GENERATION OF NEXT GENERATION RECOMBINANT AAV GENE THERAPY VECTORS THAT ADOPT 3D CONFORMATION

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Inventors: Kinjal Majumder, Clairine Larsen

The Invention

UW-Madison researchers have developed approaches to improve recombinant AAV gene therapy vectors (rAAV) therapies by improving their expression in target cells. The inventors have discovered that the AAV genome is folded into a distinct topological conformation similar to the 3D structure adopted by the eukaryotic genome, which is required for AAV to express. The inventors have engineered novel modifications into the rAAV vector genome that facilitate the formation of 3D-structures for the vector genome. Namely, they've introduced paired CTCF sequences that facilitate looping, and they're working to test a second CTCF sequence. The new rAAV vector expresses an incorporated transgene twice as efficiently and in double the number of target cells when transduced. This can improve gene therapies by robustly expressing transgenes, thereby cutting down on the amount of vector that is given to individuals, decreasing production costs long-term, and ultimately improving access to gene therapies. A remaining challenge is the small coding capacity of the typical rAAV vector, which limits the length of the transgene that can be expressed; the inventors are planning to explore the capacity of the modified rAAV vector and consider possible further improvements.

Tech Fields

- Drug Delivery : Biologics
- Drug Delivery : Other drug delivery technologies

For current licensing status, please contact Jennifer Gottwald at jennifer@warf.org or 608-960-9854