Gene therapy

Gene therapy is the therapeutic delivery of nucleic acid polymers into a patient's cells as a drug to treat disease. The polymers are either expressed as proteins, interfere with protein expression, or possibly correct genetic mutations.

The most common form uses DNA that encodes a functional, therapeutic gene to replace a mutated gene. The polymer molecule is packaged within a “vector”, which carries the molecule inside cells.

In neurooncology, the biology of neural stem cells (NSCs) has been pursued in two ways: as tumor-initiating cells (TICs) and as a potential cell-based vehicle for gene therapy.

Effective suicide gene delivery and expression are crucial to achieving successful effects in gene therapy.

Gene therapy is a promising strategy to overcome barriers to axon regeneration in the injured central nervous system. Branched polyethylenimine (bPEI: 25kDa) is one of the most widely studied nonviral vectors, but its clinical application has been limited due to cytotoxicity and low transfection efficiency in the presence of serum proteins. Here, we report cationic amphiphilic copolymers, poly (lactide-co-glycolide)-graft-polyethylenimine (PgP) that are capable of efficiently transfecting reporter genes and siRNA both in the presence of 10% serum in vitro and in the rat spinal cord in vivo. The combination of improved transfection and reduced cytotoxicity in the presence of serum as well as transfection of neural cells in vivo suggests PgP may be a promising nucleic acid carrier for CNS gene delivery [1)].

Types

Suicide gene therapy

1)
Gwak SJ, Nice J, Zhang J, Green B, Macks C, Bae S, Webb K, Lee JS. Cationic, amphiphilic copolymer micelles as nucleic acid carriers for enhanced transfection in rat spinal cord. Acta Biomater. 2016 Apr 15;35:98-108. doi: 10.1016/j.actbio.2016.02.013. PubMed PMID: 26873365; PubMed Central PMCID: PMC4829463.