Regenerative Medicine in Australia

A Strategic Roadmap for the Regenerative Medicine Sector

Prepared by Research Strategies Australia

AusBiotech

Biointelect

cell therapies

Medicines Australia

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  - Professor Bernie Tuch, Chairman, NSW Stem Cell Network
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  - Professor Megan Munsie, The University of Melbourne
  - Professor Christine Wells, The University of Melbourne
- The University of Sydney

- Professor John Rasko, Faculty of Medicine & Health, The University of Sydney,
  Head, Gene and Stem Cell Therapy Program, Centenary Institute, Head of Department, Cell & Molecular Therapies, Royal Prince Alfred Hospital
- Professor Robyn Ward, Faculty of Medicine and Health, University of Sydney

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Foreword

The Regenerative Medicine Catalyst Project (the Project) has brought together seven partners in a consortium to build the foundations for a national regenerative medicines (RM) sector 'catalyst' collaboration body. The Regenerative Medicine Catalyst Project will address priority action areas including: workforce capabilities, collaboration, funding, regulation and policy infrastructure, and Australian manufacturing capability. The Consortium and the subsequent Catalyst Body aim to support the Australian RM industry to see it thrive and drive benefits to the health of its people and Australia's economy. This *Catalysing Regenerative Medicine in Australia, Strategic Roadmap* (*Strategic Roadmap*) forms a key part of the Project.

The significance and need for the Project was highlighted in a national, sector-wide report that assessed the current state of the Australian RM sector and made recommendations on the priorities and goals, see *Regenerative Medicine: Opportunities for Australia*.[1]

Major outcomes of the Project include other reports and data that each add further to the body of evidence and understanding of the sector. The reports include:

- (This document) A researched, strategic roadmap for the RM sector's development in Australia.
- Sub-report on skills and talent specific to the sector, determining a plan to attract long-term (or patient) venture capital investment and the role of Australian biotech companies partnering with global companies, and case studies;
- Determining a sustainable funding and model structure for an RM sector 'catalyst' collaboration body;
- A regulatory white paper;
- Establishing annual data points and information resources to: map/benchmark GMP manufacturing capability and capacity; establish a model for an annual clinical trial database; and capture investments in Australian RM;
- Mapping the pathway for a typical product from early research to market, and patients receiving a therapy; and
- Mapping the global pipeline of gene and cell therapy products on the horizon.

Context

Australia has an opportunity to harness and leverage a growing and active global RM industry. If we get this right, success could be worth at least $6 billion (B) in annual revenue, 6,000 new jobs for Australia by 2035 and earlier access to ground-breaking therapies for Australian patients[2].

RM is a multidisciplinary field that seeks to develop the science and tools that can help repair, augment, replace, or regenerate damaged or diseased human cells, tissues, genes, organs, or metabolic processes, to restore normal function. It may involve the transplantation of stem cells, progenitor cells, or tissue, stimulation of the body's own repair mechanisms, or the use of cells as delivery vehicles for therapeutic agents such as genes and cytokines.

RM includes gene therapies, cell therapies, and tissue-engineered products intended to regenerate or replace injured, diseased, or defective cells, tissues, or organs to restore or establish function and structure.

Globally, the growing sector has more than 1,200 clinical trials in progress, and attracted AU$26.3B (or US$19.9B) in financing in 2020[3]. With 97 ongoing RM Phase III clinical trials or products awaiting regulatory decisions in the coming months, therapeutics companies are turning their attention to the RM sector[4]. There are also increasing numbers of gene and cell therapies being developed in and brought to Australia for patient access.

Australia has a strong and active RM industry eco-system with basic and translational research capabilities, a clinical trials framework and clinical centres that are all internationally-recognised. More than 60 companies in Australia are developing RM products and more than 130 clinical trials in progress[5].

Introduction

This *Strategic Roadmap* provides the strategic goals, objectives and priority actions for a national RM sector 'catalyst' collaboration body (the Catalyst Body). The *Strategic Roadmap* has been put together based on extensive consultation with stakeholders across the RM value chain (see Acknowledgements) in Australia and desktop research into the Australian and global RM sectors. This consultation and research process revealed the key issues facing the RM sector, which have informed the strategic goals, objectives and priority actions.

