

## **The Gene Technology Laboratory, Department of Genetic Medicine, Women's and Children's Hospital**

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The Gene Technology Laboratory is involved on the development and application of lentiviral vectors for gene therapy. Central to the laboratory is the continued development of our lentiviral vector technology, with a current emphasis on large scale production of virus and the development of complement resistant virus. In house applications of our technology include the development of gene therapy for the lysosomal storage disorders, methylmalonic aciduria and cystic fibrosis. Students with an interest in any of these areas are encouraged to discuss their interests with me.

### **References**

#### **Vector**

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Anson, DS. and Fuller, M. (2003). Rational development of a HIV-1 gene therapy vector. *J. Gene Medicine*, **5**, 829-838.

Koldej, R., Cmielewski, P., Stocker, A., Parsons, DW. and Anson, DS. (2005). Optimisation of a Multipartite Human Immunodeficiency Virus Based Vector System; control of virus infectivity and large-scale production. *J. Gene Medicine* **7**, 1390-1399.

#### **Lysosomal storage disorders**

Anson, DS., McIntyre, C., Thomas, B., Koldej, R., Ranieri, E., Roberts, A., Clements, PR., Dunning, K., and Byers, S. (2007). Lentiviral mediated gene therapy for mucopolysaccharidosis type IIIA. *Genetic Vaccines and Therapy*, **5**, 1

McIntyre, C, Roberts, AL, Ranieri, E, Clements, PR, Byers, S. and Anson, DS. (2008). Lentiviral-mediated gene therapy for murine mucopolysaccharidosis type IIIA. *Molecular Genetics and Metabolism* **93**; 411-418.

#### **Cystic fibrosis**

Limberis, M, Anson, DS, Fuller, M. and Parsons, DW. (2002). Recovery of airway cystic fibrosis transmembrane conductance regulator function in mice with cystic fibrosis after single dose lentivirus-mediated gene transfer. *Human Gene Therapy*, **13**, 1961-1970.

Kremer, K., Dunning, K., Parsons, D. W. and Anson, D. S. (2007). Gene Delivery to Airway Epithelial Cells in vivo: A Direct Comparison of Apical and Basolateral Transduction Strategies using Pseudotyped Lentivirus Vectors. *J. Gene Medicine* **9**, 362-368.