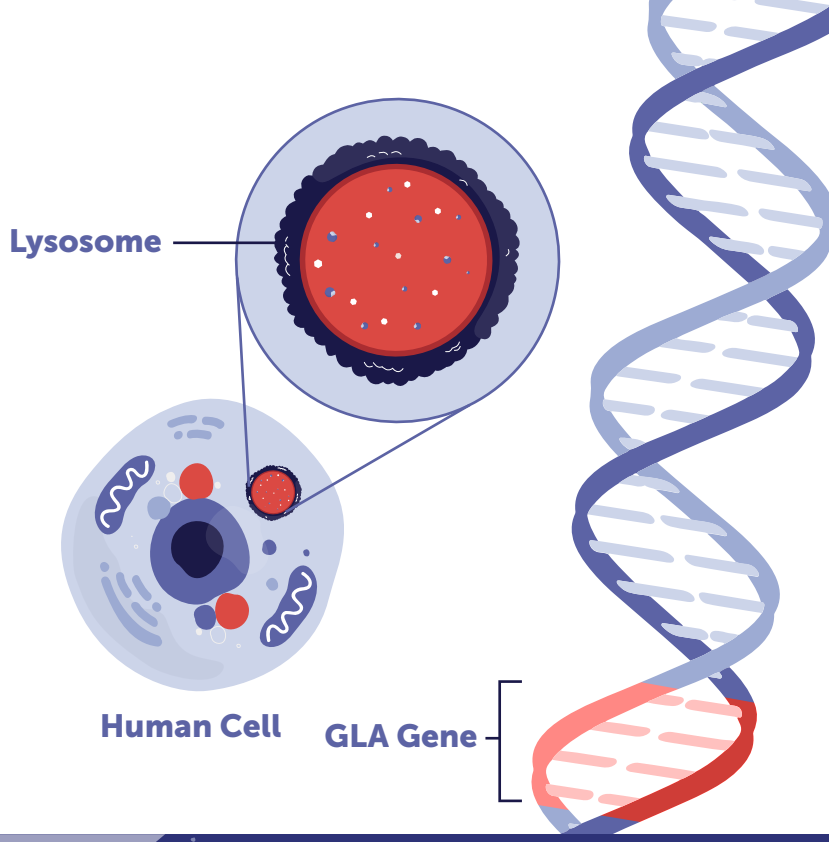
Fabry Disease and Gene Therapy

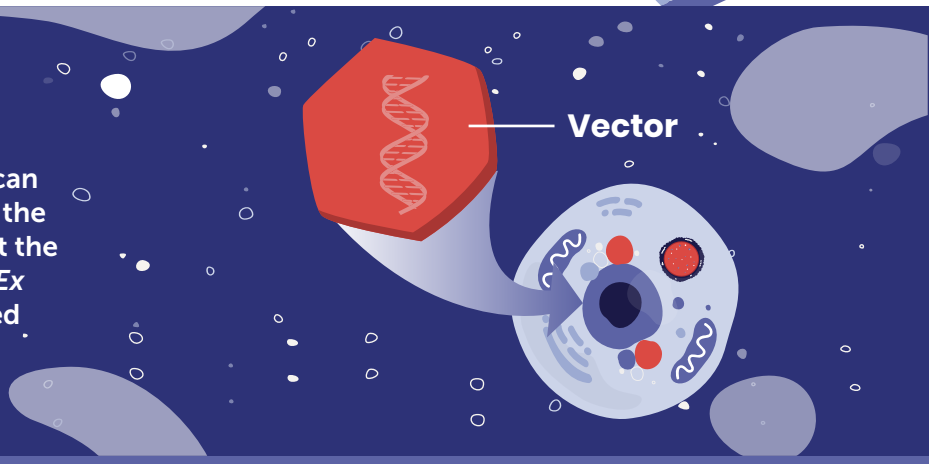


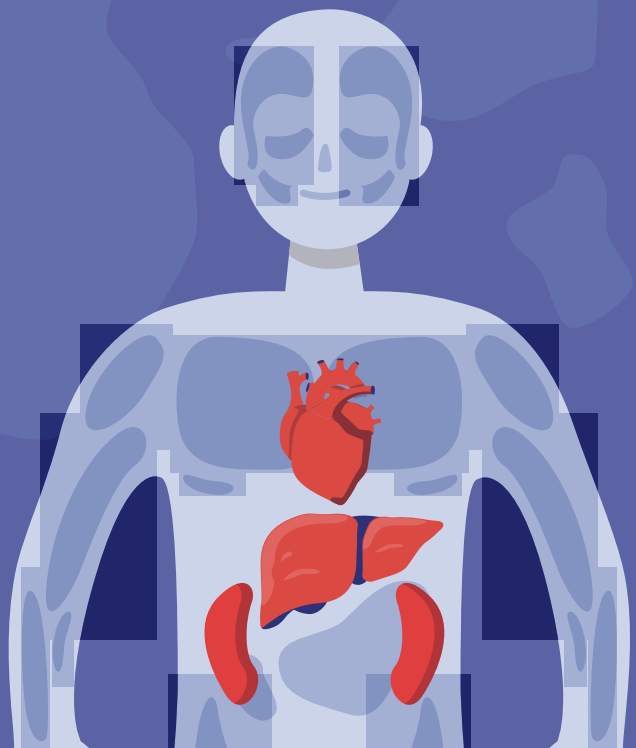
Lysosomes and the GLA Gene

Fabry disease is caused by a faulty mutation to the galactosidase alpha (GLA) gene. This gene typically instructs cells to create an enzyme called alpha-galactosidase A (α-gal A) which helps lysosomes break down a fat called globotriaosylceramide (Gb3). Lysosomes are referred to as the "recycling center" of our cells since they break down waste like sugars and fats. With a faulty gene, the cells don't produce enough of the α-Gal A enzyme— resulting in a build up of Gb3 in the lysosomes and progressive damage to the body.

Ex Vivo and *In Vivo*

In vivo and *ex vivo* gene therapy approaches can be used to deliver the working GLA gene into the cells with new instructions. *In vivo* means that the treatment is delivered directly into the body. *Ex vivo* means the person's own cells are modified outside the body, and then returned.





Gene Therapy Delivers Vector

A vector is used to deliver the working gene to the target cells. A vector, which is derived from a virus, is designed to get inside cells but the viral genes are removed and only therapeutic genes are delivered.

Vectors Target

Depending on the gene therapy approach for Fabry disease, the vectors deliver the working gene to the cells of the liver or heart to secrete the enzyme into the bloodstream for delivery to other organs, such as the kidneys. The new working gene instructs cells to make the α-Gal A enzyme and restore lysosomal function to slow or stop Fabry disease.

Visit patienteducation.asgct.org for more information

American Society of Gene + Cell Therapy