Over 2020-2021 the Project brought together seven partners in the Consortium to build the foundations for a national RM sector Catalyst Body. Formation and ongoing activity of the Catalyst Body is planned for October 2021 onwards.

Purpose

This document outlines a draft strategic roadmap for the Catalyst Body in Australia. It includes an outline of the mission and vision; strategic goals, objectives, and priority actions; and detailed discussion of the major issues informing the document.

Mission and Vision

RM represents the possibility of revolutionary, lifelong, and curative therapies to meet some of Australia's most pressing unmet medical needs. RM includes gene therapies, cell therapies and tissue-engineered products intended to regenerate or replace injured, diseased, or defective cells, tissues, or organs to restore or establish function and structure.

Developing and sustaining the necessary capacity and capability requires a coordinated approach across the public and private sectors. The role of pharmaceutical companies and biotechnology companies is key to translating science into new RM therapies. The role of governments is to continue to fund scientific discoveries while providing a regulatory environment that attracts investment, clinical trials, and new therapies into the Australian market. The uniqueness of RM in both opportunity and in challenges is why Australia requires a unified, Catalyst Body led by industry and supported by governments championing the cause.

RM therapies simultaneously provide an opportunity to reduce the burden of disease in Australia and deliver economic benefits to Australia. RM therapies give patients access to cutting-edge advances in world-class therapies, and alternative treatment options, offering significant improvements to the quality of life of Australians. They also have the potential to deliver large economic benefits through a thriving sector that provides STEM-based jobs.

RM can offer treatment for some intractable diseases and has the possibility of treating numerous conditions that have become refractory to available therapies. Some therapies have the capacity to provide 'personalised medicine' using the patient's own cells and producing a therapy that is genotype-matched, circumventing issues such as immune rejection. This will be paradigm shifting for the healthcare system, society and the national economy. Given the impressive efficacy of some of these therapies in diseases for which there are currently few or no other available therapeutic options, clinical trials are now targeting earlier stage disease(s). RM therapies that have been approved within the last few years have demonstrated the ability to increase the overall patient response rates and the duration of responses and therefore, survival.

RM today makes a difference in people's lives with a number of therapies such as graft versus host disease (GvHD) treatments and CAR-T oncology treatments, which have been approved for clinical use. The number of new RM therapies is expected to increase dramatically in the next five years.

Australia is a well-respected competitor in the global R&D market. Recent estimates show that over the past two years, the number of RM companies in Australia has doubled, with the number of research institutions involved in RM increasing from about 30 to more than 45[6]. Australia was the first country to treat an industry-sponsored patient using ground-breaking CAR-T therapy outside North America, with the first therapy approved by the Australian regulator at the end of 2018[7]. Since then, the first commercially available CAR-T therapy from Novartis has been approved in Australia and made available through the public health system.

Realising the potential health, societal and commercial benefits of RM in Australia depends upon a highly-developed and scaled capability across the entire RM value chain[8]. Without the ability to translate our excellent basic science into new therapies the benefits of RM will not be realised. Similarly, without clinical trials[9] and manufacturing[10] capabilities and capacity to test and manufacture therapies in Australia, we will not be able to deliver new therapies to the Australian population. Without the right regulatory and reimbursement pathways[11] in place, we will not gain access to new therapies, which are developing at an unprecedented pace. And without a thriving RM sector, we will miss out on the opportunity to be a manufacturing and export hub across the region.

RM differs in many ways from typical medicines. For example, manufacturing RM therapies poses distinct hurdles. Manufacturing must happen under the highest GMP standards and often close to delivery in hospital settings, and suitable manufacturing facilities are in short supply for commercial contracting. Short shelf life, temperature and hygiene control requirements, higher demands for skilled manufacturing, and small batches are all particular to RM. Similarly, there are unique challenges around how these therapies are reimbursed – one-off, curative therapies are a distinct commercial paradigm from lifelong or extended treatment with medicines. At every stage, RM poses a series of new questions to the Australian health system, patients, and RM suppliers. A national RM sector 'catalyst' collaboration body will address these questions.

