Rare Voices Australia Position Statement: 'Government response – The New Frontier: Inquiry into approval processes for new drugs and novel medical technologies in Australia'

Background

On 30 November, the Australian Government published its response to *The New Frontier – Delivering better health for all Australians* report (the New Frontier report) tabled by the Standing Committee on Health, Aged Care and Sport (the Standing Committee) on 25 November 2021.

The New Frontier report responded to the Parliamentary Inquiry into approval processes for new drugs and novel medical technologies in Australia (the Parliamentary Inquiry) and contains 31 recommendations, including dedicated sections to rare diseases and the patient voice. It also aligns with several of the priorities, actions and implementation steps outlined in the Australian Government's National Strategic Action Plan for Rare Diseases (the Action Plan).

RVA's Submission Into the Parliamentary Inquiry

As the national peak body for Australians living with a rare disease, RVA's Submission into the Parliamentary Inquiry highlighted the critical issues that continue to persist for people living with a rare disease. It also suggested how Australia could build on the existing strengths of current systems in the most coordinated and systemic way to ensure processes enable Australians living with a rare disease to access the benefits of new medicines and emerging technologies now and in the future. You can read RVA's Submission on the Standing Committee's website (see Submission #86).

RVA thanks our RVA Partner groups/organisations, as well as other stakeholders in the rare disease sector, that lodged a Submission and participated in the Public Hearing conducted by the Standing Committee as part of the Parliamentary Inquiry. As a sector, we have ensured Australians living with a rare disease have remained central to this process.

This Position Statement

This Position Statement addresses the Australian Government's response. RVA's early analysis primarily focuses on the strengths of the government's response. We note that the government has accepted several recommendations in principle only. RVA recognises that this style of language is typical for a government response. We also note that the government refers to the role of the Health Technology Assessment Policy and Methods Review (HTA Review) currently underway in response to several of the recommendations. RVA recognises that such responses are appropriate and continues to actively participate in the HTA Review as the national peak body for Australians living with a rare disease.

RVA's early analysis primarily focuses on the strengths of the government's response while, in alignment with RVA's original Submission to the Parliamentary Inquiry, also highlighting opportunities and considerations to deliver a more nuanced approach for the rare disease community.

RVA will continue to advocate to the Australian Government and key decision makers and communicate to stakeholders accordingly. RVA Partner groups/organisations and other stakeholders are encouraged to reach out to RVA to discuss the government's response and its potential implications for Australians living with a rare disease.

The Australian Government's Response to the Recommendations

In this Position Statement, RVA has addressed the recommendations in order of their significance to the estimated two million Australians living with a rare disease.

Recommendation 11: Review Repurposing of Drugs

RVA is excited by the Australian Government's acceptance of this recommendation, which is critical for Australians living with a rare disease. Many people living with a rare disease must 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.

The government's response states:

"The Australian Government through the Department of Health and Aged Care is introducing a medicines repurposing program to improve patient access to treatments by assisting sponsors to expand the approved uses of their medicines."

The response continues to state:

"The program will target medicines for which a significant public health benefit has been identified but there is little or no commercial incentive for a sponsor to pursue regulatory approval and PBS listing to make this use more accessible. The program will strengthen the voice of clinician, academic and patient groups by encouraging their active participation in identifying new usages of medicines."

As detailed below, this was a key recommendation in RVA's original Submission to the Parliamentary Inquiry.

"Providing a viable pathway for consumers to make an application for public reimbursement of an eligible technology, and in particular for new indications for medicines already approved for more common conditions. In the rare disease context, small patient numbers can greatly reduce the commercial incentive for companies to submit applications, despite unmet need."

Recommendation 1: Establish a Centre for Precision Medicine and Rare Diseases

RVA welcomes the Australian Government's acceptance of this recommendation in principle and acknowledgement that, "the Department of Health and Aged Care needs to maintain the capacity to provide Australians with timely access to new drugs and novel medical technologies, including treatments for rare diseases, and cell and gene technologies." As the government notes, RVA recognises that "… a Centre for Precision Medicine and Rare Diseases requires detailed scoping." RVA is encouraged that in its response, the government recognises the important role of the Medical Research Future Fund (MRFF). Pleasingly, RVA welcomes the government's references to the Action Plan and its vital role in providing "… guidance and direction around key goals and priorities for Australians living with a rare disease and their families and carers."

