KCNT1 EPILEPSY

HOPE IS ON THE HORIZON

FDA Patient Listening Session

04/22/2024

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FDA Patient Listening Session Meeting Summary
KCNT1 Epilepsy Foundation

On April 22, 2024, the KCNT1 Epilepsy Foundation held a Patient-led Listening Session with the Food and Drug Administration. KCNT1 Epilepsy Foundation is a 501(c)3 non-profit patient advocacy group founded by parents seeking information and support for the rare disorder known as KCNT1-related epilepsy. The mission of the KCNT1 Epilepsy Foundation is to accelerate research and drug development efforts focused on finding a cure for KCNT1-related epilepsy. The KCNT1 Epilepsy Foundation supports the KCNT1 community with educational resources and the latest research information.

Objective of Session:

The objective of the session was to provide FDA staff with the opportunity to hear about the lives of patients and families living with KCNT1-related epilepsy, the urgent need for treatments, what caregivers feel would be meaningful treatment outcomes and their risk tolerance to obtain those outcomes.

Agenda:

- Introduction & welcome from the FDA
- Foundation introduction & overview of KCNT1-related epilepsy
- Shared stories of parent / caregiver lived experience
- Moderated discussion of goals and preferences for treatments and participation in trials
- Clinician perspective
- Moment of silence
- FDA Q & A

Patients and Community Members Represented:

- Sarah Drislane – Executive Director of the KCNT1 Epilepsy Foundation, aunt of a child with KCNT1
- Nine parent/caregivers presented and spoke about their experience representing each of their children who have KCNT1-related epilepsy:
    - Julie and Jared S – parents of a 4-year-old child with KCNT1
    - Jacalyn F – parent of a 3-year-old child with KCNT1
    - Erna G – parent of a 21-year-old adult with KCNT1
    - Samantha M – parent of a 5-year-old child with KCNT1 and MAPCAs
    - Heather and Brian A – parents of a 13-year-old child with KCNT1
    - Preston M – parent of a deceased child with KCNT1
    - Lorena and Alvaro A – parents of a 3-year-old child with KCNT1

Medical Professional Attendee:

- David Bearden, MD, MSCE – Associate Professor of Neurology and Pediatrics, University of Rochester Medical Center

Consultant Attendee:

- Larry Bauer – Hyman, Phelps & McNamara, P.C.

What is KCNT1-related Epilepsy?

KCNT1-related disorders are caused by a gain of function mutation in the KCNT1 gene, which codes for a sodium-activated potassium channel present in both excitatory and inhibitory neurons. This primarily results in problems in electric electrical activity in the brain, which can cause seizures.

KCNT1-related epilepsy encompasses a spectrum of phenotypes. Earlier onset seizures, usually occurring within days to the first 6 months post birth, are associated with more severe cases and these individuals are often diagnosed with Epilepsy of Infancy with Migrating Focal Seizures (EIMFS; formerly known as Malignant Migrating Focal/Partial Seizures of Infancy (MMFSI/MMPSI)). Additional common impairments include muscle and gut issues, and the inability to hold one's head up or swallow. 10 to 12% of individuals develop pulmonary collateral arteries which can burst under pressure filling their lungs with blood. Many children develop dysphagia and are dependent on feeding tubes for nutrition and medication. Other early-onset phenotypes include Early-Onset Epileptic Encephalopathy (EOEE), West syndrome, Ohtahara syndrome, early myoclonic encephalopathy, leukodystrophy, focal epilepsy and multifocal epilepsy. EOEE results in severe cognitive and developmental impairment. It may include infantile spasms, developmental regression, and abnormal electroencephalogram (EEG) patterns. This phenotype experiences a high mortality risk.

Individuals with later onset seizures are less severely impaired and typically diagnosed with Sleep-related Hypermotor Epilepsy (SHE, or Autosomal Dominant Sleep-related Hypermotor Epilepsy (ADSHE), formerly referred to as Autosomal Dominant Nocturnal Frontal Lobe Epilepsy (ADNFLE)). ADSHE is often characterized by nocturnal seizures that are often initially misdiagnosed as sleep terrors, night terrors, sleep apnea or reflux, speech delay, and often requires multiple EEGs prior to finding the first seizure.

The Foundation is aware of >300 families around the world who include an individual who has KCNT1-related epilepsy, out of an estimated 3,000 diagnosed. The estimated incidence is ≤1.121 per 100,000 live births.

