childhood dementia INITIATIVE

Childhood Dementia Researcher Survey 2024

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Acknowledgments

In the spirit of reconciliation, the Childhood Dementia Initiative acknowledges the Traditional Custodians of Country throughout Australia and their connections to land, sea and community. We pay our respects to their elders past and present and extend that respect to all Aboriginal and Torres Strait Islander peoples today.

Thank you to Associate Professor Anthony Cook (University of Tasmania and Wicking Dementia Research and Education Centre) and Associate Professor Anthony White (QIMR Berghofer Medical Research Institute) who are members of the Childhood Dementia Initiative's Scientific and Medical Advisory Committee and contributed to the design of the survey. Thank you also to the researchers who took the time to complete the survey.

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Background

Childhood dementia is caused by more than 100 genetic disorders which are estimated to affect 1 in every 2,900 births.[1] Due to extremely limited treatment options, half of the children living with a childhood dementia disorder will die before they're 10 years old, most won't reach adulthood, and all will die prematurely. Increased research activity is needed to understand childhood dementia, develop treatments and improve care and quality of life.

This survey was conducted to assess the needs of childhood dementia researchers Australia which will inform Childhood Dementia Initiative's research strategy and advocacy activities. The survey was sent to Australian based members of the Childhood Dementia Research Alliance (439 people) in August 2024. 28 researchers completed the survey and 2 partially completed it.

Respondent demographics

Survey respondents held a wide range of positions:

- 33% lab-based research group leaders
- 23% clinician researchers
- 20% postdoctoral researchers
- 13% postgraduate students
- 13% other, including a research assistant, lab technician, project manager and a patient advocate.

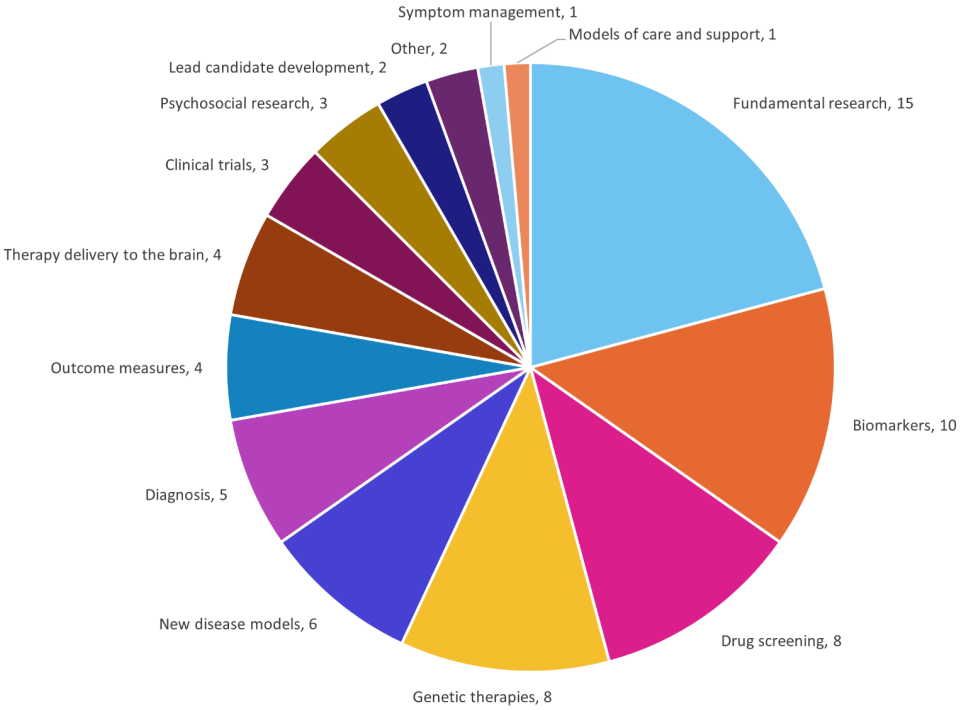
Researchers from all states of Australia were represented, none were from the Northern Territory or Australian Capital Territory (30% NSW, 30% VIC, 13% TAS, 13% SA, 7% QLD, 7% WA).

