Childhood Dementia Care: Australia's Path Forward

International models inspiring national action

Report by Gail Hilton, Churchill Fellow

2024 Churchill Fellowship to investigate comprehensive models of care for children with dementia and their families

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Report by Gail Hilton

To investigate comprehensive models of care for children with dementia and their families

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Date 1 November 2025

ACKNOWLEDGEMENTS

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This Fellowship was inspired by all the families and children affected by childhood dementia whom I have had the privilege to meet here in Australia. Over the past five years, Australian families have shared insights and stories that made this topic impossible to look away from. In the words of a mum "childhood dementia is every parent's worst nightmare". The courage, tenacity and fierce advocacy of these parents while caring for their children is both devastating and inspiring. This is a corner of children's health that most people know nothing about. These families have to fight for everything their children need in our systems here in Australia, yet in parallel they have the unwavering determination to see change for future generations. If you can all step forward as changemakers then so can others; thank you for propelling me on.

I am deeply grateful to many people who made this Churchill Fellowship journey possible. First and foremost, my heartfelt thanks go to Megan Maack, my CEO at Childhood Dementia Initiative, for her unwavering support in enabling me to undertake this project. Her belief in this Fellowship, along with the support from the entire team at Childhood Dementia Initiative, provided me with both the opportunity and resources to make this experience a reality. I extend special recognition to the extraordinary professionals who became collaborators and guides: Lauren Treat at Children's Hospital Colorado, Claire Wakefield at Stanford University, and Karsten Barton at the Frambu Centre in Norway, who went above and beyond in crafting my itinerary and sharing their expertise. To the many other healthcare professionals, researchers, and patient organisation leaders across Norway, the UK, Canada, and the USA who welcomed me into their clinics, shared their innovations, and trusted me with their most complex challenges - your openness and dedication exemplify what excellence in childhood dementia care can achieve.

Gail at Global Treehouse, Somerset House, London

ACKNOWLEDGEMENTS

To the international families who shared their experiences with me: Marlene, Øyvind and Syver in Oslo; Terri and other mums who shared for many, many hours with me in Toronto; Julia in Colorado (while eating the most delicious pastries ever) and the many families who shared their experiences and enabled me the absolute privilege of spending time with their children at the MPS Conference in Coventry - your willingness to share your stories with a stranger from Australia reflects extraordinary generosity and hope that this work might help other families. I carry your voices and experiences with me as I write this report and continue to advocate for progress. Thank you.

To my husband Steve and children Josh and Cara, who supported me at every step of this Churchill Fellowship journey - thank you for managing our home with such grace and enabling me to spend six weeks overseas pursuing this important work. Your love and encouragement made this possible, and your understanding of why this matters sustained me through the most challenging moments of this journey. I'm also grateful to my closest friends and colleagues who have been my ongoing cheerleaders throughout this process - you know who you are. Special thanks to my colleagues Maria Twigg and Ellie Da Ros, who provided invaluable feedback and proofreading support for this report, helping to ensure that the voices and experiences I encountered are represented with the care and accuracy they deserve.

Finally, my profound gratitude extends to the Winston Churchill Memorial Trust for this extraordinary opportunity to learn, grow, and contribute to advancing support for children and families affected by childhood dementia. This Fellowship has not only informed my professional understanding but has fundamentally changed how I see the possibilities for compassionate, coordinated care that honours both the complexity of these conditions and the humanity of the families who navigate them.

"The future of these children cannot solely rely on the capacity and skill of their parents"

- Øyvind Blindheim, Dad to Syver.

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EXECUTIVE SUMMARY

This Churchill Fellowship investigated comprehensive models of care for children with dementia and their families across Norway, the United Kingdom, Canada, and the United States. Australia is the first country to recognise childhood dementia as a unified group of over 100 conditions, rather than treating each disease separately. Childhood dementia affects approximately one in 2,900 babies born in Australia, with an estimated 1,400 Australians currently living with these conditions. Around 90 children die annually - comparable to childhood cancer deaths in the 0-14 age range. These children are born healthy, develop normally, then progressively lose all skills—usually in childhood. Half die before age 10, and 70% before reaching adulthood. Currently, families navigate fragmented services across health, disability, education, and social care with no systematic coordination, becoming default coordinators while caring for their children. The burden on families and inefficiency across systems is profound.

Although childhood dementia conditions currently have no cures, emerging therapies are on the horizon. Timing is critical to build the infrastructure needed to deliver these treatments when available. The recent integration of health and NDIS portfolios in Australia creates an unprecedented opportunity for cross-sector coordination. Australia has the opportunity to design world-first proactive, equitable systems serving all conditions that cause childhood dementia. Initially inspired by families sharing their story in Australia, this Fellowship was informed by extensive time with families internationally, including those currently caring for children with childhood dementia and bereaved parents who have transformed their grief into systemic advocacy.

This report is intended for: Federal and state policy makers in health, disability and education portfolios; childhood dementia families and advocacy organisations; and professionals and service providers across health, disability, education and community.

International observations

- **There are no comprehensive models of care for childhood dementia, but there are examples of best practice:** The most sophisticated care existed where professionals saw sufficient numbers of children to develop some genuine expertise. Norway's Frambu Centre, Colorado's neurodegenerative clinic, and UK's disease-specific services demonstrated that concentrated exposure enables professionals to anticipate needs, develop innovative interventions, expedite research and guide local teams.

- **Families drive innovation:** The most innovative services internationally owe their existence to parents who transformed personal experience into systematic advocacy. This pattern demonstrates both extraordinary human resilience and profound systemic failure. Sustainable care cannot depend on exceptional individual efforts by families already managing devastating circumstances.

- **Cross-system coordination is required:** Childhood dementia care extends beyond health services, requiring integration across health, disability, education, and social support. Even in countries with sophisticated healthcare systems, families become default coordinators navigating disconnected services. The UK's 'Child in Need' legislation and Canada's complex care model demonstrate structured approaches, though neither fully addresses cross-sector integration.

- **Peer support is essential:** Families across all countries—both those currently caring and those bereaved—identified connecting with others facing similar journeys as the most valuable support, often more valuable than medical interventions. Parents described peer support as 'the only place I felt understood' and 'what saved me.' Yet healthcare systems consistently undervalue peer connection as supplementary rather than essential to comprehensive care.

Recommendations for Australia

1. Establish a national approach to childhood dementia, supported by government policy and sustainable funding—an opportunity no other country has seized. This approach could enable coordinated expertise, data, research and engagement across health, disability and education systems, moving beyond the current fragmented landscape where care depends on location and individual family advocacy. Central to this must be a unified national lived experience voice, ensuring families inform policy and service design at every level.

2. Develop a national cross-sectoral model of care integrating health, disability, education, and community services to end the fragmentation where families become unwilling care coordinators and case managers. The model must be interdisciplinary, address the full trajectory from diagnosis through to bereavement, and be co-designed with families. Australia's recent integration of health and disability portfolios provides a unique opportunity to design coordinated approaches that can also formally include community services—the peer connection and navigation support families identify as most valuable, yet healthcare systems typically undervalue.

3. Recognise and resource Childhood Dementia Initiative (CDI) as the national peak body and community service provider, ensuring all families access peer support, navigation assistance, and whole-of-family support regardless of diagnosis or location. Australia should pilot an innovative model positioning CDI as convenor of integrated service coordination, bringing together health, disability, and education stakeholders around individual families to reduce the exhausting burden on parents who become default 'project managers' of disconnected systems.

4. Build specialist expertise across systems through workforce development, specialist infrastructure, and policy reform across health, disability, education, and community services. For progress in practice, professionals need sufficient exposure to childhood dementia - occasional encounters with isolated cases cannot develop the deep practice wisdom these complex, progressive conditions demand.

Right now in Australia families are navigating fragmented systems alone while caring for children losing skills and abilities—bearing burdens no family should carry. With childhood dementia now recognised as a unified group, health and disability portfolios integrated, and international evidence demonstrating what works, the foundations for transformative change are in place. Australia can be the first country to get this right, but only with committed investment and coordinated action.

KEYWORDS
Childhood dementia, paediatric care models, integrated care, family support, rare diseases, healthcare innovation, neuropalliative care, complex care, neurodegeneration, multi-disciplinary care

2025 ITINERARY

OSLO, NORWAY	
16th June	Dr Anja Lee & Paediatric Neurometabolic Team, Rikshospitalet
	Professor Ingrid B. Helland - Norwegian National Advisory Unit on Rare Disorders
	Øyvind (Dad) & Syver Blindheim, who lives with Batten Disease (lived experience) Professor Magnar Bjørås - Research Scientist, ShareLab
17th June	Frambu National Conference
	Marlene - Mum of Novalie who lives with Batten Disease (lived experience)
18th June	Multiple meetings facilitated by Dr Susann Simensen, Paediatrician and Tonje Elgaas, Psychologist, Frambu Centre
19th June	Dr Anja Lee & Regional Paediatric Palliative Care team meeting, Oslo University Hospital; Norwegian National Advisory Unit on Paediatric Palliative Care
	Luisa Klaveness, Policy Advisor, National Dementia Organisation
20th June	Multiple meetings and tour facilitated by Karsten Barton, Senior Advisor - including the Leve NÅ Team (Palliative Care), Frambu Centre

UNITED KINGDOM - LONDON	
23rd June	Laura Dale Harris - Executive Director, Global Treehouse
	Hannah Gardner - Consultant Admiral Nurse, Dementia UK
	Presentation to Professor Paul Gisson & Metabolic Team, Great Ormond Street Hospital
24th June	Paola Barbarino - CEO & Wendy Wiedner, Head of Research, Alzheimer Disease International
	Katie Burbridge - Director of Care & Executive Nurse, ACORNS Children's Hospice (via Zoom)
	Sarah Kenrick - Heads of Support & Advocacy, Batten Disease Family Association (via Zoom)
25th June	Jayne Hughes CEO and bereaved mum of Amy who had Cockayne Syndrome and Meg Simpson, Family Coordinator - Amy & Friends (lived experience)
26th June	Jayne Hughes, CEO; Paula Sullivan, Nurse Practitioner, Cockayne Syndrome, Trichothiodystrophy & Bloom Syndrome Clinic and Meg Simpson, Family Coordinator
	Guy's & St Thomas's Hospital
	Professor Lorna Fraser - Professor of Palliative Care and Child Health, Kings College, London

UNITED KINGDOM - LEICESTER & COVENTRY	
27th June	Meeting with Diana Nurse Team, Leicestershire Partnership NHS Trust
	Helena Dunbar - CEO, Kentown Support
	Toni Mathieson - CEO, LSD Collaborative & NPC UK Foundation (lived experience)
28th June	Steve Cotterell, Head of Support and Advocacy, MPS Society
27 - 29th June	MPS Matters Conference (multiple health professionals and families with lived experience)
Pre-travel	Sara Hunt, CEO and Karen Harrison, Family Support Manager (both lived experience) - Alex TLC

CANADA	
11th August	Mary Bone - CEO Canadian MPS Society, with parents impacted by Sanfilippo Syndrome (lived experience)
12th August	Louise Tunnah - Managing Director, Solutions for Kids in Pain (SKIP) - (via Zoom)
	Kira Goodman - Project Director, Canada's Paediatric Palliative Care Alliance
13th Aug	Cheryl Marcogliese - Mum of two boys with Niemann Pick C Disease (lived experience)
	Presentation to Dr Adam Rapoport & PACT Team, SickKids: Hospital for Sick Children
	Krista Keilty, Associate Chief Interprofessional Practice - Connected Care & System Integration, SickKids: Hospital for Sick Children
14th Aug	Jennifer Stinson & Fiona Campbell - Co-Directors, Pain Centre & SickKids: Hospital for Sick Children
	Kimberley Hunter - Senior Director, Clinical Programs & Team at Safehaven
15th Aug	Dr Michal Inbar-Feigenberg - Medical Director, Lysosomal Storage Disorders Program, SickKids: Hospital for Sick Children
	Terri Gortnar - bereaved mum of Claire who had Batten Disease (lived experience)

USA	
18th Aug	Julie Vitello - bereaved mum of Mila who had Batten Disease, CEO Mila's Miracle Foundation (lived experience)
19th Aug	Observation: SynGAP clinic hosted by Rose Drake, Clinic RN Coordinator.Children's Hospital Colorado
20th Aug	Observation: Rett Clinic hosted by Tristen Dinkel, NSI Rett Clinic Nurse Coordinator and Professor Tim Benke, Paediatric Neurologist, Children's Hospital Colorado
21st Aug	Observation: Special Care Clinic with Associate Professor Kourtney Santucci, Co-Medical Director of the Neurodegenerative MDC, Associate Medical Director of the Special Care Clinic, Children's Hospital Colorado
	Aj Lotz - Bereavement Coordinator, Children's Hospital Colorado
	Leighann Sremba - Genetic Counselor, Children's Hospital Colorado
22nd Aug	Dr Scott Demarest, MD - Chief Precision Medicine Officer, Neurologist and Clinical Director for the Children's Hospital Colorado Precision Medicine Institute, Co-Medical Director of the Neurodegenerative MDC
	Associate Professor Jennifer Disabato - Co-Director, Improving Paediatric to Adult Care Transition (ImPACT) Program (via Zoom)
	Maria Hopfgarten - bereaved mum of Jacob who had mitochondrial disease & Parent Champion, Courageous Parents Network (lived experience) (via Zoom)
23rd Aug	Associate Professor Lauren Treat - Neurology & Pediatric Palliative Medicine Physician, Children's Hospital Colorado
25th Aug	Professor Justin Baker - Chief of the Division of Quality of Life and Pediatric Palliative Care, Stanford Medicine Children's Health (via Zoom)
	Presentation to Professor Caroline Hastings & Professor Paul Harmatz - UCSF Benioff Children's Hospital
26th Aug	Tyler Tate - Stanford Webinar, "To Suffer What We Can't Evade" (via Zoom)

INTRODUCTION

My background is in senior leadership roles within the not-for-profit sector, where I have spent nearly 20 years managing projects, delivering services, and driving evidence-based improvements in care for communities facing significant health, education and social care needs. Most of my working life has been in service delivery to families affected by life-threatening and life-limiting childhood conditions, including childhood and teenage cancer. This has given me the opportunity to listen firsthand to families navigating these journeys, to hear from the wide array of professionals who support them and to gather insights into Australia's paediatric health system.

In 2018, returning to work from maternity leave, I joined Sanfilippo Children's Foundation—a small patient organisation focused on a single rare disease. There, CEO Megan Maack introduced me to a pattern she had observed across the rare disease landscape: numerous small patient organisations—often run by volunteer parents—each advocating for their individual rare diseases, all affecting only a small number of families in Australia. Something simply did not make sense; this siloed approach would always have limitations in driving widespread progress. There must be a better way.

Despite diagnoses of different diseases, there are a group of children who share something profound: progressive brain damage or neurodegeneration. Put simply, these are kids that are born seemingly healthy, they successfully gain skills and then begin losing them. They lose all their abilities until, sadly, they die, usually while still in childhood. Very few of these conditions have effective treatments and none have cures. The families faced common struggles navigating systems never designed for their needs while caring for their children as they deteriorated.

'Childhood dementia' was a term with roots in academic literature dating back to the 1940s, and landmark research by Professor Kenneth Nunn at Sydney Children's Hospital provided a clear Australian definition.[1] Meg's vision was to use this terminology to create a bigger grouping, to enable economies of scale to drive progress. To test this collective approach, we commissioned a burden of disease study in 2019. The results demanded attention: one in 2,900 babies born in Australia would develop a condition causing childhood dementia—comparable to cystic fibrosis in incidence and motor neurone disease in prevalence, both far better known and resourced conditions.[2] These findings led us to co-found the Childhood Dementia Initiative (CDI) in November 2020, with Megan Maack as CEO—the world's first organisation addressing childhood dementia as a collective.

As Director of Programs at the CDI, I lead our work across advocacy, care, and research, with lived experience at the centre of everything we do. Over the past seven years, I have listened extensively to families affected by childhood dementia across every Australian state and territory, at all stages of disease progression, both those currently caring and those bereaved. Given Australia's federated health system, this national perspective provided crucial insights into both the universal challenges these families face and the stark variations in care quality across different jurisdictions.

