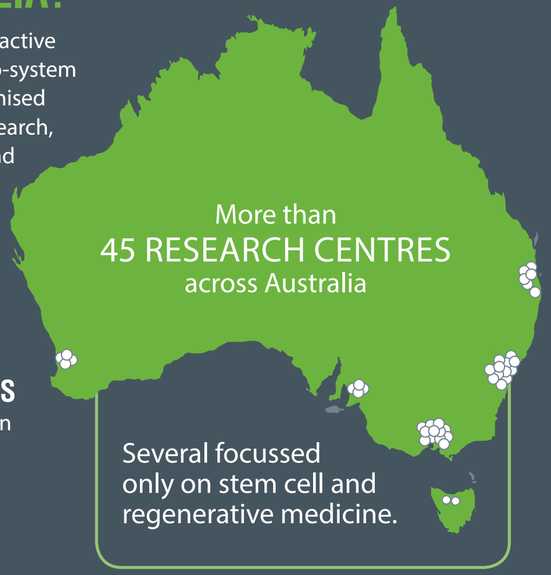
CELLULAR THERAPIES AND REGENERATIVE MEDICINE IN AUSTRALIA

WHY AUSTRALIA?

Australia has a strong and active regenerative medicine eco-system with internationally recognised basic and translational research, clinical trials framework and clinical centres.



OVER 30 CELLULAR THERAPY OR REGENERATIVE MEDICINE COMPANIES with a market capitalisation of over **$3 BILLION**

MORE THAN 30 ONGOING CLINICAL TRIALS in the research area.

AusBiotech
AUSTRALIA'S BIOTECHNOLOGY ORGANISATION

ausbiotech.org

AUSTRALIAN CLINICAL TRIALS ECO-SYSTEM ALLOWS FOR RAPID RECRUITMENT OF PATIENTS

STREAMLINED CLINICAL DEVELOPMENT PATHWAY

The regulatory pathways in Australia allow for immediate leverage of Investigational New Drug (IND)/Biologics License Application (BLA) to accelerate a global roll-out.

- This is supported by the presence of a Biologics framework (2011)
- The Therapeutic Good Administration (TGA) administers pathways such as the Rapid (14 days) Clinical Trial Notification (CTN) and Clinical Trial Exemption (CTX) schemes providing two avenues through which 'unapproved therapeutic goods' may be lawfully supplied for solely experimental purposes in humans.
- The application for CTN or CTX depends on the sponsor as well as the outcome of reviews by the Human Research Ethics Committee (HREC).
- The CTN process allows for products with US IND or EU Common Technical Document (CTD) approval to bypass further regulatory assessment in Australia.

No local study requirement for BLA and Good Manufacturing Practice (GMP) manufacturing licenses, which include collection.

- BLA or Advanced Therapy Medicinal Product (ATMP) data is usually sufficient for TGA filing, allowing for potential parallel filings as well as circumventing the requirement for local patients.
- There is also mutual recognition of GMP with the EU.

If patient recruitment is required, Australia has historically demonstrated rapid recruitment in cellular therapies.

- Low risk, single-language and stable test market for global deployment modelling.
- Large enough market locally to test deployment at scale yet small enough to enable real time fine-tuning without compromising patient safety or therapy.
- Bolstered by established, experienced clinical centres with needle-to-needle supply chain support.

AUSTRALIAN REGENERATIVE MEDICINE MILESTONES

1986

1987
2nd ever peripheral blood apheresis and stem cell transplant

1992
Demonstration of value of G-CSF in stem cell mobilisation

1993
First unrelated cord blood transplant

1996

1999
First human embryonic stem cell line created involving Australians

2002
Introduction of Commonwealth legislation to enable use of human embryos in research; establishment of New South Wales (NSW) Stem Cell Network

2003
Establishment of the Australian Stem Cell Centre

2004
First Australian human embryonic cell line

2006
Peter MacCallum Cancer Centre commence CAR-T clinical trials

2006

2009
Australian scientists generate Australia's first human induced pluripotent stem cell (iPSC) lines (following 2007 discovery from Japan); establishment of the Australian Regenerative Medicine Institute (ARMI)

2011
Establishment of Stem Cells Australia

2014
World's first clinical trial of an allogeneic iPSC-derived cell therapy product undertaken by an Australian company; world-first surgery implanting 3-D printed shinbone in Australia

2016

2016
First iPSC clinical trials undertaken in Australia; first commercial CAR-T trial patient treated outside North America

2017
CCRM Australia begins operations and launches 5-year strategic plan

2018
MTPConnect releases 'Regenerative Medicine: Opportunities for Australia' report in collaboration with AusBiotech and other stakeholders.

2018
CAR-T Cell Therapy (Kymriah) approved for use by the TGA

2019
Federal government announces $150 million into 10-year Australian Stem Cell Therapies Mission through the Medicine Research Future Fund

2019
TGA issues Australia's first T-cell therapy manufacturing license to Cell Therapies Pty Ltd

BENEFITS OF PARTNERING WITH AUSTRALIA

STRONG BASIC AND TRANSLATIONAL RESEARCH ECO-SYSTEM

Universities and institutions-based researchers are funded by the Australian Government agencies such as the National Health and Medical Research Council (NHMRC) and Australian Research Council (ARC), and by the MRFF.

Ethical oversight of medical research involving human subjects and materials is overseen by more than 200 Human Research Ethics Committee (HRECs) with expected standards set-out in the National Statement on Ethical Conduct in Human Research issued by NHMRC.

THRIVING REGENERATIVE MEDICINE INDUSTRY SECTOR

The Australian regenerative medicine sector spans the whole field from tissue engineering through to stem cell therapy to gene and immuno-therapies.

