



RESEARCH QUARTERLY

ISSUE 12: DECEMBER 2019

PARTNERSHIPS:

- NATIONAL ASSOCIATION OF EPILEPSY CENTERS
- THE EPILEPSY THERAPY PROJECT
- SUDEP INSTITUTE WITH CAMERON BOYCE FOUNDATION

INNOVATOR SPOTLIGHT

#MYCLINICALTRIAL

IN THE NEWS



WELCOME



If you want to go fast, go alone.
If you want to go far, go together.

-African Proverb

EVERY DECEMBER, the epilepsy community from clinicians, to scientists, to industry, to nonprofit advocacy organizations come together for the annual American Epilepsy Society meeting. It's a great opportunity for the Epilepsy Foundation to connect with our partners and plan for the year ahead. It's also a good time to celebrate the partnerships that the Foundation has grown with so many others in the past few years. The three specific areas of the Foundations research program (innovation, changing outcomes, and saving lives) are each stronger because of key partnerships with others in the community.

When I came to the Foundation, the Epilepsy Therapy Project (ETP) had recently merged with us. ETP's emphasis on partnerships radically changed the culture at the Foundation. Please go to page 6 to learn more about their philosophy and how that has shaped our innovation initiatives.

In our changing outcomes workstream, we continue to grow our clinical sites participating in the Epilepsy Learning Healthcare System, forging stronger connections between epilepsy center, our local Epilepsy Foundations and other organizations that can provide social services and community support to individuals

and families in our community. Please go to page 4 to hear the perspective of Dr. Nathan Fountain, president of the National Association of Epilepsy Centers, on this partnership. We are also honored to host our partners in the Rare Epilepsy Network (REN), a coalition of 30 plus rare epilepsies with the Foundation. Please go to page 2 to learn more of the new dashboard tools we have helped create to share data and results from the REN baseline survey to accelerate rare epilepsies research.

In our saving lives workstream, we are incredibly excited about our newly formed partnership between our SUDEP Institute and the Cameron Boyce Foundation to raise additional funds going towards SUDEP awareness and research (go to page 8 to learn more).

Partnerships are happening not just in the research realm, but across the board at the Foundation. For example, this past November we partnered with SK Life Science, Inc. for a first-of-its-kind Uber ride share campaign. We hope to continue such initiatives and partnerships moving forward.

I am honored and humbled to be working with such a dynamic, collaborative group of partners who are all focused on supporting those living with epilepsy.

Kind regards,

Brandy Fureman, Ph.D.
Vice President, Epilepsy Foundation

Targeting The Future

GENETICS AND EPILEPSY

Prior to genetic testing in the lab, epidemiologic studies and analysis of individual families shared the earliest evidence for genetic contributions to chronic conditions. In 1951, neurologist William Lennox studied more than 4,000 epilepsy cases, focusing on individuals living with epilepsy who did not have any evidence of structural brain damage. He examined the incidence of epilepsy in family members and differences in the concordance rates of epilepsy in identical versus fraternal twins. His seminal report concluded that although having a relative with epilepsy increase one's own risk of having epilepsy, it is not a straightforward relationship. He pioneered the idea that genetic influences on epilepsy were often multi-factorial and not always a direct one-to-one relationship.

In the rare epilepsy syndromes, most often **affecting children and representing the more severe forms** of epilepsy, the genetic culprit can be clearer than in the general epilepsy population.

Did you know? There is a **Beyond the Seizure** campaign supported by multiple companies (Invitae, BioMarin, Stoke Therapeutics, and Xenon Pharmaceuticals) to offer free genetic panel testing to kids under the age of 5.

Advances in genetic testing over the past two decades have led to a rapid increase in our understanding of the spectrum of epilepsies. The development of genomic technologies including chromosomal microarrays (which examine microscopic deletions or duplications of DNA) and next generation gene panels, whole exome and whole genome sequencing (which can do rapid sequencing of large amounts of DNA) have helped to grow understanding of the genetic underpinnings of epilepsies and allowed for acceleration of genetic discovery. Next generation sequencing has aided the identification of gene variants causing epilepsies, allowing for a higher diagnostic



Dr. Lennox and his team during a review session.
Credit: Boston Childrens Hospital

yield in people tested. Over the past two decades, we have identified hundreds of different genes linked with causing epilepsy.

