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Research Question: Can gene-editing tools treat IBDs?

Background:

Over the last four weeks, I have been conducting research dealing with the potential uses of gene editing with other diseases such as those in the IBD category. The reason I was intrigued by this was due to the fact that about a year and a half ago I was diagnosed with UC or ulcerative colitis. This disease is a genetic defect that causes my own immune system to attack my intestine. Until a few weeks ago I thought that the only viable treatment was through biological means, such as infusions which I have been receiving for a year. When Oppnet SSRP was started, one of my peers introduced me to the idea of CRISPR and gene-editing as a whole. This impacted my thinking in a large way because I was told that genetic diseases such as sickle cell could be treated, which eminently caused me to wonder how this could be implemented in the types of illnesses that impact me.

Findings:

I accomplished my research by analyzing multiple articles that deal with genetics. In the articles I chose to help me conduct this research, a variety of topics were discussed. These topics included what genes are affected by IBDs, what genes are affected by sickle cell, what gene editing is, and how gene editing treats sickle cell. I chose this approach because I wanted to find the specific genes affected by the 2 disorders that were basically the focus of my project, then I wanted to see how the sickle cell was treated specifically with CRISPR to see if that method was usable with IBDs.

In the first article, *Ulcerative colitis and Crohn's disease: distinctive gene expression profiles and novel susceptibility candidate genes,* I learned that the specific genes affect by IBDs were "16p12–q13 (IBD1), 12p13.2–q24.1 (IBD2), the major histocompatibility complex region on chromosome 6 (IBD3) and 14q11–12 (IBD4) (4–9)"(Lawrence et al., 2001). This was important for me to know as gene editing is a precise operation and knowing what is specifically affected was the first step.

From here I did the same with sickle cell, in the sense that I learned that the gene Hb is defected and is replaced with HbS, which is what caused the sickle-shaped cells (Steinberg et al., 2012). After learning about the impacted genes, I researched how gene editing is

implemented. First, RNA is created by scientists. Then that Cas-9 protein is attached to the RNA, and it is then bound to the target DNA sequence. A double-strand cut is then made and an alternative piece of DNA can then be added (Memi et al., 2018).

Conclusion:

Treating IBDs with gene-editing is a potentially permanent treatment. So far it has been tested in humans as previously mentioned with sickle cell. The only issue is that gene-editing is in its infancy and many things may go wrong. For example, a Chinese scientist named He Jiankui altered the DNA of two twin infants by altering a gene that caused them to be more resistant to some strains of HIV, but in turn, a healthy strand of DNA was inactivated.

Overall this project has sparked my interest in genetics and I hope to continue researching as more resources become available.