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Rare Voices Australia Submission: National Medicines Policy Review

October 2021

Introduction

Rare Voices Australia (RVA) welcomes the opportunity to lodge a Submission into the National Medicines Policy (NMP) Review. This Submission will formally build on the interview conducted with RVA's Chief Executive Officer, Nicole Millis, and the NMP Review Committee. Thank you for the opportunity to provide input via the interview and this Submission. RVA has provided a response to each of the Terms of Reference (TOR) Discussion Paper questions. All of RVA's responses are aligned with the priorities, actions and implementation steps outlined in the Australian Government's National Strategic Action Plan for Rare Diseases (the Action Plan). The Action Plan is the first nationally coordinated effort to address rare diseases in Australia. RVA led the collaborative development of the Action Plan which was informed by an extensive multi-stakeholder consultation process. The Action Plan was launched in February 2020 by the Minister for Health with bipartisan support. RVA is the national peak body for the estimated two million Australians living with a rare disease. RVA's person-centred focus sees us working with over 90 RVA Partner organisations (individual rare disease organisations), in addition to other stakeholders in the rare disease sector: advocates, clinicians, researchers, governments, key peak bodies and the pharmaceutical industry.

RVA would like to acknowledge the many strengths of the NMP and the intent of the Review to refresh the NMP in recognition of the substantial changes to the health landscape that have emerged since the policy was published in 2000. RVA's Submission will build on these strengths to highlight opportunities for improvement and how the NMP can provide the high-level policy framework to enable the best outcomes for Australians living with a rare disease, now and into the future.

TOR 1: Evaluate the current NMP objectives and determine whether these should be modified, or additional objectives included. This includes consideration of the proposed principles to be included within the NMP

The following principles are proposed for inclusion in the refreshed NMP:

- Equity
- A consumer-centred approach
- Partnership based
- Accountability and transparency
- Stewardship

Question A. Are these proposed principles appropriate? With regard to the proposed principles, is anything missing or needing to change?

RVA supports all of the proposed principles which align strongly with the Action Plan and in particular, the following key priority and its related actions:

Priority 2.4: *Enable all Australians to have equitable access to the best available health technology.*

Action 2.4.1: *Develop policy that supports people living with a rare disease to have timely and equitable access to new and emerging health technologies.*

***Action 2.4.3:** Ensure people living with a rare disease have equitable access to medicines with demonstrated clinical benefit for a rare disease, including those that are already funded for another condition.*

The proposed principles also clearly align with the Action Plan's foundation principles, *"person centred"* and *"equity of access".* Further alignment between the Action Plan and the principles is evident in the Action Plan's critical enablers: "multi-stakeholder involvement and engagement"; "collaborative governance and leadership"; and "state, national and international partnerships".

RVA Recommendations for Question A:

In addition to the proposed principles, RVA also calls for the inclusion of three additional principles:

- Timeliness
- Fit for purpose
- Innovation

Proposed Additional Timeliness Principle

Although "timely access to medicines" is one of the objectives of the NMP, RVA believes that "timeliness" should also be elevated to the principle level. This would more accurately reflect its critical importance to all stakeholders, particularly to patients/consumers. Timeliness is critical for the rare disease community. Many rare diseases are progressive and access to medicines is time critical. Uncertainty about when a decision regarding a health technology will be reached can add to the burden already experienced by those living with a rare disease, as well as their families and carers.

The inclusion of timeliness as an NMP principle would also help to facilitate a much-needed focus on further defining what is meant by terms such as "timely access" and identify potential ways that timeliness can be measured. Recent evidence points to deficiencies in the timeliness of reimbursement in Australia. The *Compare 5* report by Medicines Australia (2019) reported that, "It takes roughly three to four times longer for New Molecular Entities (NMEs) to achieve reimbursement in Australia (410 days) than world leaders Japan (98 days), Germany (119 days), Austria (148 days) and Switzerland (149 days)". Arguably, on average, Australians living with a rare disease wait even longer. The *Funding Rare Disease Therapies in Australia – Ensuring Equitable Access to Health Care for All Australians* report by The McKell Institute (2014) reported that "Australians are generally waiting from 2 to 4 years longer for access to rare disease therapies available in comparable countries".

Over the last five years, RVA has provided mentorship to individual rare disease patient groups, much of which focuses on their participation in health technology assessment (HTA). From this work, RVA has learned that multiple submissions are often required before a rare disease medicine is recommended for reimbursement. Similarly, from our work with the RVA Round Table of Companies, a group of pharmaceutical companies with a common interest in rare diseases and orphan drug development, RVA is aware that companies that submit applications for rare disease indications typically anticipate that it will take more than one submission to achieve reimbursement. This perception alone is damaging as it deters companies from applying and contributes to Australia failing to be identified as a priority market.