Harnessing the knowledge that erupts from advances in science is key to shared progress. **Genetic registries** play an important role in enhancing the power of genetic discovery to benefit people impacted by epilepsy. A **registry** gathers and keeps information about people with a certain condition to support and encourage research into that condition. To expedite research into the rare epilepsies, over 30 rare epilepsy organizations have joined forces with the Epilepsy Foundation, Research Triangle Institute, and Columbia University to create the first ever Rare Epilepsy Network (REN). With seed funding from the Patient-Centered Outcomes Research Institute (PCORI), the REN patient registry collects information about people living with rare epilepsies to better understand these conditions, improve treatments, and improve the lives and quality of care of people living with them. Over 1450 individuals have contributed to the registry, sharing their or their loved ones medical history and experiences on a wide range of issues. We are actively working on ways that these natural history reports could be linked with genetic testing results.

In 2019, the PCORI funding ended and the Epilepsy Foundation committed to housing the registry in-house. We do not want this important data to be lost. Along with our REN partners, we created a public dashboard for the community to begin to learn from the information collected.

Please go to <https://efa.rexdb.net> to learn more. These insights and dashboards will continue to grow over time. The REN is a production of the over 30 partners working together, and we are extremely proud to be facilitating the network to spur new insights into this area. We cannot do this alone.

Changing Outcomes:

Partnership between the Epilepsy Foundation and the National Association of Epilepsy Centers (NAEC)

Dr. Nathan Fountain is the current president of the National Association of Epilepsy Centers (NAEC), a trade organization representing over 240 epilepsy centers in the United States. We asked Dr. Fountain to reflect on what the NAEC does and what excites him the most about partnering with the Epilepsy Foundation.

The National Association of Epilepsy Centers (NAEC) was founded by Dr. Robert Gumnit in 1987. The mission of the NAEC is to support epilepsy centers in delivering quality comprehensive care to people with epilepsy, by setting standards of care, advocating for access to high quality epilepsy center services, and providing knowledge and resources to its member centers. Through Dr. Gumnit's leadership, the NAEC led the conversation about what services should be required at an epilepsy center. In 2016, we began an accreditation process to ensure that epilepsy centers had the services needed for quality care.

Epilepsy care can be broken into four different levels. **First-level** care is provided by the primary care physician. **Second-level** care is provided by a general neurologist. Most people with epilepsy are adequately treated at these levels. However, if you are still living with persisting seizures or side effects after trying at least two medications, then you should be referred to a **third-** or **fourth-level** specialty epilepsy center for follow-up. Looking for an epilepsy center, near you go to <https://www.naec-epilepsy.org/>

The strategic priorities of the NAEC fall into three main areas:

Standard setting - ensuring that epilepsy centers are providing the highest quality care

Advocacy - minimizing the financial and regulatory barriers to providing quality care

Member center support – providing comprehensive resources and administration oversight to epilepsy centers

As a professional trade organization representing epilepsy centers, we want to ensure that our epilepsy centers are providing what our community wants. That is why we find partnerships with organizations like the Epilepsy Foundation invaluable. Both organizations want to ensure that those living with epilepsy are getting the best support they can get. Historically, NAEC has worked with the Epilepsy Foundation on initiatives to improve the quality of epilepsy care in the US.

In 2018, the Epilepsy Foundation was awarded a PCORNet Learning Health System Network Pilot Collaborative grant to establish an Epilepsy Learning Healthcare System (ELHS). ELHS is designed to gather data about people with epilepsy from clinics around the country and analyze it centrally to identify practices that lead to better outcomes. When they came to NAEC for support we jumped at the opportunity to participate. The NAEC vice president, Dr. Sue Herman, has been instrumental in building up the ELHS and serves as a clinical core lead for the network.

In the past year, nine pioneering epilepsy centers across the country have joined and are testing out the pilot data infrastructure. At each clinical site, there is a local Epilepsy Foundation organization as well as rare epilepsy organizations working with the hospital to ensure that there is a seamless integration of resources.

One of the ELHS' greatest assets is the partnership between clinicians and people with epilepsy so that the clinicians know what matters to people with epilepsy. The Foundation's commitment to leading this effort is to be commended. The ELHS is an amazing opportunity for us to transform healthcare for epilepsy and NAEC is thrilled to be a partner in the effort.



