



Method for Molecular Breeding of Gene Therapy Vectors

One of the major limitations to a successful gene therapy protocol is delivery of the therapeutic gene to the tissue of interest. If a vector is unable to target the desired cell or tissue type associated with the clinical disease two main problems arise. Firstly, large doses of the vector are required, increasing the time and expense associated with vector production. Secondly, the potential for undesired side effects or toxicity in collateral tissue increases. University of Florida researchers have developed a technology to address this limitation. The technology uses combinatorial vector libraries to create novel tissue-specific gene therapy vectors, allowing for the creation of vectors individually tailored for the particular gene therapy protocol.

Applications

Useful in creating gene therapy vectors specific to therapeutic target tissue

Advantages

- ◆ Enables tissue-specific targeting of gene therapy vectors, insuring therapeutic gene expression only in the target tissue
- ◆ Reduces the risk of gene expression in collateral tissue, lowering the possibility of undesirable side effects or toxicity
- ◆ Allows for the use of smaller quantities of vector, decreasing costs, and decreasing the possibility of existing antibody responses in the patient
- ◆ Potential to create an unlimited number of tissue-specific vectors, ensuring broad applicability of this technology

The Technology

This invention pertains to the construction of a combinatorial library of chimeric adeno associated virus (AAV) vectors for tissue-specific targeting. In creating this technology, University of Florida researchers have addressed technical limitations that had previously made this technique in AAV unfeasible. To achieve this goal, inventors have both exploited molecular breeding of viruses, and utilized a novel technology of AAV vector production based on insect cells combined with AAV genome pseudotyping. The resulting technology allows users to develop novel gene delivery vectors with reduced side effects, reduced required dosage, and reduced possibilities of existing antibody responses in patients.

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