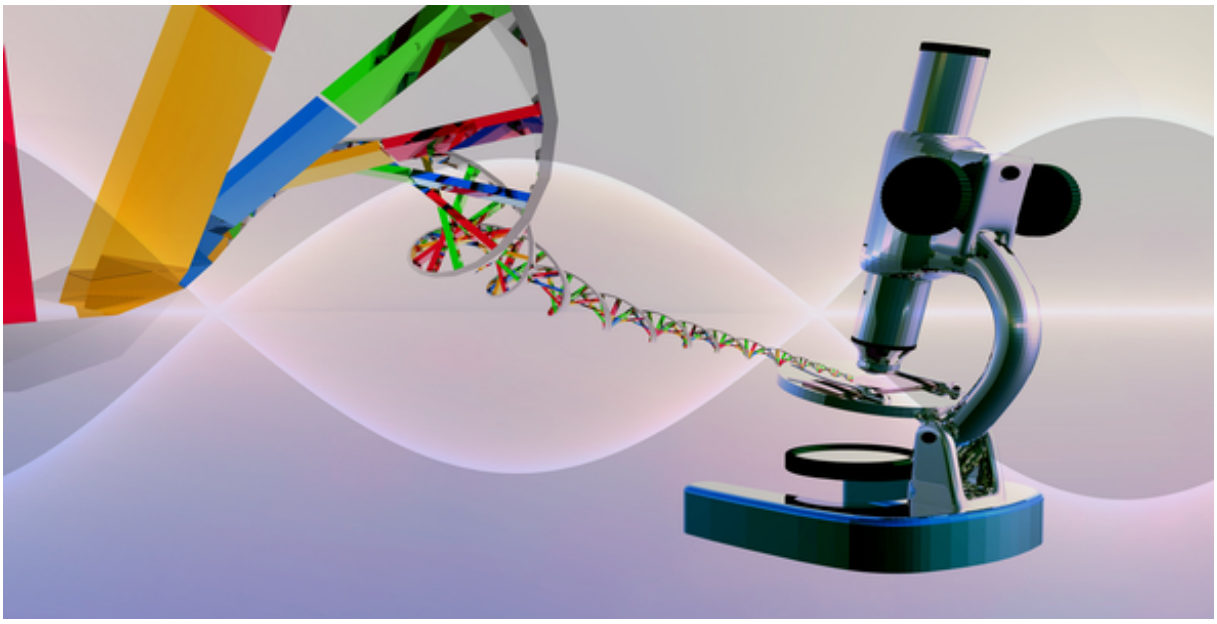
THE UNIVERSITY of NORTH CAROLINA at CHAPEL HILL

Drug Delivery

Targeted Intracellular Delivery of Therapeutic Oligonucleotides



Researchers at the University of North Carolina at Chapel Hill have developed a novel, high affinity, cell type specific method for enhancing intracellular delivery of antisense, siRNA, and microRNA oligonucleotides via targeting specific cell surface receptors capable of undergoing endocytosis. The oligo and targeting moiety can be chemically coupled to a protein carrier, which functionally increases the localized concentration of therapeutic RNA and provides biological effects in the nanomolar range.

Benefits

- **Non-Toxic** – in contrast to high toxicities of cationic lipid vehicles

- **Potent** – biological effects achieved in the nanomolar range as opposed to other peptide-oligo conjugates that require micromolar concentrations

- **Targeted** – specificity and flexible chemistry will allow for selective cell targeting

carolina blue™ opportunity

For More Information

If you would like more information about this technology or UNC - Chapel Hill's technology transfer program, please contact:

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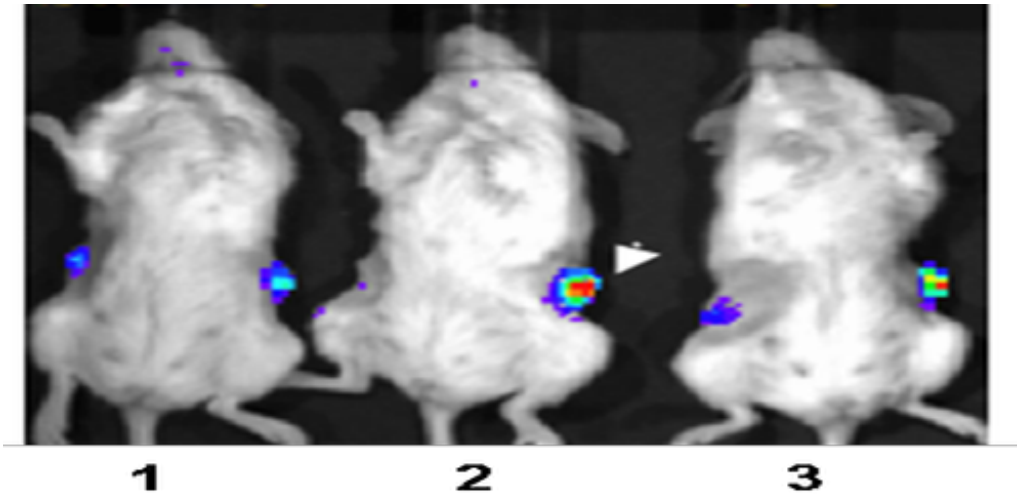
The Technology

Antisense oligos, small interfering RNAs and micro RNAs provide a promising new class of therapeutics. Despite various complications, the biggest remaining issue preventing widespread use of these RNAs as therapeutics is the absence of an effective delivery method. A number of vehicles have been generated such as viral vectors, nanoparticles, dendrimers, and conjugated peptide ligands. Each of these vehicles is associated with issues such as toxicity, lack of potency, limited biodistribution, and inadequate stability.

UNC has developed a high affinity, receptor specific delivery method that enters cells via a process of receptor-mediated endocytosis. Therapeutic oligos are paired with targeting moieties that deliver the oligo to the cell type of interest. Once endocytosis is triggered, the therapeutic oligo is steadily released into the cytoplasm where it can exert its biologic effect. This delivery vehicle utilizes oligos and a targeting moiety that can be chemically coupled to a protein carrier to increase localized concentration of the therapeutic RNA. This method achieves biological effects at nanomolar concentrations, is non-toxic, and can be easily designed to target selective cell types.

- Drug Delivery



Opportunity

UNC's Office of Technology Development seeks to stimulate development and commercial use of UNC-developed technologies. UNC is flexible in its agreements, and opportunities exist for joint development, academic or commercial licensing (exclusive, non-exclusive, and field-of-use), publishing, or other mutually beneficial relationships. A PCT application, WO2009045536 has published for this innovation.