Nucleic acid delivery systems for RNA therapy and gene editing

High throughput, combinatorial approaches have revolutionized small molecule drug discovery. Here we describe our work using high throughput methods for developing and characterizing RNA delivery and gene editing systems. Libraries of degradable polymers and lipid-like materials have been synthesized, formulated and screened for their ability to delivery RNA, both in vitro and in vivo. A number of delivery formulations have been developed with in vivo efficacy, and show potential therapeutic application for the treatment of genetic disease, viral infection, and cancer.