



Pharmaceutical Sciences Seminar Series

Wednesday, September 4, 2024
4:00pm
NCRC Building 10 South Atrium
Zoom

“Tunable polymeric nanoparticles for *in vivo* nucleic acid delivery”

Presented by:



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Abstract: The primary barrier for clinical translation of nucleic acid therapeutics remains delivery to target tissues *in vivo*. Upon entering the body, delivery vehicles encounter extracellular and intracellular barriers. It is also unclear how carrier design features impact physiological interactions with biological systems, highlighting the need for studies that elucidate these structure-function relationships. In this seminar, I will describe our work on developing polymeric nanoparticles (NPs) to deliver therapeutic nucleic acids to a variety of tissues following systemic intravenous (IV) administration. We find that gene therapy can be significantly enhanced using a new class of polymeric vehicles consisting of poly(amine-co-ester) (PACE) polymers that are designed for safe and effective nucleic acid delivery. We have also developed tools to study nanomaterial-biology interactions in animal models, including a high-throughput quantitative microscopy-based platform to measure circulation half-life and biodistribution *in vivo*. Such tools, alongside the development of novel polymeric carriers and biodistribution “enhancers”, can be used to study the structure-function relationships that guide the physiological fate of NPs in order to rationally design more effective delivery vehicles for therapeutic delivery.