Submission - Review of the National Medicines Policy

October 2021

rare cancers AUSTRALIA

Introduction

Rare Cancers Australia Ltd (RCA) is a charity whose purpose is to improve the lives and health outcomes of Australians living with rare and less common cancers.

RCA has supported cancer patients with rare and less common cancers for the last decade. Through this shared journey we have come to learn what matters most to patients, their families, and carers.

We have also been made acutely aware of the health system's failure to meet the needs of cancer patients consistently, particularly those with rare and less common cancers on non-curative pathways.

Patients frequently approach RCA looking for help to access treatment options. In more recent years, our patient support navigators have started to receive a growing number of calls from patients with complex cancers that were historically considered 'common' and who have exhausted standard treatments.

There is growing demand to support these patients – to pay for diagnostic screening tests, to find and access clinical trial treatments, to participate in special access programs or to find ways to pay for therapies that are not reimbursed.

For Australians to benefit from a revised 'National Medicines Policy', the health system must develop and adapt in such a way that all Australians – not just the wealthy – have swift and affordable access to the best therapeutics, medical technologies (including diagnostic and screening tests) and vaccines.

RCA maintains that no patient, family, or carer should be significantly out of pocket in the pursuit of diagnosing and treating their disease. It is the fundamental right of every Australian to have access to the best health care at an affordable cost.

The recommendations herein have been informed by the experiences of seven hundred patients, families and carers who contributed to our research this year.

The Scope of the Policy

Consider the definition of medicines and whether the NMP needs to be expanded to include health technologies?

Should the current NMP definition of medicines be expanded to include medical devices and vaccines?

At the policy's inception twenty years ago, medicines were predominantly classified as small molecules that were used to treat disease in populations of hundreds, if not thousands, of patients. Most of the medicines were dispensed at the community pharmacy or within the public hospital pharmacy setting.

Today we are navigating a very different healthcare landscape; particularly in the diagnosis, treatment, and management of cancers where personalised approaches to treatment are being

adopted more readily. In addition, the model of treatment for cancer patients can be as complex as administering an infused cell and gene therapy at a highly specialised treatment centre (e.g., CAR-T therapy).

Today's cancer patient is facing a journey with an intersection of multiple treatment modalities that may include personalised targeted therapies, immunotherapies, genomic testing for molecular targets, gene therapies, advanced imaging, and/or artificial intelligence application with generation of 'big data'.

RCA maintains that the scope of the policy be broadened to include therapeutics (conventional medicines; inclusive of cell therapies, biologics, gene therapies and immunotherapies), as well as diagnostic tests including co-dependant technologies, other technologies (medical technologies), and vaccines.

Broadening the scope of the policy will prevent inconsistent application of principles and helps to deliver continuity in the provision of these products and services to Australian patients.

Given the convergence of treatment modalities, diagnostic tests, and therapeutic devices, an agile policy must adequately reflect modern treatment practices.

It is noted in the 'Discussion Document' provided by the Australian Government Department of Health, that *"…feedback on the inclusion of therapeutic devices was not universally held at the Stakeholder Forum."* It states, "*…some participants highlighted the risk that an expanded policy would diminish the focus on medicines and that the TG Act defines medical devices too broadly*."

RCA agrees that including devices within the policy adds an additional layer of complexity, given the broad definition of a 'prosthesis', including anything from an orthotic device to medical sutures. While less applicable for cancer patients, further work can be undertaken by the Review Committee to define what medical technologies and devices will be included and excluded from the policy.

Broadening the scope of the policy to include therapeutics, tests, medical technologies, and vaccines supports a consistent approach to the delivery of these products and services to patients. Furthermore, it provides a platform to remove inconsistencies in wholistic treatment delivery as the same principles are applied in the policy to all products and services used to diagnose, treat, and manage cancer.

This is critical for cancer patients who require multiple modes of treatment.

A broader scope of the policy allows further reforms of the programs that underpin the policy, in a manner that is consistent with the policy's objectives.

