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11TH WORLD DRUG DELIVERY SUMMIT

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Lipid nanoparticles for delivery of therapeutic oligonucleotides

Oligonucleotides, including antisense oligos, siRNA, miRNA mimics, anti-miRs, are promising as therapeutic agents because of their ability to regulate expression of specific or networks of genes that are critical in human diseases. However, clinical translation of oligonucleotide therapeutics has had limited success partly due to their limited nuclease stability and obstacles in their *in vivo* delivery, especially to tissues other than the liver. To address these issues, a combination of chemical modifications and lipid nanoparticle (LNP)-based delivery strategy has been developed. Design of LNPs needs to balance the requirements of stability in circulation and ability to facilitate intracellular delivery. Additional consideration is needed to address hematological biocompatibility and effects on the immune system. Specific examples will be provided on several novel LNP formulations for therapeutic delivery of miR-29b mimics, anti-miR21, and antisense oligos to Akt-1 in murine tumor models.

Biography

Robert J Lee has received his PhD in 1994 from Purdue University. He was trained as a Postdoc at the University of Pittsburgh School of Medicine and worked at GeneMedicine Inc. as a Sr. Scientist and then at Endocyte Inc. as VP of R&D. He has been a Professor in the College of Pharmacy at The Ohio State University since 1997 and has more than 200 publications in the areas of targeted drug delivery systems and lipid-based nanoparticles. He has served regularly on NIH review panels and as the PI or Co-PI on many large NIH and NSF projects. He has collaborated extensively with biotech industry in his research on oligonucleotide delivery systems.

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