rare cancers Australia

Maximising **rare cancer clinical trials** in Australia

In partnership with

Cancer Trials Australia

Acknowledgement of Country

Rare Cancers Australia and Cancer Trials Australia acknowledge Aboriginal and Torres Strait Islander peoples as the Traditional Custodians of Country throughout Australia. We pay our respects to Elders past and present and extend that respect to all Aboriginal and Torres Strait Islander peoples.

We honour the deep and enduring connections of Aboriginal and Torres Strait Islander peoples to Country, culture, community, family and tradition, and recognise these as integral to health, healing and wellbeing.

We acknowledge the diversity of Aboriginal and Torres Strait Islander peoples and the many voices, knowledge systems and lived experiences that inform culturally safe and responsive healthcare.

Rare Cancers Australia and Cancer Trials Australia recognise the inequities in cancer outcomes experienced by Aboriginal and Torres Strait Islander peoples and are committed to working in genuine partnership to improve equity across cancer prevention, treatment and care.

Contents

About this report

Rare Cancers Australia and Cancer Trials Australia hosted a three-part roundtable series in 2025, Maximising Rare Cancer Clinical Trials in Australia, bringing together key stakeholders from across the clinical trial and cancer care sectors.

Held in Sydney on 24 September, Melbourne on 13 October, and Brisbane on 27 November, these roundtables explored the opportunities and challenges within Australia's current clinical trial landscape for those with rare cancers. This report summarises the discussions and insights gained from the series, setting clear areas of focus for maximising clinical trial availability and access for people with rare cancers.

We would like to acknowledge and thank all speakers and attendees who contributed their expertise and insights, particularly those with lived experience of cancer and clinical trials who generously shared their own perspectives. Their input has been instrumental in shaping this report, with the hope of improved outcomes and better experiences for people diagnosed with rare cancers in Australia through increased access to clinical trials.

rare cancers Australia

At **Rare Cancers Australia (RCA)**, our mission is to improve the lives and health outcomes of people affected by rare and less common cancers. We redefine cancer support, so no one is left behind or feels alone. Everyone is given the best possible chance to live beyond cancer.

We do whatever it takes to change the story of a rare cancer diagnosis through limitless support and advocacy. We drive change in access, affordability, and quality of care, ensuring better outcomes for the patients of today and tomorrow.

Cancer Trials Australia

At **Cancer Trials Australia (CTA)**, our purpose is to transform care for all people, by enabling world class clinical trials. We have a 30-year history of delivering excellence in clinical trial management, predominately oncology and haematology, across both metropolitan and regional hospitals. Our dedicated staff have supported the conduct of almost 3,000 clinical trials, that have recruited almost 20,000 Australian patients.

Our work helps to build clinical and industry knowledge, contributing to the development of novel therapies and the advancement of patient care, and most importantly improve outcomes for people effected by cancer. We continue to advocate for positive change in clinical trial regulation, working to ensure Australia remains an attractive destination for international clinical trial sponsors.

Rare Cancers Australia and Cancer Trials Australia would like to acknowledge supporters who helped make this roundtable series possible:

AstraZeneca

Daiichi-Sankyo

GSK

MSD

Pfizer

Supporters of this roundtable series had no editorial influence over session agendas or this final report.

Executive summary

Clinical trials are a vital part of medical research and innovation, assessing the safety and effectiveness of medicines and interventions. For people with rare cancers, they are a tool for equity and better outcomes.

Clinical trials serve a dual purpose: giving patients access to potentially life-saving treatments today, while producing the evidence required for regulatory approval and public subsidy in Australia[5]. For people with rare cancers, who face poor survival outcomes and limited access to treatments[3], clinical trials are not a last resort – they can be the only viable treatment option, considered alongside the current standard of care.

Australia is well-positioned as a leader in this space; the country has demonstrated its capability to deliver high-quality, well-conducted clinical trials across rare cancers and oncogenic subtypes, earning strong confidence from global pharmaceutical and biotechnology sponsors as a reliable destination for trial delivery, and despite its relatively small population, Australia ranks first in the world for the initiation of commercially sponsored clinical trials on a per capita basis[8]. Importantly for rare cancer patients, Australia also ranks third in the world for initiation of total number of phase I cancer clinical trials[9]. A stronger, more integrated clinical trial ecosystem can deliver clear, shared benefits across government, biopharma, health services, research institutions, and most critically, for patients.

Despite these strengths, Australians with rare cancers continue to face significant barriers in accessing clinical trials. These include limited awareness, unclear referral pathways, restrictive eligibility criteria, and, in some cases, the absence of suitable trials altogether. Many also report challenges in taking part in trials, including travel, meeting rigid appointment requirements, managing side effects and navigating the end of a trial.

Dr Kurt Lackovic and Christine Cockburn

Through a three-part, cross-sector roundtable series, we explored these issues and opportunities, with a focus on:

- how to ensure patients can identify, access, and participate.
- how to support additional research in Australia with particular emphasis on phase I clinical trials.
- how data generated in clinical trials can be better leveraged to support future regulatory approval and publicly subsidised access.

The discussions emphasised a curiosity and commitment across sectors to improving access to clinical trials for people with rare cancers in Australia. This is a shared challenge. Attendees identified actions and new approaches across the clinical trials cycle, from set-up through to Health Technology Assessment (HTA), that could improve patient experience and participation, increase the availability of trials and collection of the right data, and propel Australia as a leading destination for research and development. Building from existing strong foundations, attendees felt hopeful that we can work together to bring more trials and treatments to the people who desperately need them.

How to maximise clinical trial opportunities for people with rare cancers

1 Make it easier for rare cancer patients to identify, access and participate in clinical trials

- Support strong rare cancer indication networks, and early phase clinician networks and promote cross-network collaboration on a national scale.
- Embed consumer input into protocol development and trial delivery, including routine use of patient-reported outcome and experience measures across all cancer clinical trials.
- Encourage sponsors to engage with rural and regional sites early and support flexible or decentralised delivery models to improve geographic equity.
- Sponsors should collaborate with patient organisations to strengthen patient and public facing information and resources, including accessible and multilingual materials.
- The Australian Government should improve the national clinical trial registry through the National One Stop Shop (NOSS), by co-designing with patients with rare cancers and rare genomic subtypes, to provide accurate, timely and accessible information about available clinical trials.

2 Support and empower clinicians (investigators) to maximise clinical trial opportunities for rare cancer patients, particularly early phase

- The Australian Federal Government should increase funding for clinical trials activities in Australia, prioritising targeted fellowships and grants that give investigators the protected time needed to drive meaningful protocol development and feasibility assessments.
- Educate clinicians on the value of collaborating closely with commercial sponsors to broaden patient inclusion criteria and considering rare cancer indications where feasible, particularly within early phase clinical trials.
- Educate clinicians on the value of networked approaches to support timely connections between their patients and potential clinical trials.

3 Improve the efficiency and harmonisation of ethics and governance processes

- Facilitate faster patient access and strengthen Australia's attractiveness to commercial sponsors by establishing dedicated, prioritised review pathways for clinical trials focussed on rare cancer populations within Human Research Ethics Committees (HRECs) and site-specific governance processes.
- Implement national initiatives, such as the proposed NOSS, in a manner that improves efficiency across all institutions in regulatory and start-up activities, while preserving and protecting the performance of internationally renowned Australian trial centres.

4 Proactively increase the number of rare cancer clinical trials conducted in Australia, in particular Phase I trials

- Government, clinicians, trials groups and local affiliates of commercial sponsors, such as contract research organisations, should actively preserve and promote Australia's clinical trial capabilities internationally. Promotion should highlight Australia's exceptional start-up timelines, steady regulatory framework and financial incentives. Focus should be directed toward the country's strong early-phase trial capacity, which represents a significant opportunity for expanding access for patients with rare cancers.
- The Australian Government should accelerate the adoption of broader access to genomic testing for people with rare cancers, and a national genomic data registry, to maximise the efficient identification of patients who could be suitable for molecularly targeted clinical trials. Helping to maximise clinical trial recruitment.

5 Strengthen the pathway from clinical trials to subsidised access

- The Department of Health, Disability and Ageing should immediately clarify the evaluation of real world evidence, patient reported measures, and non-randomised and tumour-agnostic clinical trial designs in regulatory processes and decision-making, to encourage their use.
- The Department of Health, Disability and Ageing should issue clear guidance on how evidence-generation models can be utilised within HTA to safely and efficiently broaden medicine approvals for rare and molecularly defined cancer populations.

Presenters and perspectives

ROUNDTABLE 1

The value of cancer clinical trials and setting up for success

The first session explored Australia's rare cancer clinical trial landscape, identifying current strengths and defining characteristics as well as the pathways through which trials enter the country. Speakers and attendees considered ways in which Australia could attract additional clinical trials to address the current unmet needs of people with rare cancers and how Australia can position itself as a preferred destination for pivotal rare cancer studies.

Christine Cockburn

Chief Executive Officer, Rare Cancers Australia

Dr Kurt Lackovic

Chief Executive Officer, Cancer Trials Australia

As series co-chairs, Christine and Kurt shared organisational and personal reflections highlighting the current state and context of rare cancers and clinical trial delivery in Australia. Throughout the series, they drew on insights from their communities to guide discussions and encourage cross-sector collaboration.

Eva Bishop

Patient advocate

Eva shared her experience of participating in clinical trials to treat BRCA2-mutant pancreatic cancer. She implored patients to always listen to their bodies and for medical teams to ensure that every patient is fully informed of trial requirements and possible side effects. She recognised the value of taking part in clinical trials to deepen the evidence base and unlock potential treatments for future patients.

Carrie Bloomfield

Chair, Industry R&D Taskforce
Director of Clinical Operations, GSK Australia

Carrie offered her extensive pharmaceutical industry insights into how local teams work with their global counterparts in selecting hospital sites to conduct their global oncology studies. Carrie implored that for rare cancers, everything from ethics and start-up processes to study design needs to be approached differently. When compared with studies in more common diseases, rare cancer clinical trials present their own specific challenges which need to be considered to ensure success.

Professor Jayesh Desai

Chairperson, Cancer Trials Australia

Medical oncologist, Associate Director Clinical Research, Head Early Drug Development Trials Program and Deputy Director, Trials Unit Peter MacCallum Cancer Centre

Professor Desai provided an overview of the current cancer clinical trials landscape in Australia, drawing on his extensive experience in early drug development and insights from local and global data, noting Australia is a unique position to further leverage our extensive leadership in phase I trials to improve trial access for rare cancer patients. He highlighted Australia's efficient regulatory processes, high data quality, strong clinician engagement, diverse patient pool and current policy incentives as key levers in Australia's strong clinical trial ecosystem. He recognised the importance of clinician (investigator) advocacy and forums for bringing more of the right trials into Australia, based on unmet clinical need, and the great opportunity offered by early phase studies.

Bryce Davies

Early Phase Oncology Lead, IQVIA

Drawing on his experience of advancing early-stage research compounds into clinical trials, Bryce presented the perspective of Contract Research Organisations (CROs). He shared that in oncology, early-phase trials can offer broader and more flexible opportunities for rare indications. Bryce also highlighted the role local organisations can have in advocating to sponsors for additional or parallel study arms to accommodate rare indications and emphasised the importance of genomic profiling as a critical gateway for determining patient eligibility.

Elizabeth de Somer

Chief Executive Officer, Medicines Australia

Elizabeth commented on the integral role clinical trials play within Australia's health infrastructure. She emphasised the importance of horizon scanning and the scoping of unmet needs of Australian patients to ensure a balance that remains both desirable for international investment in research and drug development, whilst genuinely addressing underserved disease areas in Australia. She also commented on national policies, such as the Research and Development Tax Incentive (RDTI), as an effective attractor of international investment.

Deborah Dell

Head of Research Operations, Monash Health

Debbie identified opportunities to speed up and simplify governance processes to enable faster clinical trial start-up and reduce administrative burden. Noting that the current method of initial Human Research Ethics Committee (HREC) review – sometimes including multiple HRECs – followed by individual site governance processes can cause duplication and inefficiency. Debbie emphasised the need to streamline these processes, particularly for rare cancer clinical trials. For patients with high unmet clinical need, reduced start-up times would allow patients access to clinical trials as quickly as possible and provide a further competitive edge in attracting international investment.

Kylie Sproston

Chief Executive Officer, Bellberry Limited

Kylie presented trends in oncology trials currently being considered by ethics committees in Australia and the barriers rare cancer trials face when it comes to HREC reviews. She discussed key considerations for personalised trial designs, including whether current local infrastructure is equipped to support them. She also emphasised the value of pre-submission consultations in strengthening trial proposals by addressing potential issues early, giving studies the best chance of securing ethics approval on first review – an especially important step for early-phase trials, where preclinical toxicology data faces intense scrutiny.

ROUNDTABLE 2

Pathways to participation: Identifying, discussing and supporting trial access

In this session, attendees examined the current ways in which patients and clinicians identify and access clinical trials, considering the role of clinician networks, genomic matching, rural access and other factors that impact feasibility and participation. Understanding these practical elements is essential for ensuring that eligible patients can access the trials designed to help them. The session closed with a presentation and discussion focused on the potential of the Federal Government's National One Stop Shop (NOSS) initiative to better support patients, clinicians and sponsors to find, conduct and participate in clinical trials in Australia.

Bronwyn White

Patient advocate

Bronwyn reflected on her diagnosis and treatment of malignant peripheral nerve sheath tumour sarcoma, including decisions about clinical trials. She praised her proactive and well-informed treatment team and her rapid access to genomic testing that identified actionable biomarkers. She shared insights into the decision-making process and the information that helped her to ultimately decide to participate in a trial. Based on discussions with other cancer patients, Bronwyn highlighted that her pathway was more efficient and effective than that of many others. She wants every person diagnosed with cancer to have access to the same expertise and clinical trial options that she did.

