

WHAT IF WE COULD PREVENT CHILDHOOD BLINDNESS?



Genome surgery for eye diseases is fast approaching

Solution: Genome editing dysfunctional cells in the eye could be a cure for one type of childhood blindness.

Did you know?

The eye lends itself well to genome editing, which is why early research is happening here. If successful, the research could move to other tissues as well. Progress like this helps move toward the third UN Sustainable Development Goal of Good Health and Well-Being.

Leber congenital amaurosis (LCA) is a rare hereditary eye disease that is the most common form of childhood blindness. It appears at birth or in the first few months of life and affects 1 in 40,000 newborns. Symptoms include significant vision loss within the first year of life and rapid involuntary eye movement, ultimately ending in blindness. LCA type 10 is the most common form of the disorder and accounts for 20-30% of all LCA subtypes. No treatment currently exists.

A genetic mutation is the cause of the illness and will also be the target of a potential cure. The mutation causes the progressive loss of the eye's light-detecting cells, called photoreceptors. Editas Medicine has developed a therapy that makes use of CRISPR genome editing to correct the mutation directly in the eye. CRISPR is a precision genetic engineering tool which allows scientists to change individual letters of the genetic code with high efficiency and relative ease. The CRISPR molecules can be delivered to the eye by subretinal injection, where they enter the cells of the retina and edit the mutated gene. This intervention has the potential to restore the function of remaining photoreceptor cells and arrest further loss of vision for patients.

Editas Medicine acquired the permission of the US Food and Drug Administration at the end of 2018 to test their therapy in patients. This will be the first clinical trial using CRISPR genome editing directly in patients. Its successful use will be a milestone for medical science and could unlock the cure to many genetic diseases.



Genome editing is

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REFERENCES:

<https://www.nature.com/articles/d41587-018-00003-2>
<https://editasmedicine.com/areas-of-research/eye-diseases/>