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Rare Voices Australia submission: Parliamentary Inquiry into approval processes for new drugs and novel medical technologies in Australia

Executive Summary

Rare Voices Australia (RVA) welcomes the opportunity to provide a submission to the Parliamentary Inquiry into approval processes for new drugs and novel medical technologies in Australia. RVA is the national peak body for the estimated two million Australians living with a rare disease. RVA has provided a response to each of the Terms of Reference (TOR) and four overarching recommendations that are aligned with the priorities, actions and implementation steps of the Australian Government's National Strategic Action Plan for Rare Diseases (the Action Plan). RVA led the collaborative development of the Action Plan which was launched in February 2020 with bipartisan support.

RVA commends the Parliamentary Inquiry's clear focus on approval processes and the broad scope of the TOR to include areas such as drug development, technological innovation, repurposing of medicines, commercial incentives, personalised/precision medicines, and clinical trials. RVA recognises that these areas are closely interrelated and as such, a broad focus is necessary to achieve systemic change that is most effective.

Additionally, RVA would like to acknowledge the strengths of the Therapeutic Good Administration (TGA) and Australia's health technologies assessment (HTA) approval processes. We reaffirm our support of reforms in recent years relating to expediated pathways, parallel processes, orphan drug designations and fee waivers, which are important incentives for pharmaceutical companies. RVA also strongly supports the increasing formalisation of consumer engagement through the establishment of the Consumer Evidence and Engagement Unit; and increased transparency, timelines, and stakeholder engagement within the Pharmaceutical Benefits Advisory Committee (PBAC) and Life Saving Drugs Program (LSDP) approval processes. RVA's submission will highlight the critical issues that continue to persist for people living with a rare disease. It will also suggest how we 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.

A brief summary of challenges faced by rare disease patients

As there are limited treatment options for rare diseases, it is essential that people living with a rare disease can 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.

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.

There are many examples of an approved medicine (for a more common condition) demonstrating benefits for a rare disease. However, due to small numbers, it is not always commercially viable for companies to seek reimbursement for a rare disease indication. It is difficult, if not impossible, for a non-pharmaceutical sponsor to submit an application. Without government reimbursement, many rare disease medicines are unaffordable for people living with a rare disease and their families.

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. For many people living with a rare disease, participation in a clinical trial may be the only way to access treatment. Yet there are many challenges to running clinical trials. For example, there is no national infrastructure for rare disease clinical trials, nor a streamlined national ethics approval process.

Depending on the specific rare disease, research priorities can be different. While funding for translational research may be important for many rare diseases, some rare diseases are not yet in the position to prioritise translational research. For some rare diseases, the unmet research needs are basic discovery research or investment into data collection and natural history studies. The Action Plan states that investment into all types of research related to rare disease is needed.

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Summary of RVA's Recommendations

The below recommendations are a combined response to the TOR for this Parliamentary Inquiry. Each recommendation is closely aligned to priorities, actions and implementation steps detailed in the Action Plan.

Recommendation #1 – Build on current strengths of HTA to ensure they are applied consistently across ALL approval processes.

Recommendation #1 facilitates:

- Streamlined and timely processes.
- Parallel processes and expediated pathways.
- Greater transparency and certainty around which approval process will be used.
- Transparent and detailed timelines for all HTA approval processes.
- Effective stakeholder (consumer and clinician) engagement, both early and throughout the entire approval process.
- Key decisions throughout approval processes are appropriately guided by independent expertise that includes clinical, consumer and industry perspectives.

Recommendation #2 – Strengthen rare disease and precision health regulatory focus within HTA

Recommendation #2 entails:

- Adding two extra principles underpinning Australian HTA Processes (see Appendix 1), related to:
  1. Equity.
  2. Precision health.