The lived experience stories reveal profound inequity and severe unmet need. Families describe diagnostic odysseys lasting years. They recount being told "there is nothing that can be done, go home and love your child." They detail having to fight for everything—every piece of equipment, every support service, every moment of respite—while simultaneously watching their child slowly die. With no effective treatments or cures currently available for the vast majority of childhood dementia conditions, healthcare professionals report feeling isolated and ill-equipped, lacking the specialist knowledge and support systems to provide the complex care these children need. The comprehensive evidence detailing the profound inequity facing children with dementia and their families in Australia is outlined in the next chapter.

This combination of experiences positioned me uniquely for this Churchill Fellowship. Having spent 15 years embedded in well-resourced systems for children with life-threatening conditions—particularly cancer, where I witnessed coordinated national action drive survival rates to exceed 84%—I understood what was possible when investment, infrastructure, and integration aligned. Simultaneously, seven years listening to childhood dementia families nationwide revealed what happened when systems failed: fragmentation, isolation, and families forced to become unwilling case managers while grieving their dying children.

This Churchill Fellowship sought to understand how other countries coordinate comprehensive care around families affected by childhood dementia, and how Australia could adapt these models to benefit children, families, and the systems that support them. This took me to Norway, the United Kingdom, the United States, and Canada. Australian families themselves had been sharing examples of best practice they encountered overseas—many travel internationally to access specialists and clinical trials unavailable at home — and these insights guided my investigation.

With evidence, expert insights and lived experience painting a horrifying picture of the profound issues facing children with dementia and their families in Australia, there is a moral imperative to act. Global progress in therapeutic development means emerging treatments are increasingly within reach—but only if Australia is ready. The Australian Department of Health, Disability and Ageing is a committed partner in developing solutions, and CDI's groundwork has established the evidence base and stakeholder networks necessary for transformation. To capitalise on this unprecedented alignment, we must move quickly, learning from international best practice and adapting proven models to our context.

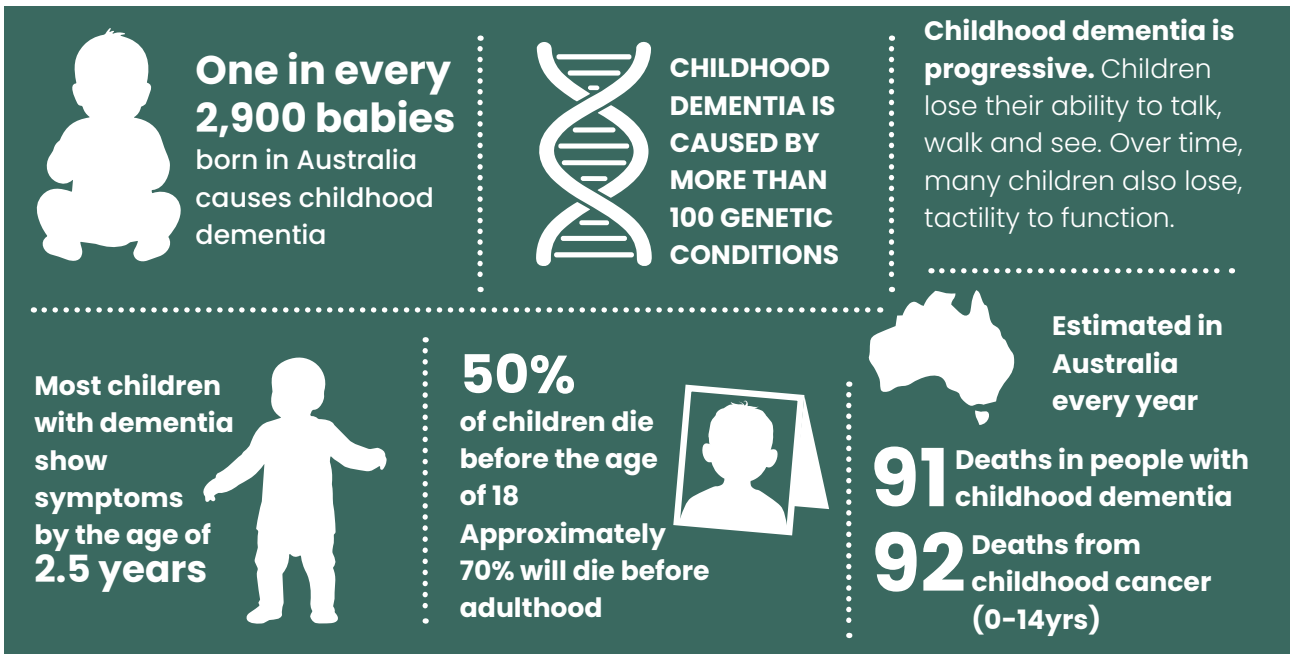
The time is now.

01. AUSTRALIA

What is Childhood Dementia?

A baby is born every 3 days in Australia with a childhood dementia disorder from which they will die. Half of all children with dementia die before the age of 10, and 70% of them will die before they reach adulthood.[2] Throughout their short lives, these children suffer and die slowly, without access to therapies or adequate care. There has been no notable improvement in survival rates for children with dementia. By comparison, death rates from childhood cancer in Australia almost halved in the 20 years between 1997 and 2017, and more than 84% of children with cancer now survive.3 Historically, the 100+ conditions that cause dementia in childhood were considered in isolation, despite the commonality of disease mechanisms, symptoms and needs. This resulted in widespread fragmentation and exclusion across care, policy and research on a global scale.4–9 Children with dementia carry arguably the highest level of unmet need in the Australian paediatric health system.

The facts



The State of Childhood Dementia in Australia

Australia is the first country in the world to consider the collective group of childhood dementia disorders in a system-wide approach.[10] CDI, founded in 2020, is spearheading this work by focusing on the presentation of childhood dementia – the symptoms and how children are impacted – rather than the biological basis of each underlying disease. This collective approach enables solutions with economies of scope and scale. Childhood dementia is complex and crosses many areas of health and social care. It does not fit neatly into any one established area, and as such, needs remain largely unmet.

Evidence has shown that:

Expertise and knowledge are limited among professionals within Australia's health system. There is no centralised source of information or resources to inform care delivery.[11,12]

Healthcare is inadequate and inequitable. There are no national standards, no specialist models of care, and no standard pathways.[11,12]

There are no treatments and cures for the vast majority of childhood dementia disorders. While advances in genomics and drug development have put effective treatments within reach globally, Australia faces a critical clinical trials gap.[13]

Recent research has documented the care and support landscape for Australian families affected by childhood dementia. Through extensive consultation with healthcare professionals and, critically, with families themselves, this research has mapped the challenges families face across the entire journey - from diagnosis through bereavement.[11,12,14] Families have articulated 'What Matters Most' to them across ten key care and support domains: healthcare professional awareness and training, diagnostic pathways, care coordination, health services, NDIS and disability services, psychosocial support, education support, palliative care, hospice and respite care, and research.[12] This work, documented in the Childhood Dementia Care and Support Landscape: Pathway Toward Report and the companion lived experience report, formed the foundation for this Churchill Fellowship and directly informed the key areas investigated internationally.[11,12]

Australia's Evolving Health Policy Landscape

Australia is at a pivotal moment for childhood dementia. Recent policy developments create unprecedented opportunities to address longstanding gaps.

Building Momentum in Policy Recognition

- The National Dementia Action Plan has recently included children as a priority population - a significant milestone that creates an opportunity to integrate childhood dementia into established dementia policy structures for the first time.[15]

- The National Strategic Action Plan for Rare Diseases (2020) provides a comprehensive framework built on three pillars: Awareness and Education, Care and Support, and Research and Data. This framework creates potential pathways for coordinated action on childhood dementia.[16]

- Rare Voices Australia's 2024 National Recommendations for Rare Disease Health Care identified eight key recommendations applicable to childhood dementia, emphasising collaboration between clinicians and researchers.[17]

The Implementation Gap

Despite these promising developments, childhood dementia remains largely invisible in policy implementation:

- No state or territory has yet developed a dedicated rare disease strategy aligned to the National Strategic Action Plan for Rare Diseases (McKell Institute 2025).

- Childhood dementia receives no specific recognition in the Paediatric Palliative Care National Action Plan.

- State dementia strategies do not yet acknowledge childhood dementia.

Australia's Opportunity

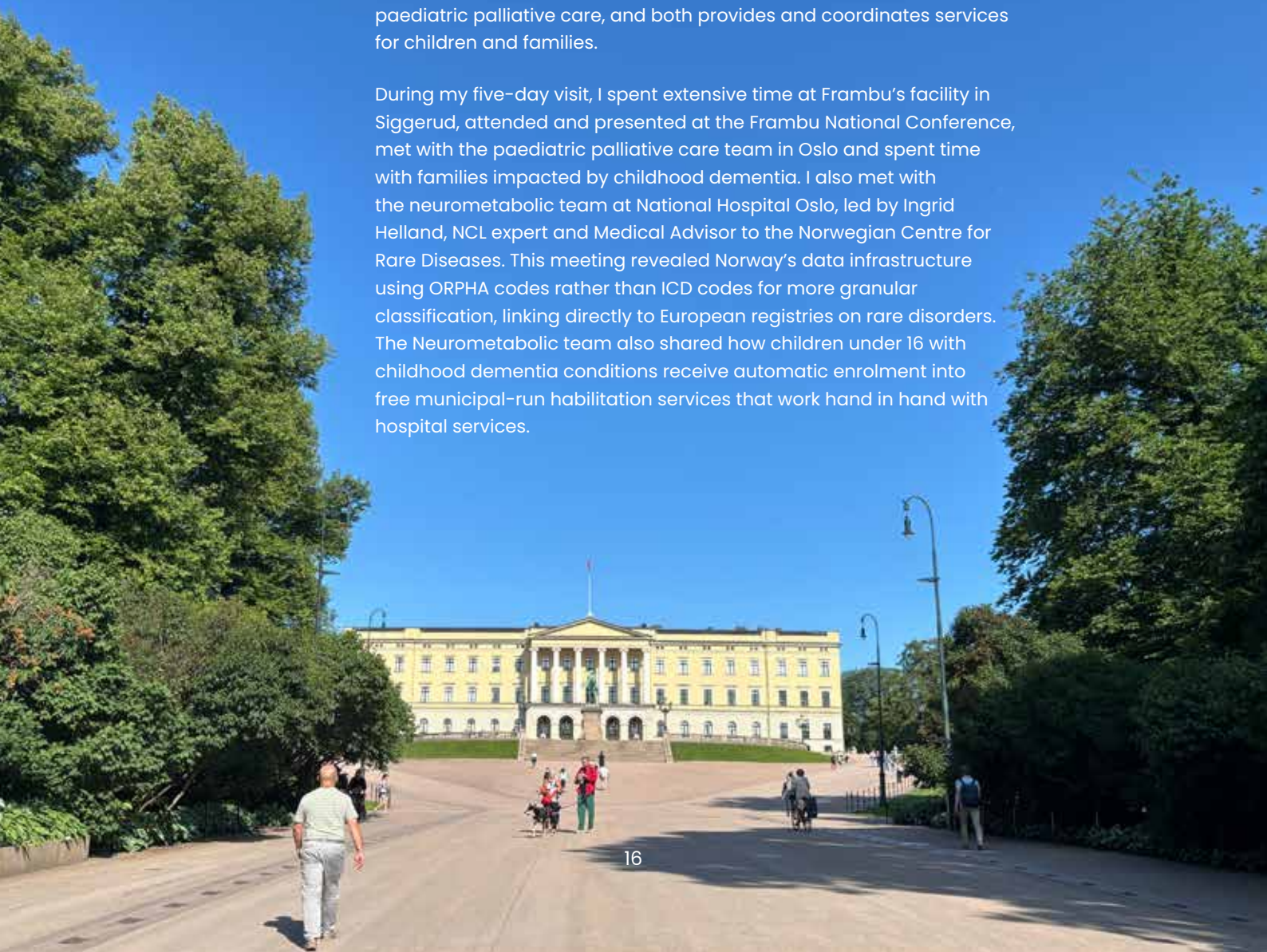
Australia's world-first collective approach to childhood dementia, combined with recent policy recognition and a clear understanding of what families need, creates a unique opportunity. The foundations are in place. The challenge now is translating policy momentum into practical change that delivers better outcomes for Australian children with dementia and their families.

For comprehensive insights on the current state of childhood dementia in Australia, including detailed family experiences and system gaps, see the State of Childhood Dementia 2024 report and additional resources at www.childhooddementia.org.

02. NORWAY

Norway's 5.4 million population faces geographic challenges similar to Australia, with 28 hours driving distance north to south and most citizens concentrated around Oslo. The healthcare system operates through university hospitals in each region with strong inter-hospital communication, following a philosophy of local care delivery when possible. Unlike other healthcare systems that treat individual rare diseases in isolation or group 'rare disease' in its entirety, Norway has created nine centres under the Norwegian Centre for Rare Diseases, each focusing on specific ICD-10 code subsets. The Frambu Centre, established in 1955, operates as the largest centre addressing over 450 rare diagnoses, among which are neurodegenerative, metabolic, mitochondrial and neuromuscular diseases. Frambu is also responsible for Leve NÅ (Living NOW), which aims to strengthen expertise in paediatric palliative care, and both provides and coordinates services for children and families.

During my five-day visit, I spent extensive time at Frambu's facility in Siggerud, attended and presented at the Frambu National Conference, met with the paediatric palliative care team in Oslo and spent time with families impacted by childhood dementia. I also met with the neurometabolic team at National Hospital Oslo, led by Ingrid Helland, NCL expert and Medical Advisor to the Norwegian Centre for Rare Diseases. This meeting revealed Norway's data infrastructure using ORPHA codes rather than ICD codes for more granular classification, linking directly to European registries on rare disorders. The Neurometabolic team also shared how children under 16 with childhood dementia conditions receive automatic enrolment into free municipal-run habilitation services that work hand in hand with hospital services.



Frambu Centre

The Frambu Centre serves approximately 6,620 registered patients across some 450 ICD-10 codes, with 79 staff including paediatricians, psychologists, social workers, physiotherapists, and special educators. Operating as the largest of nine centres for rare diseases in Norway, Frambu is organised into two primary teams: Team 1 handles neuromuscular diseases, neurodegenerative diseases, mitochondrial conditions, and metabolic disorders; while Team 2 focuses on neurodevelopmental disorders and syndromes. The organisation functions as a private foundation largely funded by the government, providing supplementary services to the existing healthcare system rather than replacing it. The centre embodies a holistic, cross-sectoral approach that extends beyond medical care to encompass education, social support, and family empowerment, positioning itself as a bridge between families and local services, with the primary goal of educating and giving guidance to patients, their families and the professionals that work with them locally.

The centre provides a 'low threshold service' which means families can self-refer and therefore directly access support without medical gatekeeping. Beyond the residential courses, Frambu offers digital consultations via video meetings, home visits for families unable to travel, information services for newly diagnosed families, individual guidance services, and professional consultation for local teams seeking advice about rare conditions.

To give a sense of scale and reach, the centre operates from a 7,000 square metre facility with 72-bed residential capacity. Recent annual statistics include 22 patient courses with 839 participants, three summer camps with 138 participants, 18 professional courses with 1,173 participants, and 161 individual guidance services reaching 941 professionals and 410 family members.

One of the primary delivery models consists of five-day residential family courses where 12-14 families stay on-site with all accommodation, meals, and childcare provided. Families receive complete care during their stay - parents can focus entirely on learning and connecting with other families while children receive specialised care grouped by functional ability rather than diagnosis. Parents participate in educational sessions covering medical aspects, educational strategies, and support approaches while having unrestricted access to ask any questions of the multidisciplinary expert team. The peer support connection between families emerges as the most consistently valued intervention, with families from entirely different diagnoses benefiting enormously from shared experiences of navigating complex systems and managing uncertainty. This whole-family approach extends to siblings and grandparents, recognising that rare conditions affect entire family systems. Summer camps serve young people aged 12-30 with programming focused on independence and peer connections, including innovations like young adults with rare conditions as paid adapted helpers.

