



Niren Murthy

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Niren Murthy is a professor in the Department of Bioengineering at the University of California at Berkeley. Niren received his PhD from the University of Washington in Seattle in Bioengineering in 2001, under the guidance of Professors Allan Hoffman and Patrick Stayton, and did postdoctoral work in the Chemistry department at U.C. Berkeley from 2001-2003 in the laboratory of Professor Jean Frechet. Niren started his independent career in 2003 at Georgia Tech's Biomedical Engineering department, and in 2012 moved back to U.C. Berkeley. The Murthy laboratory is focused on the molecular design and synthesis of new materials for drug delivery and molecular imaging.

The Murthy laboratory has developed several new nanoparticulate technologies for drug delivery, such as the polyketals and CRISPR-Gold, which have been used by numerous laboratories and companies to enhance the delivery of small molecules and proteins. The Murthy laboratory developed the hydrocyanines in 2009, which are now a commonly used class of molecules for imaging reactive oxygen species and commercially available from multiple sources. Three start-up companies have spun out from the Murthy laboratory, termed GenEdit, Microbial Medical and BioAmp Diagnostics. Niren received the NSF CAREER award in 2006, and the 2009 Society for Biomaterials Young Investigator Award.

Abstract: Therapeutic Gene Editing Enabled by New Drug Delivery Vehicles

Cas9 based therapeutics have the potential to revolutionize the treatment of genetic diseases. However, safe and effective methods for delivering the Cas9-guide RNA complex (Cas9 RNP) need to be developed before the clinical potential of Cas9 based therapeutics can be fully realized. In this presentation, I will describe non-viral delivery strategies developed in our laboratory for delivering the Cas9 RNP. The first delivery strategy is termed CRISPR-Gold. CRISPR-Gold is composed of gold nanoparticles assembled with the Cas9/gRNA ribonucleoprotein (RNP) complex, donor DNA, and an endosomal disruptive polymer. CRISPR-Gold was able to correct the DNA mutation that causes Duchenne muscular dystrophy (DMD) in mdx mice via homology directed DNA repair (HDR), with an efficiency of 5.4% after an intramuscular injection. In addition, CRISPR-Gold was able to edit the brains of adult mice and rescued mice from the repetitive behaviors caused by autism. In this presentation, I will also describe other strategies for delivering the Cas9 RNP based on encapsulation in block copolymers and conjugation with peptides. Collectively, our experience suggests that non-viral strategies for delivering the Cas9 RNP have great potential for treating DMD and other genetic diseases.