Rare Voices Australia's Detailed Position: National Consultation to improve alignment and coordination between the Medical Research Future Fund and the NHMRC's Medical Research Endowment Account

Executive Summary

Rare Voices Australia (RVA) welcomes the opportunity to provide input to improve the strategic alignment and coordination between the Medical Research Future Mission (MRFF) and the National Health and Medical Research Council's Medical Research Endowment Account (MREA), for an approach to health and medical research investment in Australia that meets the needs of all stakeholders.

About Rare Voices Australia

RVA is the national peak body for the estimated two million Australians living with a rare disease. RVA led the collaborative development of the National Strategic Action Plan for Rare Diseases[1] (the Action Plan), the first nationally coordinated effort to address rare diseases in Australia. The Action Plan includes 3 interrelated pillars—Pillar 1: Awareness and education, Pillar 2: Care and Support, and Pillar 3: Research and Data.

RVA has a strong track record in systemic advocacy for broad rare disease policy reform across government departments, including health, disability and research. RVA advocacy was critical in highlighting the need for a greater rare disease research focus in Australia, which led to the MRFF's investment in the Rare Cancers, Rare Diseases and Unmet Need competitive grant program. Collaborative development of the Action Plan further highlighted the need for more coordinated investment into rare disease research.

Rare Voices Australia and Research

In line with the Action Plan, RVA supports the need for high quality collaborative research that is person-centred and positively impacts the lives of Australians living with a rare disease. Through a formal research partnerships program, RVA is increasingly involved in research projects led by rare disease experts in hospitals and universities around Australia. RVA's roles in research range from in-kind support to develop grant applications and attend expert advisory groups, to budgeted roles, including full-time equivalent allocations for facilitating workshops and focus groups, co-designing project deliverables and plain language document reviews.

Academic researchers are increasingly recognising the value of RVA's peak body perspective, and rare disease policy expertise in their work. During 2022 alone, RVA engaged in research partnerships with 17 multidisciplinary research teams on MRFF grants and one NHMRC Partnership grant. Over 44 per cent of these grants were chosen for funding. Up to June 2023, RVA has received over 10 requests to partner with researchers on proposals for MRFF funding.

The MRFF has been pivotal in lifting the profile of rare disease research in Australia.

The Importance of Investment in Rare Disease Research

Limited data and knowledge are inherent features of rare disease. For key decision-makers at all levels, greater knowledge of rare diseases can facilitate more responsive and appropriate services for people living with a rare disease and their families and carers. The Action Plan responds to the significant potential for positive change in this space.

For many rare diseases, there are a number of barriers to effective research and no active research programs. One of the biggest challenges is that rare diseases have small patient numbers and are often very complex. Depending on the specific rare disease, research priorities can be different. For example, 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.[1]

Investment into all types of research related to rare disease is needed. This research includes:

- rare disease coding;
- data collection and registries;
- fundamental discovery research;
- qualitative research;
- pre-clinical testing; and
- clinical trials.[1]

Research into rare diseases must address existing gaps and the coordination of research projects must be prioritised. Improved policy settings, and national and international collaborations, will help to drive strong research and innovation for all rare diseases. Research into rare diseases needs to inform evidence-based policy across all systems, extending beyond health to incorporate disability, social/welfare, mental health, education, employment and housing.[1]

RVA supports any approach to streamline processes for health and medical research funding in Australia that ensures equity of research across the pipeline from fundamental discovery research right the way through to clinical translation, including equity of access to the benefits of knowledge and expertise delivered through research and embedded through policy. Streamlining governance and administration of health and medical research should not compromise but rather strengthen a person-centred, fit-for purpose approach that responds to unmet need, is truly translational—embedding research into clinical care, and promotes accountability, transparency, consistency, partnerships and diversity.

Contents

Rare Disease Research Funding: Current Landscape

Before addressing the guiding questions of this consultation, RVA would like to highlight how funding for rare disease research has changed since the Australian Government set up the MRFF in 2015, and the barriers and enablers to engaging in health and medical research as the national peak body for rare disease.