Frambu delivers targeted education for healthcare workers, teachers, and social workers, deliberately working across health, education, and social services to provide disorder-specific content with practical application in local contexts. The centre leverages international speaker networks through research connections to enhance course quality and relevance. This professional education component addresses the widespread knowledge gaps among local professionals who seldom encounter rare conditions in their practice.

Families can bring their local professionals (healthcare, welfare, daycare, education, etc.) with them to the residential courses, providing direct education and relationship-building between specialist and local services. At the conclusion of each course, families receive a comprehensive report on their child that they can use to guide and educate their local teams. This bridges the specialist-generalist gap with tailored guidance specific to each child's needs. Following the residential experience, families maintain access to consultation with the centre at any time through the Individual Guidance Service. When a child's complex needs, family conflicts, or unfamiliar conditions require more intensive support, approximately 30% of guidance service follow-ups involve face-to-face visits where Frambu staff travel to assess children in their natural environments and educate local services to provide appropriate care. This sustained connection, whether through digital consultation or on-site guidance, ensures benefits extend well beyond the five-day residential experience.

The grounds of the Frambu Centre

Staff develop concentrated expertise through repeated exposure - a single course may include ten children with the same neurodegenerative condition, providing observational learning opportunities impossible in standard healthcare settings where professionals might encounter one case per career. In gathering this deep understanding and expertise, staff at the centre develop great passion and are enabled to drive forward research into aspects of lived experience and care to better meet needs. The residential setting enables comprehensive data collection within established relationships, supporting over 30 ongoing research projects that resulted in 23 international publications in 2024 alone, with four ongoing PhD research projects. An example of Frambu's research impact is the SIBS program - a 5-session evidence-based intervention for siblings aged 8-16 of children with rare disorders, which has now been implemented internationally across Denmark, Australia, the United States, and other countries, demonstrating how concentrated expertise can develop scalable solutions for global application. International collaboration occurs through European Reference Networks (ERNs), where Frambu staff participate in syndrome meetings, contribute to clinical guidelines, and host international specialists. One staff member leads the interdisciplinary working group for neuromuscular management within ERN-NMD, demonstrating how concentrated expertise enables meaningful international engagement.

Frambu also operates Leve NÅ (Living NOW), a paediatric palliative care service with a different operational model from the centre's traditional non-treatment focus. Leve NÅ operates with a distinct focus on current quality of life through a sophisticated 5-step Care Support Needs Assessment process covering 16 comprehensive domains. The tool and process results in 3-4 priority domains, enabling families to focus on what matters most to them right at that moment. Research with families has revealed these top three priority areas: knowing what to expect in the future, help with work and social/economic needs, and managing worries and feelings.

Gail Hilton and Torun Marie Vatne, Frambu Centre

A significant trend at the centre involves moving from diagnosis-specific courses to challenge-based groupings such as pain and fatigue courses bringing together multiple conditions, progressive disease courses focusing on shared experiences, and transition courses addressing specific life stage challenges.

All courses and consultations are provided at no cost, with government 'educational money' replacing parent wages during attendance - though some families are unaware of this financial support. Service limitations seem minimal, but the team did note some equity challenges despite universal coverage principles. More resourceful families who can navigate complex systems and travel to courses are disproportionately represented, while cultural and linguistic diversity is somewhat limited despite available interpreter services.

Lived Experience: Øyvind & Syver

On the first day of my Norway visit, I met with Øyvind and Syver Blindheim, extraordinary advocates who live a few hours from Oslo. Syver is 13 and living with Batten Disease (NCL). As a result of this condition Syver is blind, one of the many challenges of this condition.

I joined them on a special day - it was their first visit to Magnar Bjørås' research laboratory, a facility that has had a significant contribution of funds due to Syver's advocacy work. The lab, located in Oslo's Science Park and at Laboratorymedis in Trondheim, employs approximately 40 staff across several startups. Ten of these staff members grow brain organoids ('brain in a dish') to test gene therapies and medications for NCL. During the visit, much of the interaction involved describing visual elements to Syver due to his functional blindness (he can see shadows and black/white only). Despite this, Syver engaged actively with the research environment, asking extensive questions about laboratory processes and research methods. Most significantly, Syver posed direct questions to Magnar about treatments, cures, and whether there would be a cure for him specifically. These interactions occurred while filmmakers documented their story as part of a planned 2027 film release, 'A Dad's Fight for Syver'. Incredibly moving, this was a demonstration of the power and impact of patient advocacy.

Øyvind's family includes Syver, aged 13 living with NCL (Batten disease), Syver's younger sister without NCL, and his wife who works 0.4 FTE. Øyvind stopped teaching and now works full-time as Syver's carer through the Norwegian government salary replacement system, which he describes as "the game changer" for his family. This policy enabled him to transition from teaching to full-time caregiving while maintaining financial stability, allowing him to focus on Syver's

Syver who lives with Batten Disease and his father, Øyvind

complex care needs, process ongoing grief related to the progressive condition, and engage in advocacy activities without financial stress. Øyvind emphasised that this policy recognises caregiving as legitimate, skilled work deserving of economic support. Like many families, Øyvind has pieced together a team to support Syver, what he describes as "a mini ecosystem around NCL that's working really well," including connections to expert clinicians, research participation opportunities, and peer support networks. Peer connection is key, and Syver maintains friendships with three other boys his age who have the same condition. Broader community support is also important, and the family accesses specialised services for blind children including camps through organisations like Foreningen Ridderrennet.

L-R: Gail Hilton, Professor Magnar Bjørås, Professor Ingrid B. Helland, Øyvind (Dad) & Syver Blindheim, ShareLab researchers

Syver who lives with Batten Disease and Professor Magnar Bjørås - Research scientist, ShareLab

Øyvind's family benefits from Norwegian social services support for equipment and progressive care needs, connections to patient organisations, and access to peer families facing similar challenges. The family's research participation includes contributing to biobanks supporting laboratory research and maintaining ongoing follow-up care through specialist neurologists with NCL expertise. Clinical care occurs through connections to specialist neurologists and ongoing follow-up protocols, though Øyvind noted these arrangements often depend on individual professionals' initiative rather than systematic care frameworks. These are observations not only from Syver's care, but issues that Øyvind has heard described by many other parents.

Øyvind explains that he believes "this is why we need stronger and well-funded professional teams with enough time who can provide help to local and regional professionals and ensure the optimal care and treatment for families."

Øyvind has become a prominent advocate for childhood dementia in Norway, contributing to parliamentary discussions (with the health committee and national NCL organisation), television programming, and public awareness campaigns. Syver himself participated in a Norwegian reality television series 'Team Pølsa', that aired in early 2025 and followed former Olympic skier Øystein Pettersen as he assembled and trained a team of six young people with disabilities to compete in a ski event, which led to increased public visibility and media attention. This exposure positioned Syver as something of a celebrity within Norway and contributed to broad public awareness of childhood dementia conditions as well as generating the significant research funding that flowed specifically to ShareLab. Syver explains that he describes his condition as childhood dementia so that "people can care for me better".

Øyvind's advocacy work operates largely independently, requiring significant personal capacity, foresight, and motivation to drive systemic change. He talked about the fact that many parents want to be advocates and create change, but that it is nearly impossible for many due to the high care needs and impacts of these diseases. He acknowledges pride in creating progress and driving improvements, but recognises the high personal cost associated with individual parent-driven advocacy efforts. "I know that without my efforts through Syver, childhood dementia would not be known in Norway, and we would not have managed to raise almost [the equivalent of] AU$3.5 million for Magnar's research". His recent efforts have included influencing the Norwegian National Dementia Organisation to lead the change needed in the future. So far, this organisation has run a fundraising campaign for childhood dementia research, giving childhood dementia even more publicity, and is an important step in including childhood dementia under the dementia umbrella. They have also expressed the need for implementing childhood dementia into the national dementia plan. All this is made possible because Syver wanted to tell the world about his disease.

> "We need stronger and well-funded professional teams."
> - Øyvind Blindheim, Dad to Syver.

Key Learnings from Norway

Norway's approach to childhood dementia care is distinguished by substantial government financial support, a well-established rare disease infrastructure, and Frambu Centre for Rare Disorders' unique role in serving specific rare conditions. Frambu represents an exceptional model, bringing families from across Norway to a residential centre for intensive multi-day courses combining professional education, peer support, and local provider capacity building. While much of Norway's approach—including its comprehensive government funding, universal municipal rehabilitation system, and residential centre model—is not directly replicable in the Australian context given different population distribution, healthcare structures, and federal-state divisions, several significant learnings emerged. Norway demonstrates how concentrated expertise develops through repeated exposure to specific conditions, how whole-family approaches address the reality that childhood dementia affects entire family systems, and how proactive government policy frameworks can create more equitable experiences for families navigating devastating diagnoses.

Frambu Model Builds Concentrated Expertise: Frambu Centre's deliberate focus on specific rare conditions enables staff to develop genuine specialisation through repeated exposure, with many staff maintaining 10-20+ years of experience seeing all children with particular conditions across Norway. This concentrated expertise serves multiple functions: staff become clinical experts who can guide both families and local care teams, and the centre serves as a research catalyst. For Australia, while a single national residential centre is impractical given geography and population distribution, the principle of deliberately concentrating expertise to build deep condition knowledge, support local providers, and fast-track research is important to note. This could be achieved through specialist centres in each state where multidisciplinary teams see sufficient patient volumes annually whilst also providing systematic outreach and guidance to local care teams, combining direct clinical expertise with system-wide capacity building.

Residential Courses Provide Intensive, Holistic Family Support: Frambu's residential course model brings entire families together for week-long intensive experiences that combine respite, peer support, professional education, and comprehensive child assessment in one coordinated intervention. These courses, explicitly positioned as 'family courses', focus on education and capacity building and address the reality that childhood dementia affects entire family systems. Families identify the peer connections formed during these intensive residential experiences as the most valuable intervention, with relationships often lasting years beyond initial courses. The model's strength lies in its comprehensiveness: families receive respite from daily care demands, children undergo thorough multidisciplinary assessment by genuine condition experts, parents and siblings access targeted education and support, and all family members develop peer networks with others facing similar journeys. The purpose-built facility with full accessibility features and dedicated spaces enables intensive focus impossible in fragmented outpatient appointments. For Australia, while establishing comparable residential facilities across states is impractical, the principle of bringing families together to build understanding and capacity rather than fragmenting support across individual appointments warrants consideration. This could potentially be achieved through regular peer connection opportunities online, or an annual conference or camps that combine clinical assessment, professional education, peer support, and respite, adapting Frambu's comprehensive approach to Australian geography and healthcare structures whilst maintaining the principle that childhood dementia requires whole-family interventions delivered intensively rather than piecemeal.

Government Policy Frameworks Create Equitable Family Support: Norway demonstrates how proactive government policy can transform the experience of families navigating childhood dementia. Families can access state-of-the-art equipment that enables community participation and meaningful experiences rather than meeting only basic medical requirements. Government salary replacement recognises caregiving as legitimate skilled work, acknowledging the professional nature of complex care provision and enabling parents to provide

care without financial devastation, whilst additional disability carer support provides practical assistance beyond what family members can provide alone. Additionally, Norway's 2017 Health Personnel Act amendment legally mandates healthcare professionals to assess and address sibling support needs, recognising that siblings of children with chronic conditions face increased risks for psychological and social problems. For Australia, these policy frameworks demonstrate how the government can proactively support families rather than placing responsibility solely on individual advocacy. While direct replication may not be feasible, the principles of equipment provision focused on participation rather than survival, recognition of caregiving as skilled work warranting financial support, and guidelines ensuring sibling needs are systematically addressed offer important guidance for policy development that could reduce the burden currently placed on Australian families.

Paediatric Habilitation Centres Support Skill Maintenance: In Norway, habilitation is a fundamental component of childhood dementia care, working hand in hand with neurology services rather than operating as a separate system families must navigate independently. Children under 16 receive automatic enrolment in habilitation services, with seamless integration between hospital and community care throughout disease progression. This approach positions habilitation as an embedded healthcare right rather than requiring complex eligibility assessments or coordination across disconnected systems. For Australia, this contrasts sharply with the current landscape where families must navigate fragmented systems across health, disability, and education sectors without guaranteed access to coordinated support. While Australia's NDIS provides individualised funding and choice once accessed, Norway's model demonstrates how habilitation can be systematically integrated as a core component of clinical care, working in partnership with health, rather than operating as a separate service requiring family advocacy and coordination. This integrated approach could inform Australian models that embed coordinated support across health and NDIS systems, rather than requiring families to navigate them independently.

03. UNITED KINGDOM

With a population of 67 million, a National Health Service (NHS) and extensive charity sector, the UK landscape offered a number of services that I was keen to explore through my Fellowship: comprehensive disease-specific care models for Inherited White Matter Disorders and Cockayne Syndrome, sophisticated patient organisations such as Alex TLC (discussed in detail below), NPUK and the MPS Society, an extensive children's hospice movement, and specialised nursing workforces including Diana Nurses for paediatric palliative care, and Admiral Nurses for dementia care.

During my Fellowship visit, I discovered two distinctive structural features that set the UK apart globally. First, the UK operates an extensive charity-funded healthcare workforce alongside its NHS - a dual-system approach largely unmatched internationally that has emerged from NHS funding constraints and centuries-old charitable healthcare traditions. Second, I learnt about the Child in Need legislative framework under Section 17 of the Children Act 1989, which provides structured multi-agency coordination for children with complex needs through regular review meetings, collaborative care planning, and clear accountability across health, education, and social care sectors. These discoveries, combined with patient organisation work far more extensive than I had anticipated, revealed how the UK's charity-healthcare partnership has addressed some of the challenges similar to those facing Australia: geographic inequities in specialist care, workforce shortages in rare disease expertise, and the need for family-centred support models.

At Great Ormond Street Hospital, I presented to the metabolic team and explored care coordination, family support systems, and equity challenges that they termed the 'postcode lottery.' My discussions with Professor Lorna Fraser, one of the world's leading researchers in paediatric palliative care, provided crucial context about the UK's approach. I spent extensive time with Jayne Hughes from Amy and Friends, Meg, the family support coordinator and Paula Sullivan one of the nurses who runs the Cockayne Syndrome/TTD clinic at Guy's and St Thomas' Hospital. These conversations provided detailed insights into how comprehensive disease-specific care models can operate in practice for rare childhood dementia conditions, detailed below. I presented a few times at a three-day MPS Society conference that brought together multidisciplinary experts from across the UK. For two days of the conference families joined us, many with children affected by childhood dementia caused by Hunter Syndrome and Sanfilippo Syndrome. I met children and their parents, heard parts of their stories, and witnessed firsthand the impacts of childhood dementia on the whole family.

The Admiral Nurse model pioneered by Dementia UK demonstrates how specialist nursing roles can scale nationally - from 216 nurses seven years ago to 481 currently - while maintaining rigorous standards through embedded clinical supervision and professional development. Through discussions with the ACORNS hospice manager, I learned about their integrated care approach that deliberately avoids segregating children with dementia, instead providing comprehensive palliative care that addresses behavioural challenges through specialised staffing and environmental adaptations. The Diana Nurse Team in Leicester exemplifies truly integrated community care, combining acute nursing, continuing care, and specialist palliative services under one unified team that follows children and their families from diagnosis through to bereavement. Their 24/7 on-call service and proactive advance care planning demonstrates how continuity of care can transform family experiences while building deep clinical expertise. During our discussions, the team described their current support of a family with a child affected by Lafora Disease, demonstrating how the model works in practice for childhood dementia cases.

Helena Dunbar's Kentown Support model operates as a strategic commissioning organisation that strengthens existing local services rather than creating parallel systems. Her 'jigsaw model' adapts to regional contexts while maintaining core principles. I also examined advocacy and support models developed by organisations like the MPS Society, BDFA, and Alex TLC - all led by people with lived experience who have identified critical gaps in childhood dementia care and responded by creating comprehensive support ecosystems spanning diagnosis, professional counselling, transition support, family respite, and bereavement services.