“Having this partnership with the Epilepsy Foundation is one of ELHS' greatest assets.”

– **DR. NATHAN FOUNTAIN, MD**
President of the National Association of Epilepsy Centers

Driving Innovation: The Epilepsy Therapy Project and its partnership with the Foundation

“Partnership had always been a core value, and we recognized that we needed greater scale and reach to have more significant impact in our mission to accelerate new therapies.”

- **WARREN LAMMERT**, Co-founder of Epilepsy Therapy Project

In 1998, Warren Lammert’s nine-month-old daughter, Sylvie, had her first seizure. As Sylvie went from one therapy to the next without finding an answer, Warren’s family joined the roller coaster ride of different therapies that is so common for those living with epilepsy. In 2002, Warren Lammert and Orrin Devinsky MD co-founded the Epilepsy Therapy Project (ETP). The goal of ETP is to foster innovation and support scientific advancements that could result in new treatments for people with epilepsy. Most importantly, the purpose is to get new therapies to people with epilepsy faster – in a timeframe that matters!

In 2007, ETP began partnering with the Epilepsy Foundation in providing commercialization grant support to academics and startups. Due to the success of that relationship, in 2012, ETP merged with the Epilepsy Foundation. That merger brought a renewed vigor and focus to the Epilepsy Foundation. Several members of ETP joined the board at the Epilepsy Foundation and pushed for us to support partnerships and to be pragmatic and solution oriented. We need to not only understand the science behind epilepsy but also the roadmap of how that science was going to benefit people in our lifetimes.

“Before Warren Lammert started the Epilepsy Therapy Project, there was little focus on bringing new and better therapies to people with epilepsy in the near term. He has acted as a major catalyst, not only providing funding (through new therapy grants) to early therapy development projects, but also connecting start-ups to resources, and facilitating important research connections. He is directly responsible for a number of new devices and therapies moving forward” – Dr. Jacqueline French, Chief Medical and Innovation Officer, Epilepsy Foundation.

The four strategic objectives of ETP are now incorporated into the Epilepsy Foundation research innovation track, and the objectives remain true to ETP’s intent. These strategic areas include:

1. **PROMOTING PARTNERSHIPS** among industry, academia, investors, people impacted by epilepsy and physicians. In 2017, we launched the Epilepsy Innovation Institute (Ei2) that supports higher-risk projects in the epilepsy space that bring together multiple disciplines together to work towards a common goal. Ei2 is currently supporting the My Seizure Gauge initiative, a public-private partnership that supports an international team working together to develop a minimally invasive seizure forecasting device. We are actively embedded with

the team, ensuring that the voice of our community is involved at all development stages. Imagine if you could forecast a seizure like you could forecast the weather? Learn more at www.epilepsy.com/myseizuregauge.

2. PROVIDING FINANCIAL SUPPORT to advance promising science that could lead to better therapies. We currently provide commercialization grants for later-stage development projects to help accelerate them on the marketplace. To celebrate the many companies that have received support through the Epilepsy Foundation partnership with ETP, watch the video: <https://bit.ly/32qtFGY>.

3. FOSTERING INNOVATION AND ENTREPRENEURSHIP so novel epilepsy projects and companies can make it to people with epilepsy. We host an annual Epilepsy Foundation Shark Tank Competition to showcase unique solutions on epilepsy products that could transform our community. Judges and the audience vote to provide funds to their favorite pitch. In 2018, we added a pitch perfect component to work with finalists before the competition. These pitches help the finalists develop their market strategy and strategic objectives to ensure a successful product. Learn more at www.epilepsy.com/sharktank.

4. PROVIDING VISIBILITY to therapies and devices in the epilepsy pipeline and increase attention to the real needs of people with epilepsy and the need for more research efforts. We host an Epilepsy Foundation Pipeline Conference that showcases therapies from preclinical to right before FDA approval. Our next conference event will be March 12-14, 2020 in Santa Clara California. Learn more at www.epilepsy.com/pipeline2020. In 2019, we revamped our pipeline tracker tool to allow people to search the catalogue of products currently in development. During our soft launch phase to test the tool, there have already been over 5,000 users searching the site. Please go to www.epilepsy.com/pipeline to learn more.

Warren's leadership has always been on building up collaborations, being solution-oriented and in focusing on the pragmatic. Partnerships are at the core of this philosophy - because we cannot do it alone. Thank you Warren for sharing your vision.



