
Research Focus Areas:

The **KCNT1 Epilepsy Foundation** is seeking proposals for a **\$70,619 award** for a translational research project focused on the development of therapies for KCNT1 disorders. The goal of this grant is to support research that can lead to the development of effective treatments for KCNT1 disorders. The project should focus on one or more of the following areas:

- Define the non-conductance functions of KCNT1 as a way to further disease understanding and find alternative treatment targets. Proposals should emphasize therapeutic potential.
- Understand cellular mechanisms, splice variants, and gene modifiers that potentially influence KCNT1 and could serve as a potential therapeutic target. Proposals should emphasize therapeutic potential.
- Preclinical testing of FDA-approved drugs previously identified in repurposing screens: This may include in vitro and/or in vivo work, to investigate efficacy for multiple patient mutations. The proposal should state why additional preclinical testing is necessary before patients are treated.
- Organization and execution of clinical trials for drugs already identified in repurposing screens: The proposal should include details of the clinical trials design, recruitment strategy, inclusion and exclusion criteria, primary and secondary outcomes, and statistical analysis plan.
- Validation of assessment tools in KCNT1 patients for use in clinical trial outcome measures, especially non-seizure outcomes: The proposal should include details of the assessment tools to be validated, the validation process, and the expected outcomes.
- Investigation of symptoms/pathophysiology outside of the brain, such as the role of KCNT1 in the lungs or cardiac symptoms. Proposals may include clinical studies or translational lab research.
- Development of gene therapies: The proposal should include details of the gene therapy approach, preclinical data, and the proposed plan for clinical translation.

Applicants are encouraged to collaborate with existing KCNT1 researchers and to leverage existing disease models and data (e.g., animal models, Ciitizen data, registry data, biobank, cell lines, etc.) and should include a statement on resource sharing in their proposal. This grant is made possible by the KCNT1 Epilepsy Foundation, its generous supporters, and the Orphan Disease Center.