Great Ormond Street Hospital for Children

UK Advocacy & Support Organisations

The UK landscape includes numerous well-established disease-specific patient organisations (charities) that provide extensive support for childhood dementia conditions. Alex TLC (leukodystrophies), Amy and Friends (Cockayne Syndrome), MPS Society (mucopolysaccharidoses), NPUK (Niemann-Pick Type C), and BDFA (Batten Disease Family Association) demonstrate a mature not-for-profit sector with sophisticated support models extending far beyond traditional advocacy roles. These organisations have typically emerged from parent advocacy, with founders often being parents who experienced gaps in support following their own child's diagnosis, operating as primary connection points between families and their specific disease communities.

Professor Lorna Fraser and Gail Hilton

Each organisation maintains detailed databases or registries of affected families within their specific conditions, enabling targeted outreach, peer connection facilitation, and coordinated support delivery from diagnosis through to bereavement. Immediate post-diagnosis support includes welcome packs, condition-specific information resources, and direct connection to other families. Organisations offer active listening services, professional counselling, help accessing disability benefits, information on housing adaptations and specialist equipment, education healthcare plans and school presentations, referrals to social care for respite, peer befriending services, and bereavement support. Some provide bespoke training sessions for schools and professionals, quarterly wellbeing meetings and advocacy support for continuing health and social care assessments, ensuring patients are being seen by the correct specialists.

Some organisations provide emergency financial assistance for families facing crisis situations, fund medical equipment not covered by NHS provision, support travel costs for accessing specialist services or research participation, and facilitate access to respite care and family breaks. Amy and Friends specifically funds transportation for families across the UK attending the Cockayne clinic, arranges accommodation, and provides play resources while coordinating the social elements that families consistently identify as most valuable.

Several organisations operate through honorary contracts enabling direct patient support within NHS settings. Sarah Kenrick from BDFA attends clinic appointments, educational meetings, and continuing healthcare assessments when requested by families, providing advocacy and emotional support within formal care systems. Karen Harrison from Alex TLC attends clinics when invited by doctors, facilitating peer support connections and serving in a social worker-type role including assistance with practical matters. Integration with clinical specialist centres varies by condition yet is integral to progress: Amy and Friends provides essential logistical and emotional support within NHS-commissioned Cockayne clinic services; Alex TLC is the lead patient representative for the NHS commissioned Inherited White Matter Disorder service (detailed below) and the MPS Society works closely with specialist centres across Great Ormond Street, Manchester, Birmingham, and other locations.

These organisations often coordinate family participation in clinical trials and natural history studies, maintain condition-specific databases enabling research recruitment, advocate for increased research funding and priority, and facilitate international collaboration between research teams. Alex TLC's involvement in developing the IWMD Service registry demonstrates how patient organisations can influence service design while maintaining clinical data quality and family engagement.

Community building occurs through Facebook support groups, WhatsApp communities organised by condition and life stage, specialised groups for bereaved families and young adults, regional family gatherings, annual conferences, and condition-specific camps. MPS Society organises regional events throughout the UK while maintaining dedicated Northern Ireland-based advocacy workers addressing geographic and cultural access needs. System navigation support addresses UK health, education, and social care complexity through guidance on Child in Need processes, special educational needs system navigation, transition planning between paediatric and adult services, and benefits application support.

BDFA provides flexible training sessions recognising professional time constraints and funding limitations, creating memory folders enabling school transitions while preserving child identity and achievements. Sarah Kenrick emphasised universal human needs underlying specialised knowledge, focusing on helping people feel safe, in control, informed, supported, and able to grow despite devastating diagnoses. Organisations have developed condition-specific resources validated by clinical experts but driven by family experience, ensuring materials address practical needs while maintaining medical accuracy.

Cultural competency and accessibility remain ongoing challenges, with organisations developing translated materials, culturally adapted communication strategies, and partnerships with community organisations to reach diverse populations including minority ethnic communities and those with limited English proficiency. Professional boundaries ensure appropriate separation between medical advice and peer support, with organisations training their staff and volunteers to recognise the limits of their expertise and referring families to appropriate clinical professionals. Geographic coverage across the UK requires sophisticated coordination given devolved health and social care systems, with organisations adapting approaches to different regulatory environments while maintaining consistent core support functions. The ‘postcode lottery’ was discussed by all organisations who are trying to respond to national systemic inequity.

The UK model demonstrates the value that the not-for-profit sector can add. Disease-specific patient organisations have evolved from individual family advocacy into sophisticated support systems providing comprehensive wraparound care that complements clinical services. These organisations fill critical gaps in psychosocial support, care coordination, peer connection, and system navigation while maintaining appropriate boundaries with clinical care, with extensive support spanning immediate crisis response through long-term bereavement care. They have all been instrumental in driving progress in clinical care settings across the UK and working tirelessly to ensure emerging therapies move through the regulatory process and reach children, which can take years.

Cockayne Syndrome Clinic

The Cockayne Syndrome Clinic operates as an annual comprehensive assessment service established in 2019 through NHS England funding at Guy's and St. Thomas' Hospital in London, following years of advocacy from Jayne Hughes and the commitment of one health professional who vowed to make it happen. The service represents unprecedented integration between NHS healthcare delivery and patient organisation support through a partnership with Amy and Friends charity, serving families across the UK affected by Cockayne Syndrome and related conditions, including Bloom Syndrome.

Rather than traditional sequential appointments, the clinic creates a 'mini-conference' atmosphere where families attend comprehensive assessments within a single coordinated day. The large multidisciplinary team includes a geneticist, specialist dentist, dietitian, neurologist, ophthalmologist, dermatologist, psychologist, occupational therapist, immunologist, and endocrinologist. Clinical expertise develops through concentrated exposure, with team members seeing multiple patients with the same rare condition annually rather than encountering isolated cases over entire careers.

Amy and Friends play a key role in the clinic organisation, and support families through all the logistics of attending clinic, including booking and funding train tickets and accommodation. A (funded) pre-clinic dinner enables peer connections and reduces medical anxiety. Clinic days are heavily supported by the Family Coordinator, who endeavours to make the whole experience fun for children and siblings. Amy and Friends hosts an annual conference where all families attend, separate from the clinical assessments, allowing families to hear directly from experts and researchers while connecting with peers facing similar challenges. Private online forums maintain national and global family connections between annual gatherings.

Guy's & St Thomas' Hospital, London

L-R - Paula Sullivan, Nurse Practitioner, Cockayne Syndrome, Trichothiodystrophy & Bloom Syndrome Clinic, Gail Hilton; Jayne Hughes, CEO and Meg Simpson, Family Coordinator, Amy and Friends.

Specialist nurses funded through the clinic model maintain contact with families through three annual touchpoints: the conference, comprehensive home visits, and clinic assessments. During annual 2-3 hour home visits to every participating family, they conduct holistic assessments covering housing, benefits, sibling needs, and care planning. They maintain proactive contact throughout the year, provide direct GP and paediatrician liaison when children are unwell, attend 'Child in Need' meetings when requested, and often prevent hospital admissions through expert triage. Additional clinic visits occur when children require more frequent assessment. The service maintains a lifespan approach with both paediatric and adult nurses, avoiding problematic care transitions.

Families participate in natural history studies and outcome measurements within the clinical setting. The team has created custom dependency and functional ability assessment tools, conducted research on measurement techniques for patients with contractures, and maintains international research collaboration with teams in Netherlands, France, Germany, America, and Japan. Clinical improvements (so far) include slight increases in average age of death, patients remaining functionally stable for longer, early problem identification, and dramatic reduction in dietary issues and pain. Family outcomes include reduced isolation, improved confidence through access to genuine expertise, better care coordination through a single point of contact, and normalised experiences for children. International replication has begun, with the Rotterdam clinic opening in 2021, while Japan and Italy are establishing similar services.

NHS England's direct funding covers clinical costs, nurse time, and travel expenses across the UK. Families from Scotland and Wales access services without referral requirements, eliminating traditional healthcare boundaries. Amy and Friends covers additional family support costs not funded through NHS channels. There are challenges; some local clinicians resist specialist recommendations that contradict conventional knowledge for rare conditions and adult services often lack appropriate equipment and expertise for children who reach 18.

The Cockayne Syndrome Clinic demonstrates how concentrated clinical expertise, proactive family support, systematic peer connection, and embedded research can transform outcomes for rare neurodegenerative conditions, creating a sustainable model that challenges traditional healthcare delivery while addressing both clinical and psychosocial needs.

> "This is unprecedented integration between healthcare delivery and family support that doesn't happen elsewhere" - Clinic Team

Alex TLC & the Inherited White Matter Disorders Service

The Inherited White Matter Disorders (IWMD) Diagnostic and Management Service represents a nationwide NHS England commissioned service established through sustained advocacy by Alex, The Leukodystrophy Charity (Alex TLC). Founded in 2004 by Sara Hunt following her sons' leukodystrophy diagnosis in 2001, the organisation developed from individual family advocacy into systematic lobbying for nationwide service provision. The development timeline demonstrates sustained advocacy effort spanning over a decade, with a service concept originating in the 2010s, an application submitted in 2016, funding secured during COVID, and full operational status achieved in 2024.

The service operates through five children's hubs and three adult hubs providing countrywide coverage, with patients able to access services from Scotland, Wales, and Northern Ireland without referral restrictions. Three levels of patient assessment address individual needs: Level 1 serves patients already diagnosed locally with expert multidisciplinary teams adding clinical registry details and confirming care plans; Level 2 addresses patients without a confirmed diagnosis through remote review teams examining local test results and recommending further molecular or metabolic testing; Level 3 provides face-to-face assessment for complex undiagnosed cases requiring comprehensive evaluation.

Alex TLC maintains unprecedented integration within NHS service delivery through several mechanisms. The organisation operates honorary contracts with each NHS Trust, enabling direct patient support within clinical settings. Karen Harrison, Support Services Manager, accompanies families to clinics when invited by doctors, providing psychosocial support, connecting families to organisational resources, facilitating peer support connections, and serving in a social worker-type role including assistance with practical matters like blue badge applications and benefits. This personal support at IWMD Service clinics improves reach to those who may not otherwise engage with charitable support services, especially those from underrepresented communities.

To further bridge gaps, Alex TLC host a 3-day Community Weekend, with Leukolabs (a UK leukodystrophy research consortium) including a Science Day for researchers, scientists, medical professionals and industry, bringing together the patient and professional leukodystrophy communities to connect, learn from and support each other, alongside supervised activities for children to give parents some respite.

Alex TLC participated fully in service development committees during commissioning without receiving funding, contributing through survey development, registry dataset development, training, and stakeholder analysis. Alex TLC hosts the central information page for the Service and Registry and medical information informed and approved by health professionals. The team disseminates monthly plain English research summaries and research website pages, helping to bridge the gap between patients and research.

Each IWMD Lead Centre maintains specified multidisciplinary teams including a consultant neurologist as clinical lead, a second named neurologist, a designated consultant neuroradiologist with dedicated MRI reporting sessions, a consultant in clinical genetics with IWMD expertise, a designated neurology specialist nurse, a consultant clinical psychologist or neuropsychologist, a consultant in metabolic diseases, a molecular genetics laboratory scientist, and an administrative data manager. Clinical Nurse Specialists serve as accessible points of contact for families, providing knowledgeable responses about conditions and care questions, reportedly reducing emergency department presentations through expert triage and advice.

The patient registry operates as a clinical database hosted by the NHS but accessed through a central website maintained by Alex TLC, enabling the charity to drive content while ensuring clinician approval of medical information. The registry collects both clinical data and patient/carer impact measurements, facilitating access to clinical trials and research. The service demonstrates significant collaboration between hubs through joint multidisciplinary team meetings, cross-consultation between sites, regular research meetings maintaining updates

on emerging therapies, and annual national clinical meetings ensuring coordination and knowledge sharing.

Service outcomes include ensuring all age group coverage rather than paediatrics only as originally proposed, enabling patient and carer self-reporting on psychosocial and physical condition impacts, facilitating direct patient-professional comparisons within registry data, ensuring patient access to Alex TLC support services at clinic appointments, and prioritising patient demands for new research, equitable care, and clinical trial access. The service maintains a lifespan approach, preventing problematic transitions between paediatric and adult care.

Current challenges include remote neurologist reluctance to refer complex cases to hubs, meeting ongoing education needs to ensure patient connection to central hubs, difficulties obtaining complete patient registry data, and service evolution beyond primarily diagnostic functions toward comprehensive care coordination. Adult services often lack appropriate equipment and expertise for complex needs patients.

The IWMD service illustrates how patient organisations can achieve systematic healthcare change through sustained advocacy, combined with collaborative relationship building. The integration between Alex TLC and NHS service delivery creates comprehensive support addressing both clinical and psychosocial family needs within sustainable funding frameworks, with NHS England direct funding covering clinical costs while patient organisation contribution provides enhanced family support and accessibility elements.

Kentown Support

Kentown Support believes every child with a life-limiting condition and their family deserves the very best care, close to home. The Kentown children's palliative care program launched in 2022 as a three-year pilot in Lancashire and South Cumbria, leading to its formation as an independent charity in 2025. Helena Dunbar, a Churchill Fellow, established this approach following doctoral research identifying gaps in children's palliative care provision. Kentown created "a first of its kind initiative leading the way in collaborative care" - a unique UK-wide model working with local services to create more coordinated, accessible and family-focused approaches. Rather than delivering direct services, Kentown works with and supports already established universal generalist and specialist teams to further their capacity, enable equitable access, and level up their service provision.

The Kentown Model for Greater Manchester

The model focuses on what matters most to families through three integrated services: nursing support, family support, and service coordination. Together, these offer wrap-around care giving families the support they need in their own home, with families accessing different components based on changing needs throughout their journey. Kentown shares and funds research and education and training resources so that healthcare professionals have the knowledge and expertise to provide wrap-around care. The organisation identifies local providers, funds expansion of proven services, and sets outcomes for commissioned partners while maintaining core principles across regions.

The model demonstrates how strategic commissioning can strengthen existing services rather than creating parallel systems or duplicating services. Access is available from point of diagnosis regardless of medical complexity, recognising that psychosocial needs often precede medical needs. This approach offers insights for childhood dementia care, where families experience significant stress before complex medical needs develop, and report feeling that they don't fit into existing systems and services. A sustainable approach to change might be to identify and train providers rather than building new services.

Helena Dunbar, CEO and Churchill Fellow, Kentown Support

"This is a first of its kind initiative leading the way in collaborative care."

Lived Experience: Mothers Driving Systemic Change

The UK's childhood dementia landscape has been fundamentally shaped by mothers who transformed their personal experience into systematic advocacy, often while simultaneously caring for their affected children. These women represent a pattern of maternal leadership that characterises much of the UK's (and beyond) innovative service development.

Sara Hunt and Karen Harrison: Sara Hunt, single mother to Alex who had adrenoleukodystrophy (ALD) and Ayden who is living with ALD, founded Alex TLC in 2004 following her sons' diagnoses in 2001. Over more than a decade, while caring for her children, she sustained the complex advocacy effort that ultimately created the NHS England-commissioned Inherited White Matter Disorders Service. Karen Harrison, mother to three sons - Cameron who is currently living with ALD, Alexander who had ALD, and Glenn who does not have ALD, joined Alex TLC 13 years ago. She now serves as Director of Support and holds honorary contracts with each NHS Trust in the Inherited White Matter Service. Despite describing herself as "just a mum," she provides psychosocial support, facilitates peer connections, and supports families to navigate practical matters like disability benefits.

Jayne Hughes: Jayne Hughes, mother to Amy who had Cockayne Syndrome, founded Amy and Friends in 2007. After years of ongoing advocacy, Jayne was successful in establishing the Cockayne Syndrome/TTD Clinic at Guy's and St Thomas' Hospital, London in 2019; Amy and Friends has become integrally embedded within this clinic model. Her advocacy while caring for her affected child helped create what the clinic team describes as "unprecedented integration between healthcare delivery and family support that doesn't exist elsewhere."

