KCNT1 EPILEPSY
HOPE IS ON THE HORIZON

Building Hope for KCNT1

November 2023

We are well into the fall season, which means ....**National Epilepsy Awareness Month** is here. (You thought we were going to say "the holidays" are coming, didn't you?)

In this issue you will find updates and reminders of ongoing programs we are participating in to build our path to new treatments.

November is a Month of Gratitude

November is a special month for a few reasons. It's not just National Epilepsy Awareness Month; it's also a time when we think about being thankful. In the United States, we celebrate Thanksgiving, a holiday dedicated to giving thanks. And there's another day called Giving Tuesday or Gratitude Tuesday when people come together to help others.

During this month of gratitude, we will highlight some of our KCNT1 Heroes! The amazing parents, researchers, doctors and dedicated supporters who are the driving force behind our mission to bring hope for new treatments for KCNT1 pediatric epilepsy. They all share a common goal: finding better treatments for KCNT1.

Keep an eye on our social media pages, we'll be featuring the heroes of the KCNT1 community! We invite you to add to our posts and include your heroes! It's a great way to celebrate the wonderful people who are helping make a difference. #KCNT1HeroesOfHope

Together for the children,

The KCNT1 Epilepsy Foundation

✅ Mark Your Calendars ✅

Parent Circle - Ever Need Someone to Talk to?
Saturday, November 11, 11:00 EST
Our second KCNT1 Support Group

Sign up for Parent Circle

Family Meeting: KCNT1 and The Heart
Thursday, November 16
12 - 1 Eastern time
For parents and clinicians, Register here

Sign up for Heart Meeting

Scientific Research Roundtable
Wednesday, November 15
For researchers and clinicians will gather virtually, to share latest discoveries.

Gratitude and Giving Tuesday – November 28

Florida Family Meet-up - Saturday, December 2
Embassy Suites
Orlando, Florida
American Epilepsy Society meeting

showing our
Gratitude
with a giveaway

In November we will be showing our GRATITUDE by giving away a SAMi Sleep Monitor and more! How to enter? Follow our IG, like our post about these Giveaway promotions and Tag one friend. Our social media ambassadors will randomly select one of you to receive the SAMi monitor or our other swag! You can also purchase one at discount to KCNT1 families when you use this link.

KCNT1 EPILEPSY
SAMi
GIVEAWAY
SAMi-3
for KCNT1 families

Advocating for KCNT1 Solutions

Follow the KCNT1 Roadshow
Georgetown
BEACON
KCNT1 EPILEPSY

Greetings from
CALIFORNIA
K
K
K
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Earlier this year we did a "roadshow" on the east coast, visiting several companies who are interested in KCNT1. This fall our we've been making stops around California, advocating for our families and uncovering golden opportunities for groundbreaking KCNT1 research! Recently, co-founder, Justin West spoke at the prestigious UCB/Stanford conference on digital health. Discussions were about the value of data and the need to capture the family experience. He also attended the Global Genes Rare Disease Advocacy Event, and met with two biotech firms who are very interested in KCNT1. Executive Director, Sarah Drislane and Justin West met with researchers and clinicians at the University of California Irvine in their epilepsy departments to pursue potential collaborations.

Offering a Glimpse into the Life of a KCNT1 Warrior

Many dedicated researchers spend their days working in laboratories and offices, often without the chance to connect with the people they strive to assist. Justin West, our co-founder, recognized the importance of inspiring these companies to grasp the real impact of KCNT1 on children and their parents. Driven by the need for swift action, Justin extended a heartfelt invitation to clinicians, researchers, and professionals from a spectrum of biotech and pharmaceutical companies to visit his home and meet his own child. Astonishingly, several individuals eagerly accepted the challenge, making their way to California. They were visibly surprised and moved by the unique nature of this opportunity, noting that such a chance had never been offered before. It's heartening to witness the commitment of these companies to gain a deeper understanding of how KCNT1 affects your children and your families. If you, too, would like to extend this offer, please reach out to us.

Powerful: Families and Researchers Meet at Epilepsy Expo

This week we participated in the Epilepsy Expo at the Disneyland Conference Center. It was an emotional gathering as warrior moms, dads, sisters in law and friends met in person for the first time. It was a powerful experience to have FOUR biotech companies seated at the same table with our families. The researchers got to meet the families and children they are developing treatments for, and the parents got to see there is hope for a better life for our kiddos with KCNT1 disorders.

