Leukaemia Foundation

Standing Committee on Health, Aged Care and Sport
PO Box 6021
Parliament House
CANBERRA ACT 2600

30 October 2020

Dear Committee Secretary

Re: Inquiry into approval processes for new drugs and novel medical technologies in Australia

The Leukaemia Foundation welcomes the opportunity to provide a submission to the *Standing Committee on Health, Aged Care and Sport* on its inquiry into approval processes for new drugs and novel medical technologies.

The Leukaemia Foundation is the only national organisation that represents all Australians living with blood cancer. We provide practical and emotional support to Australians diagnosed with a blood cancer at no cost, thanks to the generosity of the community through our fundraising efforts.

Blood cancers (leukaemia, lymphoma, myeloma and related blood disorders) are a complex set of diseases that can affect anyone at any stage of life. Advances in treatments and care are transforming the way Australians live with a blood cancer; however, incidence rates are increasing. Today, blood cancers are one of the most commonly diagnosed and biggest causes of cancer death in Australia.

The Leukaemia Foundation's priority is to ensure that all Australians living with blood cancer have access to the best therapies and treatments available, which improve time spent in remission, survival and quality of life. We therefore appreciate the opportunity to contribute to this inquiry.

The Blood Cancer Taskforce has provided a separate submission to the inquiry, which the Leukaemia Foundation fully endorses.

If you require any further information, please contact Emily Forrest, Head of Policy Advocacy on eforrest@leukaemia.org.au.

Sincerely,

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A/g Chief Executive Officer

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Leukaemia Foundation submission to the *Inquiry into approval processes for new drugs and novel medical technologies in Australia*

About us and our work

Every year, more than 17,300 Australians will be newly diagnosed with a blood cancer, and more than 5,600 people will lose their life to blood cancer or related blood disorders. This means every day, 47 Australian men, women and children will learn they have blood cancer, and sadly around 15 people will lose their life to the disease each day.

The Leukaemia Foundation's purpose is to help save lives by creating value and impact for people living with blood cancer and their families. We provide support to people living with blood cancer, including accommodation support where patients and their families need to travel for treatment; we fund and facilitate critical blood cancer research; and we advocate on behalf of people living with blood cancer for reforms that will improve diagnosis, treatment and care.

In 2019-20 alone, the Leukaemia Foundation's 37 Blood Cancer Support Coordinators gave personalised support and care to 8,459 people living with blood cancer, and we provided free, safe, self-contained accommodation to 837 families from rural and regional areas.

Demand for our services tripled during the COVID-19 pandemic, and our outreach program connected more than 3,600 families with our support staff, and over 19,000 people accessed 24/7 help and information through our dedicated information hub.

We also invested $4 million (and leveraged another $10 million) in 18 new blood cancer research projects, bringing currently funded projects to 32 in 2019/20. Over the past 30 years we have made a $51.4 million commitment to blood cancer research and built significant collaborations with Cancer Australia, Haematological Society of Australia and New Zealand, Snowdome Foundation and the Leukaemia and Lymphoma Society in the United States.

The Leukaemia Foundation is a member of the Fundraising Institute of Australia (FIA), the largest representative body for the Fundraising sector, and we comply with the FIA Code. The FIA Code is a voluntary, self-regulatory code of conduct for fundraising in Australia. It aims to raise standards of conduct across the sector by going beyond the requirements of government regulation. Its content is informed by the International Statement of Ethical Principles in Fundraising.

Changing the paradigm

For over 40 years, the Leukaemia Foundation has supported people living with blood cancer in Australia. Now we are looking forward to leading a new era of change for the Australian blood cancer community by partnering with industry, government, medical professionals and everyday Australians to realise the goal of zero lives lost to blood cancer by 2035.

Formerly two separate bodies, in 2016, the Leukaemia Foundation of Australia and Leukaemia Foundation of Queensland united into a truly national organisation, and with it began an ambitious vision for a united voice for people with blood cancer in Australia.

