Parliamentary Inquiry Position Statement: 'The New Frontier - Delivering Better Health for All Australians'

On 25 November 2021, the Standing Committee on Health, Aged Care and Sport (the Standing Committee) tabled its report into the Parliamentary Inquiry into approval processes for new drugs and novel medical technologies in Australia (Parliamentary Inquiry) in Parliament. *The New Frontier – Delivering better health for all Australians (*the *New Frontier* report*)* contains 31 recommendations, including dedicated sections to rare diseases and the patient voice. It also aligns with a number of the priorities, actions and implementation steps outlined in the National Strategic Action Plan for Rare Diseases (the Action Plan).

Trent Zimmerman MP, Chair of the Standing Committee, and its Deputy Chair, Dr Mike Freelander MP, each spoke to the report for five minutes in Parliament after the report was tabled. Rare Voices Australia (RVA) thanks Mr Zimmerman and Dr Freelander for engaging with the rare disease sector throughout the process and for their ongoing commitment to Australians living with a rare disease. Additionally, we thank all members of the Standing Committee for their work on this report.

RVA's Chief Executive Officer (CEO), Nicole Millis, was invited to a private briefing with Mr Zimmerman and Dr Freelander to discuss the report's findings immediately after the report was tabled on 25 November. The CEOs of Medicines Australia and the Medical Technology Association of Australia (MTAA) were also invited to the briefing.

Thanks once again to our RVA Partner organisations, as well as other key stakeholders in the rare disease sector, who lodged a Submission and participated in the Public Hearings earlier this year. As a sector, we have ensured that Australians living with a rare disease have remained central to this Parliamentary Inquiry throughout the process.

RVA's Submission

RVA 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 our current system in the most coordinated and systemic way to ensure processes enable Australian rare disease patients to access the fullest 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).

Next Steps

The Standing Committee included a number of the recommendations that RVA called for in our Submission and as opening witness during the Public Hearings. The recommendations in the *New Frontier* report are positive steps forward for people living with a rare disease and the entire rare disease sector. However, as these recommendations are yet to be

implemented, it is imperative that the rare disease sector continues to leverage the Action Plan to ensure that these recommendations translate to policy that transforms people's lives. RVA looks forward to the Government's response to this report and in the meantime, will continue to actively engage with key political leaders on implementation.

Related Reviews

In July 2022, the Department of Health is scheduled to expand its independent Health Technology Assessment Review. Meanwhile, the National Medicines Policy (NMP) Review recommenced in August 2021 and the consultation process continued throughout October and November. You can read RVA's Submission here. RVA also had the opportunity to participate in a one-on-one interview with the NMP Review Committee. We are confident the Review Committee now has a strong understanding of considerations from the rare disease sector. The Review Committee noted where possible, they would look to align their recommendations with the Action Plan.

As a sector, we must continue to ensure that the views of people living with a rare disease are heard as part of both Reviews. As the national peak body for Australians living with a rare disease, RVA will be advocating and engaging in consultations regarding these Reviews at every given opportunity. We will also consult with our RVA Partner organisations and other stakeholders as required.

This Position Statement

This Position Statement is designed to be read alongside the *New Frontier* report. RVA's early analysis focuses on the strengths of the recommendations. We acknowledge the breadth and complexity of the *New Frontier* report which is 360 pages long. RVA intends to undertake further analysis of the report to identify any potential gaps or concerns on behalf of Australians living with a rare disease. We will continue to advocate to Government and key-decision makers and communicate to stakeholders accordingly. RVA Partners and other stakeholders are encouraged to reach out to RVA to discuss the report and its potential implications for Australians living with a rare disease.

Recommendations: *The New Frontier – Delivering better health for all Australians*

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

In RVA's Submission, one of our key recommendations was the establishment of a Rare Disease and Precision Health Office in Government, acknowledging the importance and future promise of precision health in driving person-centred healthcare.
In its recommendation, the Standing Committee specifically states, "The objective of the Centre should be to ensure that the capacity of the Department of Health is enhanced to provide Australians with timely access to new drugs and novel medical technologies, including for rare diseases…"

In line with RVA's Submission, the recommendation also states that, "The Centre should provide advice to governments on the establishment of a dedicated regulatory Health Technology Assessment pathway for cell and gene technologies, in consultation with state and territory governments, industry, patients and other relevant stakeholders. The Centre should regularly provide advice to government on the effectiveness of those pathways and areas for further reform." Additionally, the recommendation adds, "The Centre should provide advice to the Department of Health and the Australian Medical Research Advisory Board on research priorities."