The original logo of the Epilepsy Therapy Project before the merger with the Epilepsy Foundation

Saving Lives: SUDEP Institute Partnering with The Cameron Boyce Foundation

In November, the Epilepsy Foundation and the Cameron Boyce Foundation launched a new initiative called “K(NO)W SUDEP NOW” to raise awareness about epilepsy and the risk of sudden unexpected death in epilepsy (SUDEP), particularly among youth and young adults. The new initiative currently features a public service announcement with Cameron Boyce’s parents and friends, as well as a new website - knowSUDEPnow.org to provide information on epilepsy and SUDEP as well as encourage donations.

“K(NO)W SUDEP NOW is about accelerating research and education to end SUDEP,” said Sally Schaeffer, senior director of the Epilepsy Foundation’s SUDEP Institute. “We felt this partnership was mutually beneficial given the work we are doing at the Epilepsy Foundation’s SUDEP Institute and the efforts The Cameron Boyce Foundation has embarked on to shine a light on SUDEP. The goal of this initiative is to educate people unaware of epilepsy, and empower those living with epilepsy to discuss SUDEP with their medical professional so they can reduce or mitigate their risk.”

Improving public awareness of epilepsy and SUDEP will drive more research dollars to help end SUDEP and END EPILEPSY®. K(NO)W SUDEP NOW provides a national platform to bring awareness to epilepsy and SUDEP and offers tools and resources for individuals and families to engage with their healthcare team about reducing their risk of SUDEP.

Following the passing of their son Cameron Boyce due to SUDEP, Victor and Libby expanded the focus of the foundation Cameron had established earlier this year to include epilepsy and SUDEP. “We would like to shine more of a light on epilepsy by getting more funding, more research and more people involved. And we hope that Cameron’s reach can do that,” said Victor and Libby Boyce.

Approximately 3.4 million people in the United States are affected by epilepsy. Epilepsy is the underlying tendency of the brain to produce seizures which are sudden abnormal bursts of electrical energy that disrupt brain functions. Over a lifetime, one in 10 people will have a seizure, and one in 26 will be diagnosed with epilepsy. Each year, about 1 in 1,000 people with epilepsy die from SUDEP.

For a limited time, anyone that donates \$100 or more will receive a t-shirt designed specifically for this awareness initiative. For more information, visit KnowSUDEPNow.org or epilepsy.com/SUDEP

“We would like to shine more of a light on epilepsy by getting more funding, more research and more people involved. And we hope that Cameron’s reach can do that.”

- **VICTOR AND LIBBY BOYCE,**
The Cameron Boyce Foundation

Innovator Spotlight: **JODY MCNANNAY**

Jody McNannay is the co-founder of Curadite, a medication management platform incorporating intelligent packaging, patient reminders, and a clinician dashboard for the epilepsy community. This past year, Curadite was awarded an Epilepsy Foundation Shark Tank prize to optimize the prototype presented at the competition. Just this past month, the company won the People's Choice award at the Oregon Bioscience Conference. We sat down with Jody to learn a little bit more about what inspired her to co-found Curadite and her own personal connection to epilepsy.



Q: How are you connected to epilepsy?

A: My daughter, Arianna, was diagnosed with absence seizures when she was seven. At the time, we didn't know that this was epilepsy. The doctors told us she had a seizure disorder, apparently not wanting to use the E-word with us. We were assured that this was a phase and that she would most likely outgrow it. At the age of 18, however, the seizures progressed, and we witnessed her first tonic-clonic seizure. We were not ready for it.

Q: What do you wish your doctors had told you?

A: The doctors were giving us the Pollyanna version – emphasizing that 70 percent of kids with absence seizures grow out of it by the age of 18. I wish that the doctors had also told us about the 30 percent of kids who don't. I wish that they had told us that the seizures might get worse and that there are things that we could do to mitigate those risks. I wish that they had stressed more the seriousness of medication adherence.

Q: What do you mean?

