New Horizons in Treating Disease
A Medical Revolution Begins
Getting Healthy Genes Into Cells
Vectors: Vehicles for Transferring Genes
Gene Therapy for Cancer and Infectious Disease
Should We Change Our Genes?
Gene Therapy Opens New Horizons in Treating Disease

A Medical Revolution Begins

Getting New Genes into Cells

Vectors: Vehicles for Transferring Genes

Profile
Mariann Grossman: A Pioneer in Gene Therapy

The First Success in Gene Therapy: Helping a Woman Fight Deadly High Cholesterol

Gene Therapy for Cancer and Infectious Diseases

Should We Change Our Genes?

Class Discussion
Ethical Problems in Genetics

Cover:
Gene therapy has made the difference between a life of isolation in a sterile “bubble” and an active life. Two of the children on the cover have a genetic disease of the immune system. With gene therapy, a new gene is inserted into a cell. The new gene’s DNA causes the cell to produce a protein that it would not otherwise make. That new protein helps treat or cure the disease.

Credits:
Top right: Baylor College of Medicine
Top left: March of Dimes Birth Defects Foundation
Center: The Institute for Human Gene Therapy, University of Pennsylvania
Since we humans have over 100,000 genes you wouldn't think that one or two would make a big difference. In fact, though, even a single gene can make the difference between a healthy body and one with a serious genetic disease.

Scientists have identified more than 4,000 genetic diseases caused by single defective or missing genes, and they are finding more all the time. These diseases can affect any part of the body, from the way we grow to the way our heart works, and they can affect organs throughout the body.

Many genetic diseases are inherited from one or both parents. Others are caused by changes in a person's DNA. Most individual genetic diseases are rare by themselves, but there are so many diseases that taken all together they are common, and they cause tremendous suffering. Until recently, there was no way to treat most genetic diseases. Now we are learning to treat them by changing the genes that cause them.

Gene therapy is the technique of adding genes to certain cells to change the way those cells work. In addition to treating genetic diseases, gene therapy may also help treat cancer, heart disease, AIDS, and other conditions. In this issue of Your World/Our World, you will read about several experiments -- and one successful treatment -- using gene therapy. The experiments are called "clinical trials," and we do not yet know if they will be successful.

Many people are excited about the potential of gene therapy to relieve human suffering, but they are also concerned about the potential to misuse the technology. This issue will help you understand the benefits of gene therapy, and it will encourage you to think about the challenges of defining its ethical use.

These two men both died of genetic diseases. Lou Gehrig had ALS (amyotrophic lateral sclerosis), now known as Lou Gehrig's disease, which destroys nerves in the spinal cord. Woody Guthrie had Huntington's disease, which destroys brain cells.

The human body has over 100,000 different kinds of cells.
In 1984, a twelve-year-old boy known worldwide as the “boy in the bubble” died of a rare genetic disease that left him with no immunity against even common germs. He had a genetic disease called severe combined immunodeficiency or SCID that completely crippled his immune system. To keep from being exposed to germs, the boy spent his entire life in a sterile, air-tight “bubble” chamber. This bubble helped him live longer than any other child with SCID had lived.

When the families of two baby girls, Ashanthi DeSilva and Cynthia Cutshall, learned that their children had a form of SCID, they sadly envisioned a short, protected life for their daughters. Both girls had a defective gene that did not make an enzyme called adenosine deaminase (ADA). Without this enzyme, the white blood cells (or immune cells) die, and the body cannot fight off infection.

Then in the early 1990s, these two girls became the first children ever to receive experimental gene therapy for SCID. The gene therapy added a healthy gene for ADA to their immune cells in hopes that they could develop a normal immune system.

