Docket #: S06-099

Robust factor IX minigene expression cassette (TTR)

Researchers in Dr. Mark Kay's laboratory at Stanford University have designed a new liver-specific expression cassette for inserting genes into double-stranded AAV (adeno-associated virus) vectors for gene therapy. AAV vectors are useful for gene therapy because they can be used to transduce different types of dividing and non-dividing cells of different tissues. They can establish stable, long-term transgene expression without the toxicity or human diseases associated with other viral vectors. It is believed that double-stranded AAV vectors are 10 - 100 times more robust than single stranded AAV vectors and would therefore require much smaller doses than single-stranded AAV to produce a therapeutic effect. However, a limited amount of exogenous DNA can be inserted into the double-stranded vectors. Now, Stanford inventors have linked a synthetic liver-specific enhancer to a mini promoter to generate a Factor IX vector that can be packaged into double-stranded AAV vector. This cassette was used to express Factor IX *in vivo*.

Stage of Research:
The inventors have used the expression cassette with double stranded AAV vectors for Factor IX gene therapy in mice.

Applications

- **Gene therapy** - for inserting genes that require liver-specific expression
- **Research** - studying double stranded AAV vectors

Advantages

- **Short** - small expression cassette can be packaged into double-stranded AAV vectors

- **Robust** - double-stranded AAV vectors are 10 - 100 times better than single stranded vectors
- **Lower doses** - anticipate doses 10 - 20 times lower than traditional vectors for therapeutic effect, minimizing the risk of triggering an inactivating immune response
- **Liver-specific**

Patents

- Published Application: 20070243168
- Published Application: WO2007120533
- Issued: 8,129,510 (USA)

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