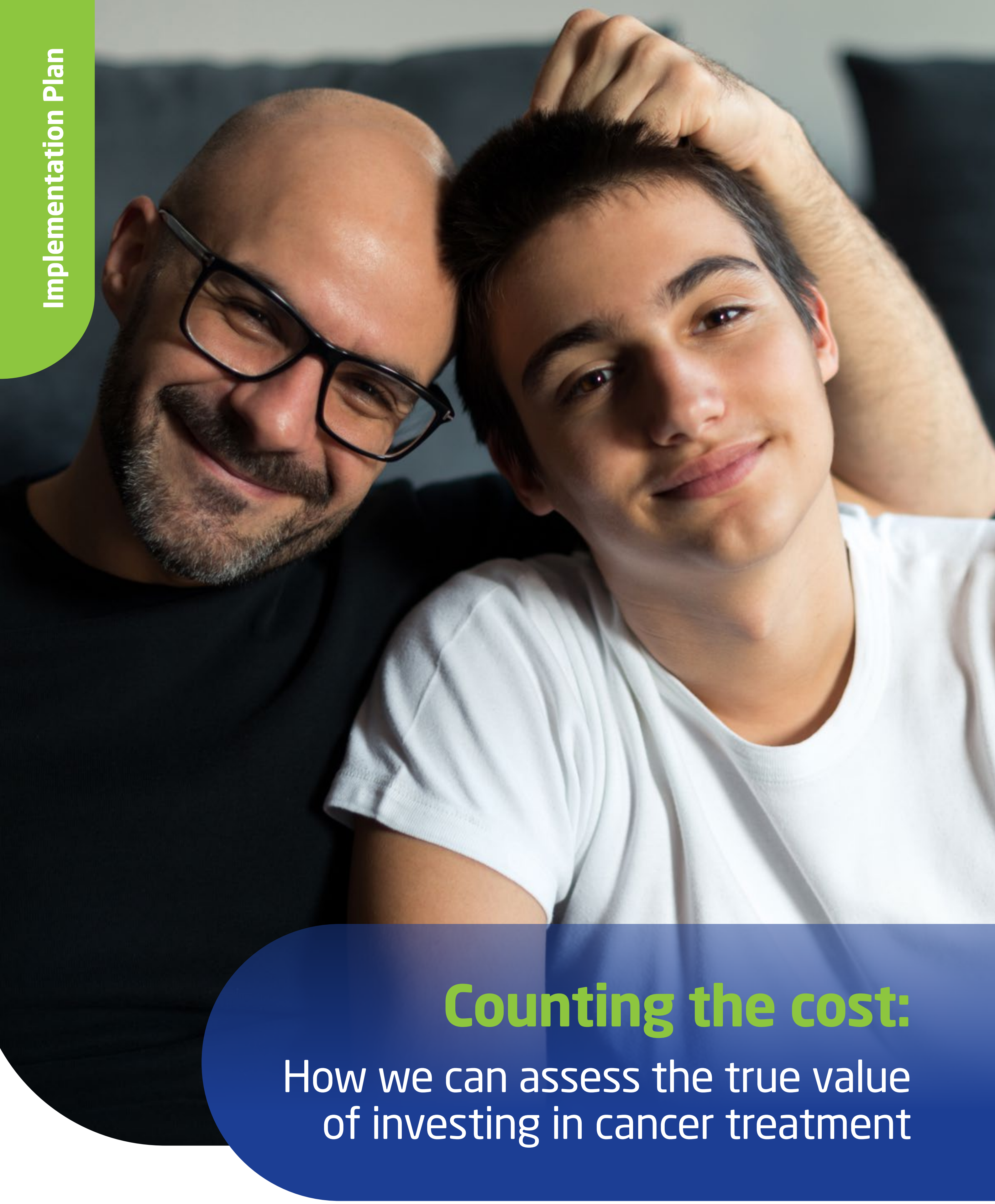
Implementation Plan



Counting the cost:

How we can assess the true value of investing in cancer treatment

HTANALYSTS

canteen

rare cancers AUSTRALIA

Acknowledgements

We would like to thank all the people who have generously contributed their time to the development of this report, especially the people living with cancer whose personal stories are included in this report, and who have helped guide our work on making our health technology assessment system more person-centred.

We would like to thank Matt Britland, from MedWise Consulting, for his work in drafting this report and Colman Taylor from **HT**ANALYSTS for his work in developing the social return on investment submission case studies.

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FOREWORD BY

Rare Cancers Australia

My wife, Kate, and I founded Rare Cancers Australia (RCA) in 2012 with the aim of improving awareness, support, and treatment of people with rare and less common cancers. One of our goals was to ensure the best treatments in the world were made available and affordable to the people who need them in Australia.

A few years later, in 2014, we launched our first crowd-funding campaign to pay for surgery for a young boy called James. Since then, we have crowd-funded almost $5million for more than 150 people, through our Patient Treatment Fund, to pay for cancer treatments that have been prescribed by their clinicians but remain unavailable through the Pharmaceutical Benefits Scheme (PBS).

The stories of two people, Lillian and Angus, from those early campaigns are included in this report. The treatments Lillian (who was 34 years old with a three year old daughter) and Angus (who was 10 years old) needed were not, at the time, available subsidised through the PBS for their rare cancers. But the success of their treatments which, in the absence of government subsidies, they both were forced to crowd-fund to pay for, is evident today. So how do we justify not funding lifesaving and life-extending treatments, when the impact can be so profound?

Even today, we are still having to crowd-fund to pay for treatments for many people living with rare and complex cancers, in part due to ongoing challenges of the Health Technology Assessment (HTA) processes. The scope of our HTA has been narrowed from its original intent to now concern itself only with the outcomes of a therapy, specifically clinical outcomes. What we are not currently measuring or considering is the cost of inaction, that is, not making the treatment available.

These consequences include but are not limited to societal impacts, for example, the impact to the lives of children orphaned. More broadly, there are significant ramifications across the immediate community of the person who dies from cancer. Similarly, the use of toxic outdated therapies brings crushing damage to people during treatment and severely impacts their economic and financial contribution to society, sometimes for the remainder of their lives.

These, and other, impacts of a disease are measurable and when considering the value of a treatment we need to consider more than just the direct clinical endpoints.

The World Health Organization defines HTA as follows:

> HTA refers to the systematic evaluation of properties, effects, and/or impacts of health technology. It is a multidisciplinary process to evaluate the social, economic, organisational and ethical issues of a health intervention or health technology. The main purpose of conducting an assessment is to inform policy decision-making.

This report shows us a pathway to consider and fully evaluate the broader value of a therapy, because the impacts of cutting-edge clinical management are not just clinical or financial. They are both personal and social and have serious ramifications, not just for the people receiving the treatments but for their families and the communities they live in.

Richard Vines
Co-Founder and CEO
Rare Cancers Australia

FOREWORD BY

Canteen

Canteen is the Australian not-for-profit that supports young people aged 12-25 impacted by cancer, whether their own diagnosis, a family member's cancer, or the death of a loved one. Our mission is to be in the corner of every young person when cancer crashes into their world.

The costs of rare cancer treatments have significant implications for young people, both those battling their own cancer, and those faced with the risk of losing a parent to cancer. Whilst investing in new therapies can have a high upfront cost, there are substantial downstream social and economic benefits.

From working directly with young people and families at Canteen I know the benefits of investing in innovative cancer treatments are so much greater than the narrow definition of benefit used by Australia's health technology assessment bodies. So, when Richard came to me with this idea about measuring social return on investment, I knew he was on to something.