Dr Malaka Ameratunga

Medical Oncologist, Alfred Health
Chair of the Victorian Rare Cancer Clinical Trial Alliance

Dr Ameratunga shared his reflections on how clinicians and clinician networks in Victoria work to effectively connect cancer patients with potentially suitable clinical trials. He noted that both formal and informal communication pathways can be effective, but that clinicians outside of such networks may not be aware of all available trials. With reference to the Victorian Rare Cancer Clinical Trial Alliance (VRCCTA) initiatives, Dr Ameratunga emphasised that successful clinical trial operations require a concerted effort from site staff, including proactively leveraging professional networks and facilitating cross-referrals. He noted that such collaboration is critical to optimising start-up activities and recruitment pathways – ensuring trials are delivered efficiently and effectively.

Professor Ben Solomon

Medical Oncologist, Group Leader of the Molecular Therapeutics and Biomarkers Laboratory in the Research Division, Peter MacCallum Cancer Centre

Professor Solomon provided his insights into patient recruitment, trial design and diagnostic methods across various genomic sub-types of lung cancer. He demonstrated how innovative trial designs, such as basket trials, can create opportunities for patients with rare cancer types and explored the efficacy data that can be gained through such approaches. Professor Solomon also explored current and emerging models of comprehensive genomic testing, a critical enabler for connecting rare cancer patients with appropriate clinical trials.

Narelle McPhee

Cancer Research Manager, Bendigo Health

Narelle spoke to the importance of conducting clinical trials in rural and regional settings, and the need to think differently to overcome additional challenges posed by greater travel distances, and at times, less well-resourced facilities. Drawing on examples of successful initiatives, Narelle demonstrated how just-in-time trial activation models, strong network collaboration and sponsor acceptance of telehealth where appropriate, can enable rural sites to conduct cancer clinical trials effectively.

Bridget Bradhurst

Chief of Advocacy, Ovarian Cancer Australia

Bridget shared the perspective of a patient organisation and their role in increasing awareness of clinical trials, as well as supporting patients and carers to access information about trials. Drawing on findings from a recent qualitative study conducted by Ovarian Cancer Australia, Bridget remarked how patients are willing and eager to take an active role in researching treatment options, including clinical trials, but need access to credible, centralised sources of clinical trial information.

Professor Hui Gan

Medical Oncologist, Austin Health

Member and former Chair of Victorian Rare Cancer Clinical Trial Alliance

Professor Gan drew on his extensive experience in rare cancer clinical trial feasibility and delivery to provide further insight to the clinician perspective of connecting patients with trials. He highlighted the practical reality that many clinicians face in which clinical trial responsibilities are often undertaken in addition to routine clinical practice. Professor Gan implored that for institutions to take on more rare trials, there needs to be strong support and commitment at an institutional level, as well as engaged clinicians.

Emeritus Professor Ian Chubb AC

Chair of the Inter-Governmental Policy Reform Group

Former Chief Scientist of Australia

Professor Chubb spoke about the currently proposed National One Stop Shop (NOSS), whilst also sharing his personal experience with cancer and a clinical trial. He outlined the potential for the NOSS to streamline trial start-up activities and provide clear, publicly accessible information on available trials. Professor Chubb emphasised that more streamlined and transparent start-up infrastructures could benefit and attract prospective researchers and sponsors, and that an up-to-date, reliable repository would be instrumental in linking patients to potential clinical trials.

Associate Professor Ben Tran

Medical Oncologist, Peter MacCallum Cancer Centre

Chair Phase I Group, Cancer Trials Australia

Associate Professor Tran, spoke to the flexibility of phase I trials and the opportunities they may present for people with rare cancers. He noted that these trials are largely biomarker-led and called for greater use of tumour-agnostic phase I trials to improve accessibility. Using real-world examples, he demonstrated how investigator-sponsor collaborations can influence eligibility criteria, how advocacy can support study activation in small cancer cohorts, and how strong relationships and scientific meetings can enable more modern study designs that support rare cancer patient participation in early-phase clinical trials.

ROUNDTABLE 3

From outputs to outcomes: making the most of clinical trial data

This session explored the data and evidence generated by clinical trials, Australia's medicines reimbursement landscape and the opportunities to work differently to ensure more trials are rapidly translated into subsidised access to therapies for Australian rare cancer patients.

Lillian Leigh

Patient advocate

Board Member, Cancer Institute NSW

Lillian shared her personal experience of how a phase I trial, which she discovered through another patient who shared the same non-small cell lung cancer rare oncogene, became her long-term treatment.

Lillian spoke to her challenges of travelling interstate for trial activities, as well as the broader logistical burdens faced by participants. She emphasised that for many people with rare cancers, trials can become treatment, and that data and reimbursement systems need to be fit-for-purpose to ensure successful trials translate into subsidised access for patients post-trial conclusion. While she remains on treatment through a compassionate access program, it is not PBS-listed and therefore unavailable to others with her cancer subtype.

Penny Shakespeare

Deputy Secretary, Health Resourcing Group, Department of Health, Disability and Ageing

Penny provided an overview on the current Health Technology Assessment process in Australia, with reference to the types of data considered by the reviewing reimbursement advisory committees, the levels of evidence assessed, and the inherent challenges involved in assessing applications for rare cancer indications.

She highlighted key initiatives led by the Department of Health, Disability and Ageing including the 2024 HTA review, research funding programs and the recent establishment of Genomics Australia. Penny also explored where real-world evidence and consumer input can be leveraged, as well as how genomics can enable more personalised approaches to care[1].

Michael Duhig

Clinical Research Manager and Developmental Psychologist, Children's Health Queensland

Drawing on lessons beyond the cancer sector, Michael discussed trial protocol design and data collection in studies involving children with neurodevelopmental disorders. He highlighted the value of caregiver-reported outcomes in determining meaningful endpoints, and the importance of optimising both patient and caregiver experience to improve recruitment and retention, support protocol adherence and enable greater flexibility in trials involving ultra-rare paediatric cohorts.

Dr Alisa (Lisa) Higgins

Director, Empiric Health

Lisa brought her experience in health economics and leadership in clinical trials outside the rare cancer setting, reflecting on how trial outputs are used in submissions and approvals for commercial use. She emphasised the importance of involving health economists as early as possible in the trial design process to help ensure high quality cases can be subsequently made to commercial authorities. Lisa noted opportunities to improve clinical operation efficiency through trial designs that can operate under umbrella regulatory approvals.

Dr Maryann Rakopoulos

Director, Servier

Maryann spoke to the considerations commercial sponsor companies weigh when adopting non-traditional study designs. She noted that demonstrated success builds confidence and momentum for future innovation and reflected on how listening to the needs of patients in Australia can drive greater flexibility, sponsor buy-in to decentralised approaches, and improved outcomes in rare cancer clinical trials.

Dr Lucille Sebastian

National Clinical Network Program Manager, Omico

Lucille discussed how alternative, decentralised study designs have worked in practice in Australia. She highlighted the importance of communications in achieving accurate feasibility assessments and effective trial recruitment. Lucille also remarked on the opportunity that genomic profiling of cancers holds in matching patients with clinical trials.

Elizabeth Pickworth

Patient advocate

Managing Director, Sidelines Consulting & Advisory

Elizabeth sat down with Christine Cockburn, CEO of Rare Cancers Australia, to discuss her experience navigating information about potential clinical trials to treat her thymic cancer, where there are currently no treatment options on the horizon.

She spoke to the power of patient-to-patient connection in both sharing knowledge and fostering a sense of support for people facing extremely vulnerable and uncertain circumstances.

Discussions at the roundtable series.

The value of clinical trials for people with rare cancers

People with rare cancers experience delayed diagnoses, limited treatment options, high levels of unmet supportive care needs and limited access to information and expertise[2,3]. While survival rates for many more common cancers have improved over the past few decades, the same increase has not been seen with rare cancers[4].

For too many people with rare cancers, affordable access to innovative treatments is out of reach. Many are significantly impacted by access delays and the high cost of non-PBS medicines. Traditional drug development and HTA processes limit treatment options, largely due to a mismatch between low incidence rates and high evidentiary standards required for approval and reimbursement. As a result, substantial unmet clinical need persists. Historically low levels of investment in rare cancer research have further constrained the development of effective treatment options[3].

In the absence of established standard of care treatment protocols, clinical trials take on an even greater value for people with rare cancers. In addition to generating evidence for new treatments and playing an essential role in the development of new interventions that improve health and quality of life outcomes, they offer eligible patients access to therapies that would otherwise be unavailable to them.

While not all trials are successful for all patients, the importance of having clinical trial options available and the hope they can provide was recognised.

Several attendees of this series have personally benefited from participation in a clinical trial, with many crediting a trial with saving or significantly extending their life.

> **"For people with rare cancers, the lack of reimbursed therapies and slow drug pipelines mean the standard of care increasingly exists within clinical trials."**
>
> **– Christine Cockburn**
> **CEO, Rare Cancers Australia**

In 2025

20,000+
people were diagnosed with a rare cancer in Australia

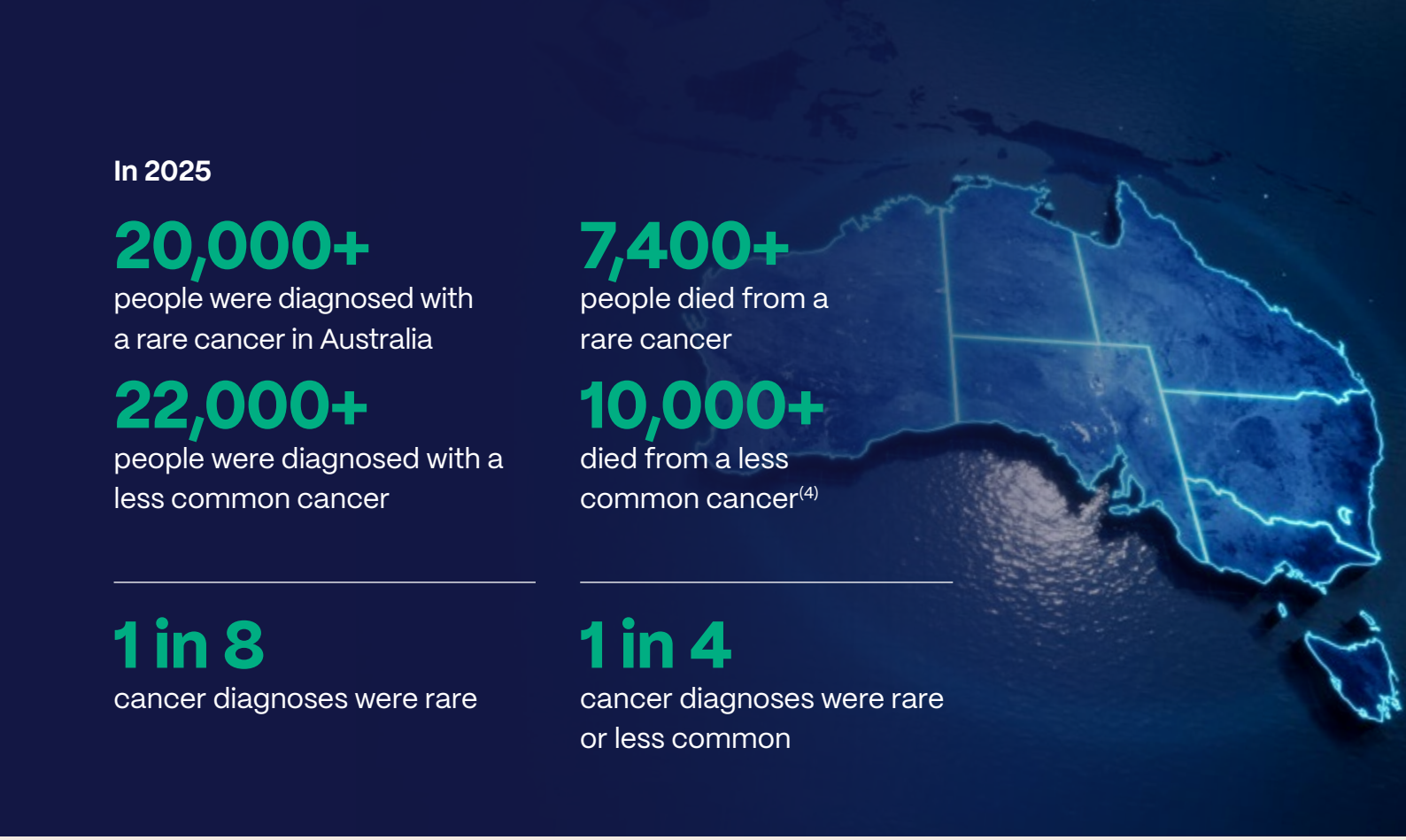
7,400+
people died from a rare cancer

22,000+
people were diagnosed with a less common cancer

10,000+
died from a less common cancer[4]

1 in 8
cancer diagnoses were rare

1 in 4
cancer diagnoses were rare or less common



A clinical trial is a research study that tests treatments or interventions to determine their safety and effectiveness. Medicines (new or repurposed), devices or treatment protocols are tested in humans to assess whether they are safe and if they can improve health outcomes for people with a particular disease[5].

Once pre-clinical laboratory and animal testing is successfully completed, a human clinical trial can be set up under highly controlled conditions. The process is deliberately rigorous, acknowledging that a potential therapy may be administered to people for the first time ever, some being patients with already very compromising health conditions.

