Gene Therapy

Understanding the role genes play in human diseases is an especially important part of biotechnology. This involves finding genes or genetic material that has been mutated in some way, and then identifying a link between those changes and a particular disease. Finding the genetic material that causes a disease enables doctors to make better diagnoses and to predict the likelihood of a person developing it.

gene therapy an experimental treatment to cure genetic disorders that involves inserting a healthy, normal form of a gene into the cells of tissues that are affected by a disorder

Gene therapy is an experimental treatment to cure genetic disorders. Its aim is to insert a healthy, normal form of a gene into the cells of tissues that are affected by a disorder. This gene can replace the mutated gene that causes the disorder. The approach to gene therapy for treating cystic fibrosis is shown in Figure 1.38. A normal gene is added to lung tissue, where it produces a normal protein. With a functioning protein, the thick mucus that builds up and harms people with cystic fibrosis is no longer produced, allowing them to breathe normally.

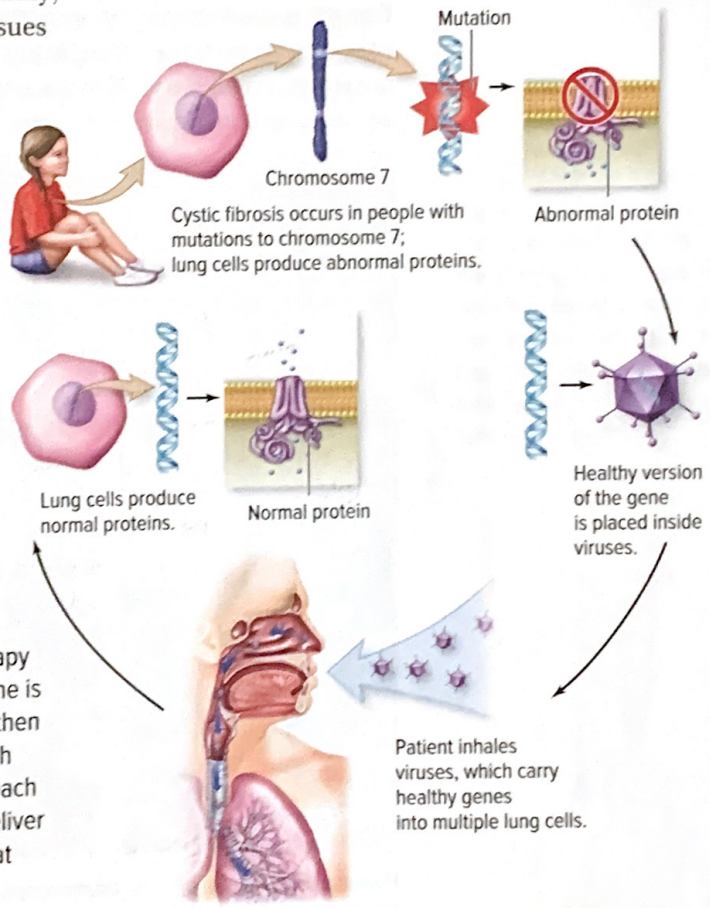

Figure 1.38 The strategy for using gene therapy to cure cystic fibrosis. A normal copy of the gene is taken up by cells in the lungs. These cells can then produce a normal protein from that gene, which can carry out its regular functions. In the approach shown, viruses act as vectors that carry and deliver the gene. The viruses have been treated so that they do not cause illness.

Extending the Connections

Gene Therapy for Donor Lungs

Scientists at the McEwen Centre for Regenerative Medicine in Toronto have developed a gene therapy technique to repair donor lungs before they are transplanted. The technique dramatically improves the success of transplants. As a result, more donor lungs are available to people who are waiting on transplant lists. Read the information provided by your teacher about this technique. Decide, from an ethics standpoint, whether this type of gene therapy is in the same category as treating the actual genetic material of an individual. Write a paragraph that summarizes your opinion, including supporting details.

Concerns about Gene Therapy

Scientists have carried out experimental treatments of genetic disorders aside from cystic fibrosis. These include treatments for sickle cell anemia (Figure 1.42) and some forms of inherited blindness. Although gene therapy has helped some people, others have experienced negative results. This caused most of the experimental treatments to be stopped until safer procedures could be developed. Nevertheless, gene therapy still holds great promise as a treatment for genetic disorders. As well, people are concerned about which genetic conditions should be considered "disorders" that deserve treatment.

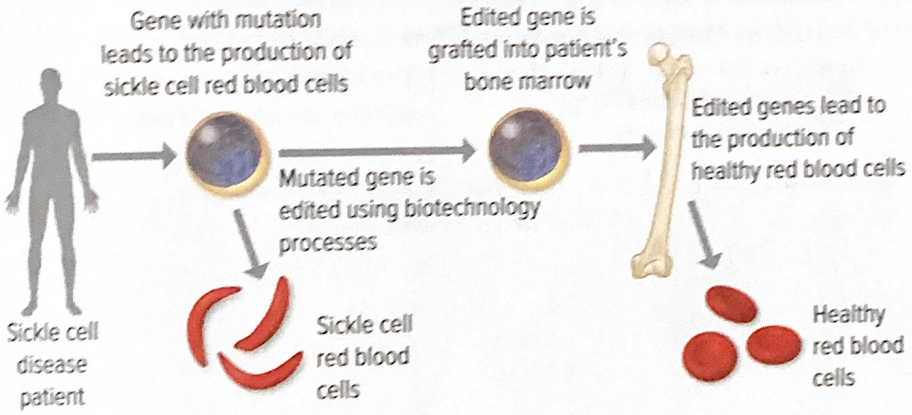

Figure 1.42 Gene therapy used to treat sickle cell anemia

Activity

Should we or shouldn't we?

Consider the scenario that has been presented by your teacher. In preparation for a debate, develop an argument for both sides of the issue. Your teacher will assign you a side to argue for in the debate. Carry out the debate according to the rules provided by your teacher.

Before you leave this page . . .

1. Why should environmental, social, and economic issues be considered when deciding how to use biotechnology?
2. Discuss one thing that concerns you about the use of biotechnology. Justify your concern with evidence collected from this concept.