Genetics and its uses in Healthcare

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Introduction

Gene editing is an evolving field that has allowed scientists to make precise changes to an organism's DNA. Using tools like the CRISPR-Cas9 has allowed researchers to target and modify specific genes to correct genetic defects or diseases or for personal benefit. This has prominently revolutionized the biotechnology field, as it has raised concerns over ethical and safety questions, but it has also been an impactful tool in modern day science.

How Genetics Cause Sickle Cell Diseases

Sickle cell disease (also known as SCD) is an inherited blood disorder which affects the shape and function of red blood cells. This disease is caused by a mutation in the beta-globin gene which codes for a protein called hemoglobin. This is important since hemoglobin carries oxygen throughout our body. The mutation produces an unusual shape of the SCD which causes the red blood cells to become crescent-shaped. Generally, a person with sickle cell disease is born with two copies of the mutated HBB gene inheriting one from each parent. The mutation is a single nucleotide change in which adenine is replaced by thymine in the DNA sequence which changes the codon GAG to GTG resulting in SCD. The abnormal red blood cells can cause vaso-occlusive episodes in which pain occurs in the chest, limbs, and back. Sickled red blood cells can also block blood flow to organs damaging health.

How the Crispr-Cas9 Works

Crispr-Cas9 edits genes by cutting into DNA and modifying the genes in a desired manner. The Crispr-Cas9 is a technology which allows geneticists to edit parts of the genome by altering the DNA sequence to change appearance or to prevent diseases. The Crispr-Cas9 is one of the easier methods of genetic modification. An enzyme called the Cas9 acts as a pair of molecular scissors and can cut two strands of DNA at the genome where DNA could be added or removed. The Crispr-Cas9 is also a good tool for treating many medical conditions.

History of Gene Therapy in Clinic & Gene Therapies

Gene therapy is a technique that uses genes to treat medical diseases or disorders. Gene therapies replace defective/missing genes with a healthier version that could help their health. DMD is a genetic disorder caused by mutations in the DMD gene. There have been recent advancements with a microdystrophin gene that uses a shorter version of the dystrophin gene, which improves muscle health and results in successful clinical trials. This allows people with DMD to stay active.

Safety and Ethical Considerations of Using Gene Editing to Treat Sickle Cell Disease

CRISPR and other gene-editing technologies serve as a way to correct the genetic mutation for sickle cell disease. However, off-target effects could lead to harmful mutations and other health problems. These types of advanced treatments may not be available for everyone, which questions affordability and people who are underprivileged but more prone to the disease. Also, long-term effects are uncertain and could be potentially harmful in the long run.

References

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