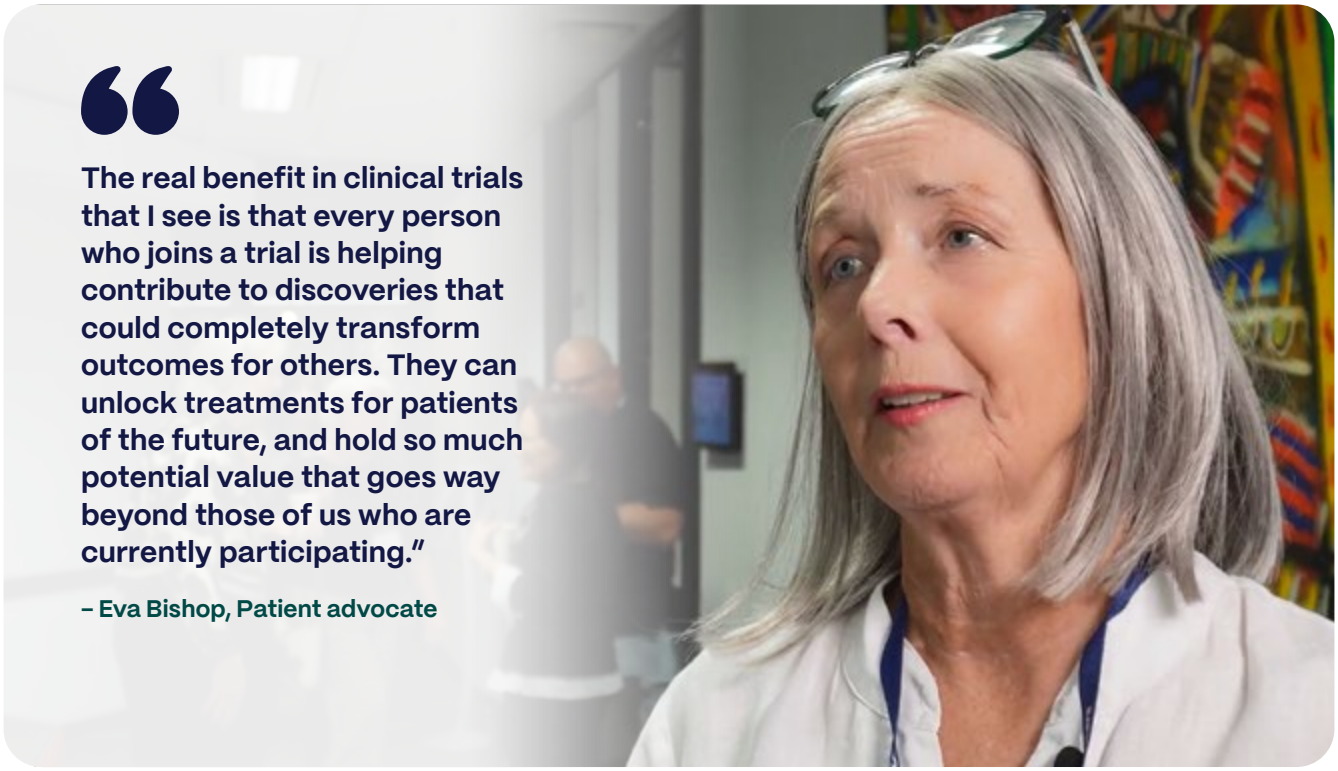
On a single trial, every participant follows the same study protocol – an ethically approved document outlining how a study is designed, how patients are to be monitored for safety, and what tests (e.g. blood samples, imaging) will be conducted and when.

Participation in clinical trials is voluntary, and patients can withdraw at any time.

Clinical trials are conducted in phases, each with a specific purpose:

PHASE I	Tests the safety and dosage of a new treatment in a small group of participants (patients). Modern Phase I trials increasingly enroll larger cohorts and incorporate biomarkers to explore biological activity and early signals of efficacy[8].
PHASE II	Evaluates the treatment's effectiveness and further assesses its safety in a larger group.
PHASE III	Compares the new treatment to standard treatments in a larger population to confirm its comparative effectiveness and monitor side effects.
PHASE IV	Conducted after a treatment is approved to gather additional information on its risks, benefits, and optimal use.

Data collected in clinical trials is required by the Therapeutic Goods Administration (TGA) for treatments to be registered and made available for the Australian public[6].



"The real benefit in clinical trials that I see is that every person who joins a trial is helping contribute to discoveries that could completely transform outcomes for others. They can unlock treatments for patients of the future, and hold so much potential value that goes way beyond those of us who are currently participating."

– Eva Bishop, Patient advocate

In 2025, RCA conducted a Cancer Lived Experience Survey, with responses from nearly 2,500 adults diagnosed with cancer and primary caregivers of adults diagnosed with cancer in Australia. Respondents diagnosed with a rare or less common cancer were more likely to be told about clinical trials as a treatment option **(36%)** and to take part in one or more trials **(15%)**, than those with a common cancer (**23%** were told about trials and **11%** took part in one or more)[7]. This reflects the increased role that clinical trials can play in the treatment of rare and less common cancers, who often have limited treatment options available.

However, significant gaps remain. Sixty percent of respondents with a rare or less common cancer said that their treating team did not mention clinical trials to them, and a further nineteen percent were told that no suitable clinical trials were available to them[7].

Given the increasingly significant role clinical trials can play as a pathway to treatment for people with rare cancers, and the importance of generating high-quality evidence to support future registration and reimbursement, this roundtable series examined what is currently working effectively within Australia's clinical trial ecosystem and what could be improved or further leveraged so that more patients know about and can take part in clinical trials – particularly patients who have no other treatment options.

People with rare cancers report a wide range of experiences when it comes to clinical trials. Some have achieved highly positive and sustained health outcomes with continued access to a treatment beyond the conclusion of the clinical trial. Others unfortunately have had no or negative responses, or felt that care decisions were overtaken by trial procedures and in some cases decided to withdraw from a trial.

A lack of clinical trials for rare cancer indications also delays access to established therapies. When people with rare cancers are not included in clinical trials, the data and evidence required for regulatory approval is not generated, widening the access gap. Novel therapies that are approved and subsidised for other cancers remain out-of-reach for people with rare cancers.

Attendees also highlighted the need to improve access to clinical trials for children with cancer. The recommendations in this report broadly apply to paediatric cancers, but more work is needed to adapt them to the paediatric setting. Particularly given the different clinical pathways and models of care involved.

Cancer clinical trials: unlocking potential in an established environment

Australia has a strong and mature clinical trials landscape. Despite its relatively small population, Australia ranks first in the world for initiation of commercially sponsored clinical trials on a per capita basis, third for the total number of phase I clinical trial initiations, and fourth for total number of phase II clinical trial initiations[8,9].

Throughout this roundtable series, Australia's world-class clinical leadership, strong early-phase trial capability, rapid trial activation processes, and supportive policy settings were recognised as key strengths that can be further leveraged to continue to attract and deliver clinical trials for the benefit of Australian patients.

While Australia currently performs well, attendees warned of the need to stay competitive and not become complacent, particularly as other countries increase their capacity and capability. Countries such as South Korea and China are growing their trial abilities, drawing on their natural advantage of large and densely residing populations. Australia's relatively small and dispersed population means we need to offer other benefits, like speed of set-up, trusted data policy incentives and novel ways to quickly identify patient cohorts, to keep pace.

Access to cancer clinical trials within Australia is inequitable, with particular challenges for patients living away from large cancer centres noted during the series, Australia's large geography and small population adding practical challenges that can easily become barriers to participation[10]. In addition, patients and clinicians can find it difficult to access reliable and accurate trial information; identifying potentially suitable studies is not always straightforward.

Even with a strong clinical trials ecosystem, too many people with rare cancers still cannot access treatments or clinical trials in Australia, and survival for rare cancers considerably lags that of the most common cancers[4]. Many patients will look for clinical trials overseas, taking on additional personal risk and paying high out-of-pocket costs, given that clinical trials are excluded from the financial assistance through Medical Treatment Overseas Program (MTOP)[11].

> **"Australia is one of the most attractive destinations to undertake clinical trials, because of the quality with which we deliver and the speed with which we can initiate them."**
>
> **– Dr Kurt Lackovic, CEO, Cancer Trials Australia**

It is imperative that Australia maintains a competitive edge to attract and deliver clinical trials, considering the many benefits for patients, clinicians, and the Australian economy – an estimated $1.1 billion a year in benefits across the entire sector[6]. Attendees reflected that a stronger and sustainable trial environment in rare cancers would further support broader clinical trial workforce development and strengthen industry capability.

Many attendees noted that the development of new cancer therapies has seen a shift from traditional immunology to more targeted agents and conjugates, resulting in more treatments being tested at an early stage and a steady rise in the number of early phase trials being started in Australia – presenting a unique opportunity for rare populations. The strength of Australia's clinical trial environment and the consistent growth in Phase I oncology trials over the past decade were highlighted throughout the series as an important foundation for further improvement[9].

However, concerns about a decline in late phase oncology trials coming to Australia were raised, despite their importance in establishing safety and efficacy in larger populations and generating evidence for registration and reimbursement. Given Australia's small and widely dispersed population, improving participation in later phase cancer trials will require stronger national coordination and systems to identify and recruit eligible patients, ensuring that geography does not limit trial feasibility or patient access.

"For people with rare cancers, access to a clinical trial is often the difference between treatment and no treatment. They play a vital role for individual patients as well as furthering our knowledge and practice for the patients of the future. Exploring ways in which we can expand on the repertoire of available trial options is of critical importance."

– Professor Jayesh Desai, Medical Oncologist, Associate Director Clinical Research, Head Early Drug Development Trials Program and Deputy Director, Trials Unit Peter MacCallum Cancer Centre

Attracting international, commercially sponsored clinical trials

Australia has a world-class clinical trials ecosystem and continues to strengthen its capabilities to remain at the forefront of global trial delivery. Raising awareness of these initiatives among international commercial sponsors was identified as a key enabler to attract and encourage the initiation of more rare cancer clinical trials in Australia. Proactive efforts are already underway; Australia's medical research strengths are being actively promoted internationally by both industry and the Federal Government.

The Medical Research Future Fund (MRFF) is a funding program which provides investment into health and medical research in Australia. It is encouraging that recent grant opportunities funded through the MRFF's clinical trials activity initiative are directed towards promoting Australian involvement in clinical trials as well as specifying for rare disease and cancer trials to be initiated here[12,13].

The Australian Clinical Trials Initiative, led by Austrade, coordinates national trade missions and strategic engagement activities to highlight Australia's competitive advantages, directly targeting the attraction of international commercial investment in clinical trials[14]. Ensuring that future engagement at international forums highlights Australia's capability to conduct complex, early-phase clinical studies, alongside promoting the broader advantages of its clinical research policy settings, could further support the attraction and initiation of rare cancer trials in Australia.

Complementing this initiative, The Australian Clinical Trials Education Centre – a separate, not-for-profit education platform developed by Australian research translation centres and partners – is expanding access to education and training for staff involved in clinical research, further enhancing the skills of an already highly capable workforce – particularly as the number of initiated trials continue to trend upward, and the accompanying economic benefit is so strong[15].

Australia's policy environment was considered generally favourable by some series participants, however the current global pressures on medicines pricing have caused concern for many about Australia's position in the global medicines

"We're seeing it at a federal level, lots of initiatives and a real effort to bring clinical trials into the norm of health service delivery"

– Deborah Dell, Head of Research Operations, Monash Health

market. The current Research and Development Tax Incentive (RDTI) policy was recognised through the series as contributing to the successful attraction of industry-led clinical trials to Australia[16]. Under this scheme, companies conducting eligible clinical trials can receive a refundable tax offset of between 38.5 and 43.5 per cent of eligible expenditure, depending on company size[17]. By effectively lowering the overall cost to sponsors of conducting research here in Australia, the RDTI remains a significant driver of sponsor-led trials entering the Australian market.

Attendees reiterated the importance of the incentive and proposed that, for rare cancers and other conditions with high unmet need, additional or targeted incentives should be considered to further reduce uncertainty for sponsors that are considering initiating their clinical trials in Australia. Building on these enablers and the additional strengths identified through the roundtable series, will further streamline trial conduct and advance the mission of positioning Australia as a truly world-class clinical trials environment.

Accelerating trial activation through system efficiency

The speed with which a sponsor company can establish or 'start-up' a trial was identified by as a critical factor in commercial decision-making regarding country selection for clinical trials.

Start-up activities include scoping hospitals or 'sites' for their capabilities, to assess whether they are able to perform every test and procedure in the trial protocol, and whether they will be able to recruit sufficient patients within a timely manner. It also includes the ethics process, a crucial but sometimes rate-limiting step to ensure patient safety – a trial cannot begin in Australia without first being approved by an Australian ethics committee.

Regulatory levels in Australia:

Therapeutic Goods Administration (TGA)

Before a clinical trial can begin in Australia, it must be authorised through one of two pathways administered by the TGA. Under the Clinical Trial Notification (CTN) scheme, a study is approved by a Human Research Ethics Committee and the TGA is subsequently notified before the trial proceeds to site-level review[18]. Or, in fewer instances, a study can be formally reviewed by the TGA itself under the Clinical Trial Approval (CTA) scheme.

Human Research Ethics Committees (HRECs)

HRECs' main purpose are to review the risks and potential benefits of clinical trials in humans, ensuring that a trial is compliant as per the National Statement on Ethical Conduct in Human Research[18]. This helps to protect the rights and welfare of patients.

Institutions

Once HREC approval is granted, each participating institution then submits the trial to their own internal governance review. Once site-specific governance approval is granted, the site can initiate trial activities, and recruit patients.

The regulatory approval pathway appears linear, but in practice can be complex and duplicative. Australia has over 200 HRECs, each operating with different capabilities, meeting schedules, and workloads[21]. Adding to this complexity, institutions may be specific about which HREC approvals they will accept, meaning a single trial protocol may be reviewed by multiple HRECS to meet all participating institutional requirements.

Each institution (e.g. hospital) also needs to undertake internal governance process, and these can vary greatly from one site to another, often linked to their level of clinical trial maturity. Taking into account the different turn-around times of HREC then site governance reviews, there can be substantial variation in the time required to initiate the same study across different sites. When a trial is conducted at multiple sites, the potential for variation in efficiency increases. These inconsistencies were identified as, at times, rate-limiting steps in recruiting patients and selecting sites who are slower at these activities.

There can also be delays in HREC approval where submissions do not meet review requirements. Some series contributors provided insights into what constitutes a good HREC submission and how submissions could be improved, acknowledging the impact of re-reviews and document amendments on approval times – particularly for ethics committees that only convene at a set calendar of meetings. For rare cancer applications, ensuring that pre-clinical toxicology data is appropriate and documentation is accurate could minimise the need for resubmission. Proactively consulting with HRECs prior to submission was flagged as an effective

way to reduce the likelihood of resubmission delays, particularly for rare cancers where less safety data, smaller patient populations, and higher uncertainty can lead to more cautious and extended review processes.

