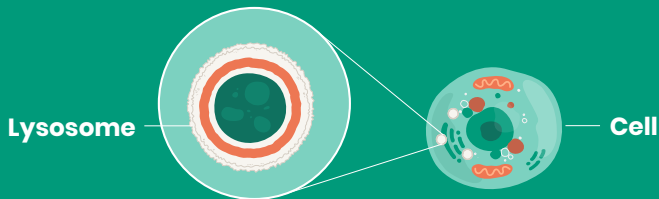


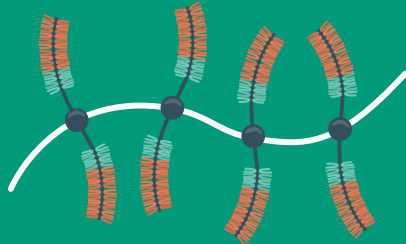
MPS: A Look at Lysosomes

What is a Lysosome?



Often referred to as the “recycling center” of our cells, the lysosome is the component that contains digestive enzymes. Lysosomes help break down excess or used materials, allowing cells to function properly.

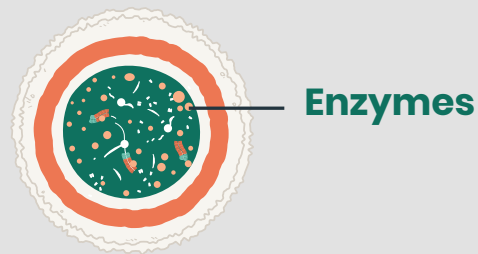
Lysosomes and GAGs



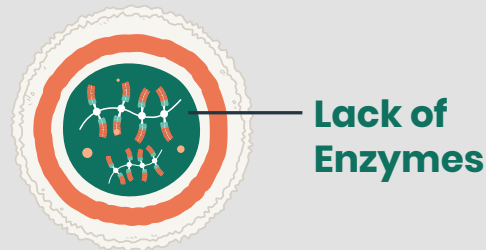
Our body uses sugar molecules called glycosaminoglycans (GAGs) to bind proteins, strengthening tissues like our skin and bones. Once they’ve served their purpose, GAGs are broken down in the lysosomes.

How MPS affects Lysosomes

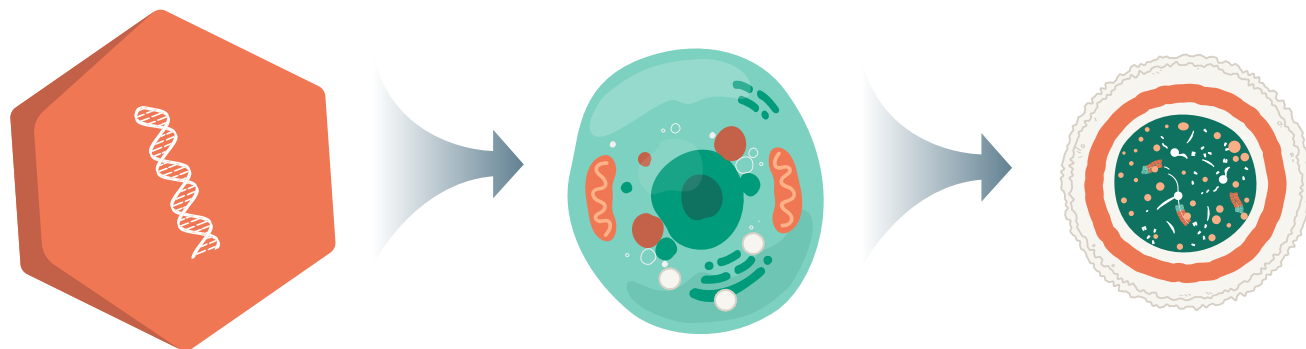
The mucopolysaccharidoses (MPS) are genetic disorders that affect how cells create the enzymes needed to break down GAGs. Without the right enzymes, GAGs accumulate in the lysosomes and cause progressive damage to the body.



Without MPS



With MPS



How Gene Therapy Can Help

Gene therapy delivers working genes into the cells in charge of producing digestive enzymes. This is done using a vector, which is often derived from viruses with all viral genes removed.

A One-Time Treatment

The new genes instruct cells to produce more enzymes, which then allow lysosomes to break down GAGs. Gene therapy for some types of MPS aims to be a one-time procedure that will slow or halt the progression of the disease.