Australian Medical Research and Innovation Two Year Priorities

Title: Submission from MS Research Australia

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1. What is the gap in Australia's health system to be addressed by this priority?

While people with relapsing remitting MS now have access to a range of disease modifying treatments, these do not work for everyone and in many cases have intolerable side-effects and risks, there is no way to prevent MS, there are no treatments for people with progressive forms of MS and there is no cure.

2. How does your area of priority address either an existing or a new health or health system challenge?

Funding for multi-disciplinary, *collaborative research networks and platforms that bring together basic researchers and clinical staff* (including allied health and nursing) in areas of research that focus on the identified priorities for the MS community will have the combined effect of *enhancing the MS research workforce*, *accelerating the translation of basic and clinical research discoveries into health outcomes for people with MS* and *continuously improve the delivery of best-practice, cost effective healthcare* interventions for people with MS, whilst working towards the prevention and cure of MS.

3. Comment on which aims and objectives your priority is likely to meet.

Funding for further research into MS as outlined below, that is conducted in multi-disciplinary collaborative platforms and partnerships and is led by the priorities identified by the collective MS community (MS organisations, people affected by MS, researchers and clinicians) will ensure that there is a *clear translational pathway that* targets the areas of greatest unmet need for people with MS and *maximises the opportunities for success*. Funding research via partnerships between healthcare providers and researchers and funding research such as public good clinical trials of interventions/medications that are not of interest to commercial funders will ensure that *cost-effective healthcare strategies* are pursued and delivered to people with MS. This approach will align researchers and clinicians and ensure that research advancements are rapidly translated into *evidence-based healthcare policies and interventions.* It will also facilitate a *collaborative, integrated, research-engaged health workforce and research community*. The end result of this approach to MS research will be the *delivery of the preventions and cures of tomorrow*, which in turn will have *economic benefits* because people with MS will have access to the treatments and interventions that optimally control disease and manage symptoms so that their long-term healthcare needs are minimised and their economic productivity is maximised.

4. Mandatory considerations – which of the mandatory considerations set out in the *Medical Research Future Fund Act (2015)* does your priority proposal address?

Tick as appropriate, no further information required.

☒ Burden of disease on the Australian Community
☒ How to deliver practical benefits from medical research and medical innovation to as many Australians as possible
☒ How to ensure that financial assistance provides that greatest value for all Australians
☒ How to ensure that disbursements complement and enhance other assistance provided to the sector

5. Outline of priority proposal:

Multiple sclerosis (MS) is caused by an autoimmune process directed against myelin, which provides an insulating layer around nerve fibres in the brain and spinal cord. The MS research sector 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. However, not all patients can tolerate or respond to the current therapies.

For patients with primary progressive MS or those entering the secondary progressive phase of relapsing remitting MS, there are currently no therapies that can halt the accumulation of disability. Further research is vital to offer hope to these patients. A cure does not exist for any form of MS.

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 directly funds investigator-led research applications, as well as coordinating national and international collaborations – 'virtual research centres' - in areas of research priority to facilitate the outcomes that could not be achieved by researchers acting in isolation. MS Research Australia also partners with other medical research funding bodies, including the NHMRC and other not-for-profit bodies.

Burden of disease on the Australian Community

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. While not as common as other diseases, the impact of MS per capita is very high, with the average utility score for people with MS as low as that for an elderly person (see http://www.msbrainhealth.org/report) and for those with more advanced disease the reduction in quality of life is equivalent to those affected by stroke and end stage cancer (Economic Impact of MS in Australia in 2010). 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, families and adult life in the community are consolidating. MS is a lifelong disease.

How to deliver practical benefits from medical research and medical innovation to as many Australians as possible

While MS has a considerable disease burden as mentioned above, MS is one of a family of autoimmune diseases in which the immune system attacks the self, other examples include type 1 diabetes and systemic lupus erythematosus. The prevalence of all autoimmune diseases is increasing worldwide. Research into these diseases has highlighted common symptoms and common biological pathways for autoimmune diseases. Progressive MS also shares many features with other neurodegenerative diseases, and similar challenges face researchers and clinicians in managing these diseases and conducting clinical trials. Funding for multi-disciplinary, multi-disease networks for interventions for identified common pathways would ensure the maximum number of people benefit from this research and mitigate any perceived risk of funding isolated disease groups. This is eminently achievable by collaboration between relevant research organisations and disease networks. Leading the way in this type of initiative, MS Research Australia is currently partnering with JDRF Australia to provide a fellowship for a researcher to investigate commonalities between MS and type 1 diabetes and identify further strategic areas of research need for future funding. Forging other similar cross-disease collaborations will lead to shared approaches to common challenges and accelerate solutions for people living with a wide range of autoimmune and neurodegenerative conditions.

