Chapter 4

Paediatric and youth cancers

4.1 This chapter examines low survival rate (LSR) cancers that affect children and young people.

4.2 Cancer Australia defines a child 'as a person aged less than 15 years', and provides the following information about cancers in this age group:

> The types of cancers that occur in children, and the way they respond to treatment, can be different from cancers that occur in adults. They can also be different from the types of cancers that occur in adolescents and young adults (aged 15–29 years) – there are often specific protocols and guidelines for the management of adolescents and young adults with cancer, which bridge the gap between children's cancers and adult cancers.[1]

4.3 However, Mr Peter Orchard of CanTeen Australia explained that the definition of a child varies across jurisdictions:

> In [Western Australia] there is a hard line drawn that will come into play in the next few months—when a young person turns 16, they are then directed to the adult setting even if they have been treated in the paediatric setting. In Victoria, with the Royal Children's Hospital, there is more flexibility; they will go up to 18. So there are just two examples of the extremes.[2]

4.4 Cancer Australia also provided an explanation of why cancer occurs in children:

> In most cases, we don't know why children get cancer. Children are too young to have the same risk factors that affect adults (e.g. environmental exposures, lifestyle, infections). Tumours occasionally develop as a result of a genetic error made in children's growing bodies.
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> …
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> In children, age is not a risk factor for cancer, but the incidence of some cancers varies with age. Some childhood cancers tend to appear in very young children and others in older children. Family history is also important because a few childhood cancers run in families.[3]

4.5 The following sections examine the most common LSR cancers in this group, the unique issues and challenges faced by this group of people with LSR cancer and the difficulties with transitioning from paediatric to adult treatment and care. Prior to

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1 Cancer Australia, *About children's cancer*, https://childrenscancer.canceraustralia.gov.au/about-childrens-cancer/what-childrens-cancer (accessed 4 October 2017).

2 Mr Peter Orchard, Chief Executive Officer, CanTeen Australia, *Committee Hansard*, 19 May 2017, p. 3.

3 Cancer Australia, *About children's cancer*, https://childrenscancer.canceraustralia.gov.au/about-childrens-cancer/what-childrens-cancer (accessed 4 October 2017).

a discussion of these issues, the section below considers the personal impact of childhood and youth cancers.

The personal impact of childhood and youth cancers

4.6 Many parents, relatives and friends of children and young people who have suffered from or are currently diagnosed with cancer shared their experiences with the committee. Childhood and youth cancer have a devastating effect on the child or young person with cancer, their family and their community:

> The impact of a child dying is pervasive. It is not just the adults who cannot rationalise the injustice of it; it is also the children—siblings, cousins, and friends. They are all suddenly faced with their own mortality because something they rationalise as being for the elderly has happened to one of their peers. While we as adults continue to grieve, so do the children—nightmares, bedwetting, anxiety, and withdrawal. It goes against nature. Parents are not supposed to outlive their children. Children are not supposed to be diagnosed with diseases devoid of survival rates. We should be able to reassure children that doctors can help them, not have them living in fear that if they were to get brain cancer they would end up like Tom and the 34 other Australian children who die from it each year.[4]

> As a parent of a child who has been diagnosed with brain cancer – words can be hard to muster to describe how this has impacted our family. It is devastating. It is all consuming. It is heartbreaking.
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> …
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> Brain cancer seems to offer one blow after another. We don't make plans. The plans we do make we often cancel. Life becomes a circle around appointment times and there is not much left in the way of finances or energy for normal social life.[5]

> We reside in country Victoria and, while I have spent time with Chloe while she has been in hospital in Melbourne, I have been on constant call to care for her brother and sister often without notice. I have had to try and find a way to calm their fears when their sister is so ill and they desperately want her and their mum and dad to come home. Not only have I had to watch my precious granddaughter in such pain and going through horrendous treatments as well as seeing the hurt and worry of her mother and siblings, I have had to watch helplessly as my younger son struggles through his emotional pain knowing there is nothing he can do to make his little girl better. This is heartbreaking for me. A parent is supposed to be able to protect their children from pain and hurt.[6]

4 Mr Simon Gray, *Committee Hansard*, 7 June 2017, p. 4.

5 Mrs Tracy Taylor, *Submission 52*, p. 1.

6 Ms Elizabeth Perry, *Submission 78*, p. 1.

> I cannot put into words the suffering our precious daughter Brooke endured, and now for my wife Olivia and I continue that suffering every second of every day. We celebrated Brooke being one of the lucky 1 in 5 survivors of Brain Cancer only to have her taken from us by this hideous disease 10 years later.[7]

