



## VECTOR MANUFACTURING

# Elimination of Immunogenic Contaminants in AAV Vector Preparations

## Brief Description of Technology

Captron technology for production of AAV vectors to reduce 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 2013/0296409  
US 2016/0194664

### INVESTIGATOR

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### LEARN MORE

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## Technology Overview

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. One major obstacle to AAV vector-based gene therapy is the inclusion of potentially immunogenic contaminants in AAV production stocks, including AAV capsid protein. The immune response mounted in response to such contaminants can result in elimination of the gene modified cells and thus can have a negative effect on long-term stability of gene therapy. Dr. Miller's group has developed a modified AAV capsid expression cassette [captron], which is too large for packaging into the AAV virions and thus eliminating a potential source of capsid antigen. Elimination of immunogenic contaminants in AAV vector preps can increase the potential for long-term, persistent expression of therapeutic transgenes.

## Applications

- Production of recombinant AAV vectors that minimizes risk of capsid gene contamination

## Advantages

- Reduced presence of immunogenic contaminants in AAV vector preparations

## 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 post-mitotic cells for persistent, potentially long-term transgene expression.