

# Supporting MPS Research

**The Irish MPS society is helping to fund Dr Brian Bigger in his research on MPS diseases. He is based at the School of Medicine, Medical Genetics at the University of Manchester**

**The Medical Genetics Research Group is part of the Research School of Clinical and Laboratory Sciences within the School of Medicine Medical Genetics at the University of Manchester**

<http://www.manchester.ac.uk/>

## **Background information**

### **MPS Stem Cell Research Laboratory**

Principal investigator: **Dr Brian Bigger**

There is considerable research strength in this partnership with the establishment of the MPS Stem Cell Research Laboratory in 2006 under Dr Brian Bigger.

Current research projects are aimed at further understanding the mechanisms of disease pathology in Mucopolysaccharidosis (MPS), development of more effective diagnostic tools for these diseases, and the generation of novel and clinically relevant therapeutic approaches to curing these diseases.

### **Current Research**

Dr Fiona Wilkinson, a postdoctoral associate, is currently examining the potential of defined stem cell subsets from bone marrow to repopulate organs that are difficult to treat in MPS, such as the brain and bones.

The risks of haematopoietic stem cell transplantation (HSCT) to the MPS patient are high due to the immune responses between the donor and patient cells. Kia Langford (research technician and part-time PhD student) is developing immunomodulators and T regulatory cells to achieve transplant tolerance using less severe chemotherapy.

In MPSIH (Hurler syndrome), HSCTs are not always successful first time. Angharad O'Leary (PhD student) is investigating the mechanisms of cell engraftment in MPS and how we can improve stem cell homing and engraftment.

Alex Smith (PhD student) is developing a combined stem cell and gene therapy approach for MPS IIIA (Sanfilippo syndrome). This involves improving the delivery of the missing enzyme by modifying stem cells in the bone marrow to produce a large quantity of enzyme to correct the disease.

Marcelina Malinowska, a visiting fellow from the University of Gdansk, Poland, is investigating the use of substrate or GAG reducing agents to improve the symptoms of MPS III subtypes.

Dr Omar N Pathmanaban, a clinical research fellow and trainee neurosurgeon is studying the role of stem cells in brain tumour formation with the help of Shahnaz Khan (technician). These techniques are very similar to those that we are using to develop stem cell therapies for MPS.

The combined expertise in the group brings together clinicians with expertise in metabolic disease, transplantation and neuroscience, the Willink Biochemical Genetics Unit for metabolic disease diagnosis and a strong group of basic research scientists working towards new therapies for MPS diseases and insights into how the diseases progress

#### **Staff members**

Dr Fiona Wilkinson  
Mr Omar Pathamanaban  
Miss Kia Langford  
Miss Shahnaz Khan

#### **PhD students**

Miss Angharad O'Leary  
Mr Alex Smith  
Visiting fellows  
Miss Marcelina Malinowska  
Miss Malani Arasadnam

#### **Collaborators**

Dr Maria Canal, University of Manchester, Manchester  
Dr Jon Cooper, King's College, London.  
Dr Cathy Merry, University of Manchester, Manchester  
Professor John Hopwood, Women's and Children's Hospital, Adelaide, Australia

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