Our vision is that Australian patients have access to world-class regenerative medicine therapies sustained by a thriving Australian RM industry.

Our mission is to create an end-to-end world-class value chain that can discover, develop, and distribute regenerative medicine, while creating jobs, commercialising research, and exporting Australian therapies to the world.

We will achieve our mission by:

- Guiding vision and strategy;
- Advancing policy;
- Mobilising resources;
- Building stakeholder engagement (incl. researchers, manufacturers, therapy suppliers, governments and patients);
- Establishing shared measurement practices; and
- Supporting aligned, globally-harmonised activities.

Strategic Goals, Objectives and Priority Actions

Table 1 Summary of strategic plan

Vision	Create an end-to-end world-class value chain (from discovery to delivery) that grants Australian patients access to world-class regenerative medicine therapies, creates jobs, and enable the export of Australian therapies to the world				
Goals	Capabilities / workforce	Collaboration	Funding	Regulation and policy	Infrastructure
Objectives	Attract, build and retain world class talent	Collaborate across the value chain	Secure long-term investment in the sector	Create a clear market access pathway that is aligned to leading global markets	Build Australian capability across the RM value chain (early stage, pre-clinical, clinical, market access, patient delivery)
Priority actions	Advocating for increased financial and structural incentives that encourage RM operations in Australia and that can attract individuals and groups with experience in commercialising RM therapies Building stakeholder engagement and mobilising resources to facilitate training and mentoring programs across the RM value chain Supporting stakeholders to improve information sharing across RM providers, clinicians, and patients especially around successful clinical models Building activities aimed at improving health system readiness by working to embed RM in standards and guidelines and as a medical specialty	Building stakeholder engagement and mobilising resources to facilitate mentoring and networking programs across the RM value chain Supporting efforts amongst stakeholders to ensure that commercial considerations are factored into early-stage research and to manufacturing capability Advocating for and contributing to policy aimed at establishing a national, coordinated approach to capability development (e.g. clinical trials, site selection, etc.)	Encouraging investment from governments and provision of broad economic incentives for private investment including a suite of targeted financing mechanisms Mobilising private investment, via international and domestic venture capital into Australia's biotechnology sector, including increasing institutional and retail investor understanding of RM Encouraging multinational companies to engage locally, to market their products in Australia and also to provide expertise and partnering to SMEs located here Encouraging more public-private partnerships (PPP)	Contributing to policy around expedited approval for RM therapies in Australia Contributing to policy discussions that seek to create definitional and decision-making alignment across the different regulatory mechanisms Advocating for Australia to have access to priority RM clinical trials Contributing to policy discussions on, and advocating for, the standardisation of international quality control processes for RM	Mobilising resources, building stakeholder engagement, and advocating for mechanisms that provide researchers early access to clinical-grade cell lines Mobilising resources and advocating for a national stem-cell registry for researchers Advocating for ongoing and increased government investment in domestic manufacturing capability and infrastructure through additional post-graduate training for the current workforce and undergraduate training for future workforce and, through support of current and emerging manufacturing infrastructure. Building stakeholder engagement and supporting activities aimed at improving health system readiness.

Attract, build and retain world-class talent

We will work with governments, public research organisations, hospitals, and companies to attract, build and retain world-class talent in Australia across the entire breadth of the RM value chain. This will include people with experience in research translation, clinical applications, commercialisation, and scale manufacturing. It will also include developing greater capability within the health system for key skills such as Good Manufacturing Practices (GMP), clinical decision-support, therapy delivery, legal, risk-sharing, and patient after-care.

We will do this by:

- Advocating for increased financial and structural incentives that encourage RM operations in Australia and that can attract individuals and groups with experience in commercialising RM therapies;
- Building stakeholder engagement and mobilising resources to facilitate training and mentoring programs across the RM value chain;
- Supporting stakeholders to improve information sharing across RM providers, clinicians, and patients especially around successful clinical models;
- Building activities aimed at improving health system readiness by working to embed RM in standards and guidelines and as a medical specialty.

Collaborate across the value chain

We will work with all stakeholders to increase the level of collaboration in the Australian RM sector. The aim is to increase the speed of development of new therapies, their commercialisation and their availability to patients. This includes the commercialisation of new home-grown therapies, as well as ensuring Australian patents gain access to international therapies.

