Gene Therapy that Delivers Light-Sensitive Proteins to Treat Eye Disorders

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Technology description

Intraocularly Delivered Adeno-Associated Virus (AAV) Treats, Prevents, or Inhibits Progression of Eye Disorders or Diseases

This gene therapy uses AAV vectors to deliver light-sensitive proteins intraocularly, treating a large number of diseases and disorders in the eye. Available eye therapies require repetitive treatments for disorders and. AAV vector-based therapy has proven to be useful in treating a number of diseases, including congestive heart failure, Parkinson’ s disease, and hemophilia, by delivering therapeutics to targeted cells. University of Florida researchers have developed a gene therapy that utilizes AAV vectors to deliver light-sensitive proteins to the eye, allowing for a one-time treatment for eye diseases. The AAV vectors have a large range of uses and can be customized to optimize the desired result, allowing for a variety of uses depending on the medical need. The therapy has been successful in treating eye injuries caused by environmental, mechanical, accidental, and genetic causes. It treats, prevents, or inhibits the onset or progression of various eye diseases.

Technology

This therapy uses AAV vectors to deliver genes to a targeted cell. The AAV vectors comprise nucleic acids capable of encoding light sensitive proteins. The therapy is performed to express certain light-sensitive proteins in the eye that are underexpressed or unexpressed targeting cells such as retinal neurons, retinal bipolar cells, retinal ganglion cells, photoreceptor cells, or amacrine cells. AAV serotypes, distinct variations within a species of viruses, are used to target specific cells based on the antigens on the microorganism’ s surface. In order to combat the immunoresponse generated by using AAVs at large doses, the viruses were mutated, causing an increase in retinal cell transduction efficiency. The AAV vectors are customizable, which allows for suitable levels and patterns of expression of the desired light-sensitive protein to be provided. The intraocular therapy has shown statistically significant improvements in condition, and the therapy has shown further promise in treating spinal injury and motor neuron diseases.

Application area

Gene therapy that uses AAV vectors to deliver light-sensitive proteins to treat, prevent, or inhibit progression of eye disorders or diseases

Advantages

Utilizes AAV vectors to target cells, delivering light-sensitive proteins exactly where they are needed
Allows for a one-time treatment, preventing the traditional repetitive care for eye diseases and disorders
Provides customized healthcare, allowing the treatment of various diseases and disorders

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