GS1-1 Development of transposon-based novel plasmid vectors for prolonged gene expression

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Non-viral vectors for gene therapy and gene functional analysis have been brought to attention because of their safety and easiness to manufacture. To date, many groups have been developed effective in vivo gene delivery methods including cationic carriers, electroporation, and sonoporation, etc.. However, gene expression after delivery to cells is transient, which may limit both the treatment of inherited disorders and analysis of gene functions.

One approach to overcome this limitation is the use of DNA Transposons. Transposons are genetic elements capable of moving from plasmid vectors to chromosomes, which stabilize therapeutic genes and enable prolonged gene expression. We constructed two kinds of plasmid vectors based on *piggyBac*, one of the most efficient transposons for integration into mammalian chromosomes. One is the “Donor vector”, which contains transposon with expression cassette of exogenous genes, and the other is “Helper vector”, which expresses transposase, the enzyme to mobilize transposons. Intravenous injection of both vectors into mice resulted in prolonged gene expression. In this symposium, we would like to discuss the design and development of integrative plasmid vectors based on transposon.