows: Getting your First Job	Junior Ballroom A/B
6:15 PM	7:00 PM	Scientific Program Committee Meeting	207-208
6:30 PM	7:30 PM	Fetal Neurology SIG – Networking Hour	251
6:30 PM	7:30 PM	Neuromodulation SIG – Networking Hour	234
7:00 PM	9:00 PM	Closing Gala Reception	Junior Ballroom & Foyer

Saturday, October 15

START	END	MEETING/SESSION	ROOM ASSIGNED
6:00 AM	12:00 PM	Speaker Ready Room	250
7:00 AM	12:00 PM	CNS Registration	Main Foyer
7:00 AM	4:15 PM	Nursing Room	210
7:00 AM	8:15 AM	Seminar 4: Education: Incorporating Patients & Families into Research	Junior Ballroom AB
7:00 AM	8:15 AM	Seminar 5: Fetal Neurology: Advances in Fetal Neurology	Junior Ballroom CD
7:00 AM	8:15 AM	Seminar 6: Diversity: Disability in Child Neurology: Society, Medicine and the Person	230-233
8:45 AM	9:30 AM	Hower Award Lecture: Leon G. Epstein, MD	Grand Ballroom
9:45 AM	12:00 PM	Symposium VI: Behavioral Neurology: Anxiety and Mood Disorders Co-occurring with Neurologic Disorders	Grand Ballroom
12:15 PM	4:15 PM	Biomedical Writing Workshop (Pre-registration required/SOLD OUT)	264
12:15 PM	4:15 PM	CNS Clinical Research Annual Workshop 2022 – Clinical Trial Design (Pre-registration required/SOLD OUT)	207-208
3:30 PM	4:15 PM	Clinical Research Breakout Sessions 1-6	TBA

CME CREDIT

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

- Please complete survey form on Survey Monkey after attending all sessions for which you are requesting credit, whether live in Cincinnati or on virtual meeting platform.
- All CME accredited livestream sessions are recorded and will be available on the virtual platform OnDemand from November 1- December 1, 2022.
- Complete online survey to claim CME credit by December 15 (11:59 pm EST)

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

No CME credit for 2022 will be issued for surveys completed after December 15, 2022.



https://www.surveymonkey.com/r/2022_CNS_CME_survey

51st Annual Meeting of the CNS Scientific Program

Times & Rooms Subject to Change (check online Virtual Program)

WEDNESDAY October 12

8:00 AM – 11:00 AM

SYMPOSIUM I:

CHILD NEUROLOGY

FOUNDATION SYMPOSIUM:

CLINICAL TRIALS IN PEDIATRIC NEUROLOGY: OUR ROLE IN IMPROVING PARTICIPATION AND OUTCOMES (I/V/O)

*Supported by the
Child Neurology Foundation*

COURSE DESCRIPTION

This 3-hour interactive symposium, is designed to raise participant awareness of the importance of clinical trials to the child neurology community and to identify strategies to overcome existing barriers to accessing clinical trials for children with neurologic conditions. We will describe ethical considerations in clinical trials and possible patient participation. Participants will also learn best practices for engaging and supporting patients before, during and after their clinical trial journey.

LEARNING OBJECTIVES

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

- Identify strategies to overcome existing barriers to accessing clinical trials for children with neurologic conditions.
- Utilize best practices for engaging and supporting patients before, during and after their clinical trial journey.

IMPACT STATEMENTS

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

- Identifying clinical trials that will benefit my patients.

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

- Effectively recommending and discussing clinical trials to my patients

ORGANIZER:

Child Neurology Foundation

Welcome

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

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

The Importance of Clinical Trials to Patients

How Clinical Trials can Impact Patient Outcomes

Tracy Dixon-Salazar, PhD
Lennox-Gastaut Syndrome (LGS)
Foundation, San Diego, CA

Common Barriers to Patient Involvement in Clinical Trials

Kimbra Edwards, PhD
CISCRP, Boston, MA

The Critical Roles of the Provider

The Importance of Clinician Involvement and Possible Roles

Bruce H. Cohen, MD, FAAN
Akron Children's Hospital; Akron, OH

Typical Barriers and Practical Considerations to Clinicians in Fulfilling these Roles

E. Martina Bebin, MD, MPA
University of Alabama at
Birmingham, Birmingham, AL

Supporting Patients:

Best Practices for Discussing Clinical Trials with Patients

Shafali Spurling Jeste, MD
Children's Hospital Los Angeles,
Los Angeles, CA

Avoiding Common

Mistakes in Discussions

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

11:30 AM – 1:30 PM

KENNETH F. SWAIMAN

CNS LEGACY LUNCHEON (I/V/O)

Awards Presented

Arnold P. Gold Foundation Humanism in Medicine Award

Jorge Vidaurre, MD
Columbus, OH

Bernard D'Souza

International Fellowship Awards

Robert K. Sebunya, M.D., M.phil
Uganda Martyrs University Nkozi,
Mother Kevin Post Graduate School,
Kampala, Uganda

Paulina C. Tejada, MD

Pontificia Universidad Católica de
Chile, Santiago, Chile

Roger & Mary Brumback

Lifetime Achievement Award

Jeffrey Buchhalter, MD, PhD
Phoenix, AZ

Roger Larson, CAE

St. Paul, MN

Michael Noetzel, MD

St. Louis, MO

(presented posthumously)

CNS/PECN Training Director Award

Tim Lotze, MD

Baylor College of Medicine,
Texas Children's Hospital,
Houston, TX

**Bhuwan Garg High School
Neuroscience Award**
Aliya Fisher
New York, NY

Tauen Chang Junior Member Awards
Mekka Garcia, MD
Laura Gilbert, DO, MBA
Riley Kessler, MD
Ezgi Saylam, MD

**Tauen Chang
Outstanding Junior Member
Post-Grad Awards**
Travis Larsh, MD
Avantika Singh, MD

M. Richard Koenigsberger Scholarship
Stephen Chrzanowski, MD

**AAP Section of Neurology
Travel Grant**
Alexis Karlin, MD

2:00 PM – 3:30 PM
**PROFESSORS & EDUCATORS OF
CHILD NEUROLOGY (PECN)**

BUSINESS MEETING (I)

Introduction and Agenda
Nancy Bass, MD
University Hospitals of Cleveland/
Rainbow Babies and Children's Hospital,
Case Western Reserve University School
of Medicine, Cleveland, OH

**Preference Signaling and
the Match**
Margie Ream, MD, PhD
Nationwide Children's Hospital,
Columbus, OH

**Forgivable Family Leave for
Trainees with Q&A**
Margie Ream, MD, PhD

**RRC Change: Program Director
Minimum FTE Support with Q&A**
Danny Rogers, MD, PhD
University of New Mexico,
Albuquerque, NM

Match Report
Leon Dure, MD
Heersink School of Medicine,
University of Alabama at Birmingham,
Birmingham, AL

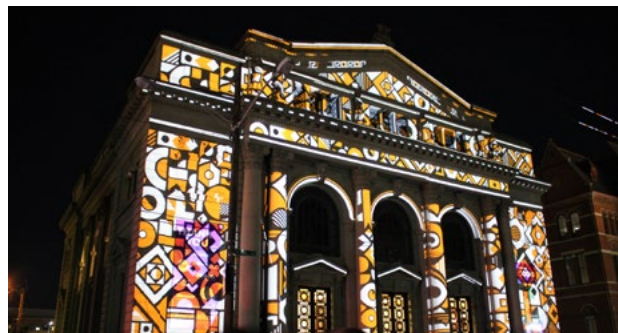
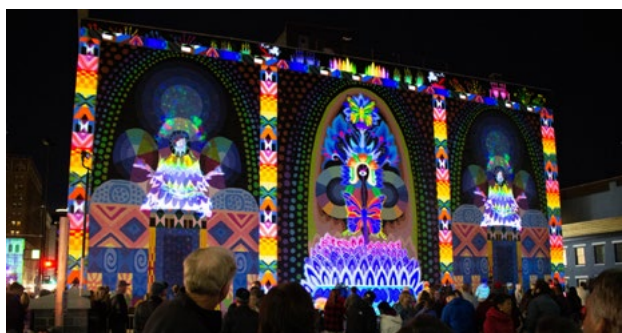
CNCDP-K12 Report
Bradley L. Schlaggar MD PhD
Kennedy Krieger Institute,
Baltimore, MD

**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,
Texas Children's Hospital,
Houston, TX

**Updates AAN Section of
Child Neurology with Q&A**
David E. Mandelbaum, MD, PhD
Alpert Medical School of
Brown University, Providence, RI

CINCINNATI NOTE OF INTEREST



BLINK – A Festival of Light & Art: October 13-16, 2022

Sometimes you get lucky. This is one of those times. Blink is a four-night annual outdoor art festival in Cincinnati that just happens to kick off on the 2nd night (Thursday) of the CNS Annual Meeting. The combination of innovative street art, projection mapping and light-based transformation of the city's classic architectural facades has become a nationally celebrated cultural magnet drawing ambulatory crowds and creating a vibe not unlike Austin, TX in 2013 on Halloween. (BTW: the city will fill up fast, so book your flights and sleeping rooms early).

Times & Rooms Subject to Change (check online Virtual Program)

WEDNESDAY | CONTINUED

3:30 PM – 5:30 PM

PECN: CME PROGRAM: EDUCATIONAL TOOLS (I/O)

COURSE DESCRIPTION

Since 2020 educational tools have evolved to include the best of both worlds including the virtual/digital platforms, exponential use of social media, and the sharing of resources across institutions. Many trainees across the country have taken advantage of participating in didactic lectures given virtually in numerous institutions. Webinars and podcasts have made their way into the day to day education of trainees and practicing child neurologists alike. Understanding and navigating the digital and social media milieu has never been more vital to our profession. Our first session aims to arm the participant with the knowledge to navigate these tools. In addition, new innovative ways of combining forces for education of our trainees as well as hybrid learning and interviewing is here to stay. With changes in harmonized milestones for resident education, ensuring trainees are receiving comprehensive exposure to the significance of equity and diversity is of vital importance. In addition, training directors have expressed the difficulty they experience in covering all the topics that are vital to residency education. In this course, the importance and impact of social media in various aspects of the career of a child neurologist, a review of a proposed curriculum in ethics for our trainees and the incorporation of topics around LGBTQ+ education will be presented.

LEARNING OBJECTIVES

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

1. Upon completion of this session, attendees will be able to identify the roles and benefits of the most commonly used social media platforms in child neurology.
2. Upon completion of this session, attendees will learn important features of a child neurology ethics curriculum and the ways to implement this in their current training programs.
3. Upon completion of this session, attendees will demonstrate knowledge of LGBTQ+ topics relating to resident education and how to implement these topics into their training curriculum.

IMPACT STATEMENT

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

1. Innovative use of social media platforms, development and implementation of an ethics curriculum specific to child neurology concerns as well as increasing their own knowledge of matters surrounding LGBTQ+ issues in residency education and patient care to result in improvements in their current practice.

PECN Digital Committee and Social Media Tools

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

Kathryn Idol Xixis, MD
University of Virginia, Charlottesville, VA

Jessica Goldstein, MD
University of Minnesota, M Health
Fairview Masonic Children's Hospital,
Minneapolis, MN

Development of a Child Neurology Ethics Curriculum

William D. Graf, MD
Connecticut Children's,
University of Connecticut,
Farmington, CT

LGBTQ: Tools for Residency Education

Jonathan Strober, MD
UCSF Benioff Children's Hospital,
San Francisco, CA

2:00 PM – 7:30 PM
EXHIBIT HALL (I)

6:00 PM – 7:30 PM
WELCOME RECEPTION (I)

Hosted by select Ohio training programs

8:00 PM – 10:00 PM
**MOVEMENT DISORDERS
VIDEO ROUNDS (I)**
(Formerly Movement Disorders SIG)

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

THURSDAY October 13

7:00 AM – 9:00 AM
PLATFORM I, II & III (I)

9:30 AM – 12:15 PM
SYMPOSIUM II:
PRESIDENTIAL SYMPOSIUM:
QUALITY AND CAPITATED CARE
(I/V/O)

COURSE DESCRIPTION

Just as healthcare has been dramatically changed by advances in molecular, genetic and systems neurosciences, there have been concurrent changes in methods of healthcare delivery and reimbursement that affect the practicing child neurologist.