Common Health Problems in KCNT1-related Epilepsy:

The following list includes the most common clinical symptoms present in:

Epilepsy of Infancy with Migrating Focal Seizures (EIMFS):

- Hundreds of daily seizures
- Primarily focal motor seizures
- Migrating seizures arise from various locations in the brain
- Hypotonia, dystonia, dyskinesia
- Respiratory issues
- Gut issues
- Cardiac issues
- Global developmental impairment
- Regression
- Intellectual disability
- Inconsolable crying and irritability
- Cortical visual impairment

Early-Onset Epileptic Encephalopathy (EOEE):

- Severe cognitive and developmental impairment

- Infantile spasms
- Developmental regression
- Abnormal EEG patterns
- High mortality risk

Autosomal Dominant Sleep-related Hypermotor Epilepsy (ADSHE):

- Focal motor and other seizures
- Cognitive impairments
- Psychiatric disorders
- Behavioral & emotional problems
- Vigorous movements during sleep
- May be mistaken for night terrors
- Delayed speech
- Memory challenges
- Constipation
- Cardiac issues

How KCNT1 Caregivers Feel:

Because all patients with KCNT1 have seizures refractory to current medications, all caregivers are desperate for therapies to improve their children's lives. *The Peds QL Family Impact Scale*, a standardized quality of life survey, showed that most of our families are under severe stress, feeling anxious and frustrated, and many of them feel sad. Top concerns include their child's seizures, delayed or halted development, decreased life expectancy, and general poor prognosis. Families live with daily fear that their child could die at any time.

Mortality:

KCNT1-related epilepsy has a high mortality rate, with 35 known deaths since 2019, 12 known deaths in 2023, and 5 known deaths to date in 2024, representing 11% of the known population. Because infants in the early onset epileptic encephalopathy group die quicky, it is likely that there are both more individuals and deaths that are uncatalogued.

Medication:

A survey of 45 families from the USA shows that most children are taking 5 or more anti-seizure medications concurrently in an attempt to stop seizures and preserve their child's development. No existing anti-seizure medication has shown efficacy in this population.

Parents Share their Experiences:

Julie and Jared, parents of Easton, age 4

Julie and Jared's third child, Easton, began experiencing refractory seizures at three months old, leading to near-death experiences and a **115-day hospital stay**. Despite trying 19 different medications, Easton still suffers from over **100 seizures daily,** many where he stops breathing. He has **lost his ability to hold his head up, follow things with his eyes, smile, laugh,** and relies on a gastrostomy tube (G-tube) for nutrition and medication.

The family uses extreme precautions to prevent illness, **as even minor sicknesses can be life-threatening** for him. They have made **significant home and lifestyle changes** including avoiding public places, homeschooling Easton's siblings, and Julie gave up her career. They also have medical equipment at home so they can avoid hospital visits. The family lives in isolation, limiting contact with others to protect Easton's health, which has strained their relationships with friends and family. Despite their **efforts to avoid infections and hospitalizations**, Easton has been **hospitalized 21 times in four years,** amounting to seven and a half months spent in medical care, underscoring the family's constant battle to keep him alive.

"We wear N95 masks inside... And we don't take him inside anywhere. That means he can't be with other kids his age... 'cause we can't risk the exposure which means he and our family are incredibly isolated."

Easton's parents

Jacalyn, parent of Ember, age 3

Jacalyn's three-year-old daughter, Ember, was developing normally, walking and starting to talk, until she experienced her first seizure at nine months old. A video of Jacalyn's daughter Ember was shown, with Ember smiling and pushing a baby walker, standing and playing with the pet cat, and then showing Ember's loss of these abilities after a period of uncontrolled seizures. Ember was experiencing up to **50 seizures a day**. Despite numerous medications, her seizures were only reduced to about 20 a day. Ember spent **85 percent of her first year in the hospital** due to unmanageable seizures.

At 18 months of age, Ember seized for 32 continuous hours, resulting in significant **developmental regression**. At age 3, Ember cannot sit, eat orally, talk, walk, or interact. Due to severe swallowing problems, she **faces issues with secretion management and has undergone surgery to clip her salivary glands**, losing her front teeth in the process. Ember also suffers from recurrent aspiration pneumonia, leading to multiple intensive care unit admissions and a collapsed lung.