In early 1994, magazines and newspapers covered the story of this apparently successful experiment. Ashanthi, then six years old, and Cynthia, who was eleven, were attending school and leading normal lives. (Researchers must still study whether their healthy immune systems are the result of the gene therapy or of other drugs they receive.) You can read about the method used for this gene therapy on pages 6 and 7 in this issue.
Recessive Inheritance Pattern

INHERITING A DISEASE CAUSED BY A RECESSIVE GENE

Sickle cell anemia is a common recessive disease among African Americans. It is related to a number of other diseases of the red blood cells caused by defects on the beta-hemoglobin gene. In this case, the gene produces a defective form of beta-hemoglobin protein. This defective protein leads to misshapen red blood cells that cannot flow through the small blood vessels (capillaries) as easily as normal cells, resulting in pain and injury to vital tissues.

Developing a gene therapy for sickle cell anemia is particularly important, since it affects so many people and since existing medical technology cannot treat all of its symptoms. However, this disease poses some unusual challenges for gene therapy. The amount of replacement beta-hemoglobin produced by a transferred gene must be very carefully regulated. Too little provides no benefit, while too much causes other problems with red blood cells. The ongoing research in sickle cell gene therapy may lead to better treatment of this and related blood disorders.

Sickle cell anemia causes the red blood cells, which are normally disc shaped, to develop a "sickle" shape. These misshapen cells cannot flow through the small blood vessels (capillaries) as easily as normal cells.

Abnormal hemoglobin genes, like the sickle cell gene, are common in parts of the world where malaria is also common. Malaria is a disease caused by a parasite that infects the red blood cells. Why do you think that malaria and diseases of hemoglobin are found in the same parts of the world? Could there be an advantage to being a carrier of an abnormal hemoglobin gene?
How do we replace a disease-causing gene with a healthy gene? First, we must find out where the gene becomes active. For instance, the gene that makes the ADA enzyme is in every cell of the body. However, when it is missing only the white blood cells malfunction; the other cells in the body work normally. Next, we must insert the new gene into the cells that need it. Just getting the ADA gene into any cell, for example, will not treat SCID; the gene must get into the white blood cells.

Removing Cells for Genetic Transfer
To treat the two young girls with SCID, researchers first removed some of their white blood cells and put a normal ADA gene into those cells. In a laboratory they cultured (grew) millions of these engineered cells. Then they injected them back into the girls’ blood in hopes that the altered immune cells would produce the ADA enzyme and build a healthy immune system.

We call this kind of gene therapy *ex vivo* or external, because the genetic transfer takes place outside the patient's body. Other genetic diseases may be treated *in vivo* or inside the body. *In vivo* methods are used when the cells that need treatment cannot be removed from the body.

Transferring Genes Inside the Body
One of the genetic diseases researchers are trying to treat with *in vivo* methods is cystic fibrosis. The CF gene leads to the build-up of a very thick mucus in the lungs, causing life-threatening infections and lung damage. To relieve these problems, experimental gene therapy for CF is targeting the cells in the lining of the lung. Since these cells cannot be removed, researchers put the healthy gene directly into the airways of the lungs. They hope the cells in the lungs will take in the new gene and make normal mucus.

You will read about how the healthy gene gets transferred to the cells on pages 8 and 9.

Are there any conditions in your family that might be inherited? Are there any illnesses that have shown up in several generations? Make a family tree of as many generations as you can, and show these illnesses. Do you think these illnesses are caused by inherited genes? By the environment your family lives in? By your family's lifestyle? Or a combination of factors?
**Career Tip: Nurse Research Coordinator**

Nurse research coordinators are responsible for recruiting and organizing patients who volunteer for a clinical trial. They make sure data on all patients are collected properly, recorded, and kept confidential. These nurses are the lifeline of any clinical trial because they are the link between science and the patient. They usually have an R.N. (registered nurse) degree, or an R.N. with M.S. (Masters of Science) degree.

**Career Tip: Genetic Counselor**

Genetic counselors understand the science of inherited diseases, and they know how to calculate the chances of you or your children inheriting a disease. They also help people deal with the emotional difficulties of discovering they have a genetic disease in their family. As our scientific knowledge about genetic diseases grows, our ability to understand how to deal with this information must also grow. For that reason, genetic counselors are becoming increasingly important resources.