LEARNING OBJECTIVES

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

1. Understand how quality improvement methodology can improve the clinical outcomes of patients and potentially result in increased reimbursement for clinical services.
2. Understand how the American Academy of Neurology develops and implements quality measures.
3. Know how learning healthcare systems can provide knowledge to improve patient outcomes that is not possible with single center efforts.

IMPACT STATEMENTS

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

1. Implement quality improvement projects based upon established pediatric quality measures.
2. Joining or creating pediatric learning healthcare systems to improve patient outcomes.
3. Assuring that care is provided in a manner that is equitable in order to eliminate existing disparities.

ORGANIZER

Bruce H. Cohen, MD, FAAN

CO-ORGANIZER

Jeffrey Buchhalter, MD, PhD
University of Calgary,
Cumming School of Medicine,
Calgary, Canada

Introduction and Discussion of the Importance of QI/QM to CNS Members

Bruce H. Cohen, MD, FAAN

Creating a Quality Improvement Ecosystem at AAN

Lyell K. Jones, Jr. MD
Mayo Clinic, Rochester, MN

Development of Child Neurology QMs at AAN

Bhooma Aravamuthan, MD, DPhil
Washington University
School of Medicine,
St. Louis, MO

Description of Rationale and Requirements for a Learning Health System (LHS)

Jeffrey Buchhalter, MD, PhD
University of Calgary,
Cumming School of Medicine,
Calgary, Canada

Descriptions of LHS in Pediatrics

Anup D. Patel, MD, FAAN, FAES

LHS for Peds/Adult Epilepsy:

Early Wins

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

Leveraging LHS to Study Health Care Disparities

Fiona Baumer, MD, MS
Stanford University School of Medicine,
Palo Alto, CA

Q&A

Bruce H. Cohen, MD, FAAN

11:30 AM – 7:00 PM
EXHIBIT HALL (I)

12:30 PM – 2:00 PM
**EXHIBITS, POSTER REVIEW &
GUIDED POSTER TOUR #1 (I)**

2:30 PM – 3:00 PM

MARTHA BRIDGE DENCKLA AWARD LECTURE (I/V/O)

Michael Shevell, MDCM, FRCP, FCAHS
Montreal Children's Hospital,
McGill University Montreal,
Quebec, Canada

Where the Next
50 Years Begins

Times & Rooms Subject to Change (check online Virtual Program)

THURSDAY | CONTINUED

3:00 PM – 5:15 PM

SYMPOSIUM III: GLOBAL NEUROLOGY: THE GLOBAL SITUATION OF CHILD NEUROLOGY PRACTICE DURING THE COVID 19 PANDEMIC AND OTHER NATURAL DISASTERS. CLINICAL CARE AND EDUCATION (I/V/O)

COURSE DESCRIPTION

This symposium will provide a global overview of the status of the child neurology practice during the COVID 19 pandemic, with emphasis in poor resource regions (low, middle, and high- income countries). The diverse panel of speakers have extensive experience in international neurology and practice in different regions, including Latin America, Africa, Caribbean, and USA. Therefore, they will present an expanded view of the current situation of pediatric neurology.

LEARNING OBJECTIVES

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

1. Describe the impact of the actual COVID 19 pandemic in the practice of Child Neurology, globally

2. Understand the use of technology in providing neurological care, specially to children and families with restricted access to care

IMPACT STATEMENTS

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

1. Use of virtual platforms to facilitate access to care
2. Delivering neurological care during the current pandemic, when access may be limited by use of available technology

ORGANIZER

Jorge Vidaurre, MD
Nationwide Children's Hospital,
The Ohio State University,
Columbus, OH

Chikungunya, Zika and COVID: Neurological Consequences and Impact in Child Neurology Care Across Latin America

Paulina C. Tejada, MD
Bernard D'Souza International Fellow
Pontificia Universidad Católica de
Chile, Santiago, Chile

Building Child Neurology Capacity in Africa During Disruptive Disasters: Ideas for Low Resourced Communities

Robert K. Sebunya, M.D, M.phil
Bernard D'Souza International Fellow
Uganda Martyrs University Nkozi,
Mother Kevin Post Graduate School,
Kampala, Uganda

Practicing Child Neurology on Conflict Zones. Lessons Learned.

Volodymyr Kharytonov, MD PhD
Clinical Hospital "Psychiatry",
Kyiv, Ukraine

The Potential for Device Technology Use in Healthcare: Applicability During Times of Reduced Access

Dave Clarke, MBBS
Dell Medical School,
University of Texas at Austin, Austin, TX

5:30 PM – 7:00 PM

EXHIBITS, POSTER REVIEW (WINE & CHEESE) & GUIDED POSTER TOUR #2 (I)

CINCINNATI NOTE OF INTEREST



National Underground Railroad Freedom Center

The National Underground Railroad Freedom Center is described as "a museum of conscience, an education center, a convener of dialogue, and a beacon of light for inclusive freedom around the globe." The museum is located in downtown Cincinnati, a short walk away from the CNS Meeting, on the banks of the Ohio River, the great natural barrier that separated the slave states of the South from the free states of the North.

FRIDAY

October 14

8:00 AM – 8:15 AM AWARD PRESENTATIONS & GENERAL SESSION (I/V/O)

*Child Neurology Foundation/
PERF Scientific Grant & Award
Announcements*

8:15 AM – 8:45 AM PHILIP R. DODGE YOUNG INVESTIGATOR AWARD LECTURE (I/V/O)

Bhooma Aravamuthan, MD, DPhil
Washington University School of
Medicine, St. Louis, MO

8:45 AM – 9:30 AM BERNARD SACHS AWARD LECTURE (I/V/O)

Steven Paul Miller, MDCM, MAS, FRCPC
University of British Columbia (BC),
BC Children's Hospital,
Vancouver, British Columbia, Canada

9:45 AM – 12:00 PM SYMPOSIUM IV: ETHICS: NEUROPALLIATIVE CARE ACROSS THE AGE SPECTRUM (I/V/O)

COURSE DESCRIPTION

Palliative care emphasizes a holistic interdisciplinary approach to the physical, psychological, social, and spiritual health and well-being of neonates, children, adolescents, and adults living with serious illness, and support for their families and caregivers. Although the lay public often equates palliative care to hospice care, the clinical domains of palliative care encompass more than end-of-life care. This symposium reviews clinical

palliative care practice guidelines and addresses important questions about “specialty” palliative care: How is neuropalliative care different than primary palliative care? How is pediatric neuropalliative care different than traditional care in child neurology? Can neuropalliative care begin at birth – or even before birth? Do we “palliate” symptoms when we offer patients promising new therapies? Do the goals of neuropalliative care vary depending on the diagnosis, stage, or severity of a neurological disorder? We discuss essential elements of neuropalliative care including diagnostic certainty, prognostic certainty (versus managing clinical uncertainty), family-centered communication, shared decision-making, and the management of pain and suffering in any care setting. This symposium will emphasize many special ethical issues in neuropalliative care such as those relating to disorders of consciousness, progressive loss of cognitive abilities or decisional capacity, and irreversible paralysis. The symposium stratifies neuropalliative care across the age spectrum. A neuro-oncologist will discuss methods of delivering bad news, assessing and explaining prognosis, assisting patients and families in the process of decision-making, and setting limits when certain types of care are objectively futile. Three child neurologists will respectively discuss pediatric, neonatal, and antenatal neuropalliative care issues such as severe neurological impairment in children and adolescents; withdrawal of life-sustaining interventions in neonates in the NICU; and care options for parents whose mid-trimester fetus has been diagnosed with a major malformation or life-limiting neurogenetic disorder. We highlight the 2022 “Clinical Guidance in Neuropalliative Care Position Statement” endorsed by the CNS.

LEARNING OBJECTIVES

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

1. Identify the various domains of palliative care and their key themes.
2. Integrate essential elements of communication, prognostication and shared decision-making into clinical practice along with special ethical considerations in neuropalliative care as it relates to disorders of consciousness.

IMPACT STATEMENTS

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

1. The domains of specialty palliative care.
2. Communicating prognostic certainty versus clinical uncertainty.

ORGANIZER

William D. Graf, MD
Connecticut Children's,
University of Connecticut,
Farmington, CT

Neuropalliative Care in Adults

Lynne P. Taylor, MD
Alvord Brain Tumor Center,
University of Washington, Seattle, WA

Antenatal Neuropalliative Care

William D. Graf, MD

Neuropalliative Care in Neonates

Monica Lemmon, MD
Duke University School of Medicine,
Durham, NC

Neuropalliative Care in Children with Severe Neurological Disorders and Neurodevelopmental Disabilities

Audrey Foster-Barber, MD, PhD
University of California,
San Francisco, San Francisco, CA

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

The one-time-only dose to stop SMA progression

ZOLGENSMA is a gene therapy for pediatric patients less than 2 years of age with spinal muscular atrophy (SMA), that is delivered as a single-dose, 1-hour intravenous infusion¹



Event-free survival

91% (20/22) of patients were alive and free of permanent ventilation at the 14-months-of-age study visit, **a primary endpoint**, and at 18 months of age^{2,a,c}



Motor milestones achieved

59% (13/22) of patients achieved the ability to sit without support for ≥30 seconds at the 18-month study visit, **a primary endpoint**^{2,a}
86% (19/22) of patients achieved one or more motor milestones by 18 months of age^{2,a}



Rapid onset

As early as 1 month post infusion, CHOP INTEND scores increased from baseline by a mean of 6.9 points (N=22)^{2,a}

The efficacy of ZOLGENSMA was evaluated in STRIVE, a completed, open-label, single-arm, multicenter, Phase 3 clinical trial of patients with SMA Type 1 (genetically confirmed bi-allelic *SMN1* deletion, 2 copies of *SMN2*, and <6 months of age at symptom onset and treatment; N=22).^{1,a,b}



Get started with ZOLGENSMA today:

Call 1-855-441-GENE (4363) or learn more at ZOLGENSMA-hcp.com

2300+
PATIENTS
TREATED AS OF
JUNE 2022^{3,d}

^aOne patient was initially classified as presymptomatic and removed from the intent-to-treat (ITT) data set included in the Prescribing Information. The patient was later confirmed to be symptomatic at baseline and included in the final ITT analysis.⁴

^bOne patient died at age 7.8 months due to respiratory failure, which was considered unrelated to treatment. One patient withdrew consent at 11.9 months of age; this patient required permanent ventilation at 11.0 months prior to withdrawal of consent. One patient discontinued participation at the age of 18.0 months, before the month 18 end-of-study visit, due to an adverse event of respiratory distress, which was considered unrelated to treatment.²

^cEvent is defined as death or the need for permanent ventilatory support consisting of ≥16 hours of respiratory assistance per day continuously for ≥14 days in the absence of an acute reversible illness, excluding perioperative ventilation.¹

^dGlobally including clinical trials, commercially, and through the managed access programs.³

Indication and Important Safety Information

Indication

ZOLGENSMA is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1 (SMN1)* gene.

Limitations of Use

The safety and effectiveness of repeat administration or the use in patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence) has not been evaluated with ZOLGENSMA.

Important Safety Information

BOXED WARNING: Acute Serious Liver Injury and Acute Liver Failure

Acute serious liver injury, acute liver failure, and elevated aminotransferases can occur with ZOLGENSMA. Patients with preexisting liver impairment may be at higher risk. Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing (e.g., hepatic aminotransferases [aspartate aminotransferase (AST) and alanine aminotransferase (ALT)], total bilirubin, and prothrombin time). Administer a systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion.

WARNINGS AND PRECAUTIONS

Thrombocytopenia

Transient decreases in platelet counts, some of which met the criteria for thrombocytopenia, were typically observed within the first two weeks after ZOLGENSMA infusion. Monitor platelet counts before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards.

Thrombotic Microangiopathy

Cases of thrombotic microangiopathy (TMA) were reported approximately 1 week after ZOLGENSMA infusion. Obtain baseline creatinine and complete blood count before ZOLGENSMA infusion. Following infusion, monitor for thrombocytopenia as well as other signs and symptoms of TMA. Consult a pediatric hematologist and/or pediatric nephrologist immediately to manage if clinically indicated.

Elevated Troponin-I

Increases in cardiac troponin-I levels were observed following ZOLGENSMA infusion. Monitor troponin-I before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards.

ADVERSE REACTIONS

The most commonly observed adverse reactions (incidence ≥5%) in clinical studies were elevated aminotransferases and vomiting.

Please see Brief Summary of Prescribing Information on the adjacent page.