The Leukaemia Foundation has developed an access strategy, '*Breaking barriers for people living with blood cancer*', which will concentrate our time, talents and resources on three critical areas for every Australian with blood cancer:

- Inform - access to trusted information and education to empower informed choices.

- Treat - access to best practice treatment and the latest trials, tests and diagnostic tools.
- Care - access to essential supportive care to improve quality of life.

That strategy is informed by the evidence-based *State of the Nation: Blood Cancer in Australia* report and the blueprint for reform outlined in Australia's first National Strategic Action Plan for Blood Cancer, and will help unite the blood cancer community in pursuit of the shared goal of zero lives lost to blood cancer by 2035.

Further information about the findings and recommendations from the *State of the Nation* report and the National Action Plan is provided below, with a particular focus on recommendations that seek to improve more consistent and equitable access to treatments and diagnostics for people living with blood cancer in Australia today.

State of the Nation: Blood Cancer in Australia

In September 2019, the Leukaemia Foundation released the *State of the Nation: Blood Cancer in Australia* report, a first of its kind analysis to quantify and articulate the challenges and opportunities that influence survival and quality of life for people living with all blood cancers.

To understand real experiences, we also undertook a survey of people living with blood cancer and their families, with over 3,200 people responding from across the blood cancer spectrum. Respondents were widely distributed across sub-types, states and territories, regional vs metropolitan, age and private health insurance status, providing a representative and statistically powerful sample.

Among its important findings were that taken collectively, blood cancers are amongst the most prevalent and deadly cancers affecting Australians today. Over 110,000 Australians of all ages are living with a blood cancer in Australia today, and by the year 2035, modelling predicts that number will increase to around 275,000 people. Over 186,000 people will die as a result of blood cancer between 2018 and 2035, and the annual cost to the health system of treating and caring for people with a blood cancer in 2035 is expected to reach $10.9 billion.

The research suggests that by removing variations in survival outcomes between metropolitan and regional areas, and ensuring consistent use of evidence-based, already-funded best practice treatment and care nationally, we could improve survival outcomes by 13 per cent – saving around 22,000 lives by 2035.

The National Strategic Action Plan for Blood Cancer

Following the release of *State of the Nation,* the Leukaemia Foundation was commissioned by the Federal Government in 2019 to establish the Blood Cancer Taskforce and develop Australia's first National Strategic Action Plan for Blood Cancer ('National Action Plan') with the blood cancer community. The National Action Plan was launched by The Hon Greg Hunt MP, Minister for Health, on 27 September 2020.

The National Action Plan provides an evidence-based blueprint to coordinate and accelerate national efforts to improve survival and quality of life for people diagnosed with blood cancer and to support their carers and families. It sets out the necessary priority areas, objectives and actions for addressing the challenges of blood cancer to achieve the vision of zero lives lost to blood cancer by 2035, underpinned by zero preventable deaths regardless of geography or background, through equitable access to best practice treatment and care for all Australians.

Through collaboration with patients and leaders in the blood cancer community, the National Action Plan identifies four major priorities to improve outcomes for people living with blood cancer and their families:

- Empower patients and their families
- Achieve best practice
- Accelerate research
- Enable access to novel and specialised therapies.

The National Action Plan includes recommendations across the entire blood cancer ecosystem: from research, clinical trials, precision medicines, treatment access and reimbursement, through to achieving best practice in diagnosis, treatment and supportive care. It builds upon a long history of hard work and success in the blood cancer community and redoubles focus on addressing the survival gaps which still exist for Australians dealing with a blood cancer.

The National Action Plan is a multi-year plan that will involve support from many levels of the community and governments. The Leukaemia Foundation and its generous supporters will be a part of ensuring the success of that plan, and the ethical and transparent collection of philanthropic support is a core value of our organisation.

The *State of the Nation* report and the National Action Plan are attached to this submission as supporting documents and we respectfully request that these are included as evidence to support the Committee's deliberations.

