Docket #: S11-234

Robust and Sustained Transgene Expression with Mini-Intronic Plasmid Vectors

Researchers in Prof. Mark Kay's laboratory have developed a robust vector that combines the ease of plasmid preparation with the stable expression achieved by minicircle vectors. This technology – Mini-Intronic Plasmids (MIP) – integrates essential bacterial elements for antibiotic-free selection and propagation within an engineered intron contained within a non-coding exon. MIPs offer an easy to implement alternative to minicircles for a variety of gene transfer/therapy, protein production and research applications where optimal transgene expression is required. In many cases, transgene expression is up to 10-times higher than that achieved by routine plasmids, minicircles or when used within a viral vector such adeno associated viral vectors (AAV).

Stage of Research
The inventors have demonstrated up to 10-fold higher in vivo transgene expression from MIP vectors than minicircle vectors in mouse livers. Biological materials are available for evaluation.

Applications

- **Gene therapy** - extrachromosomal expression of therapeutic genes without the risks associated with integration into patient genome
- **RNA and protein production** - synthesis of peptides, proteins and RNAs
- **Robust AAV vector** - robust transgene expression AAV vectors
- **Research** - vectors for creating transgenic cells and animals

Advantages

- **Easy, scalable production:**
  - standard plasmid preparation for mass production
  - RNA-out selection instead of antibiotic-selection
- **Robust, prolonged expression:**
  - persistent expression at up to 10-fold higher levels than minicircles
  - no bacterial plasmid-induced DNA silencing
  - up to 10 fold enhanced AAV-mediated transgene expression

Publications

- Lu J, Zhang F, Kay MA. " A Mini-intronic Plasmid (MIP): A Novel Robust Transgene Expression Vector In Vivo and In Vitro " Mar 2013. *Mol Ther* 10.1038/mt.2013.33
- Published International Patent Application (pending) WO 2013/119371

Patents

- Published Application: 20130210897
- Published Application: WO2013119371
- Issued: 9,347,073 (USA)

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