Toni Mathieson: Toni Mathieson, mother to three children who had Niemann-Pick C disease, leads NPUK as CEO, an organisation that has evolved over 34 years to become integral partners in clinical care delivery. Under her leadership, NPUK supports 167 living patients and around 600 families, providing wraparound care that NHS staff recognise they cannot deliver alone. Her work demonstrates how patient organisations can become essential service providers working alongside NHS specialist centres. Recognising that collaboration in rare disease is required for greater awareness and action, Toni has been instrumental in establishing the UK LSD Patient Collaborative Group and the LSD Global Collaborative, heralding a new era of advocacy.

These leaders share common characteristics: they identified crucial service gaps through personal experience, maintained sustained advocacy efforts over years rather than seeking quick solutions, and created organisational infrastructure serving far beyond their own families. However, their success raises critical questions about systemic dependence on exceptional individual advocacy rather than proactive service development responsive to identified population needs. The personal cost of this leadership model is significant, with these mothers investing decades building services while managing complex family situations, often sacrificing personal wellbeing for broader community benefit.

Key Learnings from the UK

The most striking feature of the United Kingdom's children's healthcare landscape is the extensive role of the charitable sector and its deep integration with NHS service delivery. Unlike Australia, where charitable organisations primarily provide family support and advocacy separate from health services, UK charities fund clinical positions, maintain honorary contracts enabling staff to work within NHS settings, and co-deliver services through formal partnerships. While this sophisticated charity-NHS integration creates remarkable innovations, it also creates inequities based on which conditions have well-resourced patient organisations and places unsustainable pressure on the charitable sector to fill gaps that should be addressed through systematic service planning. Despite a National Health Service and the world's most developed children's hospice care infrastructure, access to coordinated childhood dementia care remains dependent on individual, condition-specific advocacy and the postcode lottery, rather than population-level health planning.

Family-Driven Innovation Rather Than Systematic Planning: The UK's most sophisticated care models for diseases that cause childhood dementia exist because of sustained parental advocacy over decades, not proactive healthcare policy development. Sara Hunt's 10+ year effort co-creating the IWMD service and Jayne Hughes' ongoing advocacy establishing the Cockayne clinic model represent exceptional individual investment rather than systematic responsiveness to identified population needs. This demonstrates both the potential for transformational change and the problematic dependence on extraordinary personal sacrifice when systems fail to respond proactively. For Australia, this reinforces the pattern observed across all countries visited: transformational care models emerge from parents rather than proactive system design, highlighting the urgent need for systematic service planning that doesn't depend on extraordinary individual advocacy.

Partnerships Between Charities and Health Services Improve Care: Formal integration between charitable organisations and health services creates superior care models compared to either working in isolation. Alex TLC maintains honorary contracts enabling direct family support within NHS clinical settings, while the Cockayne clinic exemplifies optimal integration: NHS England funds clinical costs while Amy and Friends provides enhanced family support, creating seamless medical expertise combined with community connections. For Australia, where NDIS and health systems operate separately with minimal integration, the UK model demonstrates how formal partnerships between charitable organisations and health services can create comprehensive support without requiring families to navigate disconnected systems. However, this also raises questions about sustainability and whether charitable funding should be necessary to achieve comprehensive care that arguably should be a health system responsibility.

Disease-Specific Services Build Expertise But Create Systematic Inequities: UK disease-specific services enable staff to develop genuine, deep knowledge through seeing multiple patients with the same condition rather than encountering isolated cases. The Cockayne clinic's multidisciplinary team sees multiple patients with the same rare condition annually, building concentrated, clinical expertise that could otherwise not be achieved. However, while some conditions access comprehensive care through sophisticated charity-NHS partnerships and concentrated expertise, others navigate fragmented services without disease-specific clinics or support networks. As Great Ormond Street Hospital's metabolic team confirmed, "care quality depends on the specific condition and which team and patient organisation the child falls under," creating a two-tier system within the already vulnerable childhood dementia cohort. The ongoing postcode lottery, despite proven models of care, reveals structural barriers to scaling excellence and suggests that charitable innovation may mask systemic neglect of childhood dementia. For Australia, this demonstrates both the value of concentrated expertise and the risk that disease-specific models create inequities. CDI's collective approach offers an alternative that could build concentrated expertise whilst ensuring equitable access across all conditions rather than depending on individual organisational capacity.

Strategic Commissioning Strengthens Existing Services: Helena Dunbar's Kentown Support model commissions existing local providers to expand capacity through their 'jigsaw model'—identifying and funding gaps in local service provision rather than creating new infrastructure. This builds on community strengths whilst actively avoiding service duplication and adapting to regional contexts. For Australia, this commissioning approach offers an alternative to creating new specialist services. The Kentown model demonstrates how strategic investment in existing providers can achieve coordinated care without duplicating infrastructure, though this requires sophisticated understanding of local service landscapes and sustained funding commitments.

Legislative Frameworks Can Enable Cross-Sector Coordination: The UK's Child in Need legislation under Section 17 of the Children Act 1989 provides a structured statutory framework for coordinating multi-agency support, requiring regular review meetings to bring together health, education, social care, and voluntary sector representatives. In theory, when this works well for families affected by childhood dementia, it should reduce the burden on parents to serve as the sole connection point and source of knowledge to all services and systems, instead creating a network of informed and coordinated support across sectors around the family. For Australia, this demonstrates how legislative frameworks can mandate coordination across sectors that currently operate independently. While Australia's systems differ—with NDIS providing disability support separate from state health systems—the principle of statutory coordination requirements could address the persistent fragmentation families experience when navigating multiple disconnected systems, shifting responsibility from parents to systems. However, this would require careful adaptation to Australia's federal structure and the unique NDIS-health system interface.

> "Care quality depends on the specific condition and which team and patient organisation the child falls under." - Metabolic Team, Great Ormond Street Hospital.

04. CANADA

With a comparable geography and population, Canada also has a similar health care system to Australia in that there is a federal department responsible for policy, and the delivery of health care is devolved to the provinces. During my week in Canada, I looked at models that have been successful in creating nationally consistent approaches to the improvement of care across provinces. The Solutions for Kids in Pain (SKIP) model, detailed below, offered insights into systematic implementation of a national care standard across multiple jurisdictions, while the Pediatric Palliative Care Alliance exemplified coordinated national investment in service development and professional networking. Both models addressed challenges directly relevant to Australia's federal structure and the need for national consistency in childhood dementia care.

The Canadian Pediatric Palliative Care Alliance is a nationally funded resource center dedicated to strengthening local paediatric palliative care capacity across Canada, reducing reliance on specialist centers and enabling communities to provide care closer to home. Through awareness building, capacity development via a Project Echo Community of Practice, and national advocacy, the Alliance is developing a shared vision for paediatric palliative care. A critical insight from the Alliance is that children with progressive neurological conditions like childhood dementia typically fall within 'complex care' systems that excel at managing acute medical needs but lack integration with palliative care approaches, though some Canadian centers have successfully developed more integrated models that support whole families through progressive decline.

SickKids Hospital operates as one of the world's leading paediatric research hospitals, with a strategic plan extending to 2030 that focuses on personalised, precision medicine approaches tailored to each child. The hospital's leadership includes a CEO with a background in genetic metabolic medicine, enabling sophisticated understanding of rare disease challenges. However, Canada's provincial healthcare delivery creates tensions between national approaches and regional variation in service quality and access. During my five-day visit, I spent extensive time at SickKids discussing their integrated care program and pain management approaches and met with multiple healthcare professionals who are developing innovative care models. I gave a presentation on childhood dementia to the SickKids Paediatric Advanced Care Team (PACT) which provoked conversation about the isolation of families with individually rare conditions, the value of peer support through unified language of childhood dementia, and the unique grief and loss experience of this cohort. A focus was understanding Canada's emerging precision medicine infrastructure, particularly SickKids' approach to developing individualised treatments based on genetic variants within rare conditions. This represents cutting-edge thinking about how to move beyond standardised approaches toward truly personalised medicine. However, implementation remains in the early stages, with significant resource requirements and complex approval processes limiting systematic application.

Beyond precision medicine, Canada's approach to care coordination emerged as a crucial concept throughout my visit. Multiple healthcare professionals described complex care as "the game changer for families," providing comprehensive coordination, advocacy support, and efficient cross-specialty working. However, access to complex care requires meeting specific medical complexity thresholds, leaving many childhood dementia families without coordinated support during earlier disease stages when behavioural and educational challenges may be most significant. Ontario's 'Complex Care Kids Ontario' program provides a nurse practitioner-led care coordination model through a provincially funded hub-and-spoke system (where specialist hospitals serve as central hubs with satellite clinics in communities), with eligibility based on four domains: chronic conditions with fragility, high healthcare use, functional limitations, and family-identified needs. The program has deliberately lowered inclusion barriers for families facing structural barriers to healthcare access, such as those living more than 100 kilometers from major specialist centers. A key insight from Ontario's 10-year complex care journey is the importance of starting simple with flexible approaches and willing partners, focusing on coordinating care at the patient level across health and disability services rather than waiting for whole-system reform.

SickKids Hospital, Toronto

Inside SickKids Hospital, Toronto

I also visited Safehaven, a government-supported organisation in Toronto that provides residential, respite, and transitional care for children, youth, and adults with complex medical needs. Safehaven operates six homes across the Greater Toronto Area, with a total of 60 beds, including its 15-bed head office location, situated approximately 5km from SickKids Hospital. Depending on their needs, some clients stay for short-term respite, others live at Safehaven permanently, and some receive temporary support as they transition from hospital back home. By offering this continuum of care, Safehaven helps families remain together and ensures that individuals with complex needs can live with dignity, belonging, and quality of life. The organisation also leads a national community of practice on integrated care, connecting partners across the health, disability, and housing sectors to bridge the gap between health and community support.

Members of the team at Safehaven, Toronto

The Canadian MPS Society facilitated meetings with families affected by Sanfilippo Syndrome, providing crucial insights into lived experience within Canada's healthcare system. I also met with families impacted by Niemann Pick Type C Disease and Batten Disease, both of which cause childhood dementia. The families I met revealed both strengths and persistent gaps within Canada's approach, but broadly families consistently reported having to *"fight for everything we need."* Despite universal healthcare principles, significant inequities exist based on geographic location, with some families reporting better care in rural areas than urban centres. The autism diagnosis pathway highlighted how system design can create unintended consequences, with childhood dementia families seeking autism diagnoses to access behavioural support services. These stories revealed that even well-resourced universal systems struggle with care coordination for complex conditions that don't fit neatly within existing frameworks. A consistent theme was the disconnect between professional descriptions of comprehensive, coordinated care, and family experiences of fragmented, advocacy-intensive navigation, consistent with the Australian context.

L-R: Three parents impacted by Sanfilippo Syndrome, Gail Hilton, Mary Bone, CEO, Canadian MPS Society

SKIP (Solutions for Kids in Pain)

Solutions for Kids in Pain (SKIP) shows how to implement a national standard of care systematically across provinces and healthcare institutions. Founded by Dr. Christine Chambers after experiencing inadequate pain management for her own children despite her expertise as a pain researcher, SKIP offers insights into implementing national standards across a federated health system similar to Australia's. The SKIP project commenced with federal funding (CAD$1.6 million over four years) which has now ended. Subsequently SKIP transitioned to Dalhousie University for administrative support, maintaining operations through philanthropic grants, though sustainable ongoing funding remains a challenge.

SKIP's major achievement is creating the world's first Pediatric Pain Management health standard (CAN/HSO 13200:2023) through rigorous national and international collaboration. The national standard applies to individual hospitals, specifying responsibilities across 34 criteria, split between responsibilities for organisational leaders and healthcare teams. SKIP is integrating this standard into Canada's Accreditation framework, making pain management criteria part of hospital accreditation processes, and providing the standard enforcement mechanisms.

SKIP operates through 'clinical implementation specialists' - healthcare professionals with clinical credibility who drive practice change from within major Canadian children's hospitals using the national standard. These specialists conduct gap analyses (assessments of current versus best practice), assess institutional readiness, and help healthcare settings select specific areas for focused intervention. This incremental approach creates quick wins while building momentum for systematic change.

For childhood dementia, SKIP demonstrates how national change can be led and implemented across a health system similar to Australia's federal structure. Their approach shows the benefit of developing a world class national standard, then systematically implementing this standard across multiple healthcare institutions through clinical implementation specialists embedded within each setting. Most significantly, SKIP's model reveals that creating a national standard is only the beginning - the ongoing implementation process requiring gap analysis, institutional readiness assessment, and ongoing support to change practice represents the critical work that transforms policy into improved care delivery.

Lived Experience: Terri, Mark, Claire & Josef

Terri and Mark manage care for their family following the loss of their daughter Claire at age 9 to CLN2 Batten disease, a form of childhood dementia, in October 2024, while continuing to support their son Josef, now 8, who lives with the same condition and is currently receiving treatment. Terri works as leader of the Canadian CLN2 Foundation, which she and husband Mark founded, advocating for improved care and access to treatments for families affected by this form of childhood dementia.

Their diagnostic journey began when Claire experienced her first seizure just after her third birthday. Following months of unsuccessful seizure medications and bypassing Canadian wait times for MRI by travelling to the United States, genetic testing revealed Claire had CLN2 Batten disease in January 2018. Several weeks later, they learned that their 24-month-old son Josef also had CLN2, making them one of very few families worldwide with siblings both diagnosed so young. Terri described the physical pain of this news: *"I cried out, I fell to the floor, the physical pain ripped through me, I couldn't breathe."*

Terri, Claire, Josef & Mark Gortnar

Facing a government system that had approved but not yet funded enzyme replacement therapy, Terri and Mark launched an intensive seven-week advocacy campaign involving MPs, hospital teams, and a documentary video that reached 40,000 views. They were prepared to *"sell our house and everything we owned,"* rather than wait for government funding decisions. Their advocacy succeeded in securing funding, and both children began receiving enzyme replacement therapy through SickKids Hospital. Following their successful advocacy, the family relocated 100km north of Toronto, where Terri reports an experience of superior care quality compared to urban centers. However, significant challenges persist. Terri describes having to *"fight for everything that we need."* Josef, despite being blind and having a life-limiting condition, remains ineligible for certain types of disability support, requiring the family to go to "extreme lengths" to secure necessary care.

As treatment progressed there were stark differences between the children. For Claire, treatment began too late to significantly alter disease progression, and the family made the difficult decision to discontinue treatment due to its impact on quality of life. In contrast, because treatment began earlier for Josef, he has had a markedly different trajectory - despite significant visual impairment, he maintains mainstream school attendance and is described as *"doing really well."*

Terri Gortnar and Gail Hilton

> "I cried out, I fell to the floor, the physical pain ripped through me, I couldn't breathe." - Terri, mum to Claire & Josef

A particularly heartbreaking aspect of care and support involved a nurse who had supported Claire for five years, becoming integral to their family. As Claire approached end-of-life, this nurse left the family as she was unable to cope with the reality of the situation, leaving the family without familiar crucial care during their most critical time. When she finally made contact months later, she explained that *"Claire wouldn't have wanted me to see her like that,"* highlighting that improved training and support for healthcare workers facing the emotional demands of progressive conditions is further needed within the system.

Looking back on the last few years, Terri identified peer and family support as essential to their survival, describing Facebook groups and connections with other families as what "saved her." Additionally, having mental health support established before the crisis proved crucial - she credits her therapist, whom she had seen prior to the diagnoses, as someone *"she wouldn't have survived without having in place."*

Through the CLN2 Canada Foundation, Terri and Mark continue advocating for systemic change while managing Josef's ongoing care needs. However, Terri describes the sustainability challenges of running a patient advocacy organisation while caring for a child with complex needs - ongoing financial challenges, emotionally challenging work, and limited recognition for family-driven advocacy that achieved system changes. Terri's experience exemplifies the pattern where *"it's always families creating the change,"* rather than proactive system responses. Her priority recommendations focus on peer support and connections with other families, and mental health support established before crisis points. Her account demonstrates both the possibility of achieving transformational system change through sustained family advocacy, and the urgent need for systems to assume greater responsibility for coordinating care without requiring extraordinary family-led efforts.

