CDKL5 Program of Excellence
Pilot Grant Program

Application Title: Precision Medicine Program for CDKL5 Disorders: Therapeutic Screening Using Human iPSC Models

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Institution: Massachusetts General Hospital

We will utilize patient-derived cellular models to develop scalable small molecule assays for therapeutic discovery in CDKL5. Specifically, we will characterize neural progenitor cells, differentiated neurons, and other neural cells derived from induced pluripotent stem cells in terms of expression, metabolism, and morphology. We will then attempt to rescue these phenotypes with small molecules.