We will do this by:

- Building stakeholder engagement and mobilising resources to facilitate mentoring and networking programs across the RM value chain;
- Supporting efforts amongst stakeholders to ensure that commercial considerations are factored into early-stage research and to manufacturing capability;
- Advocating for and contributing to policy aimed at establishing a national, coordinated approach to capability development (e.g. clinical trials, site selection, etc.).

Secure long-term investment in the sector

We will advocate for the Australian RM sector, and support programs and projects that will attract increased investment into Australian RM. This includes working with governments on addressing areas where there are apparent market failures (e.g. manufacturing), attracting greater international private sector investment (e.g. multi-national pharmaceutical companies partnering with local biotechnology companies), as well as greater injections of venture capital. The aim is to secure the conditions for a vibrant commercial RM sector (both to meet Australian patient needs and also develop an export industry).

We will do this by:

- Encouraging investment from governments and provision of broad economic incentives for private investment including a suite of targeted financing mechanisms;
- Mobilising private investment, via international and domestic venture capital into Australia's biotechnology sector, including increasing institutional and retail investor understanding of RM;

- Encouraging multinational companies to engage locally, to market their products in Australia and also to provide expertise and partnering to SMEs located here;
- Encouraging more public-private partnerships (PPPs).

Create a clear market access pathway that is aligned to leading global markets

We will contribute to policy discussions to develop and align regulation with leading global markets and create clarity around the market access pathway. We will work across the different regulatory agencies and departments involved in RM regulation to ensure that there is clarity, consistency, and alignment to ensure fast access for Australian patients to new RM therapies.

We will do this by:

- Contributing to policy around expedited approval for RM therapies in Australia;
- Contributing to policy discussions that seek to create definitional and decision-making alignment across the different regulatory mechanisms;
- Advocating for Australia to have access to priority RM clinical trials;
- Contributing to policy discussions on, and advocating for, the standardisation of international quality control processes for RM.

Build Australian capability across the RM value chain (early stage, pre-clinical, manufacturing, clinical, market access, patient delivery)

We will contribute to the development of infrastructure and capability across the entire RM value chain. This will assist in taking advantage of the significant opportunities for Australian patients to access global therapies and products – and for Australian companies to collaborate with international entities seeking to bring projects into our region – as well as for Australian companies to export novel RM therapies globally. We see that there is an opportunity for Australia to be a regional hub in the Asia Pacific region known for leading research, clinical trials, translational know-how and manufacturing capabilities. However, unlocking these opportunities requires a significant uplift in our capabilities.

We will do this by:

- Mobilising resources, building stakeholder engagement, and advocating for mechanisms that provide researchers early access to clinical-grade cell lines;
- Mobilising resources and advocating for a national stem-cell registry for researchers;
- Advocating for ongoing and increased government investment in domestic manufacturing capability and infrastructure through additional post-graduate training for the current workforce and undergraduate training for future workforce and, through support of current and emerging manufacturing infrastructure;
- Building stakeholder engagement and supporting activities aimed at improving health system readiness.

Issues Informing the *Strategic Roadmap*[12]

Health system readiness

Providing RM therapies in clinical settings in Australia poses a series of challenges. It has been widely observed that there is a mismatch between existing hospital and clinical capabilities and those required for delivering RM therapies. The current delivery of healthcare is designed based on drug delivery, device-based therapies and surgery.[13] These are likely poorly suited for RM therapies[14] Related issues range from site selection, through to pharmacy capabilities, through to clinical expertise, consenting processes, oversight of non-used materials (e.g. cells and tissue), and setting realistic patient expectations.

Clinical issues begin from the point of manufacture, starting with the possibility that hospitals themselves may in part or whole become manufacturing sites for therapies. This requires significant upskilling as well as investment in infrastructure such as GMP-licenced clean rooms and bioprocessing equipment. In addition, Quality Assurance (QA) systems and Quality Control (QC) processes will need to be implemented where currently they do not exist. And, in some cases, hospitals are also required to meet various licensing requirements from the Office of the Gene Technology Regulator (OGTR) to handle genetically modified organisms.