A: Arianna was lucky growing up. Unlike others in the community, the medications were keeping her seizures under control. The medications were annoying in the sense that she would get 300 pills at once that we would have to sort. She had to take 10 pills a day (5 in the morning and 5 at night). On top of that, she had other medications for another condition. When she turned 15, she wanted more independence. I'd been trying to balance the nagging that moms have to do with giving her the opportunity to take responsibility, so I let her take

Learn about some of the resources that the Foundation provides to those starting out on the epilepsy journey at www.epilepsy.com/toolbox

over her medication regimen. It turned out that she was missing some of the doses. She wasn't missing them on purpose – but taking that many pills per day at specific times of day every single day without mistakes happens. I wish I'd known that those delayed doses would end up triggering more severe seizures.

Q: What did you decide to do?

A: We searched the market for a solution to help Arianna track her medications, but there wasn't anything out there. The smartphone apps weren't smart enough to know whether or not medication had been taken, and the plastic pillboxes that beep and flash to remind you to take medication only work if you're nearby. We realized there was no commercially available solution. My husband and I knew there had to be a way for us to track the medications without Arianna sacrificing her independence. Building on our experience working in the bioscience world, we realized it was time to leave our jobs and co-found Curadite.

Q: What is Curadite?

A: Curadite is a startup devoted to making medication adherence simple. We wanted to create a platform that would use intelligent packaging to timestamp when medication is taken. We can work with a pharmacy distributor package medication or the family/doctor can package the doses themselves. Reminders are only sent via phone if someone doesn't take the dose, and patients can opt to let loved ones know when they've taken their medication. All the timestamps are stored in the cloud, and the data is available to share with a doctor, a partner, a parent – the user decides. My daughter is currently beta-testing the product, and I haven't driven her too crazy which is a good endorsement.

Q: How have you interacted with the Epilepsy Foundation?

A: From a company standpoint, the Epilepsy Foundation helped us in multiple ways—from forcing us to further develop an epilepsy centric perspective on our business strategy and connecting us to other thought leaders in the community to supporting us financially when we won the Epilepsy Foundation Shark Tank Competition in 2019. On a more personal note, the Foundation made us feel less alone. People don't talk about epilepsy; after spending years facing the challenges of epilepsy on our own, getting introduced to the local Epilepsy Foundation made us realize that there was a passionate network of people who are working together to improve the lives of those with epilepsy. It's because of the people and the efforts of the Foundation that I joined the board for the Oregon and SW Washington chapter.

Q: Is there anything else that you would like to add?

A: I have a lot of optimism. Twenty years ago, the skepticism of immunotherapeutics was pervasive; today, it's hard to have a conversation around cancer that doesn't involve them. Ten years ago, no one would have believed that CBD would have been approved by the FDA and now we have Epidiolex. Six months ago, I witnessed the Epilepsy Foundation Shark Tank, which showcased unique ideas from new devices for seizure detection to virtual reality trainings to improved clinical decision tools. There is so much innovation happening and new opportunities for both people living with epilepsy and those cheering on the sidelines. My whole team is honored to be in a position to support Arianna and the much broader epilepsy community.



#MyClinicalTrial: Brittany's Story

FINDING A PERSONAL PASSION IN CLINICAL TRIALS

Meet Brittany, manager of patient recruitment at Engage Therapeutics, Inc., and a woman living with epilepsy. She found complete seizure control after participating in a clinical trial and is a passionate advocate for clinical trials. Brittany spoke with Liz Dueweke, manager of communications at the Epilepsy Foundation, about her journey.

Tell me about your journey with epilepsy. When were you diagnosed?

I was diagnosed when I was 9. A few weeks before I had my first seizure, I wasn't feeling well. My mom was a police officer and I was in the backseat of her car when I passed out and had my first seizure. I found out later that I was having auras (first symptom of a seizure) well before that first seizure.

What was that experience like?

It was really confusing. When they did my EEG (electroencephalography) and MRI, they couldn't find a clear reason for the seizures. My doctors would accuse me of faking it for attention. As a child I did not understand, and it upset my mother. It was hard to find a neurologist. I grew up in northern Florida and we didn't have health insurance at the time, so I didn't have access to great care. For the first ten years after my diagnosis, I was seeing a primary care physician.

Were your seizures controlled?

It wasn't until I advocated for myself and joined a clinical trial that my seizures were controlled. My seizures were stress related, so if there was an exam, or even a birthday, they were triggered. I also tracked my menstrual cycle and found they were related to that as well.

What brought you to a clinical trial?

