The Medical Advances in Gene Editing

Gene editing has taken the medical world by storm and there’s no denying that this emerging field holds great promise, with the potential to treat or cure many genetic disorders. Still, despite all the excitement surrounding gene editing, we must also pause to remember that this innovative technology carries risks too; so it’s vital to keep an eye on developments in this field as they unfold to mitigate any possible negative impacts of gene editing technologies on society.

Medical science has changed quite a bit in the last hundred years or so, but the field of genetics, the study of how our genes impact our bodies and behaviours, has changed in recent years at an unprecedented rate. The advent of gene editing technologies, like CRISPR/Cas9, is revolutionizing the medical field by giving scientists greater control over genetic manipulation than ever before. These new technologies are expected to have major ramifications for cancer treatment as well as gene therapy itself and could even lead to treatments that aren’t genetically modified at all!

Before diving into what's next for genetic engineering, it's important to understand what genome editing means. Genome editing refers to any technique that allows scientists to edit individual DNA letters within a genome, and several different methods exist. CRISPR-Cas9 is one of these methods for genome editing, but it is not synonymous with it. It is just one method for gene editing, albeit a very promising one! There are other types of gene editing that are already being used in medicine today. For example, TALENs (transcription activator-like effector nucleases) have been used in clinical trials since 2012 to treat cancer. Another type of technology called zinc finger nucleases (ZFNs) has also been used successfully in clinical trials since 2009.

CRISPR is a tool invented in 2012 by biologists Jennifer Doudna and Emmanuelle Charpentier. It stands for Clustered Regularly Interspaced Short Palindromic Repeats, which are repeating genetic elements found in bacteria that use these sequences to identify and destroy invading viruses. CRISPR was adapted into an RNA-guided genome editing tool. This revolutionary technique offers a way to permanently change DNA by deleting, adding, or replacing pieces of genetic code at specific locations within a genome.

CRISPR stands for clustered regularly interspaced short palindromic repeats. It refers to a piece of DNA that has several repeats in it, and scientists have found ways to manipulate it so that they can essentially tell a cell what to do with its genes. But let’s start at the beginning: cells have DNA, which is stored in chromosomes (yep, just like ketchup on your hamburger bun). There are many different types of cells in our bodies and each one contains unique information. For example, my muscle cells have genetic information geared toward growing and repairing muscle tissue while my blood cells have the specific genetic coding that helps them clot when needed. Just as we’ve come to rely on chemical insecticides, we may someday do away with genetic diseases. While gene editing is still in its infancy, researchers believe that it could be used to treat everything from hereditary blindness and diabetes to sickle cell anaemia. The process is also less invasive than other current treatments, and can potentially be implemented without a physical incision.

There are various concerns associated with genetic editing technology that need to be considered. In April 2018, Chinese researchers claimed to have genetically modified human embryos; though what they were doing was modifying a specific gene involved in blood disease, and a moratorium has been placed on all such experiments for now. Although there are obvious medical benefits to allowing CRISPR technology to develop further, there are many ethical and safety issues that must be debated before it’s rolled out on a widespread basis. One concern that a lot of people have raised is regulation. Gene editing techniques have been used in research for some time, but only recently have companies started to develop new tools to edit DNA in living cells. As soon as these companies begin to sell their products, how will governments react? Will gene editing treatments become regulated similarly to other pharmaceutical drugs or medical devices? If so, which agencies would regulate gene-editing therapies, and what kinds of rules and regulations would they follow?