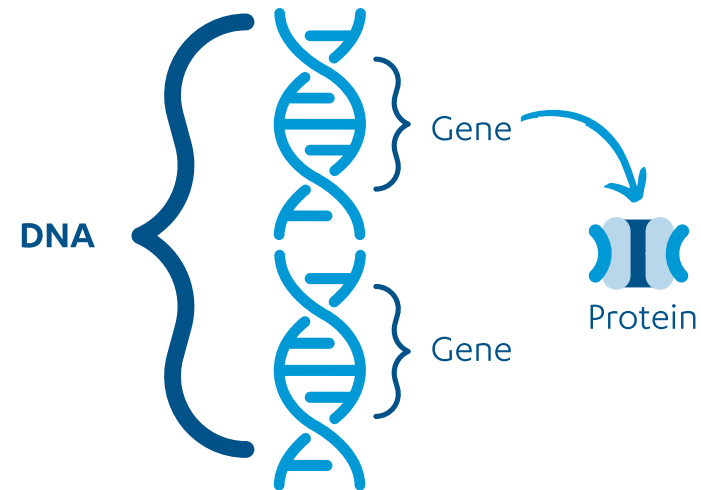


OVERVIEW OF GENE REGULATION THERAPY

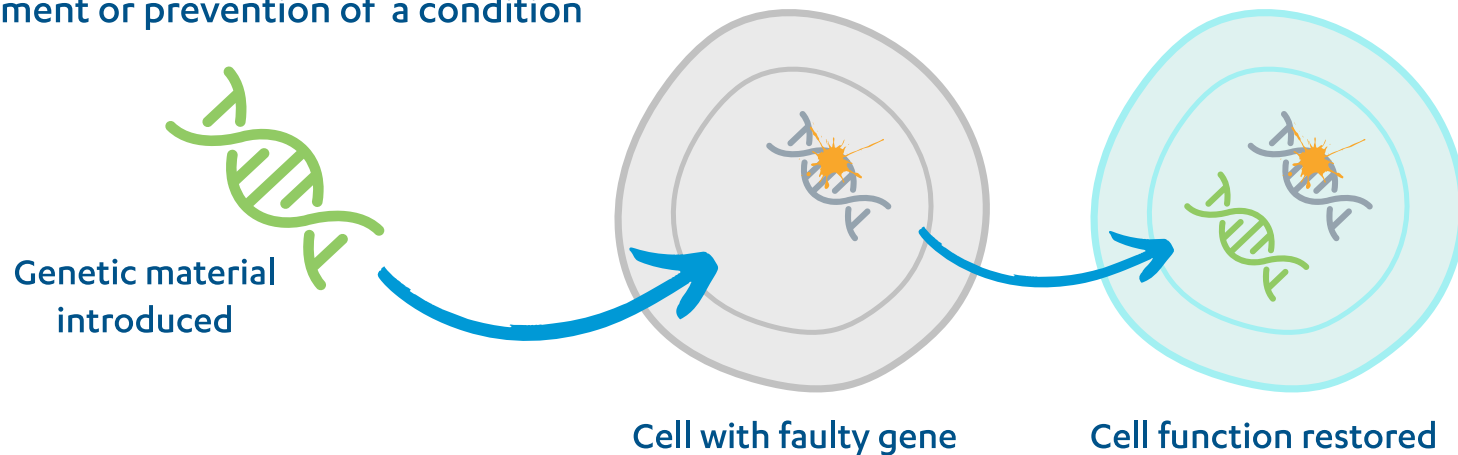
What is Gene Therapy?

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

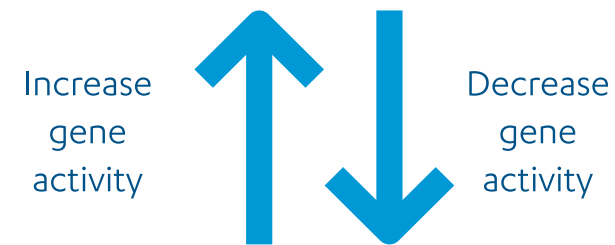
What Types of Gene Therapies Are There?

