FDA 101: Human Gene Therapy

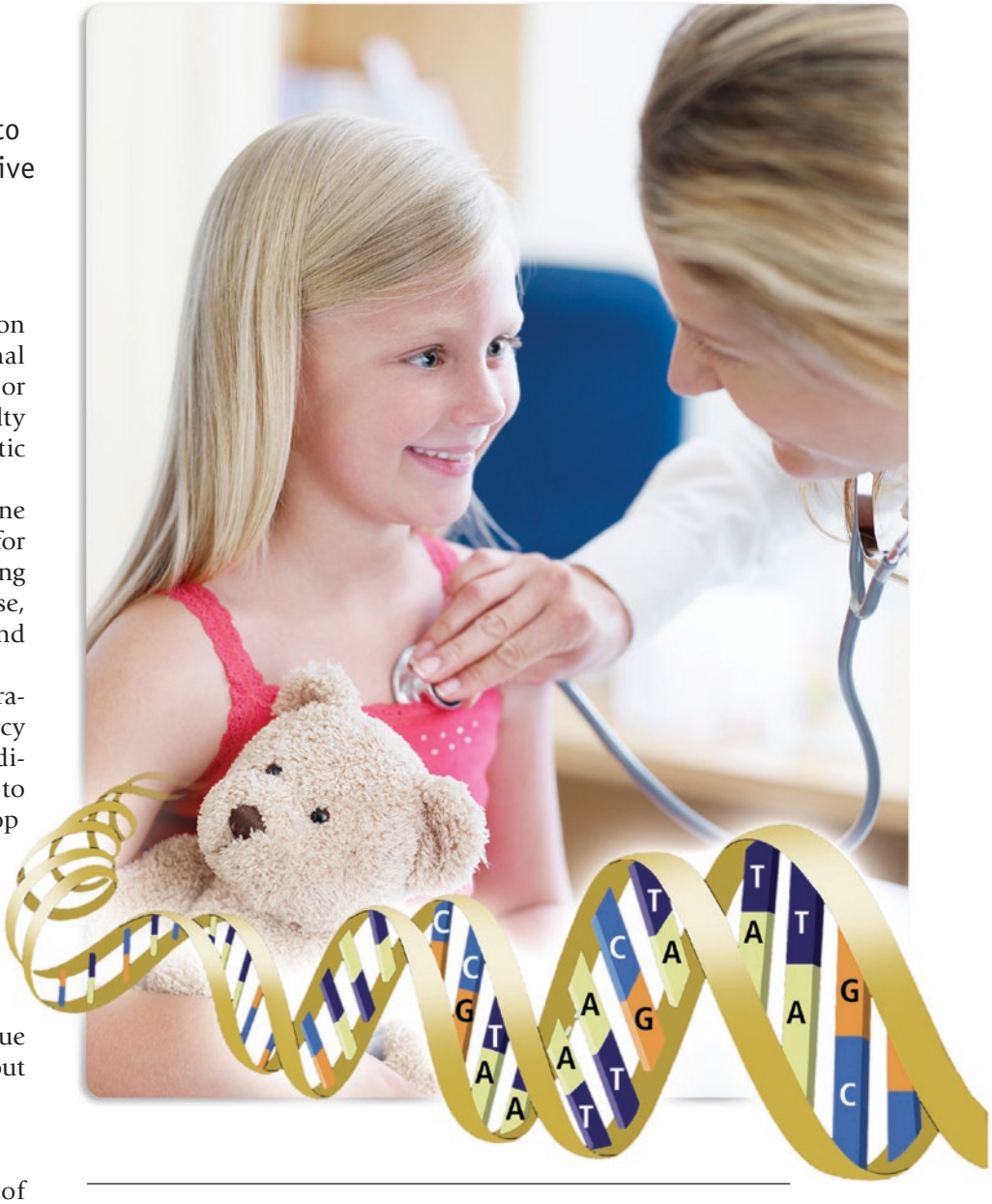
Human gene therapy, although still in the developmental stages, offers tremendous potential to pave the way for more effective treatments for many serious medical conditions.

Human gene therapy is based on the theory that disease and abnormal medical conditions can be cured or treated by replacing patients' faulty genetic material with normal genetic material.