References: 1. ZOLGENSMA [prescribing information]. Bannockburn, IL: Novartis Gene Therapies, Inc; 2022. 2. Day JW, Finkel RS, Chiriboga CA, et al. Onasemnogene abeparvovec gene therapy for symptomatic infantile-onset spinal muscular atrophy in patients with two copies of *SMN2* (STRIVE): an open-label, single-arm, multicentre, phase 3 trial. *Lancet Neurol*. 2021;20(4):284-293. 3. Data on file. Novartis Gene Therapies, Inc. 2022. 4. Data on file. AveXis, Inc. 2020.



ZOLGENSMA® (onasemnogene abeparvovec-xioi)
Suspension for intravenous infusion
Brief Summary of the Full Prescribing Information.
See Full Prescribing Information.

Rx Only

BOXED WARNING: ACUTE SERIOUS LIVER INJURY AND ACUTE LIVER FAILURE

- **Acute serious liver injury, acute liver failure, and elevated aminotransferases can occur with ZOLGENSMA.**
- **Patients with preexisting liver impairment may be at higher risk.**
- **Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing (e.g., hepatic aminotransferases [aspartate aminotransferase (AST) and alanine aminotransferase (ALT)], total bilirubin, and prothrombin time). Administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion.**

INDICATIONS AND USAGE

ZOLGENSMA is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1 (SMN1)* gene.

Limitation of Use: The safety and effectiveness of repeat administration of ZOLGENSMA or the use in patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence) has not been evaluated.

DOSAGE AND ADMINISTRATION

For single-dose intravenous infusion only.

The recommended dosage of ZOLGENSMA is 1.1×10^{14} vector genomes per kilogram (vg/kg) of body weight.

- Administer ZOLGENSMA as an intravenous infusion over 60 minutes.
- Postpone ZOLGENSMA in patients with concurrent infections until the infection has resolved. Clinical signs or symptoms of infection should not be evident at the time of ZOLGENSMA administration.
- Starting one day prior to ZOLGENSMA infusion, administer systemic corticosteroids equivalent to oral prednisolone at 1 mg/kg of body weight per day for a total of 30 days. At the end of the 30-day period of systemic corticosteroid treatment, check liver function by clinical examination and by laboratory testing. For patients with unremarkable findings, taper the corticosteroid dose gradually over the next 28 days. If liver function abnormalities persist, continue systemic corticosteroids (equivalent to oral prednisolone at 1 mg/kg/day) until findings become unremarkable, and then taper the corticosteroid dose gradually over the next 28 days or longer if needed. Do not stop systemic corticosteroids abruptly. If liver function abnormalities continue to persist $\geq 2 \times$ ULN after the 30-day period of systemic corticosteroids, consult a pediatric gastroenterologist or hepatologist.

WARNINGS AND PRECAUTIONS

Acute Serious Liver Injury, Acute Liver Failure or Elevated Aminotransferases

Acute serious liver injury, acute liver failure and elevated aminotransferases can occur with ZOLGENSMA. Hepatotoxicity (which may be immune-mediated), generally manifested as elevated ALT and/or AST levels and at times as acute serious liver injury or acute liver failure, has been reported with ZOLGENSMA use. In order to mitigate potential aminotransferase elevations, administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Immune-mediated hepatotoxicity may require adjustment of the corticosteroid treatment regimen, including longer duration, increased dose, or prolongation of the corticosteroid. Patients with preexisting liver impairment or acute hepatic viral infection may be at higher risk of acute serious liver injury/acute liver failure. Patients with ALT, AST, or total bilirubin levels (except due to neonatal jaundice) $> 2 \times$ ULN have not been studied in clinical trials with ZOLGENSMA. The risks and benefits of infusion with ZOLGENSMA in patients with preexisting liver impairment should be weighed carefully against the risks of not treating the patient. Although in the clinical trials and in postmarketing experience, asymptomatic aminotransferase elevations were very commonly reported, in the managed access program and in the postmarketing setting, cases of acute serious liver injury and acute liver failure have been reported. Some patients have experienced elevations in ALT and AST $> 20 \times$ ULN, prolonged prothrombin time and have been symptomatic (e.g., vomiting, jaundice), which resolved with the use of prednisolone, sometimes requiring prolonged duration and/or a higher dose. If acute serious liver injury or acute liver failure is suspected, consult a pediatric gastroenterologist or hepatologist. Prior to ZOLGENSMA infusion, assess liver function by clinical examination and laboratory testing (hepatic aminotransferases [AST and ALT], total bilirubin level, and prothrombin time). Continue to monitor liver function for at least 3 months after ZOLGENSMA infusion (weekly for the first month, and then every other week for the second and third months, until results are unremarkable).

Thrombocytopenia

Transient decreases in platelet counts, some of which met the criteria for thrombocytopenia, were typically observed within the first two weeks after ZOLGENSMA infusion. Monitor platelet counts before ZOLGENSMA infusion and on a regular basis afterwards (weekly for the first month; every other week for the second and third months until platelet counts return to baseline).

Thrombotic Microangiopathy

Cases of thrombotic microangiopathy (TMA) were reported approximately one week after ZOLGENSMA infusion in the post-marketing setting. TMA is characterized by thrombocytopenia, microangiopathic hemolytic anemia, and acute kidney injury. Concurrent immune system activation (e.g., infections, vaccinations) was identified in some cases. Monitor platelet counts, as well as signs and symptoms of TMA, such as hypertension,

increased bruising, seizures, or decreased urine output. In case these signs and symptoms occur in the presence of thrombocytopenia, further diagnostic evaluation for hemolytic anemia and renal dysfunction should be undertaken. If clinical signs, symptoms and/or laboratory findings consistent with TMA occur, consult a pediatric hematologist and/or pediatric nephrologist immediately to manage TMA as clinically indicated.

Elevated Troponin-I

Increases in cardiac troponin-I levels (up to 0.176 mcg/L) were observed following ZOLGENSMA infusion in clinical trials. The clinical importance of these findings is not known. However, cardiac toxicity was observed in animal studies. Monitor troponin-I before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards (weekly for the first month, and then monthly for the second and third months until troponin-I level returns to baseline). Consider consultation with a cardiologist, if troponin elevations are accompanied by clinical signs or symptoms.

ADVERSE REACTIONS

The safety data described in this section reflect exposure to ZOLGENSMA in four open-label studies conducted in the United States, including one completed clinical trial, two ongoing clinical trials, and one ongoing observational long-term follow-up study of the completed trial. A total of 44 patients with SMA received intravenous infusion of ZOLGENSMA, 41 patients at or above the recommended dose, and 3 patients at a lower dose. The patient population ranged in age from 0.3 months to 7.9 months at the time of infusion (weight range 3.0 kg to 8.4 kg). The most frequent adverse reactions (incidence $\geq 5\%$) observed in the 4 studies were elevated aminotransferases* 27.3% (12/44) and vomiting 6.8% (3/44).

*Elevated aminotransferases include elevation of alanine aminotransferase (ALT) and/or aspartate aminotransferase (AST). In the completed clinical trial, one patient (the first patient infused in that study) was enrolled prior to the protocol amendment instituting administration of prednisolone before and after ZOLGENSMA infusion.

One patient in an ongoing non-United States clinical trial initially presented with respiratory insufficiency 12 days after ZOLGENSMA infusion and was found to have respiratory syncytial virus (RSV) and parainfluenza in respiratory secretions. The patient had episodes of serious hypotension, followed by seizures, and was found to have leukoencephalopathy (brain white matter defects) approximately 30 days after ZOLGENSMA infusion. The patient died after withdrawal of life support 52 days after ZOLGENSMA infusion.

Immunogenicity

In ZOLGENSMA clinical trials, patients were required to have baseline anti-AAV9 antibody titers of $\leq 1:50$, measured using an enzyme-linked immunosorbent assay (ELISA). Evidence of prior exposure to AAV9 was uncommon. The safety and efficacy of ZOLGENSMA in patients with anti-AAV9 antibody titers above 1:50 have not been evaluated. Perform baseline testing for the presence of anti-AAV9 antibodies prior to ZOLGENSMA infusion. Retesting may be performed if anti-AAV9 antibody titers are reported as $> 1:50$.

Following ZOLGENSMA infusion, increases from baseline in anti-AAV9 antibody titers occurred in all patients. In the completed clinical trial, anti-AAV9 antibody titers reached at least 1:102,400 in every patient, and titers exceeded 1:819,200 in most patients. Re-administration of ZOLGENSMA in the presence of high anti-AAV9 antibody titer has not been evaluated.

DRUG INTERACTIONS

Where feasible, adjust a patient's vaccination schedule to accommodate concomitant corticosteroid administration prior to and following ZOLGENSMA infusion. Certain vaccines, such as MMR and varicella, are contraindicated for patients on a substantially immunosuppressive steroid dose (i.e., ≥ 2 weeks of daily receipt of 20 mg or 2 mg/kg body weight of prednisone or equivalent). Seasonal RSV prophylaxis is not precluded.

USE IN SPECIAL POPULATIONS

Pediatric Use

Administration of ZOLGENSMA to premature neonates before reaching full-term gestational age is not recommended, because concomitant treatment with corticosteroids may adversely affect neurological development. Delay ZOLGENSMA infusion until the corresponding full-term gestational age is reached. There is no information on whether breastfeeding should be restricted in mothers who may be seropositive for anti-AAV9 antibodies. The safety of ZOLGENSMA was studied in pediatric patients who received ZOLGENSMA infusion at age 0.3 to 7.9 months (weight range 3.0 kg to 8.4 kg). The efficacy of ZOLGENSMA was studied in pediatric patients who received ZOLGENSMA infusion at age 0.5 to 7.9 months (weight range 3.6 kg to 8.4 kg).

Hepatic Impairment

ZOLGENSMA therapy should be carefully considered in patients with liver impairment. Cases of acute serious liver injury and acute liver failure have been reported with ZOLGENSMA in patients with preexisting liver abnormalities. In clinical trials, elevation of aminotransferases was observed in patients following ZOLGENSMA infusion.

PATIENT COUNSELING INFORMATION

See the ZOLGENSMA Full Prescribing Information for the Patient Counseling Information.

Please visit ZOLGENSMA-HCP.com for Full Prescribing Information, including Boxed Warning.

Manufactured by, Packed by, Distributed by:
Novartis Gene Therapies, Inc.
2275 Half Day Road, Suite 200
Bannockburn, IL 60015 USA

U.S. License No. 2250

 **NOVARTIS**

© 2021 Novartis Gene Therapies, Inc.
Bannockburn, IL 60015

 **zolgensma®**
(onasemnogene
abeparvovec-xioi)
suspension for intravenous infusion

11/2021 US-ZOL-19-0202 V4

Times & Rooms Subject to Change (check online Virtual Program)

FRIDAY | CONTINUED

12:30 PM – 1:45 PM

SEMINAR 1:

CEREBRAL PALSY: WHAT IS CP? A CONSENSUS-BASED APPROACH (I)

COURSE DESCRIPTION

Cerebral palsy (CP) is the most common motor disability of childhood and is formally defined as “a group of permanent disorders of the development of movement and posture, causing activity limitation, that are attributed to non-progressive disturbances that occurred in the developing fetal or infant brain” (*Dev Med Child Neurol* 2007). Yet, we have shown ongoing variability in how we diagnose CP (*Pediatrics* 2021). We have demonstrated that neurologists and neurodevelopmentalists play an important role in CP diagnosis (*Neurology* 2020) and that the diagnostic views of people with CP differ from the views of these practitioners (*Dev Med Child Neurol* 2022). Parallel to this work, a recent CNS Open Forum Thread exemplifies the passion that our membership has in reaching a new consensus on a key question: “What is CP?”. Though previous symposia and seminar presentations at CNS have established that this question remains an open one and have provided didactic discussions on the topic, this year we propose addressing this question directly with involvement of the CNS membership – a gap highlighted by the Open Forum thread. The goal of our workshop is to use a group-think approach to determine the key uncertainties held by the CNS membership regarding the definition of CP. We have recruited renowned leaders in this field with academic, advocacy, and personal experience with CP. These leaders will facilitate breakout sessions to discuss uncertainties regarding:

1. The meaning of “non-progressive” – Michael Shevell
2. The meaning of “developing fetal or infant brain” – Ann Tilton
3. Contributions of different etiologies to CP – Michael Kruer
4. The meaning of a CP diagnosis for community members and other stakeholders – Paul Gross

We will summarize the breakout group discussions at the end of the workshop to reach a new comprehensive consensus on what the CNS membership’s addressable concerns are regarding the current definition of CP.