Key Learnings from Canada

The Canadian experience demonstrates that well-resourced healthcare systems alone don't address fundamental challenges in supporting families through devastating diagnoses and complex care journeys. Even within universal systems with strong clinical expertise and significant research investment, families continue to experience isolation, the burden of having to constantly advocate for services, and disjointed care. Importantly, Canadian healthcare professionals themselves consistently acknowledged that childhood dementia families 'fall through the gaps' of existing frameworks, providing professional validation of what families in other countries have reported. This professional recognition validates the need for developing dedicated care models rather than expecting families to navigate existing systems that are inadequate.

National Consistency Requires Innovative Sustained Implementation Models: Canada's federal structure mirrors Australia's challenge of achieving national consistency while respecting provincial/state independence in healthcare delivery. The SKIP model demonstrates how a sustained, multi-year implementation strategy can embed a single national standard consistently across different provinces through dedicated coordination, ongoing professional development, and continuous quality improvement. The Paediatric Palliative Care Alliance illustrates how federal investment can support capacity building and workforce development while enabling communities to deliver care locally. However, both models require sustained commitment spanning multiple years and dedicated teams and resources for implementation, rather than one-time policy announcements. For Australia, this highlights the need for implementation strategies that go beyond developing national standards to include structured processes that ensure standards are adopted in practice, with progress monitored, as well as dedicated coordination resources. The Canadian experience suggests that achieving true national consistency requires innovative approaches to long-term support for change across independent healthcare systems rather than relying on policy directives alone.

Precision Medicine Requires Significant Infrastructure Investment: SickKids Hospital's precision medicine approach demonstrates the substantial infrastructure needed to move beyond existing, limited rare disease treatment toward truly individualised medicine tailored to each child. Their philosophy involves extensive analysis of genetic variants, individualised treatment development, and committee-based approval processes for trials designed for individual patients (n-of-one trials). However, implementation remains resource-intensive and limited to specific cases rather than systematic application. For Australia, this highlights both the potential for precision medicine approaches and the significant investment required in research infrastructure, approval systems, and professional development to make individualised treatments accessible to childhood dementia families. The Canadian experience suggests that precision medicine represents a long-term evolution rather than immediate solution, requiring sustained commitment and realistic expectations about implementation timelines.

Complex Care Fills Late-Stage Gaps But Leaves Early Needs Unmet: Canada's complex care model provides comprehensive healthcare coordination that families and professionals describe as 'game-changing' when accessible. The model offers coordinated appointments, cross-specialty communication, advocacy support, and holistic family assessment. The integrated care program ensures that children with medical complexity transition from hospital to home with the support and training they require. Access to complex care, however, requires meeting specific criteria for medical complexity that many childhood dementia families don't reach until later disease stages. As this is primarily a healthcare-focused model, coordination across disability, education and psychosocial support services is not included. Facilities like Safehaven provide crucial respite support for medically complex children, yet for those with dementia who may have significant behavioural and psychological symptoms, there are gaps in services until children have developed significant medical needs. This creates a troubling paradox where excellent coordinated medical care exists but remains inaccessible to families needing support coordinating multi-system care during early disease progression. Australia's developing care models must consider how to provide coordination and support across systems and services throughout the entire course of the disease, rather than focusing only on healthcare and only after medical complexity increases.

Family-Driven System Change is Unsustainable: Families like Mark and Terri Gortnar's demonstrate remarkable capacity to achieve transformational system changes through sustained advocacy - in the Gortnar's case successfully bringing enzyme replacement therapy funding to Canada and influencing research investment. However, this model places extraordinary personal costs on families already managing complex care situations while providing no formal recognition or support for advocacy contributions that benefit entire communities. There is a need for systems to assume greater responsibility for identifying and addressing gaps proactively rather than relying on exceptional individual family efforts. For Australia, this underscores the importance of developing policy and practice solutions that don't depend on family advocacy to drive necessary improvements.

05. USA

The United States is one of the countries at the forefront of therapeutic development for childhood dementia conditions. This fact, combined with innovative clinical models, was my reason for visiting. My time in the USA included a week at the Children's Hospital Colorado, known for its multidisciplinary clinic model, and three days at Stanford University in California. The focus of my time in Colorado was understanding the Batten Disease and Neurodegenerative Clinic, led by Dr Scott Demarest, which represents one of the few care models globally that is specifically designed for multiple paediatric neurodegenerative conditions, detailed below. Dr Lauren Treat, a neurologist and palliative care physician at the University of Colorado and founding member of the International Neuropalliative Care Society, facilitated my visit to Children's Hospital Colorado, connecting me with the multidisciplinary teams and specialists whose work is detailed throughout this chapter.

In Colorado, I observed several multidisciplinary clinic models including SynGAP, Rett Syndrome, and Special Care clinics, providing comprehensive insights into different approaches to coordinated clinical care. These multidisciplinary clinics are an established way of working at Children's Hospital Colorado, with dedicated spaces designed to accommodate multiple specialists seeing families in coordinated appointments. Families travel from across the region and beyond—some from neighbouring states—on an annual or biannual basis to access these specialist teams, receiving comprehensive assessments and care planning in a single visit. This established multidisciplinary clinic model, detailed below, demonstrates what is possible when care is truly designed around family needs, delivering comprehensive support efficiently in a single coordinated visit. However, my observation also revealed sustainability challenges facing even well-resourced institutions, particularly in an environment where federal funding cuts are likely. Nearly every specialised clinic I observed owed its existence to patient organisation advocacy and funding—from the Rett clinic funded by Rocky Mountain Rett Association to the neurodegenerative clinic established through Mila's Miracle Foundation.

Beyond the clinic observations, I met with specialists addressing specific aspects of the care journey. Maria, a Parent Champion with Courageous Parents Network, demonstrated how parents with lived experience can be professionally integrated into healthcare systems through paid advocacy roles. Leanne, a genetic counsellor, highlighted the variability in diagnosis communication across different professionals and the ongoing challenge of matching support to family readiness. AJ Lotz, the hospital's bereavement coordinator, described systematic approaches to grief support including structured follow-up and ongoing group programs. Jennifer Disabato, co-director of transition programs, outlined the complexities of moving young adults from paediatric to adult services—a challenge particularly acute for conditions like childhood dementia where survival into adulthood is increasing. These conversations reinforced that comprehensive care requires attention to the entire disease trajectory, from diagnosis through to bereavement, with multidisciplinary professionals in the care team providing specialised support at each transition point.

The clinical models I observed were supported with and enabled by excellent data infrastructure. Federal legislation (HIPAA) supports principles of patient access to their health information, manifesting practically through Epic health records, with family portals enabling real-time access to consultation notes and complete medical records. Consultations were recorded by some professionals (technology currently being piloted) and notes immediately uploaded into the patient record. Families could access all elements of their record including upcoming appointments, consultation notes and test results. Permission can be given for access to patient records across health services where systems can integrate with Epic.

My final conversation at Children's Hospital Colorado was with Dr Lauren Treat, a child neurologist and international leader in the emerging field of neuropalliative care. One of only a handful of physicians board-certified in both neurology and palliative medicine, Lauren is a founding member of the International Neuropalliative Care Society. She participates in both general neurology and paediatric palliative care clinics, working with families to help balance the impact of disease and treatments with overall quality of life. Her focus on helping individuals with serious neurological conditions live as well as possible for as long as possible has been instrumental in establishing neuropalliative care as a distinct discipline. She emphasised the critical need to educate neurologists to have conversations about quality of life alongside discussions of emerging therapies—recognising that treatments for conditions that cause childhood dementia will likely be iterative improvements rather than cures. Good quality communication will increasingly require ongoing dialogue about what life extension might really mean for children and families.

I travelled to Stanford University in California to hear about their clinical neurology services and to understand their palliative care research program in more depth. I met with members of the research team led by Professor Justin Baker and Professor Claire Wakefield, as well as neurologists who described their approaches to caring for their patient population. Building on the conversation I had had with Dr Lauren Treat, of particular interest was Dr Grant Lin's work on neuropalliative care approaches—palliative care specifically adapted for progressive neurological conditions—that recognise the distinct challenges of these conditions in childhood. His work on neuro-prognostication (predicting and communicating disease progression in neurological conditions) represents innovative thinking about how doctors communicate with families. He discussed the need to move away from clinical language about 'normal versus abnormal' development toward approaches that recognise each child's unique journey while still providing honest information about what to expect.

Neurologist Dr Sofia Dhanani described best practice examples of communication excellence in the neurology department: a four-tier system ensuring families always have appropriate access to care and advice through the MyChart portal, with a 48-hour response time to queries, a specialist nurse advice line during business hours, 24/7 on-call neurology service, and emergency services for crises care. She also described an informal practice where neurologists provide a handover of information to local paediatricians post-appointment to ensure care continuity for families. These conversations are conducted for care excellence rather than billing. This example reinforces a recurring theme: excellence often depends on individual practitioners' commitment to quality care rather than systematic policy or infrastructure that ensures such approaches occur universally.

The families I encountered throughout my US visit shared both the benefits and persistent challenges within American systems. I met Camden's family, who had travelled from Kansas for specialised SynGAP care. They clearly valued the multidisciplinary clinics in Colorado for their expertise and coordination. Despite experiencing some information overload, families attending the clinics expressed gratitude for their existence but spoke of knowledge, support and practice gaps in local settings. Comprehensive reports from the specialist clinics act as a reference document for guiding local care. Having a centralised expert team who knows the child that local teams can access was also seen as extremely beneficial. As in Australia, access to appropriate care and services across health, disability and education in rural and regional areas is extremely challenging.

Gail Hilton at Stanford University

Once again, the power of family advocacy in establishing specialist services was particularly evident. Julia Vitarello's story (detailed below) outlines how the loss of her daughter and subsequent set-up of Mila's Miracle Foundation led to the establishment of the Batten Disease and Neurodegenerative Clinic at Children's Hospital Colorado. Even in the most advanced examples of care models, parents are instrumental in driving the improvements.

Batten and Neurodegenerative Disease Multidisciplinary Clinic

The Batten and Neurodegenerative Disease Program at Children's Hospital Colorado represents one of the most comprehensive models globally for coordinated care of childhood dementia conditions. Established in 2020 through collaboration between Mila's Miracle Foundation and the Rare Sisters Batten Foundation, the clinic serves patients with Batten disease and other neurodegenerative conditions through a comprehensive team approach. Co-medical directors Dr Scott Demarest and Dr Kourtney Santucci designed the clinic to deliberately prioritise building ongoing relationships over the disease trajectory and proactive rather than crisis-driven intervention, ensuring families have a consistent team of professionals who know their child well and can provide care and guidance as needs change over time. Many families are local whilst others travel across states to access this specialised care, which then guides their local care teams in ongoing management.

The clinic model was designed around recognition that degenerative conditions require fundamentally different approaches from developmental disorders. Operating on six-monthly visits rather than annual consultations, the clinic approach acknowledges that periods of rapid change require responsive clinical relationships. As Dr Demarest explained: *"For a degenerative condition, we wanted to create a little more of a home, and we wanted to be able to see patients more frequently, not all the time, but at the times that things are rapidly changing, you need to be able to respond to it."* The clinic's approach to family support recognises the distinct psychosocial challenges of degenerative versus developmental conditions. Whilst both require specialised support, the grief patterns and family adaptation differ significantly. The clinic provides integrated palliative care from early stages, recognising that anticipatory grief and quality of life discussions benefit from early introduction rather than waiting until crisis point.

The clinic operates two half-days per month, with capacity to see four patients per day. Foundation funding initially underwrote the position of a dedicated registered nurse care coordinator, essential infrastructure for the clinic's coordinated approach. The team includes neurology, neuropsychology, rehabilitation medicine, palliative care, speech and language therapy, physical and occupational therapy, nutrition, social work, nursing, and a family navigator. However, the model's innovation lies not just in team composition but in how the team coordinates.

Dr Santucci described the clinic's round-robin model: families arrive at 8:30am and rotate through four core consultation groups over the morning. Neurology and genetics consult together; rehabilitation medicine (physiatrist with physical therapy, occupational therapy, and speech therapy) forms another team; psychiatry provides another consultation; and the research coordinator with nurse care coordinator ensures standardised assessments are completed. In the afternoon, Dr Santucci sees patients in the Special Care Clinic, addressing primary care needs including immunisations, sleep, constipation, feeding concerns, school issues, and connecting families with dietetics when needed.

The team meets over the lunch hour to exchange information, ensuring comprehensive coordination and shared decision-making across all specialties. Dr Santucci explains: "The patients who I've seen in the morning are going to be seen in the afternoon in the MDC space, and so I'm giving them the update on all the other stuff, you know, like sleep and gait and bladder and other symptoms." This pre- and post-consultation discussion model prevents fragmentation and ensures families receive consistent rather than conflicting recommendations. The model demands a full day commitment from families, though the team has responded to feedback by creating flexibility for families who cannot tolerate extended hospital days, offering telehealth follow-up for some components when needed.

The clinic's voice banking program demonstrates the value of proactive early intervention. Speech therapists work with children in the early stages of their disease, knowing that speech articulation issues and cognitive decline will occur at different paces. By preserving the child's voice through recording sessions, the recorded voice is incorporated into assistive technologies (communication devices) that children use when natural speech becomes difficult. This represents forward-thinking intervention that improves quality of life in the longer term.

> "For a degenerative condition, we wanted to create a little more of a home, and we wanted to be able to see patients more frequently, not all the time, but at the times that things are rapidly changing, you need to be able to respond to it." - Dr Demarest

The clinic has developed concentrated expertise through deliberate condition grouping, enabling providers to see sufficient patient volumes to develop genuine rather than superficial knowledge. Dr Demarest suggested his own critical threshold: "You need to see a certain number of them. And it's not 2,000 patients, but 20 patients goes a really long way… as an individual provider, you've seen 20 patients with the same disease or with similar diseases. You're like, I think I understand this disease a little bit now."

Research is integrated into routine clinical care through standardised assessments and data collection. Dr Demarest emphasised that this research-focused approach improves clinical care by ensuring comprehensive evaluation: "Trying to be research focused, I strongly believe we improve clinical care because we have standardised templates. We know we're supposed to find out, do they have this, this, this and this issue. So I ask about it every time, and then even when the family doesn't bring it up, I ask about it." The clinic uses condition-specific rating scales including the Unified Batten Disease Rating Scale (UBDRS) and Hamburg scales depending on Batten type. Dr Santucci notes that research coordinators "make sure that we don't forget, in the course of doing symptom management, to also collect those rating scale observations."

Both Dr Demarest and Dr Santucci emphasise the clinic's critical role as new treatments emerge. Dr Demarest champions the essential role comprehensive clinical care must play in enabling access to therapies, monitoring their effects, and supporting families as treatments scale. Dr Santucci reinforces this: "Mechanistically as a hospital, I don't think we would be able to stand up participation in those kinds of protocols if we didn't already have this clinic built, because the clinic and the visit that we do in the clinic are where we're going to really be gathering the required research outcomes data to determine efficacy and natural history."

Treatments are likely to involve incremental improvements—slowing progression, stopping progression, or extending life with improved quality—rather than complete reversal of neurodegeneration. The clinic's model demonstrates the infrastructure required to embrace emerging therapies responsibly: coordinated multidisciplinary teams capable of monitoring outcomes, managing expectations, supporting decision-making, and helping families navigate the complex and uncertain journey these new options will bring. The clinic infrastructure provides a level of readiness for the future as well as improved care right now.

Lived Experience: Julia Vitarello

In December 2016, Julia Vitarello's six-year-old daughter Mila was diagnosed with Batten disease, one of the diseases that results in childhood dementia. Upon learning that no child had ever survived Mila's disease and there was no cure, Julia set out to develop a treatment for Mila. She raised nearly millions of US dollars from over 6,000 supporters and collaborated with Dr Timothy Yu at Boston Children's Hospital to develop the first-ever medicine tailored to just one person—named Milasen. After showing great promise in the first year of treatment, Mila's disease slowly continued to progress. In February 2021, Mila died.

