



Gene Therapy for Alzheimer's and Other Diseases

The University of Florida is seeking companies to commercialize a novel gene therapy for Alzheimer's disease using an AAV vector to deliver the neprilysin gene to the brain. Alzheimer's disease is the most common form of dementia among the elderly. The disorder is physically characterized by abnormal clumps (amyloid plaques) and tangled bundles of fibers (neurofibrillary tangles) composed of misplaced β -amyloid proteins in the brain. It is thought that over 4.6 million Americans currently suffer from Alzheimer's disease, with estimates of 11-16 million Americans affected by the year 2050. Over 100 billion dollars a year is spent in the United States to care for people with Alzheimer's disease. University of Florida researchers have developed a novel treatment for the disease, using AAV vectors to deliver the gene neprilysin to the brain. Neprilysin is an enzyme which functions to degrade extracellular β -amyloid in the brain. Clearance of excess β -amyloid protein, and/or prevention of buildup may function to ameliorate or prevent symptoms of Alzheimer's disease.

Applications

Potential treatment of Alzheimer's disease symptoms and/or any other β -amyloid plaque pathogenesis disease

Advantages

- ◆ Provides a potential treatment option for Alzheimer's disease, providing tremendous market opportunity with no current competitors
- ◆ Enables delivery of both a native membrane anchored Neprilysin, which is capable of removing β -amyloid in the region of the transduced cells, as well as a secreted Neprilysin, which is capable of removing extra-cellular β -amyloid from a greatly increased volume of the brain compared to the native Neprilysin, increasing likelihood of complete clearance of β -amyloid plaques
- ◆ AAV vector provides very good transduction of neurons in the brain, maximizing the efficacy of the gene therapy

The Technology

This invention consists of the gene neprilysin with a membrane anchor or a secretion peptide signal sequence cloned into adeno-associated virus (AAV) vector for gene therapy. The treatment will enable patients to produce the enzyme neprilysin, which will reduce the β -amyloid present and therefore decrease amyloid plaque formation.

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