COLLEGE OF PHARMACY
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John G. Wagner Pharmacia & Upjohn Memorial Lecture

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BSRB 1020 Kahn Auditorium
Zoom
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Title: Design of Lipid Nanoparticles That Enable Gene Therapies

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Abstract: Delivery of nucleic acid-based drugs into target cells in vivo has been a major challenge for enabling gene therapies. This barrier is now being overcome due in part to advances made in lipid nanoparticle (LNP) delivery systems. LNP systems enable the mRNA COVID-19 vaccines and there are now a host of LNP RNA vaccines and therapeutics in clinical development. Advantages of LNP RNA systems over other delivery vectors include safety, ability to re-dose, essentially unlimited genetic cargo, ease of design, straightforward manufacturing processes, lower cost, and potential for highly personalized targeted therapeutics that can be developed in a matter of weeks. In this talk I will discuss the evolution of lipid nanoparticles for delivery of RNA and DNA and illustrate lead examples of therapeutic applications of LNP RNA systems to treat cancer, cardiovascular disease and rare diseases.