In time, RVA envisions that the focus of a Centre for Precision Medicine and Rare Diseases could also include:

- Data collection and epidemiology
- Workforce and systems capacity
- Investment into rare disease centres of excellence

High level Government policy coordination in the form of a Centre for Precision Medicine and Rare Diseases would go a long way towards progressing the Action Plan's Vision: "The best possible health and wellbeing outcomes for Australians living with a rare disease."

Recommendation 2: Establish a National Genomics Testing Program

RVA's Submission noted that 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. RVA also acknowledged that gene therapy is time critical and that currently, there is no process in Australia 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 disease and will increasingly become an issue with the global trend towards personalised and precision health. It is a significant barrier preventing Australians accessing new medicines and emerging technologies.

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.

RVA welcomes Recommendation 2 of the *New Frontier* report, which calls for "… the Health Technology Assessment (HTA) process for cell and gene therapies be simplified to establish a clear and certain pathway for such therapies." In line with "equity of access" and "person-centred," which are both foundation principles of the Action Plan, we also support the recommendation's stipulation to "… establish a jointly funded national genomics testing program to provide equitable access to genomic testing nationwide. As part of the program, governments should ensure the provision of genomics counselling for all patients." Importantly, Recommendation 2 also calls 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.

Recommendation 3: Establish an Office of Clinical Evaluation

Recommendation 3 speaks to an "Office of Clinical Evaluation within the Department of Health to assess the best and most effective care for patients in the context of new and emerging health technologies." This is yet another promising recommendation for Australians living with a rare disease.

RVA's Submission 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.

We also welcome the ongoing thread throughout the *New Frontier* report that encourages international liaison and collaboration where beneficial and appropriate. "State, national and international partnerships" is one of the critical enablers of the Action Plan.

Recommendation 4: Improving the Life Saving Drugs Program

The Standing Committee recommended "… that the assessment process for the Life Saving Drugs Program (LSDP) be streamlined and delays in access to treatments be reduced by ensuring that a sponsor only need lodge one application for one Health Technology Assessment pathway."

The Standing Committee has put forward two different avenues to facilitate this recommendation. As when reforms to the LSDP were announced in 2018, RVA has always highlighted the importance of a streamlined and transparent LSDP process. RVA remains committed to working with the Australian Government regarding any potential policy reform of the LSDP.

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

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 emphasized 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 are able to 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.

People living with a rare disease can only contribute meaningfully to HTA processes if they understand the differences between the approval processes. Pillar 1 of the Action Plan is dedicated to Awareness and Education. Priority 1.2 is:

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.

RVA welcomes the Standing Committee's recommendation "… that the Department of Health increase its efforts to educate and engage with patients, clinicians, industry and the public and develop education campaigns on all aspects of the regulation and reimbursement system." RVA has been encouraging clearer and more timely communication between the Department of Health and all stakeholders in the rare disease sector for some time and we believe the proposals outlined in Recommendation 6 would go some way to addressing the issues that stakeholders have raised with RVA in the past. However, it is not the whole answer. Additionally, it is important to ensure that approval processes are fit for purpose.

Recommendation 8: Submission Fee Waivers

Due to small patient numbers, it is not always commercially viable for companies to seek reimbursement for a rare disease indication. In RVA's Submission, we acknowledged this and the need for Government to respond to current disincentives. RVA also reaffirmed our support for reforms in recent years relating to expediated pathways, parallel processes, orphan drug designations and fee waivers, which are important incentives for pharmaceutical companies.

RVA welcomes Recommendation 8, recognising that legislation and regulation can greatly inhibit the development and use of novel therapies for rare diseases.

Recommendation 9: Funding for Submissions without a Sponsor

RVA's Submission highlighted that Australia's current Health Technology Assessment (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.
While RVA supports the sentiment behind Recommendation 9, as funding is only one part of the challenge in this instance, we maintain that there needs to be 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 (OHTA) via an appropriate, supported and sustainable pathway.

Action 2.4.3.3 of the Action Plan is:

The TGA [Therapeutic Goods Administration] and OHTA must work together to develop clear processes and pathways for sponsors considering submitting applications for the repurposing of medicines already approved for use in treatment of other conditions.

Recommendation 10: The PBAC and Managed Access Programs

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. A key priority of the Action Plan is to enable all Australians to have equitable access to the best available health technology.