Scientists hope their work will one day lead to gene-based treatments for a wide range of conditions, including cancer, cystic fibrosis, heart disease, diabetes, hemophilia, wounds, and infectious diseases such as AIDS.

FDA regulates human gene therapies in the United States. The agency receives many requests from medical researchers and manufacturers to study gene therapy and to develop gene therapy products.

FDA has not yet approved any human gene therapy products for sale. There is still much to learn about how these products work, how to administer them safely, and whether the cells will continue to work properly in the body without causing adverse side effects.



Passed on from parents to children, genes are small pieces of information that tell our bodies how to operate. With gene therapy, normal genes are used to cancel out defective ones responsible for diseases or other medical problems.

How Does Gene Therapy Work?

Human genes can be thought of as extremely small pieces of informa¬tion that are present in our bodies by the trillions and tell our bodies how to operate. They are

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embedded in our deoxyribonucleic acid (DNA), the molecular structure inside the nucleus of a cell that carries the genetic instructions for making living organisms.

Passed on from parents to children, genes also determine certain physical characteristics such as eye color.

Genes may also determine the likelihood that people will develop a disease or suffer from a medical condition that is prevalent in their family history.

With human gene therapy, normal genes are used to replace or cancel out the defective genes that are responsible for a disease or medical problem. The "good" genes would then find their way to the right spot in the body and begin to do the work required.

FDA's Role

FDA's responsibility is to ensure that drugs, medical devices, and biological products (such as blood and vaccines) used in the United States are safe and effective.

Manufacturers of gene therapy products must test their products extensively and meet FDA requirements for safety, purity, and potency if they wish to sell their products in the United States.

Before studying a gene therapy product in people, the researcher or company testing the product (the "sponsor") must first obtain an Investigational New Drug Application (IND) from FDA. An IND outlines what the sponsor proposes for human testing in clinical trials.

Upon reviewing the IND, FDA decides, based on the scientific data available, whether it is reasonably safe for the drug developers to move forward with the testing.

As part of the IND process, the sponsor

- provides information on how the study will be conducted, what possible risks may be involved, and what steps it will take to protect patients
- provides data in support of the study
- informs prospective participants about the potential risks and benefits
- obtains the consent of prospective participants

Another important part of the IND process is gaining approval from an Institutional Review Board (IRB) and an Institutional Biosafety Committee (IBC). Both are boards or committees that ensure appropriate steps are taken to protect the rights and welfare of participants in studies.

Composed of scientists, doctors and lay people, IRBs can approve or disapprove clinical trials proposed to take place within their jurisdiction—usually a hospital.

The National Institutes of Health Recombinant DNA advisory Committee (RAC) also reviews gene therapy clinical trial proposals.

Further information regarding the RAC process and IBC's can be found at *www4.od.nih.gov/oba/Rdna.htm*.

Looking Ahead

Continued research, along with advancements in other cutting-edge scientific endeavors, can make human gene therapy a versatile treatment strategy at the forefront of a new age of medicine.

Potential treatments could be administered to patients through a variety of techniques. Vaccination-like injections, or possibly microscopic mechanical devices—the result of nanotechnology research—could be used to deliver normal genes into a person's body.

It is possible that certain diseases such as hemophilia could be cured or treated throughout a person's lifetime by replacing faulty genes with normal ones. Genes may also be used to offer protection for a short time, as in cases involving wounds or the need for new blood vessels.

In the future, doctors may one day be able to turn off a "faulty" gene to stop the growth of a cancerous tumor. A faulty gene could also be replaced to produce necessary proteins or enzymes that may be deficient in a patient to ensure and maintain proper body functions.

Ensuring a Safe Approach

As researchers strive toward these goals, FDA wants the public to know that patient safety is the agency's highest priority. The agency will continue to work with manufacturers and medical researchers to ensure that new gene therapy products are as safe as possible, and that people enrolled in studies will not be exposed to known unreasonable risks or harm. FDA

For More Information

Cellular & Gene Therapy (CBER)
www.fda.gov/cber/gene.htm

Human Gene Therapies:
Novel Product Development
Q & A with Celia M. Witten, Ph.D., M.D.
www.fda.gov/consumer/updates/genetherapy101507.html