Checking In
from the
Epilepsy Expo at
Disneyland
EPILEPSY AWARENESS DAY

Clinical Trial Readiness /Family meeting

We hosted a successful family gathering featuring Dr. David Bearden as our panel expert. We discussed the drug development process and the role of caregivers. A total of 63 individuals registered for the event. The meeting transcript was translated into both Portuguese and French and distributed to the respective participants. We're in the process of planning our next Clinical Trials Readiness event, slated for January.

Meeting with the FDA

We initiated our engagement with the FDA Office of Patient Affairs with an introductory meeting to familiarize them with KCNT1. We are in the process of preparing an application for a Listening Session, where we will collaborate with a few families to convey to the FDA how KCNT1 profoundly influences the lives of our children and their families.

More Bilingual Volunteers Needed

Thanks to those who signed up as interpreters and translators. We now have people who speak Korean, Spanish, Tagalog, and Russian. If you can help, sign up with this form. Sign up form

Hola
Bilingual
Volunteers
needed
Não Falo

Empowering Your Journey – Parental Support

We are thrilled to introduce our team of dedicated parents who are ready to assist you in navigating the next steps of your journey or simply provide a listening ear. Our parent support specialists are available to schedule a conversation at your convenience. Our team includes April Hawk, Justin Tanner, Abi Tanner, Amanda Abhul, Heather Patterson, Andy Ip, Kristy Salkewicz, and Tammy Williams, all extending a warm welcome to new families and offering unwavering support as we progress together.

Join Our Second Parent Circle Support Group Meeting

Our first support group meeting witnessed a strong turnout, and we warmly extend an invitation to more of you to join us for the next session on **November 11 at 11 am Eastern time**. This is an agenda-free, judgment-free space. Our parent support specialists, including April Hawk and Heather Patterson, will be on hand to facilitate the discussion. Your participation is highly valued. *Please register using this link.*

We are pleased to introduce our latest team member, Tammy Williams. Tammy is a KCNT1 warrior mom who will be acting as our Parent Engagement Coordinator, helping engage and support the parent community in many ways! If you would like to schedule a time to talk with Tammy one of our Parent Support Specialists, email Tammy.

SHARE YOUR STORY

Sharing your story helps others understand the challenges and successes you've had on your journey. These stories can be used to raise awareness and also help people understand and relate to what you're going through.

We can share these stories with researchers who work in labs, and the FDA, so they can better understand how this disease affects real children and real families. We'd like to invite you to create a short video using the link provided or schedule an interview with our foundation. Your story can inspire, educate, and have a lasting impact.
http://KCNT1.memfox.io/tjzfnh

Calls to Action

Help Build our Directory of Doctors
If you believe your doctor would be helpful to other KCNT1 families, tell them you would like to recommend them for our directory, and ask them to complete our registration: https://kcnt1epilepsy.org/doctor-registration or email Ali@KCNT1epilepsy.org

Building Assets: Patient Data
Participate in our Longitudinal Research Study on Luna
We designed and launched a longitudinal health study on our Luna DNA platform. **So far 64 families from 9 countries have signed up**, and 41 have taken the main demographics and genetic survey. There are several other surveys available.

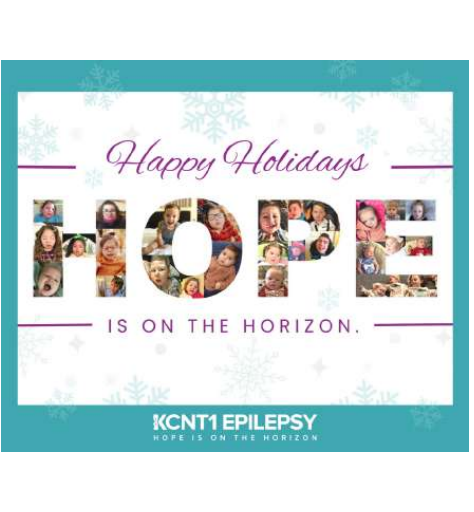
The KCNT1 Epilepsy Foundation Longitudinal (Research Registry) Study is open to all families within and outside the U.S. on the **LunaDNA platform**. This requires you to create an account for your child/ward on Luna, join our KCNT1 community, join our study, and take surveys throughout the year. See our website page, Participate in Research for instructions.