- Cynata Therapeutics and Fujifilm (Japan) have announced a license agreement (valued at $43 million overall) by which Fujifilm has acquired the right to commercialise and market Cynata's lead candidate, CYP-001, for the treatment of graft-versus-host disease (GvHD).
- Perth-based regenerative medicine company, Orthocell has announced successful trial results from the use of their CelGro membrane, used as a scaffold for the regeneration of tendons in shoulder rotator cuff injuries. Following successful approval in Europe (CE mark), Orthocell is seeking approval for CelGro from the TGA and the FDA.
- The Murdoch Children's Research Institute announced a collaboration with Organovo, a US based company pioneering the development of 3D bioprinted tissues. Using the MCRI's proprietary approach for modelling human kidney tissue from stem cells, they hope to automate the fabrication of kidney organoids.
- Researchers at St Vincent's Hospital have developed the 'BioPen', a hand-held device capable of depositing a patient's stem cells, enhanced with protective and growth factors, at the site of injury to enhance the regeneration of tissue. In the first application, it is being designed to treat osteoarthritic joints that have widespread cartilage loss.
- Polynovo Biomaterial produces and sells NovoSorb, a temporary lattice inserted ahead of skin grafts for patients with severe burns. Following successful registration with the FDA and TGA, NovoSorb has also entered the market in several countries in Asia (India, Singapore, Malaysia, Saudi Arabia).

2019 CCRM and BioCurate sign MOU

The signing of the MoU brings together leaders in drug, biologic, regenerative medicine and medical technology development to pave a viable translation and commercialisation pathway for the Australian regenerative medicine sector.

CCRM Australia is the Australian Hub of the highly successful Centre for Commercialization of Regenerative Medicine in Canada (CCRM)

Established as a not for profit with a national focus, CCRM Australia's mission is to address bottlenecks in the translation and commercialisation of regenerative medicine discoveries in Australia, many of which have the potential to cure some of the most devastating and costly diseases in the world today.

CCRM Australia's commercially focused solutions enable businesses and research partners to achieve their commercialisation objectives by providing customised country, market and industry-specific support. To date, CCRM Australia has collaborated with researchers to advance their regenerative medicine technologies, evaluated and supported promising technologies to seek investment funding, facilitated commercialisation training and worked with international biotechnology companies to set up their clinical trials in Australia. CCRM Australia continues to do so, while providing access to resources and expertise from other CCRM Hubs around the world.

GOVERNMENT INITIATIVES TARGETED AT STEM CELL RESEARCH

THE AUSTRALIAN GOVERNMENT SUPPORTS THE SECTOR BY PROVIDING INCENTIVES

The Research & Development (R&D) tax incentive, which allows for a **38.5-43.5% TAX REFUND** or offset

- The Biomedical Translational Fund
- The Medical Research Future Fund
- The Future Industries/ Sector Growth Program (VIC)
- Co-operative Research Centres (CRCs)
- Industry Growth Centre programs
- Austrade Regenerative Medicine missions

2019
THE AUSTRALIAN GOVERNMENT announces

$80 MILLION

towards the development of a dedicated CAR-T Cell therapy treatment facility at the Peter MacCallum Cancer Centre in Victoria.

The Peter Mac will also contribute

$25 MILLION

to create the Centre for Excellence in Cellular Immunotherapy which will be one of the first dedicated cell therapies treatment facility of its kind, in the world.

2003 TO 2011
the Australian Government provided over

$100 MILLION
IN FUNDING TOWARDS THE AUSTRALIAN STEM CELL CENTRE

STATE GOVERNMENT SUPPORT FOR STEM CELL RESEARCH.

- The NSW and Victorian government have provided funding towards the research and development of Somatic Cell Nuclear Transfer (SCNT) technology.
- The Victorian government formed links with the California Institute of Regenerative Medicine (CIRM) to establish collaborations between research groups based in California and Victoria.
- The Western Australia and Queensland governments have invested significantly in the sector via funding to and collaborations with universities and medical research institutes.

ACCESS TO ASIA

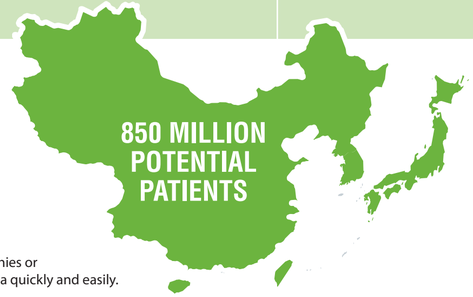
Companies based in Australia can gain easier access to Asia, which contains more than 850 million potential patients.

In addition to time zone and cultural advantages, strong and long trade and investment relationships have been fostered with countries in Asia. Regenerative Medicine-specific country-level agreements exist between Australia and Japan as well as Korea.

Geographically, supply and logistics can reach most regions in Asia as well as potentially the EU and the west coast of the US.

The clinical trials and regenerative medicine eco-system in Australia enables local companies or international company subsidiaries to obtain significant and important first in human data quickly and easily.

Historically, there is an established and proven pathway for Asian companies to obtain high quality data that is recognised by the FDA and European Medicines Agency (EMA) via Australia.



THE AUSTRALIAN REGENERATIVE MEDICINE INSTITUTE (ARMI) WAS ESTABLISHED THROUGH A
$153 MILLION
JOINT VENTURE BETWEEN MONASH UNIVERSITY AND THE VICTORIAN GOVERNMENT.

Please direct enquiries to: AusBiotech/AusBiotech Regenerative Medicine Advisory Group (RMAG) Email address: admin@ausbiotech.org Phone number: 03 9828 1400