Proposed Additional Fit for Purpose Principle

An NMP fit for purpose principle would greatly assist in policy settings being able to better respond to rare diseases. The inclusion of this principle would help guide agreements and processes to better respond to transformative therapies such as cell and gene therapies, precision medicine, co-dependent technologies and drug repurposing. A fit for purpose principle would also equip the NMP to better respond to the evidentiary challenges that come with these new and emerging technologies, as well as the ongoing evidentiary challenge inherent in the rare disease context generally. This is strongly reflected in the Action Plan:

Action 2.4.2: *Ensure funding and reimbursement pathways are fit-for-purpose and sustainable for current and new health technologies for rare diseases.*

RVA commends the work of the Therapeutic Goods Administration (TGA) and Pharmaceutical Benefits Advisory Committee (PBAC) around parallel processes. Similarly, RVA believes the PBAC is well regarded in terms of transparent timelines and consumer engagement. However, the PBAC's criteria regarding cost effectiveness is challenging in the rare disease context, as is the reliance on traditional clinical trial evidence. Meanwhile, the Life Saving Drugs Program (LSDP), which has recently been reformed in terms of its transparency and timelines, offers clear opportunities for stakeholder contribution and adopts a less restrictive approach to cost effectiveness. That said, the LSDP will only ever be an appropriate approval process for specific rare diseases due its eligibility criteria relating to prevalence and demonstration of life extension. Traditionally, the Medical Services Advisory Committee's (MSAC) expertise lies in tests, devices and blood products. In recent times, there has been significant variation in the many areas the MSAC assesses. Their level of understanding of rare disease and precision medicine is unclear. The MSAC certainly lacks transparency around timelines and formal consumer engagement. While the MSAC can use expedited processes, these processes can only be considered for resubmissions. RVA noted comments by an MSAC representative at the *Introduction to Medical Services Advisory Committee (MSAC)* webinar on 27/8/2020, regarding the length of the MSAC process being about 12 months and that "typically, applications were not successful." These comments suggest more needs to be done to ensure the process is better fit for purpose.

Rare diseases are complex. There is limited data for rare diseases and high levels of uncertainty. All rare disease applications should be routinely flagged as complex and may require additional scoping and stakeholder engagement to address potential challenges and uncertainties. An opportunity to bring together stakeholders to discuss potential challenges/gaps and troubleshoot complexities should be incorporated earlier in the process. Existing approval processes mean that such gaps are not highlighted and are often not responded to until consumer input is gathered later in the process. There is also a lack of rare disease expertise and understanding in relation to informing existing approval processes for rare disease therapies. The following Action Plan implementation step identifies this issue:

Implementation 2.4.2.3. *Ensure rare disease expertise exists, or can be accessed, on all reimbursement pathways and HTA advisory bodies.*

Similarly, a strong understanding of precision medicine and novel clinical trial designs must be evident throughout the HTA process.

Australian HTA assesses clinical effectiveness (prioritising placebo clinical trial evidence with a low tolerance of uncertainty) as well as cost effectiveness. Inherent features of rare disease (very small patient numbers and progressive, often life-threatening disease) can mean that it is challenging, sometimes impossible, and often unethical to conduct randomised placebo clinical trials. Despite the inherent limited data in rare disease, our HTA system does not seem to value or encourage other forms of evidence where appropriate, whether that is other forms of trials or the inclusion of real-world data from contemporary and emerging case studies. A fit for purpose approach is needed.

Proposed Additional Innovation Principle

An innovation principle would strengthen the NMP and equip it to better respond to emerging technological advances. This principle would help to future-proof the policy, while helping to guide and prioritise important key activities such as horizon scanning and encouraging clinical trials to take place in Australia. It was pleasing to see that the NMP Review Discussion Paper highlighted clinical trials. For many people living with a rare disease, participation in a clinical trial may be their only way to access treatment. The role of policy in attracting clinical trials to Australia was highlighted in the Action Plan:

Action 3.2.4: *Building on existing initiatives, continue to foster an environment conducive to clinical trials for rare diseases taking place in Australia*.

The close relationship between research and care is also reflected in a key priority of the Action Plan:

Priority 3.4: *Translate research and innovation into clinical care; clinical care informs research and innovation.*

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

The four objectives are:

- Access to medicines
- Quality, safety, and efficacy of medicines
- Quality use of medicines
- Maintaining a responsible and viable medicines industry

RVA feels that the interrelated NMP objectives are still very relevant.

RVA Recommendations for Question B:

Identifying how NMP objectives can be measured would greatly strengthen the current objectives. Any measures should also reflect the NMP principles. Accountability and transparency is a proposed NMP principle. To achieve accountability and transparency, these measures need to be reported on and regularly communicated to all NMP partners/stakeholders. This will enable informed multi-stakeholder discussion and facilitate benchmarking activities and continuous improvement. While different partners/stakeholders are likely to have different perspectives regarding standards, such transparent measures will help to facilitate effective discussions and reduce the disconnect between partners/stakeholders.