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

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

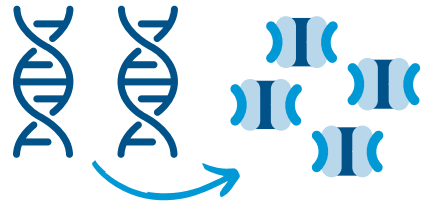
This booklet provides an overview of **gene regulation therapy**

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



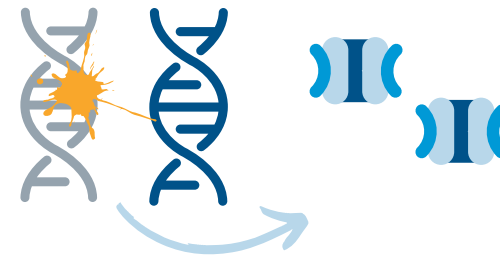
How Does Gene Regulation Therapy Work?

Every person has two copies of a specific gene

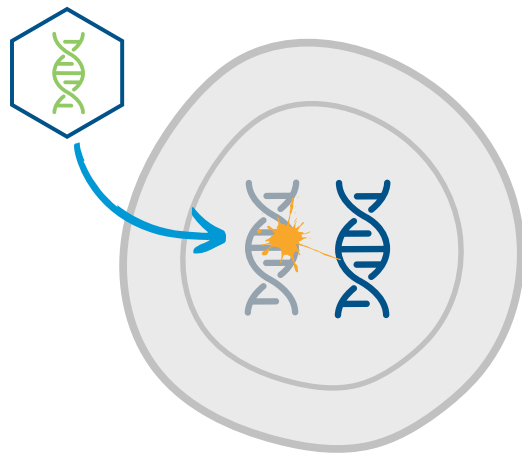


Genes code for proteins that allow the body to function properly

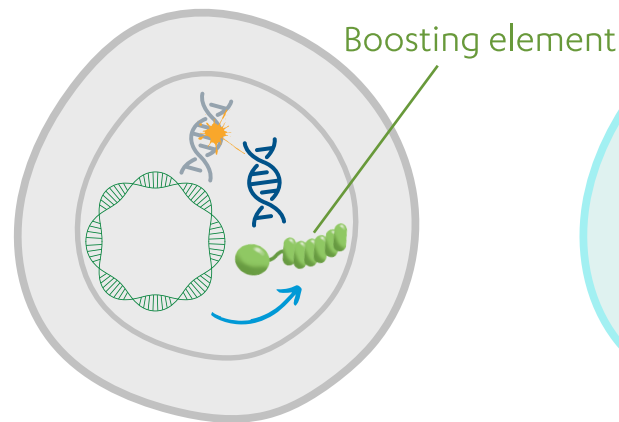
When one of the two copies of a specific gene is faulty, there may be a reduction in the amount of protein produced



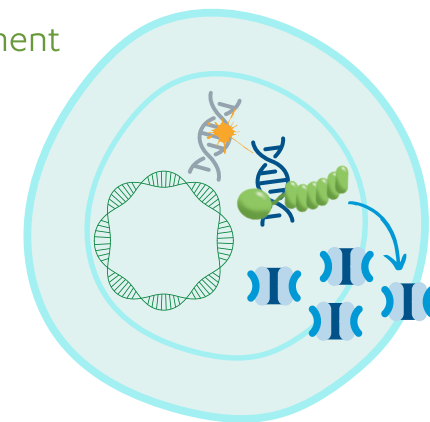
1. Potentially therapeutic genetic material is delivered to a cell's nucleus



