



VECTOR MANUFACTURING

Efficient AAV Vector Production Not Requiring Helper Virus

Brief Description of Technology

System for increased efficiency AAV vector production with higher yield and less potentially immunogenic contaminants.

BUSINESS OPPORTUNITY

Exclusive license
Sponsored research

TECHNOLOGY TYPE

Cell therapy
Gene therapy
Research tool

STAGE OF DEVELOPMENT

Preclinical *in vivo*

PATENT INFORMATION

US 7208315

INVESTIGATOR

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Technology Overview

Dr. Miller's group developed a high-yield, cell line-based system to produce AAV vectors for gene therapy applications in high titers, up to 20-fold over the traditional packaging cell/helper virus system. Adeno-associated virus [AAV] vectors are widely used for gene therapy applications due to their ability to transduce a wide variety of tissues, its persistence, and lack of pathogenicity. AAV vector-based gene therapy has reached the clinical trial stage in liver-directed gene therapy [hemophilia B] and retinal gene therapy [Leber congenital amaurosis]. AAV remains an attractive platform for development of *in vivo* gene therapies to monogenic diseases including hemophilia A, inherited retinal diseases, and Huntington's disease.

Applications

- Method for efficient production of recombinant AAV vectors for use in gene therapy
- Method for eliminating helper virus presence in AAV vector production

Advantages

- Does not require fully functional adenovirus to generate AAV, leading to less contamination by adenovirus in AAV vector preps
- Efficient production of high titers of recombinant AAV vectors of 20-fold over standard production

Market Overview

Since 2013, the gene therapy field has experienced a resurgence, with 12 different companies raising over \$650 million. There are almost 500 gene therapy candidates currently in development, with more than 200 targeting cancer, and 100 already in late-stage clinical development. The market for gene therapy-based cancer therapeutics is expected to reach up to \$13 billion by 2025. AAV is a commonly used vector for the transduction of postmitotic cells for persistent, potentially long-term transgene expression.