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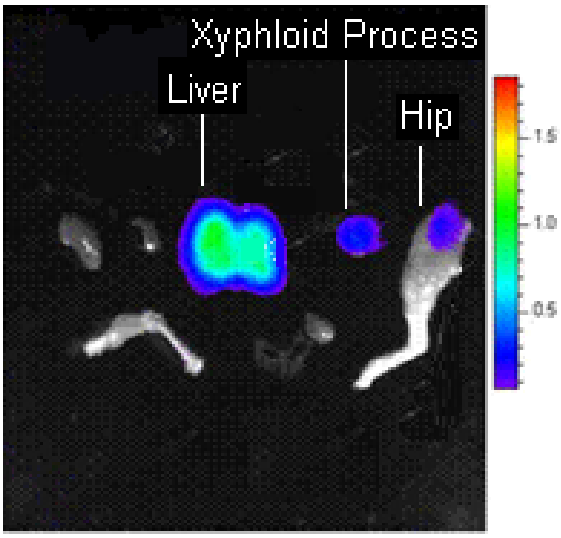
City of Hope

NATIONAL MEDICAL CENTER AND
BECKMAN RESEARCH INSTITUTE

Novel AAV Vectors Optimized for Therapeutic Gene Transfer



DESCRIPTION

Recombinant adeno-associated virus (rAAV) vectors derived from the replication defective human parvovirus AAV2 are proving to be safe and effective gene transfer vehicles because they transduce non-dividing primary cells, are low in immunogenicity and direct sustained transgene expression in vivo. This technology covers novel AAV isolates with unique tropism and transduction properties. These novel isolates may be used to transduce cells transiently, resulting in short term gene expression without genomic integration, a property of enormous importance for the applications such as derivation of induced pluripotent stem cells or expression of zinc finger proteins. In addition, when delivered systemically, these vectors display a tropism for liver and cartilage tissues. The additional tropism of the present AAV isolates for cartilaginous tissue in joints may be exploited for the treatment of bone disorders such as arthritis, osteoporosis or other cartilage/bone based diseases.

KEY ASPECTS

- There are 15 novel AAV sequences, each with different tropism and transduction properties
- *in vivo* data is available
- Novel isolates present tropism for both cartilaginous tissue and liver tissue
- Potential vector for regenerative medicine

INTELLECTUAL PROPERTY

Title	US Patent Application	Filed
CD34-Derived Recombinant Adeno-Associated Vectors for Stem Cell Transduction and Systemic Therapeutic Gene Transfer	61/330,272	4/30/2010

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