Scientific Symposium

Gene delivery using lipid nanoparticles

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Abstract

There is an increasing interest in lipid nanoparticles (LNPs), which can be used for gene delivery. They comprise of synthetic ionized or ionizable lipids for DNA complexation along with an array of natural and synthetic helper lipids for structural integrity, steric stabilization, and to enhance cellular uptake, membrane fusion, and intracellular processing. LNPs were combined with innovative nanovector plasmid DNA (pDNA) constructs that lack a bacterial backbone sequence, making them both smaller in size and less susceptible to epigenetic silencing compared to conventional pDNA. We demonstrated that the lipid composition of LNPs significantly influences their transfection efficiency and potency. For instance, phosphatidylserine (PtdSer) enhances transfection efficiency of such particles by a factor of three. LNPs with optimized lipid compositions were evaluated in vitro using, among others, physicochemical characterization and an array of hepatocyte-derived cell lines. Zebrafish (Danio rerio) embryos were used as a vertebrate screening model to identify well tolerated LNPs with favorable pharmacokinetic properties and a high transfection efficiency in vivo. Our findings suggest that this translational approach can predict the in vivo performance of lipid-based delivery systems.

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Free

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