Docket #: S06-098

Novel AAV capsids with new transduction and nonimmune properties

Researchers from Dr. Mark Kay's laboratory at Stanford University have merged desirable qualities of multiple natural AAV isolates by an adapted DNA family shuffling technology to create a complex library of hybrid capsids from eight different wild-type viruses. One of the capsids was vectorized and used to express the human transgene factor IX in mice. This AAV vector was found to be as robust or better than the best AAV vector identified in nature to date. Moreover, this capsid does not react with the human immune response. Finally, this capsid is useful for transducing cells in culture, which has been problematic for many of the AAV vectors studied to date.

Applications

- Gene therapy - library of novel AAV capsids may be useful in gene therapy applications because the novel capsids will not elicit an immune response.
- Research - the ability to isolate new AAV vectors with new or improved properties than those isolated in nature.

Advantages

- These are novel AAV vectors with new or improved properties than those isolated in nature. For instance, the investigators have found a capsid that may not elicit an immune response.

Publications

- Melo, Sandra P, Leszek Lisowski, Elizaveta Bashkirova, Hanson H Zhen, Kirk Chu, Douglas R Keene, M Peter Marinkovich, Mark A Kay, and Anthony E Oro. "Somatic Correction of Junctional Epidermolysis Bullosa by a Highly Recombinogenic AAV Variant." Mol Ther Molecular Therapy, 2014, 725-33.
- Dirk Grimm, Joyce S. Lee, Lora Wang, Tushar Desai, Bassel Akache, Theresa A. Storm, and Mark A. Kay In vitro and in vivo gene therapy vector evolution via multispecies interbreeding and retargeting of adeno-associated viruses. *J Virol.* 2008 Jun;82(12):5887-911.

Patents

- Published Application: 20070243526
- Published Application: WO2007120542
- Published Application: 20100047174
- Published Application: 20120066783
- Published Application: 20120255046
- Published Application: 20150057189
- Issued: 7,588,772 (USA)
- Issued: 8,067,014 (USA)
- Issued: 8,574,583 (USA)
- Issued: 8,906,387 (USA)

Innovators

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