METHODS FOR THE TREATMENT OF LYSOSOMAL STORAGE DISORDERS

ABSTRACT

Provided herein are methods for the treatment of lysosomal storage disorders characterized by a missing or defective secreted lysosomal enzyme. Such lysosomal storage disorders include, but are not limited to neuronal ceroid lipofuscinoses. The disclosed methods involve the transplantation of human multipotent neural stem cells into the CNS of patients suffering from the lysosomal storage disorder. Also provided herein are methods of reversing or slowing the progression of neurodegeneration in patients suffering from or at risk of developing neuronal ceroid lipofuscinoses.

Figure 1