  The addition of these two principles will strengthen and future-proof HTA to best respond to new medicines and emerging technologies. These new principles will enable the important and necessary refinement of HTA processes. Such a revision would be timely considering the last Review of HTA was conducted by the Australian Government in 2009.
- Increasing rare disease and precision health expertise within the Office of Health Technology Assessment (OHTA).
- Enabling prioritised consideration of innovative and alternative forms of evidence when appropriate.
- 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.
- Encouraging companies that are making submissions for rare disease health technologies to engage with consumers/consumers groups early in the development of their submission and to demonstrate that any research undertaken has been co-designed with consumers and includes patient reported outcome measures (PROMs).

- The establishment of a specific Advisory Committee for Precision Medicine to address gaps in current approvals processes and to acknowledge that precision medicine approval pathways will require a different evaluation paradigm than current approaches designed for treatments for common conditions, large data sets and comparative evaluation.

Recommendation #3 – Develop policy and infrastructure that responds to the strong interrelation between approval processes, clinical trials and data collection

Recommendation #3 involves:

- 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.
- Reducing barriers to clinical trials in Australia including:
    1. Operating multi-trial sites that share common resources.
    2. Streamlined, single point ethics approval processes.
- Regulatory recognition of novel clinical trial designs.
- Providing stronger incentives for co-design of trials to ensure clinical measures are more closely connected and aligned to PROMs.
- Building the capacity of rare disease organisations to facilitate their participation in the co-design and coordination of trials.
- Better linking people living with a rare disease to clinical trials in Australia and overseas, as well as to other research activity, such as data collection, registries, natural history studies and qualitative research.
- Building on existing investment into the entire rare disease research pipeline. Not all rare diseases are at clinical trial stage. Fundamental discovery research is critical in testing and progressing innovative, new and emerging technologies.
- Incentivising the inclusion of Australia in international clinical trials by providing benefits (for example, an expedited HTA approval process when Australian data is utilised). This will place added value or a 'positive weighting' towards clinical trials being conducted in Australia.
- Encouraging and incentivising not-for-profit consumer organisations to aid with clinical trial participant recruitment.

Recommendation #4 – High level Government policy coordination

Recommendation #4 facilitates:

- 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. A number of similar exemplars already exist, both nationally and overseas. Locally, the Precision Health Council in Western Australia (a ministerial council) was established in 2019. Internationally, in the United States, the Rare Diseases Office within the National Institute of Health has statutory authority and was established through the Rare Diseases Act (2002).

The focus of a national Rare Disease and Precision Health Office could include:

1. Ongoing review of legislation and health technology policy in line with scientific and genomic advancements in health technology. For example, mitochondrial donation prevents mitochondrial DNA from being inherited by a genetically related offspring. Mitochondrial donation is not yet legal in Australia.
2. Research policy and investment.
3. Data collection and epidemiology.
4. Workforce and systems capacity.
5. Investment into rare disease centres of excellence.

Detailed discussion of each term of reference (TOR)

TOR 1: The range of new drugs and emerging novel medical technologies in development in Australia and globally, including areas of innovation where there is an interface between drugs and novel therapies.

The Action Plan highlights that there are limited treatment options for rare diseases. The International Rare Diseases Research Consortium (IRDiRC) states that people living with a rare disease need research into diagnostics (including genomics), development and testing of new health technologies, and precision or personalised medicine. In this context, the development of new drugs and novel medical technologies is vital, as is novel use or repurposing of existing technologies for rare disease patients.

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. In 2020, the Nobel Prize in Chemistry was awarded to two scientists for their work on the CRISPR gene editing tool. These therapies and technologies are currently being developed. They are no longer pipe dreams, but part of current research pipelines. Australia needs to act urgently to ensure Australians living with a rare disease can experience the best health and wellbeing outcomes thanks to these transformative technologies.

As well as 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. In the rare disease sector, it is well known that developing a treatment option is only part of the challenge. Even when a treatment for a rare disease does exist, a subsidy or reimbursement may not be available in Australia, limiting access to patients.