Programs include, but are not limited to, Therapeutics Goods Administration (TGA) Regulatory Framework, Medicare Benefits Scheme (MBS), the Pharmaceutical Benefits Scheme (PBS), the Prosthesis List, the Australian Immunisation Register (AIR) and the National Blood Authority (NBA).

This will contribute to the continuity of medical product delivery to Australian patients.

Recommendation - expand the scope of the policy to be inclusive of other therapeutic, medical technologies and vaccines

The policy's title would better be represented as: "The Australian therapeutics, medical technology, and vaccines policy".

The Proposed Principles of the Policy

Are these proposed principles appropriate? Regarding the proposed principles, is anything missing or needing to change?

RCA proposes the following changes and additions to the proposed principles:

Equity of patient access – every Australian will have rapid access to the most advanced, effective, safe, and high-quality, *therapeutics, medical technologies, and vaccines* at an affordable price when they need them. The products available to patients will be comparable to the best-in-class therapies, medical technologies, and vaccines available internationally.

All patients should have equitable access to information that is easy to understand in association with the delivery and availability of *therapeutics, medical technologies, and vaccines,* including information about available clinical trial opportunities.

All partners of the policy will ensure that equity is consistently upheld as a fundamental right for every Australian irrespective of their background or personal circumstance, and regardless of the type of disease or health condition they exhibit.

Person-centred approach – members of the community (patients or otherwise) should be equal partners in shaping the policy and the regulatory, clinical trial and reimbursement programs and frameworks that underpin it to deliver on the objectives of the policy.

The programs that underpin the policy must be transparent so that members of the public are empowered to contribute to the delivery of the programs actively and meaningfully.

Patients' lived experiences will be captured to ensure the success of the policy is measurable and can shape better outcomes for the patients and families of the future.

All diverse groups of people from the community should be engaged, including groups representing marginalised individuals from diverse backgrounds, as well as Aboriginal and Torres Strait Islander representatives.

Partnership-based – the policy should establish and maintain active, respectful, collaborative, and transparent partnerships with members of the health care sector and the community to harness experience, perspectives, and knowledge.

All stakeholders should be treated as equal contributors. Patients, their families, and carers can contribute invaluable lived experience that not only inform treatment of today but help shape optimal healthcare for patients on a similar pathway in the future.

Stakeholders should include:

- Federal, State and Territory Governments (government organisations and central agencies),
- Clinical health service providers (public, private, and not for profit),
- Non-clinical health services providers (such as social workers or patient support workers),
- Health care professionals (clinical groups, physicians, and allied health workers),
- Industry representatives (pharmaceutical, biotechnology and medical technology companies),
- Members of the public and patient representatives (Australian citizens, patient organisations, and groups representing the marginalised and culturally diverse).

Agility and adaptability – the programs that underpin the policy will be agile and adaptable to respond to the needs of the individual in an evolving therapeutic and technological landscape. Where circumstances challenge the standard pathways, novel approaches will be considered to uphold the fundamental right of patients to access the best therapeutics, medical technologies, and vaccines. This will involve an approach that embraces calculated 'uncertainty' that befits rare diseases and small patient populations.

Novel approaches may be supported through the provision of real-world evidence, including patient reported outcome measures (PROMs) and patient reported experience measures (PREMs), clinical trial treatment pathways, shared data, and application of artificial intelligence (AI) to inform value for money that is reconsidered as the evidence matures.

The policy must support new and flexible ways for governments to pay for clinical products and services.

Accountability and transparency – the policy should consistently deliver on its objectives in the real-world setting.

For the policy to be meaningful it must adopt a governance structure that keeps all stakeholders accountable to upholding the agreed objectives of the policy.

Measurement of an agreed set of key performance indicators and programme outcome measures will monitor how well the policy is delivering on its objectives. This ongoing monitoring and transparent public reporting should be conducted by an independent and appointed governance committee consisting of the full breadth of stakeholders across the sector (see 'Stewardship' below) including patients, carers, and their families.

Stewardship – as stewards of the health system, all stakeholders should have a shared responsibility to ensure that the policy's objectives are met in an equitable, efficient, and sustainable manner. Patients, families, and carers will have equal position in this relationship as they are the people who the policy is designed to serve.