The development of further policy implementation mechanisms under each objective would also strengthen the NMP's responsiveness to rare diseases.

Access to Medicines Objective

The NMP Review Discussion Paper has identified a range of policy implementation mechanisms that enable access to medicines including the Pharmaceutical Benefits Scheme (PBS); the LSDP; access to medicines through public hospitals; access programs; and clinical trials. It is important to recognise that access to medicines through these mechanisms is often uncertain and comes with its own specific challenges, especially for rare diseases.

PBS

With its criteria of cost-effectiveness, it can be challenging to have a treatment recommended for listing on the PBS for a rare and thus, very small population.

LSDP

While the LSDP provides an avenue that does not require the same degree of cost effectiveness, policy does require treatment to meet prevalence (1/50000) and 'life extension' criteria. It is also a lengthier process as an LSDP application cannot begin until it has been assessed by the PBAC and not recommended for listing on the PBS. It is not yet clear how the PBAC will assess the cost-effectiveness of emerging and transformative therapies such as cell and gene therapies.

Public hospitals

Access to medicines through public hospitals is also uncertain. This type of funding is not appropriate for ongoing treatment. If a patient is approved for one course of treatment, there is no guarantee they will be approved for subsequent treatments. Nevertheless, many rare disease patients are reliant on this process as many medicines that clinicians believe would be beneficial for a number of rare diseases are currently only funded (listed) on the PBS for a more common condition. Due to the inherent very small patient numbers of rare diseases and lack of data, there is often no commercial incentive for companies to seek PBS listing for additional conditions (indications). This has resulted in rare disease patients having uncertain, inequitable, or simply no access to such treatments recommended by their clinicians. This unmet need is identified in the Action Plan:

Action 2.4.3: *Ensure people living with a rare disease have equitable access to medicines with demonstrated clinical benefit for a rare disease, including those that are already funded for another condition.*

Access programs

Australia's HTA has a strong reliance on pharmaceutical company sponsor-led applications. Where there is lack of commercial incentive, alternative supported pathways for clinicians and consumers to initiate an application for reimbursement, are required. Current policy settings do not require any collated identification of such hospital funding. The collection of this type of data would help to identify potential repurposing candidates and ensure improved quality use of medicine.

Clinical trials

Clinical trials are an important way to access treatments and should be reflected as part of an inclusive and comprehensive NMP. Clinical trials are especially important in rare disease where participation in such trials may be the only way to access treatment. Australia needs to encourage policy settings that reduce the barriers to clinical trials and develop clinical trial infrastructure. A rare disease clinical trial and registry network is needed to provide a fit for purpose response to the inherent challenges of rare disease data and research challenges. As stated in the Action Plan, clinical trials for rare diseases taking place in Australia should:

Implementation step 3.2.4.3: *Encourage the adoption of unique and appropriate trial designs that overcome rare disease research challenges.*

This fit for purpose approach also needs to be mirrored in reimbursement processes. As recommended by many in the House of Representatives Parliamentary Inquiry into approval processes for new drugs and novel medical technologies in Australia, the establishment of a precision medicine office would help to facilitate this. Specific precision and personalised medicine expertise should inform the implementation of policy relating to relevant therapies. As there are limited treatment options for rare diseases, it is essential that people living with a rare disease are able to benefit from new and transformative health technologies.

As many rare diseases are progressive, time is often critical. It is important that Australia's policy-makers do all they can to accelerate not only the scientific development of new medicines and technologies, but regulatory approval processes too. Even when a rare disease treatment option does exist, financial support may not be available in Australia which limits access. Timely and equitable reimbursement of new medicines and emerging technologies is essential. This is identified in the Action Plan:

Priority 2.4: *Enable all Australians to have equitable access to the best available health technology.*

Action 2.4.1: *Develop policy that supports people living with a rare disease to have timely and equitable access to new and emerging health technologies.*

Australian HTA processes utilise models that are designed primarily for more common diseases. This presents challenges for reimbursement decisions for medicines/technologies for rare diseases. Smaller patient numbers impact cost effectiveness, and there is often less clinical evidence available due to the challenges of conducting large-scale clinical trials. This highlights the importance of fit for purpose approaches to both research and HTA models for rare diseases.

Quality, Safety, and Efficacy of Medicines Objective

RVA strongly supports this objective and acknowledges the important role of the TGA. While arguably the most important objective, it is also the objective most impacted by the limitations of other objectives. For example, a quality, safe and efficacious medicine is no use to a rare disease patient if they cannot access it, or if their disease progresses too much before they can access it.