In scoping this recommendation, RVA strongly calls for the consideration of the recommendations RVA included in its original Submission to the Parliamentary Inquiry, including establishing a Precision Medicine Advisory Committee for current approval processes. Importantly, RVA recommended that the government establish a Rare Disease and Precision Health Office at a higher level of government positioned more appropriately for high level government policy coordination. The focus of such a national Rare Disease and Precision Health Office could include:

- Ongoing review of legislation and health technology policy in line with scientific and genomic advancements
- Research policy and investment
- Data collection and epidemiology
- Workforce and systems capacity
- Investment into rare disease centres of expertise

RVA will continue advocating for a Centre for Precision Medicine and Rare Diseases and liaising with the government and other key decision-makers.

Recommendation 2: Establish a National Genomics Testing Program

RVA welcomes the Australian Government's acceptance of this recommendation in part.

Health technology assessment process for cell and gene therapies

RVA welcomed the New Frontier report's call for "… the HTA process for cell and gene therapies [to] be simplified to establish a clear and certain pathway for such therapies." We note that the government's response points to its commitment to the HTA Review and the Strategic Agreement with Medicines Australia, which "…includes a commitment to reviewing methods for evaluating new and emerging technologies (including cell and gene therapies, and other precision based medicines) and the suitability of existing funding pathways as required."

Cell and gene therapy pathways for clinical trials

The New Frontier report called for the Australian Government to "prioritise and simplify the regulation of cell and gene therapy pathways for clinical trials in Australia," which will expedite access to treatment for Australians living with a rare disease where a proven therapy is available. In its response, the government notes that "… the TGA [Therapeutic Goods Administration] will improve and provide transparency for Clinical Trial Approval scheme applications and provide for accessible communication materials on regulation of clinical trials and an advice service to assist research and clinical organisations to support these reforms." Additionally, the response states, "A single point of entry for all approvals/notifications required to conduct clinical trials in Australia will also be implemented, with work already underway." RVA will continue monitoring the impacts of these changes, however, RVA feels that more will need to be done. In our Submission to the Parliamentary Inquiry, we recommended the development of infrastructure, including an Australian Rare Disease Clinical Trial and Registry Network.

Action 2.4.2 of the Action Plan is:

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

Recommendation 3: Establish an Office of Clinical Evaluation

RVA welcomes the Australian Government's acceptance of this recommendation in principle and its agreement "… that clinical evaluation within the Department of Health and Aged Care must continue to support patient access to new and emerging health technologies." The response points to the HTA Review to outline how elements of this recommendation have been, or will be, addressed. RVA is also pleased to see the government's willingness to engage in international liaison and collaboration where beneficial and appropriate as 'State, national and international partnerships' is one of the critical enablers of the Action Plan.

In our Submission, RVA acknowledged that in addition to a lack of investment, there is often no policy or legislative support for increased development and use of novel rare disease therapies in Australia. There can also be challenges and barriers to appropriately integrate these therapies into clinical care.

Recommendation 4: Improving the Life Saving Drugs Program

RVA welcomes the Australian Government's acceptance of this principle, in part, and states its commitment to "… providing more timely access to new medicines for life-threatening and ultra-rare conditions." The response points to the HTA Review and "… a commitment to the review addressing methods for evaluating medicines for rare diseases for PBS [Pharmaceutical Benefits Scheme] reimbursement and alternative funding pathways." RVA welcomes the government's commitment to exploring policy and legislative options that remove the requirement to 'fail' at the PBAC before being potentially considered for funding through the Life Saving Drugs Program (LSDP).

RVA remains committed to working with the Australian Government regarding any potential policy reform of the LSDP. Additionally, we will continue to highlight the importance of a streamlined and transparent process.

Recommendation 6: Improving Education and Engagement for the TGA and the HTA processes

RVA welcomes the Australian Government's acceptance of this recommendation. Health technology assessment (HTA) approval processes directly impact Australian patients' access to new drugs and emerging technologies. In RVA's Submission, in line with the Action Plan, we emphasised the importance of embedding the consumer voice throughout systems that impact rare diseases. It is particularly important in rare disease for consumers to participate in HTA processes. With limited data and uncertainties, consumers can provide much needed

narrative and 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. In our Submission, RVA recognised the existing strengths within our current system, including the work of the Consumer Evidence and Engagement Unit. RVA recognises this is an area where there has been significant progress and that this is a focus of the HTA Review in addition to codesign work for an enhanced consumer process, which is currently underway. RVA has and will continue to actively participate in such work.

