

BIOENGINEERING

PRESENTS

In vivo Delivery of Nucleic Acids, Genome Editing Tools and Cells



THURSDAY, Oct 23, 2019

12:00 – 1:00 PM

2101 ENGINEERING V

Daniel G. Anderson, Ph.D.

Harvard-MIT

Professor,

Department of Chemical Engineering

ABSTRACT:

High throughput, combinatorial approaches have revolutionized small molecule drug discovery. Here we describe our work on the combinatorial development of biomaterials for medical devices ranging from nanoparticulate delivery systems to macroscopic devices. One focus of our work is on nanoparticulate, intracellular delivery systems for RNA therapy and gene editing. Libraries of degradable polymers and lipid-like materials have been synthesized, formulated and screened for their ability to deliver macromolecular payloads inside of cells. These nanoformulations facilitate *in vivo* delivery, enabling gene suppression with siRNA, gene expression with mRNA, or permanent genetic editing using the CRISPR/Cas9 system, providing therapeutic application for the treatment of disease in both rodent and primate models. A second focus of our work is on developing biomaterials that can avoid the fibrotic response common to implanted medical devices. Using combinatorial chemistry, we have developed new materials capable of avoiding fibrosis and scar tissue formation. These show particular promise as vehicles for the immune-isolation of transplanted cells, for the treatment of diabetes. When formulated into microcapsules these materials enable functional, long-term islet transplantation in immune competent, diabetic rodents, as well as normal non-human primates.

BIOGRAPHY:

Professor Daniel G. Anderson is a leading researcher in the field of nanotherapeutics and biomaterials. He is appointed in the Department of Chemical Engineering, the Institute for Medical Engineering and Science, the Koch Institute for Integrative Cancer Research, and the Harvard-MIT Division of Health Science and Technology at MIT. The research done in Prof. Anderson's laboratory is focused on developing new materials for medicine. He has pioneered the development of smart biomaterials, and his work has led to advances in a range of areas, including medical devices, cell therapy, drug delivery, gene therapy and material science. Prof. Anderson received a B.A. in mathematics and biology from the University of California at Santa Cruz and a Ph.D. in molecular genetics from the University of California at Davis. His work has resulted in the publication of over 400 papers, patents and patent applications. These advances have led products that have been commercialized or are in clinical development, as well as to the foundation of companies in the pharmaceutical, biotechnology, and consumer products space including CRISPR Tx (Nasdaq:CRSP), Living Proof, Sigilon, Verseau Tx, and Olivo Labs.