57 - Novel cationic lipopolyplexes as gene therapy vectors

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A major obstacle in the development of gene therapy is delivery of therapeutic genes to the desired cell/tissue. The objective of our study is to use a non-viral ternary system (lipopolyplexes) to encapsulate and deliver therapeutic DNA. Our lipopolyplexes comprise a glycerol-based cytofectin, a targeting peptide and plasmid DNA.

Novel derivatives of the cationic lipids DOTMA and DOTAP have been synthesized and tested in a breast cancer cell line. A range of branched cationic peptides varying in number of residues, composition and linker to a targeting head group were also designed and prepared. The bio-physical studies demonstrated that all LPD complexes were positively charged, small (60-80 nm) and were shown to effectively condense DNA. Gel assays showed which peptides were able to protect DNA more effectively and gave high transfection efficiency. Further studies are underway investigating these systems in siRNA delivery.

Sunday, August 28, 2011 05:30 PM

Current Topics in Biological Chemistry (05:30 PM - 07:30 PM) Location: Colorado Convention Center Room: Hall E1