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Re: Medicines Australia submission to the National Action Plan for Blood Cancer

Medicines Australia welcomes the opportunity to provide a submission to the '*National Action Plan for Blood Cancer'* and the *'State of the Nation: Blood Cancer in Australia Final Report to the Leukaemia Foundation February 2019'.*

Medicines' Australia's CEO, Ms Elizabeth de Somer, is proud to be a member of the National Blood Cancer Taskforce and recognises the key role that the innovative medicines industry must play in the fight against blood cancer.

Medicines Australia is the peak industry body representing the innovative research-based medicines industry in Australia. Our members are innovative companies that research, develop, manufacture and supply new medicines, therapies and vaccines to the Australian market, including those for blood cancer. The innovative medicines industry is proud of the contribution it makes to the health and well-being of everyday Australians, as well as to the local economy. The innovative medicines industry has developed some ground-breaking discoveries. These emerging innovative medicines and therapies (such as CAR-T and precision medicine) are helping to fight previously untreatable diseases and are providing patients with better survival rates and improved quality of life. However, medicines investment is high-risk with approximately only 12% of medicines that enter clinical trials reaching approval for use by patients[1]. Therefore, our industry is highly reliant on a stable policy environment that strongly supports innovation, research and development, and commercial translation to the same levels as competitor nations.

Medicines Australia strongly supports the Leukaemia Foundation priorities, including:

- Ensuring equity of access
- Advocating for health service reform
- Accelerating research, and,
- Empowering patients

In order for Australian patients to have access to the latest innovative medicines and therapies when they need them, there needs to be a policy environment that supports innovation and acknowledges the unique role of innovative pharmaceuticals and biotherapies in enhancing Australians' health and wellbeing. Therefore, we believe that the above principles can be further defined in light of the below issues:

- Access to, and reimbursement of, novel therapies and diagnostics
- The value of precision medicine to patients and the health system
- The role of clinical trials in enhancing patient health and providing access to medicines
- The commercial translation of research (i.e. bench to bedside)

Overall, Medicines Australia would like to commend the Leukaemia Foundation's comprehensive work on the State of the Nation report and the goal towards developing a National Action Plan for Blood Cancer.

Medicines Australia strongly supports collaboration with Government, industry, health consumer organisations and other stakeholders to work towards achieving the Leukaemia Foundation's goal of Zero Lives Lost to Blood Cancer by 2035.

Please see Appendix 1 below for a more detailed discussion on the above issues in light of the State of the Nation report and the development and implementation of the National Action Plan for Blood Cancer. Please feel free to contact Betsy Anderson-Smith on banderson-smith@medaus.com.au if you would like to further discuss our submission.

Yours sincerely,

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Director of Policy and Research
Medicines Australia

APPENDIX 1

Access to, and reimbursement of, novel therapies and diagnostics

Medicines Australia firmly believes that there is a need to develop novel evaluation and reimbursement systems that appreciate the value of personalised therapies. In this way, Governments will provide access to potential cures. This will mean patients will be treated with fewer therapies, suffer from fewer adverse effects, and enable appropriate treatment to be commenced earlier, ultimately prolonging the lives of patients with blood cancer. Indeed, research indicates that pharmaceutical innovation is responsible for almost all (94.8%) of the decline in premature mortality between 1998-2015, and about half of the increase in the average age at death from cancer between 2008 and 2018. Specifically, the same research also found that 44% of the increase in the survival rate for cancer patients from 2001-2005 to 2011-2015 was due to the launch of new cancer drugs[2]. This research indicates that there is a clear benefit to patients and society at large to invest in breakthrough medicines.

As mentioned in the State of the Nation report, there are significant challenges for blood cancer patients in equitably accessing treatment and care. Indeed, if a treatment or diagnostic tool is not publicly subsidised through the Medical Benefits Scheme (MBS) or Pharmaceutical Benefits Scheme (PBS), it will be out of reach of most Australians.