Beyond manufacturing there are more general problems such as the fact that many RM products will have very short shelf lives. As such they may arrive in forms (e.g. cryopreserved) that hospital pharmacies are not readily prepared to receive.

In addition to the infrastructure aspects of these issues there are corresponding skills and workforce issues. The current clinical workforce is not trained in many of the technologies that will be used in the procurement (legal, ethical, commercial, such as risk-sharing) processes, production and delivery of these therapies.[15] As well as the RM products, there are likely to be a range of accompanying technologies (e.g. decision-support tools, monitoring and tracking software) that need to be introduced in support of RM therapies.

Finally, there are overarching issues such as how sites are assessed and selected, how risk-sharing arrangements are designed, what special access mechanisms are put in place between government, hospitals, and RM suppliers, and what long-term follow up is required with patients after therapies have been administered.

This is made more complicated in Australia given the different jurisdictions involved between state, territory and federal governments. At all stages, equitable access to RM therapies for all Australians must be considered as required under the National Health Act.

Access to clinical-grade stem cell lines

An important part of translating cell and gene research into clinical applications and commercial products is early access to clinical-grade lines. Using clinical-grade lines means that there is no ‘retrofitting’ to meet regulatory requirements when it comes to commercial development. However, using clinical-grade lines as a starting material is a complex and costly proposition, including being able to demonstrate that lines derived from different individuals and in different facilities are comparable.[16] In addition, manufacturing these lines for use in clinical trials involves a series of challenges that have not previously been faced. For example, a significant challenge is researchers gaining access to GMP-grade lines. This circumvents later complications around manufacturing compliance and other quality control standards that are used by regulatory agencies[17] which presently require this from Phase II onwards.

Documenting quality and suitability of cell lines

To deliver controlled disease modelling and drug screening maintaining the quality and suitability of cell lines is key. This requires maintaining and collating substantial documentation including ethical provenance, line derivation, culture conditions and genetic constitution of lines. This information is likely stored across multiple sources that are sometimes not publicly available, let alone available with standardised meta-data for easy access.[18]

In Australia, there is currently little oversight of what lines are made and available to researchers. As a result, lines are shared around with little ability to map their provenance or the associated ethics approvals. Further, many lines are only available in private collections and there is little knowledge of where these are and what they could be used for. In other jurisdictions stem-cell registries seek to address this by:

- Establishing a standard name and unique identifier for a cell line, making it recognisable throughout publication and data resources:
- Verifying ethical provenance and scientific evidence for pluripotency using standardised criteria;
- Offering access to information related to the individual stem-cell lines such as derivation, cultivation, genetic constitution and application;
- Providing secure, searchable and unrestricted storage of valuable data on stem-cell lines;
- Guaranteeing global visibility, confidence in ethical provenance, validation of characterisation data, and comparability with other registered lines.[19]

Other jurisdictions have successfully established registries, including for example the National Institutes of Health (NIH) Human Embryonic Stem Cells Registry in the US, the Human Pluripotent Stem Cell Registry in the European Union, and the National Registry of Human Embryonic Stem Cell Lines in Canada. In some cases, these are designed to make the translational process as smooth as possible from the perspective of regulation and commercialisation.

Planning for commercialisation of research

To effectively plan for the commercialisation of RM research, a number of considerations must be made at the early stages. This includes understanding the future clinical requirements, production demands, and the full range of associated costs. This is particularly critical given the time intensive nature and high development costs associated with RM technologies.[20]

Consideration of factors critical to translation and commercialisation by RM researchers is made more complicated by the range of actors involved along the RM development pathway from basic research to clinically validated therapy. This includes researchers, clinicians, biotech companies, pharmaceutical companies, manufacturers, regulators, payers, and patients.

Each of these will have a unique set of needs that should be factored in during early-stage research to increase the likelihood of successful translation of research. For example, consideration of GMP and other quality control requirements from the earliest stages increases the likelihood of translational outcomes.