Take Our Poll: Help Us Plan for 2024

29 families completed this poll to share their preferences and thoughts about having an in-person family and professional conference. So far, the top destinations are: Orlando, Philadelphia and Nashville! Since it is difficult for our families to travel, we are likely to host micro-conferences around the world. Our first is tentatively scheduled in Philadelphia on June 7 -- the day before the Million Dollar Bike Ride. If you'd like to help organize this event we are looking for volunteers. Fill out the poll here

We'd Love More of Your Warrior Photos

We would love some photos of your adorable warriors for Epilepsy Awareness Month and the holidays! (Yes..we said, it...) Fill out our photo release form and attach photos! Thanks!



Complete Our Report Card

Please take a moment to give us some feedback on our efforts. As a nonprofit we have a duty to use our time and donations effectively to ensure we are marching towards our mission and your feedback can help guide us.
https://forms.gle/vbaZyLHPmcXTuvUi8

Education Corner

Have You Heard of SUDEP?
Some of our families have not heard of this: Sudden Unexpected Death from Epilepsy. October 18th was SUDEP awareness day, where we bring awareness that people with epilepsy are at significantly higher risk of sudden unexpected death. Discuss it with your health care team. Watch webinars, download tools, including the Patient Handout, and talk with your healthcare team about making your safety a priority. Create a Seizure Action Plan

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RESEARCH AND DRUG DEVELOPMENT

Announcing our Science Outreach Officer

We are delighted to introduce Ali Rosenberg, PhD, as our newest team member. Ali is a scientific consultant with a strong foundation in cellular and molecular biology, microbiology, genetics, regenerative and developmental biology, and neuroscience. She brings a wealth of scientific communication expertise to the table, with a track record of delivering numerous invited talks, presentations, papers, and securing competitive grant awards.

Ali will play a pivotal role in guiding and managing our collaborations, programs, and partnerships. We are excited to have her on board, and her extensive expertise will undoubtedly contribute significantly to our mission.

Funding Research

Nationwide Children's Hospital at Ohio State: In our pursuit of groundbreaking research, we are obtaining KCNT1 patient specimens for a proof of concept/feasibility test of a gene editing method at Nationwide Children's Hospital. This exciting project holds the potential to open new doors in the search for treatments.

Mouse Model Drug Testing at Virginia Tech: We are eagerly anticipating the hiring of a dedicated lab technician at Virginia Tech to support our project. Once in place, we will provide funding to facilitate the testing of a repurposed drug in a mouse model. This endeavor aligns with our commitment to rigorous research.

Cell Model Development: Thanks to parents who are participating in our biobank so that we can create cellular models to better study KCNT1 and develop potential treatments.

Million Dollar Bike Ride Grant: We are thrilled to report that out of 29 rare disease groups that participated in 2023, KCNT1 had one of the highest submission rates for proposals! The grant reviewers are busy reviewing all the proposals now, but they can only select one to give the award to, but that means we have six left to decide which ones we will fund in 2024 ourselves, so the fundraising must continue!

Drug Repurposing Studies

What is a drug repurposing study? A drug repurposing study looks at existing medications that were developed for different health issues and analyzes whether any could be used to treat another disease. These identified drugs could potentially be used as "off-label." This approach to drug discovery can save a lot of time and resources compared to creating a completely new medication from scratch.

Three academic institutions have conducted repurposing screenings for KCNT1, Vanderbilt, University of South Australia and University of Leeds. They have identified some drugs that could potentially help inhibit the overactive KCNT1 channel. Most of this testing has only been in computer modeling or cells in a lab, so further analysis should be done to see if these drugs are safe for children and don't cause dangerous side effects.

In preparation for the papers to be published on these drugs, we are designing surveys to measure how effective a new drug could be for your child. We are setting up a study that will allow you to track the effectiveness of any off-label drugs through an online observational study on our Luna platform. This way we can see some of these drugs work well for some people and not for others and report back on it. Families and doctors will be notified of these drugs when the information comes available. (Please share your doctor's email address with us so we can notify them.)

