



15
year
anniversary

Multiple Sclerosis Research Australia

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Mission: *To use stem cells to develop innovative, safe and effective treatments accessible to all Australians who need them.*

Is the above Mission statement appropriate for the vision and goals of the Mission?

Yes, it is appropriate. As Australia's largest not-for-profit organisation dedicated to funding, coordinating and advocating for multiple sclerosis (MS) research, MS Research Australia is proud to advocate the use of stem cells to develop safe and effective treatments accessible to all Australians with MS. Our ultimate goal is to accelerate Australian MS research toward the prevention, better treatment and a cure for MS. There is ongoing research into the use of stem cells to develop treatment options and also as a therapeutic for people with MS. Further research is required in both areas and in particular, for stem cells to become a standard form of therapy in Australian clinics and hospitals for people with MS, further rigorous evidence for the effectiveness, safety and most appropriate use of stem cells is still required.

There are 25,600 people living with MS, with the average age of diagnosis being 20-40 years old. The most common form of MS is relapsing remitting MS, which is characterised by flare-ups followed by periods of recovery or remission, and affects 85% of people with MS. Over time, people with this form of MS may transition to the next phase known as secondary progressive MS. This is characterised by progressive worsening of disability over time as the nerves become damaged, with no obvious signs of remission. The remaining 10-15% of people with MS have primary progressive MS, which is characterised by worsening of symptoms and disability over time from the beginning, without any periods of recovery. It is important to prevent disease progression and to improve the quality of life of people with MS, and it is hoped that the development of innovative, safe and effective stem cell treatments and the use of stem cells to develop treatments options for people with MS will allow this by reducing progression and promoting repair.

Can the Mission statement be strengthened?

The Mission statement is appropriate.

Vision: *An Australia with a world-leading translational stem cell sector developing and delivering innovative, safe and effective stem cell treatments that improve health outcomes in partnership with patients and carers.*

Is the above Vision statement appropriate for the investment being made towards the Mission?

Yes, it is appropriate. There is a vast array of ongoing research into therapeutic use of stem cells in MS and modelling MS using stem cells. Researchers have been focusing on myelin repair to reverse the damage wrought in MS, and neuroprotection to protect nerve cells from damage.



OUTSTANDING
ACHIEVEMENT
WINNER 2018





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In MS Research Australia-supported research, Associate Professor Kaylene Young has investigated ways of activating neural stem cells to repair myelin, the insulating sheath surrounding the nerves in the brain and spinal cord, which is damaged in MS. She is also generating stem cells directly from blood cells of people with MS in order to produce myelin-producing cells to model MS. The ultimate goal of this study is to identify the molecules that interact with the MS risk genes to see if any existing drugs can be found that target them. Therapies stemming from this work could protect against the damage caused by MS.

MS Research Australia has previously funded Dr Natalie Payne, who performed extensive molecular profiling of induced pluripotent stem cells from people with and without MS to understand more about the genes that are expressed at each stage of development. This has provided clues into whether MS risk can be identified much earlier in development, and helped differentiate between the roles of genes and the environment in causing MS symptoms. Both Professor Bruce Brew and Dr Michael Lovelace were each individually funded by MS Research Australia to investigate a metabolic pathway that may modulate myelin repair via stem cells.

Can the Vision be strengthened?

The Vision is appropriate.

Does the proposed scope of the Roadmap address the scope of stem cells therapies that should be addressed in the context of the Mission?

Yes, the scope is appropriate. Research into using stem cells as a therapeutic for people with MS is still very much in the early stages and further research is required to understand the safety and efficacy of these therapies. Stem cells are also being used to model MS, with the aim of developing treatments to protect against the damage caused by MS.

Autologous haematopoietic stem cell therapy (AHSCT) is primarily an immune suppressing chemotherapy treatment. It is a one-off procedure involving a number of steps over the course of several weeks. Blood stem cells are collected from a person's bone marrow or blood, chemotherapy is then used to eliminate or partially eliminate their immune system. The individual's own blood stem cells are reintroduced back into their body via infusion to help restore and regenerate a "new" immune system. Research suggests that the 'reset' immune system is less likely to attack the brain and spinal cord.

Mesenchymal stem cell (MSC) therapy is a different treatment to AHSCT, which does not involve chemotherapy and uses a different type of stem cell which can be isolated from different tissues, including bone marrow and fat. In laboratory studies, mesenchymal stem cells secrete growth factors and other factors that can influence the immune system. The evidence to date from laboratory studies and very early stage human trials suggests that the therapeutic potential of MSCs may come from the chemicals they secrete that appear to calm the immune system and contribute to an environment that is more supportive for self-repair of the central nervous system.