> When I was 18, I was diagnosed with Gastro Intestinal Stromal Tumor [sic] (GIST) and was told by my disease had no cure and I was likely to have about one year left to live. There was no cure in 1996. There is still no known cure 21 years later. GIST is a rare cancer with low survival rates.[8]

> In September 2016 our 13 month old daughter, Isabella, was diagnosed with brain cancer. She has a grade 3 anaplastic ependymoma. It is an aggressive cancer; the most aggressive form of ependymoma. This insidious disease took over ¼ of our daughter's brain before she was diagnosed. Instead of our family watching our little girl transition from a baby to a toddler, witness her first wobbly steps, hear her learning to talk, we watched her literally fight for her life. Over the course of a week, the longest and most awful week of our lives, we stood by while Isabella endured 4 brain surgeries. We watched her suffer countless seizures, the last one requiring a MET call with staff from the ward, PICU and Emergency attending to assist to try to stabilise her. We watched as infection racked her body forcing her temperature up to 40 degrees. We watched as a ventilator breathed for her. We waited helplessly every time she was taken away to the operating theatre, not knowing if she would return to us. We listened to the neurosurgeon tell us that he had to abandon the surgery to debulk her tumour because of massive blood loss. We listened as he told us that they transfused the entire volume of blood in her body 3 times over before she was able to be stabilised. We cried when she finally woke up and said "mummy", "daddy" and "happy" (her 3 favourite words). We cried when we realised she was paralysed down her right side. We cried when we realised she could not swallow, could not eat, could not drink and could not sit up. We cried when she went mute several days after her fourth surgery. We cried a lot that week. We still cry a lot now.[9]

4.7 In addition to the emotional toll of these cancers, there are broader implications. For example, in respect of brain cancer, Love for Lachie submitted that:

> Most parents will be unable to work when their child is diagnosed with brain cancer as they need to care for their child fulltime throughout surgeries, radiation, chemotherapy and other treatments. Brain cancer is the undisputed most financially costly cancer. Parents can not work if they have

7 Mr Jonathan Karl Fretwell, *Submission 99*, p. 3.

8 Mrs Sarah McGoram, *Submission 159*, p. 1.

9 Ms Robin Berthelsen, *Submission 170*, p. 1.

> a child diagnosed; adults who are diagnosed can no longer work; treatment options that are not part of the gold standard treatment plan are incredibly expensive and for many people become completely financially prohibitive leaving them to accept their fate with standard ineffective treatment.[10]

4.8 Some of these broader effects of LSR cancers, such the loss of income, are discussed in chapter 5.

LSR cancers most commonly affecting children and young people

4.9 There are a range of LSR cancers that commonly affect children, for example, Cancer Australia identified the following cancers: leukaemia, brain and other central nervous system tumours, Hodgkin disease (Hodgkin lymphoma), non-Hodgkin lymphoma, neuroblastoma, soft tissue sarcoma, kidney tumours, melanoma, bone tumours, germ cell tumours, retinoblastoma and liver tumours.[11]

4.10 The committee heard from various submitters and witnesses that brain cancer kills more Australian children than any other disease,[12] and while 'the overall survival of some children with brain tumours has improved' in the paediatric setting, 'the groups of children with poor outcomes are becoming smaller, and therefore increasingly challenging to study'.[13]

4.11 The Australian and New Zealand Children's Haematology-Oncology Group (ANZCHOG) made a similar observation:

> Childhood cancer comprises less than 1% of the total number of new cancer diagnoses in Australia each year. This equates to more than 600 children diagnosed with cancer each year. The treatment of childhood cancer is one of the great success stories of modern medicine. Survival rates have increased from less than 30% in the 1960s to 80% in the 2000s for all childhood cancers combined. For Acute Lymphoblastic Leukaemia (ALL), the most common form of childhood cancer, the cure rate now approaches 90%. Despite these outstanding successes childhood cancer remains the leading cause of non-accidental death in children in Australia and many subtypes of childhood cancer continue to have a very poor prognosis. Unfortunately, the rate of improvement in survival for children with cancer has plateaued over the past decade.[14]

4.12 CanTeen Australia identified that cancer in adolescents and young adults (AYAs) 'has a distinct biology and responds differently to treatments that are

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10 Love for Lachie, *Submission 120*, p. 7.

11 Cancer Australia, *Types of children's cancers*, 23 August 2015, https://childrenscancer.canceraustralia.gov.au/types-childrens-cancers (accessed 4 October 2017).