LEARNING OBJECTIVES

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

1. Outline the current consensus definition of CP
2. List the key uncertainties regarding interpretation of the current consensus definition of CP

IMPACT STATEMENTS

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

1. Conferring a CP diagnosis
2. Understanding the meaning of a CP diagnosis for the patients I treat and their caregivers

ORGANIZER

Bhooma Aravamuthan, MD, DPhil
Washington University School of
Medicine, St. Louis, MO

The Meaning of “Non-progressive”

Michael Shevell, MDCM, FRCP, FCAHS
Montreal Children’s Hospital,
McGill University Montreal,
Quebec, Canada

The Meaning of “Developing Fetal or Infant Brain”

Ann Tilton, MD
LSU Health Sciences Center New
Orleans, New Orleans, LA

Contributions of Different Etiologies to CP

Michael Kruer, MD
Phoenix Children’s Hospital,
Phoenix, AZ

The Meaning of a CP Diagnosis for Community Members and Other Stakeholders

Paul Gross, BA
President, CEO & Co-Founder;
Cerebral Palsy Research Network,
Greenville, SC

12:30 PM – 1:45 PM

SEMINAR 2:

NEURODEVELOPMENTAL DISORDERS: NEUROLOGICAL AND NEURODEVELOPMENTAL CHALLENGES IN SICKLE CELL DISEASE: STROKE AND BEYOND (I/O)

COURSE DESCRIPTION

Sickle cell disease is an inherited hematological disorder that affects millions of people globally. Despite sickle cell disease being one of the first genetic diseases discovered, many child neurologists and neurodevelopmental physicians are not aware of the heavy neurological burden of this disease and therapeutic options with neurological and developmental implications. While increased risk of stroke and stroke prevention recommendations are widely known, people living with sickle cell disease also have high rates of other neurological and neurodevelopmental complications, even in the absence of brain injury. We will describe the neurological complications, neuroimaging findings, and neurodevelopmental challenges in sickle cell disease across the lifespan. Our first

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

speaker will discuss current research and guidelines with regards to stroke risk and prevention in children and adults with sickle cell disease, nationally and globally, as well as other neurological complications seen in this disorder, including seizures and headaches. Our second speaker will discuss current neuroimaging research exploring cerebral physiology, metabolism, and fMRI and new and existing sickle cell disease therapeutics and their impact on the brain in sickle cell disease. Our third speaker will discuss rates and features of neurodevelopmental disorders in sickle cell disease and current neurodevelopmental screening guidelines. Due to therapeutic advances in the last 50 years, more people with sickle cell disease are surviving into adolescence and adulthood. It is essential that child neurologists and neurodevelopmental physicians are aware of the neurological, neurocognitive, and neurodevelopmental complications of this common inherited disease as appropriate medical, developmental, and cognitive screening practices are essential to improving care and reducing health disparities for these patients across the lifespan.

LEARNING OBJECTIVES

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

1. Identify current guidelines and practice regarding management of stroke, headache, and seizures in sickle cell disease.
2. Discuss current research regarding neuroimaging in sickle cell disease.
3. Identify current guidelines and practice regarding recommendations for neurocognitive and developmental screening in sickle cell disease.

IMPACT STATEMENTS

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

1. Care of children with sickle cell disease and stroke, seizures, and headaches.
2. Care of children with sickle cell disease and neurocognitive and/or developmental issues.

ORGANIZER

Eboni Lance, MD, PhD,
Kennedy Krieger Institute,
Baltimore, MD

Update on Neurological Complications of Sickle Cell Disease: Stroke Risk and Prevention, Headaches, and Seizures

Lori Jordan, MD, PhD
Vanderbilt University Medical Center,
Nashville, TN

Advanced Neuroimaging and New Therapeutics in Sickle Cell Disease

Melanie Fields, MD, MSCI
Washington University, St. Louis, MO

Neurodevelopmental Disorders and Developmental Screening in Sickle Cell Disease

Eboni Lance, MD, PhD

12:30 PM – 1:45 PM

SEMINAR 3:

NEURO-ONCOLOGY: A CASE-BASED APPROACH TO ACUTE NEURO-TOXICITIES IN CHILDHOOD CANCER PATIENTS (I/O)

COURSE DESCRIPTION

As new and effective treatments emerge, neurologists are increasingly called upon to recognize, evaluate, and treat acute and chronic neurologic toxicities of both traditional and newer therapies for childhood cancer and brain tumors. These therapies can include the newer targeted agents as

well as immunotherapies that are used in a broad range of pediatric cancers. Emerging treatments are also used for treatment of complications associated with the tumor predisposition syndromes such as neurofibromatosis type 1 and tuberous sclerosis complex (TSC), both of which are disorders commonly managed by child neurologists. In this symposium, we offer an overview of the main acute neurological toxicities of medical treatments for childhood cancers, including traditional chemotherapy, targeted chemotherapies, and immunotherapies. We will use a case-based approach to discuss acute neuro-toxicities of traditional chemotherapy agents such as methotrexate, targeted agents such as MEK and mTOR inhibitors, and immunotherapies such as check-point inhibitors and CAR-T cell therapies. As use of these newer agents increases, there are increasing data available regarding the breadth of these toxicities as well as up-to-date management recommendations.

LEARNING OBJECTIVES

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

1. Identify and treat acute toxicities of the treatments for nervous system tumors
2. Identify and treat the acute neurological toxicities of agents used to treat pediatric cancers.

IMPACT STATEMENTS

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

1. Targeted agents
2. The diagnosis and management of acute neurological toxicities of chemotherapy for childhood cancers.

ORGANIZER

Cynthia J. Campen, MD, MScE
Stanford University, Stanford, CA

Times & Rooms Subject to Change (check online Virtual Program)

FRIDAY | CONTINUED

MODERATOR

Sonia Partap, MD, MS
Stanford University & Lucile Packard
Children's Hospital, Palo Alto, CA

Traditional Chemotherapy Agents

Nicole Ullrich, MD, PhD, FAAN
Boston Children's Hospital, Boston, MA

Targeted Agents

Cynthia J. Campen, MD, MScE

Immunotherapy

Juliane Gust, MD PhD
Seattle Children's,
University of Washington, Seattle, WA

2:15 PM – 4:30 PM

SYMPOSIUM V: NEUROIMMUNOLOGY: ADVANCEMENTS IN PEDIATRIC NEUROIMMUNOLOGICAL DISEASES (I/V/O)

COURSE DESCRIPTION

The field of neuroimmunology is changing rapidly both in the clinic and in research including in pediatric neuroimmunological disorders. This symposium will provide the latest diagnostic, evaluation, management, and treatment of pediatric neuroinflammatory disorders. Pediatric multiple sclerosis (MS), neuromyelitis optica spectrum disorder, anti-MOG antibody associated disease (MOGAD), transverse myelitis, acute flaccid myelitis (AFM), and autoimmune encephalitis will be discussed. Treatment of MS including recent clinical trials in pediatric MS will be highlighted. MOGAD is a recently described entity causing different neuroinflammatory phenotypes and recent international guidelines on MOGAD will be presented. Acute flaccid myelitis (AFM) can mimic and be mimicked by other inflammatory disorders, so features to

distinguish AFM from other disorders will be described. Increased awareness of autoimmune encephalitis, such as anti-NMDA receptor autoimmune encephalitis (anti-NMDARE), is occurring with providers and in the community. Recent international consensus treatment guidelines for pediatric anti-NMDARE will be reviewed. Moreover, the presentations will address how neuroinflammatory disorders affect patients and their caregivers. The recent international consensus guidelines and research advancements for these diseases will be included to improve clinical implementation of these guidelines.

LEARNING OBJECTIVES

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

1. Describe characteristics and evaluation of different demyelinating and neuroinflammatory disorders in children
2. Discuss treatment and management of different neuroinflammatory diseases in children, including multiple sclerosis, neuromyelitis optica spectrum disorders, anti-MOG associated disorder, acute flaccid myelitis (AFM) and mimickers of AFM, and autoimmune encephalitis

IMPACT STATEMENTS

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

1. Evaluation of a patient with suspected neuroinflammatory disease, including which ancillary tests are useful and interpretation of test results
2. Management of pediatric inflammatory diseases, including inpatient and outpatient evidence-based treatments, based upon recent research studies

ORGANIZER

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

Multiple Sclerosis and Neuromyelitis Optica Spectrum Disorders

Tanuja Chitnis, MD
Mass General Brigham,
Harvard Medical School, Boston, MA

Myelin Oligodendrocyte Glycoprotein Associated Disorders

Giulia Fadda, MD
McGill University,
Montreal, Quebec, Canada

Acute Flaccid Myelitis and Mimickers

Teri Schreiner, MD MPH
Children's Hospital Colorado,
University of Colorado, Aurora, CO

Anti-NMDA Receptor Encephalitis and Other Autoimmune Encephalitis

Grace Gombolay, MD

4:30 PM – 5:00 PM

CNS BUSINESS MEETING (I)

5:45 PM – 6:30 PM

JUNIOR MEMBER SEMINARS (I)

6:15 PM – 7:00 PM

SCIENTIFIC PROGRAM & PLANNING COMMITTEE MEETING (I)

7:00 PM – 9:00 PM

CLOSING GALA (I)

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

SATURDAY October 15

7:00 AM – 8:15 AM

**SEMINAR 4:
EDUCATION:
STUDYING WHAT MATTERS:
INCORPORATING PATIENTS AND
FAMILIES INTO PEDIATRIC
NEUROLOGY RESEARCH (I/O)**

COURSE DESCRIPTION

Historically, patients and their families have primarily served as the subjects and beneficiaries of research in child neurology. It is increasingly clear that parents and patients should also play a key role in defining research priorities, study conception and design, data analysis and interpretation, and helping results reach a broad audience. Yet, questions remain about how to best include parents and patients in the research process. In this seminar, we will discuss how to practically involve parents in research. Our three speakers have first-hand experience in patient-centered research and dissemination. Betsy Pilon, Executive Director of Hope for HIE, will use her expertise to highlight the power of patient and caregiver advocacy groups in facilitating and disseminating research. Dr. Renee Shellhaas will share her experience working with diverse stakeholders, including her experience co-leading the PERF, PCORI, and NIH-funded studies of the Neonatal Seizure Registry. Dr. Adam Hartman, will share how to align proposals with funding priorities in patient-centered design. The session will conclude with a panel question and answer session, moderated by Dr. Monica Lemmon.

LEARNING OBJECTIVES

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

1. Outline key principles of patient-centered research
2. Identify ways to incorporate parents and advocacy groups into all aspects of research, including study design,

protocol implementation, analysis, and results dissemination.

IMPACT STATEMENTS

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

1. Identifying key principles of patient-engaged research design
2. Incorporating parents and advocacy groups into study design, protocol implementation, data analysis, and results dissemination

ORGANIZER

Monica Lemmon, MD
Duke University School of Medicine,
Durham, NC

The Power of Parents and Advocacy Groups

Betsy Pilon, Executive Director
Hope for HIE, West Bloomfield, MI

Incorporating Stakeholders into Study Design and Analysis: Lessons from the Neonatal Seizure Registry

Renée Shellhaas, MD, MS
Michigan Medicine, University of
Michigan, Ann Arbor, MI

Aligning Proposals with Funding Priorities in Patient-centered Design

Adam L. Hartman, MD
NINDS, NIH, Rockville, MD

7:00 AM – 8:15 AM

**SEMINAR 5:
FETAL NEUROLOGY: ADVANCES
IN FETAL NEUROLOGY: EMERGING
IDEAS AND FUTURE LANDSCAPE
(I/O)**

COURSE DESCRIPTION

Fetal neurology is a rapidly evolving field and continues to advance with more accurate prenatal diagnoses, and improvements in neuroimaging and genetic testing. Increasing number of fetal neurologic consultations across the US allows for earlier

characterization of critical/sensitive periods of developmental neuroplasticity by identifying fetuses and neonates at risk for adverse outcomes and neurodevelopmental disabilities. Increasing numbers of fetal neurological disorders such as congenital brain malformations (disorders of cortical migration, agenesis of corpus callosum, posterior fossa malformations), genetic conditions, prenatal brain injuries (stroke, hemorrhage), or congenital infections are diagnosed in this critical/sensitive period. Timelier and more effective neurotherapeutic interventions can potentially be developed that prevent or mitigate disorders, lowering the burden of neurologic disorders across life span.