Counting the cost, which we launched in 2022, demonstrated that the value of a life is far greater than the value determined by the parameters currently used to assess the cost of medicines and the value of treatment. The report demonstrated that the true value of investing in cancer treatments, results in a three dollar return in social and economic value to the Australian community for every dollar invested in life-extending cancer treatments.

By using social return on investment (SROI) methodology it is possible to put a monetary value on the benefits that flow to families, friends, communities, and governments when a person gains years of life. Calculating a monetary value for the things that people value most, such as a stable, loving family and celebrating life milestones together, means they can be measured and valued within existing health technology assessment frameworks.

The aim of this supplementary *Counting the cost* report is to demonstrate how to implement change so that we can achieve the benefits that SROI provides. Amendment of the current PBAC Guidelines to incentivise inclusion of SROI analysis in every application would provide a fairer assessment process that prioritises patient perspectives. This report includes a hypothetical example of an application to the PBAC that includes SROI analyses under amended PBAC guidelines. It also aims to provide guidance for decision-makers on how to incentivise inclusion of SROI and a framework for implementing this change.

> This report comes at a critical time as the Australian Government reviews outdated economic equations driving the Pharmaceutical Benefits Scheme in the current Health Technology Assessment Review. We anticipate a better future for young cancer patients and families with a health system committed to equity and accessibility in healthcare and measuring what matters most to people.

We are grateful to our colleagues at Rare Cancers Australia and **HT**ANALYSTS for their ongoing dedication to bringing this issue to light and highlighting the solutions that social return on investment analysis provides. We are united in our passion and commitment to getting better, fairer outcomes for all Australians impacted by cancer.

Peter Orchard
CEO
Canteen

About **HT**ANALYSTS

HTANALYSTS has been providing boutique impact measurement and communication services for 20 years. We exist to make a powerful impact on society by driving human-centric outcomes.

Our purpose is to have a powerful impact on the health of society by connecting people with the best treatments in the fastest amount of time.

Founded in 2002, **HT**ANALYSTS has grown to become a leader in healthcare and impact assessment consulting, providing services to the healthcare industry. In recent years, our scientific rigour has proven valuable for those outside the traditional pharmaceutical world, and this has seen us grow our capabilities to include expertise in social impact measurement, government services, health ageing and disability.

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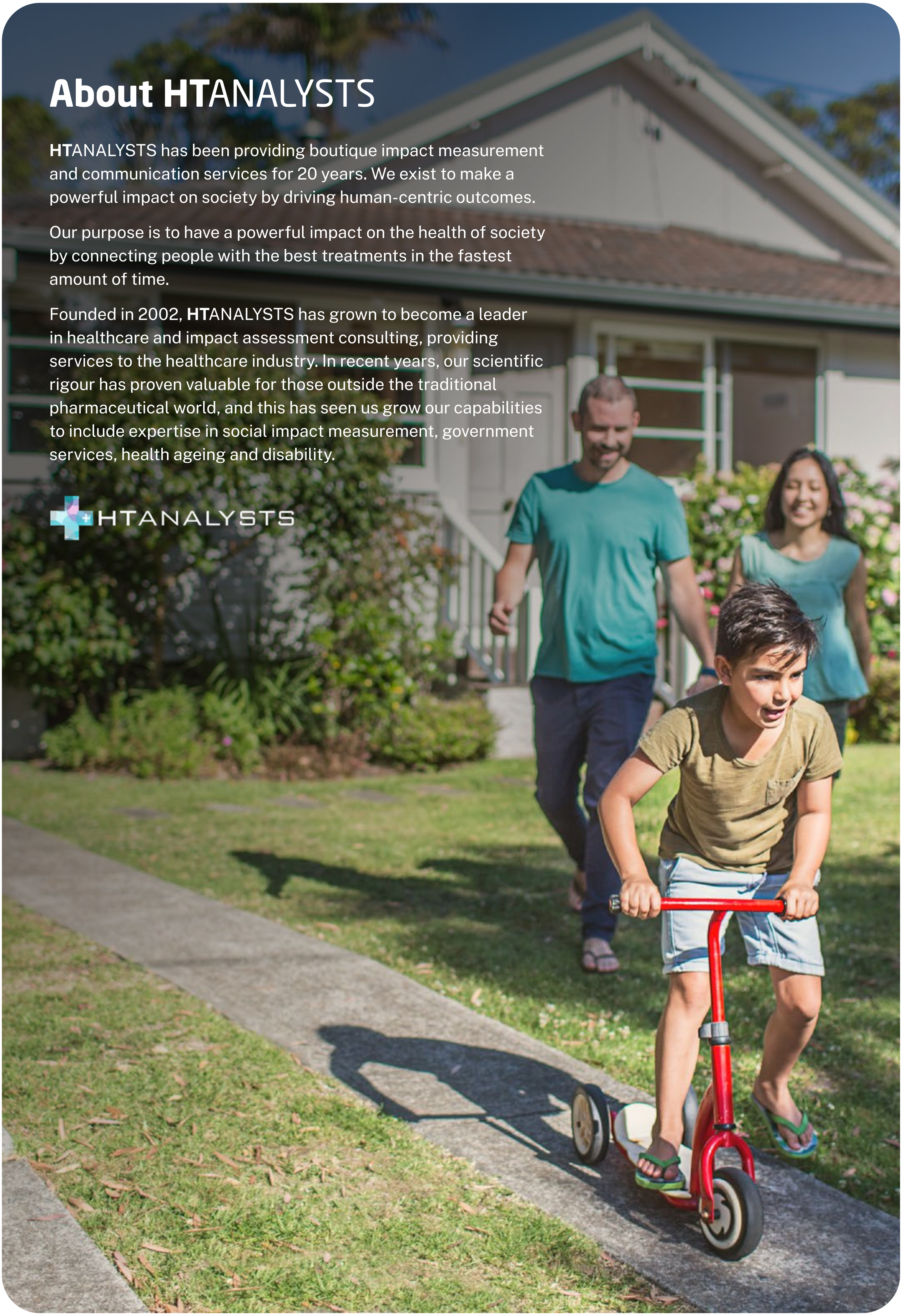


Executive summary

A cancer diagnosis has profound and enduring effects on individuals and extends its impact to loved ones, colleagues, communities, and across society. From children witnessing parental experiences with cancer, to parents caring for their children undergoing treatment, or facing the tragic loss of a loved one, the far-reaching personal consequences are clear to see.

In September 2022, Rare Cancers Australia (RCA) and Canteen launched a report *Counting the cost: the true value of investing in cancer treatment* (see QR code to download a copy). The report explored what matters most to people receiving cancer treatments, in terms of the social and personal impacts, and aimed to put a value on those benefits through Social Return on Investment (SROI) analysis. The report explored the tangible outcomes (things we can measure), such as overall survival, as well as intangible outcomes (things we cannot measure), such as spending more time with loved ones, returning to work, or experiencing healthy survivorship.

The analysis found that for every $1 invested in cancer treatments, there is $3.06 of social and economic value created.[1] It also demonstrated that, over the course of five years, investment in new technologies, therapies, and services to extend the prognosis and quality of life of people with non-curative cancer can return $3.17 billion of social value.[1] Ultimately, SROI analysis demonstrated that although investing in new therapies can have a high upfront cost, there are substantial downstream social and economic benefits in treating people with the best available technologies.

This report *Counting the cost: how we can assess the true value of investing in cancer treatment* is a follow-up to the 2022 report and aims to provide practical examples of how we might effectively integrate and evaluate social value within our Health Technology Assessment (HTA) and health system.

