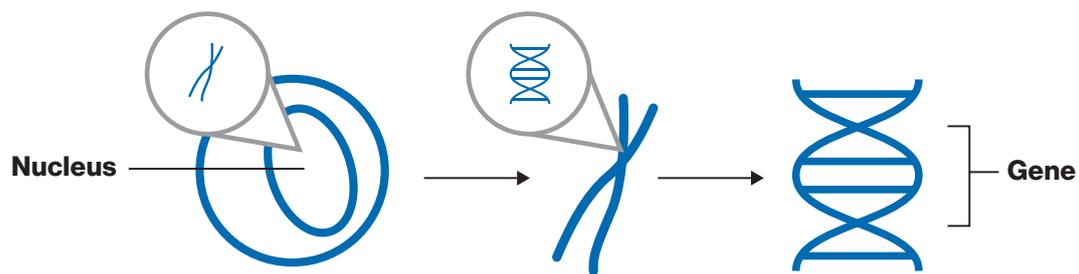


An Introduction to Cell and Gene Therapy

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 deoxyribonucleic acid (DNA) that usually carry genetic information and instructions for making proteins, which help build and maintain the body.^{1,2}



Cell

The nucleus controls the processes of the cell¹

Chromosome

Chromosomes are thread-like structures comprised of DNA tightly coiled around proteins called histones¹

DNA

DNA is code with instructions for building and maintaining an organism¹



EVERY PERSON HAS AROUND

20,000

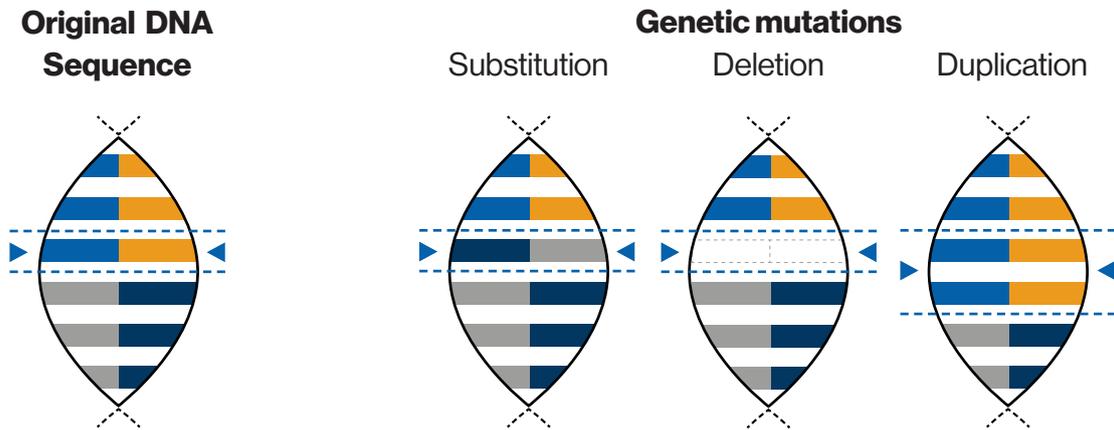
**GENES, TWO COPIES OF EACH GENE—
ONE FROM EACH PARENT.**^{1,3}

Small variations in genes result in differences in people's appearance and, potentially, health.^{1,3}

What are genetic diseases?

Genetic diseases happen when a critical piece or whole section of DNA is substituted, deleted, or duplicated. These changes are called genetic variants or mutations.³

Genetic mutations can cause genetic diseases³:

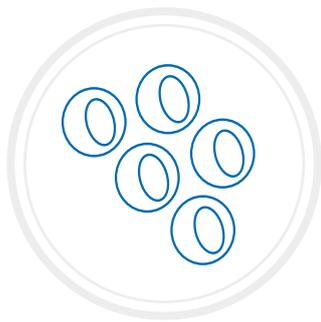


Some serious genetic diseases caused by genetic mutations can be passed to future generations.³

How do cell and gene therapies help treat genetic diseases?

Cell therapy and gene therapy are overlapping fields of biomedical research and treatment. Both therapies aim to treat or prevent diseases, and both approaches have the potential to alleviate the underlying cause of genetic diseases and acquired diseases. But, cell and gene therapies work differently.^{4,6}

The difference between cell therapy and gene therapy:



Cell therapy aims to treat diseases by restoring or altering certain sets of cells.⁴ 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



