The Centre for Blood Research

Dr. Fraser Wright
Clinical Vector Core Laboratory
Children's Hospital of Philadelphia

Wednesday, Oct 12, 2011
12:00pm
in LSC3

Life Sciences Centre
2350 Health Sciences Mall

"Advances in Gene Therapy for Hemophilia"

Description of Research Expertise:
My primary research interests are to support clinical gene therapy studies, and to investigate and develop strategies to overcome immune barriers to human gene therapy. Recombinant adeno associated virus (AAV) has demonstrated great promise in achieving efficient and durable expression of therapeutic transgenes in animal models. Excellent safety and limited efficacy have been observed in a clinical studies using AAV vectors. However, host immune responses can reduce efficiency and durability of gene transfer for some routes of administration. Designing and preparing AAV vectors that minimize activation of innate and adaptive immunes responses following in vivo administration is one important focus in my lab. This involves developing optimized vector generation and purification strategies, and 'humanizing' AAV vectors, for example by biochemical modifications, to further reduce the immunogenic profile of viral vectors. These approaches have the potential to enhance the clinical applications of recombinant AAV for therapeutic gene transfer.

Description of Clinical Expertise:
I direct a Clinical Vector Core Laboratory that designs, prepares and certifies recombinant adeno associated viruses (AAV) for use as investigational new drugs in clinical studies. The Core Laboratory follows current Good Manufacturing Practices (cGMP) required for early phase clinical studies. Several ongoing and planned clinical studies, including recombinant AAV-mediated delivery of the gene encoding retina pigment epithelium associated 65kDa protein (RPE65) for Leber's Congenital Amaurosis (Maguire et al. Safety and efficacy of gene transfer for Leber's Congenital Amaurosis. The New England Journal of Medicine 358:2240-2248, 2008), and recombinant AAV-mediated delivery of the gene encoding coagulation factor IX (f.IX) for hemophilia B (Manno et al. Successful transduction of liver in hemophila B by AAV-Factor IX and limitations imposed by the host immune response. Nature Medicine 12:342-347, 2006), are supported by the Clinical Vector Core Laboratory.

This Seminar is sponsored by:

Bayer HealthCare

Host: Dr. Ed Pryzdial, Clinical Professor Pathology and Laboratory Medicine & Centre for Blood Research

Refreshments will be served 10 minutes before the seminar
Seminar information: 604 822 7407