In advancing the focus from ‘why’ we should incorporate social value to ‘how’ we can accomplish it, it is crucial to develop and implement methodologies that capture the experiences of people accessing cancer treatments. This holistic approach will allow for a comprehensive assessment of the true value of treatments. This ensures that decisions made by organisations like the Pharmaceutical Benefits Advisory Committee (PBAC) align with principles of fairness and social justice, promoting improved treatment accessibility for every individual. Incorporating SROI into the value assessment process also promotes transparency and accountability. Stakeholders, including people living with cancer, healthcare providers, and taxpayers, gain a clearer understanding of the impact and value generated by the allocation of healthcare resources. This enhances decision-making and ensures that the allocation of resources aligns with the broader goals of healthcare systems.

Incorporating these insights and a comprehensive assessment of value and impact will not only improve treatment decisions for individuals with cancer but will also lead to better outcomes for their communities. Through the 2023 HTA Review, Australia has the opportunity to build a pioneering HTA system that integrates lived experience and prioritises what truly matters to people living with cancer, setting a new standard for comprehensive and person-centred healthcare evaluation.

Introduction

A cancer diagnosis has far-reaching effects not only on people's lives, but also on their loved ones, and society as a whole. Every day in Australia, 413 people receive a cancer diagnosis; totalling 151,000 new cases each year and 52,000 deaths.[2] Additionally, almost half of Australians will be diagnosed with cancer in their lifetime.[3] There are currently more than 1 million Australians either living with, or who have experienced cancer,[2] and cancer represents 18% of the burden of ill health suffered by Australians.[3] Importantly, rare and less common cancers account for a third of all new diagnoses and nearly half of all cancer related deaths.

As the population continues to grow and age, the incidence of new cancer cases is expected to rise. By 2031, it is estimated that 185,000 new cases of cancer will be diagnosed each year.[4] This ever-increasing number of people living with new cancer diagnoses in combination with an ageing, and hence more vulnerable, population means that without change, cancer will continue to be a significant cost to society.

There is room for optimism, however, as the field of cancer diagnostics and treatments is experiencing remarkable advancements, with innovative and personalised approaches emerging at an unprecedented rate. These new therapies increasingly offer better and more durable outcomes with fewer short- and long-term side effects. The challenge for not only Australia but every country is assessing whether these treatments are affordable and how we should assess their value as we negotiate their supply with multi-national corporations.

The tool of choice for governments and other insurance entities in assessing the worth of these new treatments is Health Technology Assessment (HTA). In Australia, it is heavily relied upon by, among others, the Pharmaceutical Benefits Advisory Committee (PBAC), the body that recommends to the government of the day what new therapies should be funded for Australians and what currently funded therapies should have their use expanded.

The World Health Organization (WHO) defines HTA as:

> *Referring to the systematic evaluation of properties, effects, and/or impacts of health technology. It is a multidisciplinary process to evaluate the social, economic, organisational and ethical issues of a health intervention or health technology. The main purpose of conducting an assessment is to inform a policy decision-making.*

The focus of PBAC is significantly narrower than the definition used by WHO, which specifically refers to social, economic, organisational and ethical issues. More emphasis is placed on clinical outcomes, as measured through the outcomes of clinical trials and patient commentary around clinical side effects.

The challenge in including a broader consideration of the impact of a treatment (or its absence) is to quantify and value it. For this reason we introduced the concept of Social Return on Investment (SROI) in *Counting the cost: the true value of investing in cancer treatment*[1] a report launched in partnership by Rare Cancers Australia and Canteen in September 2022. The report explored the broader impact of cancer interventions on society and offered a framework for evaluating the social and economic impacts associated with the provision of new treatments and also the consequential cost of denying access. The aim was to provide a more comprehensive understanding of the value of treatments to people with cancer and society. The report demonstrated that SROI can quantify the social and economic benefits stemming from improved health outcomes. These benefits include, but are not limited to, societal cohesion, increased productivity, reduced morbidity, and improved Quality of Life (QOL) for people with cancer and caregivers. By capturing these wider consequences, the value assessment process more accurately reflects the true worth of treating Australians living with cancer and the full value of pharmaceutical products to society.

This report, *Counting the cost: how we can assess the true value of investing in cancer treatments*, is the second in the *Counting the cost* series and explores in more detail how our use of HTA can adapt to better represent what matters to communities living with the condition. It will also review the use of HTA outside of Australia and demonstrate how SROI can be integrated into HTA submissions alongside traditional sections of a submission.

How do we value what matters to people living with cancer?

What is valued by individuals within a society is influenced by a multitude of factors, including their personal experiences, viewpoints, belief structures, and priorities. When these factors are combined with the characteristics of a particular disease, the impacts of its treatment, and the prevailing societal preferences, a discernible notion of significance begins to take shape. Value, in essence, is an aggregation of the aspects that people perceive as meaningful. When we evaluate this combination using a cost versus benefit analysis, the net benefits provided by a particular situation determine its worth. It is crucial that the criteria employed to measure these advantages are congruent with the concerns of the populace. For instance, if the ability to work holds importance, an associated value is ascribed to that capability. This value should be taken into account during HTA deliberations.

In practice, the assessment of value often falls short by not considering the full spectrum of benefits. Tangible outcomes, such as hospitalisations or deaths, are easily quantifiable. However, intangible elements are frequently overlooked; emotional well-being, social functioning, and personal fulfilment are important but undervalued components of patient priorities and Quality of Life (QOL).

Ignoring these intangible factors leads to an incomplete understanding of value and hampers effective decision-making.

By broadening our notion of value and embracing a holistic approach, we can make more richly informed decisions that lead to better outcomes for the people receiving the treatments, and those around them. These value measures are particularly important where traditional robust data may be difficult to obtain, such as in rare cancers and other small-population diseases.

It is critical to understand and incorporate the first-hand experience of a condition, either through personal involvement or direct engagement with individuals who have the condition, to establish value and provide effective person-centred care. In the context of cancer, lived experience encompasses the unique perspectives, challenges, and experiences of those diagnosed with cancer, as well as their loved ones. Currently, no HTA system routinely embeds lived experience in an end-to-end fashion to comprehensively consider its value and impact.

Person-centred care (placing the individual at the centre of their own care and decision-making) is well-established as best practice in the delivery of health care services. The approach of applying ‘user’ preferences could well be the next frontier for HTA. As an example, in a recent publication on outcome-based payment schemes, people with cancer and carers identified a broad range of values that mattered to them – the research group characterised these into six overarching categories (see Table 1).[5]

Table 1: Categories identified by people with cancer and associated stakeholders on values that matter to them[5]

Category	Examples
Impact on daily life and future	Continuing activities such as sports, hobbies, doing own grocery shopping, maintaining independence, working, caregiving roles, parenting,[6] independent driving and sleep quality.
Costs for people with cancer and loved ones	Especially loss of income, out-of-pocket costs, reintegration into employment, travel costs, home modifications.[6]
Quality of life (physical, psychological, social spiritual and sexual)	Such as early menopause/infertility associated with certain treatments, or bowel dysfunction and stomas in colon cancers.[6] Alopecia (hair loss) as a stand-alone side effect of many older treatments (less so with newer targeted treatments) can dramatically negatively influence self-esteem and personal identity. Hair loss can be one of the most confronting side effects of cancer treatments. It can impact self-confidence, well-being and cause humiliation for both men and women of all ages.[7] Psychological impacts can be as profound as physical ones to some. Fear of death/recurrence is common and the impact on relationships and friendship/social lives is well-documented.[6]
Impact on loved ones	Loved ones will in many cases transition to caregivers which can impact them in multiple ways including their well-being, ability to work, self-care and personal identity.[6]
Societal impact	Even a moderate impact on household income can have significant ramifications at the family level, but there are also broader effects such as loss of productivity and participation in social life. There is an obvious wider effect to society and the employer when employment is disrupted. The authors note specifically the chronic long-term effects of some treatments such as heart conditions attributed to chemotherapy.[6]
Quality of treatment	Quality use of medicine (which means people get the best possible care and treatments consistent with the evidence base) was the most important factor. Again, participants raised the long-term morbidity (side effects) which can occur as a direct result of oncological treatments. Mode of administration was also important if it meant people could be treated at home or self-manage and avoid hospital visits.[6]

How do we currently assess value in HTA in Australia?