In 2014, an Australian report found that Australians generally gain access to rare disease therapies anywhere between two to four years after comparable countries such as the United Kingdom, Canada, Germany and the Netherlands. Through RVA's work with our Round Table of Companies, a group of pharmaceutical companies with a common interest in rare diseases and orphan drug development, we know that internationally, the pharmaceutical industry's general perception is that Australia is generally a challenging market with uncertain approval processes. As such, the Australian market is often allocated lower priority by international companies. This causes great despair, frustration and confusion for Australian rare disease patients when they see that 'their' medicines are available in other countries but not here in Australia. With the pharmaceutical industry not prioritising bringing products to Australia, this creates a knock-on effect with less urgency for industry to consider Australian sites for clinical trials. These consequences are terrible for Australians living with a rare disease, particular for the many whom participation in a clinical trial is the only way to access treatment.

TOR 2: Incentives to research, develop and commercialise new drugs and novel medical technologies for conditions where there is an unmet need, in particular orphan, personalised drugs and off-patent that could be repurposed and used to treat new conditions.

RVA believes that Government should invest more funding into research for novel treatment options. This is recognised in the Action Plan which calls for a national research strategy for rare diseases to foster and drive all types of research for rare diseases (Priority 3.2), including prioritised and targeted investment into fundamental discovery research for rare diseases (3.2.2.4). Fundamental discovery research is of central importance to the development and testing of much-needed innovation in health technology for rare diseases. Currently, the full research pipeline – from basic discovery research through to clinical trials and commercialisation for rare disease therapies for Australian patients – does not often take place in Australia. Action 3.2.3 in the Action Plan calls for the support [of] collaborative research into rare diseases in Australia and internationally. Implementation recommendations for Action 3.2.3 include:

- financial incentives for research teams that can demonstrate collaboration with national, international and industry partners; and
- the development of customised research grants for rare diseases that require a degree of collaboration with national, international and industry partners.

There is also a lack of knowledge and information available to patients and rare disease organisations about novel/new treatments. The Action Plan highlights the need to better translate research and innovation into clinical care, and vice versa (Priority 3.4). Partnerships between rare disease researchers and clinicians should be supported. Implementations recommendations for Action 3.4.1 and Action 3.4.2 include:

- Research funding bodies prioritise research proposals and applications for rare diseases that can demonstrate support from, and close working relationships with, clinicians.
- Support and foster interdisciplinary research teams to encourage more person-centred research, and a dual focus on research and clinical care where appropriate.
- Support clinical teams to collect and input data, contributing to research and evidence-building

Repurposing of medicines already approved for use in treatment of other conditions plays an important role and 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 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.

While Government needs to incentivise research into new medicines and technologies, it must also identify and respond to current disincentives. Legislation and regulation can greatly inhibit the development and

use of novel therapies for rare diseases (for example, the lack of regulatory recognition of novel clinical trial designs).

TOR 3: Measures that could make Australia a more attractive location for clinical trials for new drugs and novel medical technologies.

TOR 3 closely aligns to 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.

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. 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 other challenges such as multiple and onerous ethics approval and other bureaucratic processes. There is much that needs to, and can be, done to build clinical trial infrastructure, increase economies of scale and streamline clinical trials. Existing capability and infrastructure within clinical centres should be identified and enhanced to ensure appropriate capability is available to support the operation of clinical trials for rare diseases (See implementation recommendation 3.4.2.1 of the Action Plan).

There are examples in rare disease where clinical trials have been conducted in Australia primarily as a result of strong advocacy led by rare disease families and/or clinicians. Although wonderful outcomes for these individual communities, this is neither sustainable nor equitable for rare disease research more broadly. For most Australians living with a rare disease, RVA is aware that it can be difficult to understand how best to link with research activity whether that be a clinical trial or other research activity, such as data collection, registries, natural history studies, or qualitative research, based in Australia and internationally. With appropriate support and additional capacity, rare disease organisations are well positioned to assist with clinical trial participation.

There is also a real need for education and support for rare disease organisations to enable them to participate in the design of trials, to ensure research into rare diseases is collaborative and person-centred (see Priority 3.3 of the Action Plan). Our rare disease researchers and clinicians also need further funding support to translate research and innovation into clinical care; clinical care informs research and innovation (see Priority 3.4 of the Action Plan).

