MATTHEW PORTEUS, MD, PHD
Stanford University, School of Medicine
Department of Pediatrics, Hematology/Oncology/Stem Cell Transplantation/Cancer Biology
Lorry Lokey Stem Cell Research Building, G3045 269 Campus Drive, MC 5462
Stanford, CA 94305
Phone: (650) 725-6520
Email: mporteus@stanford.edu

TITLE: Genetically engineering cells as personalized drugs to cure disease

ABSTRACT: There are currently two well-established broadly used categories of drugs as therapeutics for human disease: small molecules and biologics such as antibodies. There are several new categories of drug therapies that may expand the toolbox that clinicians might have available to treat or cure disease including nucleic acid therapeutics, gene transfer vectors (gene therapy), and engineered cell therapy.
Each of these new therapeutic categories has features that might make them uniquely qualified to address specific diseases in novel ways with improved efficacy with greater cost effectiveness than other therapies. In this talk, I will discuss our progress in using genome editing to engineer cell based therapies to treat disease. These strategies include using genome editing of hematopoietic stem cells to treat genetic diseases of the blood and immune system, engineering the immune system to be HIV resistant and engineering cells to secrete therapeutic proteins.