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

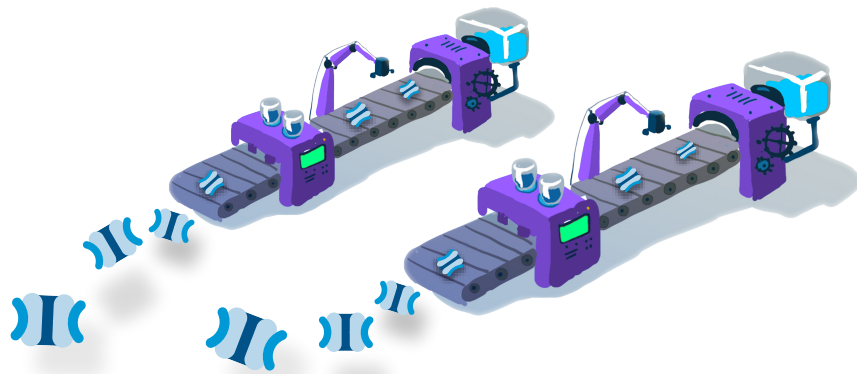


3. This boosting element tells the working copy of the gene to make more protein

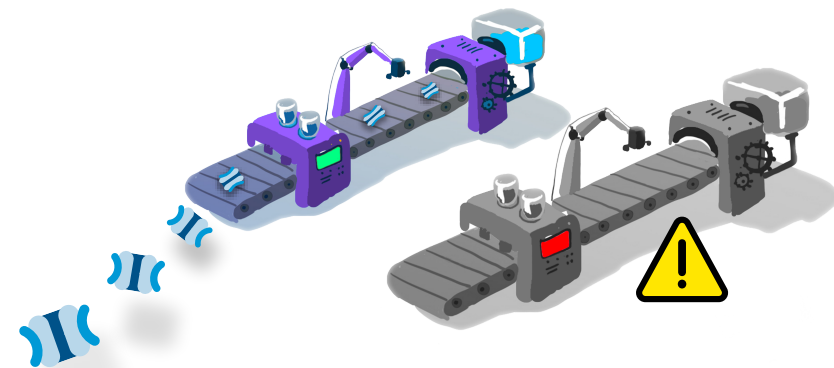


Gene Regulation Therapy Analogy

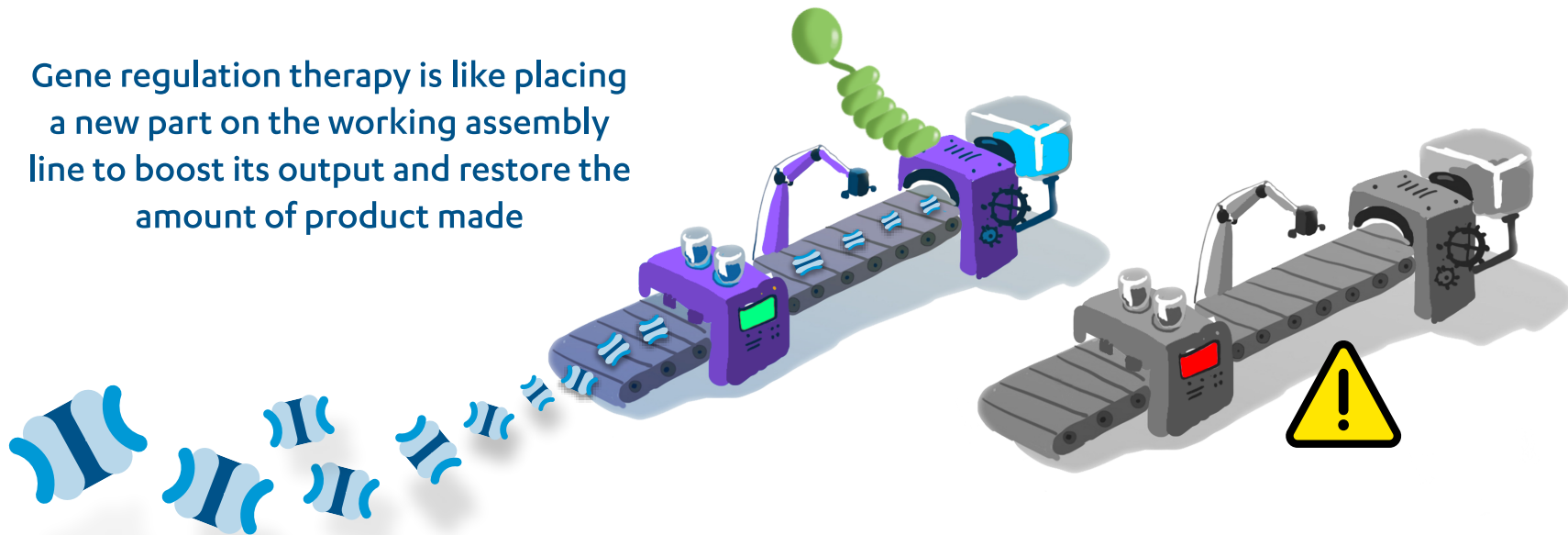
Imagine two assembly lines representing the two copies of the gene of interest, working together to make a product



If one 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 boost its output and restore the amount of product made



This educational booklet was developed by Encoded Therapeutics.



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