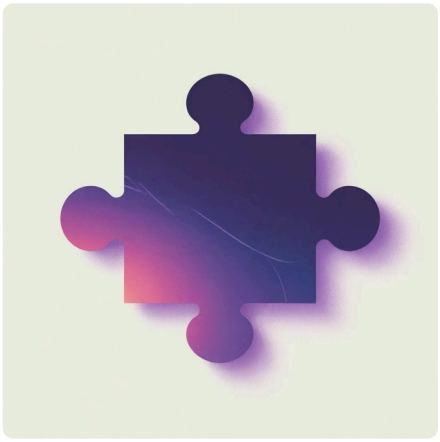

Clinical Trials: Part of the KCNT1 Puzzle

Understanding What to Expect — and Why Your Participation Matters

This guide was developed from themes discussed in a pre-recorded webinar with Dr. Brad Bryan, Seth Rotberg, Heather Bentley, and Dr. Amy Raymond

Clinical trials represent one of the most important pathways toward developing new treatments for KCNT1-related conditions. This guide, based on insights from the KCNT1 Foundation panel "Expectations and Goals of Clinical Trials," brings together expert perspectives from Dr. Brad Bryan, Seth Rotberg, Heather Bentley, and Dr. Amy Raymond to help families navigate this complex but crucial journey.

Whether you're considering participation for the first time or seeking to better understand the process, this resource will equip you with the knowledge and realistic expectations needed to make informed decisions about clinical trial participation. Every family's journey contributes to the larger puzzle of KCNT1 research, building toward better treatments and outcomes for future generations.

What Are Clinical Trials?

Clinical trials are carefully designed scientific studies that test whether new treatments are safe and effective for patients. These studies follow a structured progression through multiple phases, each serving as a critical building block toward regulatory approval and widespread availability of new therapies.

Preclinical Research

Laboratory studies conducted in cells or animal models to establish basic safety and biological activity before human testing begins.

Phase 1 Trials

Small group studies focused primarily on safety, determining appropriate dosing ranges and identifying side effects in humans.

Phase 2 Trials

Larger studies exploring whether the treatment actually works while continuing to monitor safety in a broader patient population.

Phase 3 Trials

Comprehensive studies confirming results in large groups, often comparing new treatments to current standard care options.

> "Families are the source of decision-quality data that determines whether a drug moves forward."
> — *Heather Bentley*

Your participation as a family provides the real-world data that regulatory agencies like the FDA use to determine whether a therapy can advance to the next phase or receive approval for broader use. Every participant contributes valuable information that shapes the future of treatment options for the entire KCNT1 community.

💬 What to Expect

Joining a clinical trial represents a significant decision that should never be rushed. The process involves multiple steps designed to ensure you fully understand what participation entails and can make an informed choice that's right for your family.

Informed Consent Process

You'll receive detailed documentation explaining the study's purpose, procedures, potential risks and benefits, and your rights as a participant. Take time to review thoroughly.

Question and Answer Sessions

Study teams encourage questions about expectations, time commitments, travel requirements, and any concerns you may have about participation.

Logistical Support

Learn about available support for travel, accommodations, reimbursement options, and care coordination throughout the study period.

Study Design Considerations

- Understanding placebo use and randomization
- Timeline for when active treatment begins
- Frequency and duration of study visits
- Required tests and procedures

Your Partnership Role

You're not just a subject in the research—you're a valued partner whose experiences and feedback contribute to the study's success. Following the study plan closely helps ensure data reliability and scientific validity.

> "No question is a bad question. You are the one making the sacrifice." — Seth Rotberg

⚖️ Balancing Hope and Reality

Participating in clinical trials naturally brings hope that the experimental treatment will help your child. While some participants do experience benefits, it's crucial to maintain realistic expectations about what trials can and cannot accomplish. Clinical trials are, by definition, experiments testing unproven treatments.

Maintain Realistic Hope

While hoping for positive outcomes is natural and healthy, remember that trials test experimental treatments with uncertain results. Success isn't guaranteed, but participation still contributes valuable knowledge.

Honest Reporting Matters

Accurate reporting of symptoms, side effects, and experiences—both positive and negative—helps researchers understand the treatment's true effects and guides future research directions.

Every Study Contributes

Even when trials don't lead to approved drugs, the data collected helps researchers understand what doesn't work and why, informing the design of better future studies.

The journey of drug development is filled with both successes and setbacks. Each study, regardless of its outcome, fills in another piece of the complex puzzle that is KCNT1 research. Your honest participation and realistic expectations help ensure that the scientific community learns as much as possible from every trial, ultimately benefiting future families facing similar challenges.

