

-Media Release-
**\$2 million secured for major collaborative project to tackle
Sanfilippo syndrome**

The **Sanfilippo Children's Foundation** is thrilled to announce it has secured \$2 million dollars of Federal Government funding and engaged leading researchers in Adelaide for a research project set to make great strides towards effective treatments for Sanfilippo syndrome – a rare type of childhood dementia.

Researchers from the South Australian Health and Medical Research Institute (SAHMRI), Adelaide's Women's and Children's Hospital and the University of Adelaide, in partnership with the **Sanfilippo Children's Foundation**, will create cell models that will be used to search for drugs to address Sanfilippo and provide a world-first personalised medicine model for the condition.

Associate Professor Kim Hemsley from SAHMRI is one of the project's Chief Investigators.

"We will use advances in the development of personalised cellular methods and drug screening technology to test a wide range of experimental compounds and existing medications, searching for an effective therapy for Sanfilippo," Dr Hemsley said.

Researchers will take skin cells from patients and reverse engineer them into neural cells, creating a "brain in a dish" – an individualised representation of a person's brain.

"Testing using a patient's own cells fast-tracks the research because it enables multiple drug combinations to be trialed rapidly and without risks to the children themselves."

The **Sanfilippo Children's Foundation** will fully fund the project having worked with the researchers to secure \$2 million from the Federal Government's Medical Research Future Fund (MRFF) and chipping in a further \$500,000 of their own funds.

Dr. Nicholas Smith, the head of the Paediatric Neurodegenerative Diseases Research Group University of Adelaide and the Paediatric Neurology Service at the Women's and Children's Hospital in Adelaide is the project's other Chief Investigator. He welcomes the funding as a demonstration of the Government's commitment to invest in rare disease research.

He also believes this research "has the potential to yield findings with far-reaching clinical influence on many more common neurological diseases."

Work has already started on this two-year project that will involve two other key researchers: Dr. Cedric Bardy at SAHMRI and the University of Adelaide's Professor Mark Hutchinson. A panel of Australian and international experts will help steer the project.

Megan Donnell, Executive Director of the Sanfilippo Children's Foundation said: "We are thrilled to be partnering with the Government and world-leading researchers in Adelaide to accelerate research towards effective treatments for this devastating condition.

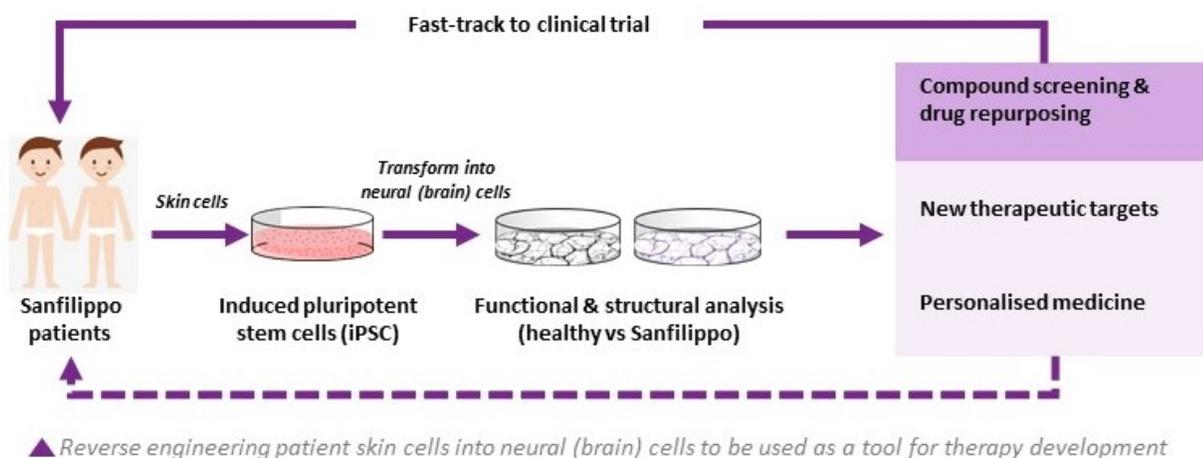


“We hope this ground-breaking method of personalised drug screening will lead to treatments that will help improve the quality of life of children battling Sanfilippo, while also potentially sparing them from invasive treatments and their accompanying side effects.”

“Five children are born every year in Australia with Sanfilippo Syndrome and there is currently no treatment or cure available. We have made it our mission to change this!”

Sanfilippo Syndrome, also known as Mucopolysaccharidosis (MPS) III, is a serious degenerative condition that causes fatal brain damage, and for which there is currently no effective treatment or cure. Life expectancy for children with the condition is just 12-20 years.

Over time the brain is progressively damaged. Children with Sanfilippo experience severe hyperactivity, disordered sleep, loss of speech, intellectual disability, cardiac issues, seizures, loss of mobility and finally death, usually before adulthood.



About the Sanfilippo Children’s Foundation: The Sanfilippo Children’s Foundation is an Australian medical charity dedicated to progressing clinical research towards a cure for the fatal childhood dementia, Sanfilippo Syndrome. The Foundation was started by Sydney mother Megan Donnell following the shock diagnosis of both her children with the condition. The mission of the Foundation is to *fund medical research so a cure can be found in time for children battling Sanfilippo today and those born with it tomorrow.*

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For more on the Sanfilippo Children’s Foundation, or to make a donation, see: www.sanfilippo.org.au. You can also follow the foundation on Facebook [@sanfilippochildrensfoundation](https://www.facebook.com/sanfilippochildrensfoundation), Twitter [@SFCFoundtn](https://twitter.com/SFCFoundtn), Instagram [@sfcfoundtn](https://www.instagram.com/sfcfoundtn)

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