Ember's seizures are increasingly aggressive and can be triggered by various factors like colds, temperature changes, and loud noises, requiring the family to administer **rescue medication two to three times a week**. Despite the challenges, Ember shows joy during activities like getting her nails painted, having her hair done, and cuddling, however, her mother laments the loss of a normal childhood for her daughter due to the relentless seizures that seem to worsen her overall health issues having a cumulative effect. Jacalyn **lives in constant worry of losing Ember**, especially during critical intensive care unit stays.

Erna, parent of Mandy, age 21

Erna's 21-year-old daughter, Mandy, suffers from KCNT1-related epilepsy. Mandy's initial symptoms appeared at three months old, with frequent seizures leading to a two-month hospital stay. Despite extensive testing and

multiple anti-seizure medications, Mandy continued to have up to **100 seizures a day** and wasn't diagnosed with KCNT1-related epilepsy until she was 16 years old.

The daily seizures have **severely impacted Mandy's development**. She is non-verbal, non-ambulatory, and relies on a G-tube for feeding. She has **cortical visual impairment, spasticity, hip dysplasia, hip subluxation, and scoliosis**. Mandy also experiences episodes of **uncontrolled chorea**, an involuntary movement disorder, lasting for weeks and affecting her ability sleep.

Mandy has **never been seizure free in 21 years**. **Seizures sometimes last up to 14 minutes,** and she requires frequent suctioning. Because of her condition, her doctors have discussed the consideration of **advanced directives and comfort care,** highlighting the severe and irreversible damage caused by KCNT1. Erna emphasizes the urgent need for research to provide a better quality of life for children like Mandy.

"Mandy's daily life is a struggle... She has never had a seizure-free day."

Mandy's mom

Samantha, parent of Charlotte, age 5

Samantha's five-year-old daughter, Charlotte, was a healthy baby, but began experiencing severe seizures at six weeks old. This led to over **50 hospitalizations** in five years. Despite extensive testing and treatments, including a medically induced coma, Charlotte continues to have intractable seizures.

Samantha shared the shock of a dual diagnosis: the presence of a rare but serious cardiac condition known as **major aortopulmonary collateral arteries (MAPCAs)**, causing Charlotte's heart to work extra hard, and leading to frequent bleeding of the arteries inside her lungs. These bleeding episodes are life-threatening, and the family lives in constant fear of Charlotte's death.

In addition to her cardiac issues, Charlotte has **severe developmental impairments**. She cannot stand, which affects her hips and spine, and has severe gastrointestinal (GI) issues requiring regular suppositories for bowel movements. She has tried numerous diet options to alleviate the **GI issues**. Her lack of strength requires her parents to catheterize her just so she can urinate. Charlotte is also **highly susceptible to infections**, complicating her condition further.

Samantha described the overwhelming precautions and extensive measures required for caring for Charlotte. Despite their efforts, Charlotte's condition continues to cause her **significant pain and suffering.** Samantha has never heard her daughter's voice or seen her smile. She emphasized the profound impact of KCNT1 on their lives. She advocates for more awareness and support for children like Charlotte, who deserve better care and a better quality of life.

> **“I’m her mom and I’m supposed to be able to fix it but I can’t.”**
>
> **Charlotte’s mom**

Brian and Heather, parents of Abigail, age 13

Brian and Heather’s daughter, Abigail, began having **seizures at the age of eight**. Initially misdiagnosed with Benign Rolandic Epilepsy, Abigail’s condition worsened, leading to back-to-back **tonic-clonic** seizures. An EEG revealed **37 tonic seizures in one night**, leading to a diagnosis of Autosomal Dominant Nocturnal Frontal Lobe Epilepsy (now referred to as Autosomal Dominant Sleep Hypermotor Epilepsy (SHE)) and Abigail started on new medication.

Despite trying numerous antiseizure medications, Abigail continued to have 6 to 12 motor seizures every night leading to safety concerns such as falling. Her condition deteriorated: she became **tired, irritable, and lost coordination, memory, and the ability to enjoy her favorite activities.** Abigail began a short-lived cannabidiol (CBD) trial that caused severe tonic seizure clusters that continued for days, with Abigail experiencing as many as **300 seizures**. Rescue medications failed to stop these seizures and the rescue medication’s side effects include fits of rage.

Over the years Abigail's condition worsened, and she has had as many as **five different types of seizures**. Good nights involved 20 seizures, bad nights hundreds. She **missed school, fell behind academically,** and lost friends due to her condition. Today, **at 13, Abigail’s health continues to decline**, with KCNT1 robbing her of a normal childhood and severely impacting her family's life. Despite their efforts and hopes for better treatment, Brian and Heather feel helpless as they watch their daughter slowly deteriorate as they wait for new treatments.

