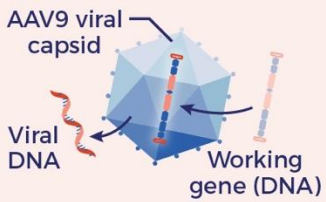
1

You do not have enough chairs for your table, so you order instructions to make them.

There is not enough protein in heart cells for typical heart function. Gene therapy is designed to provide instructions for the heart to make the protein it needs.

2

Instructions for making chairs are packaged in a box.

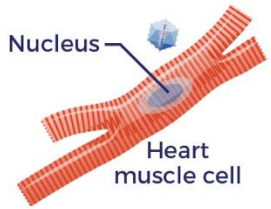


A working gene (DNA) is placed inside a capsid (vector).

A **capsid** (sometimes called a vector) is the shell of the AAV9 virus with all the viral genes removed. It is the package that delivers a working gene (DNA instructions) into cells.

3

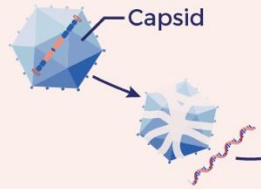
The box is delivered to your house.



Gene therapy delivers capsids to heart muscle cells.

4

You bring the box inside, open it, and take out the instructions.

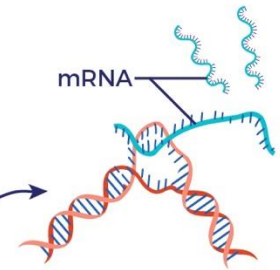


The capsids open and the working gene (DNA) is released.

Transduction: The process by which a working gene (DNA) is put into a cell using a viral vector (capsid).

5

You copy the instructions so your friends can help you.

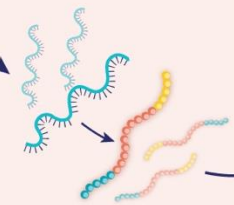


The cell copies the working gene (DNA) to make mRNA.

mRNA expression: The process by which cells copy the working gene to make mRNA.

6

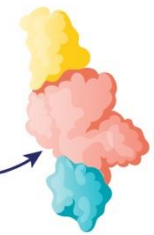
You and your friends read the instructions and put the chairs together.



The cell reads the mRNA and makes proteins.

7

You make the chairs correctly.



The proteins are made as expected.

8

You make enough chairs to go around the table.

The process repeats to make the proteins needed for cells to work as expected.

Protein expression: The process by which cells make protein from instructions in the mRNA.