22 respondents were currently engaged in childhood dementia research, and 8 were not but would like to be. Of those already engaged in childhood dementia research, 11 had been working in this field for 1-5 years, 4 for 5-10 years and 7 for more than 10 years.

Current research activity

The most popular areas that the 22 researchers were currently engaged in were: fundamental research to understand childhood dementia disorders, biomarkers, genetic therapy development and drug screening/repurposing (Figure 1).

Figure 1: Research areas that researchers are currently engaged in for childhood dementia



Childhood dementia research enablers

Top factors that researchers said would enhance their ability to do more research included more funding, more opportunities to form collaborations and more qualified staff to carry out research (which is also tied to funding) (Table 1). **All researchers said that more funding would enhance their ability to do more childhood dementia research to at least a medium degree**.

> **"More funding [is needed] - ideally long-term funding to ensure career development in the field for existing and newly-entering researchers."**

> **"Funding is the biggest limitation of Childhood Dementia research progress."**

Access to patient samples and data also rated highly. Clinician researchers highlighted the need for clinical trial infrastructure.

> **"We are often contacted by industry regarding clinical trials but often do not have the staff, resources or infrastructure within the hospital to take on extra trials."**

Table 1: Survey question: To what degree would the following enhance your ability to do more childhood dementia research?

Enabler (listed in order of weighted average*)	**% who responded 'very high' or 'high' degree**
More funding	90%#
More opportunities to form collaborations with other childhood dementia researchers	77%
More qualified staff to carry out research (63%)	63%
Access to industry partners	57%
Knowledge about disease mechanisms that could be targeted	57%
Access to patient samples e.g. tissue, blood, CSF	53%
Access to patient data e.g. natural history data	57%
Clinical trial infrastructure	50% (100% for clinician researchers)
Understanding of the childhood dementia research priorities	53%
Earlier diagnosis of patients	40%

*Very high = 5, High = 4, Medium = 3, Low = 2, Very low = 1

100% if medium, high and very high degree included

Capacity for more research

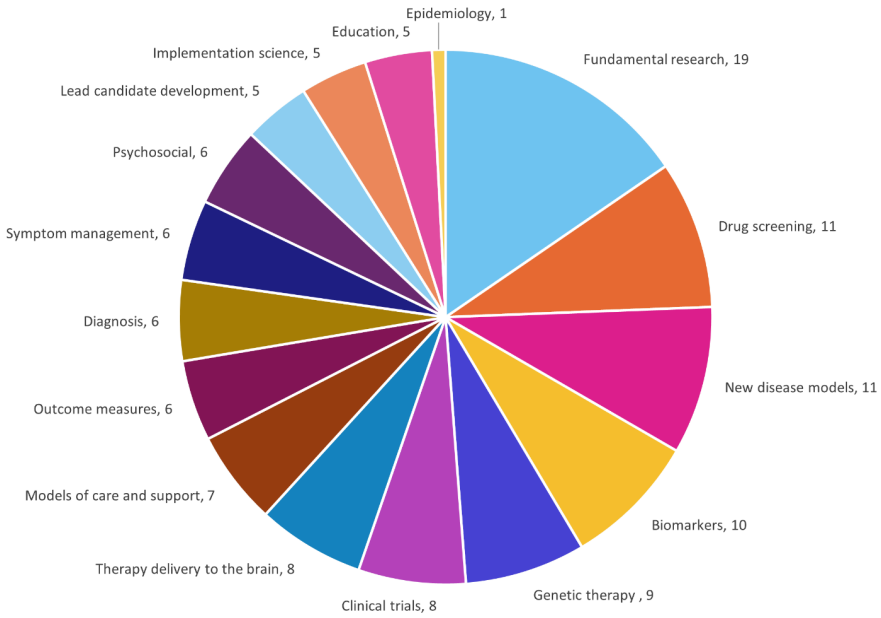
More than 50 childhood dementia-related research proposals had been submitted for funding by the researchers in the past 5 years that were not funded. The researchers cited the main reasons for unsuccessful applications as being difficulty competing against more well known and common diseases, and the lack of childhood dementia targeted funding calls/funding bodies.

