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## CDKL5 Program of Excellence 2021 Pilot Grant Program

**Project Title:** “ASO therapy in CDKL5 deficiency disorder”

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**Institution:** Boston Children’s Hospital

Pathogenic variants in the cyclin-dependent kinase like 5 (*CDKL5*) gene cause CDKL5 deficiency disorder (CDD), a severe developmental and epileptic disorder for which no effective treatments are available. We are developing ASO-switchable cellular and mouse models that will allow us to probe CDD neurobiology, define therapeutic windows for reversal of functional biomarkers, and begin to assess the feasibility of antisense oligonucleotide (ASO) treatments for certain CDKL5 mutations which cause CDD. Our application is in response to RFA of developing “Novel therapeutic approaches for CDD”.