

#### **Research Leader**

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## Focal Technology Area 1: Nano-Biomedical Research

# <u>Bio-Inspired</u> <u>Nano-Carriers for</u> <u>Sub-Cellular Targeted</u> <u>Therapeutics</u>

### Research

Our research in the nono-biomedical Focal Technology Area (FTA) develops bio-inspired nano-carriers (NCs) aimed at sub-cellular targeting of therapeutics for the treatment of cancer and metabolic diseases. Uniquely, this program's focus on drug delivery systems and includes aspects such as: design of new nano-carriers, synthesis and characterization, intra-cellular trafficking, sub-cellular recognition, and localized cargo discharge, up to the level of in-vivo proof of efficacy. The benefits of our NCs will be dramatic for both doctors and patients, providing lower drug toxicity, more specific targeting and possibly reduced treatment costs. Getting the active compound to where it is needed and effectively delivering it, is one of the holy grails in the treatment of diseases ranging from inflammation to cancer.

Our NC encapsulated therapeutics provide both patient and payer value by:

- Using approved drugs and finding better ways of delivering them to their targets.
- Using bioinspired compounds for drug delivery that may extend the product and patent lifecycles of drugs, via reformulation of existing compounds.
- Developing new drugs and drug combinations that can be delivered together to increase the efficacy and potency for unmet medical indications.

### **Applications & Products**

Our 5 year FTA has focused on the following:

- Two RNAi nanoparticle drugs; targeting breast and ovarian cancer in NCs.
- Peptidic NC delivery of Lonidamine to mitochondria.
- NC-mediated inner membrane delivery of Phosphoinositides to modulate autophagy (a metabolic disease).
- Development of two specific gene therapy plasmid DNA vectors for the treatment of cancer.