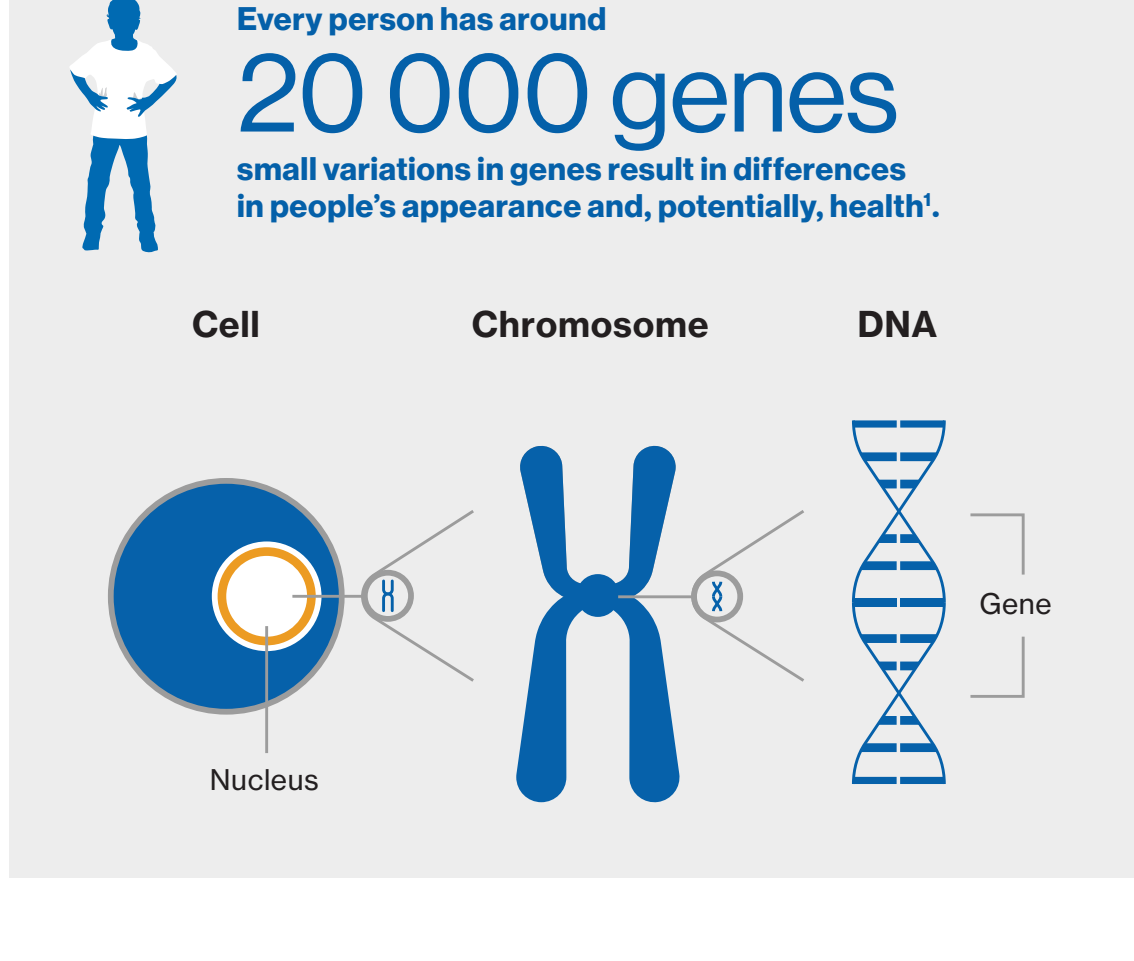


Gene Therapy

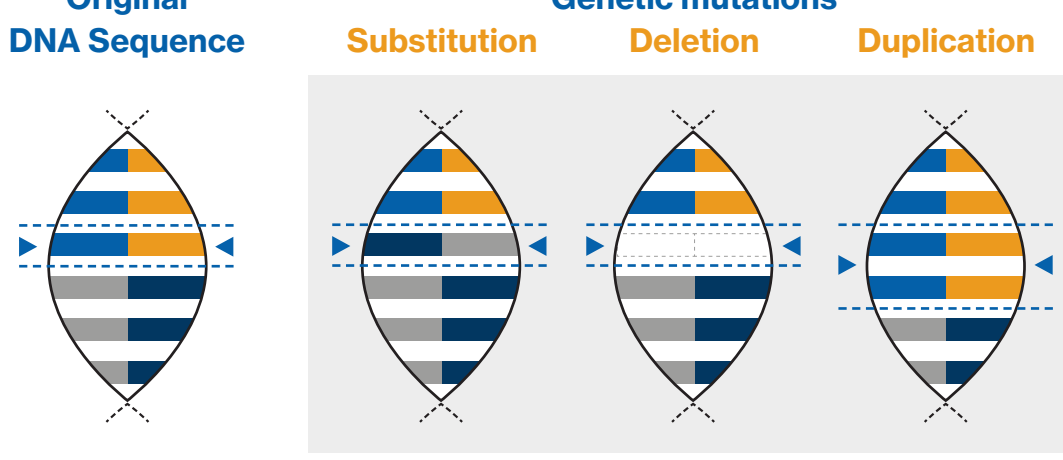
What are genes?

Genes are small sections of DNA that carry genetic information and instructions for making proteins, which help build and maintain the body¹. Every person has two copies of most of their genes - one from each parent¹.



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.



While some mutations are harmless, others can cause serious genetic diseases that are passed to future generations⁴.



Well-known genetic diseases⁴:



