

# Media Release

Mr Danny Pearson MP

Minister for Economic Growth and Jobs  
Minister for Finance



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## NEW RNA THERAPY TO TACKLE RARE CHILDHOOD DISEASES

The Allan Labor Government is supporting Melbourne researchers who have discovered a groundbreaking RNA treatment targeting rare childhood diseases, including bone marrow failure syndrome.

Minister for Economic Growth and Jobs Danny Pearson has visited St Vincent's Institute of Medical Research to meet Associate Professor Andrew Deans, the local researcher behind an innovative RNA therapy that could transform the lives of children with rare blood disorders.

In a major breakthrough, the cutting-edge RNA Prime Editing technology corrects the genetic defect that causes life-threatening blood disorders such as Fanconi anemia, potentially eliminating the need for risky and costly bone marrow transplants.

Fanconi anemia is a rare, genetic disorder that causes bone marrow failure and affects children from a very young age. About 190 young Australians are currently diagnosed with the syndrome. Sadly, more than half will die from the disease and its related conditions.

The new treatment is progressing through safety studies, in partnership with Maddie Riewoldt's Vision – an organisation dedicated to finding a cure for bone marrow failure syndrome, inspired by Maddie's tragic death in 2015 – with the aim of launching a world-first clinical trial for patients.

The Labor Government invested \$100,000 in 2023 towards the development of this RNA-based gene editing technology.

The Labor Government's investment of more than \$1 billion in health and medical research continues to drive innovation, create jobs, and deliver life-changing breakthroughs in treatment and care.

This includes more than \$30 million to support 63 research projects since 2021 – unlocking new medicines and vaccines and establishing a world-leading RNA industry in Victoria.

### Quotes attributable to Minister for Economic Growth and Jobs Danny Pearson

*"It's exciting to see this research rapidly advancing towards clinical trials, offering real hope for children and families battling these devastating diseases.*

*"Our medical research and health technology sector is the largest sector in Australia – supporting more than 30,000 jobs and enabling Victorian scientists to change lives around the world."*

### Quote attributable to Member for Northern Metropolitan Sheena Watt

*"We're proud to support world-leading research right here in our local community, bringing real hope to families affected by rare childhood diseases."*

### Quote attributable to St Vincent's Institute of Medical Research Director Professor Thomas Kay

*"Investment in medical research gives St Vincent's Institute scientists the best chance of maximising new mRNA technology to develop leading-edge therapy to combat rare childhood diseases like Fanconi anemia."*

**Media contact:** Lukas Jamieson 0477 373 170 | [lukas.jamieson@minstaff.vic.gov.au](mailto:lukas.jamieson@minstaff.vic.gov.au)

**Quote attributed to Maddie Riewoldt's Vision, Chief Executive Officer Amy Coote**

*"Maddie Riewoldt's Vision is thrilled to partner with St Vincent's Institute on this groundbreaking project. We're incredibly grateful for A/Prof Andrew Deans and his team's commitment to patient and family engagement, and thankful for the Victorian Government's investment in advancing gene editing technology that offers real hope for those living with bone marrow failure syndromes."*