Chapter 14

Nanocarriers for Vaccine and Gene Delivery Application

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ABSTRACT

Nanocarriers with various compositions and biological properties are frequently used systems for in-vitro/in-vivo vaccination and gene transfer. In recent years, developments in nanotechnology have focused on the design and synthesis of nanocarriers that have new properties and can be modified for gene and vaccine delivery. In the favorable results obtained from in-vivo studies performed, they increase interest in these developments and pave the way for their therapeutic use. Nanocarriers have become increasingly important because they can stabilize vaccine antigens and serve as adjuvants, with the advantage of easily transporting genetic material to the target site. In nanocarriers, the molecules involved are adsorbed to the surface or encapsulated in particulates. At the same time, surface modification of nanoparticles allows these systems to carry cargo molecules easily to target site. Among the most studied nanocarriers, lipidic and polymeric systems dendrimers, inorganic nanoparticles, cyclodextrins, cell penetration peptides, and ISCOMs are attracting attention.

INTRODUCTION

Gene therapy is a rapidly advancing field with great potential for treating genetic and acquired systemic diseases. This therapy involves the introduction of foreign genetic material into target cells to alter a genetic sequence. Viral and nonviral vectors are used for this purpose. Viral vectors are the most effective ones, but their applications are limited due to their immunogenicity, oncogenicity, and limited DNA size that they can carry. However, nonviral vectors are safer, less costly, and easier to produce. Moreover, the size of the genetic material that they carry is not limited. Despite improvements in the recent years, low transfection efficiency is still a disadvantage in nonviral systems (Dorraj, Carreras, Nunez, Abushammala, & Melero, 2017).

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Significant progress has been made in traditional vaccination strategies with the developments in biotechnology. In these strategies, the development of vaccine systems in combination with nanostructures, or the use of nanostructures as adjuvants, has facilitated an effective humoral, cellular, and mucosal immunity. These new approaches involve a variety of innovative methods producing effective host responses (Kalam, Khan, & Alshamsan, 2017).

Nanostructures can be designed in a complex manner to be used for treating various diseases. Especially having particles of nanosize in the designed nanocarrier systems affects the gene/vaccine-carrying capacity and the efficacy of these systems in the medical applications. The particle size in the transport systems is an important parameter for reaching the target area from the circulatory system and to be taken out of the cell. These carriers not only increase the effectiveness of the vaccine and genetic material, but also help to release them into the right place. Therefore, it is extremely important to know the physicochemical properties of the carrier system together with cellular and biological properties in nanostructure-mediated gene or vaccine transport, and to make a design by taking all these parameters into consideration.

The aim of this chapter is to provide information and examples of common nanocarriers currently used in gene and vaccine delivery. the general perspective of the article.

Gene Therapy: An Overview

The idea of using genes as medicines in treatments was first put forward in the United States in the 1970s. The emergence of this concept has led to the development of different technologies for determining human gene functions, the elucidation of effects of the mutant human genes with the updated information, and development of different technologies for DNA transmission to mammalian cells (Giacca, 2010).

Different gene manipulation methods can be applied in gene therapy to restore a specific gene function or to knock down a specific gene(s). This requires the transfer of an appropriate gene into a cell to replace a defective or a missing gene (Mellott, Forrest & Detamore, 2013).

Initially, gene therapy was used for treating inherited metabolic diseases such as autosomal or X-linked recessive single-gene disorders, cystic fibrosis), ADA-SCID, emphysema, retinitis pigmentosa, and sickle cell anemia. Later on, anemia, phenylketonuria, hemophilia, Duchenne muscular dystrophy, some autosomal dominant disorders, polygenic disorders, different cancer types, vascular disease, neurodegenerative disorders, inflammatory conditions, and other acquired diseases became targets of gene therapy (Nayerrossadat, Maedeh & Ali, 2012; Dizaj, Jafari & Khosroushahi, 2014).

About 2335 studies on clinical gene therapy were performed between 1989 and 2015, and these studies continue worldwide (Hanna, Rémuzat, Auquier & Toumi, 2016).

Almost 38% of these clinical trials were performed with virus-mediated gene transfer. A vast majority of these trials (from phase II to phase III) also involved virus-mediated experiments. Only 24 of these clinical trials in the literature have been reported to reach phase III. The majority of gene therapy targets in the clinic are cancer diseases. The first gene therapy reported in the literature was approved for Glibera, which was given for an adeno-associated virus carrying a lipoprotein lipase gene. This drug was approved by the European Union in 2012 for treating a rare form of hereditary dyslipidemia. Moreover, ONYX-15 was a first-generation oncolytic adenovirus for the local treatment of head and neck cancers in China; however, its use has not been approved in other countries yet (Lukashev & Zamyatnin, 2016; Hanna, Rémuzat, Auquier & Toumi, 2016).