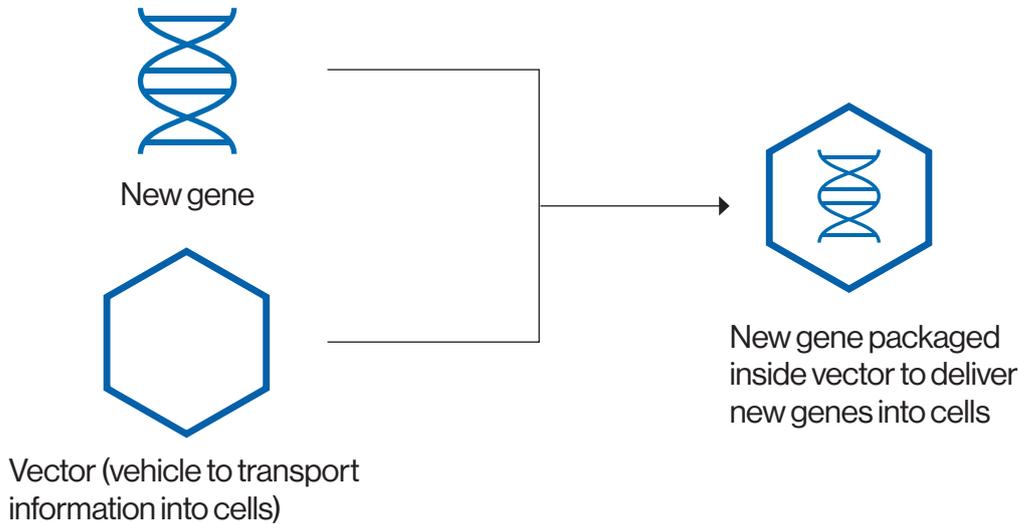
Gene therapy aims to treat diseases by replacing, inactivating, or introducing genes into cells—either inside the body (*in vivo*) or outside of the body (*ex vivo*).^{4,5}

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.⁶

How do we use cell and gene therapies?

Scientists have been researching gene therapy for over 50 years. Today, they transfer new genes into cells using transporters known as vectors, which are often made from modified viruses that do not cause disease.^{4,7}

Packaging the new genes^{5,6}:



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

Ex Vivo outside the body

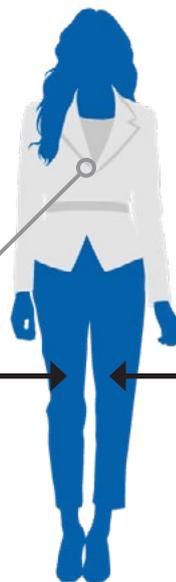
The target cells are removed from the patient, modified outside of the body, then returned to the patient



+



New gene is packaged inside vector and administered to the cells



In Vivo inside the body

The target cells remain in the patient and the therapy is administered directly to the patient



New gene is inserted or altered inside patient



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Why are cell and gene therapies important?

It is estimated that millions of people globally suffer from more than 10,000 rare diseases caused by mutations of single genes. For these patients, cell and gene therapies offer hope for previously untreatable diseases and difficult-to-treat diseases across a wide array of medical fields.^{4,5,8}



10,000

RARE DISEASES AROUND THE WORLD ARE CAUSED BY MUTATIONS OF SINGLE GENES.^{4,5,8}

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



Hematology^{4,5}



Oncology^{4,5}



Ophthalmology^{4,5}



Neurology^{4,5}



Cardiology⁹

Resources: **1.** Help me understand genetics: cells and DNA. *MedlinePlus*. US National Library of Medicine, National Institutes of Health, Department of Health and Human Services. Updated September 10, 2020. Accessed February 7, 2022. <https://medlineplus.gov/download/genetics/understanding/basics.pdf> **2.** Deoxyribonucleic acid (DNA) fact sheet. National Human Genome Research Institute. Updated August 24, 2020. Accessed February 7, 2022. <https://www.genome.gov/about-genomics/fact-sheets/Deoxyribonucleic-Acid-Fact-Sheet> **3.** Help me understand genetics: variants and health. *MedlinePlus*. US National Library of Medicine, National Institutes of Health, Department of Health and Human Services. Updated March 25, 2021. Accessed February 7, 2022. <https://medlineplus.gov/download/genetics/understanding/mutationsanddisorders.pdf> **4.** American Society of Gene and Cell Therapy. Different Approaches. Accessed February 7, 2022. <https://patienteducation.asgct.org/gene-therapy-101/different-approaches>. **5.** American Society of Gene and Cell Therapy. Gene Therapy Basics. Accessed February 7, 2022. <https://patienteducation.asgct.org/gene-therapy-101/gene-therapy-basics>. **6.** American Society of Gene and Cell Therapy. Gene & Cell Therapy FAQs. Accessed February 7, 2022. <https://asgct.org/education/more-resources/gene-and-cell-therapy-faqs> **7.** Friedmann T. A brief history of gene therapy. *Nat Genet*. 1992;2(2):93-98. **8.** What are single gene disorders? yourgenome. Updated July 21, 2021. Accessed February 7, 2022. <https://www.yourgenome.org/facts/what-are-single-gene-disorders> **9.** Hajjar R, Fuster V. Cardiac cell and gene therapies: two trajectories, one goal. *Nat Clin Pract Cardiovasc Med*. 2008;5(12):749.