> "You are contributing data that could help many others, even if this study isn't the cure." — *Heather Bentley*

This perspective helps families approach trials with appropriate hope while understanding that their contribution extends far beyond their individual experience, potentially impacting the entire KCNT1 community for years to come.

Commitment and Communication

Clinical trial participation can significantly impact your family's daily routine, requiring careful consideration of the practical implications before enrollment. Understanding the full scope of commitment helps ensure successful participation and reduces the likelihood of early withdrawal, which can affect data quality.

Visit Frequency and Scheduling

Determine how often study visits are required, their duration, and flexibility in scheduling around work, school, and other family commitments.

Financial and Travel Support

Understand what expenses the study covers, including travel costs, hotel accommodations, meals, and any compensation for time and participation.

Care Coordination Services

Learn about available support staff who can help with logistics, appointment scheduling, and coordination between the study team and your regular healthcare providers.

Withdrawal Procedures

Understand the process and implications if you need to leave the study early, including any required follow-up visits or safety monitoring.

🧭 **Be Proactive:** Maintain open communication with study staff throughout the trial. Advocate for your family's needs and discuss any challenges early. The study team wants you to succeed and will work with you to address concerns whenever possible.

Successful trial participation requires thoughtful planning and ongoing communication. Before enrolling, have honest conversations with your family about the time commitment and potential disruptions. During the study, maintain regular dialogue with the research team about any challenges or changes in your circumstances. Remember that leaving a trial early can affect the quality and completeness of the data, so approach decisions collaboratively with the study team to find solutions that work for everyone.

After the Trial

The completion of your final study visit marks the beginning of an important behind-the-scenes process that transforms your participation into meaningful scientific knowledge. Understanding what happens after the trial helps set appropriate expectations for timelines and next steps.

1 Data Analysis and Cleaning

Sponsors carefully analyze and verify all collected data to ensure accuracy and completeness. This meticulous process can take several months but is essential for reliable results.

2 Open-Label Extension Phase

Many studies include an extension phase where all participants can access the active drug while long-term safety and effectiveness data continues to be collected.

3 Results and Publication

Study results may take months to be released due to the thorough analysis required. This "quiet period" is normal and necessary for scientific integrity.

4 Post-Trial Access Planning

If the treatment shows promise, discuss options for continued access early in the process, as availability may be limited initially.

The period following trial completion can feel uncertain, especially during the data analysis phase when results aren't yet available. This waiting period, while challenging for families eager for answers, is crucial for ensuring that the scientific conclusions drawn from your participation are accurate and reliable.

Your data contributes to researchers' understanding of safety profiles, optimal dosing strategies, patient selection criteria, and the overall therapeutic potential of the experimental treatment. This comprehensive analysis helps determine whether the treatment should advance to the next phase of development or regulatory review.

> "Behind the scenes, your data helps researchers understand safety, dosing, and next steps." — Dr. Amy Raymond

Putting the Pieces Together

Every family's participation in clinical trials contributes to the larger mosaic of KCNT1 research, building toward a future with better treatment options and improved outcomes. Even when individual study outcomes remain uncertain, the collective effort of all participating families creates a foundation of knowledge that drives scientific progress forward.

Building Knowledge

Each study adds crucial pieces to our understanding of KCNT1 conditions and potential treatments.

Community Impact

Your contribution extends beyond your family to benefit the entire KCNT1 community worldwide.

Patient Stories

Every participant's experience provides valuable real-world data that shapes future research directions.

Scientific Progress

Collective datasets help researchers understand safety, efficacy, and optimal treatment approaches.

Future Hope

Today's courage and participation unlock possibilities for tomorrow's breakthrough treatments.

Participation today builds tomorrow's answers

The path of clinical trial participation requires courage, commitment, and hope balanced with realistic expectations. While not every trial will result in an approved treatment, each study contributes essential knowledge that brings the entire KCNT1 community closer to better therapeutic options. Your willingness to participate in this journey of discovery is personal and can represent an investment not just in your own family's future, but in the hope and possibilities for all families affected by KCNT1-related conditions.

The puzzle of KCNT1 research is complex, with many pieces still to be discovered and assembled. But with each family's participation, each dataset collected, and each lesson learned, we move closer to completing the picture that will ultimately lead to more effective treatments and improved quality of life for those living with these challenging conditions.

KCNT1 EPILEPSY
HOPE IS ON THE HORIZON