Engineering Biologically-Relevant Drug Delivery Systems

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Abstract:

The core of research in the Kelly Lab integrates concepts from nanotechnology, materials science, neurobiology, and disease pathology for the design and assembly of polymeric drug delivery vehicles for applications in neurologic disease. In this presentation, I will discuss how the application of knowledge from each of these disciplines frames our current work on the development of tunable and translatable platforms utilizing responsive polymeric materials in the development of next-generation neurologic therapeutics.

Although neurological disorders account for more healthy years lost than AIDS, cancers, and heart disease, modern medicine has largely fallen short in the treatment of the brain due to the presence of the blood-brain barrier (BBB), which prevents passage of 98% of small molecule drugs from the blood into the brain. Research in the Kelly Lab focuses on using the biology of specific brain disorders to establish relevant targets, and thus relevant materials to respond to these targets, for drug delivery systems. Current efforts involve the use of nano-polymersomes, self-assembled polymeric vesicles synthesized from block co-polymers, as a platform for the transport of therapeutics across the BBB and into the brain. I will discuss our work in the design of a pH and enzyme-responsive polymeric vesicle that enables active enzyme delivery in the treatment of GM1 Gangliosidosis, a neurodegenerative lysosomal storage disease. In particular, the steps utilized to design the nano-polymersomes enabled us to efficiently encapsulate and deliver an active enzyme *in vitro*. The understanding gained during the investigation of this system resulted in the creation of a tunable, polymer-based platform for disease-specific and personalized therapeutics that can address otherwise unmet needs in modern medicine.