**Career Tip: Research Doctor**

Developing new treatments for gene therapy requires a combination of very specialized skills – the technical knowledge of a Ph.D. research scientist who works mainly in a laboratory, and the medical knowledge of an M.D. who deals directly with patients. People working on genetic therapies must understand both how to transfer genes to cells and how the human body responds to disease and genetic treatment. Many research doctors have a dual degree: M.D., Ph.D.

---

**IN VIVO**

Genetic transfer takes place inside the body.

1. Cells are removed from the body.
2. In a laboratory a new gene is inserted into the cell.
3. Many copies of the cell with the new gene are grown.
4. The engineered cells are put back in the body.

**EX VIVO**

Genetic transfer takes place outside the body.

The new gene is inserted into the cells inside the body.

1. Cells are removed from the body.
2. In a laboratory a new gene is inserted into the cell.
3. Many copies of the cell with the new gene are grown.
4. The engineered cells are put back in the body.
Once we know which cells need to get a healthy gene, how do we get it there? We use a delivery vehicle called a vector. In the world of transportation, a good delivery vehicle has to drive to the right address (without accidents) and deliver its package (in good shape) to the right person. Likewise, in gene therapy a good vector has to get to its target cells without disturbing other cells along the way. Then it has to transfer its genetic package to the cell. There are different kinds of vectors for different kinds of jobs.

**Adenovirus for Internal Treatments**

If you wanted to get a new gene to the cells of the lining of the lungs, what vehicle might you use? Use one that already goes there! For example, the adenovirus (pronounced ad-no-virus) is a common cold virus that regularly infects the lungs. When it attacks the cells of the lungs, it naturally delivers its DNA to the cells.

Researchers are using these traits of the adenovirus to develop an experimental gene therapy for cystic fibrosis. They first deactivated the adenovirus so it can no longer cause disease or grow. Then they inserted a copy of the healthy CF gene into the virus. When put into the lungs, this altered virus would “infect” the lungs. Instead of catching cold, though, the patients would catch the healthy gene!

No one knows yet if this treatment will work, or what kind of problems this viral delivery vehicle might cause. After repeated doses, the body’s immune system could build up a defense against the adenovirus and reject it. The new genes could be absorbed by the wrong cells before they reach the lungs. They could disrupt the cells’ other functions.

**Retrovirus for External Treatments**

By working on cells outside the body, researchers can avoid the twin problems of the immune system’s interference and misdirected deliveries. Many experiments in external treatments use another kind of deactivated virus – called a retrovirus – as a vector. Retroviruses are used for *ex vivo* treatments because they only infect cells that are dividing. When treated outside the body, the targeted cells are grown in a culture, so they are rapidly dividing. The retrovirus quickly enters these dividing cells and delivers the new gene.

Retroviruses would not have time to work in *in vivo* gene therapy because the body’s immune system rapidly attacks most retroviruses. Even the fast-growing cells of a tumor are not dividing fast enough for the retrovirus to enter them before it gets overwhelmed by the immune system.

**Non-Viral Vectors**

Because viruses and retroviruses both have limitations as vectors, researchers are also working to build other kinds of gene delivery vehicles. Some researchers, for example, are putting a gene inside a bubble of fat called a “liposome.” Other researchers are wrapping strands of DNA with an altered gene around microscopic pellets of gold or titanium. Then they use a gene gun to “shoot” the pellets directly into the targeted cells. Scientists hope to use this method for *in vivo* treatments of skin cancer, since the tumor cells are close to the surface. They also expect to use it for various *ex vivo* therapies.
Career Tip: Cell Libraries for Gene Therapy Research

To discover the genetic cause of a disease, researchers study cells from people who have that disease. Suppose you wanted to find the genes responsible for the incurable Huntington's disease, but didn't know anyone with it. You might call a special kind of research library that collects cells from people with different diseases. These libraries then culture or grow the cells to create a “cell line.” You could order a cell line from a patient with Huntington's disease. Libraries for cell lines have made it easier for researchers to study different diseases. Without them, it would have taken longer to discover the genes responsible for cystic fibrosis, juvenile diabetes, Huntington’s disease, and others.