At present, there are few mechanisms in place in Australia that promote integration of later stage requirements into early-stage research. For example, neither the National Health and Medical Research Council (NHMRC) nor the Medical Research Future Fund (MRFF) (with the exception of a few translation-focussed grants such as the Accelerated Research Initiative Disease Teams) include this as part of funding agreements for RM research. There are emerging models for how this might be done globally that Australia

can look to. Amongst these is the Public Private Partnership (PPP) model of organisations such as the European Innovative Medicines Initiative (IMI).[21] IMI seeks to accelerate development of, and patient access to medicines in areas where there is an unmet medical or social need. It involves participation from universities, research centres, the pharmaceutical and other industries, small and medium-sized enterprises (SMEs), patient organisations, and medicines regulators and is a partnership between the European Union (represented by the European Commission) and the European pharmaceutical industry (represented by EFPIA, the European Federation of Pharmaceutical Industries and Associations). It had a budget of €3.3 billion for the period 2014-2020 to support its activities. Another such example is the Centre for Therapeutic Innovation (CTI), which facilitates collaboration between Pfizer and select academic medical centres, disease foundations, and the NIH. Its aim is to translate promising science into clinical candidates via accelerated first-in-human proof of mechanism studies.[22]

Manufacturing capability

The process for manufacturing RM therapies is very expensive and complex. RM therapies require a diverse set of infrastructure to support their delivery. The scale-up from limited laboratory facilities to automated systems for bulk production needs to be timed and planned by manufacturing providers. GMP manufacturing, which represents a significant step up in capability, is required as the RM product progresses through the clinical trial pathway. Even at the Phase I clinical trial stage, issues related to scalability need to be considered and factored into costs. In terms of cost-effectiveness, RM represents a new manufacturing paradigm compared with traditional drug development.[23]

In part this is due to the highly-skilled workforce that is required to manufacture therapies – there is less opportunity for automation than in traditional drug manufacturing. Other factors are related to the specialised requirements of manufacturing RM therapies, including GMP-graded clean rooms, storage, and supply chain logistics.

This occurs in a context in which there is currently little clarity about reimbursement for therapy producers. All in all, this creates a situation in which our manufacturing capability is limited because the return on investment for set-up is not guaranteed and therefore not attractive to manufacturers.

In other jurisdictions there has been significant investment in manufacturing capability to overcome this 'chicken or egg' situation (i.e. we need manufacturers to have a local RM industry but manufacturers will not come until there is an established RM industry to service). And while the Australian government has made some investments in this respect (e.g. the Federal Government's investment of $80 million to establish the Centre for Excellence in Cellular Immunotherapy at the Peter MacCallum Cancer Centre to increase patient access to CAR-T, or the viral vector manufacturing facility at Westmead, NSW) this is still not at the scale of investment needed to keep pace with the diverse products on the horizon. Examples from other jurisdictions also point to the important role for PPPs, such as the Vaccines Manufacturing and Innovation Centre (VMiC) in the United Kingdom, the UK Cell and Gene therapy Catapult and the 'Cellicon Valley' concept in Philadelphia. Such approaches may have an important role to play in Australia.

In addition to infrastructure, there are large gaps in the people-based capability for manufacturing. RMs require a highly-skilled workforce that does not yet exist. At present24 Australia has limited workforce capability, comprising 231 full time and 45 part-time and casual employees working across RM GMP manufacturers and an additional [24] full-time and 10 part-time/casual employees working in manufacturing facilities for clinical trials (i.e. where TGA-licensed GMP is not a requirement). This is a very small workforce and nothing like the scale that is required for Australia to take advantage of the RM opportunities available. In the short term this can be addressed through upskilling of workforce from other related areas of the medical sector, for example graduates with training in pathology or in pharmacy can receive supplemental training in GMP manufacturing requirements. There are already gains being made in this respect through partnerships

such as the UTS/SeerPharma training program. More short-term postgraduate qualifications like these will, in the short term, provide a boost to the available workforce. However, this approach in the long-term will simply transfer workforce shortages into other areas of the health and medical system and not address the overall burden.