"The childhood dementia-specific MRFF call was a good start but needed much more funding behind it...each grant was approximately half the dollar amount of the average for NHMRC Ideas grants. Thus a round that would enable funding parity with typical NHMRC Ideas grants would be welcome."

Given the right funding opportunity, researchers said that they had project ideas that they could propose across the research pipeline from diagnosis and fundamental research to drug discovery, clinical trials and models of care and support (Figure 2). In total **124 childhood dementia research projects could be proposed** by the 29 researchers who completed this question. The most popular areas for future projects were:

- fundamental research (19 projects)
- drug screening/repurposing (11 projects)
- development of new disease models (11 projects)
- biomarkers (10 projects)
- genetic therapy development (9 projects)
- clinical trials (8 projects).

Figure 2: Number of childhood dementia research projects that could be proposed given the right funding opportunity



Encouragingly, all researchers could see **broad application of their research**, not just for one disorder. The largest proportion (45%) said that their research would be piloted in one or a few childhood dementia disorders, and if successful, applicable to a wide range of disorders. Other researchers would include multiple or all childhood dementia disorders from the outset (28% and 10% respectively). Five researchers (17%) said that their research would encompass childhood and adult-onset dementia, demonstrating the potential for broad benefit.

Collaboration and knowledge sharing

Researchers were asked their preferences for sharing knowledge, connecting with other researchers and learning about childhood dementia. There was high interest in in-person symposia, a Community of Practice, webinars, written material on the CDI website, and a regular e-newsletter (more than 80% of respondents were very likely or likely to engage with all of these). One researcher also commented that further promotion

of the field and the use of childhood dementia terminology through journal reviews/ perspectives/ commentaries is needed. Researchers felt strongly that **forming international collaborations was crucial**, with 14 commenting on this topic.

> **"Researchers overseas have access to far larger patient cohorts and patient samples for clinical trial development and establishment. Collaborating with our peers overseas is critical. There are also sources of funding overseas that are accessible via partnership."**

The survey asked researchers about their use of the Childhood Dementia Knowledgebase, a database containing key information about all of the childhood dementia disorders. The majority (75%) find it very or extremely useful and it is most commonly used to understand what conditions are included under the childhood dementia umbrella, and to access data on incidence, prevalence, life expectancy and age of onset. They used the Knowledgebase to find information to reference in research papers, presentations and teaching materials, gather information to formulate new research projects, and to write grant proposals. Survey respondents also used it to identify childhood dementia disorders that they could broaden their work to. Three clinicians used the Knowledgebase to inform their clinical practice.

Survey respondents said that the most useful future additions to the Knowledgebase would be: research tools that could be shared (e.g. cell lines), disease mechanisms and information for families. It was also requested that the location of specialists/specialist centres and key researchers be listed.

Conclusions

The survey reveals substantial untapped potential within Australia's childhood dementia research community. With over 124 potential research projects identified across the research pipeline, there is clear capacity and

willingness to expand research efforts in this field. However, two critical factors are currently limiting progress: funding constraints and the need for enhanced collaboration infrastructure.

The funding challenge is particularly acute, with more than 50 childhood dementia research proposals going unfunded, largely due to competition with more widely recognised diseases and a scarcity of dedicated funding streams. The lack of funding also means that it is difficult to attract and retain experienced and qualified staff to carry out research.

The survey demonstrated researchers' enthusiasm to break down silos and design studies that could benefit multiple childhood dementia disorders or even extend to adult-onset dementia research.

International collaboration emerged as a crucial priority, particularly for accessing larger patient cohorts and additional funding sources. Strong engagement with the Childhood Dementia Knowledgebase, indicates an active and committed research community eager to expand their work and impact.

To unlock this potential, strategic investment in both research funding and collaborative infrastructure will be essential. The survey findings suggest that targeted funding calls, enhanced clinical trial infrastructure, and strengthened international research networks could significantly accelerate progress in childhood dementia research and ultimately improve outcomes for affected children and their families.

References

1. Elvidge KL, Christodoulou J, Farrar MA, et al. The collective burden of childhood dementia: a scoping review. *Brain J Neurol*. 2023;146(11):4446-4455. doi:10.1093/brain/awad242