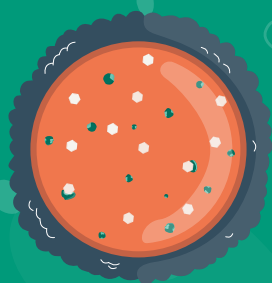
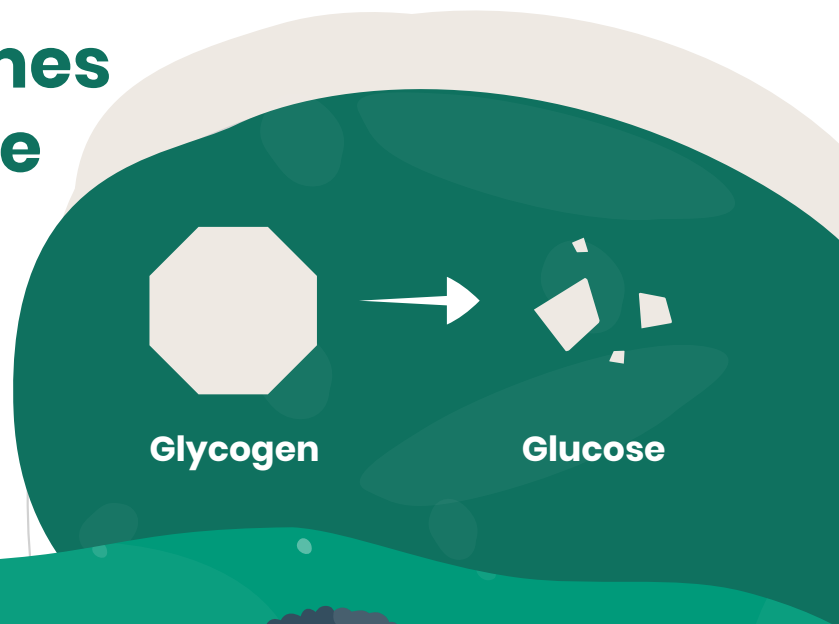


# Glycogen, Lysosomes and Pompe Disease

## The Role of Glycogen

Glycogen is a complex sugar that allows our bodies to store energy. An enzyme called acid alpha-glucosidase (GAA) helps break down glycogen into a simpler sugar called glucose. For many cells, glucose serves as the main energy source.



## A Look at Lysosomes

Lysosomes are small components of cells that help break down excess or used materials. This recycling process helps cells continue to function properly. Lysosomes contain a variety of digestive enzymes, including GAA.



## Effect of Pompe Disease

Pompe disease is caused by a faulty GAA gene, which provides the instructions for cells to produce the GAA enzyme. Without enough of the important digestive enzyme, glycogen builds up to toxic levels in lysosomes and damages organs and tissues.

## How Gene Therapy Can Help

Gene therapy can deliver working genes into the cells in charge of producing digestive enzymes. This is done using a vector, which is often derived from viruses, but modified so all viral genes are removed. The new genes can instruct cells to produce more enzymes, which then allow lysosomes to break down glycogen. This aims to be a one-time procedure that slows and controls the progression of Pompe disease.