Cell line libraries are staffed by experts in microbiology, molecular biology, cytogenetics, cell biology, and molecular genetics, as well as library science.

During the Trojan War in the 12th century B.C., Greek soldiers tried for ten years to break into the walled city of Troy. Finally they pretended to give up and left a large wooden horse as a gift for Troy. The Trojans opened their gates to let in the horse, not realizing that Greek soldiers were hidden inside. A ferocious battle followed, and the Greeks conquered Troy.

In a similar strategy, we use vectors to trick the cell into letting new genes through the cell “wall” or membrane. We hide the genes inside a virus or other vehicle that already has found a way to get into the cell.

A gene gun is used to bombard cells with gold or titanium pellets that have been wrapped with strands of DNA. The vector carrying the gene is put into an acceleration tube. Helium gas creates pressure. When the pressure reaches a precise point, a special disk breaks, sending the pellets hurtling to the cells fast enough to break through the cell membrane.

Collections of living human cells, such as the one stored here, serve the entire scientific community.
Mariann Grossman’s large Minnesota family considered her a pioneer, since no one in her family had ever shown a strong interest in science. Now, she is on the cutting edge of gene therapy research as director of one of the laboratories at The Human Gene Therapy Institute at the University of Pennsylvania.

Mariann went to college at the University of Minnesota in the 1980s. In her third year, she worked in the bone marrow transplant unit of the university’s hospital where she saw many patients with inherited genetic diseases. She saw that bone marrow transplant therapy did not always work for these people, and she felt there must be a better treatment for them. Her research led her to human gene therapy.

After college, Mariann accepted a job in a lab at the Whitehead Institute for Biomedical Research in Cambridge, Massachusetts. There she started working with a young research scientist in gene therapy named James Wilson. Since then, she has conducted research and collaborated on projects with Dr. Wilson at the Whitehead Institute, the University of Michigan, and now the Human Gene Therapy Institute at the University of Pennsylvania. She might go to graduate school one day, but she’s not ready to leave her position. “I love what I am doing,” she says. “I am learning both medicine and research. It is especially thrilling to work with patients who I may actually be able to help.”

Mariann and her colleagues have made some remarkable breakthroughs. They were the first research team to treat successfully a rare genetic disease called Familial Hypercholesterolemia (FH), which you can read about on the next page. When they began this research, every new effort was a step into the unknown, and they had to invent new laboratory strategies and techniques at every turn. For example, to treat FH they wanted to add a healthy cholesterol-related gene to the liver cells. However, very few people had ever tried to isolate human liver cells. Mariann devised a way. Then she found ways to keep the liver cells alive and grow them in a culture. Finally, she helped develop a vector to get new genes into the cells. Her colleagues often say that Mariann has a “green thumb” with cells, the way some gardeners have a special knack with plants.

Mariann’s work is completely absorbing, but she does find time to keep up with professional sports and with her own athletic loves, swimming and dancing. She also collects CDs of every kind of music, from classical to rock, jazz, and new age. Mariann is particularly excited because she is no longer the only scientist in her family. Her brother has enrolled in an M.D., Ph.D. program in gene therapy at Washington University in St. Louis. Her enthusiasm is obviously contagious.
The First Success in Gene Therapy:

Helping a Woman Fight Deadly High Cholesterol

The first proven successful treatment using gene therapy was on a 29-year-old woman who had a rare genetic disease called familial hypercholesterolemia or FH. “Familial” means that it is passed through one’s family, or is genetic. “Cholesterol” is a type of fat; if the body does not break it down properly, it will build up in blood vessels, clogging them and causing heart disease or heart attacks. The prefix “hyper” means there is too much cholesterol; the suffix “emia” means “in the blood”. So given that information, how would you describe the disease familial hypercholesterolemia?