We invite all of you to create an account for your child on Luna if you think you would like to participate in future observational studies. We also recommend that everyone keep a seizure journal. The Seizure Tracker app is recommended by several of our parents.

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luna™

Why We Use the Lu na Platform

We utilize the LunaDNA platform to administer studies to learn about how KCNT1 affects your families. Why can't we just use Survey Monkey or Google Forms? Three years ago we asked the same thing... We quickly learned that critical aspects of scientific investigations of human subjects requires taking many steps to protect your data. We are required to go through training and work under the guidance of an Institutional Review Board to ensure we are taking all the measures to protect your data and ensure your privacy. In addition, in order to publish articles in scholarly journals or share information with the FDA, only data that is generated following rigorous research and quality standards is accepted. The Luna platform provides this. It also explains why you have to take extra steps in order to share your email address with us when you enroll in Luna!

Entering Our Research Era

Enroll in the Research Registry on the Luna Platform

Our longitudinal study being hosted on the LunaDNA platform. This is our international research registry. Throughout the year we will add **new studies and new surveys**. Angel parents are encouraged to take the KCNT1 Specific Demographics survey from their child's account.

Here is a link to get started >>guides. We can schedule a time to help you create a child account, enroll and start the surveys.

How Your Participation in Observational Studies Helps *Be a Hero*

Clinical trials are like tests for new treatments, they use an intervention and then measure how it affects a pre-determine endpoint. We all want to see clinical trials for our kiddos, but sometimes we need to do other important studies before those trials, especially when it comes to our kids' health. Parents, this is where you can play a crucial role by taking part in observational studies. These studies usually involve answering questions or talking to researchers. Here's why your help in observational studies is super important:

Understanding How Treatments Affect People: We want to know how treatments affect patients in their everyday lives. This includes things like if they take their medicine properly, how well they get better, and if there are any unwanted side effects.

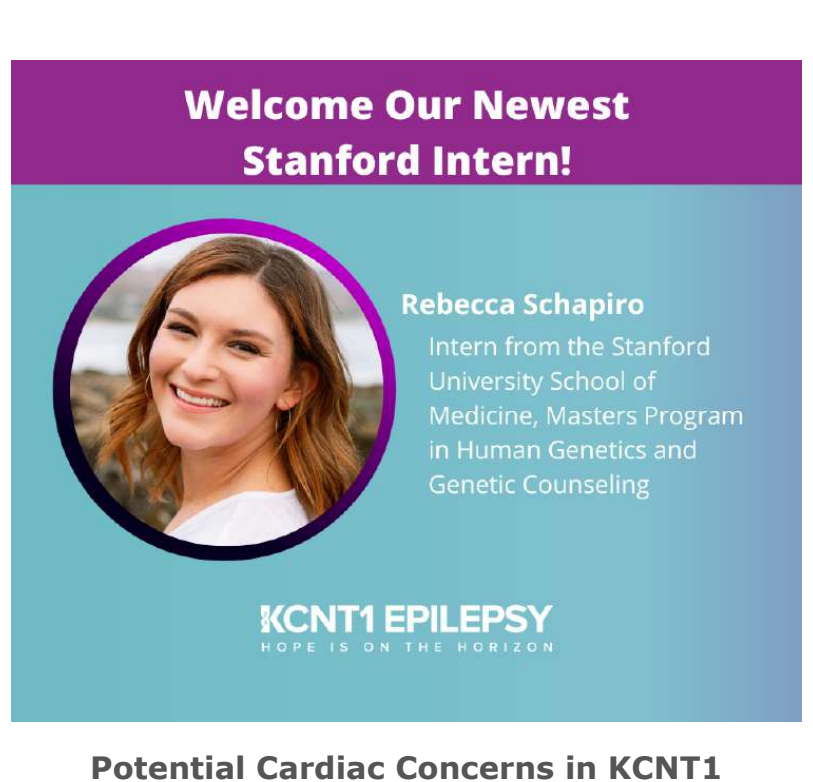
Possibly Skipping Placebos: Sometimes, the people who make the rules (the FDA) might say we need a "placebo" group in a trial. That's a group that doesn't get the real treatment. But if we gather enough information from observational studies, we might be able to skip that part and get to helping people faster.

