

# Stem cells hold hope for Hurler's syndrome

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University of Adelaide research using special adult stem cells is promising new hope for better treatments for the devastating genetic disease Hurler's syndrome.

Hurler's syndrome has a frequency of one in 100,000 live births in Australia and sufferers develop severe mental and physical disabilities and often die in their early teens.

The disease is caused by a single defective enzyme that is essential for breaking down complex sugars in cells.

The researchers are modifying adult [stem cells](#) to make them produce large amounts of the deficient enzyme, and using them to replace cells which aren't functioning properly throughout the body. Preliminary results in laboratory studies are showing improvements in [brain function](#).

"We have turned [adult stem cells](#) into little 'enzyme factories' by coupling them with a virus that makes them pump out high levels of the enzyme," says PhD candidate Matilda Jackson from the University's School of Molecular and Biomedical Sciences.

"Those stem cells can then be injected into the blood where they move around the body and become liver or bone or brain or other cells and start producing the missing enzyme. They automatically migrate to the areas of damage in the affected individual.

"So far in our laboratory studies we've measured improvements in brain

function but we're yet to complete the analysis to determine if there are improvements in other organs."

Matilda is supervised by Dr Sharon Byers, Affiliate Senior Lecturer within the School of Molecular and Biomedical Sciences and Head of the Matrix Biology Unit at SA Pathology, based at the Women's and Children's Hospital.

"There are two current treatments for Hurler's syndrome - costly enzyme replacement therapy or [bone marrow transplants](#) which require a perfectly matched donor," says Dr Byers. "And while they bring some improvement, neither of these treatments prevents damage to the brain and bones because not enough enzyme reaches either of these tissues.

"These stem cells, modified so they produce large quantities of the [enzyme](#) that people with Hurler's syndrome lack, offer great hope for a potential new therapy. If we can help reverse the disease symptoms, we could see these children able to perform normal tasks, giving them a better quality of life and increasing their life span."

Provided by University of Adelaide

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