12 October 2020

Committee Secretariat
PO Box 6021 Parliament House
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Dear Committee Secretariat,

RE: HOUSE OF REPRESENTATIVES STANDING COMMITTEE ON HEALTH, AGED CARE AND SPORT INQUIRY INTO THE APPROVAL PROCESSES FOR NEW DRUGS AND NOVEL MEDICAL TECHNOLOGIES IN AUSTRALIA

Thank you to the Standing Committee on Health, Aged Care and Sport for the opportunity to provide a submission to the 'Inquiry into the approval processes for new drugs and novel medical technologies in Australia'.

The Rare Disease Industry Working Group (RDIWG)

The RDIWG is comprised of companies who share a common interest in rare disease treatments and orphan drug development. The companies that are represented develop, manufacture and supply innovative rare disease treatments to the Australian rare disease community.

The RDIWG member companies have an ongoing relationship with Rare Voices Australia (RVA) through the RVA Round Table of companies.

The purpose of the RDIWG is to collaborate to ensure that Australians with rare and ultra-rare diseases have timely and equitable access to treatments by:

1) ensuring that reimbursement procedures and guidelines are fit-for-purpose, and

2) engaging in policy discussions to ensure sustainable access to rare disease treatments.

Australians deserve equitable and timely access to the best available health technology

The Australian Government should be commended for providing subsidised access to essential medicines to over 430 eligible patients with rare and life-threatening diseases through the Life-Saving Drugs Program (LSDP). In addition, the Australian Government subsidises access for other medicines for rare diseases via other funding pathways such as the Pharmaceutical Benefits Scheme (PBS).

We would also like to acknowledge the Australian Government's commitment to provide up to $3.3 million for activities to implement the first National Strategic Action Plan for Rare Diseases announced in February 2020. Importantly, the Action Plan's key priorities include equitable access to the best available health technology.[1]

[1] National Strategic Action Plan for Rare Disease February 2020 https://www.rarevoices.org.au/page/167/national-strategic-action-plan-for-rare-diseases on October 2020

There is more to be done with Australians waiting between two to four years longer for access to Government-funded treatment for rare diseases than in comparable countries [2, 3]

Registration and reimbursement processes must be enhanced to ensure that Australians living with rare diseases have equitable access to the latest technologies. This is in accordance with Priority 2.4 and Action 2.4.1 of the National Strategic Action Plan for Rare Diseases. In particular, it is important that areas of high unmet clinical need are supported by mechanisms for priority review and timely access to treatments and interventions.

Health Technology Assessment (HTA) was introduced in Australia in 1992. For nearly three decades, the HTA process in Australia has been considered world class and Australians have been proud of the National Health System that allows Australians access to most of the medicines they need. Australia should build on this acclaim by developing a pathway for assessing rare disease therapies which recognises the unique advantages of these medicines and technologies.

A fit-for-purpose system should be put in place for evolving therapies such as gene and cell therapies which have attributes that require special consideration. Development of such a system will mean that Australians can remain proud of the system which enables sustainable access to interventions irrespective of their health challenge.

[2] The McKell Institute 2014. Funding Rare Disease Therapies in Australia: Ensuring equitable access to healthcare for all Australians. Sydney. The McKell Institute. Accessed from https://mckellinstitute.org.au/app/uploads/McKell-Institute-Funding-Rare-Disease-Therapies-in-Australia-Nov-2014.pdf on September 2020

[3] National Strategic Action Plan for Rare Disease February 2020 https://www.rarevoices.org.au/page/167/national-strategic-action-plan-for-rare-diseases on September 2020

TOR 1: THE RANGE OF NEW DRUGS AND EMERGING MEDICAL TECHNOLOGIES IN DEVELOPMENT IN AUSTRALIA AND GLOBALLY, INCLUDING AREAS OF INNOVATION WHERE THERE IS AN INTERFACE BETWEEN DRUGS AND NOVEL THERAPIES

RECOMMENDATIONS

1. **Systematic horizon scanning of new medicines and technologies**
2. **Reviews e.g. National Medicines Policy and reimbursement guidelines include a future focus**
3. **Fit-for-purpose process for rare disease treatments and interventions**
4. **Development of innovative access mechanisms with parallel collection of long-term RWE**

A process for systematic horizon scanning of new medicines and technologies for the rare disease community should be implemented between the Department of Health, Industry and other stakeholders. This will enable planning for the future in terms of the way these interventions will be evaluated for registration and reimbursement as well as ensure that there are budgetary provisions to fund these interventions.

The proposed review of the National Medicines Policy, review of the Orphan Drug Policy and ongoing reviews of guidelines for applications for reimbursement (e.g. Medical Services Advisory Committee [MSAC] guidelines) should include a future focus so that health systems have the capacity to deal with the timely introduction of novel technologies.

A fit-for-purpose process for rare disease treatments and other rare disease interventions should be prioritised to ensure that these patients have sustainable and timely access to the most effective interventions.