Spotting Hidden Clues: Observational studies are like detectives looking for clues. They help us find out if there are things that make a disease worse or better. These clues can be tricky to see without lots of information.

Understanding Everyone: Observational research looks at all kinds of people. That means we can learn how a disease affects kids and grown-ups, boys and girls, different races, and people with different amounts of money. This helps us make sure our treatments work for everyone.

Seeing How Things Really Happen: In clinical trials, things are controlled, like a movie set. But observational studies are like watching a real-life movie. They show us how the disease behaves in the real world, where things aren't always perfect.

So, parents, your help in these studies is like being a hero in a special mission, and November is our hero month! By taking surveys or talking to researchers, you're helping us understand diseases better and find treatments that work for everyone, especially our kids. You can join in different studies to make a difference.



Welcome Our Newest Stanford Intern!

Rebecca Schapiro
Intern from the Stanford University School of Medicine, Masters Program in Human Genetics and Genetic Counseling

KCNT1 EPILEPSY
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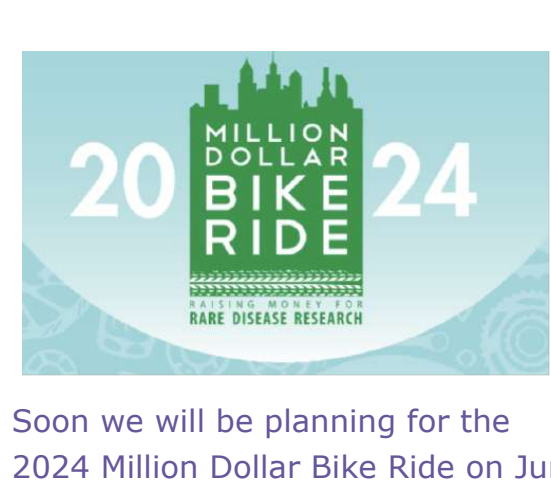
Potential Cardiac Concerns in KCNT1

Stanford University genetic counseling student, Rebecca Schapiro, has an important project on her hands. She is working hard to gather information about heart problems linked to KCNT1. Even though she has a short internship with us, she is meeting with parents, clinicians and researchers to learn as much as she can in hopes of creating a guide for clinicians and parents to advocate for their child's health. If you would like to share your experience with Rebecca, email us. We are grateful to have Rebecca put her passion for genetic counseling and strong commitment to assisting families toward this special challenge.

FUNDRAISING NEWS


20
MILLION
DOLLAR
BIKE
RIDE
24
RAISING MONEY FOR RARE DISEASE RESEARCH

Soon we will be planning for the 2024 Million Dollar Bike Ride on June 8, 2024! We hope to have many of you participate and help us secure next year's research grant from Penn Medicine and the Orphan Disease Center.

We need volunteers to help plan and organize next year's event. Please reply to this email and let us know! **We can't wait to hear from you.**

Sign up here if you want to be on our mailing list to volunteer or participate in 2024!

Girls Volleyball Charity Event

Coach Krystle Stanley, the mother of Charly, expressed her joy as her volleyball team hosted a charity night in support of the foundation. Collaborating with the Volleyball Booster Club, the team successfully raised funds in honor of Charly, and also played a crucial role in raising awareness about the importance of research for KCNT1 epilepsy! Go Team!

Thank you!
For supporting the KCNT1 Epilepsy Foundation and Pediatric Epilepsy Research
HERITAGE GIRLS VOLLEYBALL

FAMILY FUNDRAISERS

Thank you to our families who continue to find creative ways to fundraise!

Your fundraisers are an important way to help us fund the work we do and the research programs that need funding.

Host a Giving Tuesday Fundraiser

Last year we had 45 generous people donate an average of $100 to the Foundation on Giving Tuesday, the last Tuesday of November.

Our hope is they will donate again this year and ask one friend to support our mission! **Our goal is to have 100 donations on Giving Tuesday!**

We hope to see many creative fundraisers for Giving Tuesday -- which we are naming **GRATITUDE TUESDAY** this year! You can use our link to host your own fundraiser by clicking on **Fundraise** and invite your friends to be Heroes of Hope. If you need ideas or help, email us! Another simple fundraiser is to create a "Facebook fundraiser" and use the 'invite' button to share it with your FB friends. Learn how at this link!

