Joint Department of

BIOMEDICAL ENGINEERING

UNC CHAPEL HILL | NC STATE UNIVERSITY

Coulter Seminar Series Presents

"Genome editing based approaches for treating human diseases"

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Dr. Gang Bao is the Foyt Family Professor and Chair of the Department of Bioengineering, Rice University. He is also the Director of Nanomedicine Center for Nucleoprotein Machines at Rice. Dr. Bao received his undergraduate and Master's degrees from Shandong University in China, and his PhD from Lehigh University in the US. Dr. Bao is a Fellow of the American Association of Advancement in Science (AAAS), American Society of Mechanical Engineers (ASME), American Physical Society (APS), American Institute for Medical and Biological Engineering (AIMBE), and Biomedical Engineering Society (BMES). Dr. Bao's current research is focused on the development of nanotechnology and biomolecular engineering tools for biological and disease studies, including multifunctional magnetic nanoparticles, protein tagging/targeting methods, and engineered nucleases such as CRISPR/Cas9. These approaches have been applied to the diagnosis and treatment of chronic diseases such as cancer, and the development of genome editing approaches for treating single-gene disorders.

ABSTRACT

Genome editing using engineered nucleases such as CRISPR/Cas9 systems is revolutionizing life sciences and medicine. It provides unprecedented opportunities for treating many genetic disorders. However, significant challenges exist in order to treat a disease with high efficacy and safety using genome editing. In this talk I will first focus on the development of a CRISPR/Cas9 based genome editing approach to treat sickle cell disease (SCD), which is a monogenic disorder that affects millions worldwide. We have systematically optimized the CRISPR gRNA and ssODN donor template designs, and achieved high gene editing rates in CD34+ HSPCs from patients with SCD. We have also performed extensive off-target analysis of gene-edited HSPCs and demonstrated the clinically relevant levels of engraftment in immunodeficient mice. I will then illustrate the importance of analyzing gene editing outcomes at the on-target Cas9 cut-site and their biological implications. Finally, I will describe a unique delivery vehicle for in vivo genome editing by combining magnetic nanoparticles (MNP) with baculoviral (BV) vector, and discuss the challenges and opportunities of in vivo genome editing

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Presented From: 4142 Engineering Building III (NC State)
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& East Carolina University (ECU)