All stewards should consent to uphold an agreed moral and ethical framework so that conflicting stakeholder interests do not circumvent achievement of the policy's objectives.

The Objectives of the Policy

Evaluate the current NMP objectives and determine whether these should be modified, or additional objectives included.

The COVID-19 world-wide pandemic has taught us that without good health, the entire country suffers. This is a vast simplification of a complex issue but ultimately, we cannot afford to not invest in medical/scientific innovation (the best therapeutics, tests, medical technologies, and vaccines) to keep the nation as healthy as possible.

Furthermore, investment in the infrastructure to support the local delivery of these products and services will provide significant employment opportunities and add to Australia's export industry.

Healthy lives = a healthy economy, which supports a thriving nation.

RCA recommends the aim of the policy be revisited and suggest the following:

Every patient has an equal opportunity to achieve optimal health outcomes and live better and longer lives – without distinction of rurality, race, economic or social condition – through access to the best therapeutics, medical technologies, vaccines, and related services at an affordable price for patients.

As taxpayers and recipients of these treatments, patients' lived experience of their disease, clinical interventions and outcomes will be captured to help inform best practice for the benefit of the Australians of tomorrow.

The system will be agile and adaptable to respond to the needs of individuals and where conventional pathways are hampered, novel approaches to fast, provisional funded access will be taken. These may be supported through the provision of real-world evidence, clinical trial treatment pathways, shared data, and application of artificial intelligence to inform value for money that is revaluated as the evidence matures.

Recommendation: revise the overarching objective of the policy to meet the needs of individual patients.

Are these four objectives still relevant? Should any be modified, or any additional objectives be considered? If so, how and why?

RCA believes the high-level objectives of the policy are relevant with suggestions to the following:

The right to equity of patient access to innovative therapeutics, medical technology, and vaccines.

Access and value for money

Every Australian patient should have rapid access to the best therapeutics, medical technologies, and vaccines when they need them at a cost they can afford.

Within the 'universal health care framework' the Commonwealth (and with CAR-T therapy, this extends to a shared expense between State and Federal governments) subsidises the cost through MBS and the PBS. Yet the reality is that patients presenting with rare or complex cancers are more readily bearing the costs themselves – if they can afford it.

In the current policy, affordability for the community through government subsidy is considered in the context of fiscal pressures, such as an ageing population. This suggests a careful approach to spending must be adopted.

The COVID-19 pandemic has caused significant pressure on the government's budgets, particularly health. The challenge of the next ten years will be to strengthen the economy and build resilient health systems. In contrast, a struggling economy requires further investment in health to deliver long-term benefits.

RCA recommends that the terminology of spending on promising therapeutics, medical technology and vaccines is changed from an 'expense' to an 'investment', considering the long-term benefits expected to be delivered to patients, families, society, and the economy. This expectation is driven by the pipeline of scientific and technological advances that will deliver more targeted and thus reliable outcomes.

The consideration of 'value for money' needs to be realistic, rapid, and periodically reviewed as evidence becomes available. It must be considerate of the wholistic view of value and include broader societal benefits as well as utilise real-world evidence. Societal measures should extend to the economic benefits delivered in association with health or quality of life gained that flows on to direct family members and generates productivity returns for the community.

Equity of access to clinical trials

For many rare and complex diseases such as cancers, clinical trials are the standard of care. Every person should have equal opportunity to be informed of an available clinical trial.

Greater and ongoing investment in local clinical trial recruitment and infrastructure should be supported by the policy.

Broader clinical trials inclusion criteria should be considered in a way that maintains the integrity of the clinical trial but enables more rare disease and complex disease patients to access novel treatments. Formal recognition of the role of clinical trials as a 'treatment option' is necessary. Furthermore, the policy should support ethical and moral considerations in the 'treatment' potential a clinical trial offers to cancer patients who have no other options.

Equity of access for marginalised populations

About a third of Australia's population resides in regional and remote areas. The geography of Australia and its large land mass raises problems for both the government (in providing) and the regional population (in accessing) the full range of available treatments and healthcare services.

Aboriginal and Torres Strait Islander people need to be carefully considered, as research shows these communities typically have poorer access to healthcare services due to financial, language, cultural or geographical barriers.

