'It would be huge to see my granddaughter': In an attempt to cure blindness, scientists use CRISPR to edit DNA inside patients' bodies

Carlene Knight would love to do things that most people take for granted, such as read books, drive a car, ride a bike, gaze at animals in a zoo and watch movies. She also longs to see expressions on people's faces.

"To be able to see my granddaughter especially — my granddaughter's face," said Knight, 54, who lives outside Portland, Ore. "It would be huge."

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[Knight is one of] the first patients treated in a <u>landmark study</u> designed to try to restore vision to patients... who suffer from a rare <u>genetic disease</u> [called <u>Leber congenital amaurosis</u>].

The study involves the revolutionary gene-editing technique called <u>CRISPR</u>, which allows scientists to make precise changes in DNA.

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The experiment [Knight] volunteered for marks the first time scientists are using CRISPR to edit DNA when it's still inside patients' bodies.

"This is the very first time that anyone's ever actually tried to do gene-editing from inside the body," said Dr. Lisa Michaels, chief medical officer at the company sponsoring the study, Editas Medicine of Cambridge, Mass. "We're actually delivering the gene-editing apparatus to the part of the body where the disease takes place in order to correct it."

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