In the long-term, there need to be strategies in place to create additional workforce with these specialised skills. To do this, GMP skills and know-how need to be embedded within a range of relevant undergraduate programs as a core competency. Unlike the reskilling approach, this will create a pipeline of new workers. This will require deep collaboration between universities and other training providers and industry, to address the specific and evolving needs of industry. With the emergence of government initiatives such as the Job Ready Graduates package – which provides incentives for universities to orient their courses to criteria including employability, and which also provides broader scope for the participation of non-university higher education providers to provide short-courses – there are now more opportunities and financial incentives for universities to participate in this kind of co-design approach.

Standard quality control processes

Compliance with QC standards is fundamental to ensuring that RM therapies are safe for use in human patients. QC impacts the entire RM value chain and needs to be factored in across the development pathway.

For RM therapies there is currently no international QC standard. This is particularly important where therapies are being developed across jurisdictional boundaries. Without harmonisation of QC standards (and QA) a situation can arise where the release of raw materials for manufacturing is jeopardised because the QC requirements of the supplying jurisdiction are not compliant in the receiving jurisdiction where manufacturing occurs.[25] This is particularly important for Australia if we are considering a role as a potential hub across the broader Asia-Pacific region.

QC also forms a core requirement for GMP compliance. As such, it is a fundamental part of the pre-clinical phase development. There are unique challenges for undertaking QC for RM therapies. For example, because RM therapies are often produced in small quantities there can be limited quantities of product available for use in QC. The short shelf life of RM therapies means that timing of QC is much more critical than in pharmaceutical products and the complexity of developing assays for QC/QA for RM products that are cell-based requires identification of the cell type/s in a heterogeneous product that are leading to the therapeutic effect of the product. Developing potency assays can be very difficult when dealing with complex biology and therapies in which the mechanism of effect is not clearly understood.

Furthermore, standard QC procedures and mindset, which have historically governed the production and release of standard therapies (e.g. tablets or solutions for injection), do not apply to RM and require adaptation from both the manufacturers and regulators. QC also requires a pragmatic approach when dealing with life-saving treatments, especially in later lines of therapies.

As such, existing testing methods that are approved by regulators such as mycoplasma testing and sterility testing are not fit-for-purpose for next generation therapies. At the same time, specialised testing methods such as those that employ polymerase chain reaction (PCR-based) methods and rapid sterility testing have not necessarily been approved by regulators.[26]

Speed of discovery vs speed of regulation

The RM field is developing at a rate that exceeds the ability of traditional regulatory approval pathways to keep pace. In response several jurisdictions have developed specific fast-track approval pathways for RM therapies. In the US, for example, the 21st Century Cures Act of 2016 was aimed at accelerating the translation and approval of regenerative therapies for unmet patient need by lowering requirements to go through rigorous, large-scale Phase III trials, and by promoting methodological alternatives such as adaptive and other new trial designs.[27] The legislation specifically required the FDA to develop expedited pathways for RM

therapies and to develop the Regenerative Medicine Advanced Therapies (RMAT) designation to review and approve these products. This applies to cell therapies, gene therapies, therapeutic tissue engineered products, human cell and tissue products and combination products using these therapies or products.[28]

Similarly, in Japan the Regenerative Medicine Promotion Act catalysed reforms to foster the development of regenerative medicine. Japan has effectively created two expedited approval pathways. The first is the so-called 'drug track' which allows expedited, time-limited approval for RM therapies after demonstrating safety and clinical benefit. In effect this provides market approval based on a limited number of subjects and surrogate endpoints. The second pathway, the so-called 'clinical pathway' allows RM developers to make their therapies available to patients following from clinical research or trials conducted by physicians involving relatively small numbers of participants. In this pathway, even if a therapy is not approved and is prescribed off-label, it will be partially covered by social health insurance upon demonstrating a level of efficacy and safety in a clinical trial.[29]

In Australia, by contrast while there is an expedited pathway provided by Therapeutic Goods Administration (TGA), it is not available for biologicals and is only available for prescription medicines. This means it cannot be applied to all RM products. Another challenge relates to the sustainable and timely access to RM products, regarding the lack of specific and effective pathways to assess their cost-effectiveness.[30]

All considerations in this respect must be driven by primary concern for patient safety and quality. The balance between the risk and benefit to patients treated with RM therapies must be correct, particularly given the uncertainty that still surrounds some emerging RM therapies.