The Quality Use of Medicines Objective

RVA acknowledges the existing policy implementation mechanisms that relate to and support this objective. There should also be a policy implementation mechanism that identifies and monitors the use of medicines funded through hospitals not just at an individual hospital level but at a national level too. Cross jurisdictional policy needs to enable this to monitor the quality use of medicines but also to identify opportunities for appropriate service planning and investment for more equitable and sustainable access.

Maintaining a Responsible and Viable Medicines Industry Objective

It is vital that policy appropriately incentivises industry to provide innovation to address unmet need. RVA acknowledges the current measures in place, however, more must be done to enable stronger, more transparent and streamlined fit for purpose approval processes. Similarly, more should be done to encourage clinical trials in Australia. The unfortunate reality is that Australia is not a priority market for international companies. The NMP must do all it can to develop policy settings to drive change in this area. The addition of timeliness, fit for purpose and innovation principles to the NMP will greatly assist.

TOR 2: Consider the definition of medicines and whether the NMP needs to be expanded to include health technologies.

The emergence of cell and gene therapies, precision medicine, as well as an increase in co-dependent technologies has led to complexity in these therapies navigating existing regulatory and reimbursement pathways, especially when such therapies are delivered in a hospital environment. The current narrow definition of medicines arguably contributes to uncertainty regarding processes, and potentially has flow on impacts such as delays in access.

Question A. Should the current NMP definition of medicines be expanded to include medical devices and vaccines? Why or why not? How would a change in definition of medicines be reflected in the policy's high-level framework?

RVA Recommendations for Question A:

RVA's position is that the current NMP definition of medicines should be expanded. A broad definition of medicines is important to encompass newer technologies and respond in a consistent, aligned way to complexities such as co-dependent technologies. This will not only assist in future-proofing the NMP but also provide a high-level policy setting that reduces fragmentation of care. In the rare disease context, it is not uncommon for a range of health technologies to be utilised in an individual's care plan in an interrelated and co-dependent way. An expanded definition of medicines within the NMP would arguably better mirror a person's experience of care. Additionally, a broader, expanded definition would provide the NMP with a more person-centred approach.

Emerging therapies require specialist knowledge and expertise, and often specialised care in the administering of therapy. It is important that a broad definition of medicines also includes specialised and sustainable models of care. RVA recognises that different technologies require specialist expertise and the NMP would need to incorporate such expertise. While this may present initial challenges, as these challenges are addressed, this provides an opportunity for a more collaborative and less fragmented policy approach to medicines and related health technologies. Importantly, it would also set up a more appropriate policy response in recognition of the interrelation between innovation and care. Specialised technologies also often require specialised expertise in care and administration. This needs to be appropriately embedded and supported within our hospitals and other health systems. 'Sustainable systems and workforce' is a foundational principle of the Action Plan. The NMP and any other relevant policy should recognise this.

Question B. Does the policy's current title, the "National Medicines Policy", reflect the breadth of health technology developments within the policy's scope? If not, how best can these and future health technologies be better represented in the policy's title?

RVA Recommendations for Question B:

'Health technologies' is a broad, inclusive term that is being used increasingly in the mainstream. It is also the term adopted in the Action Plan. Targeted communications would increase the wider public's health literacy and familiarity with this term.

TOR 3: Assess the NMP's utility in the context of rapidly evolving treatments options, population changes, interconnected relationships, and system-wide capacities.

The NMP has an important role to play in ensuring Australia's health system continues to embrace advancements in health care to deliver high quality outcomes for all Australians. The translation of cutting-edge research into promising new treatment options (e.g. precision medicine such as cell and gene therapies) can have significant benefits for patients, particularly those with rare diseases with high, unmet clinical needs. The NMP must adequately capture the importance of, and opportunities arising from, precision medicine. With its focus on a targeted approach, precision medicine will also potentially transform the quality use of medicines to provide the right medicine(s) for the right person at the right time.

RVA acknowledges the significant related policy development and investment into advancements into health care. The rare disease sector has particularly welcomed the role of the National Health Genomics Policy Framework which outlines a shared direction and commitment between all Australian Governments to consistently and strategically integrate genomics into the Australian health system. Similarly, the Medical Research Future Fund (MRFF), which includes ongoing rare cancers and rare diseases grants, has started to address the unmet need in rare disease and drive much needed innovation. However, this work is still relatively new and much more needs to be accomplished. Such work must continue in an increasingly interrelated way, as identified in the Action Plan.

Priority 3.4: *Translate research and innovation into clinical care; clinical care informs research and innovation.*

The breadth, complexity and speed of these new treatment and therapy options becoming available, raise both regulatory and reimbursement considerations. An innovation principle as part of the NMP would strengthen this policy focus and help to enable solutions to move through these challenges.