Additionally, the Action Plan encourage the adoption of unique and appropriate trial designs that overcome rare disease research challenges (see Action 3.2.4.3 of the Action Plan). While such innovative trials exist (e.g. N of 1, adaptive, organoids, basket trials), they are not encouraged nor incentivised by Australia's current HTA processes, which are less adaptive. Double blind placebo clinical trials are still preferred best practice even when unfeasible for a particular patient cohort. This has negative significant impacts in rare disease and precision/personalised medicine development and approval in Australia.

TOR 4: Without compromising the assessment of safety, quality, efficacy or cost-effectiveness, whether the approval process for new drugs and novel medical technologies, could be made more efficient, including through greater use of international approval processes, greater alignment of registration and reimbursement processes or post market assessment.

Importance of approval processes

Approval processes directly impact Australian patients' access to new drugs and emerging technologies. Indirectly, they also greatly impact the pharmaceutical industry's appetite to bring new drugs and emerging technologies to the Australian market and to conduct clinical trials in Australia. From RVA's interactions with pharmaceutical companies, primarily through our Round Table of Companies, we are aware that Australia is seen as a challenging market for rare disease products, with rigid evidentiary criteria, and complex and uncertain HTA approval processes. Generally, pharmaceutical companies (and patients) believe that approval processes are long and cumbersome and lack transparency. Additionally, there is an expectation that rare disease products are not typically approved the first time around and multiple resubmissions are required. This perception alone is damaging as it deters companies from applying and results in Australia failing to be identified as a priority market.

Consideration of evidence

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. One of the principles underpinning Australian HTA processes is 'fit for purpose'. Despite the inherent limited data in rare disease, our HTA system does not seem to value or encourage other forms of evidence when appropriate, whether that is other forms of trials but also inclusion of real-world data from contemporary and emerging case studies.

Gene therapy is time critical. 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. This is also recognised in the Action Plan (see Action 2.4.2): Ensure funding and reimbursement pathways are fit-for- purpose and sustainable for current and new health technologies for rare diseases.

Complexity of rare disease

Rare diseases are complex, with limited data 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. There should be an opportunity to bring together stakeholders to discuss potential challenges/gaps and troubleshoot complexities earlier in the process. Existing approval processes mean that such gaps are not highlighted and are often responded to by consumer input later in the process. There is also a lack of rare disease expertise and understanding currently informing approval processes for rare disease therapies. The Action Plan highlights the need to ensure rare disease expertise exists, or can be accessed, on all reimbursement pathways and HTA advisory bodies (see Action 2.4.2.3). Similarly, a strong understanding of precision medicine and novel clinical trial designs must be evident throughout the process.

Over-reliance on pharmaceutical companies
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. 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 OHTA. The TGA 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 (see Action 2.4.3.3 of the Action Plan).

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. The HTA 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 and their families and carers, and/or rare disease organisations to support them to take a more active role in HTA processes.

Australian HTA consists of multiple approval processes, each with its own specific role and expertise, eligibility criteria and assessment models. Multiple approval pathways potentially enable a more fit-for-purpose response. However, this can only be the case if stakeholders understand the differences between the approval processes and if there is a high level of transparency about its specific expertise, timelines and consumer engagement exists. More clarity around decision-making is vital, particularly in relation to how evidence is considered in assessing clinical and other effectiveness, as distinct to how evidence is considered to assess cost effectiveness or value for money. There is a lack of transparency and concern when the consideration of these areas become merged, or when one is seen to be unduly influencing the other.

Need to build upon current strengths of HTA
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 is challenging in the rare disease context, as is the reliance on traditional clinical trial evidence. Meanwhile, the 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. However, 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 (MSAC) has expertise with tests, devices, and blood products. In recent times, there has been a massive 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 expediated

processes, these processes can only be considered for resubmissions. RVA was particularly concerned by comments made recently by MSAC representative, Dr Sarah Norris, at a recent 'Introduction to Medical Services Advisory Committee (MSAC)' webinar on 27/8/2020. Dr Norris briefly stated that the length of the process was about 12 months and that "typically, applications were not successful." The approval process should be refined to ensure that it is able to respond in a timely manner to the new and novel therapies that provide much hope for the rare disease community.

