



"Next-Generation Approaches for Rapid Production of Nanoparticles for Use in Gene & Cell Therapies"



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Abstract: Genetic medicine employing siRNA, mRNA, and plasmid DNA enable medical genetic treatments of diseases via silencing genes, therapeutic protein expression, or modifying genetic code. However,

clinical application of these drugs often requires complex delivery systems. Lipid nanoparticles (LNPs) are currently the lead non-viral means of delivering genetic drugs, with one siRNA drug for hereditary transthyretin amyloidosis – a previously untreatable disease – recently approved for clinical use by the FDA. This session will cover current challenges of the LNP technology, as well as how some of them have been overcome. It will also include examples from literature highlighting the use of LNPs in revolutionizing medicine in the fields of oncology, rare diseases, and infectious diseases. Additionally, original data will be presented to demonstrate how a microfluidic nano-formulation platform is used to accelerate all stages of nanomedicine development from discovery to manufacturing.

Biosketch: Dr. Nguyen Huu devised formulations for ocular drug delivery and studied mechanisms of age-related eye disorders during his time at the University of California, San Diego. He has extensive expertise in formulation techniques involving polymers, small molecules, and proteins, as well as experience in analytical instrumentation and assays to characterize these constructs. Dr. Nguyen Huu currently serves as the Field Application Scientist for Precision NanoSystems.