BioSpectrum the business of Bio & Health Sciences ASIA EDITION

Australian first biobank to discover new treatments for genetic muscle diseases in children

25 September 2023 | News

To improve and discover new treatments for children with genetic muscle diseases such as muscular dystrophy

The National Muscle Disease Bio-databank, co-led by Murdoch Children's Research Institute, Monash University and Alfred Health, will advance research into understanding why children develop genetic muscle diseases. The project forms part of a $2.5 million Medical Research Future Fund grant awarded to the team for research into congenital muscle diseases.

These diseases, spanning dystrophies and myopathies, are characterised by severe muscle weakness, usually from infancy, that can impact swallowing, breathing and lead to eye problems and learning difficulties. About 30 people a year are diagnosed with a congenital muscular disease in Australia of which half will have a genetic basis identified.

Murdoch Children's Dr Peter Houweling said there was an unmet need for affordable treatments that could be fast-tracked into clinical trials.

To address this, the biobanking facility housed at Murdoch Children's will collect patient information and store blood test and skin biopsy samples from children across Australia with genetic muscle disease.

A national, multidisciplinary network including paediatric neurologists, pathologists, scientists and patient advocacy groups have been assembled for the biobank project. Key collaborators include the Australian Neuromuscular Disease Registry, the Women's and Children's Hospital in Adelaide, Monash Health, the University of Melbourne, The Royal Children's Hospital, Children's Health Queensland, Sydney Children's Hospitals Network, The Harry Perkins Institute in Western Australia, Muscular Dystrophy Australia and the Fred Liuzzi Foundation.