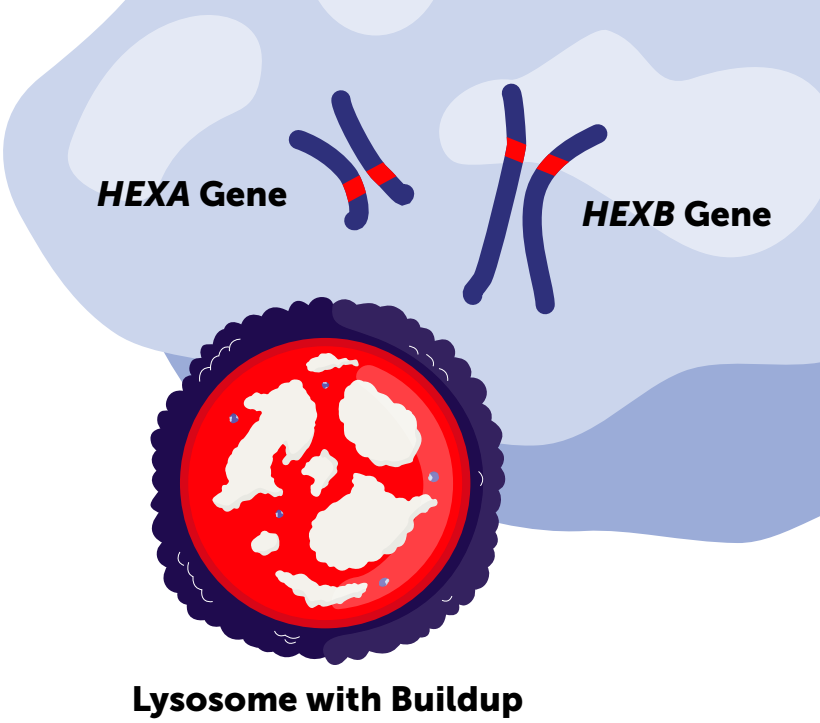
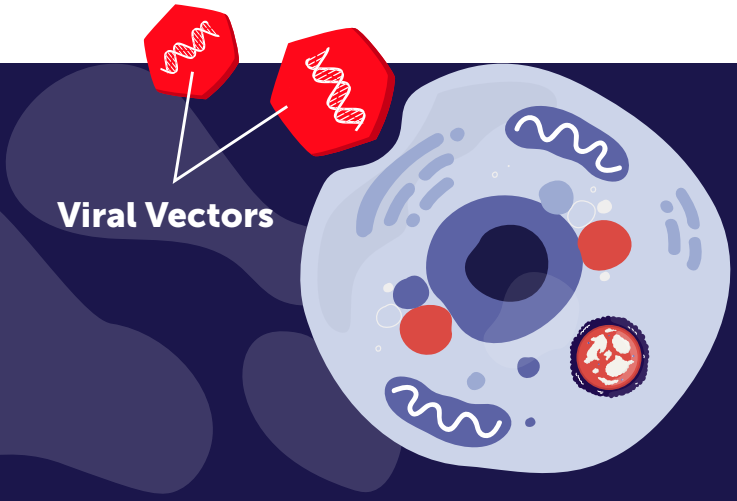
Gene Therapy for GM2-Gangliosidosis



Lysosome with Buildup

The *HEXA* and *HEXB* Genes

There are two main types of GM2-Gangliosidosis (GM2). Tay-Sachs disease is caused by a mutation to the *HEXA* gene, and Sandhoff disease is caused by a mutation to the *HEXB* gene. Although they are different genes, they both instruct the cells to produce parts of an important enzyme called hexosaminidase (HexA). This enzyme helps lysosomes break down and remove waste, allowing cells to function properly.



How Gene Therapy Can Help

Gene therapy aims to be a one-time treatment that delivers a working *HEXA* and *HEXB* gene into cells. Viral vectors are used to deliver the working genes at the same time through a single injection. The working genes can then instruct cells to make the lacking HexA enzyme to restore function in the lysosomes and remove harmful buildup.

The Role of Viral Vectors

Scientists know that viruses are good at getting into cells, so they have learned how to safely use this ability to get into cells as a vector—or carrier—to deliver the gene. But don't worry, the viral genes are removed so that only therapeutic genes are delivered. This is done using an *in vivo* approach, meaning the treatment is injected directly into the brain or spinal cord.

Clinical Trials

This investigative therapy to treat Tay-Sachs and Sandhoffs disease is currently being researched in clinical trials. Clinical trials are a required part of the research process to determine if a treatment is safe and effective. Being able to participate in a trial depends on strict criteria such as the person's age and health history.

American Society of Gene + Cell Therapy

Visit patienteducation.asgct.org for more information