Chapter 21

Genome Editing

Objectives: You will be able to

Define what gene editing is.

Differentiate what is altered to change a gene.

Identify uses of gene editing.

 Do you know that there is a way to change our body's makeup on a microscopic level? Scientists have discovered how to do just that in the late 1900s, using a process called genome or gene editing. Gene editing has changed from its original technique since then and has become efficient and cost effective enough to use in regular research since the early 2000s. (National Human Genome Research Institute, 2017) Before we learn how gene editing works, we first need to understand what genes and a few other important structures are.

21:1 What are genes?

 We have chemicals in our bodies made of atoms that combine into a structure. Some of these chemicals are called Deoxyribonucleic acid (DNA) that tell our cells what to do. Cells are the smallest functional component in an organism and too small to be seen by the human eye. Genes make up parts of DNA that have certain codes or instructions to gather proteins in a certain sequence, so our cells know what to do. Genes are a functional unit of heredity, something passed to us from our parents. This is what makes us unique! They can be as short as a few hundred bases or consist of over two million bases. We have trillions of cells in our bodies, and each one is partly made up of DNA. (Saey, T., 2017)

 For future discussion, it will be important to understand the structure of DNA. Imagine a ladder that twists as it goes up. The sides of the ladder are made of chemicals called bases. Bases are the building blocks of DNA and RNA (ribonucleic acid) and can be in different orders so different messages can be sent. DNA contains many genes made up of thousands of chemical bases. DNA has four chemicals bases called adenine, guanine, cytosine and thymine. Scientists call this DNA structure a double-stranded helix which has a backbone consisting of oxygen, phosphorous and carbon atoms (the steps of the ladder). RNA helps build the DNA ladder by sending messages and bringing building tools and formats. RNA is very important, because it is a molecule that can read, carry, and create information. This process is much more complex than this simple explanation but at this time, only a few basics are needed for understanding to the overall topic. The genes as a collection are called a genome. (Mandal, A., 2018)

21:2 What is genome editing?

 Scientists can use genome editing in bacteria, plants, animals, and even humans! Changing DNA can make a fish a different color, help a plant grow taller, or remove a disease that makes organisms (including us) sick. In the lab setting, genomes of animals are studied to observe how diseases progress within them, and that can then be applied to humans. Humans and animals have approximately 85 percent of the same genes. Think back about our DNA ladder discussed earlier. Scientists have a way of removing sections of the ladder, adding to the ladder, or swapping out bases. This allows the messages DNA sends to the cells to be changed. They can do this with a technique called CRISPR (clustered regularly interspaced short palindromic repeats). Scientists discovered CRISPR (pronounced crisper) in 1993 as a naturally occurring immune response within bacteria when invaded by a virus. When we come down with a common cold, we have been invaded by a virus. One way these viruses are fought is with CRISPR RNA. Scientists observed CRISPR RNA guiding the natural defenses to the invading virus to destroy it. Scientists learned they can create their RNA stands synthetically in the lab in 2013 and later learned how to insert it into cells that contain around twenty pairs of bases. By this approach, scientists can provide alternative formats, tools and building blocks to alter the DNA ladder, or more formally speaking, can cut, add, or replace previous DNA sequences. In early gene editing the process would take years, but with CRISPR the same goal could be reached within weeks instead of years. Now we are going to get more technical. Scientists tag DNA strands with something called PAMs (protospacer adjacent motifs) that let CRISPR know where to make the cut or other alterations. Once a cut is made by the lab synthesized RNA, DNA has a mechanism to repair itself. This could consist of reconnecting the broken ends or adding a new sequence between the broken ends. Another method after tagging of DNA is to activate or inactive a gene. This controls if the gene is sending out messages, which is also called gene expression. By doing this, functions within the cell can be turned on or off. (National Human Genome Research Institute, 2017)

21:3 Why do we want to edit genes?

 A large percentage of gene editing has been done for research purposes. Scientists want to understand what causes illnesses, how things grow, and how things duplicate. Specific applications have focused on reproduction, crops, livestock, industrial use, and for medicine. Researchers study reproduction in hopes of preventing diseases that can be inherited from generation to generation. An alternative application in reproduction is the prevention of it. Mosquitos are known to carry diseases such as Malaria, Nest Nile, and Zika, and scientists believe sterilization of mosquitos could reduce or eliminate these diseases. They hope to increase yield of crops and livestock by making them more resistant to disease and other environmental factors. Industrial benefits could include the production of safer biofuels or increasing the shelf life of food. The first application of this was used in yogurt in 2008 to make it resistant to certain viral invasions. Something that could directly benefit you, your family, and classmates are advancements in medicine. This could include ways to treat sicknesses, prevent infections, and creation of more effective and targeted medication. One hope is the ability to create immunity to HIV. Other studies include finding different causes of deafness, eradicating muscle dystrophy, and growing organs for humans! In 2015, a type of gene editing was used in a young girl to fight cancer successfully. (Nuffield Council on Bioethics, 2019)

21.4 Barriers and concerns

 Even with incredible scientific advancements such as this one, there can be drawbacks. Using gene editing tools, a target area to alter could be missed. This means a variety of errors could result, such as cutting DNA in the wrong spot or switching out DNA that was properly functioning. This could result in the creation of a mutation, which is typically what CRISPR works to correct rather than create. Mutations are a transformation of a gene within DNA that occur naturally or from environmental influences. Due to safety, use in humans has been limited. Another issue surrounding this technique and how it will be used in the future. Where should scientists draw the line when it comes to altering genes? Should humans be altered physically when it provides no health advantage? This could include changing eye or hair color, height or other physical characteristics. Could this technology be used in a way that could harm us? These are only a few questions that have been raised in response to this progressing technology. (National Human Genome Research Institute, 2017)

In review

 We learned that humans and other organisms are made up of microscopic chemicals that build together to create DNA, genes and genomes. These are present to tell our cells how to function, although sometimes the messages can cause harm by allowing the cell to function improperly and cause disease. For this reason, scientists began exploring genome editing. Benefits have included safer foods, increased output of crops and livestock, better understanding of diseases, medicine with higher sufficiency and overall, ways to keep us healthier! In completion of this chapter, ethics were also taken into consideration. What would you like to see happen with this technology?

**References**

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