> **“She can have up to 300 seizures in a day.”**
>
> **Abigail’s mom**

Preston, parent of Jack, deceased at age 6

Preston’s son, Jack, was diagnosed with KCNT1-related epilepsy and passed away recently. Jack experienced his first seizure at just three days old, initially dismissed as normal neonatal movements. At ten days old, he was admitted to the hospital, marking the beginning of **over 30 hospital admissions.**

A devastating moment came when Jack was diagnosed with the genetic disorder KCNT1, which the doctors described as terminal and impossible to predict. The parents were told that Jack wouldn't live past the age of two, but he defied those odds, living to celebrate his sixth birthday. He experienced **hundreds of seizures daily,** often **requiring sedation to sleep.** He had rescue medications that sometimes failed, leading to respiratory issues that required breathing assistance.

Jack's life was consumed by medication management and constant medical care. He had over **30 medications for seizures and sleep and** required **frequent suctioning** to clear his airway. Despite these challenges, Preston described Jack's favorite things, including fishing with his father, watching television with his parents, and music. With the help of an eye gaze device Jack was able to express his desire to be with other kids and attend kindergarten, which he did, participating in activities like riding the bus, attending field trips, and competing in the Special Olympics.

Tragically, after his sixth birthday, Jack was admitted to the hospital for the last time for **respiratory complications.** On August 24th, surrounded by his family, Jack passed away. Preston recounted the heartbreaking final moments, holding Jack as his **breathing slowed and eventually stopped.** Preston emphasizes the need for better treatments for KCNT1, wishing Jack could still be with them today.

Lorena and Alvaro, parents of Nathaniel, age 3

Lorena and Alvaro's youngest son, Nathaniel, has KCNT1-related epilepsy. Born full-term with no complications, Nathaniel started having **seizures on his third day of life**. At a routine doctor's appointment, Lorena showed videos of his twitching movements, prompting immediate hospitalization and a diagnosis of KCNT1. Nathaniel was sent home with three seizure medications, yet continued to have frequent seizures, sometimes 14 per hour, totaling hundreds of seizures in a day.

Nathaniel spent **53 days in the hospital his first year of life**, trying to get seizure control. He was eventually sent home but he was heavily medicated, often asleep all day, or crying nonstop. The parents described that he **lost the ability to smile and track with his eyes.** He also began choking on his milk, **necessitating a G-tube for feeding.** Nathaniel's low muscle tone prevents him from holding his head up or being able to swallow, requiring ongoing suctioning to prevent choking.

Nathaniel's condition is a daily struggle, and his parents feel as if they are constantly guessing how to comfort him. After numerous hospital stays and the continued decline of his health, the family made the difficult decision to **enroll Nathaniel in hospice care**, feeling it was the best option to prevent his suffering. Lorena and Alvaro expressed they are willing to take any risk with new medications to provide some normalcy and comfort for Nathaniel.

"He was asleep or crying.
There was no in-between."

Nathaniel's mom

Moderated Discussion - Goals and Preferences for Treatments and Participation in Trials:

Parents were asked about what symptoms they would most like addressed in a potential new treatment.

Parents asked for **improved seizure control**, and anti-seizure **medications that were not sedating** so that their children could be awake for more of their lives. One parent asked if the **gene mutation itself could be fixed** to

avoid the regression of functions as well as the seizures. **Developmental outcomes and quality of life** were among the most desired treatment outcomes, such that their **children could live less isolated lives with less time in the hospital** with **negative respiratory outcomes**. Samantha, whose child Charlotte has rare cardiac complications, asked for **improved cardiac therapies**, stating that the MAPCAs are a "ticking timebomb".

Samantha was asked whether other families with MAPCAs had similar stories. Samantha answered that some children with MAPCAs have died, and that parents were able to share what surgeries to get or avoid.

Parents were asked how much symptom improvement would be considered meaningful from a treatment.