It is expected that novel technologies, such as cell and gene therapies, will provide significant long-term health benefits for patients. The types of interventions in development may have limited data at the time of assessment. Therefore, there should be a focus on the development of innovative access mechanisms to ensure patients have the advantage of being able to access treatment in parallel to the long-term collection of real-world evidence (RWE).

In addition, novel technologies are likely to be associated with high upfront costs whereas the benefits may occur over a prolonged period of time. The uncertainty about long-term outcomes will require a sustainable framework for risk-sharing arrangements between manufacturers and the Government.

TOR 2: INCENTIVES TO RESEARCH, DEVELOP AND COMMERCIALISE NEW DRUGS AND NOVEL MEDICAL TECHNOLOGIES FOR CONDITIONS WHERE THERE IS AN UNMET NEED, IN PARTICULAR ORPHAN, PERSONALISED DRUGS AND OFF-PATENT THAT COULD BE REPURPOSED AND USED TO TREAT NEW

RECOMMENDATIONS

1. **Fair and equitable process for the assessment of rare disease treatments**
2. **Early engagement with the Department of Health**
3. **Review of Orphan Drug Policy**
4. **Fee exemptions based on budget impact**

CONDITIONS

The goal of appropriate incentives is to ensure that people living with rare disease have equitable and timely access to medicines with demonstrated clinical benefit for a rare disease, including those that are already funded for another condition (Action 2.4.3 of the National Strategic Action Plan for Rare Diseases).

A fit-for-purpose assessment for rare disease therapies prioritises aspects such as patient relevant outcomes and societal impact, in addition to cost-effectiveness. A clear and coordinated pathway will provide a significant incentive for rare disease companies to research, develop these and commercialise these important interventions in Australia. If these companies can be assured that funds will be available to invest in these innovations in a timely manner, it will provide certainty and predictability to these companies.

The Life Saving Drugs Program (LSDP) ensures ongoing access for patients to life-saving treatments for chronic conditions that meet certain criteria. Although significant improvements have been made to the administration of the LSDP in recent years, the program operates after a medicine has been reviewed by the Pharmaceutical Benefits Advisory Committee (PBAC). In situations where a new treatment provides similar outcomes to an existing listed treatment (e.g. second generation treatments), there is still a requirement for the sponsor to transition through a full PBAC submission prior to consideration by the LSDP Expert Panel. In this case, the PBAC evaluation may be considered inefficient, wasting valuable resource and an unnecessary step.

Other rare disease treatments that do not meet the LSDP eligibility criteria may be able to be funded on the Pharmaceutical Benefits Scheme (PBS). However, special consideration needs to be given to rare diseases being evaluated by the PBAC. Firstly, greater input from expert clinicians and relevant patient organisations would help the PBAC to understand the disease impact, patient relevance and clinical meaningfulness of the (sometimes limited) data presented, and the seriousness of the unmet need. Secondly, cost-effectiveness needs to be considered in the context of rare disease treatments which often cannot meet the ICER thresholds typically applied to treatments for more common conditions. Alternative measures of value should be considered, including societal perspectives, impact on patients, carers and

the community, and indirect economic costs. In the case of orphan drugs, the overall budgetary impact should be given more weight than the ICER.

In contrast, there are rare disease treatments and interventions that do not meet either the LSDP or PBS criteria, meaning that Australian patients do not have access to these treatments or in some cases, only have access many years after patients in other countries.

Earlier engagement with the Department of Health would be welcomed by Industry in order to be able to identify the appropriate reimbursement pathway, provide the patient voice and establish clinical need so that all parties facilitate the path to access without increasing submission churn.

The Orphan Drug Policy provides criteria for obtaining an exemption from cost-recovery fees for submissions to the PBAC. However, reimbursement submissions must occur within one year of registration or the waiver of fees does not apply. Rare disease submissions are often complex and require additional data analysis and stakeholder engagement. They are rarely recommended following the first submission. The process can take years with multiple resubmissions to PBAC and can be cost-prohibitive even with the Orphan Drug Designation (ODD) fee waiver for the first submission, with subsequent resubmissions costing $166,220 from 1st January 2021. Therefore, the timeframes for claiming exemption of cost recovery fees should be extended for orphan drugs. These changes will provide additional incentives to bring orphan drugs to Australian patients.

Rare disease treatments often treat extremely small populations of patients, meaning that the cost recovery fee structure for PBAC submissions can mean that it is not commercially viable to lodge submissions for these indications. Consideration of implementation of a fee structure based on budget impact would increase access to treatments for very small populations.

TOR 3: MEASURES THAT COULD MAKE AUSTRALIA A MORE ATTRACTIVE LOCATION FOR CLINICAL TRIALS FOR NEW DRUGS AND NOVEL MEDICAL TECHNOLOGIES

RECOMMENDATIONS

1. **Implementation of a Rare Disease Registry and Clinical Trials Network**
2. **Streamlined ethics approval especially for gene therapies**
3. **Viable pathway to sustainable access will encourage clinical development**

Building on existing initiatives there should be a continued focus on fostering an environment conducive to clinical trials for rare diseases taking place in Australia (Action 3.2.4 of the National Strategic Action Plan for Rare Diseases).