Based on anecdotal evidence from RVA's discussions with Australian researchers, until the launch of the MRFF, it was difficult to secure government funding for rare disease research. The MRFF's commitment to priority driven research has been transformative for rare disease. This is partly due to the inherent high levels of unmet need and high burden in rare disease , where knowledge is limited and treatments are few. Moving forward, it is vital that the MRFF's principles are preserved and continue to support rare disease research.

By contrast, the MREA has not been as evidently effective in responding to rare disease. Whilst the MREA may be funding rare disease research, it is not as apparent, and it is difficult to measure. Based on RVA's experience formally partnering with researchers on grant opportunities, the MRFF tends to attract and fund the majority of rare disease research in Australia. This is likely contributing to inequity in the types of rare disease research being conducted, with the balance tipped more to translational research than fundamental discovery-based research. The Action Plan and international rare disease consortia have highlighted the need for rare disease research across the entire pipeline, but the current system for funding is not adequately or consistently supporting this.

Any changes to governance and administration of health and medical research funding must ensure an equitable and effective flow of knowledge to impact for better health outcomes that is secured with strong nationally aligned policies. This would be best achieved through multistakeholder partnerships and diverse advisory structures. Nationally aligned policy is vital to ensuring consistent and equitable delivery of health innovations for the inherently small and geographically dispersed population of Australians living with rare disease.

Rare Voices Australia's Responses to Guiding Questions

RVA's responses to these questions are guided by the Action Plan and centred around addressing the health and medical research needs of people living with a rare disease.

1. What benefits should be achieved through improving the alignment and coordination of the MRFF and MREA?

Any efforts to improve alignment and coordination of the MRFF and MREA should not dismantle existing established structures. Rather they should leverage from and strengthen the integrity, transparency, consistency, multistakeholder partnerships and accountability of existing systems and approaches. In addition to governance and administrative changes that increase alignment and coordination, the roles of both funds need to be more clearly differentiated, in a way that enhances their complementarity and encourages greater collaboration across the research pipeline. The new governance model should:

- be fit-for-purpose to maximise the impact of available funding;
- be person-centred;
- strengthen partnerships—effectively engage all stakeholders in decision making, including consumers, peak bodies, Departments of Health (DoH), policymakers and hospital and health systems personnel;
- strengthen and clearly demonstrate links between health, research, and health policy for equitable dissemination and implementation of new knowledge—embedding research into healthcare;
- minimise duplication;
- decrease burden on applicants, particularly for time poor clinician-researchers who are a vital link between patients and research and have real-time knowledge of healthcare gaps;
- increase equity across the research pipeline and across health disciplines;
- value the importance of health policy as a driver of equity;
- value and resource the involvement of consumer and health policy expertise in decision making; and
- support effective policy for equitable research to healthcare translation, including reimbursed health technologies.

2. Which feature/s of the models will deliver these benefits?

Best practice in rare disease care is only possible when research is embedded in clinical care delivery. RVA was pleased to see mention of embedding research in the healthcare system as the first consideration in strengthening both the MRFF and the MREA. Any efforts to this end are vital for ensuring Australians have timely access to cutting edge technologies and clinical trials, which are the only way to access treatment for many people living with rare disease.

The Australian Government's National Strategic Action Plan for Rare Diseases and RVA's strong relationships with policymakers and health departments have enabled greater investment in rare disease research, particularly through the MRFF. Given the transformative impact of the MRFF on rare disease research, RVA strongly advises that the benefits for rare disease in this fund be safeguarded and strengthened through any new governance model.

To ensure consistency and equity in health and medical research, any new governance model must enable clear links and partnerships between research, clinical care and health policy. With this in mind, the features in proposed model 1, which maintain involvement of the DoH as part of governance-accountability, are a vital inclusion.

The Department is an important link between health and medical research and policy-driven equitable healthcare. Reducing the governance role of the DoH as recommended under models 2 and 3, risks reducing this link to health policymakers, which could lead to fragmented systems that prevent equitable delivery of innovative solutions and the true translatability of new knowledge. Research translation to policy-driven equitable healthcare is particularly important for rare disease, as Australians living with rare disease are inherently few. This scarcity is further compounded by Australia's geographical spread. Without national healthcare policies, access to innovative new health technologies and models of care will continue to be reliant on patient postcodes.

