Adeno-associated Virus Vectors for Gene Therapy Of Neurological Disorders

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**Description:**
Scientists at Yale have developed a method to deliver and stably express genes in the nervous system. Treatments based on this discovery could be used to treat a broad range of neurological diseases, including Parkinson's, Alzheimer's, epilepsy, and brain tumors. The 2002 domestic market for Parkinson’s was estimated at $1.8 billion, and current therapies leave much to be desired in terms of efficacy. Existing technologies using Herpes, Retroviral, or Adenovirus vectors are limited with respect to safety/pathogenicity, long-term expression, or the ability to infect non-dividing cells. Since our technology is based on AAV vectors, these limitations are overcome. Studies using our technology have demonstrated long-term behavioral improvement in an animal model for Parkinson's Disease. Clinical trials for Parkinson's Disease are currently underway. Gene therapy products based on this technology could have far reaching effects in the field of neurological disorders.

**Field of Application:** The multi-billion dollar neurological disease market is growing with our aging population. Gene delivery to the CNS is currently being explored by a multitude of companies and could be used treat a variety of conditions including but not limited to Parkinson’s disease, Alzheimer’s disease, Tay Sachs, Lesch-Nyan, epilepsy, and brain tumors.

**Advantages:** Our gene delivery technology, based on Adeno-Associated Virus (AAV) vectors, has many advantages over current technologies based on Retrovirus, Herpes Virus or Adenovirus vectors. AAV vectors are safer compared to competing technologies in that AAV vectors are non-pathogenic, non-immunogenic, and carry a significantly reduced possibility of generating replication competent virus. In addition, AAV vectors are capable of long term expression, can infect dividing and non-dividing cells, and have the ability to innocuously integrate into the host genome of non-dividing cells.

**Stage of Development:** This technology is covered by issued patents and has been used to correct the behavior of non-human primate models of Parkinson's disease and is currently in clinical trials for Parkinson's Disease and Canavan's Disease.

**Publications:**

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