Meet Our KCNT1HeroesofHope

Arpin Strong/Arpin International Charity Golf Tournament Raises $5,000 for KCNT1

The Foundation was chosen to be one of the beneficiaries of a charity golf tournament and was awarded $5,000. Two of our families attened this Arpin Strong Golf Tournament in Bellingham, MA on a chilly, rainy day, to represent the Foundation: Michele and Alex McKendall with their son, Teddy as well as Alex Crooks and daughter Emerson, and her grandfather, Steve. You can see them interviewed in this video on YouTube.

Bike the Pike 2023
Havertown, PA

Tyler Higgins, second cousin to a KCNT1 warrior, Leighanne, hosted his fifth bike ride! Each year this event gets larger, and this year he raised $12,000 for the Foundation! Thanks to Tyler, the participants and sponsors. We are grateful!

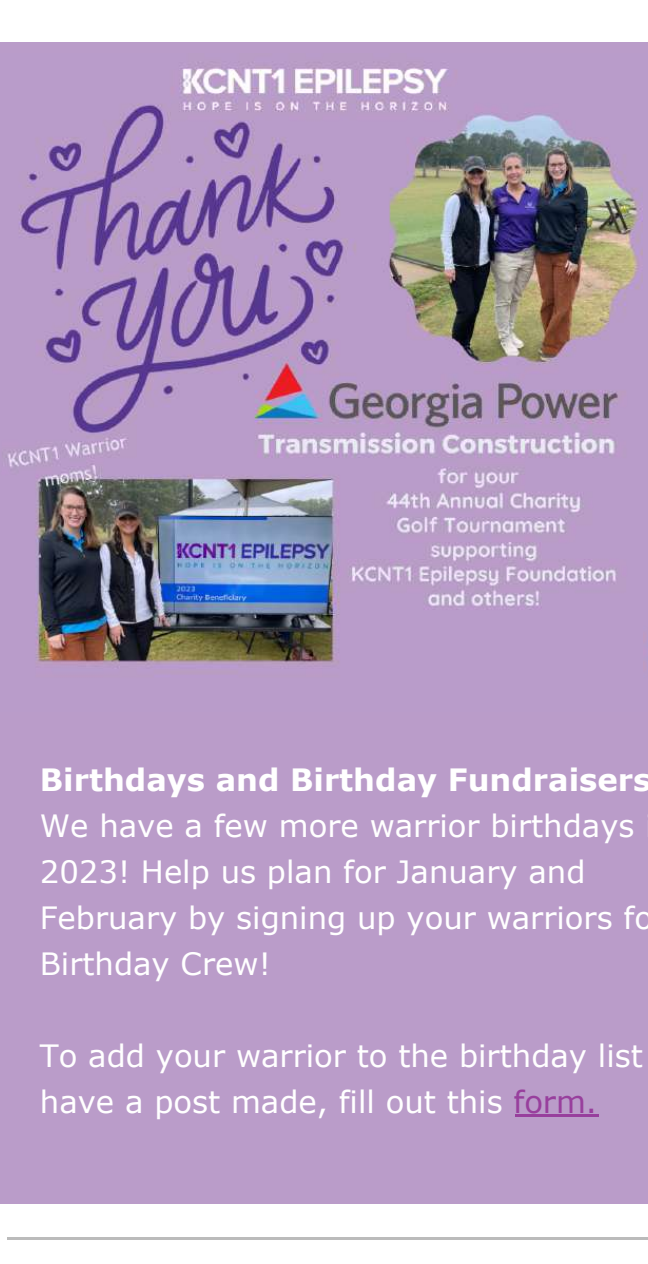
We Love Lucy

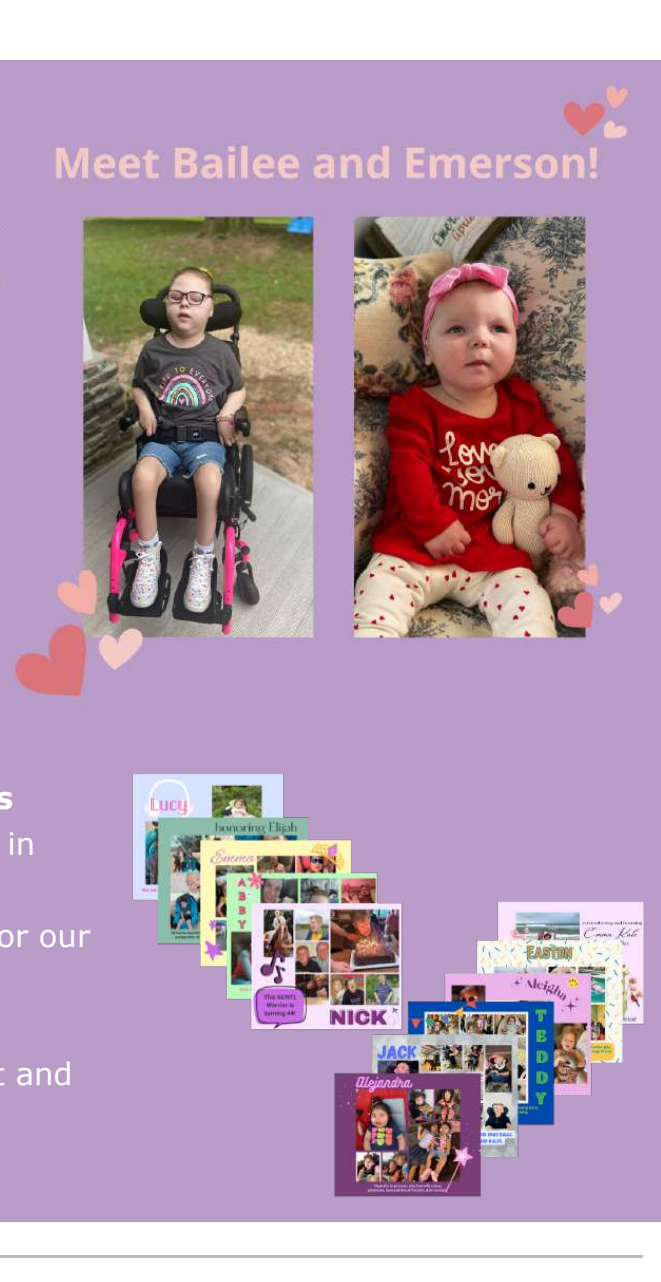
Lucy Greenblott celebrated her fifth birthday in style, as her family organized another community concert, bringing together neighbors and friends for an afternoon of music and fun. The event was not just a birthday celebration; it also aimed to raise both money and awareness for the KCNT1 Epilepsy Foundation. Everyone had a fantastic time, enjoying the music and coming together to support Lucy and the KCNT1 warriors.

Happy 5th Birthday, Lucy!
Thanks to Everyone Who Made it Special!

A Swing for Support in Peachtree City, Georgia