Barriers to enabling access to novel and specialised therapies

Australia's high quality health system is bolstered by its regulatory system which sets a high standard of evidentiary rigour and a low tolerance for uncertainty. This works to ensure product efficacy, patient safety and value for money are all considered when evaluating new therapies.

Our understanding of blood cancer disease subtypes is becoming increasingly more defined, and patient populations of those diseases are subsequently smaller and more refined. While this is of benefit to patients, allowing for the development of more targeted treatments and precision medicine, it also creates challenges and barriers for patient access to new treatments and diagnostics.

This is particularly acute with respect to evidence development and public funding for novel therapies (both medicines and diagnostics) and compounded for new therapies that require a mix of state and federal funding (for example, CAR T-cell therapies). The regulatory and reimbursement system is adjusting to the advent of precision medicine which, while increasing our understanding of blood cancers and their treatments, has brought new challenges for traditional models of development, regulatory approval and reimbursement, as well as challenges to ensuring patients have access to best practice diagnosis, treatment and care.

If a medicine, device, or service is not PBS or MBS listed, Australian patients must either privately fund, seek compassionate access via a clinical trial, or forego the therapy. In practice, this means it will likely be out of reach for most Australians, and clinicians have been reported not discussing options with patients if therapies are not publicly subsidised.

Where some patients with means can bridge the funding gap most others cannot, so concerns for equity of access necessarily follow. At the same time, patients are now more likely to be aware about treatments that may be in use overseas than ever before. In the context of evolving and

increasing consumer expectations for the health system, this can lead to substantial frustration on the part of patients and may contribute to perceptions of a 'two-tiered' health system. This increases pressure on governments and industry to address potential gaps in access to services compared to international comparator markets.

The following section outlines some of the key barriers to patient access to novel and specialised therapies, based on the evidence gathered to develop the *State of the Nation* report and the National Action Plan. Potential solutions to these access barriers are then addressed in turn. These issues are highlighted in the context of blood cancers but are no means unique to the blood cancer community.

Barriers to patient access to clinical trials

Clinical trials are one of the most important ways for people to gain access to new and novel therapies and advance our understanding of (and therefore our ability to treat) blood cancers and other diseases.

Despite the benefits, patient participation in clinical trials remains low. Very few Australians with blood cancer are involved in a clinical trial, or discuss their clinical trial options with their clinician. The Leukaemia Foundation's survey of more than 3,200 people living with blood cancer conducted as part of the *State of the Nation* report found that less than one in five patients reported participating in a clinical trial and that 68 per cent of people who did not participate in a clinical trial indicated it was because their specialist did not suggest it.

Barriers to accessing clinical trials for patients in regional and remote areas

Patients in regional and remote areas may not be able to access clinical trials due to the limited availability of trials in regional and remote locations and financial barriers created by a lack of subsidy for treatment that is not the standard of care. State-based patient assisted travel schemes (or PATS) also specifically preclude financial support for travel related to clinical trial participation in most jurisdictions.

Barriers to accessing an accurate and timely diagnosis (including genomic and genetic testing)

Access to accurate and timely diagnostics is fundamental to the treatment and care of a person living with blood cancer. As the complexity of blood cancers is better understood, so too, are the diagnostic tools used to diagnose and treat a patient.

In Australia not all tests that are recognised as clinically important are used, readily accessible or publicly reimbursed. This includes genetic and genomic testing, which is increasingly recognised as part of routine care in other markets comparable to Australia.

This leads to lower rates of testing, delayed and or inaccurate diagnoses and reducing the potential for patients to benefit from treatments targeted to their unique genetic makeup and/or the genomic markers of their disease. This can also mean that clinical trials relying on these tests may not be opened in Australia.

