



The Centre for Blood Research Seminar Series



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“Platelet Targeted Gene Therapy for Hemophilia”

Gene therapy for hemophilia has usually been targeted to the liver yet FVIII is normally made in endothelial cells where it is stored with VWF and released by endothelial agonists such as DDAVP or epinephrine. In the megakaryocyte, FVIII is not synthesized normally. If FVIII is targeted to megakaryocytes, however, FVIII is also stored with VWF but in the platelet alpha granule. Interestingly, this stored FVIII is not recognized by FVIII inhibitory antibodies and when the platelet releases VWF at the site of vascular injury, FVIII is also released and is efficacious even in the presence of high titer FVIII inhibitory antibodies. Thus FVIII targeting to platelets is effective gene therapy for hemophilia A even in patients with pre-existing FVIII antibodies.



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Tuesday, March 5th, 2013

11:00am - 12:00pm

LSC 3 - Life Sciences Centre

2350 Health Sciences Mall



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