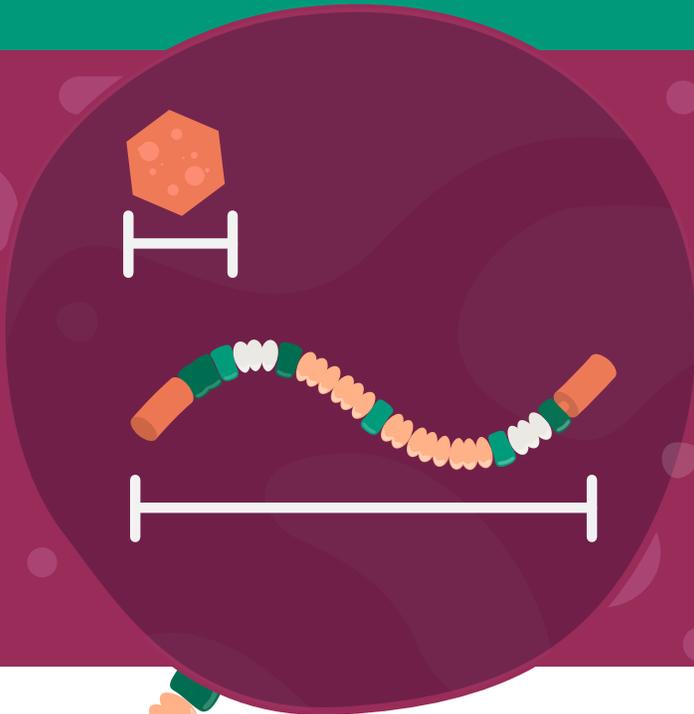
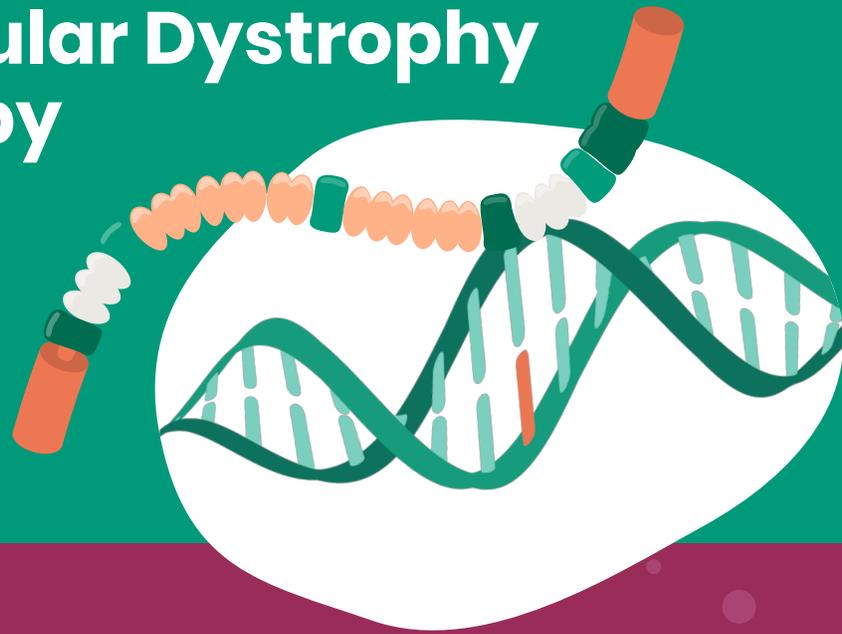


# Duchenne Muscular Dystrophy and Gene Therapy

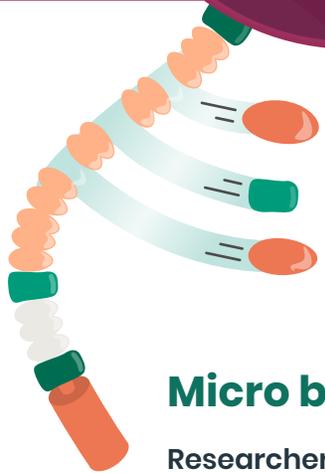
## The Role of Dystrophin

Dystrophin is a protein that strengthens our muscle fibers. In the case of Duchenne muscular dystrophy, a faulty gene affects the production of dystrophin, causing an absence of the important protein.



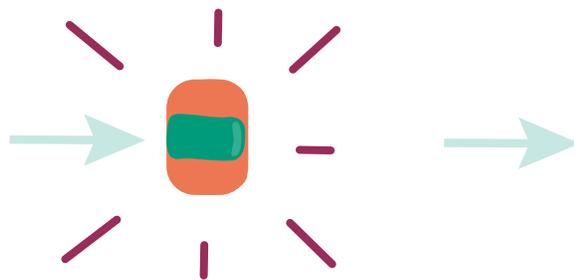
## Vectors

Genes come in a variety of sizes, and the gene that instructs cells to create dystrophin is actually one of the largest known genes in our genome. Gene therapy would deliver functional genes into the cells in charge of creating dystrophin. This would be done using vectors, which are derived from viruses with all viral material removed. However, the dystrophin gene is too big to fit inside the vectors typically used in gene therapy.



## Micro but Mighty

Researchers have developed a smaller version of the dystrophin gene called micro (or mini)-dystrophin. It contains the minimum amount of genetic code needed to instruct a cell to create the dystrophin protein correctly.



## Micro-Dystrophin Delivery

The smaller version of the dystrophin gene can fit in the vectors, which are then delivered to the muscle tissue cells. The goal is to increase dystrophin production to improve muscle strength and integrity.