Barriers to evidence development for PBS or MBS listings

A lack of evidence of benefit, or uncertainty in the evidence, may prevent the listing of a new medicine, device, or service on the PBS or MBS. Uncertainty in evidence increases as the sample data decreases. For patient populations that are rare, uncertainty of benefit is often high. This is

the challenge of rare conditions, and the stratification of blood cancer patients into ever smaller patient populations, based on genomic profiling, increases the challenges for generating evidence of benefit and for obtaining public subsidy.

This has the effort of reducing pharmaceutical company incentives to seek PBS or MBS listings for new medicines, devices, or services and in turn, could impede clinicians' access to novel and specialised therapies for blood cancer patients.

If a company does not lead an application for regulatory approval and public subsidy, it falls to the blood cancer community, which is itself fragmented, often lacks access to the required data or the data is not systematically aggregated across sites, and/or may not be experienced in application preparation (leading to low quality submissions and long regulatory timeframes).

The result is that many important medicines and diagnostics remain unfunded even when they are clinically important, standard of care therapies.[1]

Lack of evidence to support public subsidy can also have the unintended consequence of increasing the use of 'off-label' prescribing, therefore not generating the evidence required to support listings for the wider patient community and the outcomes of which are not always reported to relevant clinical registries. While representative statistics of off-label prescribing are not available, one government stakeholder consulted as part of the National Action Plan indicated it could be 'in the thousands' across all conditions.[2] This perspective accords with available research in this area in Australia; a 2012 review found that 42.2 per cent of the total treatment protocols approved for use in a specialist oncology centre were off-label and were unfunded by the PBS[3]. A survey undertaken by the Medical Oncology Group of Australia of their members in 2017 similarly found that of the respondents, 92 per cent had discussed and 68 per cent had prescribed at least one unfunded anticancer drug to their patients in a three-month period.[4]

There are a broad range of drugs currently being used off-label for blood cancer patients, where there is no TGA listing in that indication and/or a clear pathway or market incentive for a PBS listing. Through stakeholder consultations conducted as part of development of the National Action Plan we understand that, for example, bendamustine, indicated for lymphoma and CLL, is used off-label for myeloma; daratumumab, indicated for myeloma, is used off-label for AL amyloidosis; rituximab, indicated for a number of blood cancers, has also been used off-label for early stage follicular lymphoma and post-transplant lymphoproliferative disease; and tacrolimus, indicated for use in liver, kidney, lung and heart transplantations, is used off-label for haematopoietic stem cell transplantation.[5]

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[1] Such as FISH, PCR, NGS, and MRD tests; see Blood Cancer Taskforce, 2020, *Rapid Review of Evidence*, p151. Available at https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer_Review-of-Evidence-for-Action-1.pdf

[2] Ibid, p185.

[3] Mellor JD, Van Koeverden P, Yip SWK, Thakerar A, Kirsa SW, Michael M, 2012, Access to anticancer drugs: many evidence-based treatments are off-label and unfunded by the Pharmaceutical Benefits Scheme, *Internal Medicine Journal* 4 2(11):1224-9, available at https://pubmed.ncbi.nlm.nih.gov/22372936/

[4] Karikios DJ, Mileshkin L, Martin A, Ferraro D, Stockler MR, 2017, Discussing and prescribing expensive unfunded anticancer drugs in Australia, *ESMO Open* Jun 21;2 (2):e000170, available at https://pubmed.ncbi.nlm.nih.gov/28761744/

[5] Blood Cancer Taskforce, 2020, *Rapid Review of Evidence*, Table 5.3, pp189-190. Available at https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer_Review-of-Evidence-for-Action-1.pdf

Potential solutions to improve patient access to novel and specialised therapies

The *State of the Nation* report and the National Action Plan make several recommendations to address these complex and interrelated factors, which are summarised below and are relevant to the terms of reference of the Committee's inquiry.

Actions to improve access to clinical trials, including in regional and remote areas

While there are many barriers to increasing clinical trial investment and activity in Australia, there are some immediate improvements that can be made to increase patient awareness of their clinical trial options and, where trials are available, improve access to people living in regional and remote areas.