Recommendation 11: Review Repurposing of Drugs

RVA's Submission highlighted the importance of repurposing of medicines already approved for use in treatment of other conditions. These medicines play an important role and present an opportunity to address unmet need in the treatment of rare diseases.
RVA welcomes the Standing Committee's recommendation that "… the Department of Health conduct a comprehensive consultation process with industry to establish a more flexible way forward for the repurposing of drugs in Australia. This should include:

- Establishing a new pathway that incentivises the repurposing of drugs for all 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. Currently, 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 are already being 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 etc. This means that many Australians who are living with a rare disease have 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.

Recommendation 12: TGA Reform

RVA supports Recommendation 12's proposed changes to the Orphan Drugs Program. The recommendation to, "Provide automatic access to the Priority Review Pathway for all medicines granted an orphan drug designation," 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.

Likewise, we are encouraged by the focus on international liaison and collaboration where relevant, which is evident in the following recommendation: "Where the prevalence of a disease is unknown in Australia, accept evidence of prevalence in other comparable countries or, in diseases of extremely low prevalence, worldwide for the purposes of the eligibility criteria."

Recommendation 15: Membership of the PBAC and MSAC

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 said we were not clear on the Medical Services Advisory Committee's (MSAC) level of understanding of rare disease and precision medicine.

RVA supports the Standing Committee's recommendation "that the Australian Government ensure the membership of the Pharmaceutical Benefits Advisory Committee and Medical Services Advisory Committee provides the appropriate expertise for all applications." Additionally, given the challenges typically at play in rare disease, we welcome the inclusion of "…enhanced cross-membership between the two committees and the appointment of temporary members to consider individual applications" as part of Recommendation 15.
Recommendation 15 also speaks to "…the nature of health challenges in Indigenous communities, membership should include representation from Aboriginal and Torres Strait Islander Peoples." Aboriginal and Torres Strait Islander peoples are one of the priority populations identified in the Action Plan. Several factors increase the potential impact of rare diseases on Aboriginal and Torres Strait Islander people.

Recommendation 16: Increase International Collaboration

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. As such, RVA welcomes the recommendation that "… the Department of Health investigate further opportunities for the formation of an international Health Technology Assessment consortium…"

Recommendation 21: Improve Newborn Screening Program

While the Standing Committee acknowledged that newborn screening was not in the terms of reference for this Parliamentary Inquiry, they dedicated Recommendation 21 to newborn screening in response to "…calls from rare disease patient groups for more funding for treatment pathways for actionable disorders across states and territories, where identified through newborn screening."

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.

RVA welcomes the Standing Committee's recommendations regarding the NBS program, particularly the focus on "actionable disorders" rather than "treatable"; as well as recommending that "…federal, state and territory health authorities complete the standardisation of newborn screening across Australia." RVA is pleased that the Standing Committee has recognised that, currently, important work and reform is already progressing at both a Commonwealth and state and territory level. However, RVA also welcomes the Standing Committee highlighting that there is more work to be done before the NBS process is fully completed.

RVA remains committed to continuing our work with the Department of Health as well as states and territories regarding NBS.

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

In our Submission, one of RVA's 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 take place in Australia.

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. For many people living with a rare disease, participation in a clinical trial may be the only way to access treatment.

Recommendation 22, which "recommends that all levels of government prioritise and implement with urgency the harmonisation of Human Research Ethics Committee (HREC) and Site-Specific Assessment submissions into one Australian online platform and enable parallel review by HRECs and Research Governance Offices," seeks to address the multiple and

onerous ethics approvals and other bureaucratic processes currently involved in running a clinical trial in Australia.

In Australia, data for most rare diseases is not captured 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. **Recommendation 23** calls for "… the development of a national clinical trial register" and the inclusion "…of a sophisticated digital platform to collect and facilitate patient identification, patient recruitment, patient retention and completion rates for clinical trials." This recommendation 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."

Recommendation 24 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 take place in Australia.

Recommendation 25 proposes that "the Australian Government should develop a national standard approach, including nationally agreed systems and standard operating procedures to support and strengthen the capacity to conduct clinical tele-trials in rural, regional and remote areas." 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. The lack of rare disease expertise nationally is exacerbated by Australia's vast size.

RVA's Submission 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 disease and to attract industry. RVA maintains that an Australian Rare Disease Clinical Trial and Registry Network is needed, however, we support **Recommendation 26**, which states, "…the Australian Government should continue to fund Clinical Trial Networks with a particular focus on developing seed funding for Indigenous Health Clinical Trial Networks."