Submissions for therapies to be reimbursed in Australia are driven by sponsors, who are typically pharmaceutical or healthcare technology companies. These submissions are comprehensive explanations of the clinical efficacy, safety, cost-effectiveness and overall cost of a new medicine, technology or service. The evidence submitted to the PBAC or Medical Services Advisory Committee (MSAC) is typically a detailed summary of clinical trials as well as real world evidence and safety information and is analysed through a HTA framework.

Economic evaluations included in HTA are built using clinical evidence and patient Quality of Life (QOL) values. These evaluations are used to understand value and impact. For cancer, value is based on clinical trial outcomes for progression, survival and QOL. In practice, average survival is combined with QOL measures to estimate quality adjusted life years (QALYs). A QALY is used as a measure to take into consideration both the quantity and quality of life. It combines the length of life which could be gained (in years) with a measure of the individual health related QOL at that time.[8]

Utilising QALYs for value assessment presents a range of concerns. These encompass inadequate sensitivity and the omission of numerous facets that have an impact on both the individual and the wider community. For instance, the importance of significant life events and the ability to maintain employment are disregarded within this framework.

The PBAC currently focuses on evaluating the tangible value of pharmaceutical products. It assesses the cost-effectiveness of medications by comparing their clinical benefits and costs in relation to a specific alternative treatment option. This analysis primarily revolves around measurable outcomes such as improved health outcomes, reduced hospitalisations, or extended survival. While this approach provides valuable insights into the economic efficiency of treatments and therapies, it overlooks a range of benefits and impacts that extend beyond narrow health outcomes and are more important to people with cancer.

The PBAC's emphasis on efficiency rather than equity is another notable characteristic of its value assessment framework. The committee's primary concern lies in maximising the overall health gain for the population utilising the available resources. While this efficiency-focussed approach aims to deliver optimal allocation of limited healthcare resources, it may inadvertently overlook domains of equity and fairness in access to therapies. The emphasis of the PBAC on clinical cost-effectiveness has the potential to unfavourably affect patient groups that could gain from treatments but are considered less cost-effective, such as rare cancers with small patient populations. However, these treatments may in fact offer substantial value by enhancing QOL or catering to specific patient requirements. For example, prolonging the life of the parent of a young child will have immense long-term societal benefits that should be taken into consideration when assessing the value of the treatment.

> **In recent years, there have been calls to broaden the PBAC's value assessment framework to recognise a more comprehensive assessment of value, including both tangible and intangible elements. This encompasses a broader range of outcomes, such as patient-reported outcomes, caregiver burden, and social and economic benefits beyond the health system, such as productivity. By applying a more holistic perspective, the PBAC would better capture the value of treatments and therapies and address equity concerns.**

It must be noted that in Australia as well as other countries, HTA does encompass consumer input. This is typically compiled by sponsors and patient organisations or submitted directly by people affected by the disease or therapy being assessed. Criticisms of this current process include low knowledge of the existence of the process amongst the general population; uncertainty for people with cancer about how to partake in the process; the capacity of people who are ill to participate; accepting input too late in the HTA process and opaqueness of how these submissions are considered in HTA.

How do other countries assess value?

Evaluation of new medicines and medical technologies varies by country. Countries that use similar HTA systems to Australia include Canada (via the Canadian Agency for Drugs and Technologies in Health [CADTH]), the UK (via the National Institute for Health and Care Excellence [NICE]) and Scotland (via the Scottish Medicines Consortium [SMC]). These agencies play an important role in ensuring that the community has access to safe, effective and affordable medicines. While their methods of assessment and incremental cost-effectiveness ratios may differ, they share a commitment to evidence-based decision-making, transparency, and stakeholder engagement.

While there is no consensus on best-practice, there are several ways these systems incorporate broader value and impact that may be relevant to our HTA system in Australia. Although all systems include lay people involved on decision-making committees, NICE has established Citizen Councils, which are groups of people who use health and care services, carers, and members of the public, who are selected to represent a diverse range of perspectives and experiences. Citizen Councils meet regularly to provide feedback and input on NICE's work and decision-making processes. NICE has also established 'patient expert groups' for specific health conditions or areas of work, which are made up of people with personal experience of the condition or disease area.

The SMC encourages patient groups to submit evidence on the impact of new medicines on people's lives. Patient groups can provide information on the benefits and risks of new medicines and how they affect QOL. This information is considered when making recommendations. SMC also involves patients and the wider public in its review processes, including the development of review protocols and the dissemination of findings.

Meanwhile, CADTH conducts patient engagement activities, such as focus groups, surveys, and interviews, to gather input from people living with conditions and the public on its work. Both SMC and CADTH involve patients in a horizon scanning process, which identifies new and emerging medicines and health technologies that may have an impact on people's lives.

This helps to ensure that the needs and preferences of the wider community are considered when assessing new treatments.

In addition to the work of the above funding agencies, there has been considerable work undertaken by a range of groups, all with the intention of developing value frameworks to identify what is important to people living with cancer. The American Society of Clinical Oncology (ASCO) was responsible for one such value framework that attempted to assess the value of cancer treatment regimens.[9] The original 'ASCO framework' focused mainly on clinical benefit and toxicity, the same main data collected from clinical trials. While the ASCO framework stressed the importance of high-quality, well-conducted and randomised studies, they also recognised there were some limitations of such studies in decision-making. The ASCO framework highlighted that aspects important to those impacted by cancer when assessing relative value should be considered. These include the convenience of receiving therapy, not interrupting the flow of daily living, the impact on QOL and the ability to achieve personal and professional goals and milestones.[9]

Similarly, Cancer Research UK and Greater Manchester Health and Social Care Partnership (GMHSCP) commissioned a series of focus groups to explore the possibility of developing a new way of funding cancer medicine within the National Health Service (NHS) in the UK. The paper summarised the different facets of 'value', based on the outcomes deemed most important to those living with cancer (see Figure 1).[6]

In addition to clinical results, researchers pinpointed a variety of other real-world consequences and condensed them into priorities for individuals impacted by cancer. This resulted in a dedication to conduct additional research, with a broader emphasis on measures that extend beyond clinical outcomes. This particularly pertains to aspects such as post-treatment side effects and the resumption of routine daily activities. Crucially, the research aims to explore the incorporation of these values into funding determinations.[6]

Figure 1: Outcome flower showing high-level outcomes identified as important to at least one of the participants[6]

- Social well-being
- Affects sexual relationships
- Social support/ loneliness
- Family functioning

- Overall survival
- Disease-free survival
- Recurrence

- Procedure satisfaction
- Time spent on treatment
- Would repeat/ recommend
- Access to care
- Treatment delay

- Emotional well-being
- Frustration/ annoyance
- Depression/despair
- Fear of recurrence
- Anxiety
- Lack of hope
- Lack of motivation
- Worry about family risk
- Coping

- Pain
- Fatigue
- Nausea
- Lymphedema
- Osteoporosis
- Constipation/ diarrhea
- Headaches
- Dizziness
- Appetite loss
- Weight loss
- Insomnia

- Memory
- Concentration

- Need for a caregiver
- Return-to-work status
- Physical well-being
- Return to normal activities of daily living
- Eating/hearing/talking

- Reoperation
- Treatment-related A&E visits
- Readmission
- Infection requiring antibiotics
- Fertility preservation

Adapted from Lorgelly P, et al. Outcome-Based Payment Schemes: What Outcomes Do Patients with Cancer Value?6

Recognising the value of cancer treatments for real people

Lillian's story

Lillian, a lawyer, wife, and mum to a three-year-old girl, was 34 years of age when she thought she simply had a post viral cough. She also noticed a new lump just above her collarbone. This prompted Lillian to seek medical attention and after a battery of tests, she found out that she had non-small cell lung cancer (NSCLC). The shock of this news, especially for a young, fit woman, was immense.

