"Lipid-like Materials for RNA Delivery: A How-to Guide for Hacking Gene Expression"

Wednesday
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3:00 pm
Wu and Chen Auditorium
Levine Hall

Kathryn A. Whitehead
Associate Professor
Depts. of Chemical Engineering and
Biomedical Engineering
Carnegie Mellon University

Abstract

Despite the promise of RNA therapeutics, progress towards the clinic has been slowed by the difficulty of delivering RNA drugs, such as short interfering RNA (siRNA) and messenger RNA (mRNA), into cellular targets within the body. RNA therapeutics are large ($10^4 - 10^6$ g/mol) and negatively charged; they do not have favorable biodistribution properties in vivo nor an ability to cross the cellular membrane of target cells. In response to these challenges, our lab has created biodegradable, ionizable lipid-like materials called 'lipidoids' that readily formulate into nanoparticles containing RNA. Lipidoids efficiently manipulate gene expression in a variety of biological systems, including hepatocytes, white blood cells and tumors. This talk will focus on the cell-free prediction of lipidoid efficacy in delivering mRNA to mice. Furthermore, I will describe a new formulation strategy for the synergistic co-delivery of mRNA and siRNA. Together, these data demonstrate the potential of lipidoid materials to robustly manipulate gene expression in vivo.

Bio

Kathryn A. Whitehead is an Associate Professor in the Departments of Chemical Engineering and Biomedical Engineering (courtesy) at Carnegie Mellon University and a member of the McGowan Institute for Regenerative Medicine at the University of Pittsburgh. Her lab develops RNA and protein drug delivery systems and has a long-term goal of predicting the behavior of delivery materials in humans. She received an H.B.Ch.E Degree with Distinction from the University of Delaware (2002) and a Ph.D. in chemical engineering from the University of California, Santa Barbara (2007), before serving as an NIH Ruth L. Kirschstein Postdoctoral Fellow at the Massachusetts Institute of Technology (2008 – 2012). Prof. Whitehead is the recipient of numerous awards, including the NIH Director's New Innovator Award, the DARPA Young Faculty Award, the DARPA Director's Fellowship, the George Tallman Ladd Research Award, the *CMBE* Young Innovator Award, and the Kun Li Award for Excellence in Education. Prof. Whitehead was named as a Pioneer on the MIT Technology Review's Innovators Under 35 list in 2014, as well as one of the Brilliant Ten by Popular Science in 2015. Her 35+ publications have been cited over 4,500 times, and several of her patents have been licensed and sublicensed for reagent and therapeutic use.

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