Recommendation 8: Submission Fee Waivers

The Australian Government notes this recommendation.

Legislation and regulation can greatly inhibit the development and use of novel therapies for rare diseases. Due to small patient numbers, it is not always commercially viable for companies to seek reimbursement for a rare disease indication.

RVA will continue to reaffirm our support for reforms relating to expediated pathways, parallel processes, orphan drug designations and fee waivers, which are important incentives for pharmaceutical companies. We will continue to urge the government to respond to current disincentives.

Recommendation 9: Funding for Submissions Without a Sponsor

The Australian Government notes this recommendation.

RVA welcomes the government's response acknowledging that while there is currently no legal or procedural barrier to not-for-profit organisations serving as a sponsor, "… there may be practical barriers to non-profit organisations acting as sponsors for products such as repurposed medicines" although the government notes there are "… several programs and initiatives that support the treatment and management of rare diseases including the TGA's orphan drug program and PBAC [Pharmaceutical Benefits Advisory Committee] cost recovery policy." In our Submission, RVA strongly called for the need to provide "…a viable pathway for consumers to make an application".

While we note the Australian Government's response, RVA will continue to highlight that Australia's current HTA system is over-reliant on pharmaceutical company sponsor-led applications. If companies are not commercially interested in submitting an application, there is currently no viable way to assess a health technology, regardless of levels of unmet need. This means that many people with a rare disease have challenges accessing a

repurposed medicine that is prescribed by their doctor but only funded for use with a more common condition. That said, we also acknowledge that funding is only one part of the challenge in this instance. RVA will continue to advocate for a way that clinicians and rare disease organisations can work with the HTA Consumer Evidence and Engagement Unit to submit an application for public reimbursement of a technology eligible for assessment by the Office of Health Technology Assessment via an appropriate, supported and sustainable pathway.

Recommendation 10: The PBAC and Managed Access Programs

RVA welcomes the Australian Government's acceptance of this recommendation in principle, "… noting that amendments to the National Health Act can only be made by the Australian Parliament." The response goes on to state:

"While these arrangements can lawfully be given effect under current legislation, options to specifically legislate the arrangements will need to be considered by the Australian Government."

A key priority of the Action Plan is to enable all Australians to have equitable access to the best available health technology. RVA acknowledges the potential of Managed Access Programs (MAPs) and the current low uptake rates. Due to the small patient numbers and systemic data challenges in rare disease, MAPs have the potential to provide people living with a rare disease with earlier access to treatment while also collecting data and building knowledge in the long-term. RVA will continue engaging with the government regarding MAPs.

Recommendation 12: TGA Reform

RVA welcomes the Australian Government's acceptance of this recommendation in part.

Evidence of prevalence

The Australian Government accepts this part of the recommendation, stating:

"The TGA already accepts evidence to support prevalence based on epidemiological data or databases available overseas supplemented with an explanation of the extrapolation to the Australian population…"

RVA will continue to support changes to the Orphan Drugs Program that will expedite access to treatment for many Australians living with a rare disease. This aligns with Action 2.4.1 of the Action Plan:

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

Recommendation 15: Membership of the PBAC and MSAC

RVA welcomes the Australian Government's acceptance of this recommendation in principle, stating, "… it is not feasible for a standing committee to hold expertise for every specialised application it receives, and the committees are able to source external expertise to supplement the expertise of their standing membership, including when dealing with very rare diseases or specialised therapies."

We welcome the Department of Health and Aged Care's commitment to "… continue to consult with First Nations organisations when seeking nominations from MSAC, PBAC and their subcommittee." Aboriginal and Torres Strait Islander people were identified as a priority population in the Action Plan.

RVA's Submission highlighted the lack of rare disease expertise and understanding that currently informs approval processes for rare disease therapies. Action 2.4.2.3 of the Action Plan is:

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

Additionally, we stated we were not clear on the MSAC's level of understanding of rare disease and precision medicine.

RVA will continue advocating in line with Action 2.4.2.3 of the Action Plan.

Recommendation 16: Increase International Collaboration

RVA welcomes the government's acceptance of this recommendation. The response states:

"Increased international collaboration has the potential to benefit Australians through the ability to support reduced market authorisation timeframes, support the TGA to make

informed and internationally consistent decisions about therapeutic products, and reduce regulatory duplication for industry."

