Rare Voices Australia Submission: Australian Commission on Safety and Quality in Health Care's National One Stop Shop and the National Clinical Trials Front Door Proposal

Executive Summary

Rare Voices Australia (RVA) welcomes the opportunity to provide a submission to the Australian Commission on Safety and Quality in Health Care's (the Commission) the One Stop Shop and the National Clinical Trials Front Door proposal. RVA is the national peak body for the estimated two million Australians living with a rare disease.

RVA has provided a rare disease perspective for clinical trials in Australia and recommendations that align with the priorities, actions and implementation steps of the Australian Government's National Strategic Action Plan for Rare Diseases (the Action Plan)[1]. RVA led the collaborative development of the Action Plan, which was informed by extensive multistakeholder consultation. The Action Plan was launched in February 2020 with bipartisan support and RVA is now leading its collaborative implementation. The Action Plan provides guidance and direction around key goals and priorities of the rare disease sector in Australia and includes a strong patient voice.

RVA commends the vision and efforts of the Commission to ensure multistakeholder involvement in the design and build of the National One Stop Shop. RVA's submission shares the need for a nuanced approach to rare disease clinical trials in Australia and the importance for the National One Stop shop to meet the needs of all rare disease stakeholders, including researchers, clinicians, healthcare professionals, industry as well as patients and their families.

Clinical Trials for Rare Diseases in Australia

Rare diseases are often serious and progressive, so taking action is time critical. For many living with a rare disease, participation in clinical trials is the only access to treatment. Due to limited treatment options, it is essential that people living with a rare disease can benefit from new transformative treatments and health technologies through better access to clinical trials.

Even where a potential new treatment is available, the health of those waiting often progresses to debilitating and even life limiting stages before access to a clinical trial is granted. This is due to the lack of national infrastructure for rare disease clinical trials and the largely fragmented mechanisms for clinical trials approval in Australia. Delayed or lack of access to clinical trials for rare disease is further compounded by a lack of industry interest due to relatively low demand. There is much that needs to, and can be, done to build clinical trial infrastructure, increase economies of scale and streamline clinical trials for rare disease. RVA would like to see faster trial enrolment, shorter approval timelines and improvements in rare disease clinical trial data collection and interoperability.

It is vital to ensure the nuances of rare diseases are considered in the development of the National One Stop Shop. The Action Plan outlines the importance of making processes in Australia more conducive to clinical trials for rare diseases in Australia. Actions and implementations steps from the Action Plan around rare disease clinical trials are listed in Appendix A.

Contents

RVA's Recommendations

These recommendations highlight the specific needs of the rare disease community and align with the priorities, actions and implementation steps in the Action Plan.

Recommendation 1: The National One Stop Shop should encourage a national accreditation process for clinical trial sites and streamlined cross-jurisdictional ethics approval processes.

RVA strongly supports the need for a national accreditation process for clinical trial sites. Streamlined and harmonised ethics approval processes and site authorisation systems are critical to ensure timely access to the tsunami of new and emerging therapies that are on the horizon for rare diseases. Timely access to new drugs and novel medical technologies including genomics, cell and gene therapies, as well as precision medicine for rare diseases, is one of the recommendations of *The New Frontier—Delivering Better Health for All Australians*[2]. The Parliamentary report recommends significant reforms to Australia's health care system to ensure people have better and faster access to the wave of new medicines and technologies.

Australia's geographical spread, together with the very low patient numbers inherent to rare diseases, mean that recruiting patients for trials requires streamlined processes for timely approval of multi-centre trials or decentralised trials. However, there is no national infrastructure for rare disease clinical trials, nor are there streamlined national approval processes.

RVA strongly supports the need to improve the current National Mutual Acceptance (NMA) Scheme to ensure that Human Research Ethics Committees (HRECs) and trial sites across Australia trust and accept one another's processes and that these processes are made transparent to all stakeholders. Currently, the NMA Scheme does not work across all states for first in human or first in patient clinical trials, which is a major barrier for rare disease patients needing access to new and emerging therapies. This also disincentives sponsors from bringing clinical trials to Australia. Re-prosecution of a clinical trial by different HRECs across different trial sights is time intensive and means that people living with a rare disease wait too long for what could be their only access to a lifesaving treatment. Longer waits from this lack of harmonisation can also lead to the irreversible progression of rare disease pathology, and, in some cases, life threatening consequences.

Recommendation 2: The National One Stop Shop should house public facing information about infrastructure, expertise and capacity at clinical trial sites, including clinical trial staff capacity and links to registries.

