

## **Sharon Byers**

Matrix Biology Unit, CYWHS.

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The Matrix Biology Unit is situated in the department of Genetic Medicine, Women's and Children's Hospital, which is the Australasian centre for the diagnosis and treatment of children with MPS and other lysosomal storage disorders. Our research focus is the development, testing and commercialization of therapies for the mucopolysaccharidosis (MPS) group of inherited metabolic disorders.

### **Student projects:**

MPS disorders arise from a reduction or absence of an enzyme required for the turnover of glycosaminoglycan (gag) carbohydrates. As a result gags gradually build up in a wide range of cells leading to multiple organ failure. Affected children commonly die in their teens/early twenties from respiratory or cardiac failure or inanition due to severe CNS pathology. Research carried out in our unit played an important role in the clinical application of enzyme replacement therapy for MPS disorders. However, despite the success of ERT in halting most pathology associated with MPS, several tissues remain untreatable. These are the brain, cartilage and cornea. Current research is focused on alternative or supplemental treatments that address pathology in these tissues.

Students can join ongoing projects evaluating:-

- substrate deprivation therapy for MPS using RNAi or small chemical inhibitors
- gene therapy for MPS
- improved methods for recombinant MPS enzyme expression

1 or 2 honours students can be taken per year.



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