What began as a race to save Mila's life has since turned into Julia's mission to transform care and treatment for all children with fatal genetic diseases. She established Mila's Miracle Foundation to drive both the development of specialised clinical care services, and transformation of regulatory processes around individualised medicines. However, Mila's experience revealed that developing individualised medicines for single patients is not a scalable solution. The tragedy of Mila's death became the catalyst for Julia's work to transform the regulatory and therapeutic development landscape itself, recognising that complete system transformation is required if children with rare genetic diseases are to access appropriate medicines designed for them.

Gail Hilton & Julia Viterello

Julia founded the neurodegenerative clinic that became a key focus of discussion during my Colorado visit. Her foundation provided the generous donation that enabled the clinic's creation in autumn 2020, directly funding the nurse coordinator position and providing ongoing operational support for the clinic's first four years. After establishing sustainable operations through to 2024, she transitioned responsibility to the hospital system. This transition highlights a recurring challenge across exemplar care models: sustainability of funding beyond the initial investment. The clinic's success represents Julia's vision of moving beyond individual cases towards systematic solutions, creating infrastructure that enables ongoing research, care, and symptom management for multiple families, whilst building the foundation for future therapeutic access.

Beyond tangible service delivery, Julia's broader regulatory advocacy focuses on process-based approval models rather than individual product authorisation. She advocates for adapting how surgical medicine is regulated to drug development: just as surgeons are certified to perform procedures without seeking approval for each individual operation, qualified professionals should be approved to develop and administer personalised treatments without requiring separate authorisation for each patient's medicine. Julia has co-founded two companies to progress her vision - EveryONE Medicines (EOM) and N=1 Collaborative, both necessary groups to make individual medicines scalable. Her international perspective focuses on identifying progressive regulatory environments for pilot programs, with the UK currently showing the most progressive approach through Genomics UK and Harrington Oxford teams. A new policy paper published in the UK - Rare Therapies and UK Regulatory Conditions - demonstrates the progress in the approach that Julia has been pushing for. She believes Australia represents a promising pilot location due to its healthcare system structure and capacity for policy innovation.

Julia's strategic approach groups conditions by shared characteristics—what she terms "end-stage genetic diseases"—rather than maintaining disease-specific silos. She was particularly enthusiastic about the childhood dementia grouping, recognising it creates the critical mass needed to drive regulatory change whilst enabling more efficient therapeutic development and serving far more families than single-disease centres could reach.

Julia's story reinforces a recurring pattern across countries: transformational change is driven by parents rather than proactive system or policy responses. Her dual impact—creating immediate clinical infrastructure whilst championing fundamental regulatory transformation—demonstrates both the extraordinary capacity of families to drive systematic change, and the urgent need for systems to assume greater responsibility for identifying and addressing gaps before families must advocate for solutions that should already exist.

> 'These conditions can be described as 'end-stage genetic disease.'
> - Julia Viterello

Key Learnings from The USA

My visit to Colorado and Stanford revealed innovative practice, particularly the Children's Hospital Colorado's multidisciplinary clinic model demonstrating comprehensive coordinated healthcare. However, even these exemplary services in well-resourced centres are often driven by family advocacy. It should be noted that the United States healthcare landscape varies significantly state by state, with substantial differences in disability support systems and service availability creating vastly different experiences for families depending on where they live. The examples of good practice described here are not available nationwide.

Multidisciplinary Clinics Provide Coordinated, Expert Healthcare: The neurodegenerative clinic at Children's Hospital Colorado exemplifies the value of coordinated team approaches over seeing multiple specialists separately. For families, this means seeing all specialists in one place on one day, with confidence they're being seen by health professionals who are genuine experts in their child's condition, have access to up-to-date interventions and treatments, and truly know their child across the disease trajectory. The clinic's pre- and post-consultation team meetings ensure comprehensive coordination and shared decision-making across all specialties, preventing fragmentation and ensuring families receive consistent rather than conflicting recommendations. Crucially, these clinics provide roadmaps for care that local teams can follow, extending their expertise beyond the specialist centre to support families in their home communities. This 'medical home' approach—where a consistent team provides a base for care coordination—builds expertise not just within the specialised clinic but throughout the broader network of local providers, ensuring consistent, informed care regardless of geographic location. Families also have a team they can contact at any time with questions, rather than navigating multiple unconnected specialists who may provide conflicting advice or require repeated assessments. The integrated team approach enables innovative interventions like voice banking, where speech therapists work proactively with children before they need speech therapy, recognising that speech articulation issues and cognitive decline occur at different paces. In the Australian context, where families typically navigate multiple separate specialist appointments without coordination, this demonstrates that childhood dementia care could benefit significantly from multidisciplinary models that prioritise relationship-building and systematic coordination, whilst building capacity in local care teams through clear care roadmaps.

Building Concentrated Expertise Requires Seeing Enough Patients: Dr Demarest notes that in rare disease you are always learning alongside patients and he is careful to note that the 20-patient threshold for meaningful expertise development is arbitrary, but that he has found this is the number where he begins to have a framework for a disease. This finding suggests that distributing rare disease care across many providers, each seeing occasional cases, produces superficial rather than sophisticated knowledge. The concentrated expertise model, where specialists physically see sufficient numbers of patients with similar conditions, enables providers to recognise subtle disease patterns, anticipate complications, and develop innovative interventions, like voice banking, that require deep condition understanding. This suggests that Australia's childhood dementia care should bring families to specialist centres in each state where multidisciplinary teams see sufficient patient volumes annually, rather than the current model where children see individual consultants in isolation. The expertise gained through concentration benefits all families through improved clinical knowledge, research advancement, and innovation development that would be impossible with fragmented care delivery.

Family-Driven Change Creates Inequities and Unsustainable Burden: Patient organisation funding and advocacy as the driver of change creates inequity both across diseases and geographically. Organisations with greater capacity and fundraising capability establish world-class services for their specific conditions, whilst other families navigate fragmented systems without specialised support. The lack of proactive state or federal policy means that responsibility for driving change is once again placed on those impacted by these diseases. Julia Vitarello's story demonstrates this pattern: her extraordinary efforts created immediate clinical infrastructure whilst championing fundamental regulatory transformation. This raises sustainability challenges for funding and exemplifies the precariousness of innovation dependent on individual advocacy rather than systematic service planning. For Australia, CDI's collective approach represents an opportunity to address these inequities through policy and investment supporting all conditions, rather than depending on extraordinary individual family efforts.

Emerging Neuropalliative Care Field Responds to the Childhood Dementia Experience: Paediatric palliative care, a relatively young field, was borne out of the oncology space. The emerging field of neuropalliative care represents important progress, creating specific research and approaches to progressive neurological conditions, now one of the highest burdens of disease eligible for paediatric palliative care. Dr Lauren Treat at the University of Colorado, a founding member of the International Neuropalliative Care Society who practices in both general neurology and paediatric palliative care clinics, emphasised the critical need to educate neurologists in quality-of-life conversations alongside therapeutic discussions. As treatments for childhood dementia emerge—likely as iterative improvements rather than cures, like the 40-year journey that brought childhood leukaemia survival rates to current levels—families will need support navigating decisions about what life extension means for their child's quality of life. Stanford's neuropalliative care research, particularly Dr Grant Lin's work on neuro-prognostication, represents innovative thinking about family communication that will result in significant practice improvements for families impacted by childhood dementia. In childhood dementia, the timing of palliative care introduction, the nature of anticipatory grief, and the uncertainty of disease progression differ substantially from oncology contexts. Australia should invest in neuropalliative care research and specialist practice to ensure childhood dementia families receive appropriate support throughout their journey. Dr Treat's visit to Australia in 2025 represents an opportunity to advance this field domestically.

CONCLUSION

Across four distinctly different healthcare systems, the fundamental finding is clear: no country has developed comprehensive care models for childhood dementia. Care for these children must extend far beyond health services alone; integrated approaches spanning health, social care, disability support, education, and psychosocial support for families are required. This reality creates coordination challenges that every country struggles to address. Whilst some have developed models that better manage cross-sector coordination, others rely on fragmented, single-sector approaches. However, this universal challenge positions Australia uniquely for proactive transformation. Through Childhood Dementia Initiative (CDI), Australia can design comprehensive systems from the outset rather than make reactive improvements within single sectors.

The most compelling evidence demonstrates that concentrated expertise transforms both care quality and innovation capacity. The neurodegenerative clinic in the USA, Frambu's condition-specific residential courses in Norway, and the UK's disease-specific clinics all reveal that genuine expertise develops through repeated exposure rather than occasional encounters. This concentrated exposure creates professionals with deep practice wisdom who develop intuitive understanding of disease trajectories and effective interventions. These expert centres become innovation hubs enabling research impossible in fragmented settings, whilst developing sophisticated multidisciplinary teams that can integrate specialised considerations including neuropalliative care, rehabilitation medicine, and complex care coordination. When combined with systematic knowledge transfer to local teams, concentrated expertise extends benefits whilst maintaining geographic accessibility. These expert centres also position healthcare systems for emerging therapeutic developments, as concentrated patient cohorts and sophisticated infrastructure enable clinical trials and precision medicine approaches that fragmented care cannot support.

Care coordination emerged as critical at two distinct levels. Within health systems, families navigating multiple unconnected specialists receive conflicting advice and undergo repeated assessments, highlighting the need for coordinated multidisciplinary approaches that prioritise relationship-building and systematic information sharing. Beyond health services, the gap between health, disability, education, and social support systems creates even greater challenges. Families become default coordinators across systems that operate independently with different eligibility criteria, funding mechanisms, and philosophical approaches. Successful models demonstrate that both types of coordination require deliberate design rather than expecting families to bridge disconnected services.

Peer support consistently emerged as essential intervention across all countries. Families identified connecting with others facing similar journeys as the most valuable support they received, regardless of healthcare system sophistication. These connections provide irreplaceable benefits: practical knowledge from lived experience, emotional support from shared understanding, reduced isolation, and collective advocacy strength. Yet healthcare systems consistently undervalue peer connection, treating it as supplementary rather than essential to comprehensive care.

All four countries struggle with balancing geographic equity against expertise concentration. Even in the UK's compact geography, families experience a postcode lottery in accessing care. Norway's residential courses and the USA's hub-and-spoke approaches offer promising hybrid solutions through periodic intensive contact combined with local team support, yet these still place significant burden on families. This tension between expertise concentration and geographic accessibility requires thoughtful approaches that don't simply disperse expertise thinly across many sites, whilst acknowledging the real challenges families face in accessing distant specialist centres.

Formal partnerships between charitable organisations and health systems, particularly evident in the UK's integrated model, demonstrate how these traditionally separate sectors can work together to enhance family support and service innovation. For Australia, where health services and charitable organisations often operate in isolation, these international examples reveal significant opportunities for resource leverage, innovative delivery, and sustainable funding diversification when partnerships are formalised rather than remaining informal.

Even the most successful innovative programs face persistent funding sustainability challenges. The USA's Medicaid uncertainties, the UK's charity dependency, Norway's political funding cycles, and Canada's provincial variations all highlight the precarious nature of childhood dementia services regardless of broader healthcare system design. Sustainable innovation requires systematic funding approaches rather than dependence on philanthropic support or political goodwill.

However, the most critical finding transcends any single innovation or service model: across all four countries, parent advocacy drives system change whilst creating unsustainable burden. The most innovative childhood dementia services owe their existence to parents who transformed personal tragedy into systematic advocacy—Julia Vitarello's neurodegenerative clinic in the USA, Jayne Hughes' Cockayne Syndrome clinic in the UK, and similar stories in Norway and Canada. These parent advocates demonstrate remarkable capacity to create new services, influence regulatory frameworks, and establish support networks benefiting entire communities. Yet this model places an extraordinary burden on families already managing devastating diagnoses and complex care whilst providing no systematic recognition or support for their advocacy contributions. The success of parent-driven innovation highlights both human resilience and systemic failure. Sustainable childhood dementia care cannot depend on exceptional individual efforts to identify and address service gaps that governments and health systems should proactively address.

Australia's opportunity lies in learning from global successes whilst deliberately avoiding documented pitfalls. The convergence of CDI, Australia's healthcare innovation capacity, and international best practice evidence creates unprecedented opportunity for comprehensive transformation rather than incremental improvement. Most importantly, Australia can design services serving all childhood dementia conditions equitably rather than depending on individual patient organisation capacity or extraordinary family advocacy to drive necessary improvements. Australia can also position itself for emerging therapies by building the clinical expertise and coordinated infrastructure needed to deliver precision medicine and participate in international trials as treatments develop. This represents fundamental transformation toward proactive, government-led systemic change that breaks the pattern evident across all countries visited. The following recommendations translate these international learnings into specific, actionable steps for the Australian context, prioritising approaches that address the full spectrum of identified needs, whilst ensuring sustainability and equity across all childhood dementia conditions.

> "The most compelling evidence demonstrates that concentrated expertise transforms both care quality and innovation capacity."
> - Gail Hilton

RECOMMENDATIONS

RECOMMENDATION 1:
Establish a National Approach

Australia should establish a national approach to childhood dementia, supported by government policy and sustainable funding. This represents a unique opportunity: no country studied has implemented a comprehensive national approach to childhood dementia. All struggle with geographic inequity and condition-based disparities, where care availability depends on location and whether individual patient organisations exist for specific conditions.

A national approach would enable coordinated engagement across Australia's health, disability, education and community systems, ensuring these sectors work together rather than operating in isolation. It represents a proactive, government-led systemic response rather than dependence on individual family advocacy to drive improvements. The childhood dementia grouping, established in Australia over recent years, should be formally embedded in health, disability, and education systems and policies.

1.1 National Lived Experience Voice

Central to the national approach must be a collective national voice of lived experience. Across all countries studied, a consistent gap exists between what professionals believe they provide and what families experience. Healthcare professionals may believe they offer coordinated care, while families describe isolation and fragmentation. Services may consider themselves family-centred, while parents report fighting for every form of support.

CDI should enable a structured national lived experience voice, ensuring families inform policy development, service design, and system improvements at every level—nationally and across jurisdictions. This ensures progress is guided by those who understand these conditions first-hand, without placing unsustainable burden on individual families to drive change through personal advocacy.

RECOMMENDATION 2:
Develop a National Cross-Sectoral Model of Care

Childhood dementia care extends far beyond health services alone. Families navigate multiple disconnected systems—health, disability, education, and community services—with no coordination and no shared information. They become unwilling care coordinators, disease experts and case managers while caring for their seriously ill children. Healthcare professionals may not recognise this burden, believing they provide comprehensive care, when in reality families experience only fragments of what they need.

A national model of care for childhood dementia must integrate these systems. The model of care should:

- **Be interdisciplinary in approach**, bringing together multiple professional perspectives rather than isolated specialists providing conflicting advice.
- **Address the full disease trajectory** from diagnosis through to end-of-life and bereavement, recognising that families need support at every stage—not just during acute medical crises.
- **Create space for innovation and emerging best practice**. Examples from the USA's integrated neuropalliative care approaches and Norway's early intervention programs demonstrate how concentrated expertise enables clinical innovation that fragmented systems cannot support.
- **Be co-designed with families and state and territory delivery systems** to ensure it is both practically implementable and genuinely responsive to the needs of those it serves. Co-design with families is essential to bridge the gap between professional assumptions and lived reality.

The inclusion of community services as a formal component of the model of care represents a particularly novel element for Australia. International examples, particularly from the UK, demonstrate the significant value community organisations bring in providing peer support, accessible information, navigation support, and family-centred coordination—elements that families consistently identify as essential. Families universally identified peer support as the most valuable support they received, yet healthcare systems typically undervalue it as supplementary rather than essential to comprehensive care.

