Brad Hazzard
Minister for Health
Minister for Medical Research

MEDIA RELEASE

Monday 15 October 2018

NSW LEADS TRIAL TO SAVE MORE BABIES' LIVES

A Sydney baby is the first in the world outside of North America to receive lifesaving gene-replacement treatment under a NSW Government $2 million newborn screening pilot.

Health Minister Brad Hazzard said in an Australian first, all babies born in NSW and the ACT are now offered screening for the deadly condition Spinal Muscular Atrophy (SMA), which is the leading genetic cause of infant death in Australia.

"This is a tragic condition – in some cases, babies are born so weak they only survive a few weeks. This trial will potentially give those babies a much greater chance at life," Mr Hazzard said.

"Within a month of introducing SMA to the newborn bloodspot screening program, a baby girl was diagnosed before any symptoms had begun and she is now getting lifesaving gene-replacement therapy."

The routine heel prick screening will also now include Primary Immunodeficencies (PID) – a range of serious disorders that weaken the immune system that occur in 1 in 40,000 births and are usually fatal in a baby's first year of life.

The additional screening over two years is being funded by the NSW Government through Paediatrio, the NSW paediatric research collaboration between Sydney Children's Hospitals Network, Children's Medical Research Institute and Children's Cancer Institute.

Sydney Children's Hospitals Network paediatric neurologist Dr Michelle Farrar said early screening for SMA and some PID conditions will greatly improve outcomes.

"It will help detect these rare and life-threatening conditions in newborns and improve access to clinical trials and early treatment," Dr Farrar said.

"One approach to treatment is gene-replacement therapy, given as a one-off dose before symptoms arise and could save a baby's life."

The newborn bloodspot program screens for more than 25 medical conditions, including cystic fibrosis, primary congenital hypothyroidism, phenylketonuria and congenital adrenal hyperplasia.

SMA occurs in one in every 10,000 births and affects motor nerve cells in the spinal cord, causing progressive muscle weakness through to adulthood.

Julie Garvey | Minister Hazzard | 0488 569 746