In particular, the National Action Plan makes two recommendations which the Leukaemia Foundation submits should be considered as part of the national reform agenda to improve clinical trial participation and access in Australia.

National Strategic Action Plan for Blood Cancers (pg.47-48)

Action 4.1 – Develop key performance indicators (KPIs) for a discussion of clinical trial options with patients

4.1.1 The National Action Plan recommends commissioning a pilot study to examine the implementation of a KPI for a clinician-led discussion regarding enrolment in clinical trials if and where available and appropriate. A KPI for a clinical trials discussion could be piloted in blood cancers (or a subset of blood cancer patients). The results generated may have wider application for cancer patients more broadly and on a national level. The aim is to increase active consideration of clinical trials in treatment planning and empower patients to have greater engagement and understanding of their clinical trial options.

4.1.2 The establishment of a KPI for a discussion of available clinical trial options with patients would leverage existing national clinical trial reform agendas and be led by the Australian Commission on Safety and Quality in Health Care as part of their existing program of work. Once the systems and methods for KPI reporting are developed, these would be embedded in blood cancer optimal care pathways and clinical guidelines and there would be training and change management delivered to clinicians to support their implementation.

Action 4.2 – Increase access to clinical trials in regional and remote areas, including a national approach to prioritising tele-trials

The National Action Plan recommends building on existing work being undertaken at the state/territory and federal level (including the regional and remote clinical trials infrastructure program) to promote and support patient participation in regional clinical trials and tele-trials through:

4.2.1 Ensuring that blood cancer optimal care pathways and clinical guidelines document the importance of discussing clinical trial research options with patients, including those living in regional and remote areas.

4.2.2 A national approach to blood cancer research supported by the Blood Cancer Research Program, with regional trial sites pre-approved for ethics and governance to

streamline trial opening, leveraging work already underway through the national reform agenda in clinical trials.

4.2.3 A skills audit of regional and remote workforce requirements (including the primary care workforce) and an infrastructure audit of facilities to enable clinical trial participation at these sites.

Actions to ensure an accurate and timely diagnosis (including genomic and genetic testing)

While a timely and accurate diagnosis is fundamental to quality and safe clinical practice and patient outcomes, major challenges exist in achieving an accurate and timely diagnosis, the delivery of diagnostic services to patients and in the notification of all blood cancers to state cancer registries.

The National Action Plan recommends developing Australian-specific guidelines for diagnostics in blood cancer, followed by a review of Australia's capacity to meet those guidelines – including options to address under-notification of cases to state cancer registries. These actions are outlined in full below.

National Strategic Action Plan for Blood Cancers (pg.34-35)

Action 2.2 – Develop guidelines for diagnostics and review Australia's capacity to meet these guidelines

2.2.1 Review existing international diagnostics guidelines in blood cancer and develop Australian-specific guidelines for minimum and recommended testing requirements for different types of blood cancer in Australian healthcare settings. These guidelines should be integrated with clinical guidelines (Action 2.1).

2.2.2 Following the development of diagnostic guidelines for different types of blood cancer, undertake a wider strategic assessment of blood cancer diagnostics service delivery across Australia. This would include workforce development needs in metropolitan and regional areas, as well as potential options for improving accuracy, timeliness and efficiency in diagnostic services nationally. This strategic assessment should also identify options to address issues with under-notification of cases to state cancer registries.

2.2.3 The Enabling Access Working Group (Action 4.3) should engage with governments, regulators and the blood cancer community to:

a. Coordinate evidence to support the development of applications for MBS reimbursement of diagnostics that are standard of care but are not yet listed.

b. Continue important reforms to MSAC processes for MBS listings, focusing on greater transparency and the rapid adoption of diagnostics, which have been demonstrated to be cost-effective that direct patients to the most effective therapies. This should include enhancing consumer understanding of and engagement with the MBS listing process, drawing experience from improved consumer engagement in PBS processes

Actions to improve evidence development, including for PBS and MBS listings

Both the *State of the Nation* report and the National Action Plan make recommendations to improve evidence development for new and novel therapies, including recommendations to help clinicians and decision-makers better capture data and evidence from off-label prescribing.

