Other Experimental Therapies in Neimann Pick Diseases

How to Cite This Article: Taghdiri MM. Other Experimental Therapies in Neimann Pick Diseases. Iran J Child Neurol. 2015 Autumn;9:4(Suppl.1): 25.

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Liver or bone marrow transplantation has been unsuccessful in the treatment of NPA and NPC.

Enzyme replacement therapy is being developed for patients with NPB who have no neurologic symptoms.

No evidence indicates that climethyl sulfazixe or cholesterol.

Lowering agents improve neurologic symptoms in NPC.

Reliable parental diagnosis uses Molecular investigations on chrionic DNA when familial mutations have been clearly defined.

No specific treatment is available for NPA.

Cataplexy could be improved by clomipramine or modafinal.

The following reports illustraye yhe range of experimental therapied for NDP:

- Hematopoietic stem cell transplantation(HSCT) did not modify the neurological course.
- Retroviral mediated transfer using acid sphingomyelinase (ASM) cDNa in cultured fibroblasts of affected patients resulted in up to 2 16 fold increase in ASM activity.
- Direct intracerebral transplant of neural progenitor cells into the mouse model of NDP-A.
- Treatment with tamoxifen in combination with vitamin E, Did not have significant effect in the mouse model of NPD-C.
- Another approach is based on the observation that neursteroids, made in the central nervous system.
- Neurosteroidogenesis and treatment with allopreganolane in the NPD-C mouse delayed neurological symptoms.
- Bone marrow-derives mesenchymal stem cells in NPC-C mice suppressed neurogical inflammation and resduced cerebellar pathology.

Keywords: Neimann-Pick disease; Bone marrow transplantation; Enzyme replacement.

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