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BIO 294

Writing assignment #4

October 14, 2022

Gene therapy helps those with a rare form of blindness see better at night

The article published by the US News on October 14, 2022, discusses an experimental gene therapy being tested on Leber congenital amaurosis in hopes to improve the night vision of adults with childhood-onset blindness. Leber congenital amaurosis (LCA) is the most common type of inherited retinal degeneration dysfunction. It causes progressive loss of photoreceptors and overall vision. One of the known causes of LCA is mutations in the GUCY2D gene. This gene is crucial because it is found in the transition space of rod photoreceptors, and is responsible for encoding a key protein needed in retinal photoreceptor cells to convert light to brain signals. In this ongoing trial, researchers have used adeno-associated virus (AAV) gene therapy approach, in which a non-enveloped virus is engineered and used to deliver DNA sequences with intended therapeutic applications directly to target cells. The article also briefly discusses the current results of the study. For instance, patients who have participated in the study have shown significant improvements in the function of their rod photoreceptors, which are responsible for the low light vision and light sensitivity. Two examples of patients who had little visual function and extremely abnormal light sensitivity were able to show improvements after 8 days of treatment, Overall, this study shows promising results in improving vision for those with LCA.

Works Cited

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