Recommendation 27: Research and Development

RVA welcomes the Standing Committee's recommendations to:

- "Develop additional reforms to data exclusivity timeframes to support research and development into new drugs and novel medical technologies in areas of unmet need.

- "Consider future funding initiatives for novel drug discovery and support research and development partnerships in Australia. This would assist new drugs and novel medical technologies in early stage and pre-commercial development."

Recommendation 28: The Patient Voice

The *New Frontier* report dedicates 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 emphasized the importance of embedding the consumer voice throughout systems that impact rare diseases.
RVA welcomes all elements that form Recommendation 28. The recommendation reads, "The Department of Health integrate the patient voice upfront into the Health Technology Assessment system" and goes on to propose, "The Australian Government provide funding for organisations to support participation in the HTA process, including for very rare disease patient groups that have limited capacity for fundraising or access to alternative funding." While we support these improvements, again, we 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 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.

RVA strongly supports the following elements of Recommendation 28:

- "The Department of Health provide patients and stakeholders with a concise sponsor's submission summary to help facilitate their own involvement in the Health Technology Assessment process.
- "The Department of Health should consider making patient evidence compulsory for certain applications, and should consider the role of patient evidence in the decisions of the Therapeutic Goods Administration.
- "The Department of Health should notify relevant patient groups of the outcome of the assessment process by all HTA bodies."

Recommendation 29: Improving the HTA process

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 Pharmaceutical Benefits Advisory Committee (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 is challenging in the rare disease context, as is the reliance on traditional clinical trial evidence.

RVA welcomes the Standing Committee's recommendation that, "The independent Health Technology Assessment Review reassess relevant aspects of the Health Technology Assessment process to 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." Additionally, we commend the Standing Committee for highlighting "… that 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. We believe the following recommendations will assist in addressing these issues:

- "The Department of Health publish data on application processing times and positive recommendation rates for the Pharmaceutical Benefits Advisory Committee and other Health Technology Assessment bodies. In addition:
- The Department of Health should publish Health Technology Assessment processing times annually, benchmarked against other nations with advanced HTA processes.
- "The Australian Government, in collaboration with relevant stakeholders, develop a suite of clear and measurable benchmarks to track the Commonwealth's implementations of the recommendations made by the Committee and accepted by the Australian Government. – These agreed benchmarks along with measurable KPIs/metrics should be developed in such a way as to best facilitate the Department of Health, including its agencies and other relevant statutory bodies, in the tabling of an annual update to the Australian Parliament."

Recommendation 30: Review of HTA

The Standing Committee has made a number of recommendations for the Australian Government's independent Health Technology Assessment Review (which is scheduled to commerce in July 2022) to consider.

RVA looks forward to playing an integral role in this consultation in 2022 on behalf of Australians living with a rare disease and welcomes all of the Standing Committee's recommendations. From a rare disease perspective, the following recommendations are of particular importance:

- "Streamlining the interaction between hospitals and the Health Technology Assessment system

- "Streamlining the interaction of the Therapeutic Goods Administration, the Pharmaceutical Benefits Advisory Committee, the Medical Services Advisory Committee and other Health Technology Assessment bodies
- "Cooperation and harmonisation between Australian Health Technology Assessment bodies and equivalent bodies overseas
- "Increasing the use of Real World Evidence in Health Technology Assessment
- "Introducing a scoping process that includes patients and clinicians at an early stage to agree on the framework that the submission will be considered.
- "Improving the independent review process for HTA decisions, including the potential for this to be made available to groups of patients and clinicians in addition to sponsors."

Recommendation 31: MSAC

RVA's Submission noted that in recent times, there has been a massive variation in the many areas the Medical Services Advisory Committee (MSAC) assesses. As already 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 is able to respond in a timely manner to the new and novel therapies that provide much hope for the rare disease community.

RVA fully supports the Standing Committee's recommendation that, "The Department of Health expand the independent Health Technology Assessment Review in July 2022 to include Medical Service Advisory Committee processes." Likewise, RVA supports the following recommendations:

- "The Medical Services Advisory Committee increase the involvement of clinicians in its assessments of technologies with which its members lack relevant expertise
- "The Therapeutic Goods Administration introduce parallel processing of applications with the Medical Services Advisory Committee
- "The Medical Services Advisory Committee consider developing international collaboration for complex assessment proposals
- "The Medical Services Advisory Committee publish a full calendar timeline of meeting agenda and outcomes, including dates when minutes and Public Summary Documents will be made public
- "The Department of Health establish a benchmarking system for MSAC assessments, including benchmarking against comparable overseas organisations."