CRISPR can fix gene that causes intellectual impairment in men–fragile X syndrome

The fragile X syndrome is the most common form of intellectual impairment in men, affecting 1 out of 3,600 boys. Now, scientists at the Whitehead Institute used the CRISPR/Cas 9 gene editing method to restore activity to the fragile X syndrome gene in affected neurons. Their work, performed on human brain cells in a dish, paves the way for trying the technique on the brain, with the hope that it may treat a host of genetic conditions.

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The team produced the first evidence that removing methylation, which are molecular tags that keep a mutant gene shut off, can reactivate the gene and restore the fragile X syndrome neurons.

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The researchers hypothesized that methylation can blanket the nucleotide repeats and shut down the gene's expression. To test this, postdoctoral researchers Shawn Liu and Hao Wu from [researcher Rudolf] Jaenisch's lab developed a CRISPR/Cas9-based technique which can add or delete methylation tags from a specific stretch of the DNA. Removing the tags proved to renew the FMR1 gene expression, turning it back into a normal gene.

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Jaenisch's team was able to reverse the abnormal electrical activity associated with the fragile X syndrome. Neurons rescued by this procedure were grafted into the brains of mice, with the FMR1 gene remaining active for at least three months. This promises that correcting methylation can lead to a sustained remedy for the disease.

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