I decided to pursue a master's degree in public health and got accepted to Boston University. I found out about the research being done at Massachusetts General Hospital. I decided to reach out and obtain a job as a graduate research assistant in neurology. My principal investigator in my lab then referred me to a doctor at the Brigham and Woman's Hospital in Boston.

I had a seizure while at the Epilepsy Monitoring Unit at Brigham and Woman's. I was there to be reviewed as a possible brain surgery candidate. They found some sclerosis on my hippocampus. However, there was no definite plan that brain surgery would stop my seizures. A physician at the EMU had told me I would be an ideal candidate for a clinical trial for Onfi, so I decided to pursue that option.

USE THE HASHTAG **#MYCLINICALTRIAL** TO SHARE YOUR STORY.

What was the experience of a clinical trial like?

As a person who didn't know much at the time, I remember getting the informed consent and not reading it. I just initialed at the bottom of each page. As a research coordinator, I do not suggest people do this. I encourage possible research patients to read every word of the informed consent and ask questions to your research team.

During the clinical trial, I was reimbursed for my time, received free medical scans and lab work, along with physician check-ups which were all covered by the sponsor. The clinical trial was testing how sleepy Onfi makes you. I had to wear a sleep watch and attend visits with the clinical research team. I am still very tired from taking Onfi, I need 8-10 hours of sleep a night, but being tired is better than having seizures. Overall it was a really great experience, but it's important to understand what is going on.

And you were able find seizure control?

Yes. I haven't had a breakthrough seizure in over 2 years. This all led me to focus on clinical research. I now work with Engage Therapeutics, Inc. on their clinical trial efforts. I work with study coordinators and principal investigators to help them with patient recruitment.

Do you think others should try clinical trials? If so, why?

If you're uncontrolled and tried multiple treatments, and your physician thinks it is a good idea, I think clinical trials are very useful. You can get some very good health data from the tests they perform that you can apply to your own care.

It also advances the science for people with epilepsy. There are people who have seizures that are not controlled by current treatments. Even if it doesn't work for you, they can get the data on why it may not work for you to help someone else.

What do you wish other people with epilepsy knew about clinical trials?

I wish they knew they weren't lab rats. Scientists are not trying a treatment just for the sake of trying it, as it is very difficult to get a clinical trial up and running. They are working to get data to make it the best kind of medication or therapy on the market.

Also, if you are on the placebo for the trial, your doctor will be sure to manage your standard of care. You can also withdraw at any time in case you don't end up liking the experience. The entire effort is meant to help the patient.

I believe that clinical research is very important in bringing new medication and treatments to the public, because without individuals joining trials, we would never get new treatments on the market.

SHARE YOUR STORY

We want to hear your clinical trial story. Tag us on Facebook, Twitter, or Instagram and use the hashtag #MyClinicalTrial to share yours.



Think Big with Dr. French - Research Roundtable in Epilepsy

Dr. Jacqueline French is the Chief Innovation and Medical Officer at the Epilepsy Foundation

For this partnership issue, I wanted to focus on the Research Roundtable for Epilepsy (RRE), which the Epilepsy Foundation hosts annually. The RRE was created to allow a diverse group of interested parties to come together to discuss thorny issues and roadblocks related to bringing new therapies (drugs and devices) to the clinic. Attendees include companies developing new therapies, clinical researchers who are running trials, researchers, government representatives from the National Institutes of Health (NIH) and the Food and Drug Association (FDA), and most importantly, people with epilepsy who will eventually use the therapies. This year, the Epilepsy Foundation will convene the roundtable on May 14-15, 2020, in Washington, D.C.

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The research roundtable in epilepsy is an opportunity for people who have different types of expertise, and different viewpoints to all focus on one issue.
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Planning for the meeting is quite intense. The roundtable members select the topic a year in advance and then meet weekly to shape the agenda and consider speakers. We believe in representing the different voices in the epilepsy community and actively solicit input from other patient and physician groups such as the American Epilepsy Society, Citizens United for Research in Epilepsy (CURE), and the Epilepsy Leadership Council (ELC). Once the roundtable has concluded, the speakers and attendees collaborate on a manuscript that is submitted to a high impact journal, so that the conclusions can be widely disseminated.

