

# 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 <sup>1)</sup>.

## Types

### Suicide gene therapy

<sup>1)</sup>

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.

From:

<https://neurosurgerywiki.com/wiki/> - **Neurosurgery Wiki**

Permanent link:

[https://neurosurgerywiki.com/wiki/doku.php?id=gene\\_therapy](https://neurosurgerywiki.com/wiki/doku.php?id=gene_therapy)

Last update: **2024/06/07 02:59**