It took several weeks and appointments to get the final definitive diagnosis, which revealed that Lillian had a very rare form of lung cancer, called ROS1 lung cancer. This subset of lung cancer affects just 1-2% of the 13,000 new cases of lung cancer in Australia per year.[10]

At that time, in the opinion of her treating oncologist, the best medicine available (which was a targeted therapy against ROS1) was not available on the PBS. This medicine cost up to $8,000 per month and was only accessible to Lillian due to a large crowd-funding effort initiated by her oncologist. She feels lucky to have been diagnosed quickly, which she puts down to her family (who are all in the medical world) and her connections. She managed to start treatment relatively quickly, too. Unfortunately, the promise of this medicine (and the fact it was shrinking the tumour) was tempered by a specific side-effect she experienced, and despite reductions of dose, it was decided that she had to stop treatment.

> **I was out of options. By about six months after my diagnosis, I was faced with the question of – is it chemotherapy or do I join a clinical trial?**

Around this time, like many people faced with a diagnosis in this technological world, Lillian started meeting others online. Many of these people had a similar diagnosis to herself, including a medical researcher who told her about a clinical trial in Melbourne. He explained this was a phase one clinical trial and answered all her questions about the logistics of entering such a trial. At her next appointment with her oncologist, before he could say anything to her about her options, she asked him specifically if she could join this trial.

Lillian managed to get access to a novel and targeted treatment through her own research and by directing her health care team. Ever since then, she has been on the same medication at the same dose with few side-effects and the cancer is under control. Being able to access a targeted therapy rather than the older conventional chemotherapies made such a difference to Lillian.

Lillian recalls the day that she learned she was being accepted into the clinical trial. She was about to go into a wedding banquet when she got a call in the car park – she was elated. She knew that she did not want to endure the 'pains of chemotherapy' and, through her research, knew that the prognosis for her specific type of lung cancer was not good, even with chemotherapy.

By the time Lillian started on her new targeted therapy, many of her initial symptoms had come back with even greater ferocity than before. She recalls coughing so much she fractured a rib. Within a day of starting treatment, the cough had completely gone. Since that time, the side effects have been minimal and tolerable, while the therapy continues to work.

It was not just the avoidance of side effects that Lillian values but also the ability to treat herself by simply taking some tablets before bed. By avoiding the lengthy hospital-based infusions required for chemotherapy treatment, Lillian can retain some degree of normality in her life, which is an '... invaluable part of the targeted treatment'. She admits she can never fully switch off and forget that she has cancer, but the value she places on this near-normality is profound.

> **Dealing with side effects throughout treatment means your life stops. There's a whole financial side of it. Socially, you're taken away from your catch-ups, your children's drop-offs at school, you know, there's all that.**

Lillian also places a significant value on 'not looking like a stereotypical cancer patient'.

> **When I meet people and their mum, dad or family member had been diagnosed, they see me and go, 'Huh? Cancer patients can look like that?'. I'm like, that's the least I can do to show that we actually come in all forms. It helps take away that fear and stigma. It is hard to put in words, but I think it's the further impact to society around cancer.**

Lillian doesn't want people to think that getting such a targeted therapy means your life does not change; it does, beyond recognition – cancer has that effect. We need to address the long-term emotional impacts of cancer; healthy survivorship needs to be a long-term consideration of care.

But first, being able to access the treatments that meet the needs of people with cancer at the time they need them is vital because people simply do not have time to wait.

AUTHOR'S NOTE

Lillian's access to her treatment has meant that she continues to be a great mum to her daughter and partner to her husband. She continues to significantly contribute to society through her employment and volunteer work and simply by living, gives remarkable hope to all Australians diagnosed with cancer.

It should be noted that, sadly, the treatment Lillian receives has not been subsidised for funding on the PBS for others with her type of lung cancer. The small number of people diagnosed with Lillian's cancer means the company that owns the treatment has never applied for funding reimbursement, because under the current assessment protocols it would be highly unlikely to be recommended. Considering the evident value of the therapy not only to Lillian and her family, but to society more broadly, would shift the measure of value applied to the treatment and potentially make this impact a reality for others who could benefit.

Tahli's story

Tahli is a young woman and mother living with cancer. Her illness started during the COVID lockdown, when she was already juggling home schooling with running a small business. This was thought to be the cause of her increasing lethargy and rapid weight loss. It was after a walk one day along the beach that Tahli first noticed a few little welts on her stomach and back, which she initially thought were from sandfly bites. The welts became itchy, so she went to the doctor, who gave her a skin cream which settled them. Her skin then became increasingly itchy and so the doctor prescribed a stronger cream and reassured Tahli. Eventually the rash subsided, but then a few weeks later she developed a cough that wouldn't settle – a chest x-ray was taken which showed pneumonia. After a course of antibiotics, Tahli was sent for a CT scan to make sure everything was fine.

It wasn't fine. Tahli was subsequently diagnosed with lymphoma and referred to an oncologist for treatment. After undergoing a series of scans and biopsies, Tahli started on a combination chemotherapy that was scheduled for six rounds. Initially the tumour reduced in size, but as the treatment progressed so did the cancer. This characteristic of cancer to evade and evolve in such a way that it can continue to defeat more traditional treatments has been and remains a huge barrier to treatment.

> **Chemo was shocking. The first few rounds I went in on a Monday, came out Saturday morning. 24/7 for five days straight. Bags would finish, I'd have a quick shower, then back on. Because I was in hospital I was away from my husband and kids, and that made it so much worse.**

An alternative chemotherapy was prescribed for Tahli with the aim of progressing to a stem cell transplant. Sadly, the cancer also proved intractable to this process and her specialists determined that a new innovative therapy called CAR-T was her next best option.

The challenge was that CAR-T did not at the time attract government funding and the cost for a private payment would be many hundreds of thousands of dollars. Ultimately, CAR-T was made funded by government but this involved significant delays that resulted in Tahli having to undergo another round of treatment, radiation therapy this time, in order to keep her alive until CAR-T worked its way through the labyrinth of government and pharma regulation.

> **It was just bad news after bad news and you're like OK how much more can I do? But I had heard good things about CAR-T, and it was the only hope I had left.**

> **When starting CAR-T I was feeling physically exhausted from all the prior treatments, mentally over it. It'd been over a year. If this doesn't work, then what? My oncologist said if you don't have this you're going to die. I looked at my family and thought, I don't have a choice. It was all really scary, and you just hope that there's no side effects. But for me, CAR-T was by far the easiest out of everything I went through.**

Within 30 days of receiving CAR-T therapy she had a scan and her disease had completely disappeared.

One of the hardest things about Tahli's initial treatments was seeing the impact it had on her family, especially her children. The turmoil on the family with the chronic disappointment, of having to tell them that the therapy wasn't working time and time again, was a constant stress. She felt this was even harder for her family than for herself. The stress and trauma Tahli and her family faced during her treatment pathway was incalculable.

Continually reporting failed treatments and possibly fatal outcomes took a huge toll on everyone involved, culminating with Tahli virtually saying goodbye to her children as she started CAR-T.

