CDKL5 Program of Excellence
2018 Pilot Grant Program

Project Title: "Quantitative SRM assays for optimization of CDKL5 protein replacement therapy"

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CDKL5 (cyclin-dependent kinase-like 5) deficiency disorder (CDD) is a nervous system disorder characterized by severe intellectual, movement, and developmental defects during infancy caused by a defect in the gene CDKL5. One potential therapeutic strategy would be protein replacement therapy, re-introducing CDKL5 protein or an essential domain to the nervous system to help restore its function. To advance this strategy, we will use a powerful, cutting-edge technology to provide the tools for detecting the delivered CDKL5 protein and downstream targets of CDKL5, and will determine how well this rescue strategy works on human neural progenitor cells grown in a dish. The tools developed will be valuable to the CDKL5 research community and the rescue strategy, if successful, can subsequently be tested in an animal model and, in the future, in humans.