11 - 11 - 2024

News: RNA editing

Recently, Wave Life Sciences, a biotechnology company in the US, became the first company to treat a genetic condition by editing Ribonucleic acid (RNA) at the clinical level.

Ribonucleic Acid (RNA) editing

- Ribonucleic Acid (RNA) editing is the process of modifying Messenger RNA (mRNA) nucleotides, after Deoxyribonucleic acid (DNA) creates mRNA but before it begins protein synthesis.
- mRNA is made up of portions called exons and introns. Exons eventually code for a protein whereas the introns are non-coding parts and are removed from the RNA before it's used to make a protein.

Types

There are three types of RNA modifications i.e., addition, deletion, and substitution. Addition is when a nucleotide is inserted. Deletion is when one is removed while substitution refers to the replacement of one nucleotide with another.

Mechanism

- The technique involves a group of enzymes called adenosine deaminase acting on RNA (ADAR).
- Scientists pair ADAR's effects with a guide RNA (or gRNA) that guides ADAR to a specific part of the mRNA, where the ADAR does the designated job.

Clinical Use

- Wave Life Sciences used RNA editing to treat α-1 antitrypsin deficiency (AATD), an inherited disorder through a therapy dubbed as WVE-006.
- RNA editing shows promise for treating Huntington's disease, Duchenne muscular dystrophy, obesity, Parkinson's disease, neurological conditions, heart diseases, and more.

Ribonucleic acid (RNA)

- > RNA is a nucleic acid present in all living cells.
- > It is structurally similar to DNA but typically single-stranded.
- ➢ Its backbone consists of alternating phosphate groups and ribose sugars, with bases adenine (A), uracil (U), cytosine (C), and guanine (G).

Types of RNA

- Messenger RNA (mRNA): Carries genetic information from DNA to ribosomes for protein synthesis.
- Ribosomal RNA (rRNA): Forms the core of the ribosome's structure and catalyses protein synthesis.
- Transfer RNA (tRNA): Transfers amino acids to ribosomes during protein synthesis.
- > Regulatory RNAs: Play roles in gene expression regulation.

Functional Significance

- RNA plays essential roles in cellular processes like building cells, immune responses, and transporting amino acids.
- > Certain viruses use RNA as their genetic material.