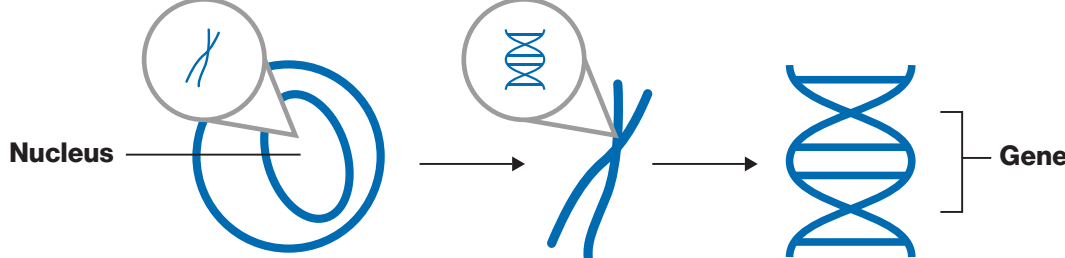


An introduction to cell and gene therapy

What are cells and genes?

Cells are the basic building blocks of all living things, and genes can be found deep within cells. Genes are small sections of DNA that carry genetic information and instructions for making proteins, which help build and maintain the body¹.

Genes are a part of DNA¹:



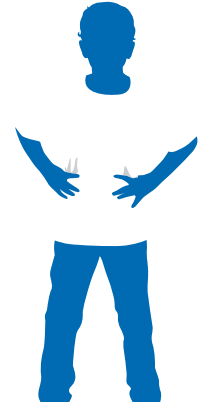
Cell

The nucleus controls the processes of the cell

Chromosome

Chromosomes are thread-like structures made up of DNA tightly coiled many times around proteins called histones

DNA



EVERY PERSON HAS AROUND

20 000

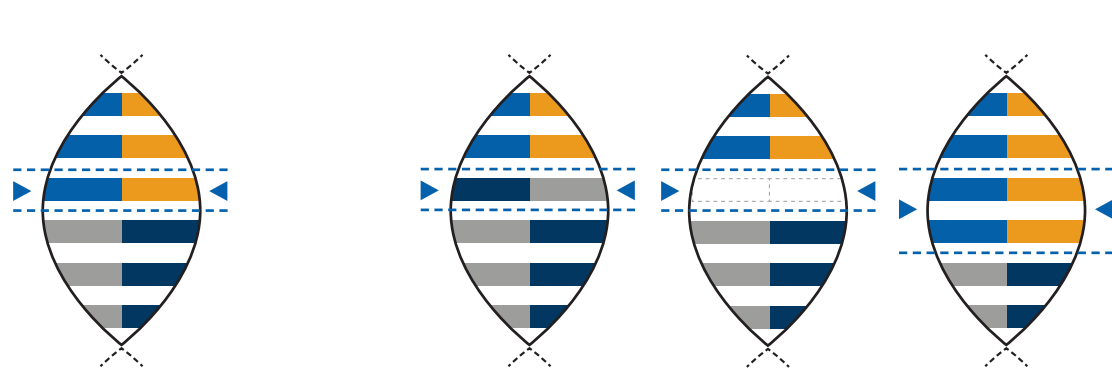
GENES AND TWO COPIES OF EACH OF THEIR GENES — ONE FROM EACH PARENT.

Small variations in genes result in differences in people's appearance and, potentially, health¹.

What are genetic diseases?

Genetic diseases happen when a critical piece or whole section of DNA is substituted, deleted or duplicated². These changes are called genetic mutations³.

Genetic mutations can cause genetic diseases²:



Original DNA Sequence

Genetic mutations

Substitution

Deletion

Duplication

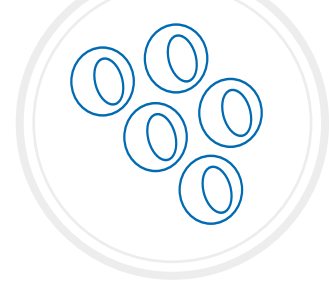


Some serious genetic diseases caused by genetic mutations can be passed to future generations⁴.

How do cell and gene therapies help treat genetic diseases?