The Fetal Neurology Consortium was founded in 2020 and has identified several challenges faced by the interdisciplinary team of clinicians who must recognize and overcome diagnostic limitations while offering accurate and compassionate prenatal counseling and management guidance into postnatal years. This symposium is being submitted as part of efforts of the consortium to disseminate knowledge on advances in fetal neurology. The sessions will include presentations in the areas of advances and future scope of fetal neuroimaging, neurogenetics and neurotherapeutics.

The course is designed for fetal-neonatal neurologists, child neurologists and trainees with special interest in the field of fetal and perinatal neurology, perinatology, and early origin of neurologic disorders.

LEARNING OBJECTIVES

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

1. Define advances in the field of fetal neurology focusing on neuroimaging and neurogenetics.
2. Identify emerging neurotherapeutics for prenatally diagnosed neurologic disorders.

Times & Rooms Subject to Change (check online Virtual Program)

SATURDAY | CONTINUED

IMPACT STATEMENTS

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

1. Diagnostic work up for fetal neurologic disorders.
2. Complexities in prenatal counselling and management of fetal neurologic disorders.

ORGANIZER

Sonika Agarwal, MBBS, MD
Children's Hospital of Philadelphia,
Perelman School of Medicine at
the University of Pennsylvania,
Philadelphia, PA

**Fetal Neurology Consortium
and Registry Workgroup – Fetal
Neurology Program Survey Results**
Sonika Agarwal, MBBS, MD

**Advances in Fetal Neurogenetics:
Emerging Ideas and Future
Landscape**

Lisa Emrick, MD
Baylor College of Medicine,
Houston, TX

**Advances in Fetal
Neuroimaging: Emerging
Ideas and Future Landscape**

Tomo Tarui, MD
Tufts Medical Center, Boston, MA

**Advances in Fetal
Neurotherapeutics and
Interventions**

David Neal Franz, MD
Cincinnati Children's Hospital/University
of Cincinnati College of Medicine,
Cincinnati, OH

7:00 AM – 8:15 AM

SEMINAR 6:

**DIVERSITY: DISABILITY IN
CHILD NEUROLOGY: SOCIETY,
MEDICINE AND THE PERSON (I/O)**

COURSE DESCRIPTION

Health care disparities are real and profound for people with disabilities. Just as racism plays an important role in perpetuating health care disparities, so does ableism. For example, a recent survey of physicians found that only 41% were very confident about their ability to provide the same quality of care to patients with disability (Lezzoni et al. 2021). As child neurologists, our intimate roles caring for disabled children make addressing ableism in our field an imperative. In this symposium, we will consider disability and ableism in the contexts of society, medicine and self/family. Appropriate language and frameworks for thinking about disability will be introduced. Participants will learn practical tools so that their child neurology practice can progress beyond disease management and include optimizing function and promoting inclusion in society as part of routine neurologic care.

Specifically, Dr. Kim will introduce our topic by defining ableism, providing examples, and introduce different models for framing disability. Dr. Christy will discuss ableism in medicine, including the historical evolution of the language we use to describe disability, and how ableism from physicians contributes to health disparities for disabled people (Lezzoni et al. 2021). Dr. Cejas will discuss personal/internal ableism: how disabled people holding these views about themselves impacts their health, and how physicians can help, as well as introducing tools that can help us think about an individual's function (International Classification of Funct...; Rosenbaum and Gorter 2012).

Finally, Dr. Barber will offer discussions of two illustrative cases to highlight these various perspectives on disability and moderate a discussion among the audience and all speakers.

LEARNING OBJECTIVES

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

1. Recognize that ableism is omnipresent and identify ableism in society, in medicine, and in personal/family dynamics.
2. Begin the process of changing the culture of child neurology to decrease ableism and improve neurologic care for children with disabilities.

IMPACT STATEMENTS

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

1. Using up-to-date, precise and respectful language when talking about disability with professional colleagues, patients, their families, and in society.
2. Moving beyond disease management for our patients to include optimizing function and promoting inclusion in society as part of routine neurologic care.

ORGANIZER

Danielle Guez Barber, MD PhD
Children's Hospital of Philadelphia,
Philadelphia, PA

**An Introduction to
Disability and Ableism**

Young-Min Kim, MD
Loma Linda University Children's
Hospital, Loma Linda, CA

**History of Ableism in
Child Neurology**

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

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

Ableism and the Individual

Diana M. Cejas, MD, MPH
University of North Carolina at Chapel Hill, Carolina Institute for Developmental Disabilities, Chapel Hill, NC

Panel Discussion, Q&A and Case Studies

MODERATOR

Danielle Guez Barber, MD PhD

- Diana M. Cejas
- Alison Christy, MD, PhD
- Young-Min Kim, MD

8:45 AM – 9:30 AM

HOWER AWARD LECTURE (I/V/O)

Leon G. Epstein, MD
Ann & Robert Lurie H. Children's Hospital of Chicago, Chicago, IL

9:45 AM – 12:00 PM

SYMPOSIUM VI: BEHAVIORAL NEUROLOGY: SPANNING THE DIVIDE: ANXIETY AND MOOD DISORDERS CO-OCCURRING WITH NEUROLOGIC DISORDERS (I/V/O)

COURSE DESCRIPTION

Anxiety and mood disorders commonly co-occur with neurologic disorders of childhood. These psychiatric symptoms arise from shared neural circuits, often interact with neurologic symptoms, and can negatively impact quality of life in our patients. In this session, we will review the increased prevalence of mental health disorders in neurologic conditions and the role of Child Neurologists in recognizing and managing symptoms. We will discuss the shared neural mechanisms of movement, motivation, emotions and behavior. In addition, we will use movement disorders (tic disorders) and epilepsy (Tuberous Sclerosis) as models for how neurologic symptoms

and psychiatric symptoms can coexist and impact each other. Finally, we will discuss the rising prevalence of anxiety and mood disorders in children and adolescents and the impact on child neurology patients. Given the high rates of co-occurring psychiatric conditions in neurologic disorders, it is important that we have the tools to recognize symptoms and understand how to approach management in our patients.

LEARNING OBJECTIVES

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

1. Understand the neurophysiology underlying the close relationship between neurologic disorders and psychiatric symptoms.
2. Understand the role of child neurologist in the care of patients with neurologic disorders and co-occurring psychiatric symptoms.

IMPACT STATEMENTS

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

1. Identification of anxiety and mood disorders in youth with neurologic disorders
2. Understanding the relationship between psychiatric and neurologic symptoms in youth

ORGANIZER

Jennifer Vermilion, MD
University of Rochester, Rochester, NY

Overlapping Neural Circuits in Movement Disorders and Mood Disorders: Implications for Diagnosis and Treatment

Jonathan W. Mink, MD, PhD
University of Rochester, Rochester, NY

Tourette Syndrome: Bridging the Border between Neurology and Psychiatry

Jennifer Vermilion, MD

Tuberous Sclerosis Complex Associated Neuropsychiatric Disorders: Insights and Opportunities

Tanjala T. Gipson, MD
University of Tennessee Health Sciences Center, Memphis, TN

Understanding and Addressing Psychiatric Comorbidities in Child Neurology

Devin C. McNulty, PhD
Ann & Robert H. Lurie Children's Hospital, Northwestern University Feinberg School of Medicine, Chicago, IL

12:15 PM – 4:15 PM

CNS CLINICAL RESEARCH ANNUAL WORKSHOP 2022 – PEDIATRIC NEUROLOGY CLINICAL TRIALS – TRIAL DESIGN (I)

COURSE 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:

1. Have an understanding of different types of clinical trials including the strengths and weaknesses of each study type as it applies to their specific area of research
2. Support their clinical research by identifying correct statistical analysis methods and study design specific sources of funding.

IMPACT STATEMENTS

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

1. Initiate new clinical research projects.
2. Meaningful engage in existing clinical research projects.

ORGANIZER

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

CO-ORGANIZERS

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

Welcome

Ariel Maia Lyons-Warren, MD, PhD

Introduction to Clinical Research Study Design

Jennifer Vermilion, MD
University of Rochester,
Rochester, NY

*Breakout Sessions***Finding the Right Grant for Your Clinical Research Study**

Adam L. Hartman, MD

Statistics by Study Design: Selecting the Right Type of Analysis for your Clinical Research Study

Paul S. Horn, PhD
Cincinnati Children's Hospital
Medical Center, Cincinnati, OH

*Coffee Break & Networking***How to Get Involved in Multi-Site Clinical Research Trials**

Darcy Krueger, MD PhD
Cincinnati Children's Hospital
Medical Center, Cincinnati, OH

Q&A**12:15 PM – 4:15 PM
BIOMEDICAL WRITING WORKSHOP
(I)**

COURSE DESCRIPTION

This interactive workshop for novice writers offers techniques to promote better manuscripts and enhance the likelihood of publication. Topics include avoiding writer's block, responding effectively to revision requests, and practical techniques to improve writing clarity. Numerous text examples illustrate practical ways to improve manuscript writing and organization skills, and the relaxed atmosphere promotes audience participation.

LEARNING OBJECTIVES

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

1. Recognize barriers to successful publication
2. Develop strategies for overcoming writer's block
3. Be able to more effectively revise manuscripts and respond to reviewers and editors
4. Understand the requirements for republication, use of patient materials and privacy concerns

IMPACT STATEMENTS

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

1. Publication of clinical and research articles that promote progress medicine by disseminating new ideas and information.
2. More efficiently planning and creating of manuscripts and interacting effectively with journal editors.

ORGANIZER AND PRESENTER

E. Steve Roach, MD
University of Texas Dell Medical School,
Austin, TX

Introduction:**Why Manuscripts are Rejected**

E. Steve Roach, MD

Outwitting Writer's Block

E. Steve Roach, MD

*Break***Revising Manuscripts & Responding to Reviews**

E. Steve Roach, MD

Rules of the Road: Permissions, Consents, and Other Potholes

Phillip L. Pearl, MD
Boston Children's Hospital,
Boston, MA

Meet the Editors

- Yasmin Khakoo, MD, FAAN
Memorial Sloan Kettering Cancer
Center, Weill Cornell Medical
College, New York, NY
- E. Steve Roach, MD
- Phillip L. Pearl, MD



It is now more important than ever
to find the cause of your patient's hypotonia.

COULD IT BE AADC DEFICIENCY?

Accurate identification of disease manifestation can help improve the care and management of patients with AADC deficiency.

➤ Visit **PTC Therapeutics** at booth **1818** to learn more about
Aromatic L-amino Acid Decarboxylase (AADC) deficiency.

Industry-Sponsored Satellite Sessions

Industry Sponsored Satellite Sessions are independently staged and accredited or non-accredited educational or product theater events. A gateway fee making them accessible to attendees is paid by the presenters.

WEDNESDAY October 12

PRODUCT THEATER 1: **Biogen: The Latest NURTURE Trial Data on Pediatric Patients**

Wednesday, October 12, 3:00 PM - 4:00 PM
Exhibit Hall A, Duke Energy Center

Speakers

Diana Castro, MD
Neurologist and Neuromuscular Physician
Founder of Neurology and Neuromuscular Care Center
Founder of Neurology Rare Disease Center
Denton, TX

Gyula Acsadi, MD, PhD
Pediatric Neurologist, Chief of Pediatric Neurology
Connecticut Children's Medical Center
Hartford, CT

THURSDAY October 13

PRODUCT THEATER 2: **A Treatment Option for Seizures Associated with CDKL5 Deficiency Disorder (CDD)**

Thursday, October 13, 7:00 AM - 8:00 AM
Room 206, Duke Energy Center

This presentation will review the data of a randomized, controlled trial that evaluated the efficacy and safety of an antiseizure treatment in patients with refractory epilepsy associated with CDKL5 deficiency disorder (CDD).

M. Scott Perry, MD
Head of Neurosciences
Director, Jane and John Justin Institute for Mind Health
Medical Director, Genetic Epilepsy Clinic
Cook Children's Medical Center

PRODUCT THEATER 3: **GENENTECH: EVRYSDI**

Thursday, October 13, 12:30 PM - 1:30 PM
Exhibit Hall A, Duke Energy Center

Join us at an interactive symposium about Evrysdi to explore community and expert perspectives

SATELLITE SEMINAR 1: **Practical Clinical Management of Lennox-Gastaut Syndrome**

Thursday, October 13, 2022
12:30 PM - 1:00 PM: On-site Check-in and Lunch
1:00 PM - 2:30 PM: Satellite CME Seminar
Rooms 200 & 205, 2nd Floor, Duke Energy Center

PRE-REGISTER AT www.millermeded.com/LGS
Pre-registration does not guarantee seating. On-site registration may be available, space permitting.