There are excellent examples of Australian institutions and HRECs delivering these processes effectively and efficiently, which have received global recognition contributing to Australia's reputation for fast and high-quality trial activation. Elevating all trial sites, particularly those with early-phase capabilities, to a consistent standard of performance could strengthen national metrics for study start-up and delivery, while also broadening recruitment reach across Australia. Ensuring that the regulatory and ethics environment in Australia remains consistent, fast and efficient was identified as crucial for maintaining Australia's international reputation, and for ensuring that patients can access new treatments and clinical trials as quickly as possible.

The role of alternative trial designs and the early integration of health economics was discussed, observing that many current trial set-ups, both within and beyond oncology, are effectively built as single-use structures: complex, resource-intensive, and dismantled once the trial is completed.

The National Mutual Acceptance (NMA) scheme is a policy framework that allows multiple institutions to rely on a single HREC approval before completing their own site-specific governance processes[22]. Implemented nationally in 2017, the NMA is a key mechanism for streamlining ethics review and reducing duplication[22]. However, some institutions remain reluctant to accept external HREC approvals, particularly for studies involving novel therapies or early phase designs – precisely the types of trials that are often most relevant to rare cancers. Discussions attributing the inconsistency in uptake as a reflection of Australia's state-based health networks and associated complexity. Any national initiative of streamlining the ethics process would require navigating these internal borders. Where institutions do not fully participate in NMA, further optimisation of internal HREC and governance processes becomes critical, especially for trials targeting rare populations with significant unmet need. Dedicated pathways within both HRECs and site governance structures – supported by out of cycle meetings, prioritised reviews and accelerated decision-making – were suggested as possible options to meaningfully reduce delays.

It was noted that site activation bottlenecks often stem from contract and budget negotiations and the completion of required documentation. Standardised fee schedules and clearer expectations were proposed as practical solutions to improve consistency and speed across sites. A defined, fast-tracked review pathway for rare cancer trials was also recommended to ensure these studies can open quickly and reach the patients who need them most.

Attendees of the roundtable series.

Early phase trials: a rare opportunity

Rare cancer patients are often excluded from later stage clinical trials because these studies typically require large participant numbers and apply strict, specific eligibility criteria that smaller patient populations cannot satisfy, such as forbidden prior lines of therapy[23]. Throughout the series, the substantial opportunity offered by early-phase trials for rare cancer patients was highlighted; trials which have the ability to be more flexible in eligibility criteria, can branch across tumour types and be green-lit with low patient recruitment targets.

As novel compounds and innovative trial methodologies emerge, the conventional linear model of drug development has evolved. It was noted that this has created new avenues for rare cancer participation that older trial designs could not accommodate. These studies are exploratory and prospective by design and are increasingly driven by tumour biology, such as genomic biomarkers and immunologic mechanisms of action, rather than tumour site alone. This shift allows patients with rare cancers, for whom traditional tumour-site-specific trials are rarely feasible, to access investigational therapies that would otherwise be unavailable.

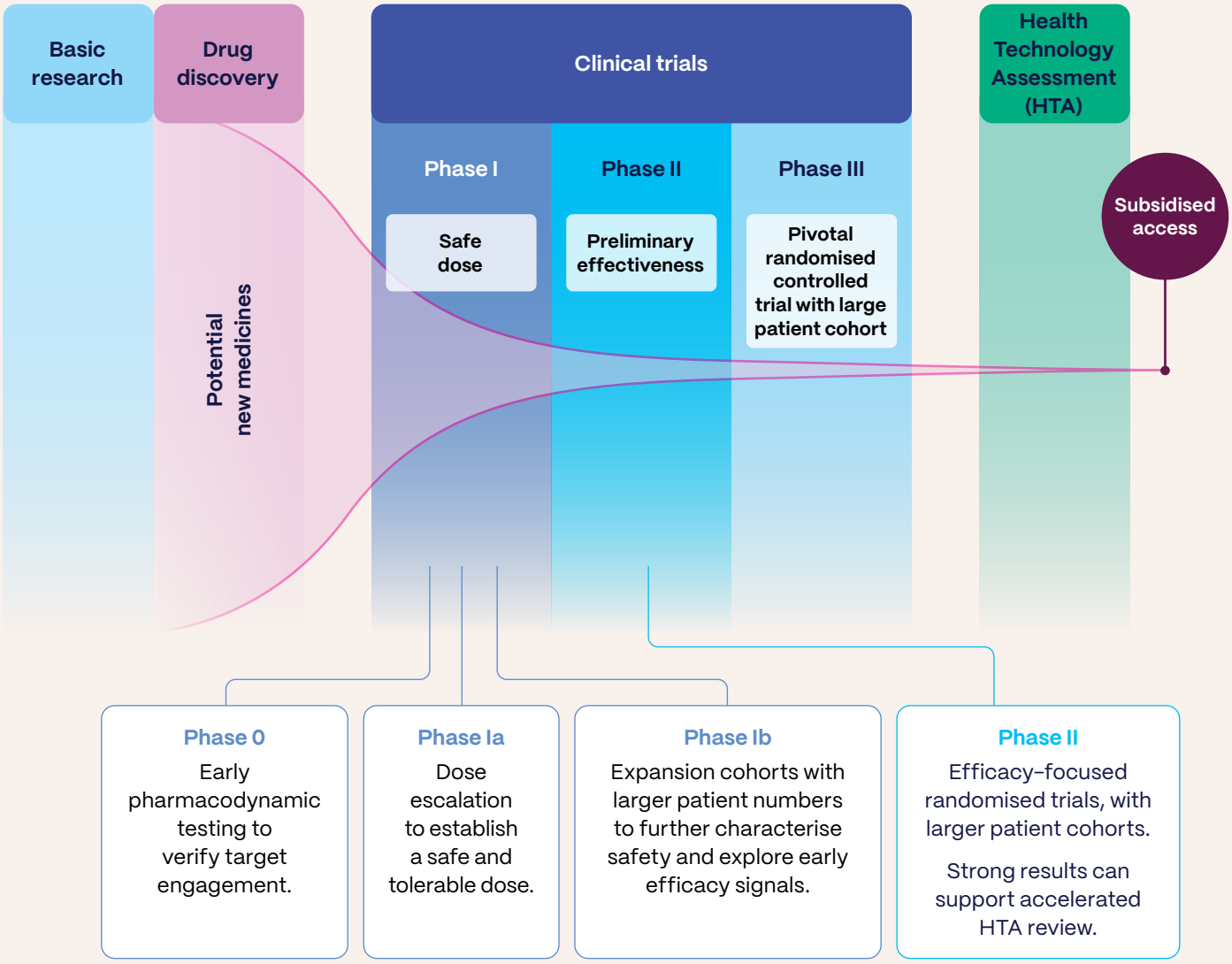


Figure 1. Overview of Drug Development and Clinical Trial Phases. Adapted from PhRMA "Clinical Trials," based on Tufts Center for the Study of Drug Development (2014) and U.S. FDA clinical trial phase descriptions[50]

Advocating for the trials that people with rare cancers in Australia need

Throughout the series, attendees returned to the principles of demand-signalling and advocacy to bring in the clinical trials that are needed for the Australian population, particularly those with rare cancers. There was recognition of the importance of a proactive approach from investigators, which could be strengthened through national initiatives, to seek and secure trials for those with high unmet clinical needs, rather than waiting for sponsors to initiate trials which have the tendency to represent more global interests.

Investigator-led trials

Strong relationships and close collaboration between investigators and commercial sponsors were seen as a critical opportunity to expand clinical trial access for rare cancer populations, benefiting both patients and commercial interests. Practical examples of the power of this approach were presented at this roundtable series – demonstrating how early phase investigators can have the ability to:

- influence eligibility criteria of clinical trials to include rare cancers.
- advocate for parallel protocols in rare cancers.
- initiated novel studies in rare cancers all together (Investigator-initiated trials).

One example outlined how investigator conversations and advocacy were successful in convincing a receptive, collaborative commercial sponsor to set up an additional, exploratory patient cohort. The clinical trial – initially planned to focus on a single tumour type of cancer – was subsequently expanded after investigators demonstrated that the relevant genomic alterations were present across multiple tumour types. Unlocking a new therapy option for a rare cancer population through the establishment of an additional dedicated cohort in a rare paediatric cancer.

Investigators can also provide crucial input into protocol design, particularly to support inclusion of patients with specific genomic markers. Other examples raised included allowing wider study-visit windows for imaging assessments and enabling the use of accredited local pathology services rather than mandating a single central laboratory, for the collection of routine blood tests.

"The investigator-sponsor relationship is key. Bringing rare cancer trials to the fore really requires a lot of planning and I think that the earlier that we talk to each other and the earlier we start planning these trials, the better the outcomes will be."

– Dr Maryann Rakopoulos, Director, Servier

Tumour-agnostic dose expansion cohorts were identified as an opportunity for patients with shared genomic targets to participate regardless of tumour type, transforming the potential for identifying effective treatments for people with particularly low incidence cancers. Although not always successful, there are examples of a documented tumour response in a patient with a rare genomic subtype, in a completely different cancer indication from the original cancer type being explored[25].

Throughout this series Australian clinicians were recognised as international leaders in a range of cancer specialties. Strengthening their ability to guide and expand research directions has the potential to improve outcomes for patients with underserved diagnoses, while also enabling sponsors to better understand how their therapies may benefit additional disease indications. This is mutually valuable and beneficial for patients, clinicians and the pharmaceutical / biotechnology industry.

Sponsor and Contract Research Organisation advocacy

Local sponsor teams and Contract Research Organisations (CROs) play a critical role in facilitating access to clinical trials and ensuring the right trials come to Australia for the benefit of patients with rare cancers.

CROs, sponsors, and trial groups often have broader visibility of emerging therapies and areas of unmet clinical need across cancer indications. Operating across multiple institutions and clinician networks, attendees noted that these groups can act as effective connectors between rare cancer populations and potential treatment opportunities. Reducing silos within the clinical trial environment was consistently identified as a key enabler of greater collaboration and more efficient trial development.

It was also noted the importance of clear communication and advocacy within sponsor companies, with local teams attempting to secure trial sites in Australia from significant global competition, based on Australia's strengths. CROs also have the ability to advocate directly to sponsors for the addition of parallel studies or single-arm cohorts, complementing clinicians and manufacturers to expand trial opportunities and lines of research for people with rare cancers.

Patient-led demand signalling

People with lived experience expressed frustration with the challenges they have faced in finding suitable clinical trials, with differences in access based on where they are being treated and how well connected their oncologist may be. One attendee highlighted that it felt nonsensical that they were ready and waiting for a clinical trial, but there was no way to show that to sponsors or investigators outside of their own multidisciplinary and care teams. Attendees discussed ways to better signal demand with less onus placed on individual clinicians, for example creating a national register where patients (or clinicians on their behalf) could show that they are open to considering trials, with some key characteristics of their cancer. Linking this to My Health Record was suggested as one way to minimise duplication and leverage existing platforms.

Discussions at the roundtable series.

Improving trial matching through universal genomic testing

Advances in genomic medicine are transforming cancer diagnosis and treatment. The rise in 'tumour-agnostic' treatments, which effectively target a genomic biomarker across a range of cancer types, is unlocking potential treatment options for patients with rare cancers[26].

Genomic testing can shorten diagnostic pathways and reduce diagnostic uncertainty, avoid ineffective or toxic treatments, and support timely access to targeted therapies and clinical trials[27]. Many Phase I trials are increasingly focused on targeted therapies defined by genomic biomarkers, rather than histology, allowing the set-up and expansion of clinical trials that may have been previously impossible due to low patient numbers.

Attendees discussed the value of comprehensive genomic profiling in identifying potential treatments and trials for people with rare cancers. While not every patient will have an identifiable and actionable genomic target, for some it can completely change their options. However, genomic profiling is not yet the standard of care in Australia.

In some studies, separate biomarker screening is performed for each individual clinical trial to determine whether patients may be eligible. This inefficient method contributes significant costs and time, delaying recruitment. Having awareness of genomic alterations prior to attending an initial trial screening visit could help avoid these inefficiencies and support identification of appropriate trials earlier in patients' treatment journeys.

Series participants emphasised the importance of equitable and accessible genomic testing for individual patients to support the attraction of additional clinical trials to Australia. This is particularly relevant given the substantial proportion of oncology clinical trials in which genomic subtypes and biomarker status are part of the eligibility criteria[28]. **Ideally, if every patient had genomic testing at diagnosis and the data were held in an appropriately managed national registry, prospective clinical trial cohorts could be quickly identified, whereas at present, they are invisible to investigators and sponsors. This would also support demand-signalling, giving more confidence to sponsors to launch clinical trials in Australia with genomic eligibility criteria. Some attendees also observed that Australia's relatively small population places it in a unique position to feasibly implement routine genomic testing – an opportunity to set Australia apart from other countries when sponsors are looking for trial sites.**

This proactive approach to matching patients with clinical trials is already being successfully employed by Omico through the public-private funded Cancer Screening Program (CaSP), which provides free genomic profiling for people with advanced or incurable cancers, and those with an earlier diagnosis of a poor-prognosis cancer[29]. More than 2,300 patients have been supported to access a clinical trial through Omico's programs to date, including at least 16 trials which otherwise would not have been initiated in Australia[29]. CaSP exemplifies the power of collaboration between government, sponsors, clinicians and patient advocates in proactively seeking trials and new treatments. Centralised national communication for identifying eligible patients was recognised as a critical success factor for such programs, as well as for other clinical trial initiatives led by, or delivered in partnership with, Omico.

With the recent establishment of Genomics Australia, the publication of the *Framework for Genomics in Cancer Control*, and the evidence, networks and infrastructure developed through Omico and the Zero Childhood Cancers Program, attendees saw this as a critical moment to seize the benefits of precision oncology by mainstreaming of genomic-led cancer care in Australia, to the benefit of cancer patients and to our economy[30].

The reality of trials: recruitment and participation

Despite Australia's strong clinical trials environment and the well-established value of trials for people living with rare cancers, the reality for many patients is that participation remains out of reach.

