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Neuroblastoma funding left redundant by access scheme sitting idle

The Albanese government is tight-lipped on the fate of funding allocated for a rare children's cancer despite lobbying by families and advocates.

By JAMES DOWLING

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Millions of dollars earmarked for fighting a form of rare and deadly children's cancer is sitting unused, with advocates and families arguing its reallocation could benefit dozens of children facing cancer relapses.

Neuroblastoma – a type of solid tissue tumour formed from residual embryonic nerve cells throughout the body – is the most lethal form of cancer for children under five, and treatment typically runs at a price in the hundreds of thousands of dollars.

Health Minister Mark Butler allocated $20m over two years to bankroll the drug treatment difluoromethylornithine (DFMO) in July last year, despite a decision from the Therapeutic Goods Administration on its viability still pending.

However, in October the drug's supplier, Norgine, also opted to begin an early access program that subsidised care. The $20m in federal funding was still outlined in the

Mid-Year Economic and Fiscal Outlook despite this.

Neuroblastoma Australia argued the remaining funding should be invested in CAR-T cell therapy research in Australia, an emerging treatment for neuroblastoma.

Neuroblastoma Australia chief executive Lucy Jones.

Currently, CAR-T cell treatments are limited to experimental trials overseas averaging a price of $700,000. The advocacy group estimates the net price to establish such a trial in Australia would sit at $10m.

Mr Butler flagged at the time that his funding would be a one-off investment in lieu of potential funding by the Pharmaceutical Benefits Scheme. A media release said it would fund the drug “until Norgine establishes its compassionate access scheme”.

DFMO was formally approved for domestic use by the Therapeutic Goods Administration in early April.

“It’s about Australia being left behind. On one hand, it’s actually done extremely well getting DFMO available so quickly for free. But it would be great to build on that success and to actually have this option of treatment (CAR-T cell therapy) available here, because there are very few options here for relapse,” Neuroblastoma Australia chief executive Lucy Jones said.

“The money was set aside for children with neuroblastoma … So we really would love to see the continued prioritisation of these children with that funding, which should still be available.

“There are very young children having to endure the most terrible treatments, and then half of them aren’t actually surviving despite everything being thrown at them. It became a personal mission to try and change the situation.

Jack Schofield, 5, with his mum Jaimee Thompson, dad Trent and brother Ted, 7.

“Their families have to use a social network. They have to sacrifice their own privacy and their child’s privacy to get the funding, and there are the families who just don’t have the means to do that. It’s just very inequitable.”

High-risk neuroblastoma carries a 50 per cent survival rate accounting for the standard treatment of chemotherapy, stem cell transplants, radiation therapy and immunotherapy. DFMO was found to halve relapse rates, but its utility for patients who have already relapsed is in question.

Half of all patients are expected to relapse within their first year of care. One such patient is Jack Schofield, 5, currently undergoing chemotherapy and immunotherapy after seeing little success with an $800,000 US-based cancer vaccine trial.

“Jack is, by nature, an incredibly kind and thoughtful little boy. He’s the one to offer you the last lick of his ice cream and his last lolly,” mum Jaimee Thompson said.

“Eighty-six per cent of his bone marrow was cancerous, and he had lesions in his bones from the top of his head through to his toes. It’s a pretty gruelling treatment campaign for him to get better.

> *“The monetary amount itself is incredibly daunting; it’s so much work to raise that amount of money. You barely sleep through those periods of time because you’re just kind of trying to keep your head around everything, plus you’re trying to be present for your kids and give them what childhood they can get in this setting.”*

“Jack’s missed out on making friends … all his mates are currently moving on and going to school and learning, when he’s just got to come into hospital and get hooked up and endure more treatments. We’ve lost a lot, I lost my job as a result. It’s been incredibly challenging for everyone in our family.

“The monetary amount itself is incredibly daunting; it’s so much work to raise that amount of money. You barely sleep through those periods of time because you’re just kind of trying to keep your head around everything, plus you’re trying to be present for your kids and give them what childhood they can get in this setting.

“I don’t even know how to articulate the level of … anxiety, stress, fear, all of it bundled into one that you’ve got to somehow live with on a day-to-day basis.”

The Australian and New Zealand Children’s Haematology/Oncology Group and Neuroblastoma Australia submitted a proposal to government in January for how $10m of the residual DFMO funding could be reinvested into CAR-T cell therapy trials across Australia.

Sydney Children’s Hospital children’s cancer specialist Tony Trahair said families were being handed difficult and expensive ultimatums on treatment options when Australia’s domestic cell therapy research infrastructure could realistically be scaled up to tackle neuroblastoma trials.

“I think the research community is fairly cohesive, and has been very focused on trying to develop and bring new therapies to clinical practice,” Dr Trahair said.

“Australia has always had a fair bit of strength and depth in children’s cancer research.

Children’s oncologist Toby Trahair.

“It would be nice to (see) that some of these trials are more easily available, or more easily accessible, within Australia, rather than families having to go overseas.”

Despite questions, Mr Butler did not say how much of the fund remained, or how it would be reallocated.

“Thanks to the Albanese Labor government, all patients have successfully transferred to the Norgine Expanded Access Program and have been provided immediate free access to treatment,” he said.

“We look forward to continuing to work with Cancer Australia, Neuroblastoma Australia and the Australian & New Zealand Children’s Haematology/ Oncology Group on their mission to provide world class care and treatment to children and young adults with cancer.”

Opposition health spokeswoman Senator Anne Ruston said she was “proud to have fought for this funding for Australian children with neuroblastoma to secure access to lifesaving treatment and ... will continue to work closely with patients and their families on the support they need”.

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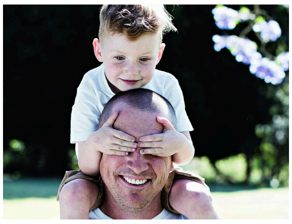

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James Dowling is a reporter in The Australian’s Sydney bureau. As an intern at The Age he was nominated for a Quill award for News Reporting in Writing for his coverage of the REDcycle recycling scheme. When covering health he writes on medical innovations and industry.

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