Podcast: Fighting blindness with CRISPR. Ophthalmologist in groundbreaking study explains how gene editing could treat a once-incurable disease

Congenital eye disorders can rob children of their eyesight at a young age and severely diminish their quality of life as they grow up. Over 60 percent of cases of blindness among infants are caused by such inherited conditions, and for many years, there was little modern medicine could do. But that's beginning to change as the gene-editing revolution advances, enabling physicians to replace and modify the damaged DNA responsible for these disorders.

In early March, researchers for the first time ever treated a patient born with Leber congenital amaurosis (LCA), a rare genetic condition that causes severe visual impairment, using CRISPR. To administer the therapy, scientists inject a harmless virus carrying the instructions to produce CRISPR-Cas9 directly into the patient's eye, where it is expected to edit out a mutation in the CEP290 gene responsible for the most common subtype of LCA known as Type 10.

Although a gene therapy for a different LCA subtype has been approved by the FDA, the recent effort to correct the mutation in CEP290, if successful, will offer the first treatment for LCA10. The gene-editing procedure also marks the first attempt to edit DNA inside a living patient, signaling a significant step forward in biomedicine.

The groundbreaking treatment raises all sorts of questions, most pressing among them: is the procedure safe, and will it really restore sight? On this episode of Science Facts and Fallacies, geneticist Kevin Folta and GLP editor Cameron English sit down with Dr. Mark Pennesi, Associate Professor of Ophthalmology at Oregon Health and Science University, to get some answers. Pennesi is one of several specialists conducting the BRILLIANCE clinical trial to evaluate the novel CRISPR therapy for LCA10.

Mark Pennesi is a professor of ophthalmology at Oregon Health and Science University who specializes in degenerative retinal disorders. He completed a combined M.D./Ph.D. at Baylor College of Medicine. Visit his website

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