

Australian Medical Research and Innovation Five Year Strategy

Title: Submission from MS Research Australia

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Multiple Sclerosis (MS) and MS Research Australia

MS Research Australia is the research arm of MS Australia. Its mission is to accelerate research toward the prevention, better treatments and a cure for multiple sclerosis (MS). MS Research Australia partners with relevant medical research institutes and scientists around Australia, to directly fund research as well as coordinating national and international collaborations in areas of research priority to MS. MS Research Australia also partners with other medical research funding bodies where relevant, including the NHMRC, to provide research grants.

MS is the most common acquired chronic neurological disease affecting young adults and costs the Australian health system and society over \$1 billion p.a. There are 23,000 people living with MS in Australia and this number is increasing. People are more likely to be diagnosed in early adulthood and three quarters are women. This has profound effects on an individual's ability to fulfil expected life roles at a stage when careers, relationships, and adult life in the community are consolidating, with resulting impacts on work, family, and social life.

MS is caused by an autoimmune process directed against myelin, which provides the insulating layer of nerve fibres in the brain and spinal cord. The medical research sector in MS is very strong and basic research has identified many of the genetic and environmental triggers of MS as well as a number of the disease processes involved. This past research has resulted in improvements to management and therapies for the most common form of MS, relapsing remitting MS, which translates to earlier diagnosis, less hospitalisations and slower disability accumulation compared to a decade ago.

For patients with primary progressive MS or those entering the secondary progressive phase of relapsing remitting MS, there is a very limited response to these therapies. Further research and drug development is vital to offer hope to these patients. A cure does not exist for any form of MS.

Challenges to address

The gaps in the research landscape for MS are those that have been identified in the wider health system and MS Research Australia welcomes the introduction of the MRFF to address these issues. Specifically, MRFF investment is required to increase the capacity for the translation and implementation of findings from social, allied health and clinical research and increase the efficiency of the research currently being done. Empowering the health system workforce to build their research capacity and embed research throughout the health system can be achieved by funding multi-disciplinary and cross-disciplinary networks, collaborations and partnerships that bring together academics, clinicians, allied health professionals and basic researchers. These collaborators will learn from each other and facilitate the bidirectional exchange of information on priorities and unmet needs of healthcare consumers, and the methodology, expertise and infrastructure required for the successful completion of robust evidence-generating health research. Facilitating collaborations across the academic and health system spectrum will also ensure that pathways for utilising and translating fundamental research discoveries are rapidly identified and put in train. Funding for public good clinical trials (such as low-cost health interventions and off-patent drug repurposing) with little or no commercial investment interest or potential is also an area of significant unmet need. This need can be met by providing funding directly for public good clinical trials in a partnership model with health authorities/payers, hospitals, and not-for-profit disease-specific organisations. Embedding research across the health system will also build research capabilities and facilitate the conduct of public good clinical trials and implementation of cost-effective health interventions. Providing funding that enables frontline healthcare practitioners to devote a proportion of their time to research activities will foster a culture of continuous improvement and efficiency in

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healthcare delivery and ensure that healthcare delivery remains at the forefront of best-practice and evidence-based. Another key challenge is ensuring that developments in healthcare and the delivery of healthcare meet the needs and priorities of the end-user of the research, healthcare consumers. By partnering directly with the not-for-profit organisations that represent people affected by a condition, the MRFF will ensure that they are utilising an already established network of stakeholders with a thorough understanding of the challenges, unmet needs and priorities of the population.

Solutions and investment pathway

There are several ways by which translation and efficiency of research could be improved. Funding of **multi-disciplinary, multi-disease networks** (such as for autoimmune disease and neurodegenerative diseases) would increase translational opportunities and research outcome exposure. Research into autoimmune diseases, such as MS and type 1 diabetes, has identified common symptoms and common biological pathways for autoimmune disorders and translation of interventions that target these areas deserve funding. In particular, this initiative would reduce barriers to collaboration (challenge 4), and support research and innovation from concept to delivery (challenge 5). Leading the way in this type of initiative, MS Research Australia is currently partnering with JDRF Australia, the funding body for research into type 1 diabetes, with generous support from the Macquarie Group Foundation, to provide a fellowship for a researcher to investigate commonalities between the two diseases and identify further strategic areas of research need for future funding. Implementation of research for wider goals for autoimmune and neurodegenerative diseases is eminently achievable by MRFF investment in collaboration between relevant research organisations and disease networks.