Had CAR-T been available earlier in Tahli's treatment cycle there would have been dramatically less physical and mental trauma associated with her cancer. Even more worrying is that, had the funding of CAR-T been delayed further, she may not have lived through the journey. Tahli's story is a compelling illustration of how physical, mental, familial and societal impacts of cancer are real and when they are valued properly, build more balanced arguments for investment in cancer treatment.

Tahli is now cancer-free although, like all survivors, lives with the lingering side effects of her early toxic treatments. And again, like all people with cancer, she is haunted at the prospect of cancer's return. That said, she is happy and grateful that her last option proved the best option and is now back contributing to society as a mother, partner, businesswoman and powerful patient advocate.

Angus's story

Angus is a 19-year-old university student, currently studying a Bachelor of Business and a Bachelor of Creative Intelligence Innovation and getting ready for his working life. But his life, resilience and plans have already been well and truly tested.

Growing up he had been a healthy boy; social, active and ready to take on the world. But his life changed beyond recognition at just 10 years old when his mum, Trish, found a series of lumps on the side of his neck. After many, many tests, Angus was diagnosed with Hodgkin's lymphoma, stage 2A.

At a time when most children are preparing for school camps, Angus was preparing for something quite different. Due to his diagnosis at a young age, Angus cannot now recall many details, but he knows his personality changed instantly from one of an interested and intrigued kid to someone quiet and withdrawn.

> **I was just numb; I couldn't fathom how such a young kid could have cancer.**

The health care team reassured Angus and his family that the cancer was very treatable and that the chance of relapse or complications was slim. They started Angus on a combination of treatments and after six months, Angus had a CT scan that showed the cancer was no longer visible, and his treatment was finished.

Angus had a very strong support network from his friends, family and community. His best mates even shaved their heads, so he wasn't the only bald student walking around school! However, around a year later the lumps had returned. He knew this time that the cancer was back. The lumps were in almost identical position to the first ones, but this time they were bigger. Soon after, a PET scan confirmed his fears – the cancer had spread to both sides of his neck and his abdomen.

> **How could this happen? Wasn't my cancer meant to be gone? Wasn't it highly treatable?**

Chemotherapy was started again but was a lot more aggressive than the first time. However, it wasn't slowing the cancer growth and made Angus extremely sick. The health care team added radiotherapy in an attempt to gain control, which slowed the cancer growth but not enough. He had to undergo a stem cell transplant, which Angus describes as the worst 31 days of his life. Angus couldn't leave his room for a long time.

There was a drug that could help make sure that any residual cancer would be killed off. This would make it possible for Angus to leave hospital, regain his social life and go back to school, soon after the transplant. This drug could make such a difference to Angus, who was now a 12-year-old boy. He didn't want to lose his hair, he didn't want to miss school anymore, he wanted to see his mates, he wanted to learn and to get back to being a regular kid.

The drug that could give all this value to Angus was not reimbursed, and private health would also not cover it. It cost $11,000 per dose and he needed 16 doses. RCA helped Angus and his family crowd-fund to make this possible.

> **Looking back now I can't believe that we had to crowd-fund to save my life. Still to this day it causes me massive anxiety, PTSD, thinking about it. I don't even want to think about what would have happened if I didn't raise the money. Rare Cancers gave us hope when all hope was lost. Thanks to the help of the generous donations of people around Australia, and maybe the world, we were able to purchase my treatment. And after 11 doses a clear PET scan revealed that my cancer was completely gone. And to this day, over six years later, I've been completely cancer-free.**

Angus reflects on all the families who can't raise that amount of money for cancer treatment. He thinks about the fact that for his family, there was no plan B.

> **Maybe my parents would have re-mortgaged the house and gone into debt or I would have had to try a treatment with more risks and side effects. I was so young, and I just assumed that when you get sick, people help you to get better, right?**

Without this treatment, Angus may have never experienced teenage years where he could actually feel like a teenager; formals, year 12 graduation and now, university.

> **The fact that inequity involving cancer treatments is still seen in a country like Australia baffles me… Cancer is horrible, not just on the parents, not just on the patients, but on everyone involved. Having to see a brother or sister, a grandparent or a friend, go through treatment is scary enough, but imagine being in charge of paying for lifesaving treatments because they aren't covered by the PBS.**
>
> **This is the reality that faces many Australians, including myself. To think that cancer treatment is viewed as a cost or a burden instead of an investment is dreadful. Certain treatments that could extend or even save someone's life are inaccessible due to the financial aspects of the treatments or because they aren't approved. This has got to change.**

AUTHOR'S NOTE

Upon meeting Angus, it is evident that he will make an outstanding and lasting contribution to Australian society. Yet without the extraordinary efforts of his family he was in very real danger of a devastating outcome. The longer-term value of the therapy that arguably saved Angus's young life remains a strong example of the societal benefit gained from investment.

What can SROI analysis add to the assessment of value in HTA?

As we contemplate the funding challenges associated with providing treatments for Australians living with cancer, it becomes evident that a re-evaluation of our approach to cancer treatment is needed. The mere fact that most people navigate their illness without awareness of superior and alternate therapies than those prescribed underscores the inadequacy of maintaining the current methodology. Ultimately, the tool kit Australian clinicians have to treat their patients is determined by the HTA and PBAC process. It could be suggested that the constrained evaluation criteria for reimbursement lead to clinical decision-making being confined to what is included on the PBS.

Additionally, the categorisation of Australia as a secondary market for the pharmaceutical industry cannot be regarded an acceptable rationale for preventing people in Australia from accessing the most effective treatments. Australians uphold a deep belief in and reliance upon our social contract, which essentially asserts, 'you contribute through hard work, adherence to the law, and tax payments, and we, as your government, commit to being there for you in times of need'.

Crucially, the flip side of this social pact is the implicit pledge that, 'we will use your hard-earned taxes judiciously'. Therefore, the solution lies neither in unquestioningly meeting any price demanded for a therapy as and when required, nor in delaying or rejecting essential treatments for Australians.

Currently, our use of HTA is narrowly focused, primarily centred around clinical data derived from trials and limited patient input. In order to enhance decision-making fairness and effectiveness, a broader perspective is required, one that encompasses both the impact of providing the treatment and the ramifications of denying access. The concept of SROI represents a potential tool capable of providing this comprehensive outlook.

SROI offers a structured approach to quantifying the tangible and intangible consequences of a treatment. Its use would ensure funding recommendations are made within a framework that mandates a holistic evaluation of the disease's effects as well as the potential outcomes of granting or denying access to treatment.

> Importantly, SROI can be integrated into Australia's existing regulatory and assessment mechanisms, serving as a supplement to the data models currently used by PBAC. Its capacity to measure the social and economic implications also fosters uniformity throughout the decision-making procedure.

Incorporating SROI into the HTA evaluation process additionally promotes transparency and accountability. Through a systematic appraisal of the societal and economic advantages stemming from healthcare investments, a range of stakeholders – including people with cancer, healthcare providers, and taxpayers – can attain a clearer understanding of the consequences and value resulting from the allocation of healthcare resources. This is a feature that is noticeably absent in the present scenario.

The following pages contain a detailed hypothetical example of what a submission to the Australian PBAC might look like if it were to include SROI measures.

Example of how to incorporate SROI in PBAC submissions

It is essential to recognise that benefits from cancer treatment extend beyond the clinical realm. People undergoing cancer treatments often face unique challenges and experiences that cannot be fully captured by traditional clinical endpoints alone. These challenges may include aspects such as symptom and side-effect management, emotional well-being, continuity of employment, access to treatment, and the ability to engage in daily activities.

The following is an example of a hypothetical treatment, and how we can incorporate SROI assessments into PBAC submissions.