People with lower socioeconomic backgrounds may also be disadvantaged with the changing delivery of medical interventions and associated tests. Aside from the cost of the treatment itself or the test, there are often financial barriers for cancer patients including travel and accommodation costs; some associated with loss of income. Through RCA's research we found that one in two cancer patients are financially struggling due to the burden of paying for treatments and associated costs.

The policy should promote the use of tele-trials and tele-health in rural and regional Australia to help solve issues of access caused by geographical barriers.

Attracting global innovation

Funded access to the best modern therapeutics, medical technologies and vaccines must be equivalent to those available in comparable countries.

Unequivocally the investment in effective and safe medical innovation supports jobs and economic growth.

Commercialisation-funding pathways within Australia must be easier to navigate so that the country continues to attract overseas investments from global pharmaceutical, medical technology, and biotechnology industries. Australia must be seen as a desirable launch destination and location for investment in clinical trial research.

As disease populations become smaller and more individualised, the traditional commercialisation pathways that rely on large, global multi-national companies are at risk. These populations provide less commercial opportunity for global companies to realise return on investment in bringing products to Australia.

Patients with rare, less common, and complex cancers are already bearing the consequence of this – a growing number are having to resort to crowd funding to travel overseas for treatments, while others simply run out of options.

If funding government is a significant barrier, traditional funding models could be expanded upon to incorporate more novel approaches where government, industry and other stakeholders excluding the individual have shared financial responsibility.

Quality, safety, and efficacy

RCA believes the policy should be updated to include mention of fast-tracked and provisional approval pathways, and the relevance of these to meeting the needs of complex diseases.

Formal incorporation of these real-world outcome and evidence measures into the regulatory process, as well as big data and machine learning, can aid the extension of registered products beyond their approved use.

This will facilitate access to cancer therapies that are able to deliver benefit for multi-cancers but only have clinical trial data in a select few. One obvious example is checkpoint inhibitors that are effective in cancers that express PDL-1/L but are registered and reimbursed for one cancer type at a time. Many cancer patients expressing this phenotype have missed out on the opportunity for potential lifesaving treatment with check-point inhibitors.

Quality use of medicines

According to the policy, *"The quality use of medicines (QUM) is focused on reducing preventable harm and promoting the achievement of optimal health outcomes with reference to medicines use, by ensuring the right patient receives the right medicine* at the right time."*

RCA suggest this wording be changed to "*optimal treatment when it is needed*".

RCA would like to point out the following about the broad level quality use of therapeutics, medical technologies, and vaccines:

Data linkage, data recording, ethics, and ownership

RCA is aware that large amounts of data related to diagnosis, management and treatment of cancers are being generated by multiple stakeholders with competing interests.

Healthcare produces a steady and growing data stream from research, medical records, devices, prescription data, MBS item claims, past preferences for services, genomics, biology and more. This data will be key in delivering patient-centric personalised cancer care.

The resultant data must be stored in data banks, data clouds or registries with privacy and ethical protections in place for personal health information.

Currently, data is being accumulated across a range of public and private data banks, which will present a significant challenge for patients and the delivery of health outcomes if not systematically managed.

An added layer of complexity lies in the intellectual property, or legal ownership of the information. Who owns the data and how readily it will be shared will have significant impact on delivering the best outcomes to cancer patients.

This is particularly true in the case of the discovery and patenting of genetic signatures that have known linkages to important disease characteristics of a particular cancer subtype.

The policy should state that patient data generated within Australia must be available to Australians as well as foster data-sharing mechanisms.

The policy should present a position on data ownership and RCA suggests that patients should have the rights to their own data.

To achieve delivery of health outcomes and optimum uses of therapeutics, medical technologies and vaccines, data must be linked within different systems within hospitals and community treatment settings to increase overall efficiency. This reduces the reliance on patients or their families to relay complex medical information, histories, or results. Feedback from patients we support is that 'My Health Record' is less than optimal in meeting these needs.

Integration of real-world evidence, patient reported experiences, patient reported outcomes as well as clinical observation data will support best practice for future patients. It will also provide a data source to measure the effectiveness of the policy.