It is critical that Australia attracts and incentivises pharmaceutical companies to conduct rare disease clinical trials in Australia and enables patients to participate in international clinical trials without leaving Australia (See Action 3.2.5 of the Action Plan). Currently, there is a lack of coordinated infrastructure to support a national approach for rare disease clinical trials with very small patient numbers. 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). This would make sharing resources easier and facilitate multi-site trials.

Registries are also vital clinical trial infrastructure, yet there is no national rare disease registry or clinical trial network in Australia despite high levels of unmet need. RVA supports the need for the National One Stop Shop to link with registries and the proposed inclusion of a database for rare diseases and treating clinicians. Australian clinicians and specialised healthcare staff with specific expertise in rare disease are few and geographically spread. Many primary healthcare professionals

may not even know where to refer their patients to specialists with knowledge around clinical trials. Therefore, a central location for both patients and primary health care professionals to access this information is vital to timely access to treatment. Australian based and international sponsors would also benefit from easy access to information about trial sites to fast track their planning and decision making.

Recommendation 3: The National One Stop Shop should incentivise the sharing of information related to, if/how patients and the public are/were involved in the planning of clinical trials. Where possible, it should also enable patients and public to participate in trial design.

Clinical trials designed by researchers and clinicians without direct consultation with patient target groups have been known to miss the mark when it comes to recruitment and retention. What matters to patients does not always align with the questions or intentions of researchers and clinicians. RVA encourages the National One Stop Shop to educate researchers and sponsors around the importance of including patients and public early in the planning and design stages of clinical trials. The value of the patient voice across all areas of drug development and approval was also highlighted in a dedicated section of *The New Frontier—Delivering better Health For all Australians.*

The National One Stop Shop should ask sponsors and clinical trial leaders to disclose information around patient involvement in their clinical trial design. Furthermore, all stakeholders involved in planning a clinical trial and their roles should be clearly disclosed and readily available on the National One Stop Shop. These should be shared together with real time updates on safety and outcomes that include patient-reported outcome measures (PROMs) and patient-reported experience measures (PREM's).

Recommendation 4: The National One Stop Shop should facilitate the adoption of unique and appropriate trial designs that overcome rare disease research challenges (see Appendix A, Action 3.2.4.3 of the Action Plan), and it should be responsive to innovative new treatments and technologies.

Extremely small patient numbers and a lack of existing knowledge are a major barrier to rare disease clinical trials. Not only do small patient numbers make large scale double-blind placebo-controlled clinical trials impossible, but they also make economies of scale inaccessible. Learnings from the impact of COVID-19 have revealed the value and success of decentralised clinical trials. Decentralised trials can overcome the geographical barriers to accessing clinical trials in Australia. This type of trial would also increase equity of access (a foundation principle of the Action Plan) and enrolment numbers, which ultimately strengthens the data that can be put forward for approval and reimbursement processes.

The need to support innovative trial designs for rare diseases cannot be understated, particularly considering rapidly moving advancements in precision/personalised medicine and cell and gene therapies. Cell and gene therapies are becoming increasingly relevant for people born with rare single gene disorders. Advances in genetic testing and the intention to integrate genetic testing into health care mean the number of people diagnosed with genetic disorders will grow. Therefore, ensuring that the National One Stop Shop is built to respond to such growth and innovation is vital.

Recommendation 5: The National One Stop Shop should facilitate multistakeholder collaboration and integration of clinical trials into health care.

RVA is committed to ensuring that every way forward in the rare disease sector is collaborative and person-centred, and this is emphasised throughout the Action Plan. Access to participation in clinical trials should not be limited by geographical location, or practitioner interest or knowledge. All systems and centres must be integrated, and health care professionals must work together to ensure that information is shared, and clinical trial access is embedded in health care independent of jurisdiction.

RVA is excited by the proposed functionality of the National One Stop Shop to facilitate information sharing. Information about where and when clinical trials are happening should be easily accessible to all stakeholders, including patients, researchers, clinicals and health care providers. There should also be automated systems to ensure that all interested stakeholders are notified of forecast opportunities and when a clinical trial is recruiting.

Recommendation 6: The National One Stop Shop should encourage and enable not-for-profit consumer organisations to aid with clinical trial design and participant recruitment.

RVA supports the real time availability of clinical trials information proposed by the National One Stop Shop, from planning in the initial stages right through to implementation, monitoring and data analysis. Consumer organisations are well placed to share information about clinical trials with their communities and facilitate co-design and recruitment. Therefore, RVA would encourage the National One Stop Shop to include direct sharing of real time updates with relevant patient organisations around forecast opportunities, opportunities for co-design, recruitment timelines and clinical trial outcomes.