Clinical trials and medicines access programs support the earliest access to novel treatments. Access to clinical trials and medicines access programs are not discussed in the current NMP. This is a significant limitation. For many people living with a rare disease, participation in a clinical trial may be their only opportunity to access treatment. It is important that the NMP provides the required high-level fit for purpose policy settings that appropriately prioritise access to clinical trials and medicines access programs. Key factors to consider include:

Health Literacy

The principle of accountability and transparency is dependent on increased health literacy. Identification of priority populations and targeted education and communication is important. The Action Plan has identified seven priority populations including Aboriginal and Torres Strait Islander people; people living in regional, rural and remote areas; people from culturally and linguistically diverse (CALD) backgrounds; and people experiencing socio-economic disadvantage. It is important to continue investing in the ongoing sustainability of evidence-based, credible peak bodies and services, especially for those that cater for priority populations.

Equity and Sustainability

Financial and geographic barriers to accessing novel treatments are an equity issue. People should receive equitable and consistent care regardless of where they live.

In rare disease, reimbursement decisions often limit access to medicines to children only. These decisions are often due to the reliance on data from clinical trials which often excludes adults. Population subsets such as these cause inequity and distress within individual disease patient communities.

For Australians living with a rare disease, the rapidly changing landscape of research, diagnostics, emerging therapies and understanding the value of existing therapies for rarer conditions has increased inequities in accessing medicines. See the 'Proposed Additional Timeliness Principle' on page 3 for more detail. 'Equity of access' is one of the foundation principles of the Action Plan.

Real-world Evidence

The use of real-world evidence to inform HTA has the potential to address some of the evidence gaps relating to uncertainty. Managed Access Programs are currently used in a very limited way and often with a narrow primary focus of managing price uncertainty rather than building evidence. RVA calls for the further development of Managed Access Programs to have a greater focus on real-world evidence and consumer centricity to address data gaps and accumulate knowledge. Currently, in Australia, there is no process for translating and utilising valuable real-world data as it emerges. Yet, this remains a potentially invaluable strategy to facilitate timely regulatory approval and to enable equitable therapeutic access. This is a long-standing equity issue for rare diseases and will increasingly become an issue with the global trend towards personalised and precision health. This is a significant barrier preventing Australians accessing new medicines and emerging technologies.

Drug Repurposing

The repurposing of medicines already approved for use in the treatment of other conditions plays an important role and presents an opportunity to address unmet need in the treatment of rare diseases. Existing medicines funded for more common conditions can often be clinically beneficial as treatments for rare diseases. Clinicians often prescribe off-label use of medicines in the treatment of rare diseases.

Australian patients are unable to reap the full benefits of repurposing of medicines as reimbursement of health technologies for rare diseases is challenging, even for an approved medicine for a more common condition. For rare diseases, there are a lack of transparent and equitable pathways for repurposing existing treatments that have already been reimbursed for more common conditions. This is a common and systemic issue for rare diseases.

Companies commonly state that it is not feasible for them to submit an application for new indications due to extremely small patient numbers, lack of conventional clinical trials, governance requirements that would require alteration of core data sheets etc. This means that many Australians who are living with a rare disease are forced to rely on the uncertainty of off-label use or self-fund (often equating to thousands of dollars in costs) their access to a medicine that is recommended by their clinician. This is both unsustainable and inequitable. It is difficult, if not impossible, for a non-pharmaceutical sponsor to submit an application. Without Government reimbursement, many rare disease medicines are simply unaffordable for Australians living with a rare disease and their families.

Question A. How has the NMP been able to maintain its relevance and respond to the changes in the health landscape?

RVA acknowledges the many strengths of the NMP which, given the policy was first published in 2000, has served the majority of Australians well over the past two decades. That said, there have been substantial changes to the health landscape that have emerged in recent times which make this Review a necessity, particularly for Australians living with a rare disease.

RVA Recommendations for Question A:

The NMP must respond to the cross-jurisdictional complexity for nationally funded treatments delivered as part of state-funded health systems and hospitals. Importantly, the NMP must inform and guide related policy and agreements such as the National Health Reform Agreement to strengthen interconnectivity to facilitate timely, equitable and safe access to new medicines for Australians living with a rare disease in line with the Action Plan:

Action 2.4.1: *Develop policy that supports people living with a rare disease to have timely and equitable access to new and emerging health technologies.*

Such policy must also consider the importance of workforce capacity. A foundational principle of the Action Plan is 'Sustainable systems and workforce'.

Question B. How could the NMP be refreshed so that the policy framework is able to better address current and future changes in the health landscape? What is missing and what needs to be added to the policy framework, and why?