RVA has concerns about imparting all accountability on only two parties; namely the National Health and Medical Research Council CEO and the Minister, as proposed in models 2 and 3. RVA acknowledges that more layers of accountability, outlined in model 1, will require better coordination, but this greater accountability will increase transparency and partnerships and streamline implementation of new knowledge and processes.

In addition to maintaining links to DoH, the new governance structure should enable advice and input from every possible stakeholder group, including consumers and peak bodies, policymakers, federal, state and territory hospitals and health administrators, early, mid and late career researchers, clinicians, clinician-researchers and any other expertise required for the equitable delivery of health and medical research funds.

RVA supports the intention to streamline administration of funding and reduce the burden of grant applications for all stakeholders, as outlined in the discussion paper across all 3 proposed models. Due to very nature of rare disease, the majority of rare disease specialists are clinician-researchers. Clinician-researchers are uniquely positioned to highlight the gaps in rare disease and work on research that directly addresses these gaps. Supporting rare disease clinician-researchers will ensure that research translates into clinical care and clinical care informs research.[1] It is vital that these invaluable skills are recognised, supported and promoted in the governance and administration of Australian health and medical research funds.

3. **What elements of the existing arrangements for the MRFF and the MREA work well and should be retained? Which feature/s of the models will help ensure these elements are preserved?**

As mentioned previously, the priority driven research foundations of the MRFF have worked particularly well for rare disease, due to the inherently high levels of unmet need in this space. RVA has been pleased to see initiatives of the MRFF that recognise the value of building evidence for policy reform. This was particularly notable in the recent call for high-cost therapies enabled research, which will drive policy around health technology assessments for rapidly emerging new and novel health technologies for Australian's living with rare disease. Based on these observations, RVA recommends retaining and strengthening this current approach and focus of the MRFF and would like to see these links to building evidence for policy strengthened in the MREA.

The original principles of the MREA and the MRFF are well intentioned, but more needs to be done to increase accountability, transparency, objectivity and robustness around how the funds are delivered. RVA commends the commitment of the Health Minister and the leaders of these two funding bodies to ensuring governance that strengthens the original principles of both funds.

4. Which aspects of the current arrangements could be changed to deliver the most appropriate and effective change, and why? Which feature/s of the models will help deliver this change?

The success of the MRFF for rare disease, highlighted in the previous sections, should be leveraged to improve the response of the MREA to rare disease, which funds the majority of fundamental discovery research in Australia. Without access to this funding, the causes of most rare disease will remain elusive, and Australia will not be competitive in this space on the international stage.

RVA recommends keeping these two funding streams separate and strengthening their existing principles to more clearly delineate their different but complimentary focuses, as originally intentioned. This aligns with practices in the United Kingdom's Office for Strategic Coordination of Health Research, as outlined in the Discussion Paper.

If instead, the MRFF and the MREA are merged, it is imperative that existing effective foundations and principles are incorporated and strengthened, and robust systems are in place for more transparent reporting and accountability around the use of funds.

The roles of consumer representatives and peak body expertise should be more explicitly included and adequately resourced in the new governance advisory structure. Peak bodies can add value in an advisory role with their broad-brush knowledge base of issues affecting large groups of Australians. Peak bodies including, RVA, Research Australia, Consumers Health Forum of Australia, National Aboriginal Community Controlled Health Organisation, and the recently formed Australian Multicultural Health Collaborative under the Federation of Ethnic Communities' Council of Australia should be invited to these structures.

There should also be an ongoing commitment to improving governance and administration of health and medical research funding in response to feedback, change and new knowledge. RVA recommends keeping and aligning regular reviews of priorities and strategy for the consistent and coherent delivery of the both the MREA and the MRFF. While the priority driven nature of the MRFF requires more regular reviews, coordinating and following the MRFF's approach for 2 and 5 yearly reviews may improve alignment and delivery of the 2 funds and enable the greatest impact on health and wellbeing outcomes.

Given the lack of transparency around how much rare disease research is funded under the MREA, RVA recommends embedding and strengthening data collection and evaluation as part of any new administrative and governance framework. This will support continuous improvements in funding decisions across both funds and increase accountability for an effective, fair and equitable approach to distributing health and medical research investments.

–END–