Docket #: S00-013

Increasing the size of rAAV-mediated expression cassettes in vivo

Researchers at Stanford have developed and patented rAAV vectors that can be used to transfer long nucleic acids into cells. Recombinant AAV vectors are limited by a restricted amount of exogenous DNA that can be placed into the vector. The strategy designed here overcomes this problem by using two different rAAV vectors, each containing a portion of an expression cassette to reconstitute gene expression *in vivo*. By strategic placement of cis DNA elements, up to 70% of the exogenous level of gene expression from a single vector can be achieved *in vivo*.

Please see the issued patent (below) for additional information.

Applications

- Persistent and safe therapeutic gene delivery
- *In vivo* gene therapy for reconstituting large expression cassettes of up to 9 kb

Advantages

- Accommodate cDNAs that normally will not fit in rAAV vectors
- Can add large regulatory or cis DNA elements that require specific regulatory elements

Publications

- Nakai H, Storm TA, Kay MA. Increasing the size of rAAV-mediated expression cassettes in vivo by intermolecular joining of two complementary vectors. Nat Biotechnol. 2000 May;18(5):527-32.

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