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The advances and challenges of Gene Therapy for Duchenne Muscular Dystrophy

Since the discovery of the dystrophin gene (DMD gene) thirty years ago, several therapeutic approaches have been investigated to treat Duchenne muscular dystrophy (DMD). This includes cell therapy, exon jumping, exonic knockout, and the CinDel method. In this article, we present the challenges of developping a treatment for DMD and the advances of these various approaches. We included the new CRISPR-Cas9 system, which permits not only major progress in the development of new treatments based on genome editing but also the production of new animal models.

Mini Review **Published Date:- 2017-07-24**

Progress in the development of Lipoplex and Polyplex modified with Anionic Polymer for efficient Gene Delivery

Nucleic acid-based therapy has become an increasingly important strategy for treating a variety of human diseases. In systemic therapy, a therapeutic gene must be delivered efficiently to its target tissues without side effects. To deliver a therapeutic gene such as plasmid DNA (pDNA) or small interfering RNA (siRNA) to target tissues by systemic administration, cationic carriers such as cationic liposomes and polymers have been commonly used as a non-viral vector. However, the binary complex of therapeutic gene and cationic carrier must be stabilized in the blood circulation by avoiding agglutination with blood components, because electrostatic interactions between positively charged complexes and negatively charged erythrocytes can cause agglutination, and the agglutinates contribute to high entrapment of the therapeutic genes in the highly extended lung capillaries. One promising approach for overcoming this problem is modification of the surface of cationic complexes with anionic biodegradable polymers such as hyaluronic acid, chondroitin sulfate, or polyglutamic acid. As another approach, we recently developed a sequential injection method of anionic polymer and cationic liposome/therapeutic gene complex (cationic lipoplex) for delivery of a therapeutic gene into the liver or liver metastasis. In this review, we describe recent advances in the delivery of therapeutic genes by lipid- and polymer-based carrier systems using anionic polymers.

Editorial **Published Date:- 2017-06-23**

CRISPR genome editing: A general view

CRISPR technology has presented a path forward for genomic engineering and gene modification. The framework for the use of CRISPR technology to manipulate the human genome is of great interest and the form of its development and application has excited the researchers and biotech communities as the number of publications citing CRISPR gene targeting system has rose predominantly as indexed in PubMed. From a technical standpoint of view, most of us think that this would be relatively straightforward process, but technical feasibility is never the only consideration in doing experiments. Much of the discussion about CRISPR engineering has revolved mostly around its ability for treating disease or editing the genes of human embryos. In the real sense, what the biologists desire about CRISPR is its specificity: the ability to target and determine particular DNA sequences in the genome circuit.