Cell therapy and gene therapy are overlapping fields of biomedical research and treatment⁵. Both therapies aim to treat, prevent, or potentially cure diseases, and both approaches have the potential to alleviate the underlying cause of genetic diseases and acquired diseases⁶. But, cell and gene therapies work differently.

The difference between cell therapy and gene therapy:



Cell therapy aims to treat diseases by restoring or altering certain sets of cells or by using cells to carry a therapy through the body⁵. With cell therapy, cells are cultivated or modified outside the body before being injected into the patient. The cells may originate from the patient (autologous cells) or a donor (allogeneic cells)⁶.



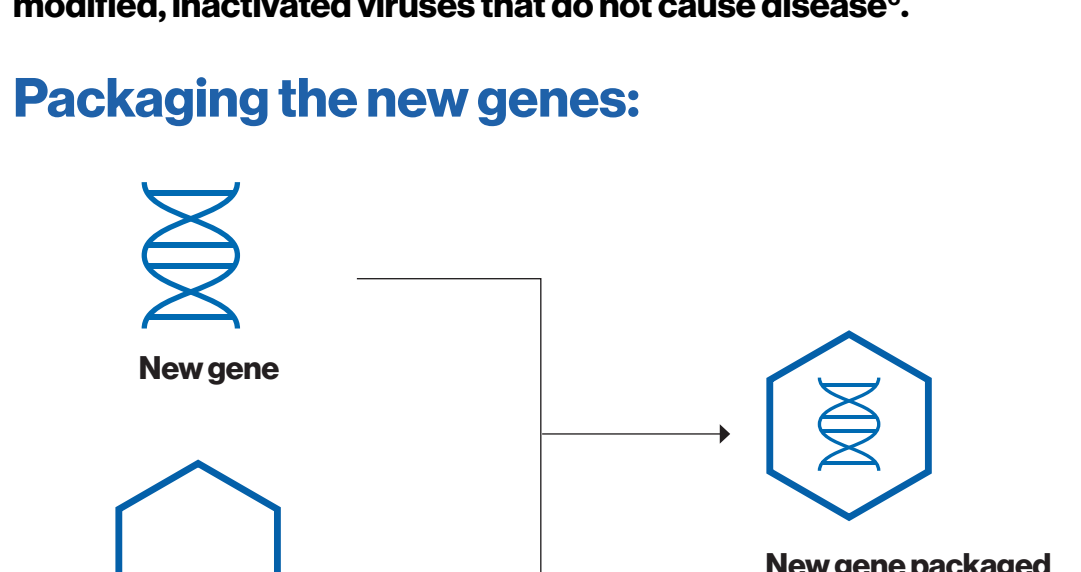
Gene therapy aims to treat diseases by replacing, inactivating or introducing genes into cells—either inside the body (in vivo) or outside of the body (ex vivo)⁶.

Some therapies are considered both cell and gene therapies. These therapies work by altering genes in specific types of cells and inserting them into the body.

How do we use cell and gene therapies?

Scientists have been researching gene therapy for over 50 years⁵. Today, they transfer new genes into cells using transporters known as vectors, which are often made from modified, inactivated viruses that do not cause disease⁶.

Packaging the new genes:

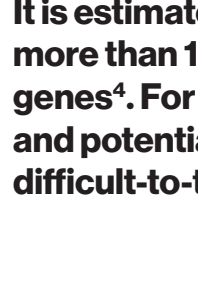


Once packaged, there are two ways to deliver the new gene—ex vivo or in vivo



Why is cell and gene therapy important?

It is estimated that millions of people globally suffer from more than 10 000 rare diseases caused by mutations of single genes⁴. For these patients, cell and gene therapies offer hope and potential cures for previously untreatable diseases and difficult-to-treat diseases across a wide array of medical fields⁷.



10 000

RARE DISEASES AROUND THE WORLD ARE CAUSED BY MUTATION OF A SINGLE GENE⁴.

Cell and gene therapies offer hope for a wide array of untreatable diseases and difficult-to-treat diseases in⁷:



Hematology



Oncology



Ophthalmology



Neurology



Cardiology

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