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Short Communication

Nanoparticles In Gene Therapy

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INTRODUCTION

Gene therapy has emerged as an effective treatment method for a variety of illnesses, including diabetes and cancer, in recent years. Gene therapy utilising genetic engineering, as well as gene delivery methods, have been extensively researched [1]. Engineering efficient gene-delivery vectors with minimal cytotoxicity is a key problem for scientists[2]. Many indicators of toxicity and adverse effects have been seen in viral vectors that have been employed as gene delivery vehicles. To circumvent the physiological hurdles that viral vectors face, non-viral vectors for gene transfer have been investigated and developed. Cationic lipids are good non-viral gene carriers because of their strong gene integration capacity, high transfection efficiency, and simplicity of production.

However, there are significant drawbacks to using cationic lipids in therapeutic settings, such as poor repeatability due to large batch-to-batch variability and instability. Biodegradable polymeric nanoparticles, on the other hand, have been explored for a variety of reasons, including their tiny particle size, good physiological stability, ease of surface functionalization, and long-term release profiles. However, one important drawback of polymeric nanoparticles is their low transfection effectiveness, which limits their therapeutic use[3]. The different forms of nanoparticles and their uses in gene therapy, as well as the technical and ethical problems surrounding their usage in gene therapy, will be discussed in this study.

Gene therapy methods

In general, gene therapy can be accomplished by introducing naked DNA into target cells; however, due to the negative charge of large DNA molecules and the negative nature of the cellular membrane, some nucleic acid-based medicines are unable to cross the cellular membrane using simple passive diffusion methods. As a result, it's critical to utilise a vector to aid in the transfer of DNA molecules into the cell. In order to introduce genes into cells, both somatic gene therapy and germline gene therapy, whether *in vitro* or in vivo, require vectors. Vectors are divided into two types: viral and non-viral vectors. Viral vectors have been demonstrated to be a very effective delivery strategy in gene therapy due to their inherent capacity to transduce their own genetic material into host cells. Furthermore, viral vectors have large packing capacities of up to 50 kb and may carry a variety of single- and double-stranded genetic material, broadening their therapeutic potential substantially. Cancer, neurological disorders [4] retinal illnesses, haemophilia[5], and arthritis are only a few of the diseases that may be treated with viral vectors. However, viral vectors have a number of disadvantages, including the difficulty of scaling up and designing them, as well as their proclivity for immunotoxicity. Non-viral vectors encompass a wide range of non-viral delivery techniques, from physical means like electroporation, ultrasound, and magnetofection [6] to chemically constructed carriers like nanoparticles and polymers. Non-viral vectors are safer than viral vectors in that they are less likely to trigger immunological responses and mutagenesis, as well as being more cost-effective and easier to create.

Nanoparticles

A particle, according to nanotechnologists, is a tiny entity that moves and functions as a single unit. Particles can be divided into fine and ultrafine particles, with sizes ranging from 100 nm to 2500 nm and 1 nm to 100 nm, respectively.

Nanoparticles are particles having at least one dimension less than 1 m that fall into the latter category. Because of their unusually high surface area and quantum size effects, some feel they should be classified as a new state distinct from the traditional liquid, solid, gaseous, and plasma states. Despite their one-of-a-kind nature, nanoparticles can only exist in two forms: amorphous (non-aggregating) or crystalline (aggregating). Nanomaterials are made in two ways: top-down nanofabrication, which includes breaking down big (bulk) structures into smaller components, and bottom-up nanofabrication, which involves building nanostructures using individual atoms.

Most common Nano particles used in gene therapy

Because of their nanometric size, high surface-to-volume ratio, and stability, nanoparticles are good candidates for gene carriers. They can also have their surfaces modified to allow them to bind an infinite number of ligands and receptors. Some nanoparticles, on the other hand, can encapsulate nucleic acids and release them within target cells. Nanoparticles are an excellent candidate for non-viral vectors in gene therapy because of these characteristics, as well as their lack of immunogenicity. Here we are discussing in brief different nano particle being used for this process.

Chitosans were initially utilised in gene therapy *in vitro* in 1995 as a self-aggregating complex [7]. The fact that the main amine backbone in chitosans is protonated (i.e., positively charged) in acidic circumstances makes the molecule soluble in organic solvents is the reason for its appeal in gene delivery. Numerous research on the medicinal application of chitosans have been published, both *in vitro* and in vivo. GI delivery of chitosan-insulin gene complexes to diabetic rats was successful [8].

Due to their unique characteristics, dendrimers have been used in a variety of applications over the years, including medication and gene delivery. PAMAM dendrimers, which include a core of either ammonia or ethylenediamine and a few branching sites, were the first attempts at utilising dendrimers as a gene delivery vector.

Gold nanoparticles have also been shown to be an effective gene therapy delivery method. Their appealing characteristics, including as inertness, simplicity of thiol linkage functionalization, and plasmon resonance, have sparked a lot of study into their medicinal potential [9] performed one of the early investigations on gold nanoparticles as nucleic acid vectors, successfully inhibiting T7 RNA polymerase transcription with gold nanoparticles functionalized with trimethylammonium thiol.

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