How to ensure that financial assistance provides that greatest value for all Australians

Value for the MRFF will be derived through funding MS research with clear and rapid translational timelines. Past research into MS has decreased time to diagnosis, improved management, reduced hospitalisation and slowed disability outcomes for the MS patient population, which has reduced costs across the health system. Integrating research into the health system and funding research that focuses on clear translational priorities will provide the most benefit in the first two years of the MRFF. MS Research Australia has a well-respected research review process that closely mirrors that of the NHMRC, and has a strong track record in providing funding for the strongest research that will improve outcomes for people with MS in Australia. MS Research Australia is also well positioned through close interaction with our stakeholder groups (see below) to understand the key priorities and unmet needs in MS research. Therefore, by partnering with MS Research Australia to fund multi-disciplinary, collaborative research platforms and networks, the MRFF will ensure that the financial investment in MS research is well targeted and provides the greatest value for Australians.

How to ensure that disbursements complement and enhance other assistance provided to the sector
Similarly to the NHMRC, MS Research Australia provide direct research funding for research projects, and capacity-building salary support. We also provide incubator grants to fund pilot studies and travel awards for researchers to collaborate overseas for short periods. Funding from the MRFF for collaborative endeavours such as the national and international, multi-disciplinary, collaborative platforms for priority areas in MS research, would complement these investigator-led grants. Funding of public good clinical trials of potentially high impact therapies including ‘off-label’ or off patent medications is not possible through current mechanisms, and would benefit from a partnership approach in which ‘treatment costs’ are borne via the health system (hospitals/PBS) and philanthropic/ NFP and MRFF funding can provide the additional costs associated with a clinical trial. Funding for cross-disease researcher networks, is also a current gap and will maximise the use of the knowledge and outcomes for broader patient groups and maximise value for money in funding research. Another way to ensure rapid translation is to include clinicians at the earlier, basic research levels of a project or intervention. MS Research Australia is in the planning stages of a scheme which will provide paired funding for a researcher and clinician, to allow the pair to collaborate on research and implement findings at a clinical level. MRFF support of such initiatives would aid rapid translation and uptake of research findings.

6. What measures of success do you propose and what will be the impact on health care consumers?

- For National and International MS Collaborative Platforms – meetings per year, collaborative outcomes per year, research outcomes against identified milestones/barriers, new publications, development of policies /recommendations and clinical implementation.
- Autoimmune and neurodegenerative disease cross-disciplinary networks and common research areas – as above for MS collaborative platforms.
- Public good clinical trials for potentially high impact therapies – clinical trial design milestones, patient recruitment milestones, clinical trial end points, research outcomes (new publications, policies and/or recommendations), and implementation milestones.
- Initiatives which increase translation of clinical research into MS (such as the clinician and researcher partnership or clinical fellowships) – numbers of applications and award of funding in this category, research outcomes, production of new publications, policies and/or recommendations, measurement of uptake of research outcomes in the healthcare setting.

7. Please outline any linkages your proposal has with stakeholders, policy agendas and other health and medical research funding agencies.

As a key partner working with the MRFF, MS Research Australia would bring the advantage of a well-established stakeholder network and associated priority-setting and governance processes. MS Research Australia interacts with *healthcare practitioners* and *researchers,* universities, medical research institutes and hospitals to fund and coordinate research around Australia. MS key opinion leaders, epidemiologists and public health experts are part of our advisory boards and steering committees, and Governance Boards. We partner with the *NHMRC* to co-fund MS researchers. We approach *trusts and foundations and philanthropic organisations* and work in partnership with them to fund research. We receive roundtable funding from the *pharmaceutical industry* and interact with industry to facilitate clinical trials access for healthcare practitioners and patients. We have funding partnerships with the NSW Office of Health and Medical Research for a number of key platforms. MS Research Australia works closely with our sister organisations, the state-based MS societies and MS Australia, who provide care, services and advocacy for people with MS, and we interact directly with *consumers* in the MS community through a range of activities, communications and enquiries. We are currently undertaking a major survey of the MS community on the priorities for MS research.