



Gene therapy for treating sialidosis

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Experience: Graduated from Department of Medicine, NTU, and obtained Ph.D. degree from Institute of Molecular Medicine, NTU. Trained as a Pediatrician in NTU Hospital, and has been Visiting Staff, Deputy Head of Department of Pediatrics, and Head of Medical Genetics.

Market Needs: Sialidosis is a rare autosomal recessive inborn error of metabolism, and currently there is no effective treatment for this disease. Patients usually have symptoms including myoclonic seizure, unsteady gait, and loss of vision. Developing a new therapy for sialidosis is an urgent need.

Our Technology: We created a mouse model for sialidosis, and we have constructed a viral vector. We first proved the function of this vector in cultured cells. We then injected this vector into sialidosis mice, and we demonstrated that their brain pathologies decreased after treatment, and the motor functions of the treated mice were also improved.

Strength: We are the first in the world to develop a treatment for sialidosis mice.

Competing Products: There is no other similar product in the world.

Intellectual Properties:

- (1) Our team has successfully developed a gene therapy for human disease.
- (2) There has been no patient published about the treatment of sialidosis

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