FH patients often die of heart attacks as young adults or even as children. These people have a defective gene that does not produce a low-density lipoprotein (LDL) receptor in the liver. A receptor is a special cell part that combines with chemicals and allows them to enter cells where they can be used or disposed of. Without the LDL receptor, the liver cannot break down cholesterol. To fight this disease, Dr. James Wilson and Mariann Grossman (see previous page) found a way to insert a gene for the LDL receptor into liver cells.

The first woman to receive this gene began producing enough LDL receptors to lower her cholesterol to a less dangerous level. After the operation she said, “Now I hope to live to see my daughter graduate from high school.” Since then Dr. Wilson’s team has treated other patients with FH, including several young children.

Each treatment requires a major, risky operation – and a lot of drama. Surgeons carefully remove part of the liver and rush it to Mariann Grossman in her lab. She can’t lose a second. She must keep the liver cells alive while she loosens them into separate cells. She divides them into 1,500 separate petri dishes. For the next three days, these liver cells divide until there are billions of them. Then they are bathed with a retrovirus that contains the gene for the LDL receptor. The treated cells are then injected into a vein in the patient that leads to the liver. If every part of the operation works, the liver will soon begin to break down cholesterol in the blood.

Dr. Wilson’s team is thrilled that this method may be helping to save lives, but they want to find other ways to get new genes into the liver that do not require such difficult surgery. They hope to find a vehicle to take the new gene directly to the liver cells. Then they could treat more people with different kinds of cholesterol problems and liver disorders. The work of gene therapy is only just beginning.

Gene Therapy for Familial Hypercholesterolemia:

1) Doctors remove 15% of the patient’s liver.

2) These liver cells have an abnormal LDL receptor gene. They are separated and cultured into billions of liver cells in a laboratory. (Not shown in illustration.)

3) A retrovirus vector is used to transfer the gene for the LDL receptor to cultured liver cells.

4) The cultured cells now have normal LDL receptor genes.

5) The cultured liver cells are injected into a vein that leads to the liver. Liver cells begin to make the LDL receptor, and the patient’s level of harmful cholesterol drops.
Gene therapy may one day be used for much more than adding a healthy gene to treat inherited diseases. It might also be used to treat some cancers and infectious diseases. In these cases, scientists may add a gene that has nothing to do with what caused the disease. Instead, the new gene would change the way the disease works.

**Fighting Cancer...**
A common treatment for cancer is chemotherapy. With chemotherapy, patients receive a high dose of strong drugs that kill the fast-growing cancer cells. The proper dose for chemotherapy is a delicate balance: too much and the healthy cells die; too little and the tumor cells survive. Gene therapy could help make this balance easier to achieve.

*...by Strengthening the Guards...*
Chemotherapy can kill the bone marrow cells and weaken the immune system. Researchers are looking for ways to keep the immune cells healthy. In one experiment, they remove some bone marrow cells before chemotherapy. They give the cells a gene – called the Multiple Drug Resistant Gene, or MDR – that guards them against the toxic effects of chemotherapy drugs. Then they culture billions of these treated bone marrow cells. After the patient receives chemotherapy, researchers put the cells with the MDR gene back into the patient. They hope these cells with MDR will survive and produce healthy immune cells, while the tumor cells die.

*...and Weakening the Tumor*
Other experiments use gene therapy to send in “suicide” genes to kill tumor cells in patients with incurable brain cancer. Researchers use a deactivated virus that carries the gene for an enzyme called thymidine kinase (TK). The virus makes this enzyme inside the cell it has invaded. The enzyme kills cells that are dividing when they are exposed to an anti-viral drug called ganciclovir (gän-si-klö-vear). The cell is said to “commit suicide” since, with the new gene, it makes a protein that leads to its own destruction. Researchers first give the patient the virus with the suicide gene and then the ganciclovir. Although some non-tumor cells may have taken up the TK gene and are exposed to ganciclovir, only the tumor cells are actively dividing. Since the ganciclovir only kills dividing cells, scientists hope the treatment will selectively kill the brain tumor, but not the healthy cells.