One of the critical enablers of the Action Plan is, 'State, national and international partnerships.' As identified in the Action Plan, given the small populations and complexity involved in rare diseases, strong ongoing partnerships are invaluable. Global collaboration and the sharing of knowledge and expertise are often required to ensure the best outcomes for people living with a rare disease.

Recommendation 21: Improve Newborn Screening Program

RVA welcomes the Australian Government's acceptance of this recommendation in principle.

Newborn bloodspot screening (NBS) is an important program that supports the earliest possible diagnosis of numerous rare diseases. Priority 2.2 of the Action Plan is:

Ensure diagnosis of a rare disease is timely and accurate.

On 13 June 2023, the Department of Health and Aged Care updated its website to state they are "… working with states and territories to expand Australia's NBS programs, and make sure all babies born have access to the same screening." This milestone agreement ensures timely and consistent screening and wrap around care. Read the media release issued by the Department of Health and Aged Care for more information about these changes.

The response states:

"The Australian Government notes the Committee's call for increased support for treatment pathways for babies diagnosed with a disorder through NBS… The Australian Government, in collaboration with states and territories, will consider these clinical management pathways in developing implementation arrangements."

RVA will continue to work with the Department of Health and Aged Care, and states and territories, regarding NBS expansion.

Recommendations 22 to 26: Improve the Clinical Trial System in Australia

In RVA's Submission, one of our key recommendations was to develop policy and infrastructure that responds to the strong interrelation between approval processes, clinical trials and data collection. Action 3.2.4 of the Action Plan is:

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

It is critical that Australia attracts and incentivises pharmaceutical companies to conduct rare disease clinical trials in Australia, enabling people living with a rare disease 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. For many people living with a rare disease, participation in a clinical trial may be the only way to access treatment.

Recommendation 22

RVA welcomes the Australian Government's acceptance of this recommendation in principle.

This recommendation seeks to address the multiple and onerous ethics approvals and other bureaucratic processes currently involved in running a clinical trial in Australia. Data for most rare diseases is not captured in Australia in either health information systems or registries and there is no coordinated strategy to collect, measure, build and translate data that does exist. For many rare diseases, there are many barriers to effective research and no active research programs.

The government's response to this recommendation points to the National One Stop Shop platform for Clinical Trials and Human Research Approvals, and the related National Clinical Trials Front Door. In response to Recommendation 25, the government states that "The aim of the National One Stop Shop is to make it easier for patients, researchers, industry representatives to find, conduct, participate and invest in high quality and ethical research in Australia."

Beginning in late 2021, RVA attended several public and private consultations to contribute rare disease perspectives at different phases of the National One Stop Shop project. RVA also prepared two written submissions. Leveraging key priorities and actions in the Action Plan, the first Submission provided rare disease perspectives to the development of the

National Clinical Trials Governance Framework. Also leveraging the Action Plan, RVA's second Submission addressed the proposed national SSA minimum core elements and proof-of-concept web platform, which was developed in consultation with subject matter experts across all jurisdictions in Australia. Read more and download our submissions via RVA's website.

Recommendation 23

The Australian Government notes this recommendation, which aligns with Priority 3.1 of the Action Plan:

Enable coordinated and collaborative data collection to facilitate the monitoring and cumulative knowledge of rare diseases, informing care management, research and health system planning.

RVA is encouraged by this statement in the government's response and looks forward to the evolution of the National Clinical Trials Front Door:

"A key proposal explored as part of this process is the potential for a national recruitment portal – that is, the development of a sophisticated platform to facilitate patient identification and recruitment."

Recommendation 24

RVA welcomes the Australian Government's acceptance of part of this recommendation in principle and its overall intent.

The recommendation states that, "National standards should include standardising clinical costs and fees that are competitive with international fees." Again, this recommendation aligns with Action 3.2.4 of the Action Plan:

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

Digital technologies and practices

The Australian Government accepts this part of the recommendation in principle and points to the National One Stop Shop for Clinical Trials and Human Research Approvals "… to position Australia as a global leading destination for medical research including clinical

trials." RVA is encouraged by the development of a Teletrials Compendium, "… developed in collaboration with jurisdictions and key government agencies to support a consistent national approach to implementation of teletrials in Australia. This will particularly support and enable clinical trials in rural, regional and remote areas." People living in regional, rural and remote areas are identified as a priority population in the Action Plan; where people live can have a significant impact on their ability to access services. The lack of rare disease expertise nationally is exacerbated by Australia's vast size.

