Novel synthetic regulation of gene expression

Value Proposition
Gene therapy is an emerging market that is expected to grow at an astounding annual rate of 48.9%. Although gene therapies have yet to be approved in the United States, several are currently being evaluated in over 4,900 clinical trials – of which many are backed by major pharmaceutical companies like Pfizer and GSK. A major challenge of gene therapy is to target only the intended cell- and/or tissue-type. For this reason, many gene therapies are delivered using adeno-associated virus (AAV) vectors with gene-specific promoters; however, off-target AAV tropism and inadequate promoter regulation many times lead to the expression of the delivered gene in unintended cells/tissues. For these reasons, methods that can restrict the expression of the delivered gene within specific cells/tissues are needed for the safe implementation of gene therapy.

Technology
Drs. Marengo and Garcia-Blanco have recently developed a technique that promotes the stringent control of gene expression within specific cell types by combining gene-specific promoters with cell type-specific splicing exons and microRNA-binding sites. This invention has the potential to enhance the stringency of AAV-delivered gene therapies, or it could be utilized in stand-alone nucleic acid delivery systems (e.g. nanoparticle-, lipofection-, electroporation-mediated delivery). The latter would overcome several of the limitations of viral-based gene therapies, including immunological neutralization by the host and restrictions on transgene size.

Advantages
This methodology enforces multiple modes of cell-type-specific transcriptional regulation before the desired gene can be encoded, thereby preventing “leaky” expression within unintended cell types.

Patents

- Patent Number: 9,284,575
  Title: SYNTHETIC REGULATION OF GENE EXPRESSION
  Country: United States of America

- Patent Number: 9,845,481
  Title: SYNTHETIC REGULATION OF GENE EXPRESSION
  Country: United States of America