LIPID NANOPARTICLES FOR THE DELIVERY OF NUCLEIC ACIDS

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3.1 INTRODUCTION

As with the development of recombinant DNA technology, gene therapy emerged as a promising technology that would drastically improve the practice of medicine for treating inherited and acquired diseases. The idea underlying gene therapy is to treat human disease by the transfer of genetic materials into specific cells of the patient [1]. Nucleic acids, the fundamental elements that precisely controlled the expression of proteins, are very appealing therapeutic candidates due to the simplicity of the drug development strategy and minimal side effects compared with conventional drugs. Moreover, the discovery of RNA interference (RNAi) by Fire and Mello in the late 1990s has opened up an entirely new field of "gene therapy," using small RNA fragments that potently knockdown the target gene expression with high specificity and efficiency [2]. Gene therapy has hardly made its mark in medicine. Successful implementation of gene transfer in the clinic requires drug delivery techniques that can efficiently and specifically deliver nucleic acids to the site of action after systemic administration.

The physicochemical properties of nucleic acid, such as vulnerability to nucleases, high anionic charge content, and high molecular weight (MW), preclude naked unformulated molecules from performing their functions after systemic delivery. Therefore, a delivery vector should facilitate nucleic acid therapeutics to access the

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