Doctors try first CRISPR editing in the body for blindness

By Associated Press, adapted by Newsela staff on 03.23.20
Word Count **583**
Level **590L**

Dr. Mark Pennesi, who leads Oregon Health and Science University's involvement in the trial (center right) looks on as staff at school's Casey Eye Institute perform the first-ever in vivo CRISPR gene edit procedure for the BRILLIANCE clinical trial in Portland, Oregon, on a patient who had an inherited form of blindness. It may take up to a month to see if it worked to restore vision. Photo: Kristyna Wentz-Graff/OHSU via AP

Scientists have used CRISPR inside someone's body. This is the first time this has ever been done.

Genes are made up of DNA. Genes tell a specific part of the body how to grow and work. Genes are passed down from parents to children. CRISPR is a specific area of DNA. CRISPR technology is a powerful tool. It is used to edit DNA. Scientists hope to use it to treat diseases.

The treatment took place in Portland, Oregon. It was for an inherited form of blindness. The treatment is supposed to bring back the patient's vision.

Results may take up to a month. Doctors will wait to see if the first attempts are safe. Then they plan to test it on 18 children and adults.

Making People Who Are Blind See

"We literally have the potential to take people who are essentially blind and make them see," said Charles Albright. Albright runs Editas Medicine. The company helped develop the treatment. They worked with another company called Allergan. He is excited about what the treatment can do. "We think it could open up a whole new set of medicines to go in and change your DNA."

Jason Comander is an eye doctor. He works at Massachusetts Eye and Ear in Boston. The hospital plans to put patients in the study. He said the treatment marks "a new era in medicine." CRISPR "makes editing DNA much easier and much more effective."

The people in this study have Leber congenital amaurosis. The condition is caused by a gene error. The body cannot make a protein needed for sight. This condition cannot be treated with regular gene therapy. Gene therapy is when doctors supply a replacement gene. Gene therapy requires genes to be a certain size. The gene needed for this condition is too big.

CRISPR Comes With A Risk

Instead, scientists are trying to cut out the mistake. They are using CRISPR to do this. The hope is that after the treatment, the gene will work as it should.

The treatment takes an hour. Doctors use a tube the size of a hair. They drip three drops of liquid under the retina. The liquid has gene editing tools in it. The retina is a part of the eye. It is the lining at the back of the eye. It is where the cells that sense light are.

"Once the cell is edited, it's permanent," said Eric Pierce. He is a scientist at Massachusetts Eye and Ear.

CRISPR treatment, though, comes with a risk. It might accidentally change other genes. The companies have worked to make sure that does not happen, Pierce said.

Experts were hopeful about the new study.

"The gene editing approach is really exciting. We need technology that will be able to deal with problems like these large genes," said Jean Bennett. Bennett is a researcher at a university.

Kiran Musunuru is also a scientist at a university. He said the treatment should work. Tests on human tissue and animals were successful.

The gene editing tool stays in the eye. It does not travel to other parts of the body. So "if something goes wrong, the chance of harm is very small," he said.

First Time Treatment Was Used Inside Body

The treatment is the first time CRISPR was used inside the body.

Other scientists are using CRISPR to edit genes outside the body. They are trying to treat diseases like cancer.

All of these studies are done with government approval.