Science Heads

Editing Genes to Cure Disease

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Around 7,000 diseases or conditions in humans are known to be caused by genetic mutations. One of these conditions, spinal muscular atrophy (SMA), affects around 25,000 Americans. World-wide, 1 in every 6,000 – 10,000 babies born each year are afflicted with this potentially life-threatening condition.

The DNA of humans contain a gene named SMN1 which if mutated can cause SMA. Fortunately, the human genome also contains a backup copy of this gene called SMN2. If this backup copy is normal, then the effects of SMA can be mild. But if both genes are mutated, the condition can lead to severe physical limitations and eventually death. Babies born with Type 1 SMA have a life expectancy of under 2 years.

Gene Editing Holds Promise for a Cure

Researchers at Harvard University are working on a cure. David R. Lui, PhD leads a team looking for a cure using a technique called Base Editing. They described their technique recently in a peer-review article published in the journal ***bioRxiv***. His team modifies the genetic code of a harmless virus programming it to modify the base pairs of the backup SMN2 gene in cells that the virus infects. So far, this technique has been shown to cure SMA in mice and it works effectively in model cells that are very similar to human cells. Testing on humans remains to be done.

The hope is one day soon medical science may have found a cure for SMA and thousands of people could live longer and healthier lives.

Reference: https://www.genengnews.com/base-editing/one-time-treatment-base-editing-shows-promise-vs-sma/

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