

# Stem Cell Therapies Mission – Roadmap

The Stem Cell Therapies Mission will provide \$150 million under the Medical Research Future Fund (MRFF) to support stem cell research to deliver innovative, safe and effective treatments.

Research advances over the last ten years have established the potential of stem cells to develop novel treatments. Stem cells may restore function to damaged tissues, be used to engineer replacement tissues and organs, or boost the body's ability to heal itself. Human stem cells can also be used in the lab to better understand what happens to the body during disease, allowing us to develop and test new drugs without any risk to patients. These stem cell applications have the capacity to provide innovative treatments for many chronic and inherited diseases with major unmet clinical needs, while also having the potential to revolutionise drug development practices.

Australia has been, and continues to be, a global leader in stem cell science, with over 300 research laboratories and more than 30 companies focussed on regenerative medicine and cell therapy. Nationally and internationally, the pace of development in this sector is accelerating, with more stem cell therapies now moving through clinical development.<sup>1</sup> Investor and industry interest is also rising, with cell therapy development attracting US\$7.6 billion in financing in 2018.<sup>2</sup>

## Our Mission

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

## Our 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.

## Scope of the Mission

### Stem Cell Types

Funding provided within the Mission will encompass research on:

- Tissue stem cells
- Pluripotent stem cells
- Tumour stem cells

### Applications of these stem cell types

- The use of stem cells for novel *cellular therapies* and *tissue engineering*, including the application of gene-modified stem cells.
- The use of stem cells for *disease modelling*, including as models to identify underlying disease mechanisms, screen for new treatments or enhance preclinical studies.

## Funding Principles

- Research should target the development of innovative, safe and effective treatments
- Mission investment should focus on research quality and opportunity for patient impact
- Adherence to best practice by health professionals and researchers is essential
- Long term outcomes should improve clinical delivery and commercial development of innovative, safe and effective treatments
- Multidisciplinary collaboration, including international collaboration, should be facilitated

## Priority, timing and phasing investment

Funding will be made available across the 10 years of the Mission. The relative priority for funding and phasing of schemes within this Mission will vary. Some activities will also seek funding from other investment areas within the MRFF and/or external funding partners.

<sup>1</sup> 268 cell therapy clinical trials were underway around the world at the end of Q1 2019 – 31 in phase III.

<sup>2</sup> Regenerative Medicine Opportunities for Australia MTPConnect, October 2018

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## Funding priorities

### Targeted research funding

Research funding may focus on collaborative multi-disciplinary teams to deliver targeted health translation research. To address the need for collaboration and treatment focus, schemes may incorporate existing international approaches for building disease-related teams with a treatment outcome. Such teams are recommended to have patient advocates to address the disease complexity. It would be viewed positively if projects could leverage external sources of funding. Funding may be staged to support:

- Pilot projects – designed to nucleate teams to show proof of concept for a potential treatment
- Disease-focussed team programs – larger scale milestone-based team funding focussed on developing treatments and moving a potential treatment from preclinical to clinical trials
- Research on ethical, societal and regulatory issues relating to stem cell treatments

### Clinical translation

To ensure the delivery of safe and effective stem cell treatments, the Mission may focus on early phase clinical trials, clinical trials registers, including improved engagement of patients and community in developing novel treatments. Potential support for clinical translation could include:

- Early phase clinical trials
- Stem cell clinical trial registers
- Clinical grade stem cell lines
- Stem cell ethics advisory committees
- Improved regulatory frameworks for stem cell treatments and international harmonisation
- Education for patients regarding engagement with clinical trials

### Ethics, engagement and policy

A key objective of the Mission is to engage with the community around their understanding and expectations of stem cell medicine, and their views on novel technologies. The Mission must also work to ensure safe and effective treatments, which will require strong regulatory and ethical frameworks. To deliver these outcomes, support is needed for:

- Patient and advocate information portals
- Research and clinical guidelines for the stem cell sector
- Clarification on reimbursement pathways for different applications of stem cell medicine
- The economics of the development and delivery of stem cell therapies

### Capacity building and workforce

This sector needs to develop strong capacity building and training. In particular, support is needed for upskilling in clinical delivery, regulatory frameworks, commercialisation, and cellular manufacturing. Such capacity building may be funded through the MRFF, NHMRC and/or Industry or Education funding if applicable. Potential capacity building activities to support careers in stem cell research include:

- Industry and clinical fellowships
- Mentorship programs within health institutions or industry who have had success in translation
- Cell manufacturing scholarships
- Education on regulation and development of treatments
- Funding for a national stem cell medicine network

### Commercialisation

Opportunities exist within the MRFF, including within this Mission, to increase collaboration across the value chain thereby accelerating the development of world-leading treatments for export. This in turn will generate jobs. Commercialisation activities may be funded via the Mission, other programs under the MRFF, or other Government initiatives, including the Biomedical Translation Fund. Leveraged support may also be sought from private capital, including philanthropy and the commercial sector. Potential support for these activities could include:

- The MRFF Biomedical Translation Bridge and BioMedTech Horizons programs
- The Biomedical Translation Fund
- Incubators

### Infrastructure

The 10 year MRFF Investment Plan includes support for 'soft' infrastructure, such as data portals. Some aspects of this Mission may be addressed using this MRFF mechanism. Building Australian manufacturing capability will need to be addressed in stages using funds from outside the Mission such as through the National Collaborative Research Infrastructure Strategy and/or State Government and commercial sector funds.