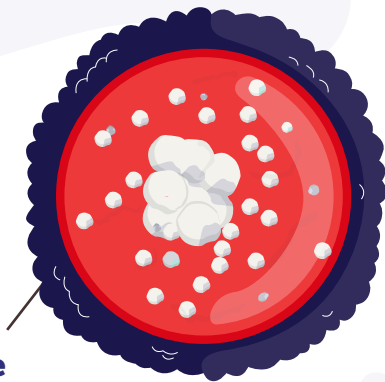


Gene Therapy for GM1 Gangliosidosis

The *GLB1* Gene and GM1

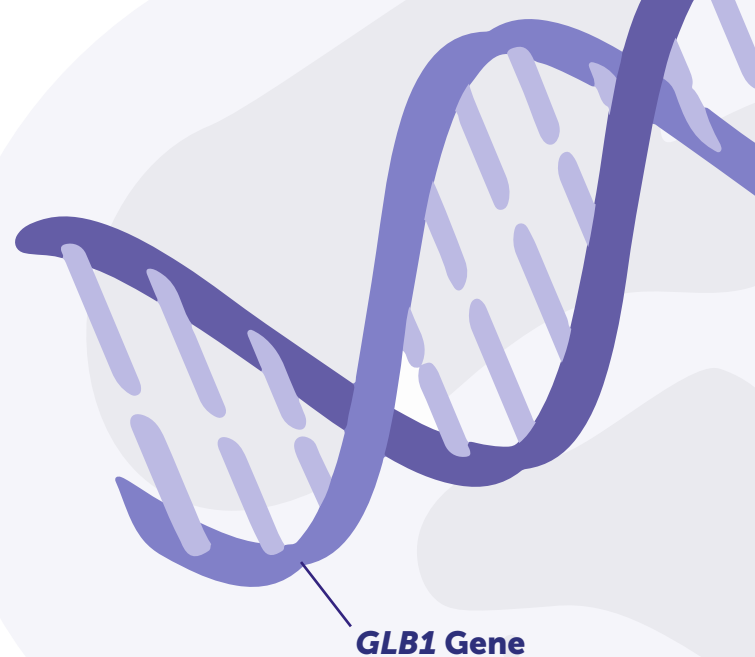
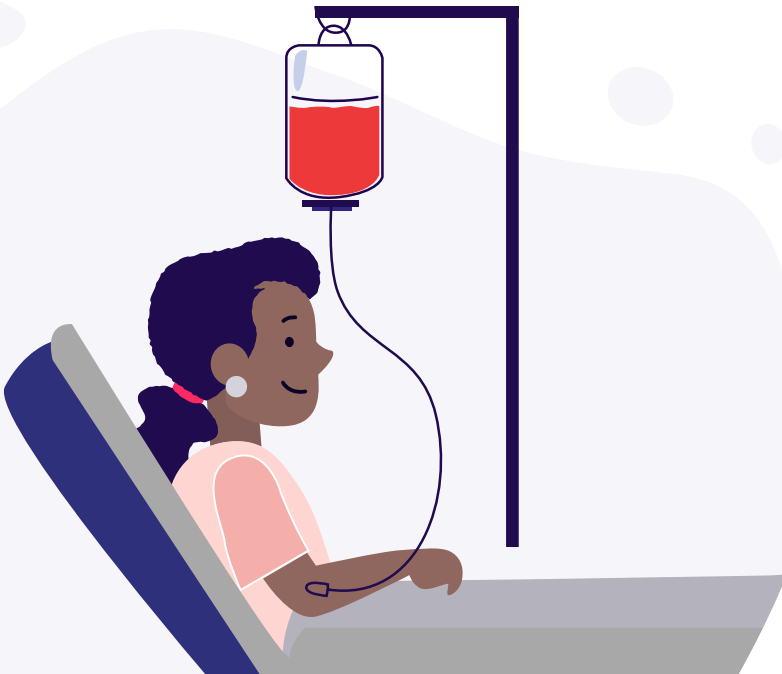
All forms of GM1 are caused by a mutation or change to the *GLB1* gene. This gene instructs cells to produce an enzyme called beta-galactosidase-1 (β -gal), which helps break down fats and sugars in the lysosomes within cells. Due to the faulty *GLB1* gene there is not enough of the β -gal enzyme causing waste to build up in cells and tissues which becomes toxic.



Lysosome

Delivering the Vector

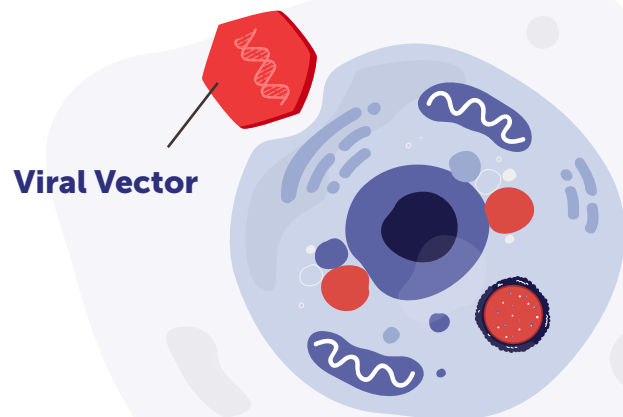
Scientists know that viruses are good at getting into cells, so they have learned how to safely use this ability to get into cells as a vector—or carrier—to deliver the gene. But don't worry, the viral genes are removed so that only therapeutic genes are delivered.



GLB1 Gene

How Gene Therapy Can Help

Gene therapy aims to be a one-time treatment that delivers a working *GLB1* gene into the body. There are currently two routes that gene therapy for GM1 can be given—intracisternal, meaning injected at the base of the skull—and intravenous, which is into a vein. A vector is used to deliver the working gene that can instruct cells to produce the β -gal enzyme. The goal is to restore how lysosomes function to remove toxic materials and prevent their build up.



Viral Vector

The Role of Clinical Trials

This investigative therapy to treat forms of GM1 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. People need to meet certain eligibility criteria to participate in clinical trials, which can include age of symptom onset and health history.