Lack of clarity and transparency around approval pathways for gene therapy

There is a very obvious lack of clarity and transparency around approval pathways for new medical technologies, and in particular, for gene therapies. RVA has heard multiple messages from the Department of Health that all gene therapies would be evaluated by the MSAC. However, a rare disease gene therapy is currently on the PBAC Agenda. While RVA has no issue with this end decision, RVA is very concerned about the impact of such uncertainty regarding which approval process to use. In this instance, the patient group believes it has resulted in a six-month delay in deciding which approval pathway was more appropriate. Such a delay is unacceptable for rare disease patients and can result in irreversible disease progression.

During the first hearing of this Inquiry, the Department of Health said the decision to consider gene therapies via the MSAC was patient-centred, by prioritising where a medicine will be administered to the patient. RVA does not agree with messaging that such a decision is patient-centred. RVA knows that rare disease patients actually place more value on transparency, timeliness, effective consumer engagement, and knowledge and understanding of their disease and its impact. HTA is a comparator system. If the MSAC considers gene therapy they are likely to have no experience with assessing comparative current therapies, or knowledge of the particular patient cohort. The accumulation of knowledge in rare disease is vital. Similarly, RVA has heard the explanation that MSAC handles more complex applications. RVA's understanding is this also refers to complex funding arrangements as it seems that the MSAC handles applications that relate to complex funding arrangements between federal and state Governments.

For the MSAC process, it is vital that clear timeframes to reach and publish outcomes, similar to the PBAC's timeframes, are implemented and made public. RVA Partners (specific rare disease organisations) report a higher level of confidence in the PBAC rather than the MSAC pathway due to the increased certainty provided by clear timelines and transparent consumer engagement processes. This certainty is vital as many rare diseases are progressive. 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.

Appendices

A: Australian Government HTA Processes are guided by the:

- Vision: Australians have timely, equitable and affordable access to the cost-effective health technologies needed to manage their health.
- Goal: to maximise beneficial health outcomes to the Australian population within the overall funds available whilst being cognisant of the other important goals of the health system.
- Objectives: use the best available evidence and efficient methods to inform robust decisions about market entry and the subsidised use of health technologies. The Australian Government HTA system should also continually improve the evidence base for assessment and operate according to agreed principles.

Principles underpinning Australian Government HTA Processes

Australian Government HTA processes reflect the following principles:

- Sustainable
- Transparent, accountable, and independent
- Consultative and reflective of Australian community values
- Administratively efficient
- Flexible and fit for purpose
- Informed by robust and relevant evidence

B: Case studies – Off-label access to repurposed medicines for rare diseases

One source of inequity, uncertainty and confusion for rare disease patients is the lack of clear pathways to access treatments that are on the PBS for more common conditions. There are several aspects to this:

1. Patients needing to self-fund a treatment that they would receive as a Government subsidised treatment if they had a more common condition.
2. Ongoing off-label use of the treatment
    a. Reliance on seeing a prescriber who has an understanding of the rare condition and the benefits of off-label use.
    b. Reliance on hospital funding which only considers individual applications – no certainty regarding ongoing access.
3. Uneven funding of the treatment as in-patients. This leaves patients in some locations accessing the treatment while patients in another location cannot access it or alternatively, creates uncertainty about whether or not they will have access to the treatment as an outpatient or during subsequent admissions.

Case studies have been provided to the Standing Committee separately and in confidence.

C: Hospital data

In the rare disease context, companies often state that it is unfeasible to submit for new indications, due to extremely small patient numbers, lack of conventional clinical trials etc. This means that many people 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) to access a medicine that is recommended by their clinician. This is both unsustainable and inequitable.

RVA has sourced de-identified data from one of Australia's largest public hospitals (that also includes a children's hospital) around their funding of off-label medicines. This is a sample from 2017 to August 2020. The data shows 570 instances of off-label hospital use that relate to a total of 30 medications for a total of 144 different indications/ reasons.

Detailed data reports have been provided to the Standing Committee separately and in confidence.