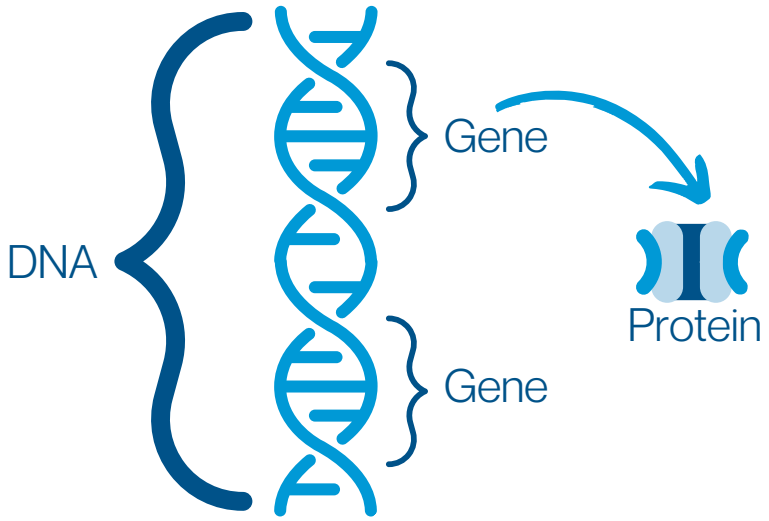
OVERVIEW OF GENE REGULATION THERAPY

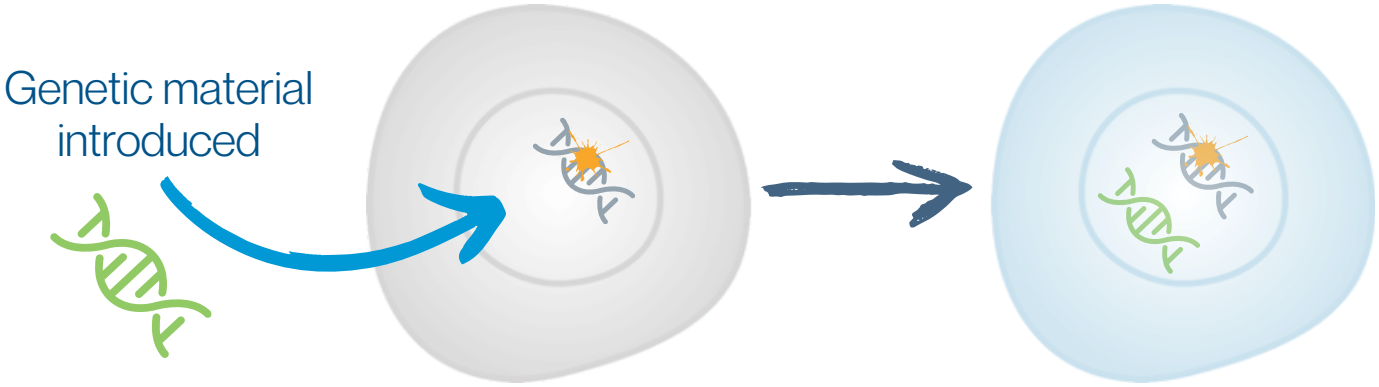
What is Gene Therapy?

Genes are sections of a person's DNA that contain the codes for proteins and enzymes that control how the body looks and functions.

Genes that don't work properly may cause medical conditions.



Gene therapy is the use of genetic material in the treatment or prevention of a condition.



Representative example of a gene therapy approach

What types of gene therapies are there?

There are a number of gene therapy approaches being studied, including:

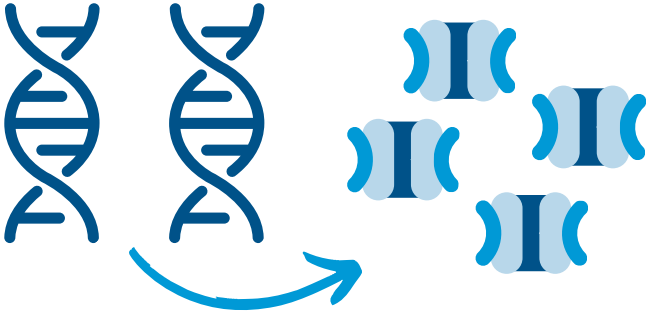
- Gene replacement / addition
- Gene editing
- Gene silencing
- Gene regulation

This booklet provides an overview of **gene regulation therapy**.

Gene regulation therapy is the use of genetic material to influence the activity of an existing gene with the aim of preventing, stopping, or slowing the effects of a condition.

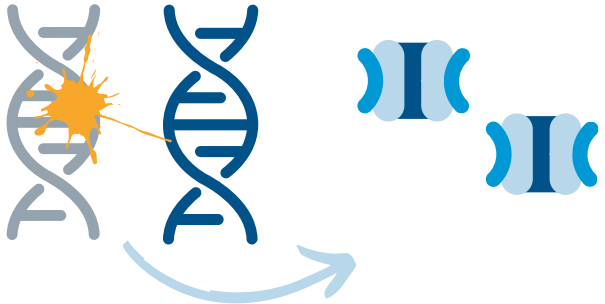
How does gene regulation therapy work?

