An introduction to cell and gene therapy

What are cells and genes?

Cells are the basic building blocks of all living things, and genes are the instructions that control how these cells function. Genes are a part of DNA, which is found in the nucleus of a cell.

DNA that carry genetic information and instructions for making proteins is called genetic material.

Genes

What are genetic diseases?

Genetic diseases happen when a critical piece or whole section of DNA is missing, altered, or extra. These changes can affect the processes of the cell.

Why is cell and gene therapy important?

Genetic diseases happen when a piece of DNA is missing, altered, or extra. These changes can affect the processes of the cell.

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

- Cardiology
- Neurology
- Oncology
- Ophthalmology
- Hematology
- Dermatology
- Gastroenterology
- Urology
- Pulmonology
- Rheumatology

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

How do we use cell and gene therapies?

To deliver the new gene—ex vivo or in vivo.

EX Vivo

GENE THERAPY: IN VIVO

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Once packaged, there are two ways to deliver the new gene—especially in vivo:

1. Modified, inactivated viruses that do not cause disease
2. Modified viruses that do not cause disease

Packaging the new gene:

New gene packaged inside vector to deliver to cell and gene therapy:

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