RVA is also encouraged by the government's intent to support information sharing:

"More broadly, the Australian Government is working to support information sharing across the health system, both for near real time clinical information and data sets to inform population health measures."

Recommendation 25

RVA welcomes the Australian Government's acceptance of this recommendation. The response states "… that a national standard approach, including nationally agreed systems and standard operating procedures, will support and strengthen the capacity to conduct clinical teletrials in rural, regional, and remote areas."

As noted, people living in regional, rural and remote areas are one of the priority populations identified in the Action Plan. Where people live can have a significant impact on their ability to access services.

Recommendation 26

RVA welcomes the Australian Government's acceptance of this recommendation in principle. The response states:

"The Australian Government will explore the most effective ways to support CTNs [Clinical Trials Networks] to strengthen sector capability and collaboration with the aim of embedding evidence-based care in the health system. This includes meeting the needs of, and achieving outcomes for, First Nations people and communities…"

In RVA's Submission, we supported this recommendation. RVA also called for the development of an Australian Rare Disease Clinical Trial and Registry Network; national infrastructure to support rare disease clinical trials to accelerate clinical trials in rare

diseases and to attract industry. RVA maintains that an Australian Rare Disease Clinical Trial and Registry Network is needed.

Recommendation 27: Research and Development

The Australian Government notes this recommendation. The response states:

"The Australian Government considers that additional periods of data exclusivity should be made available for orphan drugs and vaccines, but only where thorough analysis of the issue and evidence demonstrate this is necessary to encourage investment in these important areas."

Recommendation 28: The Patient Voice

RVA welcomes the Australian Government's acceptance of part of this recommendation in full and other parts in principle. The New Frontier report dedicated an entire section to the importance of the patient voice.

'Person-centred' is a foundation principle of the Action Plan and Action 2.1.5 is:

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

In RVA's Submission, in line with the Action Plan, we emphasised the importance of embedding the consumer voice throughout systems that impact rare diseases.

Patient engagement in HTA

The Australian Government accepts this part of the recommendation and RVA welcomes the Department of Health and Aged Care's commitment "… to continuing engagement with consumers and integrating the patient voice into the HTA system." We also welcome the commitment that "… the Commonwealth and Medicines Australia will work to co-design and implement an Enhanced Consumer Engagement Process to capture consumer voices in respect of applications to the PBAC. It is intended that the process will capture the consumer and patient voice at an earlier stage of consideration of applications to list new medicines, or to expand indications for existing medicines. It is also intended that this process, once implemented, will be capable of informing reimbursement submissions for particular medicines. This work will complement continuing process improvements for when and how consumers are engaged and involved in HTA processes."

Notification of new applications

The Australian Government accepts part of this recommendation and notes that the Department of Health and Aged Care "… already uses an electronic notification process in the form of e-bulletins for both the PBAC and MSAC process." RVA is also encouraged to note the redevelopment of the TGA website, which the response states "… will offer patients more options to subscribe to topics of their interest."

Submission summaries

The Australian Government accepts this part of the recommendation in principle. RVA encourages the provision of plain English summaries to consumers as part of HTA decision-making.

Patient evidence in PBAC and MSAC decisions

RVA reiterates the government response's acknowledgement of "… presenting patient evidence and highlight[ing] the importance of including patient-relevant outcomes. This includes outcomes that are directly relevant to the patient as well as those that reflect improvements in quality or length of life, family and societal outcomes and outcomes that relate to health disparities, such as equity of access, and areas of unmet clinical need."

Patient evidence in TGA decisions

The Australian Government accepts this part of the recommendation and RVA is encouraged by the response's statement that the "… TGA is better defining and communicating its use of Patient Reported Outcomes (PROs) and Real-World Evidence (RWE)…"

Notification outcomes

The Australian Government accepts this part of the recommendation and RVA acknowledges the launch of the HTA Consultation Hub in December 2021. We support the intent that "… this portal will also be used to provide additional information about MSAC outcomes…"

Implementation funding

The Australian Government accepts this aspect of the recommendation in principle. RVA welcomes the Department of Health and Aged Care's acknowledgement of "… the substantial time and resources required of consumer organisations to participate in HTA processes. Many consumer organisations… do not have the capacity to provide the type of input requested or to generate consumer evidence for HTA processes." RVA acknowledges that several initiatives are aimed at improving engagement and communication with patients and consumer groups regarding HTA-related matters.

