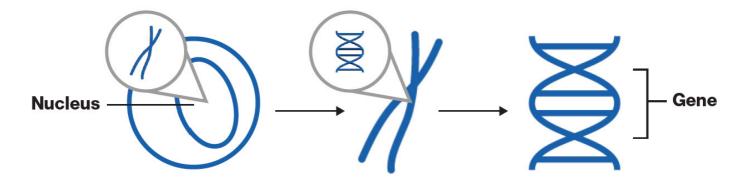
What is cell and gene therapy

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.



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

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. 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 treatment⁶. 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:

1/2

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.

Learn more about how we use cell and gene therapies and why they are important

View the infographic

References

- 1. National Institutes of Health (NIH) U.S. National library of medicine. What is a gene? Available at: https://ghr.nlm.nih.gov/primer/basics/gene. Last accessed April 2019.
- 2. National Institutes of Health (NIH) U.S. National library of medicine. What kinds of gene mutations are possible? Available at: https://ghr.nlm.nih.gov/primer/mutationsanddisorders/possiblemutations. Last accessed April 2019.
- 3. National Institutes of Health (NIH) U.S. National library of medicine. What is a gene mutation and how do mutations occur? Available at: https://ghr.nlm.nih.gov/primer/mutationsanddisorders/genemutation. Last accessed April 2019.
- 4. World Health Organization. Genes and human diseases. Available at: http://www.who.int/genomics/public/geneticdiseases/en/index2.html. Last accessed April 2019.
- 5. Friedman T. A brief history of gene therapy. Nat Genet. 1992; 2: 93-98.
- 6. American Society of Gene & Cell Therapy. Different approaches. Available at: https://www.asgct.org/education/different-approaches. Last accessed April 2019.

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List of links present in page

- 1. https://prod1.novartis.com/about/novartis-gene-therapies/what-cell-and-gene-therapy
- 2. https://prod1.novartis.com/news/media-library/introduction-cell-and-gene-therapy
- 3. https://ghr.nlm.nih.gov/primer/basics/gene
- 4. https://ghr.nlm.nih.gov/primer/mutationsanddisorders/possiblemutations
- 5. https://ghr.nlm.nih.gov/primer/mutationsanddisorders/genemutation
- 6. http://www.who.int/genomics/public/geneticdiseases/en/index2.html
- 7. https://www.asgct.org/education/different-approaches