This includes new models for clinical trials and systematic evidence development and working in collaboration with industry and governments to develop strategies for evidence development for therapies that do not have public subsidy or where there are access challenges due to the complexity of funding arrangements or the type of therapy.

New approaches to evidence development in clinical trial settings could be deployed to more systematically tackle barriers to evidence development and ensure more equitable, timely access to emerging therapies.

The *State of the Nation* report refers to one potential model for systematic evidence development. The concept of the 'Right to Trial' program is to support systematic evidence development and provide a mechanism for the more systematic evaluation of off-label use and re-purposing of drugs.

State of the Nation: Blood Cancer in Australia report

Supporting evidence development for new therapies and tests (see Action 2.2)

"A Right to Trial program could support more systematic evidence development and clinician-led or patient-led submissions for new therapies. This would *provide a mechanism for the more regular and systematic use and evaluation of off-label medicines, and could reduce dependence on industry to conduct the research needed to advance potentially curative therapies.*

The Right to Trial Program would need to be developed to ensure that therapies accessed through the program met required eligibility criteria, such as the criteria used to determine off-label use, or applications for compassionate access, where safety criteria can be prescribed and met, and are in routine use already today. In addition, there would need to be clear entry and exit timelines to limit potential unintended consequences vis-a-vis the PBS. This would provide a more systematic and scientific mechanism for the evaluation of medicines that are used off-label and more equitable access to emerging therapies.

Such a program, properly designed, would reduce inequities of access to therapies where evidence is in development. Moreover, while the program could be piloted for blood cancers it could easily be extended to a wider range of conditions over time; it need not be blood cancer specific" (pg. 100).

This concept of an evidence development model outlined in the *State of the Nation* report takes its inspiration from alternative approaches to evidence development that have been successful in supporting access to therapies in use internationally. Models like this can be used to develop robust control groups in the context of small patient populations — ultimately supporting more effective and systematic evidence development and progress towards a cure.

The Leukaemia Foundation is currently supporting a clinical trial that has a similar model to that proposed in the *State of the Nation* report, called the Blood Cancer Genomics Trial.

Case study: Blood Cancer Genomics Trial

The Leukaemia Foundation's has a set of research priorities including precision medicine, novel therapies and innovative clinical trials which have driven our investments into the use of genomic and genetic testing and the access to novel therapies through clinical trials. One of these research initiatives is the Blood Cancer Genomics Trial.

The Leukaemia Foundation has partnered with the Australian Genomics Cancer Medicine Program to use the Molecular Screening and Therapeutics clinical trials and immunotherapy platform at Sydney's Garvan Institute to pilot a study (Blood Cancer Genomics Trial) to test the feasibility of rapid turnaround genetic testing, multidisciplinary tumour board reporting and genetically directed targeted therapy for patients with relapsed or refractory high-grade haematological malignancies.

The Leukaemia Foundation is supporting the Blood Cancer Genomics Trial, in partnership with Tour de Cure, headed by Professor Steven Lane at the Queensland Institute of Medical Research (Brisbane) and Professor Hamish Scott, University of South Australia, SA Genomics (Adelaide).

We envision that the Blood Cancer Genomics trial will consist of a combination of basket and umbrella trials. There are over 100 different types and sub-types of blood cancer making it difficult to do traditional large-scale clinical trials. Basket and umbrella trials provide an alternative method to provide access to new therapies under a clinical trial setting in discreet patient populations.

Clinical basket trials test the efficacy of new therapies in patients with different types of cancer but similar genomic mutation or biomarker in that cancer. The genome of each patient is compared with the genome of the patient's cancer, to discern the underlying cause of their cancer and target treatment accordingly.

