17.1G: Biotechnology in Medicine

From manipulation of mutant genes to enhanced resistance to disease, biotechnology has allowed advances in medicine.

Learning Objectives

• Give examples of how biotechnology is used in medicine.

Key Points

• The study of pharmacogenomics can result in the development of tailor-made vaccines for people, more accurate means of determining drug dosages, improvements in drug discovery and approval, and the development of safer vaccines.

• Modern biotechnology can be used to manufacture drugs more easily and cheaply, as they can be produced in larger quantities from existing genetic sources.

• Genetic diagnosis involves the process of testing for suspected genetic defects before administering treatment through genetic testing.

• In gene therapy, a good gene is introduced at a random location in the genome to aid the cure of a disease that is caused by a mutated gene.

Key Terms

• **gene therapy**: any of several therapies involving the insertion of genes into a patient’s cells in order to replace defective ones
• **pharmacogenomics**: the study of genes that code for enzymes that metabolize drugs, and the design of tailor-made drugs adapted to an individual’s genetic make-up

• **immunodeficiency**: a depletion in the body’s natural immune system, or in some component of it

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**Biotechnology in Medicine**

It is easy to see how biotechnology can be used for medicinal purposes. Knowledge of the genetic makeup of our species, the genetic basis of heritable diseases, and the invention of technology to manipulate and fix mutant genes provides methods to treat the disease.

Pharmacogenomics is the study of how the genetic inheritance of an individual affects his/her body’s response to drugs. It is a coined word derived from the words “pharmacology” and “genomics”. It is, therefore, the study of the relationship between pharmaceuticals and genetics. The vision of pharmacogenomics is to be able to design and produce drugs that are adapted to each person’s genetic makeup. Pharmacogenomics results in the following benefits:

1. Development of tailor-made medicines. Using pharmacogenomics, pharmaceutical companies can create drugs based on the proteins, enzymes, and RNA molecules that are associated with specific genes and diseases. These tailor-made drugs promise not only to maximize therapeutic effects, but also to decrease damage to nearby healthy cells.

2. More accurate methods of determining appropriate drug dosages. Knowing a patient’s genetics will enable doctors to determine how well the patient’s body can process and metabolize a medicine. This will maximize the value of the medicine and decrease the likelihood of overdose.

3. Improvements in the drug discovery and approval process. The discovery of potential therapies will be made easier using genome targets. Genes have been associated with numerous diseases and disorders. With modern biotechnology, these genes can be used as targets for the development of effective new therapies, which could significantly shorten the drug discovery process.

4. Better vaccines. Safer vaccines can be designed and produced by organisms transformed by means of genetic engineering. These vaccines will elicit the immune response without the attendant risks of infection. They will be inexpensive, stable, easy to store, and capable of being engineered to carry several strains of pathogen at once.

Modern biotechnology can be used to manufacture existing drugs more easily and cheaply. The first genetically-engineered products were medicines designed to combat human diseases. In 1978, Genentech joined a gene for insulin with a plasmid vector and put the resulting gene into a bacterium called *Escherichia coli*. Insulin, widely used for the treatment of diabetes, was previously extracted from sheep and pigs. It was very expensive and often elicited unwanted allergic responses. The resulting genetically-engineered bacterium enabled the production of vast quantities of human insulin at low cost. Since then, modern biotechnology has made it possible to produce more easily and cheaply the human growth hormone, clotting factors for hemophiliacs, fertility drugs, erythropoietin, and other drugs. Genomic knowledge of the genes involved in diseases, disease pathways, and drug-response sites are expected to lead to the discovery of thousands more new targets.
Genetic Diagnosis and Gene Therapy

The process of testing for suspected genetic defects before administering treatment is called genetic diagnosis by genetic testing. Depending on the inheritance patterns of a disease-causing gene, family members are advised to undergo genetic testing. Treatment plans are based on the findings of genetic tests that determine the type of cancer. If the cancer is caused by inherited gene mutations, other female relatives are also advised to undergo genetic testing and periodic screening for breast cancer. Genetic testing is also offered for fetuses to determine the presence or absence of disease-causing genes in families with specific, debilitating diseases.

Genetic testing involves the direct examination of the DNA molecule itself. A scientist scans a patient’s DNA sample for mutated sequences. There are two major types of gene tests. In the first type, a researcher may design short pieces of DNA whose sequences are complementary to the mutated sequences. These probes will seek their complement among the base pairs of an individual’s genome. If the mutated sequence is present in the patient’s genome, the probe will bind to it and flag the mutation. In the second type, a researcher may conduct the gene test by comparing the sequence of DNA bases in a patient’s gene to a normal version of the gene.

Gene therapy is a genetic engineering technique used to cure disease. In its simplest form, it involves the introduction of a good gene at a random location in the genome to aid the cure of a disease that is caused by a mutated gene. The good gene is usually introduced into diseased cells as part of a vector transmitted by a virus that can infect the host cell and deliver the foreign DNA. More advanced forms of gene therapy try to correct the mutation at the original site in the genome, such as is the case with treatment of severe combined immunodeficiency (SCID).

Figure 1: Gene Therapy: Gene therapy using an adenovirus vector can be used to cure certain genetic diseases in which a person has a defective gene.