RECOMMENDATION 3:
Establish Childhood Dementia Initiative as the National Community Service Provider

3.1 Core Service Provision

Families across all countries studied consistently identified peer connection as the most valuable support they received—often more valuable than medical interventions. Parents described peer support as "the only place I felt understood" and "more helpful than any appointment." Yet in Australia, accessing this support depends on whether a child's specific condition has a dedicated patient organisation. Some families find vibrant communities; others find nothing.

CDI should be formally recognised and resourced as the national community service provider for childhood dementia, ensuring all families have access to comprehensive community support regardless of their child's specific diagnosis or geographic location.

This national approach ensures equity across all childhood dementia conditions, particularly benefiting families whose children have conditions without dedicated disease-specific patient organisations. Service design and delivery should be continuously informed by family feedback, recognising that lived experience reveals gaps and challenges that professionals may not perceive.

Operating as a 'one-stop shop,' CDI should provide:

- **High-quality, accessible information and education** about childhood dementia, addressing families' current need to become their own researchers when professionals report limited knowledge of these conditions.
- **Peer support programs** connecting families facing similar journeys, recognising this as essential intervention rather than optional extra.
- **Navigation support to help families** access and coordinate care across disconnected systems, addressing the reality that families describe themselves as "project managers" of their child's care.
- **Whole-of-family support** recognising the profound impact on siblings and parents that healthcare systems often overlook in their focus on the diagnosed child or children.
- **Support through critical transition periods** when families report feeling most isolated and abandoned by services.

3.2 Innovative Care Coordination Role

Currently, parents spend significant time as default care coordinators, repeatedly explaining their child's condition to disconnected professionals across health, disability, and education systems. Families describe this as exhausting and demoralising. Healthcare and disability professionals may not realise the extent of this burden, believing families have adequate support when families in fact experience a severe lack of support and profound isolation.

Australia should investigate and pilot an innovative model positioning CDI as convenor of integrated care coordination around individual families. Drawing on the UK's 'Child In Need' approach, this would involve CDI bringing together education, disability, and health stakeholders responsible for a child's care to:

- Share information about the child's current status and evolving needs—information families currently repeat separately to each professional.
- Coordinate planning across sectors with the family's priorities central to decision-making.
- Reduce the coordination burden on parents who should be caring for their children, not managing professional communication.

This pilot would test whether community organisation-led coordination can effectively reduce fragmentation and family burden while improving cross-sector integration. Design and evaluation should centre on family experience, measuring success by reduced burden and improved coordination as families perceive it, not as systems measure it.

3.3 Lived Experience Leadership

The most innovative childhood dementia services internationally owe their existence to parents who transformed personal tragedy into systematic advocacy. This pattern demonstrates both human resilience and systemic failure. Progress cannot continue to depend on extraordinary individual efforts by families already managing devastating diagnoses and complex care.

As the national community service provider, CDI is positioned to facilitate meaningful lived experience participation in research, policy development, and across consumer advisory and advocacy bodies. This includes providing support, training, and infrastructure to enable families to contribute their expertise without unsustainable personal burden. People with lived experience should have genuine influence over decisions, not tokenistic consultation after directions are set.

RECOMMENDATION 4:
Build Concentrated Specialist Expertise Across Systems

Concentrated expertise transforms care quality and innovation capacity. International evidence demonstrates that professionals need to see sufficient numbers of children with childhood dementia to develop deep practice wisdom. Occasional encounters with isolated cases cannot build the expertise these complex conditions demand.

Families consistently reported that professionals who had seen multiple children with similar conditions provided dramatically better care—they anticipated needs, offered proactive solutions, and understood disease trajectories. In contrast, families seeing professionals encountering a condition that causes childhood dementia for the first time described having to educate their clinicians, receiving generic advice inappropriate for progressive conditions, and felt their child's unique needs were invisible.

Australia should strategically develop concentrated childhood dementia expertise across health, disability, education, and community service systems.

4.1 Health System Specialist Approaches

State-based multidisciplinary childhood dementia clinics should be established, enabling health professionals to see sufficient numbers of children (ideally 20+, as demonstrated by effective models in the USA and Norway) to build genuine expertise. Families who accessed specialist clinics internationally described them as "life-changing," finally meeting professionals who "got it" without lengthy explanations.

These clinics should:

- **Provide coordinated multidisciplinary assessment and ongoing care,** ending the pattern families describe of seeing multiple unconnected specialists who provide conflicting advice and require repeated explanations of their child's history.
- **Enable research and data collection**, impossible in fragmented settings.
- **Incorporate specialised elements** including neuropalliative care, rehabilitation, and complex care coordination—expertise families report is critically lacking in current Australian care.
- **Position Australia to participate in emerging clinical trials** and deliver precision medicine as therapies develop. The USA's neurodegenerative clinic model demonstrates how concentrated patient cohorts and sophisticated infrastructure enable participation in trials and compassionate use programs impossible in fragmented care settings.

Families are already travelling internationally to access trials and specialists—Australia should provide this expertise domestically. Clinic design and service delivery should be co-designed with families to ensure models genuinely meet their needs rather than professional assumptions about what families need.

4.2 National Disability Insurance Agency (NDIA) and Disability System Specialist Approaches

Children with childhood dementia have complex, progressive conditions that don't fit standard NDIA ways of working. Families report challenges across the entire disability system—not just with planning, but in finding service providers who understand progressive conditions, support workers who know how to adapt as children lose skills, and therapists who can provide appropriate interventions for neurodegenerative conditions.

Parents describe feeling they must repeatedly 'prove' their child's deterioration at each planning meeting—a traumatic requirement that fails to recognise the progressive nature of these conditions. They report difficulty finding service providers willing to take on complex care needs, support workers who understand the day-to-day needs of a child losing abilities, and therapists trained in neuroprogressive approaches rather than developmental delay models.

Specialist childhood dementia expertise is needed within the NDIA and across the broader disability sector:

Within the NDIA:

- Specialist Planners with deep understanding of childhood dementia trajectories and support needs.
- Specialist pathways for progressive conditions that don't require families to re-justify needs at each plan review.
- Decision-makers and reviewers who understand the evidence base for childhood dementia supports.

Across the disability service sector:

- Specialist Support Coordinators equipped to support children with complex, progressive conditions.
- Support workers trained in childhood dementia care, including understanding of disease progression, palliative approaches, and family grief.
- Therapists (e.g., occupational therapy, physiotherapy, speech pathology) with expertise in neuroprogressive conditions and palliative and rehabilitation approaches.
- Equipment providers who understand evolving needs as conditions progress.

This concentrated expertise would enable more appropriate planning, support coordination, and service delivery based on understanding of disease progression rather than requiring families to educate every professional they encounter or fight for each support as their child deteriorates.

4.3 Education System Expertise Development

Similar expertise development is needed within education systems to support children with childhood dementia and their siblings. School is an essential connection to community and peers for all children. Families report that schools often have no experience with progressive conditions, leading to inappropriate expectations, inadequate support, and siblings whose grief and trauma go unrecognised in school settings. Families report extra layers of isolation and lack of support through education transitions and are required to become experts in their child's education too.

The mechanisms for building this expertise should be co-designed with education stakeholders and families, recognising that approaches may differ from health and disability models while maintaining the core principle: concentrated exposure enables genuine expertise. Teachers and school support staff who have supported multiple children with progressive conditions develop invaluable understanding that benefits all subsequent families.

4.4 Community Service Expertise

CDI should lead and deliver concentrated expertise in community services, building specialised knowledge in supporting families affected by childhood dementia. CDI should act not only as a service provider but as an expert knowledge holder, innovation driver, and capacity builder for the community sector's response to childhood dementia.

Importantly, CDI's expertise must be built from and continuously informed by lived experience, ensuring community services remain responsive to what families actually need rather than what professionals assume they need.

4.5 National Equity

Geographic location and specific diagnosis should not determine care quality. Families in rural, remote and regional areas report significant challenges accessing care, both in Australia and internationally. Families whose children have less common conditions report being told "we've never seen this before" at every appointment, while those with slightly more common conditions may occasionally access disease-specific clinics (Rett Syndrome clinics are available in some areas in Australia, for example).

To prevent this 'postcode lottery' and diagnosis-based inequity, these specialist approaches should be embedded within national frameworks. National coordination ensures consistent quality and equitable access while allowing appropriate local and state-based delivery. Success should be measured by whether families—regardless of location or diagnosis—report receiving knowledgeable, coordinated, and compassionate care in a timely manner

CHURCHILL FELLOWSHIP: FOUR KEY RECOMMENDATIONS

International learnings for Childhood Dementia care in Australia

1

NATIONAL APPROACH

Government-supported policy and funding ensuring equity across all conditions and locations

2

CROSS-SECTORAL MODEL OF CARE

Integrates health, disability, education and community services. Co-designed with families

3

CDI AS NATIONAL SERVICE PROVIDER

'One-stop shop' for information, peer support, navigation and coordination. Led by lived experience

4

CONCENTRATED SPECIALIST EXPERTISE

Build expertise across health, NDIS, education and community services through concentrated exposure

CORE PRINCIPLES

- **LIVED EXPERIENCE LEADERSHIP** — Families inform all progress without unsustainable burden
- **CO-DESIGN** — With families and delivery systems at every stage
- **NATIONAL EQUITY** — No 'postcode lottery' based on location or diagnosis
- **FULL DISEASE TRAJECTORY** — Support from diagnosis through end-of-life and bereavement
- **INNOVATION AND EMERGING PRACTICE** — Neuropalliative care, early intervention, clinical trials
- **CROSS-SECTOR INTEGRATION** — Systems working together around families, not in silos

AUSTRALIA'S UNIQUE OPPORTUNITY:

No country has implemented a comprehensive national approach to childhood dementia. We can be first—learning from international best practice while avoiding their pitfalls.

DISSEMINATION

Implementing comprehensive childhood dementia care will require engagement across multiple stakeholders. To explore how the findings from this report might inform implementation plans the following steps will be undertaken:

- **Communication**
- **Integration with Key Strategic Projects**

Communication

Findings from this report will be disseminated to:

- Families with lived experience across all childhood dementia conditions (essential partners in all design and implementation decisions).
- Federal Minister for Health, Disability and Ageing.
- Key contacts at the Department of Health, Disability and Ageing.
- NDIS leaders.
- Health, disability, education and community professionals.
- Major children's hospitals and paediatric networks.
- Patient organisations and advocacy groups.

Communication will be via:

- Conference presentations (including but not limited to: National Lysosomal Storage Disorder Conference, Sydney (2025); International NCL Conference, Queensland (2025); International Dementia Conference, Sydney 2026 (abstract submitted); Alzheimer Disease International Conference, France 2026 (abstract submitted).
- Opportunities for published thought leadership pieces.
- Presentations for rare disease bodies and patient organisations.
- Webinar for CDI connections including families.
- Policy briefings for government representatives.

Integration with Key Strategic Projects

The timing of this Churchill Fellowship creates unprecedented opportunities for integrating international insights into Australian policy and service development. Fellowship findings provide crucial international evidence to strengthen the National Childhood Dementia Unit (NCDU) business case, which proposes a AU$12.64 million investment to establish Australia's first virtual centre of expertise for childhood dementia. The concentrated expertise observed in Norway's Frambu Centre, Colorado's neurodegenerative clinic, and UK's integrated services validates the NCDU's virtual centre approach. International clinical models and community care will directly inform the proposed Childhood Dementia Model of Care and National Reference Centre.

Fellowship insights are being integrated into CDI's 5-year strategic plan launched in November 2025. The international evidence on peer support as essential intervention, professional education needs, and cross-sector coordination challenges directly informs CDI's strategic direction and service development priorities. Current additional research into Australian multidisciplinary clinics, Rare Care Centre, and Rett clinic models over a 3-month timeline will complement international findings for strategic planning. The integration of health and NDIS into one ministerial portfolio creates an unprecedented opportunity for implementing coordinated approaches based on international evidence. These Fellowship findings on cross-system coordination can inform improved integrated service delivery. This critical policy window positions childhood dementia care to benefit from enhanced cross-sector collaboration, with international examples demonstrating how coordinated approaches can eliminate the fragmentation that families consistently identify as their greatest challenge.

It is important to remember, that even though these findings provide deep insights into what is possible, next steps must centre on the voices of lived experience here in Australia. As we progress, families will be engaged every step of the way. Parents will be invited to hear about these findings, to discuss what elements might work here from their lived experience, and we will co-design solutions to improve care for the future.

This Churchill Fellowship has highlighted that all around the world it is parents who are leading change for childhood dementia. Imagine what we can do if we combine the power of family advocacy with policy change, appropriate levels of investment and a professional, national organisation charged with leading this change.

The future starts now.

GLOSSARY

ACORNS - Children's hospice organisation in the UK providing palliative care services

Alex TLC - Alex, The Leukodystrophy Charity - UK patient organisation supporting families affected by leukodystrophies

Amy and Friends - UK charity supporting families affected by Cockayne Syndrome and related conditions

BDFA - Batten Disease Family Association - UK patient organisation

BPA - Personal assistance system in Norway providing carer support hours

CDI - Childhood Dementia Initiative - Australia's national organisation addressing childhood dementia collectively

Child In Need - UK legislative framework under Section 17 of the Children Act 1989 providing structured multi-agency support coordination

CLN2 - Neuronal Ceroid Lipofuscinosis Type 2, a form of Batten disease

Complex Care - Comprehensive care coordination model for children with medical complexity, providing integrated support across multiple specialties

ERN-NMD - European Reference Network for Neuromuscular Diseases

Frambu - Frambu Resource Centre for Rare Disorders in Norway, providing specialised support for families affected by rare conditions

IWMD - Inherited White Matter Disorders - a group of conditions affecting brain white matter

LSD - Lysosomal Storage Disorder

MD Clinic - Multidisciplinary clinic providing coordinated care across multiple specialties

MPS - Mucopolysaccharidoses -a group of inherited metabolic disorders

NCL - Neuronal Ceroid Lipofuscinosis - a group of inherited neurodegenerative disorders also known as Batten disease

NCDU - National Childhood Dementia Unit - proposed Australian virtual centre of expertise

NDIA - National Disability Insurance Agency - the department that delivers the NDIS

NDIS - National Disability Insurance Scheme - Australia's disability support system

NHS - National Health Service - UK's public healthcare system

NPC - Niemann-Pick Type C - an inherited metabolic disorder affecting cellular cholesterol transport

NPUK - Niemann-Pick UK - the organisation representing all Niemann-Pick diseases

Neuropalliative Care - Specialised palliative care approach for progressive neurological conditions, distinct from traditional oncology-based models

PACT - Paediatric Advanced Care Team at SickKids Hospital, Toronto

Precision Medicine - Medical approach tailoring treatment to individual genetic characteristics

SickKids - Hospital for Sick Children in Toronto, Canada

SIBS - Evidence-based program addressing sibling support needs in rare disease contexts

SKIP - Solutions for Kids in Pain - Canadian national pain management implementation program

SynGAP - Gene associated with intellectual disability and autism spectrum disorder

TTD - Trichothiodystrophy, a rare genetic condition related to Cockayne Syndrome

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Amy and Friends - Cockayne Syndrome support and clinic information.
Available at: https://www.amyandfriends.org

Dementia UK - Admiral Nurse Program information and service delivery models.
Available at: https://www.dementiauk.org

Frambu Resource Centre for Rare Disorders - Service delivery models and research publications.
Available at: https://frambu.no/en

SKIP (Solutions for Kids in Pain) - Implementation resources and health standards.
Available at: https://kidsinpain.ca

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Personal Communications and Interviews

Interviews and conversations conducted during Churchill Fellowship travel (June-August 2025) with healthcare professionals, families with lived experience, and patient organisation leaders across Norway, United Kingdom, Canada, and United States of America. Specific individuals and organisations are acknowledged throughout the report text with appropriate consent and context.

Note on Sources

This report prioritises direct observation, interviews, and firsthand experience gathered during the Churchill Fellowship travel period. References are provided for quoted material, organisational information, and policy documents that informed the analysis. Personal experiences and reflections from fellowship activities do not require citations, consistent with Churchill Fellowship reporting guidelines that emphasise learning through direct engagement rather than literature review.