AAV Vector-Based Anti-Inflammatory Therapeutic for Macular Degeneration and Other Tissue-Specific Inflammatory Diseases

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Technology description

Gene Therapy Vectors Deliver Secretable, Cell-Penetrating Viral Therapeutics to Targeted Host Cells

This AAV vector-based therapeutic treats inflammatory responses in mammals by delivering tissue-specific anti-inflammatory therapeutic proteins to targeted host cells. These transvected host cells then inhibit a variety of pro-inflammatory syndromes, which cause or exacerbate diseases and disorders caused by chronic inflammation. AAV vector-based gene therapy is useful in treating a number of diseases, including congestive heart failure, Parkinson’ s disease, and hemophilia, by delivering therapeutics to targeted cells. Unfortunately, no available treatments combat tissue-specific inflammation, such as dry age-related macular degeneration. Age-related macular degeneration affects as many as 11 million people with global cost of visual impairment at $343 billion in the United States alone. University of Florida researchers have developed AAV vector constructs that are optimized for delivering anti-inflammatory peptides to selected mammalian cells and tissues. This tissue-specific treatment addresses symptoms of oxidative stress and inflammation by delivering secretable, cell-penetrating anti-inflammatory proteins to treat specific inflammatory diseases, such as dry age-related macular degeneration.

Technology

AAV vector-based therapy has been utilized in treating a number of genetic diseases; however, gene therapy has not traditionally been used to treat inflammatory diseases. This tissue-specific method of treating inflammation targets inflammatory responses by administering AAV vectors coupled with secretable, cell-penetrating anti-inflammatory proteins derived from viruses. These modified viral proteins penetrate target host cells; these suitably-transvected host cells then inhibit the key cellular inflammatory pathways that cause or exacerbate a variety of chronic progressive diseases, disorders, and conditions. Researchers have used this AAV vector-based therapy to treat dry age-related macular degeneration as an example of the therapy’ s use in inhibiting pro-inflammatory responses in a number of mammalian diseases.

Application area

AAV vectors deliver modified viral protein as tissue-specific therapy for chronic inflammation

Advantages

Delivers viral proteins to host cells, providing gene therapy treatment for inflammation
Delivers gene product directly to affected cells, increasing chance it can be used for many other tissue-specific inflammatory diseases
Targets two key pro-inflammatory signaling pathways within cells, giving it the capacity to treat diseases over prolonged period of time

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