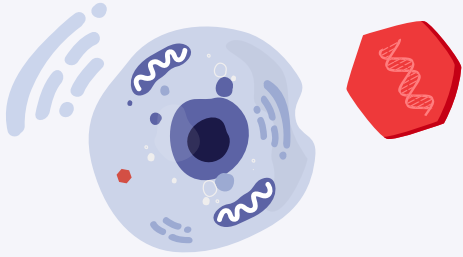


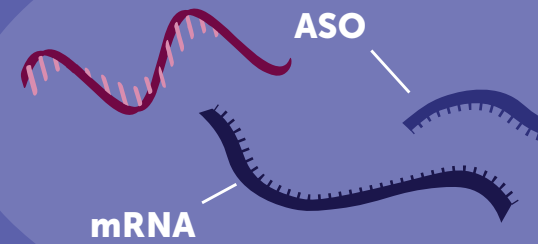
What are the Different Gene Therapy Approaches?

Gene Therapy is the use of genetic material to treat or prevent disease. This material is usually DNA or RNA, which are strings of molecules with the information to instruct cells to produce proteins.



One type of gene therapy is **gene addition**, which adds in a working gene that has the instructions for the cell to make more of a desired protein. A vector is often used to deliver the working gene to the cell's nucleus. This gene will live in the nucleus which gives a greater chance of being a permanent change and is only given one time.

RNA Therapy is the use of shorter sequences of genetic material in RNA format to treat or prevent a disease. There are many different types of RNA therapy because there are so many types of RNA sequences and sizes that can affect cell functions. Some of these types include mRNA, ASOs, miRNA, Ribosomal RNA, siRNA and tRNA. These therapies often involve **gene silencing**, which silences a gene to stop it from creating a toxic protein. Receiving repeat doses is common for these types of therapies because they do not permanently change any of our DNA.



Gene Editing is a type of gene therapy that corrects pieces of DNA by changing or deleting the information **within** the affected individual's gene rather than adding a new gene like gene addition. Gene editing uses technology that is highly precise to make these types of changes.

Cell Therapy is the transfer of a specific cell type(s) into a patient to treat or prevent disease. Depending on the cell therapy, the cells can come from either the affected individual or an unaffected donor.

Gene-Modified Cell Therapy is a combination of gene and cell therapy. It first removes a person's own cells from the body. Certain cell types are then treated by adding a working gene or modifying the affected one. The modified or treated cells are then returned to the person.



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