Recommendation 7: The National One Stop Shop should participate in horizon scanning for emerging treatments and medical technologies.

Horizon scanning is a critical mechanism for ensuring the National One Stop Shop is responsive to new medical technologies and can rapidly identify and flag changes or innovations that might warrant adaptations to approval systems. A focus on horizon scanning for transformative medicines is also emphasised across several recommendations and by a broad range of stakeholders in *The New Frontier—Delivering Better Health For all Australians*.

Eighty per cent of rare diseases have genetic origins and advancements in genomic medicine and cell and gene therapies are opening a world of life saving and potentially preventative or curative new treatment options. It is vital that the National One Stop is prepared for these transformative technologies and has a system to inform relevant stakeholders.

The National One Stop Shop should have inbuilt capability to enable all stakeholders to flag clinical trials for particular treatments in Australia. This would enable those with capacity or interest to identify, sponsor and facilitate a trial. Furthermore, it presents a way for the National One Stop Shop to be a sounding board for seemingly smaller voices to identify areas of particular clinical or medical interest to Australians.

Recommendation 8: The National One Stop Shop should be used to collect data on gaps and strengths across clinical infrastructure and resources to inform a national strategy that enables timely access to clinical trials that is not impeded by geography or bureaucracy.

RVA commends the National One Stop Shop's ambition to collate and share transparent clinical trials information in one centralised location. Additionally, we see that it also has immense potential to inform and adapt the way forward for clinical trials in Australia. RVA sees the National One Stop Shop as a valuable data source for research to identify gaps, limitations and strengths in clinical trials infrastructure, governance and approval processes at national, state and territory levels. Identifying strengths can inform best practice in locations where limitations are obvious, and highlighting the gaps creates impetus for advocacy into clinical trials policy reform.

Recommendation 9: The National One Stop Shop should be simple to navigate and accessible by all stakeholders, including Indigenous Australians, CALD communities and those living with disability.

RVA supports the need for plain language statements and information that is easy for all stakeholders to read and understand. Special consideration should be given to cultural nuances and to reducing language barriers to accessing information housed on the National One Stop Shop, including for Aboriginal and Torres Strait Islander people and those of CALD background (Implementation step 2.1.2.3 of the Action Plan). In line with this, recommendation 26 of the *The New Frontier—Delivering better Health For all Australians* suggests that the Australian government focus on developing seed funding for Indigenous Health Clinical Trial Networks. Furthermore, many people living with rare disease live with a range of disabilities, so it is critical that this platform be accessible to everyone independent of their ability. To this end, RVA supports intentions to allow all interested stakeholders to trial and test the National One Stop Shop website for usability before it is launched.

References

1. Australian Government Department of Health. (2020). *National Strategic Action Plan for Rare Diseases.* https://www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases

2. Parliament of the Commonwealth of Australia. House of Representatives Standing Committee on Health and Aged Care and Sport. (2021). *The New Frontier—Delivering better health for all Australians. Inquiry into approval processes for new drugs and novel medical technologies.* *https://parlinfo.aph.gov.au/parlInfo/download/committees/reportrep/024755/toc_pdf/TheNewFrontier-DeliveringbetterhealthforallAustralians.pdf;fileType=application%2Fpdf*

Appendix A – Excerpts from the National Strategic Action Plan for Rare Diseases

Action 3.2.4

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

Implementation

3.2.4.1. Develop recommendations to encourage and enable more clinical trials for rare diseases to take place in Australia.

3.2.4.2. Increase the economies of scale of research into rare diseases by, for example, operating multi-trial sites that share common resources.

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

Action 3.2.5

Investigate and promote options that enable Australians living with a rare disease to participate in clinical trials and other research activity, both in Australia and internationally (without needing to leave Australia).

Implementation

3.2.5.1. Identifying and maximising utilisation of available resources and assets to the extent possible, link people living with a rare disease to research activity, such as data collection, registries, natural history studies, qualitative research and clinical trials based in Australia and internationally.

3.2.5.2. Investigate and promote options for a Trials Enabling Program (TEP) for trials for rare diseases in Australia, leveraging a partnership approach that involves philanthropy and industry in the absence of relevant clinical trials in Australia.

Action 3.4.2

Identify, leverage and enhance existing capability and infrastructure to ensure appropriate and experienced resourcing is available within clinical teams that deliver rare disease care.

Implementation

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

3.4.2.2. Support clinical teams to collect and input data, contributing to research and evidence-building.