First attempt at using CRISPR to edit genes inside the body targets inherited form of blindness

Patients are about to be enrolled in the first study to test a gene-editing technique known as CRISPR inside the body to try to cure an inherited form of blindness.

People with the disease have normal eyes but lack a gene that converts light into signals to the brain that enable sight.

The experimental treatment aims to supply kids and adults with a healthy version of the gene they lack, using a tool that cuts or “edits” DNA in a specific spot. It’s intended as a onetime treatment.

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Two companies, Editas Medicine and Allergan, will test this in up to 18 people around the United States, including Massachusetts Eye and Ear in Boston, starting this fall.

This gene editing in people after birth is different from the controversial work a Chinese scientist did last year — altering the DNA of embryos at conception in a way that can pass the traits to future generations. The DNA changes in adults that the new study aims to make will not be inherited by any offspring.

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[R]esearchers believe [CRISPR] has great potential to cure or treat many diseases caused by gene flaws that have no good treatments now.

Read full, original post: First CRISPR study inside the body to start in U.S.