GX118 for children and young teens with high-grade glioma

High-grade glioma places a substantial burden on children and young adults, impacting not only their lives but also the lives of their families. These aggressive brain tumours can lead to severe symptoms, cognitive impairments, and dramatically reduced quality of life. Average survival is measured in months rather than years and only 25% of people survive more than one year.[11] The limited treatment options and poor prognosis further compound the challenges faced by these individuals. However, there is potential promise in the form of GX118, a targeted therapy specifically designed to address tumours with an identifiable mutation. GX118 offers hope by targeting the underlying genetic abnormalities driving the tumour growth, potentially leading to improved outcomes and a better quality of life for children and young adults diagnosed with high-grade glioma. The precision and effectiveness of GX118 hold promise for transforming the landscape of treatment options and providing hope for young people with glioma and their families living with the reality of this devastating disease.

As described, the submission to the PBAC would follow a 'top down' approach to estimating value through the clinical trial evidence for GX118 as well as economic modelling. The clinical trial evidence would be used to estimate the benefit of treatment through improved survival as well as other clinical parameters such as response rate and safety endpoints. This data would be combined with QOL assumptions to estimate QALYs gained. For example, treatment with GX118 might provide young people with glioma with five extra years of life on average. During this time, QOL is estimated to be 70% of 'normal'. When combined, young people with glioma are assumed to gain 3.5 extra QALYs (5 years x 70% = 3.5 QALYs).

Importantly, GX118 might cost the Government a total of $100,000 per patient per year. The additional average cost of GX118 can then be compared to the QALYs gained providing a cost per QALY gained ratio of approximately $143,000 ($100,000/0.7).

The PBAC would use this ratio as the primary determinant of value. The committee would also consider other factors such as clinical need, impact on carers, equity considerations and alternative available treatment options. However, there is a lack of transparency with respect to the mechanics of decision-making and these other factors are not routinely assessed in submissions or during a PBAC meeting. This leaves an asymmetry of information for people with cancer and the general public as to what influences decisions. Similarly, people with glioma and their families view the QALY paradigm as insufficient relative to their lived experience and value constructs.

In contrast, a submission that encompasses the traditional cost per QALY analysis as well as a SROI analysis would more thoroughly examine the factors that affect the lives of people with cancer and their families. To begin, SROI involves engaging with stakeholders directly impacted by the disease and the therapy, including people living with cancer and caregivers, to understand their priorities and how they might be influenced by this new treatment. By capturing these impacts, the analysis can effectively reflect the broader value of the therapy.

Impacts for people with cancer

Insights gathered from stakeholders would be combined in the new submission with other data sources to construct a theory of change and to illustrate how these impacts translate into value. For instance, young people with glioma report experiencing improved well-being due to treatment with GX118, enabling them to participate in social activities, attend school, engage in family gatherings, and interact with friends. Moreover, they gain the ability to contemplate their future, engage in employment opportunities, and plan for higher education.

Figure 2: The SROI captures and values these through a range of value endpoints for children

- Increased school attendance
- Enhanced fulfillment
- Improved mental health
- Increased income/savings
- Increased hope for the future
- Improved social life/time with family

Impacts for parents of children with cancer

When young people with glioma respond to GX118, it has a powerful impact on their parents' mental well-being. Not only that, it also alleviates some of their caregiving responsibilities, enabling them to focus on their own health and the well-being of other children. Additionally, from a financial standpoint, parents can engage more in paid work, and out-of-pocket (OOP) medical and non-medical expenses are reduced.

Figure 3: The SROI captures and values these through a range of value endpoints for parents

- Improved mental health
- Better ability to take care of own health
- Better ability to care for other children
- Increased income/savings
- Reduced OOP medical costs
- Reduced OOP non-medical costs

Impacts for the Government

The Government stands to gain significant value through the SROI framework when considering the use of GX118. Unlike traditional submissions, adding SROI captures a wide range of value elements such as reduced hospital visits, resulting in health system cost savings. Moreover, improved individual patient health leads to a lower burden of illness and comorbidity more broadly. Additionally, as parents return to work, the Government benefits from increased productivity and tax revenue and reduced provision of benefits.

Figure 4: The SROI framework provides a comprehensive assessment of the benefits that extend beyond direct healthcare expenses

- Reduced hospitalisation costs
- Reduced comorbidity costs
- Increased tax revenue
- Reduced unemployment payments

Measuring the impact

In the next step, the value endpoints are monetised through valuation techniques. One way of doing this is to understand what people would be willing to pay to achieve an outcome such as improved mental health or achieving further education. The endpoints are then summed into categories including:

Productivity
capturing employment and business benefits

Economic
capturing reduced expenses and increased income

Social and emotional
capturing hope and family time

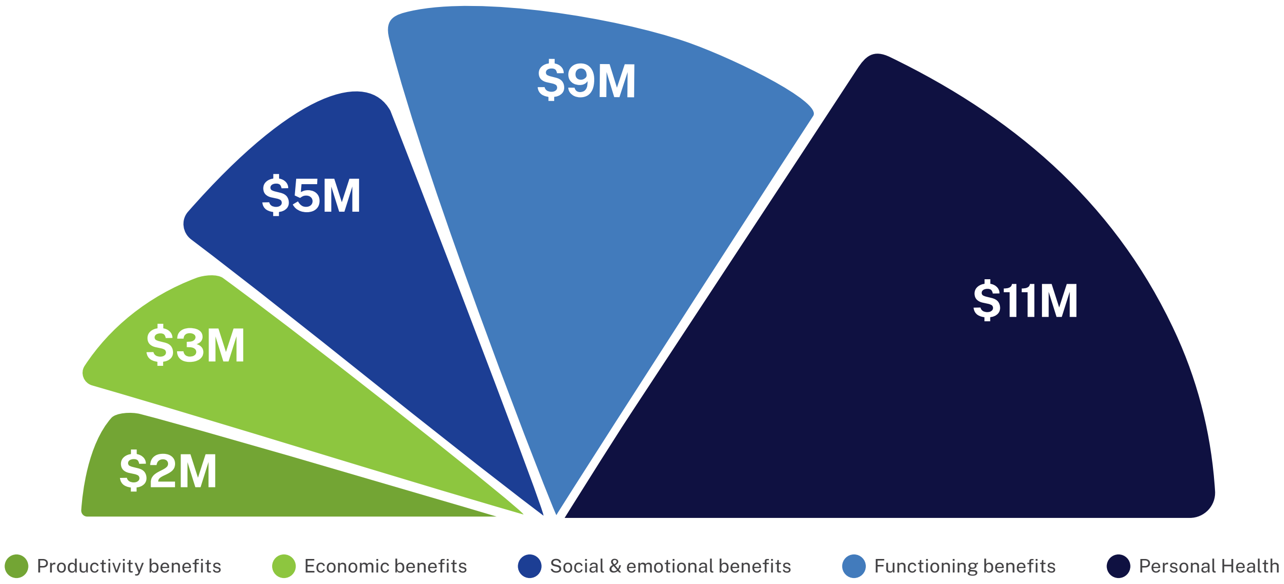
Role functioning
capturing going to school and being with friends

Personal health
capturing mental health and other personal health outcomes

It is estimated that around 100 young people would be eligible for GX118 in Australia per year, costing the government $10m annually. However, the SROI estimates that treatment would lead to substantial societal benefits, totalling $30m per year. This is achieved through:

TOTAL COST
$10M
PER YEAR

TOTAL BENEFIT
$30M
PER YEAR



Assessing the value

In evaluating the value of GX118, the PBAC would be equipped with compelling evidence. Firstly, they would have access to a cost-effectiveness estimate of approximately $143,000 per QALY gained, providing a quantitative assessment of the intervention's efficiency. Additionally, the committee would be presented with an impressive SROI ratio of 3:1, illustrating that the benefits outweigh the costs when considering outcomes that matter to patient populations and their families.

