Therapeutic Genome Editing for Genetic Diseases

Monday, November 5, 2018 | 12:00PM - 1:00PM

The Jill and John Freidenrich Center for Translational Research (FCTR)
800 Welch Road, Room 180

Genome editing provides a mechanism to precisely change the DNA sequence of cells and holds promise for the treatment and prevention of single-gene disorders. These lectures will describe the developing of precision genome editing to a broad variety of human cell types in order to develop novel curative cell-based therapies for genetic diseases affecting various of organ systems.

MATTHEW PORTEUS, MD, PHD

“Genome Editing of Stem Cells to Cure Genetic Diseases of the Blood and Immune System”

Dr. Matthew Porteus is a Professor in the Department of Pediatrics and a member of the Institute of Stem Cell Biology and Regenerative Medicine and of the Child Health Research Institute at Stanford University. His primary research focus is on developing genome editing as an approach to cure disease, particularly those of the blood but also of other organ systems as well. His research program has made important discoveries in advancing the field of genome editing including the first use of genome editing using engineered nucleases in human cells and optimizing the use of the CRISPR/Cas9 system in primary human stem cells. He also works as an attending physician on the Pediatric Hematopoietic Stem Cell Transplant service at Lucile Packard Children’s Hospital where he cares for children under going bone marrow transplantation for both malignant and non-malignant diseases. His goal is to combine his research and clinical interests to bring innovative curative therapies to patients.

NATALIA GOMEZ-OSPINA, MD, PHD

“Beyond the Blood: Engineering the Hematopoietic System to Treat Multisystemic diseases”

Dr. Natalia Gomez-Ospina is an Assistant Professor in the Division of Medical Genetics. For her clinical practice, she sees patients with suspected genetic disorders. She leads the Program for Inherited Metabolic Disorders (PIMD) at Stanford whose mission is to provide more comprehensive care for patients with neurometabolic disorders and spearhead the preclinical development, and clinical testing of gene and cell-based therapies for these diseases. Her research program is on developing genome editing of hematopoietic stem cells as therapies for a class of child neurodegenerative diseases known as lysosomal storage disorders.

LIMITED LUNCH PROVIDED, FIRST COME, FIRST SERVED!

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