We began the roundtable in 2016 and have focused on the far-reaching issues relating to drugs and device development incorporating new trial designs. In our first RRE, we discussed reducing placebo exposure. Most trials are done as controlled trials where there are two groups: one is the therapy being studied, the other is a placebo or “sugar pill” added to whatever treatment the trial participant is already on before the study. These trials typically last 3 months or more, but there are new trial designs that could allow a participant to exit early if the intervention is ineffective. These discussions were published and already there are studies incorporating these new designs into their trials. In 2018, we discussed trial designs that could be used for trials in the rare epilepsies, where only a handful of trial subjects might be available. In 2019, we focused on making clinical trials more efficient, and by doing so, streamlining the process of getting a drug to the clinic. In the upcoming session, we will discuss different ways epilepsy patients could be grouped for study in trials. One option is to group individuals by the underlying cause of the epilepsy such as a specific genetic mutation to study in a trial. Another option is to group people by epilepsy syndrome, such as Lennox-Gastaut Syndrome or Juvenile Myoclonic Epilepsy, irrespective of the cause. A third option is to group people by the seizure types that they have such as tonic seizures or absence seizures. Each way could be appropriate, depending on what is known about the mechanism by which the drug or device has its action. The goal of this meeting is to begin developing a framework around when to use what option.

The research roundtable in epilepsy is an opportunity for people who have different types of expertise, and different viewpoints to all focus on one issue. We cannot do this alone. By bringing all stakeholders together, we have a group that can effectively advance several important agendas, clearing the path to successful therapeutic development.

Clinical Trial Portal List

Clinical trials are the way new treatments are tested for safety and effectiveness before being approved and made available to people with epilepsy.

Go to [EPILEPSY.COM/clinical_trials](https://www.epilepsy.com/clinical_trials) to learn more.

Rare Epilepsy Syndrome Trials

CANNABIDIOL AS AN ADD-ON THERAPY IN TUBEROUS SCLEROSIS COMPLEX

This study is looking at how effective and safe cannabidiol (CBD) is in people 1 year to 65 years old living with Tuberous Sclerosis Complex (TSC). CBD is given in addition to their current anti-seizure medications.

PREVENTING EPILEPSY USING VIGABATRIN IN INFANTS WITH TUBEROUS SCLEROSIS COMPLEX

This Phase IIb trial will test whether earlier treatment versus standard treatment with vigabatrin in infants with tuberous sclerosis complex (TSC) will have a positive impact on developmental outcomes at 24 months of age. It also tests whether early treatment prevents or lowers the risk of developing infantile spasms and refractory seizures. It is a randomized, double-blind, placebo-controlled clinical trial design. Infants under the age of 6 months diagnosed with TSC but without history of seizures or infantile spasms may be eligible.

FOCUSED ULTRASOUND FOR TREATMENT OF EPILEPSY

The University of Virginia Comprehensive Epilepsy Program is conducting a research study on the use of focused ultrasound to treat deep lesions in the brain causing intractable epilepsy in adults 18 to 80 years old. The study will evaluate the effectiveness and safety of an investigational device that uses ultrasound or sound waves from outside the head to treat seizures that are not well controlled by medication and are due to a small growth of abnormal cells in the middle of the brain, most commonly hypothalamic hamartomas.

PERAMPANEL STUDY FOR INFANTS WITH EPILEPSY

This clinical research study is being done to learn more about the safety of Perampanel (E2007), the study drug, and how well it's tolerated in infants, from 1 month to less than 24 months old (<2 years), who have epilepsy.

Focal (Partial-Onset) Seizures Trials

ESLICARBAZEPINE ACETATE (ESL) AS FIRST OR LATER ADD-ON THERAPY FOR THE TREATMENT OF PARTIAL- ONSET SEIZURES

This study is looking at how effective and safe eslicarbazepine acetate (also known as ESL) is in people 18 years of age or older with partial-onset (focal) seizures, when it is added to their current anti-seizure medication(s).

PFIZER A0081096: LOOKING FOR CHANGES IN EYESIGHT FROM USING PREGABALIN

This study will look at people between 18-65 years old, taking their own anti-seizure medications in addition to either the study drug (Pregabalin) or a placebo. The aim is to look for changes in eyesight.

EPILEPSY FOUNDATION'S HUMAN EPILEPSY PROJECT (HEP2): RESISTANT FOCAL SEIZURES STUDY

This study, the Human Epilepsy Project 2: Resistant Focal Seizures (HEP2) is designed to better understand the challenges of living with focal seizures that do not respond to medication and determine biomarkers of epilepsy severity and treatment response.

