

TITLE: Therapy of MPS IIIB with stem cells and gene therapy

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RESEARCH PROJECT DESCRIPTION (brief overview of background, hypothesis, methods, role of medical student, funding and relevant publications -- SHOULD NOT EXCEED ~ 250 WORDS)

We have several projects available testing timing of genetic correction and using gene therapy with AAV or stem cell mediated gene therapy with lentivirus correction of neural and mesenchymal stem cells. MPS IIIB is a lysosomal storage disease also known as Sanfilippo Syndrome which results in progressive neurodegeneration. We assess delivery of the therapy and correction of disease as assessed by circadian studies, behavioral tests and biochemical assays.