Effective stakeholder collaboration and patient-centred approaches were seen as critical enablers for successful trial delivery in rare cancer populations, from trial initiation through to close-out. As highlighted by the World Health Assembly, cohesive collaboration between community stakeholders – including clinicians, sponsors, and regulators – together with active engagement with ethics committees, trial groups, and patient communities across the entire trial lifecycle, leads to improved trial delivery and enhances the overall value of clinical trials[31].

When recruitment is conducted effectively, the benefits have the potential to flow to all stakeholders. Trial sites gain investment and experience, sponsors can meet recruitment targets with greater certainty, and patients gain faster access to potential new treatments[32].

The primary measures of performance for local commercial sponsor teams and trial sites are:

1. The ability to recruit the required number of patients.
2. The speed with which clinical trials can be initiated.

Following substantial upfront investment by biotech and pharmaceutical companies – including research and development, investigational product manufacturing, obtaining regulatory approvals, staff training, investigational product importation, and site activation activities – successful patient recruitment represents the tangible return on investment in the conduct of clinical trials in Australia. Sites that fail to meet recruitment targets miss out on per-patient funding and diminish their reputation for attracting future studies.

The need for individual sites to continue performing well on recruitment to maintain Australia's national reputation as an effective clinical trial ecosystem was also recognised. Further strengthening recruitment and trial delivery through coordinated ways to identify patients, actions to increase access for people living outside of metropolitan areas and those from culturally and linguistically diverse backgrounds, and continually improving recruitment practices, could all help to deliver even faster recruitment and wider delivery, which would attract further high-quality and relevant studies to the region.

Power in clinician networks

Attendees discussed the real pressures on clinicians, who need up-to-date and reliable access to information about open and upcoming clinical trials, as well as accurate medical histories for each individual patient. In the absence of one national or international database, clinicians have developed different methods of tracking trials and navigating options.

The ability of networks to broaden connection and influence, while retaining intimate knowledge of local capabilities and stakeholders, was identified as a key enabler of improved trial delivery.

Clinician networks are shaped by a range of factors, including hospital and departmental structures, individual clinician engagement, and the quality of communication, including between clinical sites. Contributing clinicians at the roundtable series described the methods of how they typically become aware of clinical trials, relying strongly on local institutional meetings and informal professional networks. A key limitation identified with these networks is that they often operate within closed circles, meaning trial opportunities may not reach clinicians outside these groups. This dynamic can perpetuate inequities in patient access, where some clinicians are aware of trial opportunities while others are not.

Strong examples of local and state-based networks were discussed, including the VRCTTA and NSW Early Phase Clinical Trials Alliance (NECTA). These networks have driven collaboration and an increased focus on early phase and rare indication clinical trials. Their value has been recognised by clinicians and through state funding[20]. There was appetite from attendees for stronger and better connected clinical trials networks, within and across states.

Registering the connections

During facilitated discussions, attending clinicians of this series noted that despite the availability of trial registries and digital systems, confidence in the accuracy and reliability of the information they provide remains inconsistent. In some cases, registries indicate that trials are actively recruiting when in reality, they are not, creating false hope for patients and clinicians alike. Poorly designed and

Professor Jayesh Desai, Medical Oncologist, Associate Director Clinical Research, Head Early Drug Development Trials Program and Deputy Director, Trials Unit Peter MacCallum Cancer Centre.

difficult-to-navigate interfaces further limit their utility and accessibility. In the absence of a central, trusted, and up-to-date trial repository, clinicians continue to rely on local networks for trial awareness, investing effort in expanding these connections rather than engaging with any additional platforms.

In one positive example, Cancer Trials Australia provides clinician access to a web accessible and filterable list of trials open to accrual across their service Members (currently 19 Victorian hospitals). Because this information is updated weekly from operational platforms, it is the most up-to-date information available to clinicians spanning multiple hospitals.

As well as trial information, clinicians require comprehensive and consistent information on their patients to determine trial suitability – including clinical history, genomic profile, and prior treatment responses. Clinicians who attended the series noted that registries and patient records can be fragmented or non-standardised, resulting in clinicians, who are the critical link between patients and trials, facing significant barriers in identifying and facilitating appropriate trial participation.

Trials in hospitals: not just a bonus

There is evidence indicating that conducting clinical trials delivers broader institutional benefits beyond any research outputs, where hospitals can receive 'spill-over' effects through conducting trials. This includes strengthened research workforce capability, as local clinicians, trial coordinators, pharmacy, and laboratory staff gain hands-on experience through trial delivery – bringing significant external investment or new technologies into the participating institutions. In addition, local patients gain access to fully subsidised investigational medicines that would otherwise not be available locally[32].

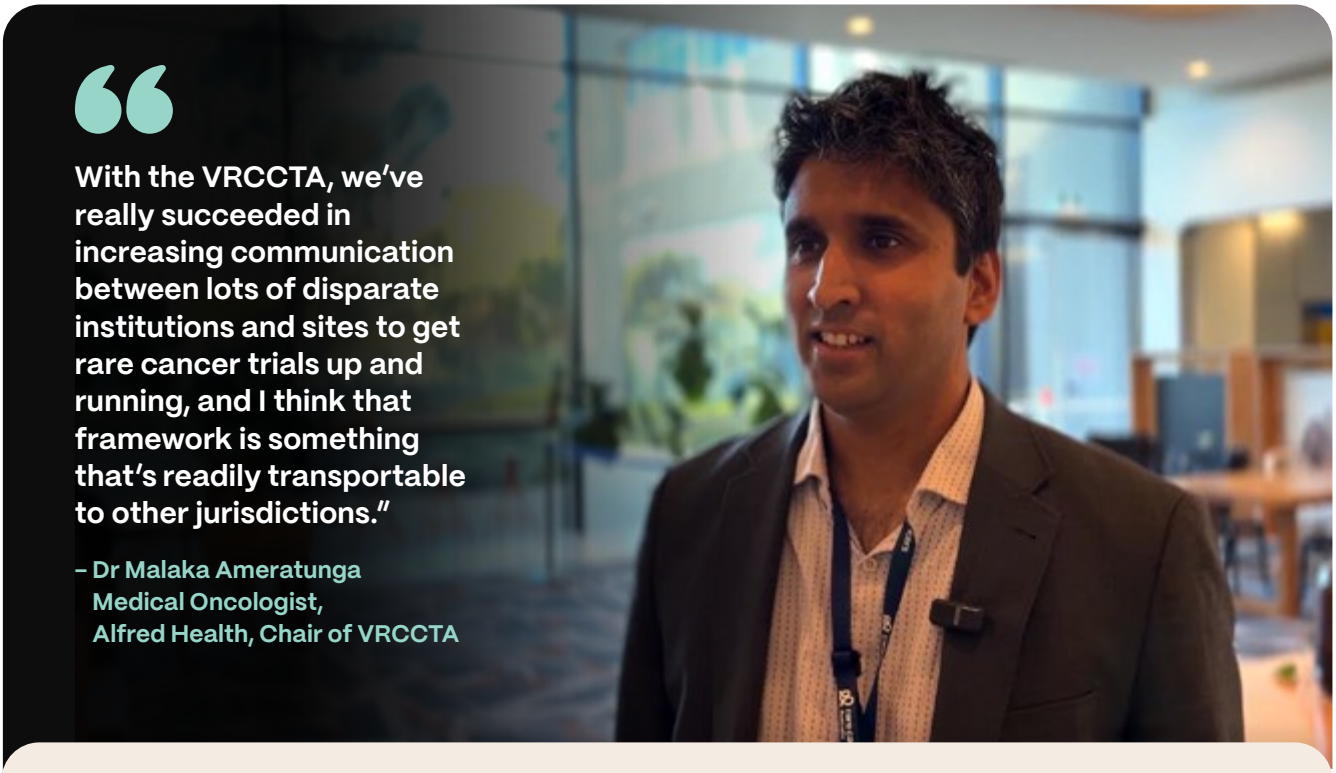
To realise these benefits, Australian health services engaged in clinical research must allocate sufficient time and capacity for clinicians to participate in both active and upcoming trials. Attendees emphasised the importance of introducing institutional performance metrics that explicitly recognise and support the conduct of trials in rare cancers and other high-unmet-need indications.

Clinicians attending the series also reflected on the significant personal commitment required to support clinical trials, often undertaking trial-related responsibilities in addition to their standard clinical workload and outside normal working hours. Attendees noted that participation in rare cancer trials frequently relies on a small number of highly dedicated oncologists who are willing to invest substantial personal time in conducting and advocating for research in this space.

It is encouraging to see future funding and grants – with explicit reference to rare cancers being released through the Medical Research Future Fund (MRFF)[13]. The continuation of these types of fellowships and targeted funding streams that prioritise rare cancer clinical trials represents a clear and positive commitment to improving access and research locally[12]. Sustained investment of this kind is essential to ensure clinicians have the capacity and protected time required to design, lead and deliver rare cancer trials, and to build long-term expertise within Australia's clinical research workforce.

The roundtable discussion also focused on strategies to broaden clinician networks beyond established metropolitan hubs, with particular emphasis on improving education and engagement for fellows and trainees across medical disciplines, as well as the importance of extending these efforts to clinicians practising in regional, rural, and outer metropolitan areas to support more equitable access to clinical trial opportunities.

For clinicians, clear, contemporaneous information about the patients they are consulting, and the trials that are available can increase efficiency and lessen burden, in trial connection.



With the VRCCTA, we've really succeeded in increasing communication between lots of disparate institutions and sites to get rare cancer trials up and running, and I think that framework is something that's readily transportable to other jurisdictions."

– Dr Malaka Ameratunga
Medical Oncologist,
Alfred Health, Chair of VRCCTA

The Victorian Rare Cancer Clinical Trial Alliance (VRCCTA)

The VRCCTA exemplifies collaborative network-based approaches to improving outcomes for people with rare cancers through clinical trials. The Alliance brings together oncologists, research nurses, trial coordinators, and specialist clinicians from metropolitan and regional institutions across Victoria to support early identification of rare cancer patients and timely referral to appropriate trial opportunities.

Under the current chairpersonship of Dr Malaka Ameratunga, and previously Professor Hui Gan, the VRCCTA has established strong partnerships with 17 Victorian trial sites and 18 commercial sponsors and contract research organisations. The Alliance also maintains active connections with government agencies and broader cancer collaborative groups, strengthening coordination across the clinical trial ecosystem.

Rather than sponsors approaching individual sites sequentially, the VRCCTA assesses rare cancer trial feasibility at a network level. While a single site may lack the capacity to initiate a study or recruit sufficient patient numbers, coordinated participation across the Alliance enables cross-referral and shared recruitment, increasing the likelihood that rare cancer trials can be activated and successfully recruited to. This shared model distributes responsibility and mitigates the inherent uncertainty associated with rare disease studies.

The Alliance also undertakes capability mapping of local laboratory services, providing sponsors with early clarity on which protocol-specific tests can be supported during feasibility assessments. In parallel, the VRCCTA proactively reviews the pipeline of rare cancer trials, provides protocol input at early feasibility stages, and engages with collaborative tumour groups to inform tumour-specific clinicians of upcoming trial opportunities.

The VRCCTA promotes a 'just-in-time' recruitment model, underpinned by consistent and open communication between sites and investigators within shared geographic and health service regions. The Alliance demonstrates what coordinated, clinician-led collaboration can achieve and provides a practical model for strengthening the rare cancer clinical trial landscape nationally.

Empowering and supporting patients

Finding and making informed decisions about clinical trials

Learnings from RCA's Cancer Lived Experience Survey reinforce the central role of specialists in trial awareness. Four in five (83%) respondents who had participated in a clinical trial said they had first heard about trials as a treatment option through their specialist (including oncologists, haematologists, and surgeons). Others identified general practitioners, nurses, personal online searches, and patient communities or forums as sources of information[7].

> **We've heard from our patient community that information access can be really challenging but is an incredibly important thing."**
>
> **– Bridget Bradhurst, Chief of Advocacy, Ovarian Cancer Australia**

Attendees discussed opportunities to increase general awareness of clinical trials to reduce the dependency on specialists and ensure more people are made aware of trials.

As clinical trials are experimental, there can be misconceptions and stigma about their safety and legitimacy. Attendees noted that these misunderstandings – such as fears of not receiving active treatment or beliefs that trials are only a last resort – can undermine efforts to engage effectively with both patients and carers. They emphasised the importance of normalising, rather than promoting, clinical trials by directly addressing misconceptions and challenging misinformation. Doing so is essential for building patient confidence, supporting informed decisions, and enabling fairer access to trials.

Clinical trial navigators, cancer navigators and patient organisations were recognised as important sources of information and support for patients who are considering trials. These roles can significantly improve awareness, and facilitate information sharing, particularly for patients, who are often unfamiliar with how to navigate the clinical trials system. This support is most effective when delivered in collaboration with treating clinical teams.

2025 Cancer Lived Experience Survey

How did you first learn that clinical trials were a cancer treatment option for you?

