Biology Seminar

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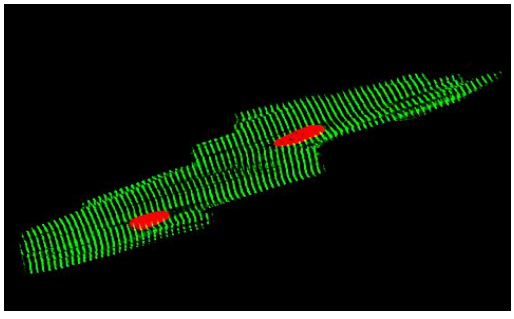
Applications of Gene Editing and Regenerative Medicine for the Correction of Muscle and Heart Disease

We seek to delineate the mechanisms that govern development, disease and regeneration of the heart and other muscles and to build upon this knowledge to restore muscle function during disease and aging. As one approach toward this goal, we are applying CRISPR/Cas9-mediated genome editing to promote muscle repair in Duchenne muscular dystrophy (DMD), which is caused by mutations in the dystrophin gene. We have established new cellular, genetic, and biochemical tools, as well as novel strains of genetically modified mice, for optimizing gene correction in vivo. We have also discovered a new myogenic stem cell with potent regenerative activity for adult muscle, providing a means of promoting muscle regeneration and repair. Opportunities and obstacles in the path toward regeneration and permanent correction of diseases of muscle and the heart will be discussed.



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