12 See for example, Brain Cancer Discovery Collaborative, *Submission 60*, p. 1; Love for Lachie, *Submission 120*, p. 1; Children's Hospital Foundation, *Submission 274*, p. 2.

13 Children's Cancer Research Unit (CCRU), *Submission 88*, p. 4.

14 Australian and New Zealand Children's Haematology-Oncology Group (ANZCHOG), *Submission 237*, p. 2.

otherwise successful in paediatric or older adult populations'.[15] In respect of survival rates for AYAs, CanTeen Australia stated that:

> Although overall survival rates are good…at approximately 88%1, this masks poorer outcomes seen in several high lethality cancers for this age group. Five-year survival for cancers such as Acute Myeloid and Acute Lymphoblastic Leukaemias and Brain and Bone cancers are still exceptionally low at between 61.3% and 65.6% with Sarcoma only slightly higher at 76.7%, with others such as Rhabdomyosarcoma and Lung and Adrenocortical Carcinomas having 5 Year survival rates well below 40%, and Hepatic Carcinoma only 20.6%.[16]

Unique challenges and issues

4.13 The committee heard from a number of parents and professional organisations about the particular challenges and issues faced by children and young people with cancer.

4.14 For example, The Kids' Cancer Project stated that '[t]he challenges of new anti-cancer drug development for childhood cancers that are faced globally are exacerbated in Australia because of our relatively small population'.[17] These challenges generally arise because of 'the rare nature, smaller population, limited access to tumour samples, more limited bodies of research knowledge and therefore reduced funding opportunities'.[18]

4.15 The Kids' Cancer Project also noted that '[w]e have seen the improvement in prognosis of several [childhood] cancers that have had dedicated, focussed funding from the Federal government', but:[19]

> The rarity of several childhood cancers means that they are not covered by the burden of the population which the current National Health and Medical Research Council [(NHMRC)] funding model is based on.[20]

4.16 The Children's Cancer Research Unit also discussed challenges arising from the NHMRC funding model, asserting that:

> …characteristics of low survival rate cancers can make it more difficult for associated research grant proposals to be considered "well designed (or to have) a near flawless design". The fact that a particular cancer is characterised by poor survival rates can reflect a more limited research base, leading to less scientific knowledge. This can mean a greater need for more open-ended research grant applications seeking to (for example) identify treatment targets, or biomarkers of response. However, these more

15 CanTeen Australia, *Submission 128*, p. 3 (citations omitted).

16 CanTeen Australia, *Submission 128*, p. 2 (citations omitted).

17 The Kids' Cancer Project, *Submission 136*, p. 3.

18 The Kids' Cancer Project, *Submission 136*, p. 5.

19 The Kids' Cancer Project, *Submission 136*, p. 2.

20 The Kids' Cancer Project, *Submission 136*, p. 3.

> open-ended proposals can be viewed by grant review committees and reviewers as "fishing expeditions" that may be less likely to be considered to have "objectives that are well-defined, highly coherent and strongly developed (and be either) well designed (or have) a near flawless design". Similarly, low survival rate cancers may have fewer experimental models (cell lines, mouse and other animal models) available for study. It can also be challenging to access statistically informative and representative sample cohorts, or patient cohorts for clinical trials. Reduced resources for research could therefore also lead to reduced "scientific quality" and "significance and innovation" scores for NHMRC project grant applications, as well as negatively impacting the team's "track record".[21]

4.17 Indeed, clinical trials were identified by The Kids' Cancer Project as 'the single most important factor contributing to the dramatic improvements in survival rates for children with cancer over the past forty years'.[22]

4.18 In speaking of access to clinical trials for children, Dr Chris Fraser of ANZCHOG noted that:

> The fact that childhood cancer is relatively rare in one way assists our ability to conduct clinical trials because the care is very centralised. Essentially, all of these children are cared for in one of eight children's cancer centres around the country.[23]

4.19 However, ANZCHOG raised a number of obstacles to running clinical trials, including the expense of clinical trials, reluctance by pharmaceutical companies to run trials in Australia due to the small population size, and accessing targeted drugs.[24]

4.20 The importance of clinical trials focussed on children and young people was similarly emphasised by CanTeen Australia, which noted that AYAs face particular challenges:

> Compared to paediatric and older adult populations, AYAs have experienced relatively poorer survival gains and reductions in mortality, in part driven by poorer access to clinical trials. Embedding clinical research within standard paediatric care has been the single most important driver of the dramatic improvements in childhood cancer survival rates seen over the past 40 years. Compared with the approximately 45% of younger children with cancer in Australia who currently participate in potentially lifesaving clinical trials, AYA participation rates remain low at approximately 10%.
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> The rarity of some cancers which disproportionately impact this age group is another reason for the poorer improvements in length of survival and mortality. Despite improvements in the diagnosis and treatment of common cancers that have resulted in dramatic reductions in mortality, early

21 CCRU, *Submission 88*, p. 2.

22 The Kids' Cancer Project, *Submission 136*, p 3. See also ANZCHOG National Patient and Carer Advisory Group, *Submission 125*, p. 6.