Umbrella clinical trials, conversely, test the efficacy of therapies in patients who have the same type of cancer but different gene mutations or biomarkers in that cancer. In umbrella trials, patients receive treatment based on the specific mutation or biomarker found in their cancer, which allows for flexibility and reflexivity of treatment within a trial as new targets and drugs are found.

This clinical trial is in the final stages of development and designed to test the feasibility of genetic testing of blood cancers and genetically directed targeted therapy for patients who have failed previous therapy and would otherwise have poor outcomes. The trial will utilise translational biomarker analysis to support two sub studies – a basket sub study, and an umbrella sub study.

The fundamental premise of the Blood Cancer Genomics Trial is that innovation in clinical trial design may expedite translation of discovery into improved health outcomes. It is anticipated that the findings and structure of the trial will provide a new model of rapid clinical translation and access to world-leading innovative treatment for the management of high-risk blood cancers in Australia, with findings that have global impact.

The rapidly evolving technology landscape for blood cancers presents challenges for patients, governments, service providers, researchers, clinicians and industry - in what is an already complex regulatory, funding, and research landscape.

Consultations and evidence gathered in developing the National Action Plan found that risks to patient access to novel and specialised therapies arose from three overlapping issues:

> Issue 1: Market barriers to evidence development and regulatory applications
>
> Issue 2: Inequitable access to novel and specialised therapies by jurisdiction due to the high cost of therapies and complex funding arrangements
>
> Issue 3: Equity of access risks to existing and emerging therapies, with an emphasis on cellular therapies (such as stem cell transplants and CAR T-cell therapy).

These complex and interrelated issues require sustained leadership and focus to remedy. The National Action Plan therefore recommends establishing a multidisciplinary Enabling Access Working Group, including consumers and other relevant specialists, to work across the blood cancer community and provide a structured approach to addressing these issues in turn. The recommendation is provided in full below.

National Strategic Action Plan for Blood Cancers (pg. 49)

Action 4.3 – Establish an Enabling Access Working Group to improve equitable access to novel and specialised therapies

4.3.1 A multidisciplinary Enabling Access Working Group should be established, including consumers, to work across the blood cancer community and address challenges for patient access to novel and specialised therapies. The Working Group would have three tasks:

- Develop a short-list of clinically important medicines and diagnostics that do not have public subsidy and where there are market barriers to evidence development. Work with the Federal Government and the blood cancer community to coordinate an approach to evidence development for each therapy, which could include funding investigator-led clinical trials, or coordination of research and regulatory applications, including provisional registration, which may require participation in a registry to enable access to a novel therapy.
- Commission a review of access to novel and specialised therapies by state and territory to identify disparities in access to standard of care therapies and develop a plan to improve equity of access nationally.
- Engage with Government to develop a strategy to optimise supply of suitable stem cell donors for Australian and international patients and to ensure equity of access to cellular and emerging therapies, including CAR T-cells for all Australians.

The Enabling Access Working Group would consider and complement work that is already underway to improve access to new therapies and diagnostics, including projects to be delivered through the MRFF, for example, the Health System Preparedness for Cancer and Paediatric Healthcare Initiative.

Conclusion

Thank you for the opportunity to contribute to this Inquiry. Both the *State of the Nation* report and the National Action Plan make a number of recommendations that are relevant to the terms of reference, as summarised in this submission to the Committee.

As a patient-centred organisation, our driving motivation is for reforms that enable patients to access the treatment they need, when they need it, and ultimately reduce the number of Australians every year who die from blood cancer.

Decision-making in healthcare, as in many other policy areas, has moved positively in the direction of *nihil de nobis, sine nobis* – nothing about us, without us. It is essential to more fully engage consumers and to incorporate their views into decisions that so directly affect their health and healthcare options.

Improved transparency and better integration of the consumer voice in enunciating the value of new and novel therapies will, we believe, improve both the evaluation process and subsequent implementation of reforms.