

**Mechanistic Insights and Advances in RNAi Therapeutics Platform****Karyn Schmidt, PhD**Principal Scientist  
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RNAi therapeutics are a clinically validated approach for the treatment of a wide range of debilitating diseases with high unmet medical need. Achieving safe and effective siRNA delivery was key to turn the scientific possibility of RNAi into a whole new class of medicines for patients with limited or inadequate treatment options. The presentation will cover our recent advances in delivery technologies for liver, CNS and eye. Ongoing efforts to maximize the potential therapeutic benefit of RNAi therapeutics focus on improving our mechanistic understanding of the RNAi pathway and the effects of chemically stabilizing modifications on potency and duration of effect by examining target organ exposure, intracellular trafficking, Ago2 loading and target RNA silencing. Enhanced understanding of the RNAi pathway has also enabled development of molecules capable of rapid and complete reversal of RNAi-mediated target gene silencing activity in vivo.



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**Host:** Dr. Johanna Carroll**Date:** Monday, November 13<sup>th</sup>, 2023**Time:** 3:00 PM**Place:** Room 254 (MC) Mechanical Engineering Bldg.