23 Dr Chris Fraser, Chair, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 19.

24 ANZCHOG, *Submission 237*, pp 4–5.

> diagnosis programs for rare cancers have not improved over the last 20 years and diagnosis often remains slow, resulting in the cancer being diagnosed at a more advanced stage.
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> In addition, rare cancer treatments have not advanced at the same pace as those for common cancers and it is likely that many patients with rare cancers are receiving suboptimal care; hence a rare cancer diagnosis is often accompanied by a very poor prognosis. AYAs diagnosed with a rare cancer are significantly more likely to die from their disease, with these cancers being responsible for the majority of cancer-related deaths in this age group.[25]

4.21 Further, CanTeen Australia submitted that, in circumstances where people experience paediatric cancers in their 20s:

> …ideally they should be able to be part of a paediatric trial. We forget the fact that it is a paediatric trial; what we do remember is that it is a trial in this particular topic cancer. If they have got that type of cancer, they should be able to be part of it.[26]

4.22 The difficulty faced by young adults was also noted by ANZCHOG, which stated that the issue of eligibility for clinical trials for young people between the ages of 14 and 18 'is a bit of a grey area'.[27] Dr Fraser elaborated:

> Adolescents and young adults have some poorer outcomes in some types of cancers, and they are not enrolled as frequently on clinical trials. There is also a discrepancy sometimes between the treatment the same patient with the same sort of cancer might receive in a paediatric institution compared to in an adult institution. And there might be discrepancies between the treatment they might receive in a private adult institutions and a public institutions, for example.[28]

Transitioning to adult treatment

4.23 The committee heard that there are particular challenges faced by cancer patients who transition from paediatric to adult treatment and care. For example, CanTeen Australia informed the committee about the 'disruption to treatment' experienced by these patients:

> If they are having treatment and then at 16 they have to be bumped across to a new institution, a whole new team needs to pick them up at that point. In terms of research, it is that, by definition, they are still a child but they are not able to be part of a paediatric trial because they are considered to be too old for a paediatric setting. And the hard rule around paediatric trials is that they have to happen in a children's hospital that has been approved by [the Children's Oncology Group (COG)]. They have teams that go around

25 CanTeen Australia, *Submission 128*, pp 2–3 (citations omitted).

26 Mr Orchard, CanTeen Australia, *Committee Hansard*, 19 May 2017, p. 3.

27 Dr Fraser, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 22.

28 Dr Fraser, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 23.

> the world accrediting hospitals for COG trials, but they will not look at any hospital other than a paediatric hospital. So a 16- or 17-year-old will not be able to participate in the trial because they cannot attend a setting.[29]

4.24 This was also discussed by Professor David Walker:

> **CHAIR:** I understand there is a huge difference, if I can put it that way, in regard to the way children compared with adults get treated for exactly the same disease. So if you are moving from the paediatric area to the adult area it is quite often a bit of a shock. Do you find that?
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> **Prof. Walker:** There is no doubt about that. In fact, I think that is one of the reasons why the outcomes for children's cancers—for some cancers—have improved to some extent over the years. They get better coordinated care. Their care is centralised, by the way, so therefore a lot of the patients are either available for, or have access to, the latest trials. There is no doubt that there is a greater appetite for coordination of care and longitudinal care in the paediatric medical community compared to adults.
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> …
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> **Prof. Walker:** …even young adults, particularly those ones transitioning through: they find they are in between and they do not get either. They do not get the benefit of either.
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> **CHAIR:** I understand that when you move from being a paediatric patient to AYA you do not have the same team. Is that correct?
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> **Prof. Walker:** That is true for a lot of things. Kids who have long-term problems lose contact with the team that has been looking after them. Team care is far less applied in adult medicine compared with children's medicine, in a variety of fields. So, yes, it is really quite difficult when kids get older, whether it be brain cancer or other neurological problems like spina bifida and things like that—but we are getting off topic. But that is absolutely true. Absolutely true.[30]