**A Dead End for the AIDS Virus**
The retrovirus known as HIV (Human Immunodeficiency Virus) causes the deadly disease AIDS. So far, it has out-tricked all the drugs and vaccines that we have thrown at it. Some researchers now think that we may be able to treat AIDS by using gene therapy to interfere with the way HIV reproduces itself.

When HIV infects a cell, it makes a protein called “rev.” The rev protein helps HIV reproduce; without it, the infection cannot spread. Some researchers are putting a gene for an altered form of rev into HIV. This altered rev keeps the normal rev protein from working, so the virus cannot reproduce. Other researchers are developing other ways to disrupt HIV’s reproduction.
Cancer and Genetics
Some forms of cancer seem to run in families. Why do some people inherit the tendency to develop a particular cancer? Genetic researchers are discovering genes that are linked to certain forms of colon cancer, breast cancer, skin cancer, and others.

In the future, scientists will probably develop genetic screening tests for these genes. For people who have a family history of certain cancers, these tests may either relieve their fear by showing that they do not carry the gene – or confirm that they have a high risk of developing cancer. Learning that they carry a cancer gene can encourage people to take better care of themselves and to detect their cancer at an early, more treatable stage.

Genetic testing may one day identify genes associated with many types of diseases. Many people are concerned that genetic test results could be misused. What would happen if health insurance companies required genetic tests for people applying for insurance? They might refuse to cover people with “suspicious” genes, or charge them higher premiums. What if employers refused to hire these people? Do we have a right to genetic privacy?
Every advance in science presents society with choices about how we use the new knowledge we have gained. For example, think of how we use the computer. What if society had decided that there should be no personal computers, or that computers could only be used for business and that games were illegal? Similarly, society must make choices about how we use biotechnology.

For example, should we use biotechnology to change something about ourselves that we do not like, such as our hair, our eyes, our size, our memory, or our athletic ability? Have you ever said to yourself, “If only I could change my....”? Depending on how society guides the use of gene therapy, kids in the future may have that chance.

Gene therapy is already trying to treat previously untreatable diseases. As we learn more about human genetics, we may also be able to use gene therapy to select what traits we will have. Should we?

Many people agree that we ought to use gene therapy to treat a fatal genetic disease. However, the line between a genetic disease and a genetic difference can be very hard to draw. Should we try to “cure” baldness, deafness, blindness, color blindness, dwarfism, or other non-fatal conditions? What differences should we try to change? Some people think we should only use gene therapy to change things that affect our health, and not for “personal enhancement” to improve our looks or abilities. They warn that using gene therapy to “improve” people could lead to “social engineering” to encourage certain traits and eliminate others. We might become followers of a “genetic fashion” and everyone would be the same. Others wonder whether there is any ethical difference between traditional plastic surgery to change a person’s cheek bones and gene therapy to change a person’s eye color.

Some people fear that gene therapy may one day be used to oppress certain groups. History has many examples of people who tried to create a “master class” or to “cleanse” ethnic differences in their populations. What might they have done with gene therapy?

To safeguard against the potential abuse of gene therapy, some people want to limit gene therapy research to treating disease. Others want to prohibit research into gene therapy on the reproductive cells that would carry genetic changes into future generations. (At present, however, there is no technology even to begin such research; all genetic therapies now and for the foreseeable future are limited to the non-reproductive cells of a single individual.) Is it appropriate to limit these areas of research? Is it possible?