Moreover, the PBAC's decision-making process would be enriched by considering both tangible and intangible drivers of value, as estimated through the SROI analysis. This comprehensive evaluation allows for a deeper understanding of the broader impact and outcomes associated with GX118. The intangible value, which may encompass factors difficult to quantify directly, could be correlated with qualitative feedback received from patient communities. By incorporating these patient perspectives, the PBAC would engage in a more informed and holistic assessment, exploring the realities and aspects that truly matter to those affected by the condition.

Such a robust and comprehensive evaluation framework provides the PBAC with a solid foundation to make an evidence-based and insightful decision regarding the value and potential recommendation for reimbursement of GX118.

Conclusion

As discussed in the first *Counting the cost* report, the HTA Review offers a unique opportunity for us to ensure that our HTA system adequately values the people at the centre of it. In our assessments of therapeutic products, we must recognise that end-point data collected in clinical trials, whilst vital in creating an evidence base and informing reimbursement, do not represent the full extent of their value.

> Balancing the financial costs of investing in emerging cancer treatments with the benefits derived from effective cancer treatment is crucial. Improved quality of life, prolonged survival, and the subsequent physical, psychological, social, and economic benefits for people with cancer, their loved ones, employers, and society are all significant, and should be incorporated into our assessment process.

SROI can offer a comprehensive approach to evaluating the return on investment delivered by cancer treatments, by considering a broader set of outcomes and priorities. Incorporating elements such as improved health-related quality of life, reduced caregiver burden, enhanced productivity, and societal contributions, SROI results in a more accurate representation of the value created by cancer treatments.

We should take the opportunity the HTA Review provides and explore the practicalities of data collection necessary for SROI analysis; where does responsibility lie and what assessment framework should advisory bodies, such as PBAC, use to consider it? Should communities of people living with diseases be enabled to contribute data to SROI models that affect them in a systematic way? Are sponsors and investigators motivated to collect broader data to demonstrate SROI for regulatory consideration and to deliver cutting-edge therapies to patients faster?

As Australia undertakes the 2023 HTA review, we should embrace the opportunity to shift the focus beyond mere survival and incorporate the concept of healthy survival into HTA processes. Evaluating treatments based on their longer-term safety profiles and impact on quality of life will lead to more comprehensive and person-centred decision-making in cancer care. This new approach to decision-making would allow highly skilled clinicians to undertake disease management based on innovative approaches, not just therapies that represent a narrow view of value for money. By embracing a more inclusive and comprehensive approach that incorporates SROI, we can ensure the HTA system in Australia assesses the true value of cancer therapies and improves outcomes for individuals living with cancer, their families, and society.

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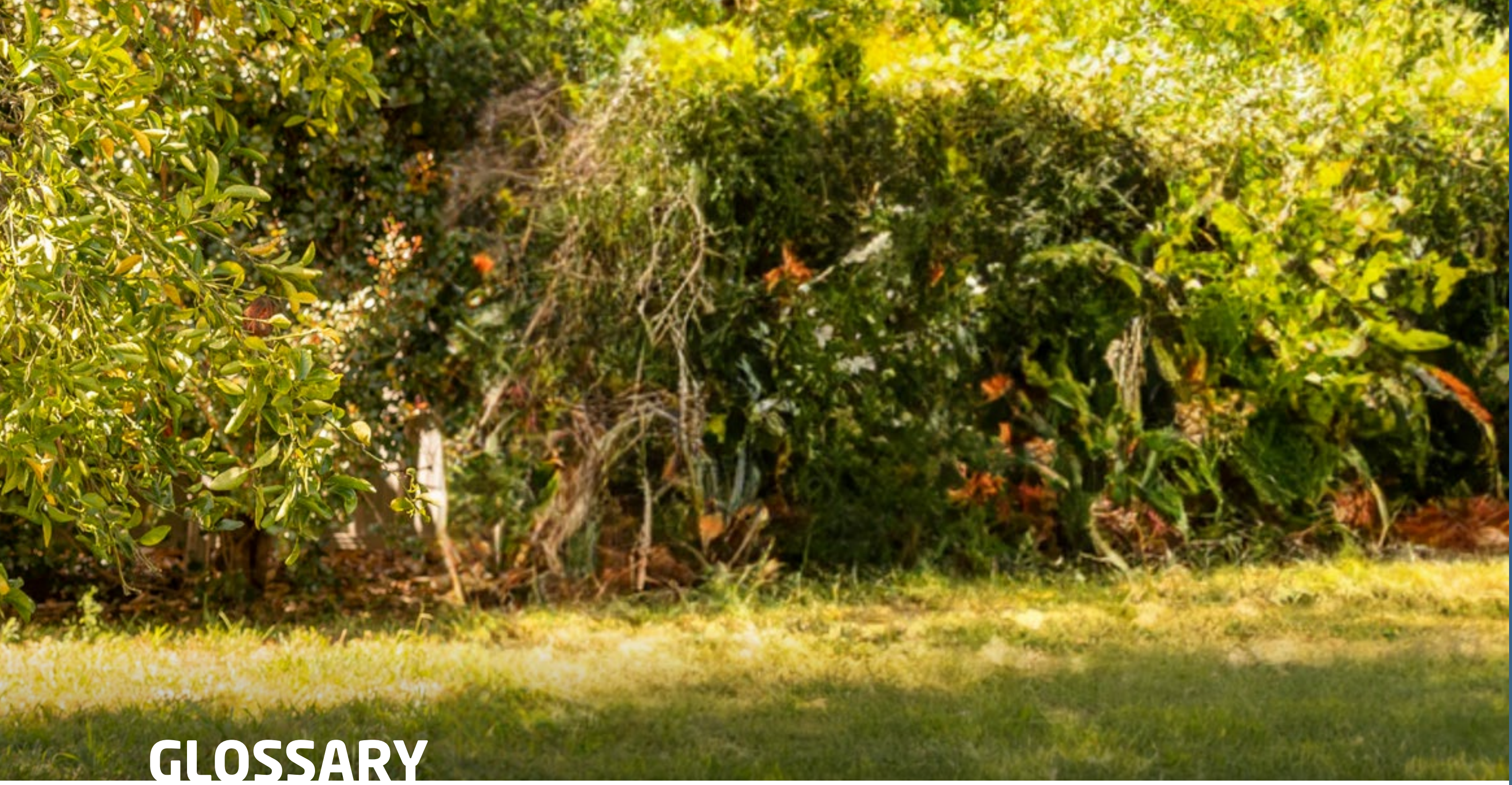

GLOSSARY

AYA	Adolescent and young adults
ASCO	American Society of Clinical Oncology
CADTH	Canadian Agency for Drugs and Technologies in Health
DSCRT	Desmoplastic small-round cell tumour
GMHSCP	Greater Manchester Health and Social Care Partnership
HTA	Health Technology Assessment
MSAC	Medical Services Advisory Committee
NHS	National Health Service
NICE	National Institute for Health and Care Excellence
NTRK	Neurotrophic tyrosine receptor kinase
NSCLC	Non-small cell lung cancer
OOP	Out-of-pocket
OS	Overall survival
PBAC	Pharmaceutical Benefits Advisory Committee
PBS	Pharmaceutical Benefits Scheme
PFS	Progression-free survival
QOL	Quality of Life
QALY	Quality Adjusted Life Year
RCA	Rare Cancers Australia
SMC	Scottish Medicines Consortium
SROI	Social Return on Investment

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