Australia relies on a system of health technology assessment with respect to the reimbursement of medicines or therapies via the Pharmaceutical Benefits Advisory Committee (PBAC) and the Medical Services Advisory Committee (MSAC). The PBAC and MSAC incorporate cost-effectiveness principles to ensure that there is a balance between the health gains from a new medicine or therapy and the price sought by the responsible person. The challenge with innovative precision therapies lies in the uncertainty in demonstrating cost-effectiveness. This is often due to small patient populations in clinical trials and high research and development costs, which can mean there is a lack of commercial incentives for companies to undertake clinical trials and reimbursement submissions.

Australia's regulatory and reimbursement medicines evaluation systems have served us well over the last few decades. However, with the evolution of more specialised medicines, including cell and gene therapies, there is a need to ensure that the health policy environment remains fit for purpose and evolves with technology. Whilst there are already non-standardised pathways through the regulatory process, there is a need to discuss how the traditional approach to reimbursement and health technology assessment can keep pace with the next generation of major scientific advances in medicine.

Precision medicines and personalised therapies are disruptive and do not fit the traditional health technology assessment process. The potential for precision medicines to be curative means the Government could move to innovative evaluation and payment models which focus on the predictive value of therapy to an individual patient, the community, and the wider health system.

In addition, with the development of electronic health systems (e.g. My Health Record), there is the opportunity to incorporate real-world evidence and patient reported outcomes in regulatory/reimbursement evaluations to supplement the data from early-phase single-arm clinical trials. In this way, a real-world data ecosystem also enables companies to do cost-effective research on medicines use beyond traditional clinical trials. It should be noted that there is currently no formal mechanism for capturing real world data (e.g. for off label use) that allows clinicians to submit products through the regulatory and reimbursement processes.

The value of precision medicine to patients and the health system

Medicines Australia supports biopharmaceutical research and development into personalised therapies to enable a deeper understanding of disease mechanisms and the ability to target therapies to defined patient populations. Precision medicine involves the use of tests to guide drug/dose selection, to predict which patients are likely to respond to treatment or have a higher risk for adverse events, and allows for the targeted treatment of disease.

The benefits of precision medicine are vast, and include:

- Faster times to optimal therapy due to diagnostic tests that reduce inefficient prescribing
- Increased patient compliance due to improved effectiveness and/or a more favourable safety profile
- Increased probability of clinical benefit in medical research due to a focus on narrower, well-defined patient subpopulations
- A reduction in health care costs due to early diagnosis and prevention of advanced disease, a reduction of ineffective treatment, and avoidance of additional care related to adverse reactions or sub-optimal therapeutic response

As stated above, there is a need to improve the current regulatory and reimbursement policies to foster continued innovation for precision medicine to ensure the full value of targeted therapies and companion diagnostics is realised.

The role of clinical trials in enhancing patient health and providing access to medicines

Medicines Australia has long advocated for, and been supportive of, policies that enable Australia to be seen as an attractive destination for clinical trials. Australia already has many advantages as a place to conduct clinical trials. This is in large part because Australia is home to some of the world's best researchers and health professionals and boasts a world-class research infrastructure, a stable socio-political environment, and high clinical and research standards that ensure confidence in the scientific conclusions reached by clinical trials conducted in Australia. The innovative medicines industry is committed to ensuring that Australia maintains its reputation as a preferred destination for clinical trial investment. According to MTPConnect's report 'Clinical Trials in Australia', data from 2015 showed that 1360 clinical trials were started that year (including 473 industry sponsored trials) supporting approximately 7000 jobs – a $1.1 billion in investment[3].

Indeed, clinical trials can provide many benefits with respect to the economic and physical health of Australians including:

- Early patient access to cutting edge therapies, providing patients with improvements in their health and quality of life
- Improvements in the knowledge of participating healthcare professionals
- Creation of high skill jobs and a robust workforce
- Provision of opportunities for Australian scientists and medical researchers to be at the forefront of medical research
- Generation of taxable income and a source of government revenue

Unfortunately, there are some impediments to Australia's attractiveness as a market for clinical trials including:

- Complex arrangements to establish a trial including variable arrangements for clinical governance and ethics approval, leading to unnecessary delays in the initiation of multicentred clinical trial sites
- Lower recruitment rates and slow timelines potentially leading to trial closure
- High trial costs
- Inconsistent processes between states and territories
- Lack of a coordinated long-term strategy

Unless Australia is prepared to continue to reform the trials environment to remain an attractive location for global clinical trials and develop a long-term strategy, Australia runs the risk of significant competition from lower cost geographies such as the Asia Pacific and Latin America. As international competition intensifies, Australia will have to address these challenges.