Two dedicated mothers, Tammy Williams and Alex Crooks, are both employed at Georgia Power, came together last year. Fate brought them together when they realized that both of their daughters had been diagnosed with KCNT1. Given the suggested rarity of KCNT1 and the extensive workforce of over 8,000 employees at Georgia Power, the likelihood of this remarkable connection is astonishing. A huge thanks to Georgia Power Transmission Construction for selecting our Foundation as one of the beneficiaries of their charity golf tournament. We appreciate Georgia Power's generosity and the Warrior Moms who spent the day representing the KCNT1 community! This will make a real impact, driving awareness and progress for our cause!


KCNT1 EPILEPSY
HOPE IS ON THE HORIZON
Thank you
Georgia Power
Transmission Construction
for your 44th Annual Charity Golf Tournament supporting KCNT1 Epilepsy Foundation and others!
KCNT1 EPILEPSY


Meet Bailee and Emerson!

Birthdays and Birthday Fundraisers
We have a few more warrior birthdays in 2023! Help us plan for January and February by signing up your warriors for our Birthday Crew!

To add your warrior to the birthday list and have a post made, fill out this form.

WE CAN DO THIS TOGETHER

Your dedication makes all the difference. Let's work together to create a brighter future for those affected by KCNT1. We are truly grateful for your unwavering support! Email us if you have questions!

Donate

Our Contact Information
{{Organization Name}}
{{Organization Address}}
{{Organization Phone}}
{{Organization Website}}

Looking for something?
Check out our Linktree list of links!
https://linktr.ee/kcnt1

{{Unsubscribe}}