Parents answered that while eliminating seizures was their goal, even **reducing seizures would greatly help**, as seizures make their children exhausted and caring for their children during seizures, which occur day and night, prevent the entire family from sleeping. Abigail's parents said a **25% reduction would change their lives**. Jacalyn said that **even one less seizure** would be a massive improvement, as Ember's seizures are catastrophic and aggressive, and Ember's body is exhausted after a seizure and she has no energy, and that this further diminishes Ember's ability to fight illnesses when she is sick. Jacalyn said she is desperate for even a small change. Samantha answered that if Charlotte could **hold up her head, sit up, smile, or grasp items in her hands** it would increase her and her family's quality of life.

"Even reducing it by one seizure would make a world of difference for her... her seizures are so catastrophic and aggressive.

Ember's mom

Parents described their difficulty in interpreting their nonverbal child's needs and not knowing where their child was feeling pain and how to help them feel better. Julie described how Easton will cry for hours on end and how no specialist can pinpoint a specific issue, and that their **inability to diminish his pain** is one of the worst aspects of this disorder. Parents described their fear and guilt that they are wrong about their interpretation of their noncommunicative children's needs.

Parents were asked what kind of information they would need to see before enrolling their child in a clinical trial.

Some parents answered that they would want to see success in preclinical tests and understand the risks and side effects. Other parents described their desperation, the horrors of watching their children experience this disorder, their helplessness, and that **they would do anything and try anything to help their child** even potentially improve. Parents express that **their children are already taking risks** using medications not meant for KCNT1 epilepsy, and using these medications feels like being in trial with many potential side effects. The moderator spoke to an unpublished caregiver survey showing that 20% of caregivers are **willing to risk severe side effects** for significant improvements or a cure.

“We would be willing to do essentially anything… we are borrowing medications intended for something else and they are not working.”

Easton’s mom

“It feels like we are given a band-aid when we need stitches…”

Charlotte’s mom

Clinician Overview and Key Insights

After the families spoke, Dr. David Bearden presented key insights into the burden of disease and unmet medical needs based on his experiences as a clinician. The summary and key insights are below:

- KCNT1 is one of the most severe genetic disorders he treats and is among the most refractory to conventional seizure medications.
- Many painful comorbidities with most children experiencing chronic pain of unknown cause, chronic lung disease, spasticity, and occasionally, abnormal blood vessels that can bleed into the lungs.
- Associated with early mortality, with some studies describing more than 50% of children dying before the age of 10.
- Ultra-rare disease, but the increased accessibility of genetic testing means that more families are identified yearly around the world.
- Current anti-seizure medications in this condition have almost no effect beyond a handful of patients, though most patients are taking at least five anti-seizure medications attempting to minimize seizures.
- He attempts to preserve development with supportive therapies and remove sedating medications that aren’t helpful.
- Patients need extensive care teams of specialists including a neurologist, gastroenterologist, nutritionist, cardiologist, and multiple therapists.
- Many parents stop working and interacting with society to provide care for their child.
- In future therapy parents desire even small improvements in their children and less time in the hospital.
- He expects that seizure control can lead to developmental improvements.
- There are some therapies in the pipeline in the next few years for KCNT1-related epilepsy, which are desperately needed.

Moment of Silence

A moment of silence was observed to honor the children that have died from KCNT1-related issues.

Q&A / Comments from the FDA

FDA staff expressed appreciation for the presentation and particularly to the families for their open discussion of the challenges they and their children experience with KCNT1-related epilepsy.

One FDA staff member recounted that he heard the families state that a reduction as small as even one fewer seizure would be a clinically meaningful change for their children and themselves. This staff member also acknowledged the parents' difficulty in determining whether one is making the right choices for their children and validated the parents' decisions and concerns.

Willingness to Travel for Trials and Interest in Decentralized Trials
FDA staff acknowledged the fragility of those with KCNT1-related epilepsy and asked about the parents' willingness to travel to sites for clinical trials, their ability to participate, and their views on the burden and potential risks involved in a clinical trial. Parents answered that they would prefer something near where they live or home-based to accommodate their family situation, but that they would do whatever it takes for the possibility of a better treatment, and would figure out how to make it work, even if it meant moving.