Every person has 2 copies of a specific gene.

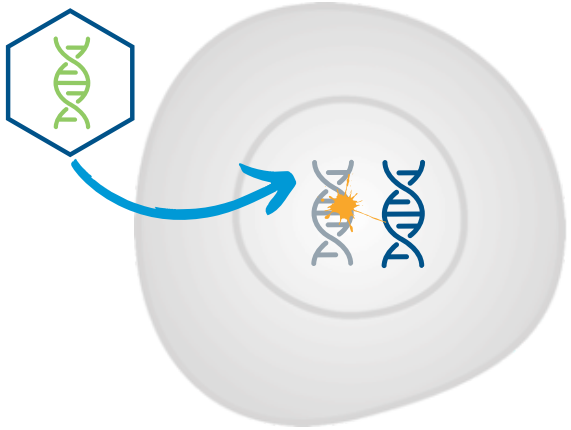


Genes code for proteins that allow the body to function properly.

When 1 of the 2 copies of a specific gene is faulty, there may be a reduction in the amount of protein produced.

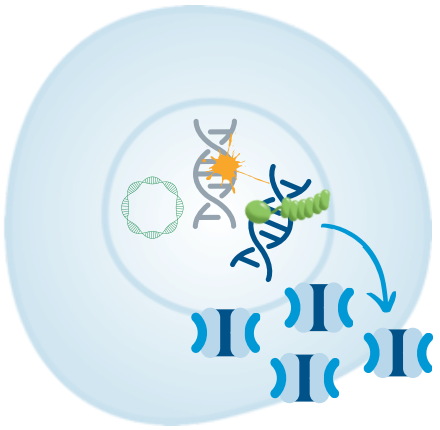


1. Potentially therapeutic genetic material is delivered to a cell's nucleus.



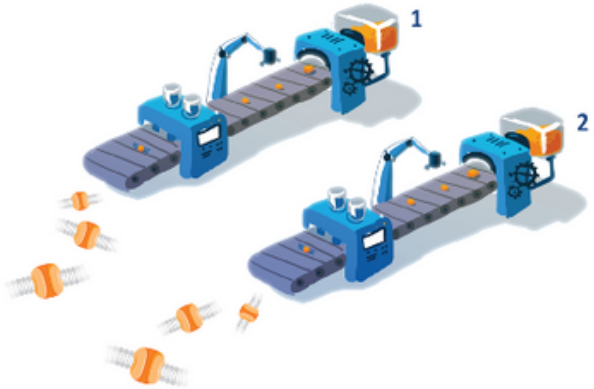
2. This genetic material lives outside the cell's own DNA where it produces a boosting element for the gene of interest.

3. This boosting element tells the working copy of the gene to make more protein.

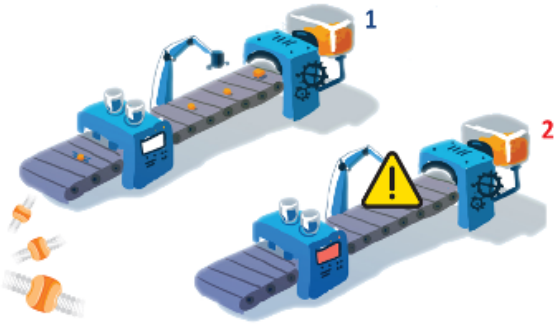


Gene regulation therapy analogy

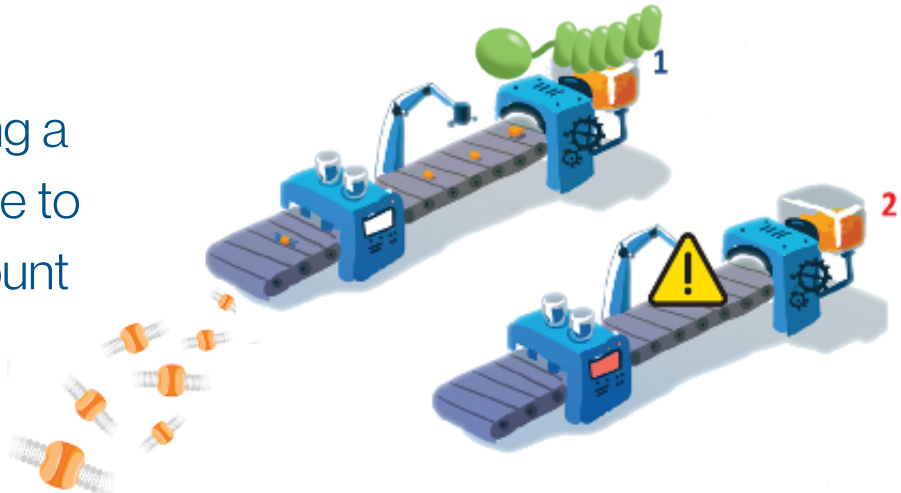
Imagine 2 assembly lines representing the 2 copies of the gene of interest, working together to make a product.



If 1 assembly line does not work, there is a reduction in the amount of that product made.



Gene regulation therapy is like placing a new part on the working assembly line to boots its output and restore the amount of product made.



This educational booklet was developed by Encoded Therapeutics.

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