Program Overview

Lennox-Gastaut syndrome (LGS) is a debilitating developmental and epileptic encephalopathy (DEE) characterized by multiple seizure types, diffuse slow spike-and-wave complexes on encephalograms, and cognitive impairment. Most patients are treatment-refractory and have life-long disability. Drop seizures are hallmark features of LGS, most notably tonic seizures. However, most patients will develop between 3 and 5 seizure types which wax and wane during disease progression. Generalized tonic clonic seizures (GTCs) are commonly observed and, even though they can occur at any point during the syndrome manifestations, usually occur in later stages of LGS. In addition to being associated with bodily injury and hospitalizations, GTCs are a primary risk factor for SUDEP (sudden unexpected death in epilepsy). In addition, as in other DEEs, LGS patients are significantly affected by developmental delays and behavioral issues. In this seminar we will review LGS and its clinical diagnosis and management, with practical focus on rational therapy choices that optimize patient management and may affect long-term outcomes.

Learning Objectives

After completing this activity, the participant should be better able to:

- Review clinical features and diagnostic challenges associated with Lennox-Gastaut syndrome
- Discuss seizure end points in clinical trials of Lennox-Gastaut syndrome and how they translate into clinical practice
- Review secondary outcomes that are unique to Lennox-Gastaut syndrome management and reasonable expectations for patient care

Faculty

Katherine Nickels, MD (Program Chair)
Associate Professor of Neurology
Mayo Clinic
Rochester, Minnesota

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

Joseph E. Sullivan, MD
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

Physicians – maximum of 1.50 *AMA PRA Category 1 Credit(s)*[™]

Jointly provided by Postgraduate Institute for Medicine and Miller Medical Communications, LLC.

Grant Source: This live activity is supported by an independent educational grant from Zogenix, Inc.

SATELLITE SEMINAR 2: A Spotlight on the Management of AADC Deficiency: Experience With Investigational Intraputamenal Gene Replacement Therapy

Thursday, October 13, 2022
5:30 PM – 7:00 PM
Rooms 200 & 205, 2nd Floor, Duke Energy Center

PRE-REGISTER AT: <https://cvent.me/gnaMbn>

Speakers

Jennifer O'Malley, MD, PhD
Stanford University School of Medicine

Sudhakar Vadivelu, DO
Cincinnati Children's Hospital

Richard Poulin & Judy Wei
Parents of a child with AADC deficiency

Grant Source: PTC Therapeutics

PRODUCT THEATER 4:

Alexion: Pediatric Cases of Neurofibromatosis Type 1 (NF1) With Symptomatic, Inoperable Plexiform Neurofibromas (PN)

Thursday, October 13, 6:00 PM – 7:00 PM
Exhibit Hall A, Duke Energy Center

Program Description

Alexion invites you to a branded presentation featuring 3 cases of pediatric patients with Neurofibromatosis Type 1 with symptomatic, inoperable Plexiform Neurofibromas (NF1 PN). The presentation will discuss patient diagnosis, treatment, and care.

Speaker

Audrey Green-Murphy, DO, MSc
Co-Director NF Clinic
Valley Children's Hospital
Madera, CA

Pediatric Hematology-Oncology, Pediatric Neuro-Oncology

Dr Green-Murphy is a board-certified pediatric hematologist/oncologist and fellowship trained pediatric neuro-oncologist in Central CA. She is a member of Children's Oncology Group and currently serves as a Sub-PI for her institution. Her clinical interests include pediatric neuro-oncology, neurofibromatosis type 1, neurofibromatosis type 2/schwannomatosis syndrome, brain tumor survivorship, cancer predisposition syndromes, evidence-based treatment of children with underlying genetic syndromes, and increasing access to care for rural/underserved patients with cancer predisposition syndromes.

FRIDAY

October 14

SATELLITE SEMINAR 3: **The Latest Update on Metachromatic** **Leukodystrophy: Screening, Diagnosis, and Emerging** **Treatments to Improve Quality of Life of Patients**

Friday, October 14, 12:00 PM – 2:00 PM
Room: 200/205

Pre-registration link: www.cmeoutfitters.com/CNS2022

Speakers

Florian S. Eichler, MD – Moderator
Associate Professor of Neurology
Massachusetts General Hospital
Harvard Medical School
Boston, MA

Laura A. Adang, MD, PhD
Assistant Professor of Child Neurology
Children's Hospital of Philadelphia
Philadelphia, PA

Rachel E. Hickey, MS, LCGC
Genetic Counselor, Care Coordinator of Leukodystrophy
Clinic
Ann & Robert H. Lurie Children's Hospital of Chicago
Chicago, IL

Grant Source: Supported by an educational grant from
Takeda Pharmaceuticals U.S.A., Inc.

**Session links provided on virtual platform and
on CNS website 2022 Annual Meeting page**

SATELLITE SEMINAR 4: **Ending the Diagnostic Odyssey Genetic** **Diagnosis in Children Affected by Epilepsy**

Friday, October 14
5:00 PM registration
5:30 PM – 7:00 PM educational session
Room: 200/205

Pre-registration link: <https://na.eventscloud.com/website/44418/>

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

Speakers

Neil A. Hanchard, MD, PhD
Adjunct Associate Professor
Department of Molecular and Human Genetics
Baylor College of Medicine
Houston, Texas

Ingo Helbig, MD
Assistant Professor of Neurology
Perelman School of Medicine at the University of
Pennsylvania
Philadelphia, Pennsylvania

J. Michael Graglia, MBA, MA
PATIENT ADVOCATE
Co-Founder & Managing Director
SynGAP Research Fund
Palo Alto, California

Lacey Smith, MS, CGC
Genetic Counseling Program Manager
Epilepsy Genomics at Boston Children's Hospital
Boston, Massachusetts*With special guest and parent
advocate Mike Graglia

Grant Source: Supported by an independent
educational grant from GeneDx|Sema4



An invitation

Join us at an interactive symposium about Evrysdi to explore community and expert perspectives

Thursday, October 13
12:30 pm to 1:30 pm ET

Exhibit Hall,
Duke Energy Convention Center
Cincinnati, OH

Visit us at **Booth #2013**

To learn more
about Evrysdi, visit
[Evrysdi-hcp.com](https://evrysdi-hcp.com)

Genentech
A Member of the Roche Group

© 2022 Genentech USA, Inc.
All rights reserved. M-US-00017561(v1.0) 09/2022



ALERT

What other symptoms of **Rett syndrome** are asking for your attention?

Rett syndrome is a rare neurodevelopmental disorder that primarily affects girls¹

With no FDA-approved treatment for Rett syndrome (RTT), treatment is symptomatic and supportive.^{1,2} But there are additional challenges of daily life with RTT that can take a physical and emotional toll on caregivers^{3,4}—including symptoms like syndrome-related behaviors, breathing issues, mood disturbances, and nighttime behaviors.^{5,6}

Considering these and other manifestations of Rett syndrome may greatly impact care—giving voice to the unique challenges and unspoken needs of this disorder.



Together with caregivers, you can help identify more opportunities for collaborative and comprehensive care for individuals with Rett syndrome.

Uncover the full impact of Rett syndrome.



Visit us at
Booth #1724

References: 1. National Institute of Neurological Disorders and Stroke. Rett syndrome fact sheet. Accessed August 2, 2022. <https://www.ninds.nih.gov/rett-syndrome-fact-sheet> 2. Gold WA, Krishnaraj R, Ellaway C, et al. Rett syndrome: a genetic update and clinical review focusing on comorbidities. *ACS Chem Neurosci*. 2018;9(2):167-176. 3. Mori Y, Downs J, Wong K, et al. Longitudinal effects of caregiving on parental well-being: the example of Rett syndrome, a severe neurological disorder. *Eur Child Adolesc Psychiatry*. 2019;28(4):505-520. 4. Palacios-Ceña D, Famoso-Pérez P, Salom-Moreno J, et al. "Living an obstacle course": a qualitative study examining the experiences of caregivers of children with Rett syndrome. *Int J Environ Res Public Health*. 2019;16(41):1-13. 5. Fu C, Armstrong D, Marsh E, et al. Consensus guidelines on managing Rett syndrome across the lifespan. *BMJ Paediatr Open*. 2020;4(1):e000717. doi:10.1136/bmjpo-2020-000717 6. Killian JT, Lane JB, Lee H-S, et al. Caretaker quality of life in Rett syndrome: disorder features and psychological predictors. *Pediatr Neurol*. 2016;58:67-74.

The CNS Junior and Early Career Forum

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

The Junior and Early Career Forum was born out of a recent “International Collaboration of the Young Members” spanning the international child neurology organizations including ICNA, AOCNA, ACNA, EPNS, and the CNS. Representatives from each of the international/regional child neurology societies have been meeting for over a year to share ideas and discuss new programs that could benefit Junior Child Neurologists worldwide; the CNS is represented by Dr. Alexander Cohen and Dr. Ariel Lyons-Warren. Several ideas have been taken root that will, we hope, increase international collaboration and foster Global Neurology connections. 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.

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 Program;
4. the John M. “Jack” Pellock Resident Seminar on Epilepsy for CN and NDDDD residents in their final year of training; and
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 now expanding its junior member and early career programming this year with a new set of brief career-focused talks interwoven throughout the meeting, that will also be given by early/mid-career members with the theme of “things I knew last year”. These will be held in a dedicated “open” meeting space immediately below the main meeting room (Grand Ballroom A/B) to allow attendees to listen in between scientific sessions while they grab coffee, tea, and snacks. Topics range from 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. There will also be multiple opportunities to meet current and past award winners as well as editors of the top child neurology journals.

Finally, we will also be hosting a new 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; and
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”. We look forward to seeing everyone in Cincinnati!

Date	Time	Topic
Launching Your Career		
Wed	3:00 PM	What to look for in a job offer: young academic perspective
Wed	3:15 PM	What to look for in a job offer: young private practice perspective
(30 min break)		
Managing Your Career		
Wed	4:00 PM	When and how to ask for a raise in academia
Wed	4:15 PM	When and how to ask for a raise in private practice
(30 min break)		
Meet the Award Winners		
Wed	5:00 PM	Meet the award winner #1: Monica Lemmon
Wed	5:10 PM	Meet the award winner #2: Nina Schor
Wed	5:20 PM	Meet the award winner #3: Kenneth Mack
Making New Friends		
Thur	5:00 PM	How to network at a meeting
Thur	5:15 PM	Do I need a recruiter?
Meet the Editors		
Fri	12:00 PM	Meet the Editor #1: Yasmin Khahkoo
Fri	12:10 PM	Meet the Editor #2: Renee Shellhaas
Fri	12:20 PM	Meet the Editor #3: Marc Patterson
Finding Your Yoda		
Fri	1:45 PM	How to find mentors at your (existing/new) institution
Fri	2:00 PM	How to become a good mentor
Following the Money		
Fri	4:30 PM	Effort, Salary, and Fractions
Fri	4:45 PM	How to read a P&L statement

Duke Energy Convention Center Exhibit Hall A

EXHIBITS & POSTER REVIEW

EXHIBIT HALL

WEDNESDAY
2:00 PM-7:30 PM

6:00 PM - 7:30 PM

Welcome Reception

Supported by local Ohio hosts:

- Akron Children's Hospital
- Cincinnati Children's Hospital
- Nationwide Children's Hospital
- University Hospitals: Rainbow Babies & Children