Focal or Generalized Seizures Trials

STEREOTACTIC LASER ABLATION FOR TEMPORAL LOBE EPILEPSY (SLATE)

This study is looking at the safety and efficacy of MRI-guided laser ablation therapy with Visualase™ (laser ablation may also be called laser interstitial thermal therapy or LITT) for seizures coming from the temporal lobe in people who are taking seizure medicines.

EFFECTIVENESS OF INHALED STACCATO ALPRAZOLAM IN TREATING AN EPISODE OF FOCAL OR GENERALIZED SEIZURES

A new clinical trial is looking at how effective and safe orally-inhaled alprazolam (also known as STAP-001) is in people 18 years of age or older with focal or generalized epilepsy when given at the time of a seizure episode. This study is commonly referred to as StATEs (Staccato Alprazolam Terminates Epilepsy Seizures).

Epilepsy Foundation in the News

NEW THERAPY COMMERCIALIZATION GRANT AWARDEE Cadence Neuro secures 15 million in series A financing to commercialize a novel neuromodulation therapy for epilepsy that was developed at the Mayo Clinic in Minnesota. Read the Press Release: <https://www.cadenceneuro.com/news>.

DR. SONYA DUMANIS, SENIOR DIRECTOR OF INNOVATION, was interviewed by Proactive at the Bio Investor Forum about ways that the Foundation works to support biotechs in epilepsy. Check out the interview: <https://www.youtube.com/watch?v=FvOPmtM1SI0>

Epilepsy Foundation Announces Shark Tank Finalists

The Epilepsy Foundation has selected five finalists to participate in the annual Epilepsy Foundation Shark Tank taking place on March 12, 2020, in Santa Clara, CA.

- **Aditya Kadambi** from Mocxa for their automated privacy enhancement for seizure videos
- **Atila Borbath** from Synergia Medical for their NAO-VNS product
- **Boris Goldstein, PhD** from Brain Scientific for his graphene electrodes in under the skin EEG implants
- **Jong Woo Lee, MD** along with Product Architectures for their night-time SUDEP prevention device
- **Keely McArthy**, parent of a child living with epilepsy, for her emergency epilepsy band

Congratulations to the finalists and we look forward to hearing their pitches!

Upcoming Research Grants

SUDEP BIOMARKER CHALLENGE

> Submissions **open now**

Prize for \$800,000

AES/EF CLINICAL RESEARCH TRAINING FELLOWSHIPS & JUNIOR INVESTIGATOR RESEARCH AWARDS

> Applications due **January 8, 2020**

Award up to \$50,000 for 1 year

Do you have questions or a research funding opportunity to share? Email **GRANTS@EFA.ORG**

Want to donate funds to research? Go to: **DONATE.EPILEPSY.COM/DONATE-RESEARCH**



CREATE YOUR OWN BRAIN ART TO END EPILEPSY®

You can create and share your own unique Brain Art at EndEpilepsy.org/BrainArt. And please encourage everyone in your care and in your professional and family network to do the same. Brain Art with stories and messages help bring epilepsy into the light and promote awareness, which is critical in our fight to END EPILEPSY®. Please visit EndEpilepsy.org/BrainArt to view powerful stories of others in the fight.

Pipeline Conference
March 12-13, 2020

Shark Tank
March 12, 2020

Community Day
March 14, 2020

Santa Clara Marriott
Santa Clara, California

Dr. Sylvia Olvera

EPILEPSY FOUNDATION | **END EPILEPSY**

Upcoming Conferences

AMERICAN EPILEPSY SOCIETY ANNUAL MEETING 2019

December 6-10, 2019, Baltimore, MD

> meeting.aesnet.org/about/future-meetings

PIPELINE CONFERENCE 2020

March 12 -14 2020, Santa Clara, CA

> epilepsy.com/pipeline2020

SLC13A5 RESEARCH ROUNDTABLE

March 23, 2020, Dallas, TX

> <https://tessresearch.org/>

Have a conference that you want to share? Email LSCHREIBER@EFA.ORG

Mission

To lead the fight to overcome the challenges of living with epilepsy and to accelerate therapies to stop seizures, find cures, and save lives.