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researchers hope they would grow into healthy tissue and thereby restore the person’s vision.

“This would be an example of precision medicine, in that we’d first identify which of the handful of genetic mutations that can cause retinitis pigmentosa is carried by the patient, and then fix it specifically,” says Stephen Tsang ’98PS, who is the László Z. Bitó Associate Professor of Ophthalmology, Pathology, and Cell Biology at Columbia University Medical Center and one of the paper’s senior authors.

CRISPR, which stands for “clustered regularly interspaced short palindromic repeats,” is an adaptation of a natural defense mechanism that some bacteria use to identify and disable the DNA of viral intruders. It has not yet been approved for manipulating the human genome, in part because it can sometimes cause unintended modifications. Tsang and his colleagues are now working to show that their corrected cells are safe for transplantation.

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