



Department of Biomedical Engineering
Graduate Seminar



Dr. Niren Murthy, Ph.D.

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Subject: *In vivo* delivery of Cas9 ribonucleoprotein and donor DNA with gold nanoparticles

Abstract:

Cas9 based therapeutics have the potential to revolutionize the treatment of genetic diseases. However, safe and effective methods for delivering Cas9 protein, gRNA and donor DNA need to be developed before the therapeutic potential of CRISPR based therapeutics can be realized. In this presentation, I will describe a non-viral Cas9 delivery vehicle, termed CRISPR-Gold, which can induce homology directed DNA repair (HDR) *in vivo* by directly delivering Cas9 protein, gRNA, and donor DNA. CRISPR-Gold is composed of gold nanoparticles assembled with the Cas9/gRNA ribonucleoprotein (RNP) complex, donor DNA, and an endosomal disruptive polymer. We have been able to demonstrate that CRISPR-Gold can correct the DNA mutation that causes Duchenne muscular dystrophy (DMD) in mdx mice via HDR, with an efficiency of 5.4% after an intramuscular injection and with minimal levels off-target DNA damage. In addition, CRISPR-Gold was able to deliver Cas9 RNP into the brain, after an intracranial injection, and rescue mice from FMR1 related autism, via deletion of the mGluR5 gene via non-homologous end joining (NHEJ). CRISPR-Gold is a non-viral delivery vehicle that can generate HDR and NHEJ *in vivo* and has potential for treating muscle and neurological diseases.

Bio:

Dr. Niren Murthy is a professor in the Department of Bioengineering at the University of California at Berkeley. Dr. Murthy's scientific career has focused on the molecular design and synthesis of new materials for drug delivery and molecular imaging. The Murthy laboratory has been recently focused on developing non-viral delivery vehicles that can deliver Cas9 protein, gRNA and Donor DNA *in vivo*.

Date and time: Tuesday May 4th, 2021

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