THURSDAY

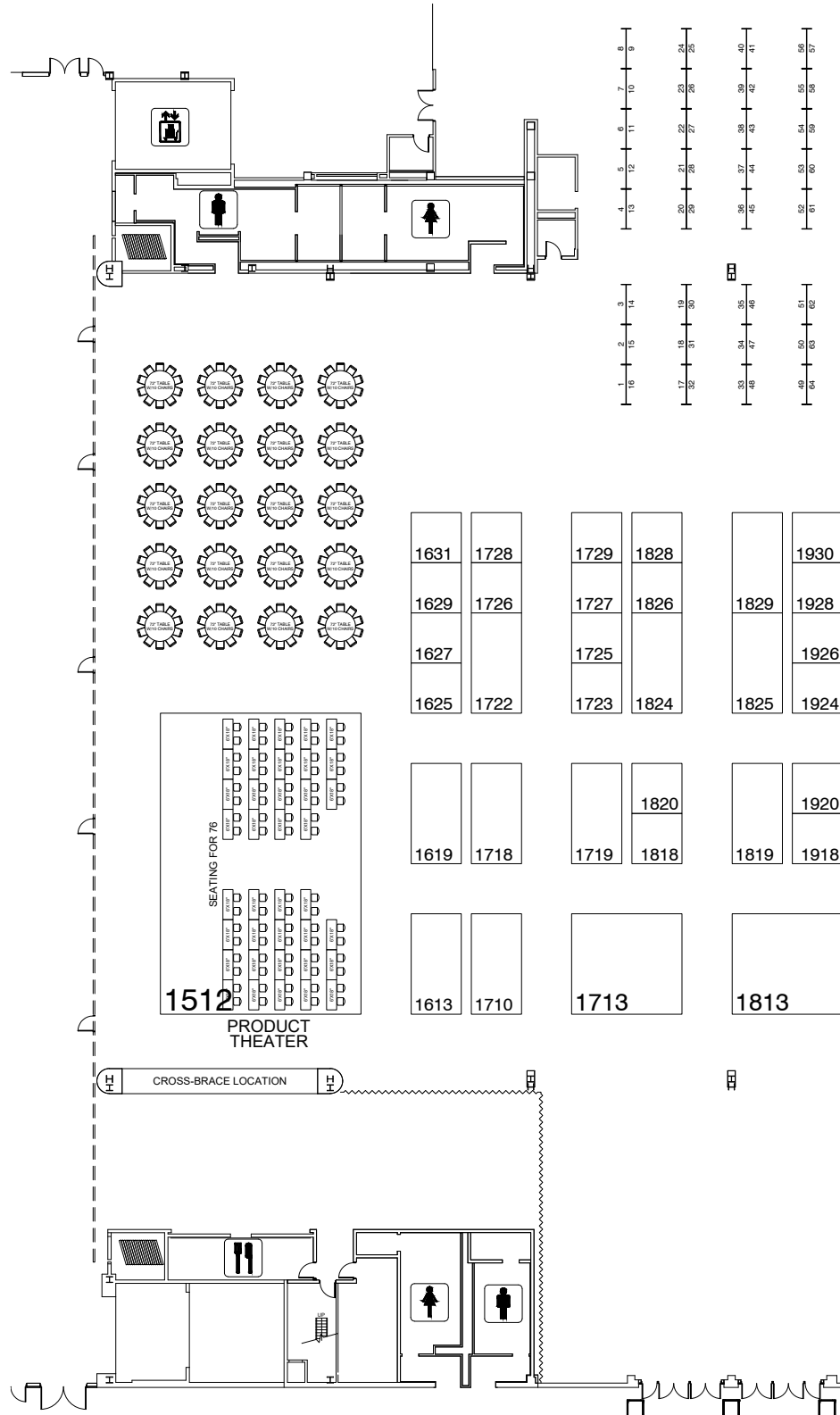
11:30 AM-7:00 PM

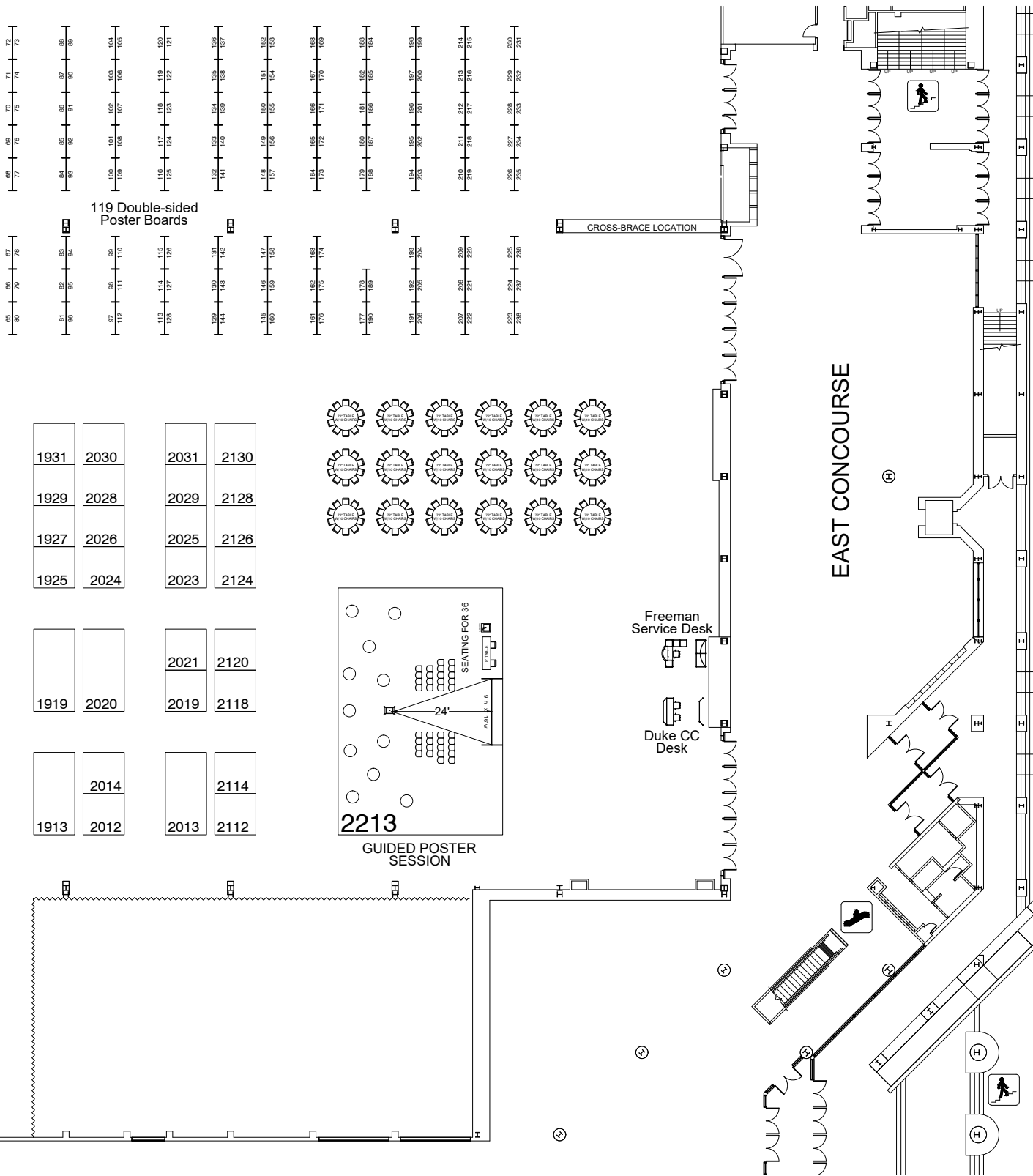
Lunch served
12:45 AM-2:00 PM
Poster Review

Wine & Cheese Reception

5:30 PM-7:00 PM

Poster Review





Exhibitors

AbbVie (#1728)

AbbVie's mission is to discover and deliver innovative medicines that solve serious health issues today and address the medical challenges of tomorrow. We strive to have a remarkable impact on people's lives across several key therapeutic areas. For more information about AbbVie, please visit us at www.abbvie.com. Follow abbvie on Twitter, Facebook, Instagram, YouTube and LinkedIn.

Acadia Pharmaceuticals, Inc. (#1722 & #1924)

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. For more information, visit us at www.acadia-pharm.com.

BRONZE SPONSOR

Alexion, AstraZeneca Rare Disease (#2029 & #2128)

Alexion, AstraZeneca Rare Disease focuses on serving patients and families affected by rare diseases through the discovery, development and commercialization of life-changing medicines. Our research efforts are in novel molecules and targets in the complement cascade and its development efforts on hematology, nephrology, neurology, metabolic disorders, cardiology and ophthalmology.



American Board of Psychiatry & Neurology, Inc. (ABPN) (#2014)

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.

Amneal Pharmaceuticals (#2019)

Amneal Pharmaceuticals is focused on the development, manufacturing and distribution of specialty and generic drug products. Amneal markets a portfolio of branded pharmaceutical products in the U.S., with a primary focus on central nervous system and endocrine disorders.

Association of Child Neurology Nurses (ACNN) (#1927)

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

Astellas Gene Therapies (#1729)

Astellas Gene Therapies is an Astellas Center of Excellence developing genetic medicines with the potential to deliver transformative value for patients. Based on an innovative scientific approach and industry leading internal manufacturing capability and expertise, we are currently exploring three gene therapy modalities: gene replacement, exon skipping gene therapy, and vectorized RNA knockdown.

Atlantic Health System (#2021)

Atlantic Health System is powered by a workforce of more than 18,000 members and 4,800 affiliated physicians dedicated to building healthier communities. Serving more than half of the state of New Jersey, Atlantic Health is comprised of more than 500 site of care all with a mission to design and deliver high quality innovative and personalized health care, to improve lives for patients, consumers and caregivers.

Biocodex, Inc. (#2118)

Biocodex, Inc. is the U.S. subsidiary of Biocodex, a family-owned multi-international pharmaceuticals company founded in France in 1953, with proven expertise in treatment for the central nervous system. As the maker of DIACOMIT, we are dedicated to providing education and support to our healthcare providers, affected individuals, and their families.

BRONZE SPONSOR

Biogen (#1913)

At Biogen, our mission is clear: we are pioneers in neuroscience. Biogen discovers, develops and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases as well as related therapeutic adjacencies.



BioMarin Pharmaceutical Inc. (#1718)

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.biopharm.com to learn more.

Bionano Genomics (#1920)

Bionano Genomics is a provider of genome analysis solutions that can enable researchers and clinicians to reveal answers to challenging questions in biology and medicine. Through Lineagen, we provide genetic testing and counseling services for patients with clinical presentations consistent with neurodevelopmental disorders. For more information, visit: www.lineagen.com.

Cadwell Industries, Inc. (#2028)

Cadwell is listening to our customers and responding to the ever-changing needs of brain monitoring. Arc EEG Software supports all three Arc systems: Apollo+, Essentia, and Zenith, to enable at-home ambulatory EEG, clinical EEG, in-hospital EEG, and high channel-count intracranial monitoring with direct cortical stimulation for epilepsy monitoring and neurosurgery.

Children's Hospital Los Angeles (#2030)

Children's Hospital Los Angeles is the No. 1 pediatric hospital in California, No. 1 on the West Coast and among top 10 in the nation ranked by *U.S. News & World Report*. Care is led by world-class physicians who are faculty members of the Keck School of Medicine of USC.

Children's National Hospital (#2124)

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.

LOCAL HOST

Cincinnati Children's (#2130)

With some of the nation's most respected physicians and researchers, Cincinnati Children's Division of Neurology is a leader in the latest research and treatment advances. The depth and breadth of our expertise, along with the novel therapies and clinical trials available, attract patients from across the country and around the world.



BRONZE SPONSOR

GeneDx – Sema4 (#1613)

GeneDx, LLC, is an industry leader in genomics and genetic testing. For over 20 years, GeneDx has been at the forefront of genetic innovation, pioneering new technologies and gene discovery, enabling greater diagnostic accuracy for patients and families. When experience matters, GeneDx is your answer.



BRONZE SPONSOR

Genentech (#2013)

Considered the founder of the industry, Genentech, now a member of the Roche Group, has been delivering on the promise of biotechnology for more than 40 years. Genentech is a biotechnology company dedicated to pursuing groundbreaking science to discover and develop medicines for people with serious and life-threatening diseases.



Epilepsy Alliance Ohio/Epilepsy Alliance America (#1926)

Since 1953, the Epilepsy Alliance Ohio has been dedicated to supporting those impacted by epilepsy in local communities by confronting the spectrum of challenges created by seizures. This is accomplished through direct services such as support groups, counseling services, camps, group homes, advocacy and much more. EAO is a proud founding member of Epilepsy Alliance America.

Fulgent Therapeutics, LLC (#1826)

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 oncology, anatomic pathology, infectious and rare diseases, and reproductive health.

Glut1 Deficiency Foundation (#1725)

The Glut1 Deficiency Foundation is a nonprofit patient advocacy organization dedicated to improving lives in the Glut1 Deficiency community through our mission of increased awareness, improved education, advocacy for patients and families, and support and funding for research.

