



# Stem Cell Therapies Mission

## Roadmap



Theme  
Research  
missions

The Stem Cell Therapies Mission will provide \$150 million over 9 years under the Medical Research Future Fund (MRFF) to support stem cell research to deliver innovative, safe and effective treatments.

Research advances over the past 10 years have shown the potential to develop new treatments using stem cells. 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 be used in the laboratory to better understand what happens to the body during disease, allowing us to develop and test new drugs without any risk to patients. These applications could provide innovative treatments for many chronic and inherited diseases that have major unmet clinical needs, and may also revolutionise drug development practices.



### Scope

The Stem Cell Therapies Mission invests in research on:

- **tissue stem cells** — stem cells or progenitor cells in human tissues that play a role in tissue repair and homeostasis
- **pluripotent stem cells** — human embryonic and induced pluripotent stem cells, which can differentiate into any specialised cell type of the human body and play a key role in regenerative medicine
- **tumour stem cells** — cell types within a cancer that can initiate a tumour, or drive tumour growth or regression after treatment

The Stem Cell Therapies Mission includes applications of these stem cells and their derivatives for:

- development of novel cellular therapies and tissue engineering, including using gene-modified stem cells
- disease modelling to identify underlying disease mechanisms, screen for new treatments or enhance preclinical studies



### Our goal

To support world-leading translational stem cell research that develops and delivers innovative, safe and effective stem cell medicines to improve health outcomes, in partnership with patients and carers.



### Our mission

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



## Funding principles

Activities funded under this mission should:

- target the development of innovative, safe and effective treatments
- support disease modelling to screen for new treatments or enhance preclinical studies
- focus on research quality (as determined by an independent peer-review panel) and likely impact on patient outcomes
- ensure that health professionals and researchers adhere to best practice
- generate long-term outcomes that improve clinical delivery and commercial development of innovative, safe and effective stem cell-based treatments
- facilitate multidisciplinary collaboration, including international collaboration and engagement with industry
- ensure meaningful engagement with patients, their carers and consumer groups at all stages of the research, wherever possible

## Priority areas for investment

Funding will focus on:

- accelerating the development of safe, effective and affordable stem cell-based therapies
- generating new treatments using human tissues made from stem cells (disease modelling for drug development)
- building the health system and commercial sector to deliver these transformative treatments for the community

### Clinical translation

To ensure the delivery of safe and effective stem cell treatments, the mission may support:

- 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 about engaging with clinical trials
- improved engagement of patients and the community in developing novel treatments

### Targeted research funding

Funding may focus on collaborative multidisciplinary 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. These teams should include patient advocates to address disease complexity. The ability to use external sources of funding would be an advantage. Funding may be staged to support:

- pilot projects — designed to support and nurture teams to show proof of concept for a potential treatment
- disease-focused team programs — larger-scale milestone-based team funding, focused on developing treatments and moving from preclinical to clinical trials
- research on ethical, societal and regulatory issues relating to stem cell treatments

### **Capacity building and workforce**

To improve capacity and training, the mission may support upskilling in clinical delivery, regulatory frameworks, commercialisation and cellular manufacturing. Capacity-building activities to support careers in stem cell research may include:

- industry and clinical fellowships
- mentorship programs within health institutions or with industry that have been successful in translation
- scholarships for cell manufacturing

- education on regulation and development of treatments
- facilitation of cross-network activities

Capacity building may be funded through the MRFF using National Health and Medical Research Council selection processes, or through the Australian Government Department of Industry, Science, Energy and Resources or the Australian Government Department of Education, Skills and Employment, if applicable.

## **Enabling priorities**

### **Commercialisation**

The MRFF has opportunities outside this mission to increase collaboration across the value chain. This will accelerate the development of world-leading treatments for export and generate jobs. Commercialisation activities may be funded through the MRFF or other government initiatives, including the Biomedical Translation Fund. Support may also come from private capital, including philanthropy and the commercial sector.

### **Infrastructure**

The MRFF has opportunities outside this mission to support 'soft' infrastructure, such as data portals. Building Australian manufacturing capability will be addressed in stages using funds from the National Collaborative Research Infrastructure Strategy, state and territory governments, and the commercial sector.

### **Ethics, engagement and policy**

This mission includes opportunities to embed community engagement in funded research projects, and support research on the ethical, legal and social implications of stem cell-based therapies. Additional support from outside the MRFF will be needed to develop and implement strong regulatory and ethical frameworks, and improve community understanding of stem cell science and its clinical translation.