Cystic fibrosis



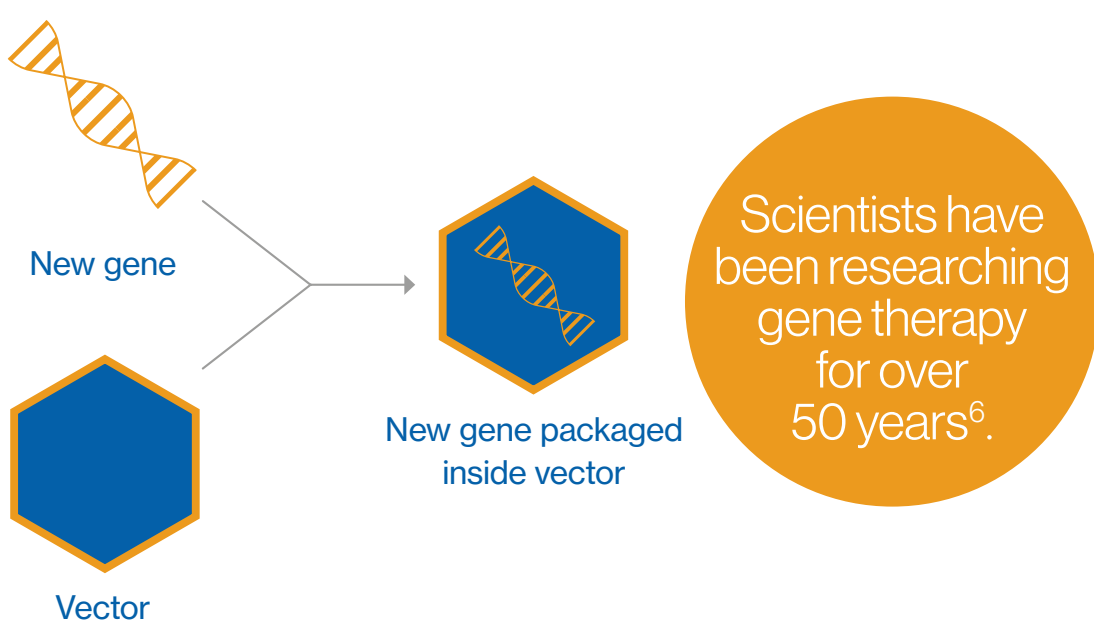
Hemophilia



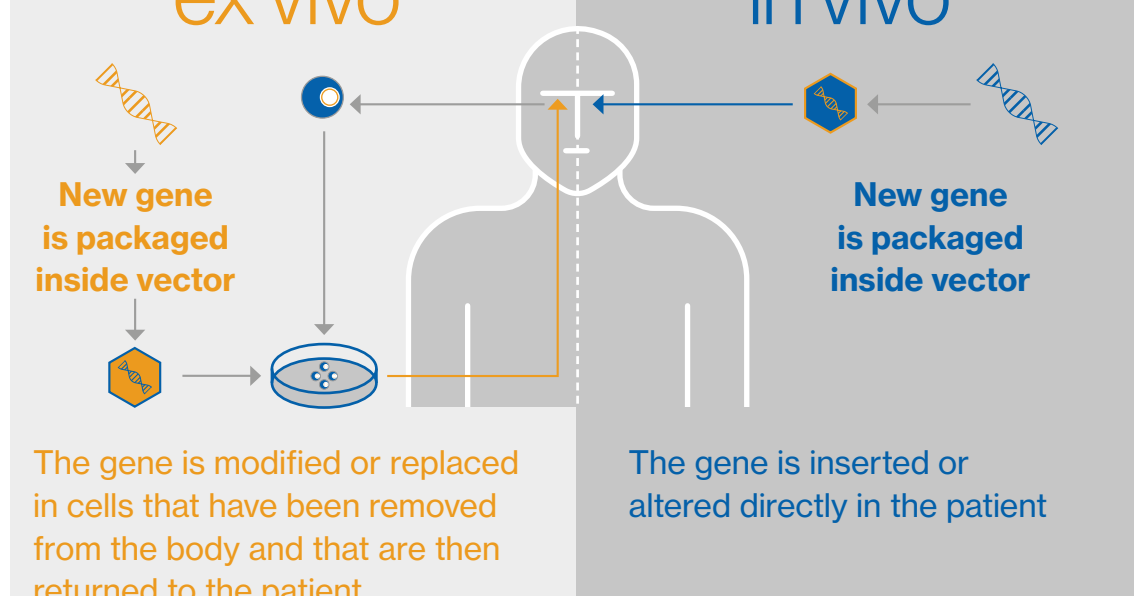
Sickle cell anemia

What is gene therapy?

Gene therapy aims to treat or prevent a wide array of genetic diseases by suppressing, replacing or augmenting mutated genes with functioning copies⁵. New genes are transferred into cells using transporters known as vectors, which are often made from modified, inactivated viruses⁵.

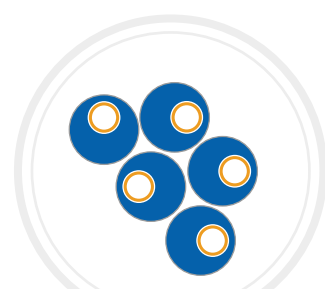


There are two ways to deliver gene therapy⁵



What is the difference between gene therapy and cell therapy?

Gene therapy and cell therapy are overlapping fields of biomedical research and treatment, but they are not the same⁷.



Cell therapy aims to treat diseases by restoring or augmenting certain sets of cells or by using cells to carry a therapy through the body⁷.



Gene therapies aim to treat or prevent diseases by modifying, repairing or replacing a mutated gene⁷.

Why is gene therapy important?

It is estimated that **millions of people** globally suffer from more than **10 000 rare diseases** caused by mutation of a **single gene**⁴.



For these patients, gene therapy offers hope, through its potential to treat previously untreatable diseases across a wide array of medical fields⁸:



Ophthalmology



Cardiovascular diseases



Neurology

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