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## CDKL5 Program of Excellence 2022 Pilot Grant Program

**Project Title:** “Hematopoietic stem cell gene therapy as a therapeutic approach for CDKL5 Deficiency Disorder”

**PI:** Joseph Anderson, PhD

**Institution:** University of California, Davis

In our current proposal, we will be evaluating a stem cell gene therapy approach for CDKL5 deficiency disorder (CDD). We will be genetically modifying blood forming stem cells with a functional copy of CDKL5 and evaluating these cells in a CDD-specific mouse model. Upon transplantation, mice will be evaluated for engraftment of the gene modified cells. Successfully engraftment mice will then be subjected to behavior and motor tests to evaluate if the therapeutic cells have rescued any of the CDD-related phenotypes. If successful, this therapeutic approach for CDD will be further evaluated to progress it toward a Phase I human clinical trial.