RVA Recommendations for Question B:

Clinical trials often present the only opportunity for a patient with a progressive rare disease to access treatment. It is critical that Australia attracts and incentivises pharmaceutical companies to conduct rare disease clinical trials in Australia, enabling Australian rare disease patients to participate in international clinical trials. Currently, there is a lack of coordinated infrastructure to support a national approach for rare disease clinical trials with very small patient numbers. The Action Plan acknowledges that more needs to be done to attract these clinical trials to Australian sites:

Action 3.2.4: *Building on existing initiatives, continue to foster an environment conducive to clinical trials for rare diseases taking place in Australia.*

There is arguably a role for the NMP in achieving this. Additionally, the NMP should recommend the use of novel trial designs, such as basket trials and N=1 trials that facilitate precision, gene and cell therapy trials. For decades, the rare disease community has heard about and waited eagerly for the future promise of gene and cell therapies, gene editing, genomics, precision and personalised medicine. The NMP should acknowledge that regulation and approval pathways for precision medicines must be fundamentally different from current pathways. This aligns with the Action Plan:

Action 2.4.2: *Ensure funding and reimbursement pathways are fit-for-purpose and sustainable for current and new health technologies for rare diseases.*

The NMP should also assist in enabling the implementation of a precision medicine subcommittee to acknowledge that the evaluation of precision medicines requires a different paradigm and expertise in contrast to the evaluation of therapies for large cohorts well suited to randomised control trials.

Rare disease registries

Registries play a critical role in clinical trial infrastructure yet there is no rare disease registry or clinical trial network in Australia. Additionally, there are numerous challenges such as multiple and onerous ethics approval and other bureaucratic processes. There is also a real need for education and support for rare disease organisations to enable them to participate in the design of trials.

The Action Plan identifies the need for a national, coordinated and systematic approach to the collection and use of rare disease data, including registries. It also highlights that rare disease researchers and clinicians require further funding support to translate research and innovation into clinical care.

Action 3.1.3: *Improve rare disease data collection and use, including best-practice safe storage, data sharing, custodianship, analysis, reporting and privacy requirements.*

Action 3.1.4: *Develop a national approach to person-centred rare disease registries to support national standards, best practice and minimum data sets.*

Action 3.2.4:: *Building on existing initiatives, continue to foster an environment conducive to clinical trials for rare diseases taking place in Australia.*

Implementation 3.4.2.1: *In partnership with industry, philanthropy and trial sites, identify and enhance existing capability and infrastructure within clinical centres to ensure appropriate capability is available to support the operation of clinical trials for rare diseases.*

Priority 3.4: *Translate research and innovation into clinical care; clinical care informs research and innovation.*

The NMP must include policy relating to registries and systematic, effective data collection to provide essential clinical trial infrastructure and evidence to support timely approval and funding pathways for emerging rare disease therapies.

TOR 4: Consider the centricity of the consumer within the NMP and whether it captures the diversity of consumers and their needs and expectation.

The NMP notes the fundamental role of consumers in achieving the policy's four objectives and identifies responsibilities for consumers. However, there is little detail regarding implementation mechanisms for involving consumers other than the following acknowledgement: "…ensuring consultation with consumer representatives when new arrangements are contemplated".

A foundation principle of the Action Plan is "person-centred" and a key action is:

Action 2.5.1: *Embed the voice of people living with a rare disease and their families and carers throughout structures and systems that impact rare diseases.*

The establishment of the Office of Health Technology Assessment (OHTA) Consumer Evidence and Engagement Unit is positive and signals important progress. Further investment is required to get the full benefits from such a unit. While the unit has clearly advanced consumer engagement, much more work could be progressed in regard to consumer evidence with adequate funding. Developing increased rigour and incorporating it into consumer evidence would greatly strengthen the quality use of medicines focus.

RVA acknowledges the important role played by consumer nominees/representatives on various HTA committees. However, there are limited guidelines and selection criteria for such roles. Australia is also a culturally and geographically diverse nation, yet there is no acknowledgement in the NMP of the diversity of consumers and their specific needs. We must continue to strive to capture and embed more diverse and representative voices. These voices must capture the seven priority populations identified in the Action Plan including Aboriginal and Torres Strait Islander people; people living in regional, rural and remote areas; people from CALD backgrounds; and people experiencing socio-economic disadvantage.

Question A. How can the NMP's focus on consumer centricity and engagement be strengthened? Is anything missing, and what needs to change?

Consumers regularly experience being 'last' in the consultation process and current policy and processes often prioritise patient reported measures (e.g. 'personal utility' is lower in the hierarchy of evidence). Additionally, consumers routinely provide comment as part of HTA processes without full transparency regarding what has been proposed by the sponsor. Earlier involvement of consumers at all stages of the development and approval of therapies has the potential to strengthen outcomes for patients.