Risk Tolerance for New Innovative Therapies
FDA staff acknowledged that we are in an era where new innovative treatments are becoming available for genetic diseases and emphasized that lessons are being learned, including the possibility of serious, irreversible side-effects or even the risk of fatality. The question was posed to the families asking what their thoughts were about this level of risk. Parents responded that on a daily basis their children already face mortal risk: from potentially fatal seizures, from Sudden Unexpected Death in Epilepsy (SUDEP), and even from something as innocuous sounding as going to the park or shopping center [where they might encounter an infection]. Samantha commented that "We are already fighting for [my daughter's] life everyday…so I would rather have her pass with us trying and doing something that could lead to even better insights, than not trying anything at all."
Additional caregiver comments:

> *"I feel like there's no other option at this point*."
>
> "*Our children take that risk every second of every day."*

The Q&A session concluded with FDA staff expressing their appreciation and gratitude for the parents sharing their experiences, and their hope that this day would be seen as a turning point for KCNT1. The KCNT1 Epilepsy Foundation thanked the FDA staff for their time and for the opportunity to present this overview of KCNT1-related epilepsy and share caregiver stories at this FDA Patient Listening Session.

FDA Divisions Represented

- **Office of the Commissioner (OC) – 3 offices**
  - OC/OCPP/PAS - Office of Clinical Policy and Programs/Patient Affairs Staff (organizer)
  - OC/OCPP/OOPD - Office of Clinical Policy and Programs/Office of Orphan Product Development
  - OC/OCPP/OPT - Office of Clinical Policy and Programs/Office of Pediatric Therapeutics
- **Center for Biologics Evaluation and Research (CBER) – 4 offices**
  - CBER/OCBQ/DIS/PSB – Office of Compliance and Biologics Quality/Division of Inspections and Surveillance/Program Surveillance Branch
  - CBER/OCD – Office of the Center Director
  - CBER/OCD/PS – Office of the Center Director/Policy Staff
  - CBER/OTP/PSPS – Office of Therapeutic Products/Policy and Special Projects Staff
- **Center for Drug Evaluation and Research (CDER) – 9 offices**
  - CDER/OCD/PFDD – Office of the Center Director/Patient Focused Drug Development Staff
  - CDER/OMP – Office of Medical Policy
  - CDER/OND/ODES/DCOA – Office of New Drugs/Office of Drug Evaluation Science/Division of Clinical Outcome Assessment
  - CDER/OND/ON/DNII – Office of New Drugs/Office of Neuroscience/Division of Neurology II
  - CDER/OND/ORDPURM/DRDMG – Office of New Drugs/Office of Rare Diseases, Pediatrics, Urology and Reproductive Medicine/Division of Rare Diseases and Medical Genetics
  - CDER/OTS/OB/DBI – Office of Translational Science/Office of Biostatistics/Division of Biostatistics I
  - CDER/OTS/OCP – Office of Translational Science/Office of Clinical Pharmacology
  - CDER/OTS/OCP/DCEP – Office of Translational Science/Office of Clinical Pharmacology/Division of Cardiometabolic and Endocrine Pharmacology
  - CDER/OTS/OCP/DCPI – Office of Translational Science/Office of Clinical Pharmacology/Division of Cancer Pharmacology I
- **Center for Devices and Radiological Health (CDRH) – 6 offices**
  - CDRH/OCD – Office of Center Director
  - CDRH/OPEQ/OHTIII – Office of Product Evaluation and Quality/Office of Health Technology III
  - CDRH/OPEQ/OHTIII/DHTIIIB - Office of Product Evaluation and Quality/Office of Health Technology III/Division of Health Technology IIIB
  - CDRH/OPEQ/OHTIV/DHTIVB - Office of Product Evaluation and Quality/Office of Health Technology IV/Division of Health Technology IVB
  - CDRH/OPEQ/OHTV/DHTVB - Office of Product Evaluation and Quality/Office of Health Technology V/Division of Health Technology VB
  - CDRH/OSPTI/OEID/DPCD – Office of Strategic Partnership and Technological Innovation/Office of Equity and Innovative Development/Division of Patient Centered Development
- **Non-FDA Attendees**

- **Reagan Udall Foundation**
- **National Institutes of Health (NIH)**
  - NIH/NCATS – National Center for Advancing Translational Sciences

Disclaimer

Discussions in FDA Patient Listening Sessions are informal. All opinions, recommendations, and proposals are unofficial and nonbinding on FDA and all other participants. This report reflects the KCNT1 Epilepsy Foundation's account of the perspectives of patients and caregivers who participated in the Patient Listening Session with the FDA. To the extent possible, the terms used in this summary to describe specific manifestations of KCNT1-related epilepsy, health effects and impacts, and treatment experiences, reflect those of the participants. This report is not meant to be representative of the views and experiences of the entire KCNT1-related epilepsy patient population or any specific group of individuals or entities. There may be experiences that are not mentioned in this report.