Implementation of a Rare Disease Registry and Clinical Trials Network would improve the National co-ordination of data collection and patient identification for trial participation in rare diseases with very

small numbers. Additionally, the development of registries and better access to registry data by all stakeholders would facilitate the review of treatments.

Harmonised and streamlined ethics approval across State and Territory boundaries will facilitate the inclusion of Australian patients in clinical trials.

However, patients and clinicians are more hesitant to participate in clinical trials if there is no viable pathway to sustainable access, and sponsors are also less likely to bring clinical trials to Australia. An active clinical trial environment in Australian is not possible without certainty of timely access.

TOR 4: WITHOUT COMPROMISING THE ASSESSMENT OF SAFETY, QUALITY, EFFICACY OR COST-EFFECTIVENESS, WHETHER THE APPROVAL PROCESS FOR NEW DRUGS AND NOVEL MEDICAL TECHNOLOGIES, COULD BE MADE MORE EFFICIENT, INCLUDING THROUGH GREATER USE OF INTERNATIONAL APPROVAL PROCESSES, GREATER ALIGNMENT OF REGISTRATION AND

RECOMMENDATIONS

1. **Pre-submission process should be enhanced for LSDP and PBS rare disease treatments**
2. **Fit-for-purpose evaluation pathway for rare disease treatments that do not meet LSDP criteria**
3. **PBAC criteria for rare disease treatments to have broader focus**
4. **Consumer hearings should be held for all rare disease treatments**
5. **Clarity with regard to reimbursement pathways for new interventions e.g. gene and cell therapy**

REIMBURSEMENT PROCESSES OR POST MARKET ASSESSMENT

Processes for rare disease interventions should be improved to ensure funding and reimbursement pathways are fit-for-purpose and sustainable for current and new health technologies for rare diseases (Action 2.4.2 of the National Strategic Action Plan for Rare Diseases).

In a similar way that the Australian Technical Advisory Group on Immunisation (ATAGI) considers vaccines before the PBAC, consideration of products destined for the LSDP by the LSDP expert panel prior to the PBAC would simplify the process. The two-step LSDP process is unnecessarily lengthy particularly with regard to the need to be rejected by the PBAC prior to consideration by the LSDP Expert Panel.

The PBAC pre-submission process for rare and ultra-rare disease medicines should be enhanced. In the case of products to be listed on the LSDP, the inclusion of a LSDP representative as part of the multi-stakeholder review panel would ensure the specific challenges of the disease are well understood and provide clarity on the likely funding pathway prior to PBAC submission.

A fit-for-purpose reimbursement pathway for rare disease treatments that do not meet LSDP criteria, including development of PBAC guidelines for rare disease treatments and education and advice to evaluators on the issues associated with rarity and the importance of reviewing these treatments through

a different lens to treatments for non-rare conditions. PBAC criteria for rare disease treatment should value rare disease treatments with a broader focus than cost-effectiveness criteria such as societal perspectives, the impact on carers, broader community care and indirect economic costs.

Importantly the patient voice should be incorporated as part of all pathways. Consumer hearings should be held for all rare disease treatments in order that the patient voice is heard in particular with regard to the effect of the condition on the life of patients and their families and the impact that a new treatment will have to these patients. In accordance with Action 2.1.5 of the National Strategic Action Plan for Rare Diseases, the voice of people living with a rare disease as well as families and carers should be embedded throughout structures and systems that impact rare diseases. Rare disease organisations should work with the HTA Consumer Evidence and Engagement Unit to take a more active role in HTA processes.

Managed access programs and other outcomes-based arrangements, particularly in the case of small populations and uncertainty of the data, would provide early access to patients while collecting data to reduce uncertainty over time.

Rare disease expertise should be developed within the Office of HTA (Action 2.4.1.4 of the National Strategic Action Plan for Rare Diseases) and the evaluation template should include content and explanations which focus on the differences as a consequence of rarity.

The overall reimbursement process for rare diseases is unnecessarily lengthy meaning that Australian patients have to wait for treatments for very prolonged periods of time. Consideration should be given to ongoing negotiation rather than rejection, particularly with regard to price and population, after the first evaluation thus reducing submission churn.

The advent of new technologies has meant that the reimbursement pathway is uncertain even if the technologies appear similar. The current uncertainty on pathways for novel technologies requires clarity and guidance with regard to the respective pathways as well as the development of guidelines for reimbursement submissions for these technologies. While MSAC guidelines are currently under review, it does not appear that there has been a future focus with the incorporation of guidelines for reimbursement submissions for future agents.

In summary

The RDIWG welcomes the opportunity to work with the Australian Government and other stakeholders on fit-for-purpose reforms to the current reimbursement procedures and guidelines so that Australians have sustainable access to rare disease treatments.

Yours sincerely

[redacted]

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On behalf of the Rare Disease Industry Working Group (RDIWG)

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