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There has been ongoing research into directing neural stem cells to repair myelin, with the ultimate goal of harnessing the regenerative capacity of these cells for the treatment of MS. Furthermore, researchers are using stem cells from people with MS to generate myelin-producing cells. The aim of this study is to identify the molecules that interact with the MS risk genes to see if any existing drugs can be found that target them.

Are the key funding principles appropriately articulated?

Yes, the key funding principles are appropriately articulated.

Should any funding principles be further strengthened in other areas of the document?

MS Research Australia believes that both national and international collaborations should be facilitated, and funding specifically for collaborative activities in MS is highly desirable. Multidisciplinary collaboration will accelerate translation of research outcomes into clinical practice.

Are there other principles that should be followed?

MS Research Australia believes that funding should be directed towards building strong regulatory and ethical frameworks for effective stem cell treatments, which is a new and complex area. Many stem cell therapies are being offered at an increasing number of private clinics without clinical trial evidence of safety and efficacy and therefore strong regulatory and ethical frameworks are of high importance. Developing these frameworks should involve examining the attitudes of major stakeholders – including people who are being treated, consumer and advocacy groups, scientists, clinicians and regulators – regarding access to and regulation of innovative stem cell therapies, as well as reviewing the regulatory provisions used to oversee the use of stem cell therapies and assessing how regulatory exemptions are being used in Australia compared with international jurisdictions.

Do the six funding priorities capture the key areas of focus for the sector?

Yes, they do. MS Research Australia strongly believes that both national and international collaborations should be facilitated, and funding specifically for collaborative activities in MS is highly desirable. Multidisciplinary collaboration will accelerate translation of research outcomes into clinical practice.

The ethics, engagement and policy priority is very important as it is necessary to build strong regulatory and ethical frameworks in this area to ensure safe and effective treatment. Many stem cell therapies are being offered at an increasing number of private clinics without clinical trial evidence of safety and efficacy and therefore it is necessary to address this. In addition, as part of this priority, it is important to educate those who are being treated. Therefore, funding towards patient information portals would be highly beneficial, especially since many of these therapies involve some risks.





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Are there any specific areas of actions that can be suggested under each priority?

MS Research Australia agrees with the actions suggested under each priority, which will ultimately benefit people with MS and MS researchers. There are particular actions that MS Research Australia believes are of high importance.

Pilot projects and disease-focused team programs as part of the targeted research funding priority, will promote multidisciplinary collaborations and fast-track translation of MS research to the clinic, which will ultimately improve the quality of life for people with MS. In addition, research on ethical, societal and regulatory issues relating to stem cell treatments is important. This should involve examining the attitudes of major stakeholders – including people who are being treated, consumer and advocacy groups, scientists, clinicians and regulators – regarding access to and regulation of innovative stem cell therapies, as well as reviewing the regulatory provisions used to oversee the use of stem cell therapies and assessing how regulatory exemptions are being used in Australia compared with international jurisdictions.

Early phase clinical trials as part of the clinical translation priority are of high importance to assess the safety of stem cell therapies, and to also move these therapies into larger clinical trials and ultimately into the clinic. MS is a varied disease, and no two people exhibit the same symptoms. Therefore, it is important that there are a wide variety of treatment options available for people with MS. Improved regulatory frameworks for stem cell treatments are required in order to provide adequate protection for people with MS without affecting the clinical freedom of clinicians too much who wish to innovate with stem cells and people who want to be part of the innovation. In addition to the actions under this priority, it would be worthwhile to identify manufacturers and practitioners that are offering stem cell therapy in Australia to determine what clinical indications are being targeted and assess the claims that are being made about the safety and clinical efficacy, with reference to current best evidence.

People with MS and advocacy groups need to be adequately educated on stem cell therapies, and therefore funding towards patient and advocate information portals under the ethics, engagement and policy priority are important. AHSCT is not widely available in Australia for people with MS, and as a result of this, many Australians are travelling overseas to receive treatment in countries where there is very little regulation. Furthermore, people who are being treated need to be educated on what these treatment options entail so they can make informed decisions.

MS Research Australia also believes that capacity building is of high importance to support careers in stem cell research in MS. Capacity building is an investment in the future of MS research by creating opportunities for early career researchers to gain scientific training, to learn from leading MS experts and nurture a commitment to cure MS.