4.25 Clinical Associate Professor Nicholas Gottardo of ANZCHOG also informed the committee that transitioning to adult treatment 'is a bit of an issue', which varies across states, but that:

> …in general, we would not be transitioning a patient during treatment. If we have taken a patient who is 16 or 17 under our care, we will complete the therapy that is prescribed for that particular patient. Then a transition model would be developed with a particular clinician or hospital, depending where that care was best served. So, generally, we would not be transitioning a patient [mid-treatment]. That may occasionally happen as a patient gets well beyond 18 years of age and potentially has a resistant

29 Mr Orchard, CanTeen Australia, *Committee Hansard*, 19 May 2017, p. 3.

30 Professor David Walker, *Committee Hansard*, 6 June 2107, p. 50.

> tumour that is not responding to the treatment that we have delivered up front.[31]

4.26 Clinical Associate Professor Gottardo identified the 'wider issue' for transitioning patients as:

> …having a pathway of coordinated care for a child or an adolescent—or even a child survivor of cancer—into the adult environment, where they are much more left to their own devices, as opposed to the more paternalistic paediatric model where we kind of take care of everything. That type of care can certainly be disjointed. We are now much more aware of this issue and we are setting up transition clinics et cetera to try and have a smoother transition between our service and the adult service.[32]

4.27 Clinical Associate Professor Gottardo acknowledged the evidence received by the committee that some children and young adults 'fall between the gaps', and although it is not a 'major problem' for children up to 16:

> …I think the 16- to 18-year-olds fall between the gaps. Often children's hospitals' business model is younger children, so there are often restrictions on being able to accept children between 16 and 18. Different states have different rules on it. It can also depend on whether the child, or the young adult, ever gets referred to a paediatric centre. Sometimes we just never find out about them, and we may have a clinical trial available.
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> Many of our clinical trials with the children's oncology group go into their early 20s—some of the sarcoma trials go into their 30s—and we would be able to enrol such patients in a trial. But the adult sector are not part of those oncology groups and therefore would not be able to and may or may not have access to trials. But the data certainly suggests that that is the group that falls between the gaps for enrolling in clinical trials. If they are admitted to a paediatric centre then there is no difference, but if they are admitted to an adult centre then they seem to have very low enrolment in an up-front clinical trial.[33]

4.28 Indeed, Mr Robert Perkins—whose son was 17 at the time he was diagnosed with a GBM malignant tumour and passed away at the age of 21—shared his experience that his son was too old for a children's hospital, and that '[t]here was little or no support for adolescents who are dealing with their own mortality in a hospital system that is mostly dealing with mature adults'.[34]

Committee view

4.29 The committee cannot adequately express its thanks to the individuals who shared their personal experiences of paediatric and youth cancer. The devastation of

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31 Clinical Associate Professor Nicholas Gottardo, Deputy Chair, ANZCHOG; and Chair, Central Nervous System Tumour Subcommittee, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 23.

32 Clinical Associate Professor Gottardo, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 23.

33 Clinical Associate Professor Gottardo, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 23.

34 Mr Robert Perkins, *Submission 184*, p. 1.

cancer is often compounded when a child or young person—who has barely commenced their life—is diagnosed. The committee wants to acknowledge the bravery and resilience of these children and young people, and their families, who in the face of great personal tragedy strive for knowledge and solutions not only for their own benefit but also in a quest to spare other families the same trauma.

4.30 Recommendations elsewhere in this report are applicable to the challenges facing children and young people with cancer; the committee hopes that action is taken so that all people with LSR cancers face improved prognoses in the future and that significant in-roads are made to improve the diagnosis and treatment of all LSR cancers. In particular, the committee hopes that greater financial support for innovative clinical trials, increased flexibility in clinical trial design and access, and improved ethical and governance approvals will see more research into LSR cancers affecting children and young people.

4.31 The committee is concerned about the transition from paediatric to adult oncology care where it appears, at least in some settings, that children are abruptly removed from paediatric oncology services and moved to adult oncology services.

4.32 The committee notes that this change from paediatric to adult oncology services is the responsibility of the state and territory health systems. The committee encourages the states and territories to consider their current arrangements for transitioning children and young people from paediatric to adult oncology services, and ensure that this occurs in a consistent and co-ordinated way that ensures continuity and quality of care in the best interests of each individual patient.

Recommendation 8

4.33 The committee recommends that, through the Council of Australian Governments Health Council, the Australian government leads a process to ensure that arrangements for transitioning children and young people from paediatric to adult oncology services occurs in a consistent and co-ordinated way that preserves continuity and quality of care in the best interests of each individual patient.