(208 responses, prompted if respondent said they had participated in a clinical trial)

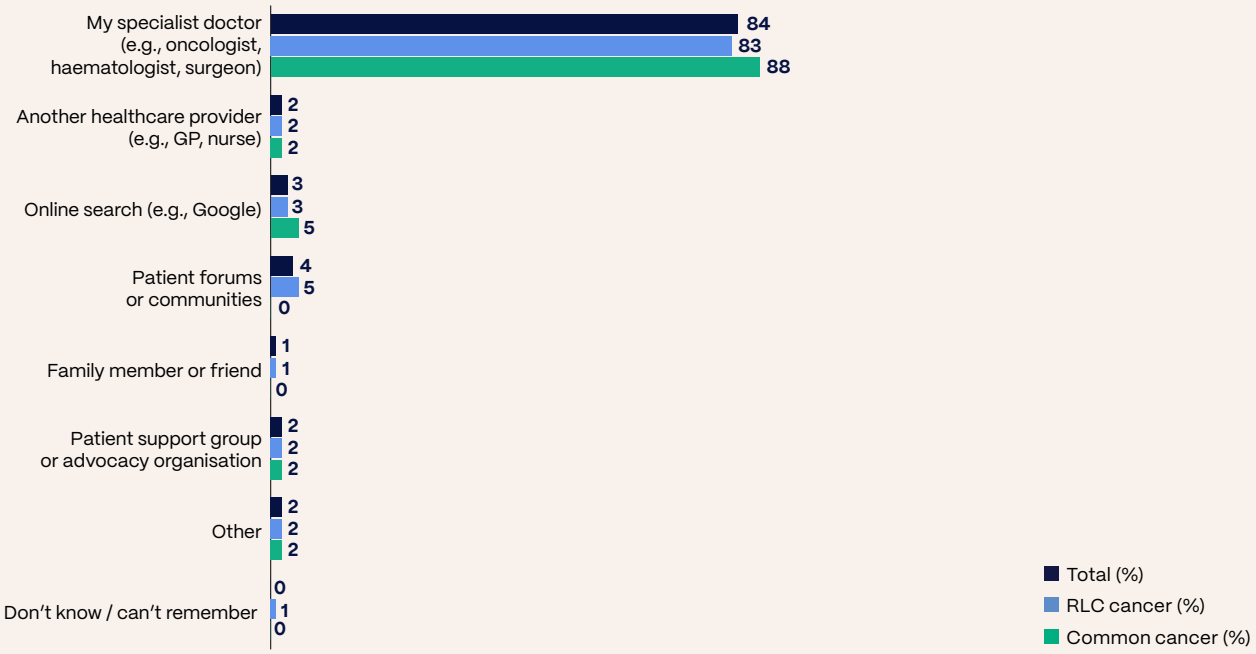


Figure 2. Source: Cancer Lived Experience Report (2025, p. 41)[7].

Patient organisations are also well placed to identify emerging and common issues affecting patients, drawing on collective experience to highlight the concerns most pressing for the community at any given time. Ovarian Cancer Australia's qualitative research into the perspectives of Australians with ovarian cancer including how they learn about, access and participate in clinical trials found that patients are motivated not only by personal benefit, but also by altruism and a desire to contribute to future advances, a sentiment shared by patient advocate speaker Eva Bishop. At the same time, participants expressed a strong preference for a centralised and credible source of information to help them independently identify relevant trial opportunities[33].

People with lived experience shared that the decision to take part in a clinical trial is not an automatic 'yes' and that reliable and understandable information is crucial. Possible side effects, the intensity of the treatment schedules and how a clinical trial may impact a patient's life and family come into consideration.

> **"Please remember that we are terrified and looking for information. Information gives us a sense of control over the situation however tenuous, about our treatment options and about the experiences we might expect to have. My experience with the support groups is that a lot of people are looking for treatment options outside of their clinicians' appointments also and we only have one person to think about. Having patient accessible information about available clinical trials opens the door to a greater audience and potential candidates."**
>
> **– Bronwyn White, Patient advocate**

Supporting patients to take part in clinical trials

Attendees identified that embedding patient perspectives from the earliest stages of trial design delivers benefits for everyone. Early and meaningful patient engagement improves awareness among clinicians and patients of upcoming trials, supports sponsors to meet recruitment milestones with greater confidence, and, most importantly, enables more equitable patient participation. This approach enhances the overall patient experience, from trial entry through to participation and closure.

Clinical trials can place significant demands on patients. These requirements are designed to ensure the safety of patients and to ensure that robust, decision-ready data is generated. While some assessments are widely regarded as immovable elements of a study's schedule – particularly those critical to time-sensitive primary endpoints, such as pharmacokinetic blood sampling – discussions with trial participants and study sites indicate that not all visits carry the same level of data collection or burden for patients. Certain study visits are relatively low impact, potentially involving little more than a brief consultation with the investigator, routine vital signs, and limited safety blood tests.

For patients travelling interstate – or even within metropolitan areas – these visits can be frequent and disruptive, compounding logistical and personal burdens while work and family responsibilities continue. Attendees emphasised the importance of recognising and addressing avoidable burdens to patients, particularly where flexibility can be introduced without compromising scientific integrity. Sponsors and trial sites should ensure timely reimbursement of patient costs, including travel and accommodation, to eliminate or minimise out-of-pocket expenses during trial participation, thereby improving the clinical trial experience and reducing financial barriers for rare cancer patients.

Encouraging the use of accredited local pathology laboratories and imaging services was also identified as a potential method to improve patient experience. However, this approach must be applied with caution, as it relies on the ability of local providers to meet consistent standards and deliver comparable results across jurisdictions in Australia – early consultation with sponsor and sites is needed for meaningful implementation.

Bronwyn's story

Our first keynote speaker in Melbourne considers herself as one the luckiest cancer patients in Australia, with an experience that she believes should be the standard of care, yet one that remains out of reach for many, due to gaps in referral pathways, precision medicine access, and awareness of early phase trial potential.

Bronwyn White, living with malignant peripheral nerve sheath tumour, a type of soft tissue sarcoma.

"My cancer story is five years in the making, starting in late 2020, where after requesting an ultrasound from my GP, I was told that I had an 11cm mass in the muscle. Possibly, likely, cancer.

Even before I had left the room, the GP contacted a cancer liaison, who called until I had an appointment with a specialist the very next day. In a whirlwind of scans and biopsies, I was diagnosed with malignant peripheral nerve sheath tumour sarcoma.

During the next years, I underwent radiation and surgery and was able to keep some semblance of normalcy in an otherwise very un-normal situation. Until scans revealed that my cancer had metastasised, with lesions popping up around my body. I was worried and so was my team.

My clinical team had the foresight to have genomic testing performed on my tumour, revealing an oncogene that can be targeted, making me eligible for clinical trials at the Peter MacCallum Cancer Centre. I joined a trial in February 2022, and by May 2024, my lesions had reduced by 94%. For three and a half years, I remained on that same treatment – at first through an early phase trial and then through a compassionate access program, once the trial ended.

I reflect on what went right for me in my cancer care, and how it happened. I had a GP that was able to connect with a liaison and then a specialist. I had genomic testing performed so that when the time came, I could be indicated for not one, but two clinical trials. I live in Melbourne near the world class Peter MacCallum Cancer Centre, full of clinicians who are connected and aware of the trials available.

Connecting with other patients, I know that this isn't always the case. For some indications, there are no trials available. Those living rurally cannot realistically participate without completely upheaving their lives at great expense. Some are never made aware or have trials discussed at all.

I strongly believe genomic testing should be standard for all cancers, particularly rare, with systems in place to help GPs and clinicians be aware of and connect patients, everywhere, with potential clinical trials."

Raising the bar for rural and regional access

While there has been a steady increase in the number of sites opening clinical trials across Australia over the past 10 years, this growth has been concentrated predominantly in metropolitan centres, particularly larger cancer centres have more capacity to undertake trials, with more larger cancer centres that have more capacity resources including staff, laboratories, imaging and pharmacies [10]. In a rural or regional setting, the lack of such resource is a significant disadvantage to attracting clinical trials, leaving rurally based patients having to travel to participate in a clinical trial, or not participate at all. Attendees remarked that not all patients are able to travel to take part in a trial – particularly trials with intensive study schedules. And for those who are able, the time away from home and the costs of travel and accommodation can be difficult for them and their loved ones.

> **"For our rural and regional participants, if they have to travel and there isn't any rapid participant reimbursement, then it makes it really difficult for them to participate."**
>
> **– Narelle McPhee, Cancer Research Manager, Bendigo Health**

Attendees across the series agreed that investment in rural and regional clinical trials in Australia delivers clear value. Expanding trial activity beyond metropolitan centres increases the likelihood of successful recruitment for sponsors by improving access for rural and regional patients, while also enhancing the participation experience for individuals who would otherwise be required to travel long distances or stay away from home for extended periods. Additional benefits identified included the development of capability and experience within rural trial sites and an enhanced local workforce. However, attendees also noted that these benefits rely on successful delivery, as where rural sites are unable to meet trial requirements, there may be uncertainty in engaging with them for future studies.

Attendees discussed measures needed to ensure clinical trials can be delivered effectively with the importance of thinking differently about trial design and delivery – creating a future in which participation of rural sites in trials becomes the norm rather than the exception, so that regional and rural Australians are included and better supported to take part.

The use of a 'just-in-time' ethics model to support rural site participation and faster trial activation was explored. Under this approach, ethics approval is sought for multiple potential sites during the initial review process, with some sites remaining dormant on the HREC approval[34]. This enables sites to be activated rapidly when an eligible patient is identified, reducing administrative start-up timelines and allowing local patients to be screened and enrolled more quickly.

It was noted that for this to be successful, there needs to be open communication and activity from ethics committees, sites and sponsors, with a clear understanding of each location's capabilities – to ensure that collected data is of high quality, and that participants are monitored safely.

The use of telehealth was a recurring theme for improving patient experience and broadening access to trials, particularly in supporting rural participation. Virtual study visits and assessments can reduce travel burden for patients, with certain clinical procedures, such as blood tests, conducted through local pathology services. This model also enables a separation of responsibilities between metropolitan and regional hospitals, where visits that require infrastructure or capability that isn't available at a regional site can be undertaken at a metropolitan site. However, less complex visits can be supported at the regional centre, significantly minimising a patient's travel burden and simultaneously supporting regional workforce development.

For rare cancer patients, who may have only a single suitable trial available nationally, geographic location can be a deciding factor in whether participation is possible.

The tele-trial model is an approach to decentralised clinical trials, using telehealth to link a primary trial site with separate satellite sites[35]. With dedicated funding announced in 2020 to support its expansion across six Australian states, the tele-trial model aims to build trial capability and infrastructure in regional and remote locations while enabling participants to be monitored and supported at local health services[36].

While the tele-trial model introduces additional regulatory and governance complexity for ethics committees and sites, with consideration of possible increased costs for sponsors, attendees emphasised that improved recruitment efficiency and the enhanced likelihood of patients remaining on trial can offset these challenges.

Participants highlighted that successful implementation requires coordinated approaches to travel assistance and site administration, to ensure geographic location does not remain a barrier to accessing appropriate clinical trial opportunities.

A family-centred approach to delivering paediatric clinical trials was highlighted, in which the entire family is considered the 'patient'. Some attendees noted that for families from regional or remote areas, reimbursement for travel and accommodation to metropolitan centres from sponsor companies can extend beyond the child to cover multiple caregivers and family members. Although drawing on concepts from specific study settings, the discussion reinforced that caregivers play a central role in a patient's treatment experience and in effective clinical trial participation. Caregivers often support the broader family unit, facilitate transport to trial sites, recall medical history and information, and often help manage treatment administration if given orally. Enabling and supporting caregivers is a critical mechanism for increasing and improving patient participation in clinical trials.

A commitment to clinical trial reform: The National One Stop Shop

The Australian Government has invested in, and is committed to, significant reform of the Australian clinical trial ecosystem through the **National One Stop Shop (NOSS)**[37]. The NOSS is predominantly envisaged to be a digital platform designed to streamline approval and reporting processes for human research across Australia.

Recent investments of $18.8 million in the 2024–25 Federal Budget, followed by a further $13.6 million investment through the MRFF, have supported the design and early development of the NOSS and foundational clinical trial reform[38].

The NOSS is being progressed under the leadership of Professor Ian Chubb, Chair of the Inter-Governmental Policy Reform Group (IGPRG), who has emphasised its potential to benefit people with rare cancers and researchers by improving transparency and accessibility across the clinical trial pipeline.

Attendees of the series highlighted the need for better, more accessible data to support decision-making by patients and clinicians and noted that Australian clinical trial start-up processes could be further streamlined. Discussion focused on how the NOSS, or any national clinical trials platform, could be implemented in a way that supports the rare cancer trial environment, rather than entrenching inequities or being unfit for that purpose.

To ensure patients can be appropriately assessed for eligibility, trial information made publicly available to patients and clinicians must be clear, accurate, and not misleading. For people with rare cancers, this includes transparent communication about specific eligibility requirements, such as biomarker-defined subgroups, as well as when and where trials are opening, recognising that site activations may occur in stages.

Participants also emphasised the importance of clear recruitment timelines, with regular updates indicating whether recruitment windows are being extended, closed early, or whether cohorts are opening on an intermittent basis.

By increasing visibility of trial start-up pathways, the NOSS has the potential to further strengthen international sponsor confidence in initiating trials in Australia and provide accurate, up-to-date information for patients, clinicians, and industry on upcoming and actively recruiting trials, including where they are being conducted[39].

If implemented effectively and supported by sustained investment, the NOSS has the potential to reduce fragmentation, further strengthen Australia's attractiveness to global sponsors, and deliver faster access to innovative therapies, particularly for people with rare cancers.

Attendees recognised that many elements of Australia's current clinical trial ecosystem already function effectively and cautioned the risk of diminishing these strengths through the introduction of a national system. They emphasised that the NOSS, and any other trial platforms, should be designed, implemented, and evaluated through active and ongoing engagement with clinicians, patients, and industry to ensure existing capabilities that underpin one of the strongest trial ecosystems in the world are preserved and enhanced.

Failed success: when trials do not translate into access

While Australia has some of the strongest clinical trial activity in the world, there has been comparatively slow growth in the number of therapies receiving public subsidy for rare cancer indications in Australia[40]. Only one quarter of innovative medicines supplied globally between 2016 and 2021 were listed on the Pharmaceutical Benefits Scheme (PBS) in Australia[41]. The average time from TGA registration to PBS listing for new medicines that improve health outcomes is 670 days[42], and for many rare indications that registration and listing will never come.

Innovative therapies at the forefront of modern medicine are currently being trialled and are likely to become the new standards of care for many rare cancers – yet the existing assessment frameworks aren't keeping pace with these advancements, resulting in delayed and less efficient patient access to emerging treatments.

This imbalance can leave patients in a precarious position following the conclusion of a successful clinical trial. Compassionate access schemes can bridge the gap, but when it is many years, or an indefinite period between a trial concluding and an application for registration and reimbursement, this can breed uncertainty and inequity. The broader patient population with the same diagnosis are unable to access the therapy in Australia until it has progressed through all regulatory and reimbursement assessment processes, if at all. Patients and clinicians spoke of the frustration they feel when access to a potentially effective therapy is held at the mercy of commercial decisions regarding whether and when therapies for rare indications are brought to the Australian market.

