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Liver disease breakthrough delivers hope to sick children

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Rhiannon Lunney with her son Cooper, 3, who has just had a liver transplant / Picture: Richard Dobson

AN exciting breakthrough in treating genetic liver diseases in children could one day prevent kids from needing a transplant.

The team at the Children's Medical Research Institute at Westmead has shown how gene therapy can replace the defective gene responsible for genetic liver disease and allow the organ to begin functioning properly.

It can cure genetic liver diseases in adult mice and produce a "transient effect'' on infant mice whereby the disease went away but later returned. The institute's Gene Therapy Research Unit head Ian Alexander said the challenge was far more vexing in a still-growing liver but "the idea there, would be to prevent the need for liver transplantation".

Gene therapy is a relatively new field which aims to correct the DNA code and fix genetic disorders at the source.

This research into genetic liver disease builds on the use of “adeno-associated viral vectors” which have already proven to be highly successful in clinical trials for the treatment of adult haemophilia. “We have been tweaking this technology and building on it to make it appropriate for the growing liver,” Professor Alexander said.

The research is jointly funded by the National Health and Medical Research Council and proceeds from today’s annual Jeans for Genes Day.

It is a significant advancement for kids like Cooper Hammond, 2, who was born with a urea cycle defect and was in an incubator until a liver transplant last December. “It would mean the world for a family to not have to go through that (transplant),” his mother Rhiannon Lunney said.

Rhiannon Lunney with her son Cooper, 3, who has just had a liver transplant / Picture: Richard Dobson