

## **CDKL5 Program of Excellence Pilot Grant Program**

**Application Title:** Development of a High-content drug repurposing screen using CDKL5 patient derived iPSC neurons

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Despite substantial recent progress in many rare diseases, there has been an almost complete lack of progress in understanding of how mutations in CDKL5 affect the mammalian brain or individual brain cells. Using the latest techniques and methods this project aims to rectify these gaps in our knowledge. In particular, our project will determine the precise changes in thousands of genes that occur in brain cells (neurons) and the brain itself as a consequence of CDKL5 mutation. We will then use this information to determine how these changes affect the behavior and activity of brain cells. These results will provide a foundation for discovering new drugs for CDKL5. As a first step along this road, we will apply computer algorithms developed by us to see if we can computationally predict drugs that may be capable of restoring specific functions to CDKL5 deficient neurons.