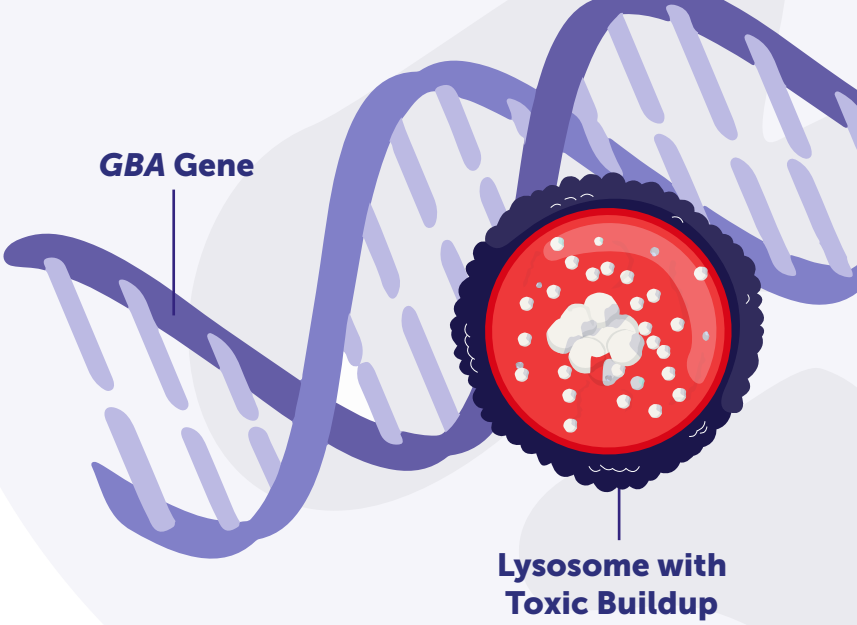
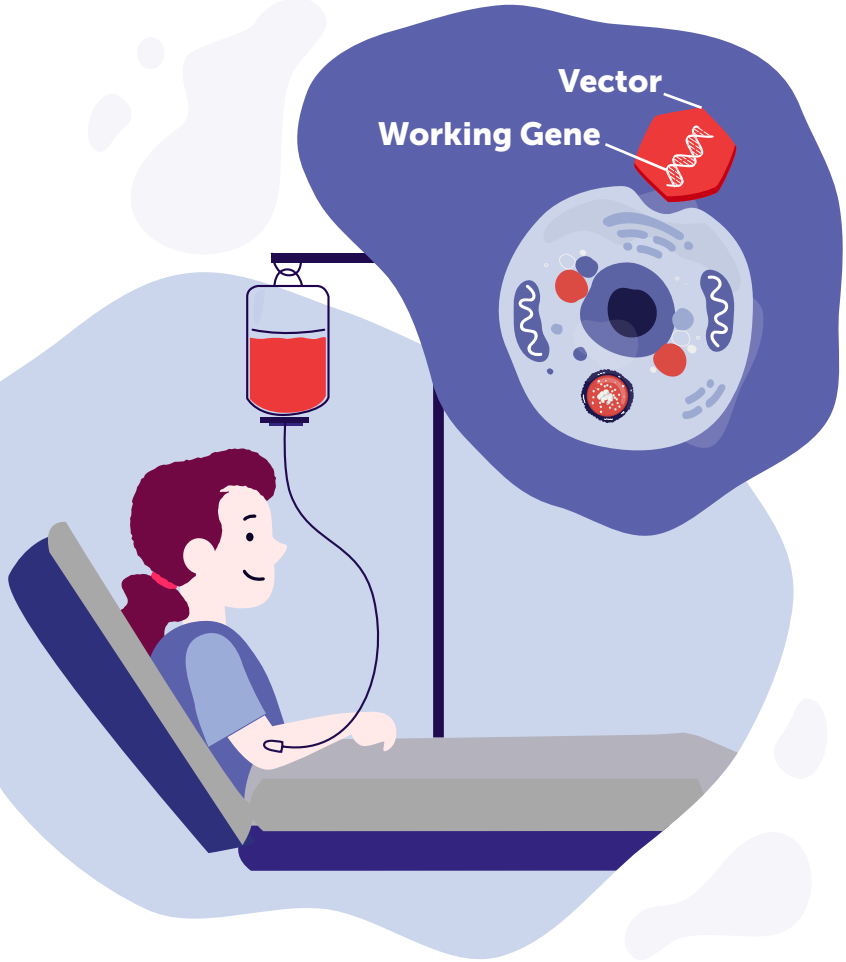
Gene Therapy for Gaucher Disease



The *GBA* Gene

Gaucher disease is caused by a mutation or change to the *GBA* gene. This gene instructs cells to produce an enzyme called beta-glucocerebrosidase (GCase), which helps break down sugars and fats called glycolipids within the lysosomes of cells. Due to the faulty *GBA* gene, there is not enough of the GCase enzyme, causing waste to build up in cells and tissues and become toxic.



How Gene Therapy Can Help

Gene therapy aims to be a one-time treatment that delivers a working *GBA* gene into cells. A viral vector is used to deliver the working gene that can instruct cells to produce the GCase enzyme to break down the toxic buildup of wastes in the lysosomes and prevent further build up.

Delivering the Viral Vector

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 only therapeutic genes are delivered.

In Vivo or *Ex Vivo*

Gene therapy can be delivered using an *in vivo* approach, meaning the vector carrying the working gene is given directly into the body via injection. Another approach is *ex vivo* where a person's hematopoietic stem cells (HSCs) are removed from the blood and then modified in a specialized lab. A process called conditioning then clears space for these modified cells to be returned to the body through an IV infusion.

The Role of Clinical Trials

This investigative therapy 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 criteria such as the type of Gaucher disease, and the person's age and health. At this time, people who have received one type of gene therapy approach will not be able to receive the other, so careful consideration must be given to which clinical trial is right for an individual.

American Society of Gene + Cell Therapy

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