Data generation, data storage and its use within the Australian healthcare setting will require governance to prevent misinterpretation and inequities.

Artificial Intelligence (AI)

AI has the potential to transform cancer diagnosis and treatment and can revolutionise traditional models of healthcare.

Medical expertise in cancer care can be enhanced by the agility and precision of machine learning and deep learning over the next ten years. Harnessing the profound potential of AI in cancer care will depend on how well it is integrated into healthcare frameworks.

The industry is moving very fast internationally, and Australia can't afford to fall behind. The policy must support AI research, commercialisation and creation of AI healthcare guidelines and industry standards.

RCA also recommends the establishment of clear ethical frameworks to guide the weight that is placed on machine learning and algorithms versus human beings in clinical decision making.

Health literacy and information delivery standards

A minimum standard of information provision is required to ensure that all people can access and use information associated with an intervention in ways that promote and deliver optimal health outcomes.

Information should be simple and straightforward and be available in different languages and a variety of formats (digital/hard copy and graphically produced).

Patients should be informed sufficiently so that they are equipped to contribute to decisions relating to treatment or testing in partnership with their physician or clinical team – if they choose. This helps maintain an equal balance of power in the clinical team and patient relationship.

RCA supports the review of the legislation that prevents industry from talking about treatments and clinical trial data that are yet to be approved by the TGA. This is a barrier to knowledge sharing for patients, families, carers, and the public. We do not endorse this in a promotional capacity but rather in supporting empowered patients, families and carers that can contribute in multistakeholder settings with equal significance.

Creating a Person-Centred Policy

How can the NMPs focus of Consumer centricity and engagement be strengthened is there anything missing and what needs to change?

Despite all the good intentions, there is a vast disparity between the idea of person-centred healthcare policy and how that translates in the lived experience of cancer patients.

There is growing recognition that as taxpayers and the direct recipients of healthcare, the patient's perspective is becoming increasingly important.

There is still a significant power imbalance across the health system in the way patients are consulted and their input considered. Patients, carers, and families need to be respected as equal stakeholders with a valid and meaningful contribution to make.

Over seven hundred patients consulted via RCA's research agreed that patients' roles in the health care system should include being able to have a greater influence on service delivery, access, design issues and decision making.

RCA believes that patients should have representation at every touchpoint within health care decision making frameworks. Patients, families, and carers bring unique and rich perspectives to decision making forums.

The 'Discussion Document' refers to the 'Australian Charter of Healthcare Rights' (Patient Rights Charter) which describes what consumers can expect when receiving health care. Yet like the current version of the NMP, there is no mechanism to uphold these patient rights. Most patients we consulted were not aware of the Patients' Rights Charter or the NMP.

Currently having knowledge of the Patients' Rights Charter or the NMP does not change a patient's reality in terms of access to breakthrough therapeutics, medical technologies, and vaccines. Without a mechanism for accountability for the policy and charter, patients are powerless in navigating their treatment journey or trying to share knowledge to improve the journey for others in the future.

A patient-centric policy would formally capture patient preferences within the programmes that underpin the policy, and directly influence the recommendations made by the Committees to the Minister for Health.

A patient's expert guidance, derived from lived experience, can add value and insight to committee meetings, designing clinical trials, service delivery design of infrastructure, and how to deliver information. Enhanced patient influence will in turn aid the breakdown of logistical and clinical silos.

As the evidence base for emerging therapeutics, medical technologies and vaccines becomes more 'uncertain', it is logical that patient, carer, and family perspectives can play a critical role in providing clarity in decision making.

While RCA recognises there have been steps taken to increase public (consumer) representation within current frameworks, the patient perspective remains complementary to the discussion. The public/patient contribution must be fully integrated within the programmes. Person-centricity demands greater patient representation and recognition of the value derived from lived experiences.

Options to improve the NMP's governance

RCA strongly suggests that there be a formalised Patient Advisory Group or group of representatives from Patient Organisations that have a similar forum with the Health Department as Industry and can advise on issues relating to access and patient rights.

Should you require further information please contact:

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