In rare disease, it is particularly important for consumers to participate in systems that impact rare diseases (e.g. HTA processes). This is identified in the Action Plan:

Action 2.1.5: *Embed the voice of people living with a rare disease and their families and carers throughout structures and systems that impact rare diseases.*

With limited data and the uncertainty involved in rare disease, consumers can add much-needed context to the data presented in HTA. It is critical that HTA processes formally embed, capture, and promote the voice of people living with a rare disease and their families and carers. The OHTA Consumer Evidence and Engagement Unit is a great initiative that will assist in the provision of education and support to people living with a rare disease, their families/carers and rare disease organisations that wish to take a more active role in HTA processes.

RVA Recommendations for Question A:

RVA calls for the NMP to align with the following priority and actions detailed in the Action Plan:

Priority 1.2: *Ensure Australians living with a rare disease have access to information and education that enables them to be active participants in their rare disease journey.*

Action 1.2.2: *Improve consultation and communication between policy-makers and the rare disease community.*

Action 2.1.5: *Embed the voice of people living with a rare disease and their families and carers throughout structures and systems that impact rare diseases.*

Specifically, the NMP must emphasise early engagement with consumers in the development of clinical trial design, translational research, as well as regulatory and reimbursement applications. Consumer representatives should be appointed to advisory roles wherever possible and be given appropriate training regarding effective consumer representation.

Policy that supports consumer-led applications for access to therapies must also be developed. Australia's current HTA system is over-reliant on pharmaceutical company sponsor-led applications. If companies are not interested in submitting an application for commercial reasons, there is currently no viable way to assess a health technology, regardless of levels of unmet need. This means that many people living with a rare disease encounter challenges accessing a repurposed medicine that is prescribed by their clinician but only funded for use for a more common condition. There needs to be a sustainable way that clinicians and rare disease organisations can work with the OHTA Consumer Evidence and Engagement Unit to submit an application for public reimbursement of a technology eligible for assessment. Additionally, the OHTA Consumer Evidence and Engagement Unit should be given a broader remit to support building the health literacy and capacity of consumers, especially from very small disease groups to enable effective engagement in decision-making systems that directly impact them.

TOR 5: Identify options to improve the NMP's governance; communications, implementation (including enablers) and evaluation.

The NMP identifies stakeholders/partners with responsibilities for advancing each of the four objectives. The discussion consists of a high-level list of outcomes that partners/stakeholders are responsible for delivering under each objective and emphasises the importance of collaboration and the interrelationships between each objective. The medicines policy landscape has matured since the NMP was first published in 2000. Roles for partners/stakeholders will not remain static over time. This will change expectations and opportunities for the increased involvement of consumers, clinicians and researchers. There is also an opportunity for greater collaboration between the Commonwealth and State Governments. The NMP must help to enable and connect to the agreements that sustain this type of collaboration, such as the National Health Reform Agreement.

Over time, engagement with consumers and other stakeholders regarding medicines policies and HTA processes has been strengthened. This has resulted in the pressing need for timely, targeted and transparent communications with all stakeholders. Improved communication, including clear links between various policies and initiatives that are associated with the NMP, would reduce the perception of fragmentation and lack of coordination relating to medicines policy in Australia. All policies, programs and agreements must show how they link back to the NMP for increased transparency and accountability. Ensuring that this ongoing linkage is communicated across all policies, programs and agreements, would promote public recognition of a strategically aligned approach that also promotes the visibility of key partners/stakeholders and their work in delivering the NMP's objectives. Additionally, there is an opportunity to consider how a two-way exchange of information for partners/stakeholders to raise and consult on specific NMP issues can be better facilitated. This could be explored by leveraging established structures of consultation (e.g. the current processes in place to support comments on submissions to the PBAC).

Questions A. What opportunities are there to strengthen governance arrangements for the NMP? What would these be, and why?

RVA Recommendations for Question A:

Generally, the policy principles and objectives are strong and the emphasis on consumer-centricity is welcomed. The Action Plan acknowledges the importance of involving people living with a rare disease at every level of decision-making. One of the critical enablers of the Action Plan is "collaborative governance and leadership" and it is important that the NMP team continues to work collaboratively with all partners/stakeholders.

Question B. How can communication about the NMP be enhanced or improved?

RVA Recommendations for Question B:

In line with the Action Plan, RVA highlights the need for improved consultation and communication between policy-makers and the rare disease community.

Action 2.5.1: *Embed the voice of people living with a rare disease and their families and carers throughout structures and systems that impact rare diseases.*

This is essential in enhancing current communication about the NMP.

C. What would be effective mechanisms to support communication about the policy?

RVA Recommendations for Question C:

Incorporating reporting on both strategic and tactical actions to implement the NMP into other agreements such as the National Health Reform Agreement is critical. This will assist in increasing the visibility of and accountability for achieving the NMP's principles and objectives across both state and territory health systems. One of the Action Plan's critical enablers is "State, national and international partnerships". Such collaboration is vital in facilitating effective and accurate communication about the NMP to partners/stakeholders and the wider Australian community.

