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What is GENE THERAPY?

What are genes and what do they do?

Genes are small sections of DNA. Every person has two copies of each gene, one inherited from each parent. Most genes are the same in all people, but a small number of genes are slightly different between people. These small differences contribute to each person's unique features.

Genes act as instructions to make the functional building blocks of the cell, enabling them to function normally. A genetic mutation is a change in the structure of a gene or group of genes. Such changes can be passed on from parent to child. Many mutations cause no harm but others can cause genetic disorders.

Faulty gene passed on from a parent can cause genetic disorders

How is gene therapy designed to work?

Built on
decades of research

The aim of gene therapy is to use genes to treat disease using different approaches.

Gene therapy has been approved as a treatment for several rare inherited diseases and there is much ongoing research to find ways to treat even more.

For more information about gene therapy, please consult your healthcare provider.

Gene editing
Inserting, deleting or replacing DNA at a specific site.

Gene expression alteration
Inactivating, or ‘knocking out’, a mutated gene that is not functioning properly.

Gene addition / insertion
Introducing a functioning gene into the body to help treat a disease.

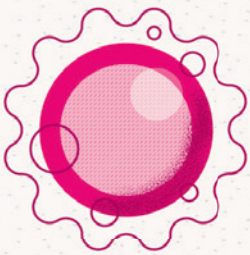
What is ex vivo autologous HSC gene therapy?

A person's body contains stem cells throughout their life. The body can use these stem cells to make new cells when it needs them.

Blood stem cells, otherwise known as haematopoietic stem cells (HSCs), are cells that are found in your bone marrow. These cells are capable of producing copies of themselves (self-renewal) and of producing bloods cells of all types, including white blood cells, red blood cells and platelets.

Ex vivo HSC gene therapy uses a patient's own (autologous) HSCs to correct a genetic disorder by inserting a functioning copy of the gene into the patient's cells outside of the body, and then returning these cells to the patient.

Haematopoietic stem cells (HSCs) produce all types of new blood cells



Useful terms

Genes
Small sections of DNA that contain the instructions for individual characteristics, like eye and hair colour, and how to make proteins, the functional building blocks of the cell. Proteins are responsible for making sure that the cells in the body function properly.

Autologous cells
Cells that come from the patient themselves.

Engraftment
When stem cells are accepted by the patient's body.

Ex vivo
Outside of the body.

Gene therapy
A technique that aims to use genes to treat disease.

Mutation
A change in the structure of a gene or group of genes. Such changes can be passed on from parent to child. Many mutations cause no harm, but others can cause genetic disorders.

Clinical trials
Research studies performed in people that are aimed at evaluating a therapy. They are the main way that researchers find out if a new treatment, like a new drug, is safe and effective in people.

Blood stem cells or haematopoietic stem cells (HSCs)
Cells capable of producing copies of themselves (self-renewal) and of producing blood cells of all types including white blood cells, red blood cells, and platelets.

Viral vector
A virus that has been engineered to be non-infectious, and is used instead to transport functioning genes into cells.

References

- Genetics Home Reference [Internet]. Bethesda (MD): Published: March 22, 2021. Available from https://ghr.nlm.nih.gov/primer/basics/gene
- Wang D, Gao G. Discov Med 2014;18(98):151–161

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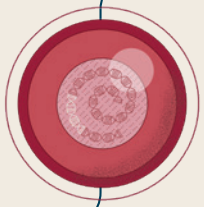
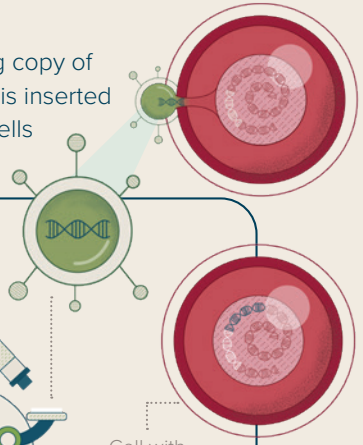
Ex vivo HSC gene therapy at a glance

1 **Step 1**
Blood stem cells are collected from the patient

2 **Step 2**
The stem cells are selected and purified



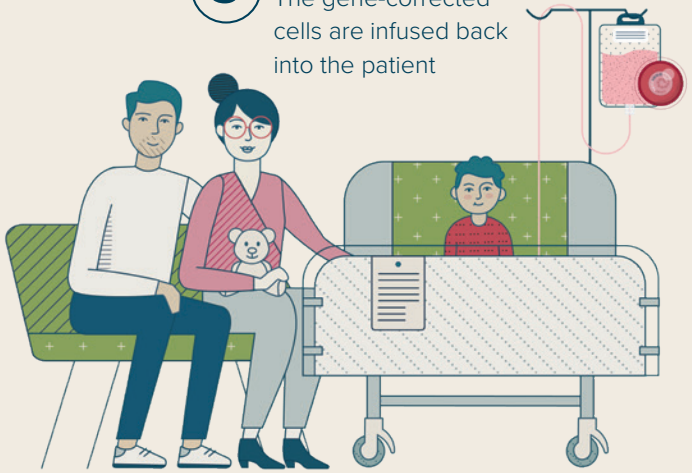
3 **Step 3**
A working copy of the gene is inserted into the cells



Cell with faulty gene

4 **Step 4**
The patient is conditioned to receive treatments

5 **Step 5**
The gene-corrected cells are infused back into the patient



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