From an industry perspective, the uncertainty and difficulty of regulatory processes were also considered to be frustrating. Many agreed that improved clarity and predictability regarding the likelihood of successful market access could be an important driver of increased, successful applications for registration and reimbursement.

Attendees highlighted the lack of traditional data and evidence as significant barriers to attain subsidised access for people with rare cancers. Discussions explored how complex HTA processes could be better adapted to accommodate evolving, biomarker–driven and tumour–agnostic therapies, and the ways in which greater flexibility in data collection, including better use of patient reported measures, could overcome those challenges. It was considered possible that evidentiary standards required by regulators could be maintained whilst navigating the increased uncertainty that comes with small patient populations.

Discussions at the roundtable series.

Lillian's experience

Lillian Leigh – navigating rarity in lung cancer

"I am standing here, 11 years after being told that I had six to nine months to live, because of a clinical trial. Through genomic testing, I discovered that my cancer was driven by a rare oncogene. I accessed non-subsidised, targeted therapy for months, but unfortunately could not tolerate the treatment.

Then something extraordinary happened. I connected with a medical researcher in the United States who had the same rare oncogene-driven lung cancer. He told me about a Phase I clinical trial in Melbourne, a first-in-human trial for a new targeted therapy. He assured me about the trial schedule, what to expect, he made it seem possible. I told my clinician, and the screening visit was organised.

I started flying from Sydney to Melbourne, initially every week, then every three weeks, then every six weeks. It was exhausting. It was expensive. It meant time away from my daughter, from work, from normal life. But my tumours began to shrink all over my body.

Eventually, thanks to a sponsor decision, I was able to move my visits to Sydney. The difference that made to my life and ability to sustain treatment alongside it is immense. The trial finished years later, and I have been able to access the medicine via compassionate access ever since.

The medication is approved by the FDA. It's approved by the TGA. It's now on the PBS for another cancer indication which is around four or five times larger in population size. But that approval does not extend to my rare cancer.

Why? I don't think it's because of science. I think it's because it's rare. It's because of a commercial reality. But we do need to find solutions for the patients caught in this gap between what's commercially viable and what's medically necessary."

Navigating evidentiary uncertainty

Attendees discussed the considerations faced by HTA assessors and decision-makers when assessing clinical trial data and supplementary evidence to approve and subsidise medicines for rare cancer indications. In rare cancers, evidentiary packages are often constrained by small patient populations, resulting in trials that are smaller and associated with greater statistical uncertainty compared to clinical trials in more common cancers, where thousands of patients may be recruited globally to demonstrate efficacy and safety endpoints[1].

There was recognition that achieving more equitable access to new treatments requires decision-making frameworks capable of navigating this uncertainty without compromising evidentiary standards. Reimbursement decisions must continue to demonstrate clinical benefit, safety and value for taxpayer investment. While the roundtable identified strategies to strengthen rare cancer trial activity, including increasing Australia's participation in global studies and improving recruitment, it was acknowledged that, for some rare cancer cohorts, patient numbers will remain inherently limited. In these circumstances, supplementary evidence sources may be required to support regulatory and reimbursement submissions.

The growing use of real-world evidence to complement trial data was highlighted as a key development. Participants noted increasing acceptance of this evidence in submissions for rare conditions and its growing acceptance in health technology assessments, but that more guidance on acceptable and beneficial use of real-world data were needed.

- **Real-world data** – defined by the International Network of Agencies for Health Technology Assessment as data collected during the routine delivery of health care, outside clinical trial conditions.
- **Real-world evidence** – is the clinical evidence regarding the usage and potential benefits or risks of a health technology derived from analysis of real-world evidence[1].

Participants noted that the more submissions incorporating real-world data and evidence are accepted and recommended for listing, the more likely it will be that manufacturers and sponsors will gain confidence in submitting future applications that also utilise these types of evidence. Clear and transparent feedback between the Department of Health, Disability and Ageing, PBAC and sponsors regarding evidentiary expectations and the rationale underpinning decisions was identified as critical to supporting more consistent and more timely uptake.

Other novel approaches to data collection and analysis, including the use of synthetic data or emulation trials, the extrapolation of trial results across population-level linked data sets and tumour-agnostic analysis, were seen as emerging fields with great promise but with a current need for caution. The rapidly evolving capability of artificial intelligence was seen as an enabler, for example using advanced statistical modelling and real-world data to replicate the variability and structure of clinical datasets, thereby generating 'synthetic data' for evaluation purposes[43].

In the context of small patient populations, it is essential to maximise the collection of meaningful data. Some attendees also noted the restrictions and rigid approaches that can exclude patients from data reporting, which is particularly detrimental when working with a very small population. One example of a more flexible approach was to use multiple comparators where there was not a defined standard of care, rather than just one other treatment as a comparator. Maximising data was considered important not just in demonstrating efficacy and safety, but also in showing the value for patients and what they consider most important, particularly for indications that are rare and less well-researched or documented. Attendees noted there can be a disconnect between trial endpoints and patient values and preferences, with an opportunity to better integrate patient-reported outcomes and social value into HTA frameworks. This should be achieved alongside a continued focus on maintaining a positive and supportive clinical trial experience for participants.

Gaining insight from patient reported measures (PRMs)

Patient reported outcome measures (PROMs) and experience measures (PREMS) are structured assessments that capture what patients themselves are experiencing in their healthcare, including when participating in clinical trials[44]. While PRMs are not the only way patients communicate their experiences, they are a vital component of a more responsive and person-centred cancer system. The Australian Cancer Plan identifies the use of patient reported measures as a key enabler of person-centred, high-quality cancer care[45].

Attendees discussed the types of data that can be obtained through PROMS and PREMS and highlighted that it is not always apparent to the trial participants exactly how the data is used as evidence – sometimes seen as an afterthought, with assessments and treatment decisions largely dominated by clinical indicators such as tumour size or biomarker presentation. PRMs can include patient reported severity of side-effects through patient diaries, efficacy questionnaires and overall experience of the trial and treatment[46]. For health technology assessors, these measures can present valuable information as clinical trial endpoints that demonstrate meaningful improvements in quality of life. They may also be incorporated into economic evaluations, contributing directly to the estimated benefit of a medicine as HTA bodies assess submissions for public subsidy[47][1].

> **"I often wished the questionnaire could capture the things that really mattered to me—how the trial drug allowed my husband and me to continue working and taking care of our family. How my celebration milestones impacted my community. The ordinary moments that became extraordinary because I was alive to experience them."**
>
> **– Lillian Leigh, Patient advocate**

Participation in a clinical trial is influenced by many factors such as logistics, family situation and the quality of life the patient wishes to maintain. Integrating questions in PRMs to capture these considerations not only strengthens the evidence base for future submissions but also helps trial teams to better understand where the pressure points are for patients, identify opportunities to improve the overall experience and help to support patients to stay on a trial by managing side effects and other patient reported issues.

Regardless of these formal measures, when sites listen to patients and sponsor companies are receptive with intent, favourable protocol amendments can occur without compromising data quality or patient safety. These amendments may include adjustments to study schedules or the provision of additional logistical supports, such as travel and accommodation assistance, to enable patient participation and support future recruitment. Attendees emphasised the importance of open, direct communication between patients, site staff, and sponsors to support continuous improvement.

An opportunity for systemic change

Within HTA processes, higher levels of uncertainty are typically associated with lower prices at which the government is willing to subsidise medicines – sometimes too low for manufacturers to accept, leading to protracted negotiations where all parties suffer, none more so than patients who are awaiting access[48].

While the discussion focused on approaches to address uncertainty, participants also noted that increasing the government's willingness to pay could enable access to medicines for rare indications where uncertainty is unavoidable. Attendees emphasised that for rare cancers with high unmet clinical need, existing value and subsidy thresholds may need to be reconsidered to better reflect the realities of limited evidence and the potential patient benefit.

By recognising the need for a different approach on rare, rather than continuing in the same way with the same result of people with rare cancers and subtypes being left without equitable access, attendees saw potential to overcome many of the current barriers. A promising example of this was the pan-tumour or tumour-agnostic assessment of medicines. Since the conclusion of the roundtable series, PBAC has made two positive recommendations for a broad listing of immunotherapy for people with any type of advanced or metastatic cancer, including rare indications that are not yet registered by the TGA[49], enabling based on clinical judgement, using the latest evidence of potential benefit or harm to assess a patient's eligibility rather than their cancer type alone. One of these recommendations is now a reality, with a world-first tumour-agnostic PBS listing made from 1 March 2026[19]. This is a significant step towards equity of access for those patients who could benefit from immunotherapy and a welcome signal of the maturation of Australia's registration and reimbursement landscape and the ability to find workable solution for rare populations. Attendees were buoyed by the prospect of applying similar concepts in other therapy areas.

It was widely recognised in discussions that Australia's HTA system is outdated and lacks the agility needed to respond to a rapidly evolving therapy landscape. Many references were made to the potential impact of long-awaited reform through implementation of *The Health Technology Assessment Policy and Methods Review: Final Report* – published in September 2024 with 50 recommendations to improve the efficiency of health technology evaluation and deliver faster, more equitable access to medicines[1]. This was recognised as a significant opportunity to strengthen systems in a way that can benefit patients, clinicians, sponsors and the entire clinical trial industry over the coming years. Importantly, the increased focus on the assessment of high unmet clinical need areas, such as rare cancers and horizon scanning was considered a pivotal moment to shift from a reactive to a proactive approach to attracting the trials and therapies that Australian patients need. Several recommendations within the HTA Review came up in discussions, including:

Better and more consistent collection and use of real-world data.
– Recommendations 27 and 31

Methods for assessing non-randomised and observational evidence.
– Recommendations 35

Guidance on methods for assessing tumour-agnostic therapies.
– Recommendations 38

Identification of areas of high unmet clinical need and faster access to medicines for these populations.
– Recommendations 8, 20 and 44

Alongside HTA reforms, the NOSS initiative and the increased focus on expanded access to genomic-led cancer care were considered critical opportunities to embed the specific needs of people with rare cancers in policy and system change as initiatives are implemented over the coming decade. Attendees emphasised the importance of integration and using these reform initiatives to build on what is working well, in a way that promotes the critical role of clinical trials in enabling faster patient access in the near term and long term.

Realising the potential in *rare*

Clinical trials provide patients with earlier access to innovative therapies, with the potential to improve health outcomes and advance standards of care. They also deliver substantial benefits to Australia, driving investment, skilled employment, and strengthening our national research capability.

Through this three-part roundtable series, participants identified barriers and practical opportunities to strengthen Australia's strong yet imperfect cancer clinical trials ecosystem, to deliver even greater access and benefits for people with rare cancers in Australia. There was a united recognition of the need to work collaboratively and across sectors to maintain and boost the local and national clinical trial capability and capacity and further incentivise the initiation of rare cancer trials in Australia; and improve referral pathways, the quality and accessibility of clinical trials information, and they ways in which patients and clinicians quickly and effectively find and access clinical trials. Further, collective efforts to modernise data governance and evidence frameworks and adopt different approaches for rare population can support more effective use of data and evidence generated through rare cancer research for regulatory approval and reimbursement, giving patients faster access to promising therapies.

What is evident from the series is that progress will require sustained, deliberate collaboration between government, regulators, industry, clinicians, researchers, and patient communities to translate discussion into measurable reform.

Panel members at Sydney roundtable.

How to maximise clinical trial opportunities for people with rare cancers

1 Make it easier for rare cancer patients to identify, access and participate in clinical trials

- Support strong rare cancer indication networks, and early phase clinician networks and promote cross-network collaboration on a national scale.
- Embed consumer input into protocol development and trial delivery, including routine use of patient-reported outcome and experience measures across all cancer clinical trials.
- Encourage sponsors to engage with rural and regional sites early and support flexible or decentralised delivery models to improve geographic equity.
- Sponsors should collaborate with patient organisations to strengthen patient and public facing information and resources, including accessible and multilingual materials.
- The Australian Government should improve the national clinical trial registry through the National One Stop Shop (NOSS), by co-designing with patients with rare cancers and rare genomic subtypes, to provide accurate, timely and accessible information about available clinical trials.

2 Support and empower clinicians (investigators) to maximise clinical trial opportunities for rare cancer patients, particularly early phase

- The Australian Federal Government should increase funding for clinical trials activities in Australia, prioritising targeted fellowships and grants that give investigators the protected time needed to drive meaningful protocol development and feasibility assessments.
- Educate clinicians on the value of collaborating closely with commercial sponsors to broaden patient inclusion criteria and to consider rare cancer indications where feasible, particularly within early phase clinical trials.
- Educate clinicians on the value of networked approaches to support timely connections between their patients and potential clinical trials.

3 Improve the efficiency and harmonisation of ethics and governance processes

- Facilitate faster patient access and strengthen Australia's attractiveness to commercial sponsors by establishing dedicated, prioritised review pathways for clinical trials focussed on rare cancer populations within Human Research Ethics Committees (HRECs) and site-specific governance processes.
- Implement national initiatives, such as the proposed NOSS, in a manner that improves efficiency across all institutions in regulatory and start-up activities, while preserving and protecting the performance of internationally renowned Australian trial centres.

4 Proactively increase the number of rare cancer clinical trials conducted in Australia in particular Phase I trials

- Government, clinicians, trials groups and local affiliates of commercial sponsors, such as contract research organisations, should actively preserve and promote Australia's clinical trial capabilities internationally. Promotion should highlight Australia's exceptional start-up timelines, steady regulatory framework and financial incentives. Focus should be directed toward the country's strong early-phase trial capacity, which represents a significant opportunity for expanding access for patients with rare cancers.
- The Australian Government should accelerate the adoption of broader access to genomic testing for people with rare cancers, and a national genomic data registry, to maximise the efficient identification of patients who could be suitable for molecularly targeted clinical trials. Helping to maximise clinical trial recruitment.