Medicines Australia therefore calls for the following solutions to ensure Australia remains an attractive destination for clinical trials:

- The Australian Government, through the Council of Australian Governments (COAG), to work with industry and the State and Territory Governments, to find agreement for regulatory harmonisation and mutual recognition that optimises clinical trial initiation and shortens commencement times
- Government and clinical trials stakeholders to work together to enhance patient access to clinical trials through improved coordination, promotion of the value of clinical trials, harmonisation of patient records, and enhanced tele-health initiatives to improve patient recruitment in remote and regional areas

The commercial translation of research (i.e. bench to bedside)

Medicines Australia believes that engagement and partnership with industry will assist in the translation of research into clinical/commercial outcomes. The global pharmaceutical industry is an essential part of translating research into commercial ideas and onto patient outcomes, and the priorities of the National Action Plan for Blood Cancer should align to capture the skills, knowledge and scientific expertise of the industry.

Medicines Australia supports the idea of an International Blood Cancer Research Mission. We believe this will enhance Australia's reputation as an attractive research destination, encourage and enhance the skills and knowledge of Australian researchers, and ensure health system quality, effectiveness and sustainability.

Specific comments on the State of the Nation report

Inequity of access

Medicines Australia agrees with the State of the Nation report that there are substantial variations in treatment and care depending on the State a patient lives in, whether they live in a metropolitan area or regional area, and whether they are treated in a public or a private hospital setting (or both).

For example, many patients with haematological malignancies are treated as inpatients in the hospital setting due to the severe nature of their conditions. These treatments are high in cost and are covered by the hospital. However, the ability of a hospital to bear these costs varies greatly between hospitals and states. In the public setting hospitals will decide whether to cover these treatments which can lead to patients in the public setting missing out on the latest innovative medicines.

Conversely in the private setting all treatments, even those given as inpatients, are covered by the PBS creating an inequity across the hospital system depending on a patient's location or ability to afford private hospital cover.

Diagnostic reimbursement

The State of the Nation report highlights that 'precision medicine is now at the forefront of innovative cancer treatment, targeting specific changes in a patient's tumour DNA to treat the disease, while also taking into account the genetic variations between people who have been influenced by environmental and lifestyle factors. These advances are transforming both the diagnosis and management of patients.'

Whilst we acknowledge that diagnostic techniques are advancing rapidly, the reimbursement of diagnostics is not keeping pace. Availability of testing varies widely between hospitals and several tests such as minimal residual disease (MRD) testing or molecular testing are not currently available via the MBS. In order to achieve optimal patient outcomes, testing needs to be widely available and affordable for all patients irrespective of where they are treated.

Without accessibility and affordability of new advances in diagnostic technologies, the result will be misdiagnosis and poor treatment planning. For some sub-types, this may result in the wrong treatment, contributing to poorer health outcomes for patients and health system inefficiencies.

Examples of this include emerging treatments targeting specific mutations or cell markers. The identification of prognostic factors is also advancing rapidly. Diagnostic tests such as the measurement of MRD are being increasingly used to tailor treatment strategies and assess the risk of relapse in patients. However, as stakeholder feedback in the State of the Nation report identifies (figure 3.27) there is a significant out of pocket expense associated with accessing MRD testing.

Treatment and transition of patients from pediatric to adult

Treatment paradigms vary greatly between paediatric and adult/elderly patients and cut offs also vary by disease, state and hospital. Often clinicians specialise in one or the other setting, so there can be an interrupted continuum of care when patients transition from paediatrics to adults which may lead to sub-optimal treatment strategies and outcomes.

References

1. PhRMA, Medicines: Cost on Context. Available at: http://www.phrma.org/cost
2. Lichtenberg FR. Pharmaceutical Innovation Improves Patient Outcomes and Reduces Hospital Demand: Measuring the Impact of Pharmaceutical Innovation in Australia 1998-2018.
3. MTP Connect, Clinical Trials in Australia. https://www.mtpconnect.org.au/clinicaltrials