Exhibitors

Invitae (#1619)

Invitae's mission is to bring comprehensive genetic information into mainstream medical practice to improve the quality of healthcare for billions of people. Our goal is to aggregate the world's genetic tests into a single service with higher quality, faster turnaround time and lower prices. Visit www.invitae.com.

SILVER SPONSOR

Jazz Pharmaceuticals (#1819)

Jazz Pharmaceuticals is a global biopharmaceutical company whose purpose is to innovate to transform the lives of patients and their families. Within our neuroscience portfolio, a priority focus area is exploring treatment options for rare and severe forms of epilepsy through our world-leading GW Cannabinoid Platform. Jazz is headquartered in Dublin, Ireland and has employees around the globe, serving patients in nearly 75 countries.



LivaNova (#1829)

As pioneers of the VNS Therapy™ system, we continue to advance medical device solutions for people affected by drug-resistant epilepsy. We strive to help where it counts, where it truly matters the most. Sharp, responsive and effective – at LivaNova we serve health and improve lives.

Mallinckrodt Pharmaceuticals (#1631)

Mallinckrodt is a global business consisting of multiple wholly owned subsidiaries that develop, manufacture, market and distribute specialty pharmaceutical products and therapies. To learn more about Mallinckrodt, visit www.mallinckrodt.com.

BRONZE SPONSOR

Marinus Pharmaceuticals, Inc. (#2012)

Marinus is a commercial stage pharmaceutical company dedicated to the development of innovative therapeutics for seizure disorders.



MedTech International Group (#1629)

MedTech International Group is a mission-driven company. For us, that means providing world-class LED illumination solutions that help doctors and healthcare professionals around the world. Based in Houston, Texas, MedTech International Group has emerged as a leader in LED illumination for over 5 years and expanded our products for a variety of medical, dental, and veterinary practitioners.

National Institute of Neurological Disorders and Stroke (NINDS) (#2023)

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

National Organization for Disorders of the Corpus Callosum (#2126)

The National Organization for Disorders of the Corpus Callosum (NODCC) is a 501(c)(3) nonprofit established in 2002 for individuals with disorders of the corpus callosum, their families and professionals. The NODCC has become the leading organization for disorders of the corpus callosum seeking to raise the profile, understanding and acceptance of these disorders through education, networking, advocacy, and being a catalyst for research.

LOCAL HOST

Nationwide Children's Hospital (#1710)

Nationwide Children's is ranked among the top 10 best children's hospitals for Neurology and Neurosurgery by US News. Unique areas of focus include stroke, intracranial hypertension, 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. (#1719 & #1723)

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 Inc. (#1727)

At Neurogene, our focus is to develop life-changing genetic medicines for patients and their families affected by rare, devastating neurological diseases. Our mission is to turn devastating neurological diseases into treatable conditions, to improve the lives of patients and families impacted by these rare diseases.

Neurotech, LLC (#2031)

Neurotech specializes in EEG services: in-home long-term aEEG, continuous hospital EEG monitoring, and research. Accredited by the Joint Commission, our in-home, long-term EEG monitoring services improve our patients' comfort and provide a cost-effective alternative to a hospital stay. Neurotech cEEG Partners provides hospitals with continuous EEG monitoring in the ICU and EMU to improve patient outcomes.

Nobelpharma America (#1627)

Nobelpharma America is focused on the commercialization of pharmaceuticals and medical devices that expand treatment options for people with rare diseases. In 2019, NPA became the first wholly owned global subsidiary of Nobelpharma Co., Ltd., which is based in Tokyo Japan.

Novartis Gene Therapies (#1713)

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. We are powered by an extensive manufacturing footprint, in capacity and expertise, enabling us to bring gene therapy to patients around the world at quality and scale.

NS Pharma (#1928)

NS Pharma is a highly focused, research-driven biopharmaceutical company working in rare diseases. Our current goal is to optimize the potential of exon-skipping therapy in treating Duchenne muscular dystrophy (DMD).

Parent Project Muscular Dystrophy (#2026)

Parent Project Muscular Dystrophy fights to end Duchenne. We accelerate research, raise our voices to impact policy, demand optimal care for every family, and strive to ensure access to approved therapies. Our Decode Duchenne program provides free genetic testing (diagnostic and carrier) for families living in the US or Canada.

Passage Bio (#2120)

Passage Bio is a clinical-stage genetic medicines company on a mission to provide life-transforming therapies for patients with CNS diseases with limited or no approved treatment options. As we work with speed and tenacity, we are always mindful of patients who may be able to benefit from our therapies.

Pediatrix Medical Group (#2024)

Pediatrix Medical Group is a provider of maternal-fetal, newborn and pediatric subspecialty services. Starting as a single neonatology practice in 1979, we have evolved into a multi-specialty medical group – capable of delivering a women's and children's continuum of care – meeting the needs of our patients and hospital partners.

Religen Inc. (#2114)

ReligenDX is focused on developing diagnostic solutions in Mitochondrial Medicine, Neurological Disease, and Genetics. We work with parents, patients, advocacy organizations, and healthcare providers to find solutions while developing and implementing new and innovative diagnostic testing, playing a pivotal role in the overall course of treatment.

RosmanSearch (#1931)

RosmanSearch is a highly specialized recruitment firm. Our mission is to place quality providers with quality practices, academic departments and hospitals nationwide. We are the only search firm in the country with dedicated teams specializing solely in neurosurgery, neurology and urology.

Sanford Health (#2025)

Sanford Health, the largest rural health system in the United States, is dedicated to transforming the health care experience and providing access to world-class health care in America's heartland. The integrated health system has 47 medical centers, 2,800 Sanford physicians and advanced practice providers, 170 clinical investigators and research scientists and world clinics in 8 countries around the globe.

Sarepta Therapeutics (#1825)

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 (#1930)

Sentynl Therapeutics is a U.S.-based biopharmaceutical company focused on bringing innovative therapies to patients living with rare diseases. With a focus on commercialization, Sentynl looks to source effective and highly differentiated products across a broad spectrum of therapeutic areas to address unmet needs. Sentynl is committed to the highest ethical standards and compliance with all applicable laws, regulations, and industry guidelines.

BRONZE SPONSOR

PTC Therapeutics, Inc. (#1818 and #1820)

PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. Our mission is to provide access to best-in-class treatments for patients who have an unmet medical need. To learn more, please visit www.ptcbio.com.



Exhibitors

The Sturge-Weber Foundation (#1929)

The SWF will be exhibiting the latest SWS research updates, annual events program calendar, educational materials and patient engagement events. Learn more about partnership opportunities to advance quality of life and care.

BRONZE SPONSOR

Takeda (#1919)

Takeda is a patient-focused, values-based, R&D-driven global biopharmaceutical company committed to bringing Better Health and a Brighter Future to people worldwide. Our passion and pursuit of potentially life-changing treatments for patients are deeply rooted in our distinguished history in Japan since 1781.



Texas Children's Hospital (#1925)

The Neuroscience Center at Texas Children's Hospital is comprised of pediatric neurologists, neurosurgeons, neuropsychologists and neurophysiologists. Our comprehensive team of experts provide care for patients from across the United States and around the world with a diverse array of neurological conditions ranging from the most common to the most complex.

Traverse Therapeutics (#1625)

Traverse Therapeutics is a biopharmaceutical company dedicated to identifying, developing and delivering life-changing therapies to people living with rare disease.

UCB, Inc. (#1813 and #1918)

UCB is a global biopharmaceutical company committed to discovering and developing innovative medicines that transform the lives of people living with severe diseases.

United Mitochondrial Disease Foundation (#1726)

Every 30 minutes, a child is born who will develop a mitochondrial disease by age 10, although the actual number of children born with the disease is thought to be much higher. The United Mitochondrial Disease Foundation powers the research, education, and support that is advancing treatments for patients and families affected by mitochondrial disorders. Visit umdf.org for more information.

Upsher-Smith Laboratories, LLC (#1824)

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.upsheer-smith.com.

UT Health Austin Pediatric Neurosciences at Dell Children's (#2112)

UT Health Austin Pediatric Neurosciences at Dell Children's, has assembled a team of highly specialized clinicians to treat children and adolescents with medical conditions of the central nervous system. Our inter institutional partnership with Dell Medical School at The University of Texas at Austin allows for multidisciplinary collaborations.

Variantyx, Inc. (#1828)

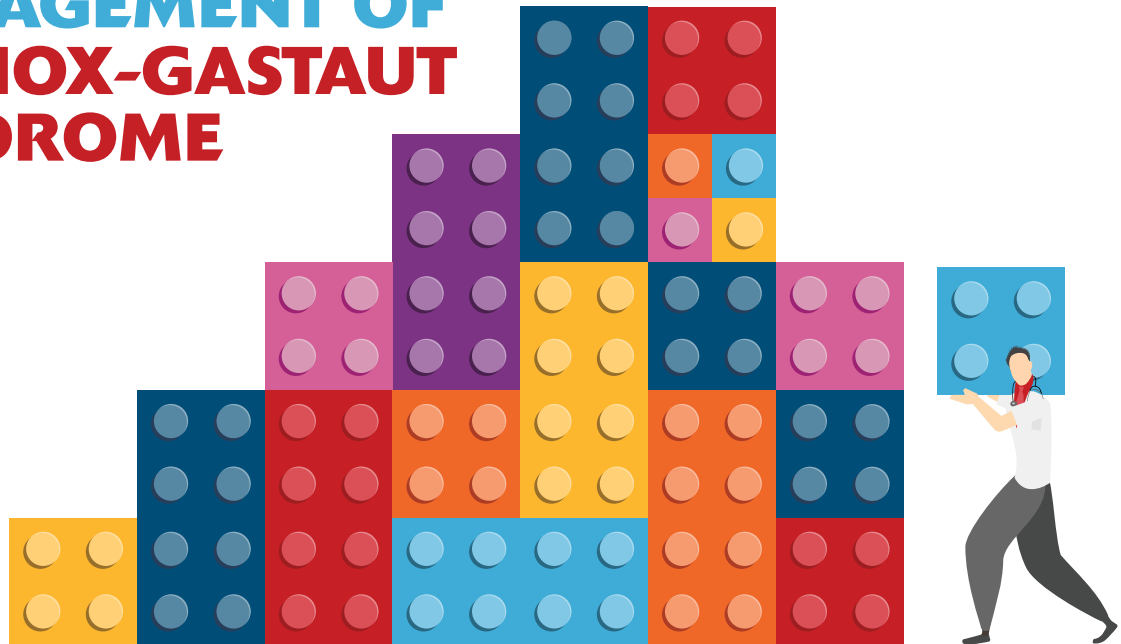
Variantyx is a precision medicine company using a whole genome analysis platform to provide state-of-the-art diagnostic solutions for rare genetic disorders. Our Genomic Unity® testing portfolio includes genome, exome, and phenotype-driven analyses. Learn more about our comprehensive testing for patients with neurological disorders at www.variantyx.com.

Xenon Pharmaceuticals (#2020)

Xenon Pharmaceuticals is a clinical stage biopharmaceutical company committed to developing innovative therapeutics to improve the lives of patients with neurological disorders. We are advancing a novel product pipeline of neurology therapies to address areas of unmet medical need, with a focus on epilepsy. For more information, please visit www.xenon-pharma.com.

JOIN US FOR A SATELLITE CME LUNCH SEMINAR

PRACTICAL CLINICAL MANAGEMENT OF LENNOX-GASTAUT SYNDROME



THURSDAY, OCTOBER 13, 2022

12:30 PM – 2:30 PM

Duke Energy Center
Rooms 200 & 205, 2nd Floor

PROGRAM CHAIR

Katherine Nickels, MD
Associate Professor of Neurology
Mayo Clinic
Rochester, Minnesota



PRE-REGISTER AT:

www.millermeded.com/LGS

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(s)*[™].

Jointly provided by Postgraduate Institute for Medicine and Miller Medical Communications, LLC.



Postgraduate Institute
for Medicine



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

This seminar was staged in conjunction with the 51st Annual Meeting of the Child Neurology Society. A staging fee was paid to the CNS to present this session. CNS is not responsible for content or CME credit, nor does it endorse the sponsoring organization or its products and/or services.

We look forward to seeing you...



*...at the Duke Energy Center in Cincinnati, Ohio and
to partnering with you at future CNS Annual Meetings.*



52nd CNS Annual Meeting

October 4 - October 7, 2023
Vancouver, BC, Canada



53rd CNS Annual Meeting

November 11-14, 2024
San Diego, California