5 Strengthen the pathway from clinical trials to subsidised access

- The Department of Health, Disability and Ageing should immediately clarify the evaluation of real world evidence, patient reported measures, and non-randomised and tumour-agnostic clinical trial designs in regulatory processes and decision-making, to encourage their use.
- The Department of Health, Disability and Ageing should issue clear guidance on how evidence-generation models can be utilised within HTA to safely and efficiently broaden medicine approvals for rare and molecularly defined cancer populations.

References

1. Australian Government Department of Health Disability and Ageing. *Health Technology Assessment Policy and Methods Review – Final report*. 10 September 2024. [cited 1 February 2026]. Available from: www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-final-report
2. Rare Cancers Australia. *Now it's personal: Access and equity for all*. 2025. [cited 1 February 2026]. Available from: www.rarecancers.org.au/reports-submissions/now-its-personal-access-and-equity-for-all/
3. Senate Standing Committee on Community Affairs. *Equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer*. 2024. Parliament of Australia. [cited 1 February 2026]. Available from: parlinfo.aph.gov.au/parlInfo/download/committees/reportsen/RB000168/toc_pdf/Equitableaccesstodiagnosisandtreatmentforindividualswithrareandlesscommoncancers,includingneuroendocrinecancer.pdf
4. Cancer data in Australia [Internet]. 2025 [cited 1 February 2026]. Available from: https://www.aihw.gov.au/reports/cancer/cancer-data-in-australia/contents/overview
5. Australian Clinical Trials. *What is a clinical trial?* 2023 [updated 8 November 2023. Cited 2 February 2026]. Available from: https://www.australianclinicaltrials.gov.au/about/what-is-a-clinical-trial
6. Clinical Trials [Internet]. n.d. Medicines Australia. [cited 2 February 2026]. Available from: www.medicinesaustralia.com.au/policy/clinical-trials/
7. Rare Cancers Australia. 2026. *Cancer Lived Experience Survey Report 2025*. [Cited 10 February 2026]. Available from: www.rarecancers.org.au/advocacy/rare-cancer-lived-experience-survey/
8. Kelly R, Guo, C., Desai, J., Tran, B. *Changing trends in phase 1 oncology clinical trials*. Contemporary Clinical Trials Communications 2024;37:*101239*.
9. Hitchen N, Shahnam, A., Manoharan, S., Topp, M., Mileshkin, L., Lim, A. M., Whittle, J. R., Luen, S. J., Solomon, B., Lackovic, K., Desai, J., & Tran, B. *Trends in phase 1 oncology clinical trials across Australia; Analysis of ClinicalTrials.gov 2012–2022*. Asia-Pacific Journal of Clinical Oncology. 2024;20(6):731–7.
10. MTPConnect. *Australia's Clinical Trials Sector: Advancing innovative healthcare and powering economic growth*. 2024 [Cited 10 February 2026]. Available from: mtpconnect.org.au/wp-content/uploads/2025/07/MTPConnect-2024-Australias-Clinical-Trials-Sector-Report.pdf
11. Australian Government Department of Health Disability and Ageing. Medical Treatment Overseas Program: Commonwealth of Australia, 2026 [updated 2 January 2026. Cited 2 February 2026]. Available from: www.health.gov.au/our-work/medical-treatment-overseas-program.
12. The Department of Finance. *Grant Opportunity GO7923*. Australian Government. 2025. [Cited 10 February 2026]. Available from: www.grants.gov.au/Go/Show?GoUuid=328e663e-0b64-442a-84dd-574039f451dd
13. The Department of Finance. *Grant Opportunity GO8232*. Australian Government. 2026. [Cited 10 February 2026]. Available from: www.grants.gov.au/Go/Show?GoUuid=01b50890-22c3-449b-9f1b-1d44b77d7e37
14. Australian Trade and Investment Commission. *Australia presents first class clinical trials capability to the world*. Austrade. 2025. [cited 10 February]. Available from: www.austrade.gov.au/en/news-and-analysis/media-centre/media-releases/australia-presents-first-class-clinical-trials-capability-to-the-world
15. Australian Health Research Alliance (AHRA). *Education platform expands nationally to enhance clinical trials workforce*. 2024. [updated 6 December. Cited 10 February]. Available from: ahra.org.au/2024/12/06/education-platform-expands-nationally-to-enhance-clinical-trials-workforce/.
16. Australian Government Department of Health and Aged Care. *MRFF – Rare Cancers, Rare Diseases and Unmet Need Clinical Trials Initiative*. Australian Government Department of Health and Aged Care. 2023. Available from: www.health.gov.au/our-work/mrff-rare-cancers-rare-diseases-and-unmet-need-clinical-trials-initiative#about-the-initiative.

17. Department of Industry Science and Resources. *Industry Research and Development (Clinical Trials) Determination 2022*. 2022 [Cited 10 February]. Available from: www.legislation.gov.au/F2022N00078/latest/text
18. Clinical Trials Project Reference Group. *National Standard Operating Procedures for Clinical Trials including Teletrials in Australia*. 2021 [Cited 2 February 2026]. Available from: www.australianclinicaltrials.gov.au/resources/national-standard-operating-procedures-clinical-trials-australia
19. Australian Government Department of Health, Disability and Ageing 2026, *World first cheaper immunotherapy treatment listing for patients with advanced or rare cancers*, Australian Government Department of Health, Disability and Ageing. [Cited 11 March 2026]. www.health.gov.au/ministers/the-hon-mark-butler-mp/media/world-first-cheaper-immunotherapy-treatment-listing-for-patients-with-advanced-or-rare-cancers
20. NSW Government 2026, *Funding for Australian-first alliance boosting access to life-saving clinical cancer trials, ministerial media release*, NSW Government, Sydney. [Cited 10 March 2026]. Available from: www.nsw.gov.au/ministerial-releases/funding-for-australian-first-alliance-boosting-access-to-life-saving-clinical-cancer-trials
21. National Health and Medical Research Council. *List of Human Research Ethics Committees registered with NHMRC*. 2026 [updated 4 February 2026. Cited 2 February 2026]. Available from: https://www.nhmrc.gov.au/research-policy/ethics/human-research-ethics-committees
22. National Health and Medical Research Council. National Mutual Acceptance of ethical and scientific review for multi-centre human research projects conducted in public health organisations [FACT SHEET, March 2024] Available from: https://www.clinicaltrialsandresearch.vic.gov.au/national-mutual-acceptance
23. Subbiah V, Othus M, Palma J, Cuglievan B, Kurzrock R. *Designing Clinical Trials for Patients With Rare Cancers: Connecting the Zebras*. American Society of Clinical Oncology Educational Book. 2025;45(3):e100051.
24. Jaki T, Burdon A, Chen X, Mozgunov P, Zheng H, Baird R. *Early phase clinical trials in oncology: Realising the potential of seamless designs*. European Journal of Cancer. 2023;189:112916.
25. Pant S, Schuler M, Iyer G, Witt O, Doi T, Qin S, et al. *Erdafitinib in patients with advanced solid tumours with FGFR alterations (RAGNAR): an international, single-arm, phase 2 study*. The Lancet Oncology. 2023;24(8):925-35.
26. Tateo V, Marchese PV, Mollica V, Massari F, Kurzrock R, Adashek JJ. *Agnostic approvals in oncology: Getting the right drug to the right patient with the right genomics*. Pharmaceuticals (Basel). 2023;16(4):#434.
27. Subbiah V, Kurzrock R. *Imperative of Comprehensive Molecular Profiling as Standard of Care for Patients With Rare Cancers*. JCO Oncology Practice. 2025;21(12):1734-7.
28. Novotech. *Precision Oncology Clinical Trials & Statistics 2024 – White Paper.* [Cited 2 February 2026]. Available from novotech-cro.com/whitepapers/precision-oncology-clinical-trials-statistics-2024; 2024.
29. OMICO. *Omico's impact report: From momentum to mainstream Delivering precision oncology for Australians with advanced and incurable cancers*. 2025. [Cited 2 February 2026]. Available from: omico.org.au/wp-content/uploads/2025/12/Omico-Impact-REPORT_Online-pdf-version_2025_FINAL.pdf
30. Cancer Australia. National Framework for Genomics in Cancer Control. 2025. [Cited 2 February 2026]. Available from: www.canceraustralia.gov.au/sites/default/files/2025-02/national-framework-for-genomics-in-cancer-control-cancer-australia-january-2025.pdf
31. Gobat N, Slack C, Hannah S, Salzwedel J, Bladon G, Burgos JG, et al. *Better engagement, better evidence: working in partnership with patients, the public, and communities in clinical trials with involvement and good participatory practice*. The Lancet Global Health. 2025;13(4):e716-e31.
32. Bentley C, Cressman S, van der Hoek K, Arts K, Dancey J, Peacock S. *Conducting clinical trials-costs, impacts, and the value of clinical trials networks: A scoping review*. Clinical Trials. 2019;16(2):183-93.
33. Williams N, Russell H, Bradhurst B. *Exploring clinical trials awareness, information access and participation amongst Australians with ovarian cancer: a qualitative study*. Supportive Care in Cancer. 2025;33(3):176.

34. Clinical Oncology Society of Australia (COSA). Pilot implementation of the Australasian tele-trial model: Access to clinical trials closer to home using tele-health (Completion Report). 2021. [Cited 2 February 2026]. Available from: www.cosa.org.au/media/332811/cosa-tele-trials-model-report-2021-web-final.pdf

35. Symons T, Woollett A, Zalcberg J, Eckstein L. *Implementing Decentralized Clinical Trials in Australia through Teletrials: Where to From Here?* Therapeutic Innovation & Regulatory Science. 2025;59(6):1199-203.

36. Underhill C, Woollett A, Buzza M, Freeman J, Evans W, McBurnie J, et al. *Implementing Teletrials to Improve Equity of Access for Regional Patients With Cancer: Report From the Victorian Teletrial Collaborative*. Asia-Pacific Journal of Clinical Oncology. 2026;22(1):50-8.

37. Australian Clinical Trials. National One Stop Shop for health and medical research: Commonwealth of Australia. 2025 [updated 26 November 2025]. Available from: www.australianclinicaltrials.gov.au/national-reforms/national-one-stop-shop-health-and-medical-research.

38. $13.6 million to cement Australia's status in world-class clinical trials. [press release]. Australian Government Department of Health and Aged Care, 2 October 2025. Available from: https://www.health.gov.au/ministers/the-hon-mark-butler-mp/media/136-million-to-cement-australias-status-in-world-class-clinical-trials

39. Australian Government Department of Health and Aged Care. *Budget 2024-25: Cheaper medicines*. Canberra: Australian Government. 2024. Available from: www.health.gov.au/sites/default/files/2024-05/budget-2024-25-cheaper-medicines_0.pdf

40. Rare Cancers Australia. Counting the cost: The true value of investing in cancer treatment. Accessed 1 February 2026. Available from: www.rarecancers.org.au/reports-submissions/counting-the-cost-the-true-value-of-investing-in-cancer-treatment/

41. Medicines Australia. *Pharmaceutical industry facts and the value of investing in medicines*. Medicines Australia.1 ed, 2025. [Cited 2 February 2026]. Available from: www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2025/08/MA-facts-and-figures-brochure_V1.0_280825.pdf

42. Australian Government Department of Health and Aged Care. FOI 25-0413 LD, FOI 25-0418 LD, FOI 25-0423 LD – Incoming Government Brief. 26 June 2025. Available from: www.health. gov.au/resources/foi-disclosure-log/foi-25-0413-foi-25-0418foi-25-0423-ld-incoming-government-brief-2025

43. Alloza C, Knox B, Raad H, Aguilà M, Coakley C, Mohrova Z, et al. *A Case for Synthetic Data in Regulatory Decision-Making in Europe*. Clin Pharmacol Ther. 2023;114(4):795-801.

44. Balitsky AK, Rayner D, Britto J, Lionel AC, Ginsberg L, Cho W, et al. *Patient-Reported Outcome Measures in Cancer Care: An Updated Systematic Review and Meta-Analysis*. JAMA Network Open. 2024;7(8):e2424793.

45. Cancer Australia. Australian Cancer Plan, *Strategic Objective 4.5.1, Design and embed patient reported experience and patient reported outcomes into national performance monitoring and reporting for all providers, to assess services for all population groups and establish an evidence base*. Cancer Australia; 2023 [Cited 2 February 2026]. Available from: https://www.australiancancerplan.gov.au/actions/4.5.1

46. Alger E, Aiyegbusi OL, Dueck AC, Minchom A, Pe M, Peipert JD, et al. *International Consensus-Driven Recommendations for Patient-Reported Outcome Research Objectives in Early Phase Dose-Finding Oncology Trials: OPTIMISE-ROR*. Journal of Clinical Oncology. 2026;0(0):JCO-25-01625.

47. Lambert S, McKenzie M, Coronado AC, Caissie A, Tracey L, Watson L, et al. *Real-World Patient-Reported Outcome Measure Implementation: Challenges and Successes of a Pan-Canadian Initiative to Improve the Future of Patient-Centered Cancer Care*. JCO Oncology Practice. 2025;21(8):1087-96.

48. Medicines Australia. *Managing uncertainty: Discussion Paper*. 2022. [Cited 19 February 2026]. Available from: www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2022/11/HTA-DP-Managing-Uncertainty.pdf

49. Pharmaceutical Benefits Advisory Committee. *PBAC meeting outcomes: December 2025 Intracycle meeting*. 2025.[Cited 19 February 2026]. Available from: https://www.pbs.gov.au/info/industry/listing/elements/pbac-meetings/pbac-outcomes

50. Pharmaceutical Research and Manufacturers of America. *Clinical Trials*. [Accessed 3 March 2026] Available from: https://phrma.org/policy-issues/research-development/clinical-trials

Facilitated Discussion Questions

Contact us

Enquiries about this report and RCA's advocacy:

policy@rarecancers.org.au

+61 2 4862 2768

Support for patients and their families:

support@rarecancers.org.au

1800 257 600

Send a letter:

Rare Cancers Australia
PO Box 440
Bowral NSW 2576

www.rarecancers.org.au

General enquiries:

info@ctaust.org

+61 3 8559 7244

www.cancertrialsaustralia.com