It's important to highlight that funding is not the only barrier for consumers — they also need a pathway that is appropriate, supported and sustainable. RVA reiterates the important role of the HTA Consumer Evidence and Engagement Unit, which is well positioned to assist in the provision of education and support to people living with a rare disease and their families and carers, and/or rare disease groups/organisations to support them to take a more active role in HTA processes. Equally as vital is building the capacity of rare disease organisations to facilitate their participation in the co-design and coordination of trials.

Recommendation 29: Improving the HTA process

RVA welcomes the Australian Government's acceptance of this recommendation in principle. The response notes that changes to the National Health Act can only be made by the Australian Parliament.

There are many strengths in Australia's current HTA processes, however, these strengths do not exist consistently across all approval processes. This is a real issue as every approval process assesses rare disease and precision health technologies.

RVA commends the work of the TGA and PBAC around parallel processes. Similarly, RVA believes the PBAC is the gold standard in terms of transparent timelines and consumer engagement. However, its criteria regarding cost effectiveness are challenging in the rare disease context, as is the reliance on traditional clinical trial evidence.

HTA Policy and Methods Review

The response reiterates that the terms of reference for the HTA Review "… will consider HTA policy and methods for all medicines and vaccines, highly specialised therapies, and other health technologies (for example a pathology test or an imaging technology) that improve

health outcomes associated with the aforementioned technologies." For Australians living with a rare disease, it's vital that relevant aspects of the HTA process ensure there are future pathways for treatments and therapies that do not fit neatly into the current system such as rare cancers, antimicrobials, orphan drugs, and precision medicines. As highlighted in the New Frontier report, "… precision medicine approval pathways will require a different application assessment than current approaches designed for treatments for common conditions, with large data sets and comparative evaluations."

RVA's Submission also highlighted the lack of transparency across certain elements of the HTA process. RVA will continue advocating for HTA processes that are equitable for Australians living with a rare disease.

Reporting implementation of report recommendations

For increased transparency, RVA is encouraged by the statement that the "… Australian Government will consider appropriate reporting for implementation of review recommendations, noting many of the recommendations relate to existing initiatives that already have reporting mechanisms in place."

Recommendation 30: Review of HTA

RVA welcomes the Australian Government's acceptance of this recommendation. The response points to the Strategic Agreement with Medicines Australia and the terms of reference for the HTA Review.

As noted, RVA—alongside the rare disease sector—has been actively participating in the HTA Review. We will continue this important work as equitable access to health technology is a key priority of the Action Plan.

Recommendation 31: MSAC

RVA welcomes the Australian Government's acceptance of some parts of this recommendation in full and some parts in principle.

Increased clinician involvement in MSAC

The Australian Government accepts this part of the recommendation. RVA's Submission noted that the MSAC's level of understanding of rare disease and precision medicine is unclear. RVA is encouraged by the response's indication that "From time to time targeted

consultation may also occur with an individual who has specialist knowledge or expertise that can inform MSAC's deliberations."

International collaboration

The Australian Government accepts this part of the recommendation, and the response states that "The Australian Government and the MSAC will explore opportunities for appropriate international collaboration with similar HTA agencies." RVA is pleased to see the government's willingness to engage in international liaison and collaboration where beneficial and appropriate as 'State, national and international partnerships' is one of the critical enablers of the Action Plan.

Inclusion of MSAC in HTA Policy and Methods Review

The Australian Government accepts this part of the recommendation. In RVA's response to the New Frontier report, we fully supported this recommendation. The government's response states, "The Reference Committee [for the HTA Review] will consider policy and methods used by the MSAC, focussing on those used for evaluating cell and gene technologies and medicines that are improved by the use of a diagnostic test."

Benchmarking for MSAC assessments

The Australian Government accepts this part of the recommendation in principle.

RVA's Submission noted that in recent times, there has been a massive variation in the many areas that the MSAC assesses. As noted, the MSAC's level of understanding of rare disease and precision medicine is unclear. We also highlighted the lack of transparency around timelines and formal consumer engagement and our concern that applications were typically unsuccessful. RVA called for the refinement of the approval process to ensure that the MSAC can respond in a timely manner to the new and novel therapies that provide much hope for the rare disease community.

The government's response states:

"The Department of Health and Aged Care is continuing to streamline and digitise the processes to support the public funding of health technologies through the progressive roll out of the Health Products Portal to manage applications considered by the MSAC. Alongside this work, the Department of Health and Aged Care is developing a metrics framework to measure and report on MSAC assessments."