It is also necessary to incorporate reporting on strategic and tactical actions to implement the NMP in Government departments that have key responsibilities for ensuring timely, equitable and safe access to therapies including via HTA. Such departments include the TGA, PBAC, MSAC and the LSDP.

Input must also be sought from a range of stakeholders, including consumers, to assess the effectiveness of implementation and to identify tactical actions to address areas where improved implementation is required (e.g. in timeliness and equity of access to medicines for Australians living with a rare disease). One of the critical enablers of the Action Plan is "multi-stakeholder involvement and engagement." This is critical in the ongoing ability of the NMP to work towards the best health and wellbeing outcomes for Australians living with a rare disease.

Robust reporting and evaluation measures will provide the NMP team with the ability to provide accurate and detailed reporting to all partners/stakeholders.

TOR 6: Review the NMP partners and provide options for building greater accountability including addressing conflicts of interest.

The achievement of the NMP's objectives relies heavily on the ability of the partners/stakeholders listed under each objective to work together to implement the NMP. Further consultation on how each partners'/stakeholders' expertise can be better utilised and how those involved can best work together is necessary. A more balanced approach is needed. The reliance on multiple partners/stakeholders working collaboratively to deliver the NMP objectives highlights the importance of establishing strong accountability measures.

As noted throughout this Submission, RVA calls for increased levels of transparency. We particularly reiterate the importance of managing conflicts of interest. It is important to acknowledge power imbalance, inequity and the limited resources of some partners/stakeholders. Greater strategic alignment across medicines policy priorities is needed, in addition to transparency and accountability from all NMP partners/stakeholders in their delivery of the NMP's objectives.

A. Question A. How should the NMP's 'partnership-based' approach be defined?

RVA Recommendations for Question A:

It is not enough to simply list partners/stakeholders – it is imperative to also acknowledge how the partners/stakeholders can work together optimally. Partners/stakeholders must be supported by implementation mechanisms, guides and processes to facilitate effective partnerships.

Additionally, researchers must be included as a partner/stakeholder. It is critical that we encourage partnerships between rare disease researchers and clinicians. The Action Plan clearly identifies the need to better translate research:

Priority 3.4: *Translate research and innovation into clinical care; clinical care informs research and innovation.*

Question B. What is missing from the policy's reference to the NMP partners? Are there other partners that should be included in the policy? Who would they be and why?

RVA Recommendations for Question B:

The NMP team must work with peak bodies with HTA experience such as RVA to provide information, education and support to consumers to enable them to actively engage with and provide feedback on the implementation of the NMP. Working with RVA will ensure that a key priority of the Action Plan is addressed:

Priority 1.2: *Ensure Australians living with a rare disease have access to information and education that enables them to be active participants in their rare disease journey.*

C. How could the NMP be refreshed to support greater accountability amongst the NMP partners? How could the partnership approach be improved?

RVA Recommendations for Question C:

Multi-stakeholder evaluation of and reporting on the implementation of the NMP would support greater accountability among all NMP partners/stakeholders. One possible way to achieve this would be to expand the Medicines Australia's *Compare 5* report. This multi-stakeholder collaboration would enable partners/stakeholders to identify tactical actions to address outcomes from the evaluation.

The above recommendations are consistent with the following critical enablers in the Action Plan: "multi-stakeholder involvement and engagement"; "collaborative governance and leadership"; and "state, national and international partnerships"

D. How are conflicts of interest currently managed and should more be done to address this amongst the NMP partners? What approaches could be taken?

RVA Recommendations for Question D:

RVA does not believe a sufficient balance currently exists between greater transparency and avoiding conflicts of interests. Current policy settings result in consumers regularly being excluded from medicines/ HTA discussions/negotiations and having access to critical information. Often, consumers are attempting to engage with HTA with limited visibility of what information is included in a proposal. Mandating greater transparency and accountability that requires parties to report on interactions and how potential conflicts of interest have been managed will strengthen consumer-centricity and ensure conflicts are appropriately and visibly managed. It is important that the OHTA Consumer Evidence and Engagement Unit, as well as consumer-centred organisations with experience in HTA, are supported to continue providing relevant information and foster consumer engagement and awareness.

Some stakeholders have suggested that lessening restrictions on industry/consumer communications would improve transparency. RVA believes that a more appropriate and less risky approach is to reduce consumers' reliance on industry. It is important that other key NMP partners/stakeholders, such as the Department of Health, clinicians and peak bodies, take a more active role in communication activities. This will increase transparency and accountability while mitigating conflict of interest risks.

References

1. Commonwealth of Australia (Department of Health) 2020 ***The National Strategic Action Plan for Rare Diseases***

2. Medicines Australia (2019) ***Compare: Comparison of Access and Reimbursement Environments 2019*** Edition 5