Blindness causing gene corrected by CRISPR in stem cells

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion and analysis.

Researchers used CRISPR to correct a blindness-causing genetic defect, the first time a defective gene has been successfully fixed in patient-derived stem cells, which may be the first step toward using gene editing to cure a disease.

The successful correction of a gene causing retinitis pigmentosa by researchers at Columbia University is thought to be the first such use of CRISPR, and a significant step on the way to using gene therapy for personalized treatment of health conditions.

For the study, published in Nature: Scientific Reports, researchers took a sample of skin from a patient with retinitis pigmentosa, using it to create stem cells with the patient's DNA in them.

Using CRISPR, the researchers were able to successfully edit the gene RGPR, which is responsible for the degenerative condition. The gene is especially difficult to edit because it contains many repeats and tight-binding nucleotide pairs. Potentially, the cells can be transplanted back into the patient to treat the condition.

Read full, original post: Blindness-causing genetic defect corrected using CRISPR