Funding is also needed for **“public good clinical trials”** which aim to demonstrate the safety and efficacy of ‘off-label’ or off patent medications or interventions for potentially high impact therapies. This type of research is impossible in the current climate of pharmaceutical industry-led clinical trial funding and would need a collaborative approach where clinical trials are funded through a partnership model. In this model the usual suppliers of a treatment being tested (e.g. PBS, hospitals) continue to fund that treatment for a new group of patients and the remainder of the clinical trial costs are covered by a philanthropic, not-for-profit or industry partner. Funding for “public good clinical trials” would facilitate translation of research into health outcomes (challenge 1), embed research universally across the health system (challenge 2), maximise productivity within the health and research system (challenge 3). In this model, investment from the MRFF could be deployed to cover the costs that would normally be incurred by the health system (i.e. the suppliers above) and partnerships could be sought through an application process for specific initiatives of treatments and diseases (such as the use of vitamin D as a preventative or treatment for MS, or comparative trials of ‘off-label’ procedures or treatments against best available approved pharmaceutical therapies).

Funding to **bridge the gap between proof-of-concept research and full commercial development** is also a significant priority. Support for pre-clinical work-up of promising compounds and devices would greatly increase translational research activities and the up-take of potential new therapies by industry partners. This can again be done by supporting collaborations in which basic researchers, clinicians, and individuals with expertise in regulatory affairs and clinical trials are brought together. Early involvement of industry, regulatory, clinical and clinical trials expertise at proof-of-concept and pre-clinical stages will ensure that product development is targeted, practical and trials are optimally designed to maximise the potential for a successful outcome that will meet the requirements of health technology assessment bodies. These collaborations would also benefit from a partnership funding model in which MRFF, not-for-profit patient-focussed organisations, philanthropists and industry can contribute. Funding research with commercialisation potential via this mechanism will address the mandatory consideration to ensure that financial assistance from the MRFF provides the greatest value for all Australians via the efficient and targeted use of funding. It will also maximise productivity within the health and research system (challenge 3), reduce barriers to collaboration (challenge 4) and support research and innovation from concept to delivery (challenge 5). Organisations such as the International Progressive MS Alliance, of which MS Research Australia is a managing member, have adopted this model in the development of

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their strategy and in the funding of major collaborative networks to target translational research for progressive forms of MS. Involvement of an 'Industry forum' at all levels of the Alliance's governance, grant review and collaborative structures has provided invaluable early insight and guidance on the way forward to accelerate solutions for progressive forms of MS that will have the best chance of success once they reach the health technology assessment stages.

A funding gap exists for direct funding of **national collaborations between researchers and clinicians with a common interest**. Collaborative funding ensures outcomes that could not be achieved by researchers acting in isolation. These include focusing attention on patient-centred priorities, furthering translation and implementation of findings, and pooling of expert knowledge, resources and infrastructure. It will also help to avoid research duplication and maximise the mutual use of expensive research infrastructure that may be available in either the academic sector or the healthcare sector. MS Research Australia partners with scientists and clinicians around Australia, to coordinate national and international collaborations – 'virtual research centres' in areas of research priority to MS. Investment by the MRFF in collaborative efforts such as these, would reduce barriers to collaboration (challenge 4), and support research and innovation from concept to delivery (challenge 5). Investment in the coordinating body, or partnership with the coordinating body, rather than the members of the networks themselves, is a gap in the current NHMRC funding model and encourages participation and judicious use of funds. Not-for-profit disease-specific organisations are already well positioned to bring together multiple stakeholders, identify patient-centred priorities, and assemble expert collaborators around a common goal. They are also adept at collaborating with other not-for-profits within and across disease areas.

The MRFF also needs to focus on the need to embed research into the health system through encouraging clinician involvement in research. Involving clinicians at the concept level of research discovery will help to guide the development and rapid translation of research findings into practical and needed interventions. As also identified as a priority area in the McKeon review, further funding needs to be made available for **clinical research fellowships and pairs of clinician-researchers working on a common research project**. MS Research Australia is in the planning stages of a scheme which will provide funding for a researcher and clinician, to allow the pair to collaborate on research and implement findings at a clinical level. This scheme will be funded through an application process which is peer reviewed in a way which is similar to the NHMRC. The MS Research Australia Research Management Council perform this function for MS Research Australia grants. These 'paired fellowships' would allow the MRFF to 'purchase' research time of clinical professionals and also ensure that the research project was completed to high level research standards with experts in specific methodologies or analysis techniques. The paired fellowship scheme would facilitate translation of research into health outcomes (challenge 1), embed research universally across the health system (challenge 2), maximise productivity within the health and research system (challenge 3), would reduce barriers to collaboration (challenge 4), and support research and innovation from concept to delivery (challenge 5).

Overall, MS Research Australia believes that increasing translation and implementation in the ways listed above would facilitate translation of research into health outcomes (challenge 1), embed research universally across the health system (challenge 2), maximise productivity within the health and research system (challenge 3), would reduce barriers to collaboration (challenge 4), and support research and innovation from concept to delivery (challenge 5).