The debate about the ethics of gene therapy will continue for as long as people push the frontiers of knowledge about the human genetic code. During your lifetime, gene therapy may affect you in many ways. You can become an informed participant in the ongoing effort to set guidelines for this powerful new medical tool.
The following cases give examples of dilemmas that might grow out of our use of genetics. What do you think is the ethical way to handle these situations?

It helps to use principles or guidelines when making ethical decisions. For example, should decisions be based on individual well-being? The prevention of harm to yourself or others? Fairness? Justice? Truth? Personal independence? What principles might you use to make an ethical decision?

There are many differing viewpoints on these questions. Our society has not yet decided what direction to take. Read these cases, and reflect on what direction you think we should take.

Case 1: Treating Familial Hypercholesterolemia with Gene Therapy
The year is 1997. Five-year-old Lorese has familial hypercholesterolemia (see page 11). Gene therapy could give her a more normal cholesterol level and a longer life; however, it is very expensive.

Do you think Lorese should be able to have gene therapy? Who should be involved in deciding? Parents? Doctors? Should cost or the question of who pays be a factor?

Case 2: I Want To Play Basketball!
The year is 2001. Harold is the shortest kid in his class. He makes good grades, has many friends, and is a superb athlete. His favorite sport is basketball, but he knows he could never pursue basketball professionally because he is too short.

Scientists have developed a gene therapy that causes people to grow taller. Harold's personal physician believes that gene therapy should be used only to improve a person's health, not just to help someone grow taller.

Harold knows people who have changed their hair color, the shape of their nose, and who have had surgical face lifts. Harold wants to be tall.

Is there an ethical difference between plastic surgery and gene therapy when they are performed for cosmetic rather than health reasons? Is it ethical for society to allow – or not allow – science to change the genetic structure of an individual?

Case 3: Withholding Information About A Genetic Condition
The year is 2005. Cheri is a recently married 25-year-old eager to start a family. Her father recently died of a genetic disease. This disease does not appear until people are in their thirties, and then they slowly lose their nerve function until they die ten or twenty years later.

Cheri has a 50% chance of carrying this fatal gene, for which there is no treatment or cure for adults. (There is a cure for children, but only if they are treated immediately after birth.) If she does have the gene, each of her children will also have a 50% chance of inheriting the gene. She can find out if she has this gene either by waiting to get symptoms of the disease or through genetic testing.

She does not want to be tested because she does not feel she could handle the possible bad news. She also does not want her future children to be tested, because if they should test positive, she will know that she will come down with the disease herself.

By not testing her children, she will deny them their only opportunity to be cured of the disease if they do carry the gene.

Do you think Cheri has an ethical obligation to test her children? Do you think she should be tested before having children?

Case 4: Who Decides Who Receives Gene Therapy?
The year is 2009. Scientists have developed a low-cost, simple gene therapy to treat one form of hereditary deafness. However, the treatment must be done before a baby is four weeks old. Susan and Juan both have this form of deafness, and so does their newborn baby Eric.

Doctors encourage them to give Eric gene therapy so he will be able to hear. They refuse. They say deafness is not a defect, and that deaf people live fully rewarding lives with a culture of their own. They want Eric to grow up in this culture of deafness, and they do not think mainstream culture should eliminate valuable differences among people.

Is it ethical for Susan and Juan to refuse gene therapy for Eric? ■

Note to Teachers: See Teachers’ Guide for ideas and lesson plans for this activity.
Dear Students:

We are pleased to provide you with this issue of Your World/Our World. We hope you find it an interesting way to learn more about biotechnology. Biotechnology can be important to you for two reasons:

1. During your lifetime there will be tremendous discoveries in this field, and you'll want to understand what those discoveries mean for you, your friends, and your family.

2. You can help make those discoveries if you decide to continue to study science and math.

Either way, we hope you join us in discovering the promise of biotechnology for our world. We are pleased to acknowledge the support of the companies listed. Their support makes this project possible.

Sincerely,

Jeff Davidson
Executive Director
Pennsylvania Biotechnology Association