Encourage clinical trials of new RM therapies in Australia

For patients to gain early access to RM medicines it is important that Australia is an attractive place for multi-national developers to launch and develop new therapies. The importance of clinical trials can be seen in trials currently underway at the Hudson Institute of Medical Research looking into how amniotic epithelial cells could be used as a therapy for the acute phase of COVID-19.[31] A key aspect of this is developing incentives for companies to invest in the conduct of clinical trials in Australia. The different phases of the development pathway are integrally connected: we require a regulatory system that allows Australians access to new therapies that in turn encourages investment in R&D by providing early access to new therapies via clinical trials. This is particularly important in the context of RM therapies where market entry is uncertain and unpredictable. In addition, there is need to build and expand the network and infrastructure for testing in the R&D phase. Having the infrastructure (e.g., in genomics, precision medicine) to continue to attract innovation and placement of trials, prior to regulatory approval and reimbursement processes.

It is widely acknowledged that the regulatory and reimbursement processes for new therapies is a core driver for companies in determining their launch sequence. Significant time lags between registration and reimbursement, for example, are likely to put Australia's potential as an early wave launch country at risk.

At present, the review times for the Clinical Trial Approval (CTA) scheme (previously called the Clinical Trial Exemption scheme – CTX) are a barrier to the clinical trial activities of RM companies. Of the two pathways for the approval of clinical trials, the Clinical Trial Notification (CTN) scheme is generally not available for Class 4 biologicals (that most RM therapies fall under) and, therefore, the CTA route is usually used for these therapies going into clinical trials in Australia. The exception to this is if an overseas entity, such as the FDA, has previously approved a clinical trial, and this is accepted by the TGA, thereby allowing for the more time-effective CTN route to be used for a trial in Australia. Currently, there is no fixed time for completion of review (compared to the 30-day turnaround by the FDA on an Investigational New Drug (IND) application), and there is no opportunity to update the application should more information become available while under review. This causes uncertainty and affects long-term strategy, which is especially damaging for SMEs. The delayed

clinical trial start times can affect investor confidence and frustrate patients, who are keen to have access to potentially lifesaving medication through participation in these clinical trials.

The TGA has already introduced a priority and provisional registration pathway to provide access to new and innovative therapies and is increasing collaboration with regulators in other jurisdictions to continue to ensure that Australia is an early wave launch country. In the case of RM, there are additional nuances that need to be considered. For example, RMs are often targeting small patient populations and therefore identifying participants for clinical trials can be even more difficult. So clinical trials may present an additional hurdle to launching new therapies in Australia. In the case of having to engage in multi-site trials, this can be very complex, occurring across multiple jurisdictions and with every study needing ethics and governance approvals before it can commence.[32]

Access to capital

The barriers to investment in RM in Australia are in many ways no different from those associated with life sciences investing in Australia in general. Low levels of venture capital (VC) funding, geographical isolation and gaps across the research translation pathway[33] are all well accepted constraints.

However, there are a range of unique challenges facing RM companies. Amongst them, the costs associated with GMP-quality products in the pre-clinical phases, regulatory uncertainty, and questions around the health economics are all acknowledged[34] as barriers to increasing investment into Australian RM.

Equally important is the fact that these are cutting-edge technologies with few having completed the path the patients yet. Therefore, there are limited success stories for the RM sector to point to when trying to show the future returns for investors. Like other innovative technologies, RM is still being met with enthusiasm for investing, evidenced by the large investments here and elsewhere globally, despite the future being unknown.

Data on the global investment landscape in RM shows that there is increasing investment in the RM sector. The Alliance for Regenerative Medicine (ARM) reports that in 2020 $US19.9b investments was raised by RM companies, up from just US$9.8b in 2019, US$13.3b in 2018 and US$7.5b in 2017.[35] The trend of increasing investment is continuing in 2021 with US$14.4b raised in the first half of the year which is 71% of what was raised in the full year 2020[36].

For Australia to increase the pool of funding there are two options that need to be pursued: increasing and evolving the local funding sector, and gaining greater access to the international funding sector. Growing the local investment sector first and foremost requires increasing the RM-literacy amongst local investors – institutional and retail alike.

In the short term, however, the most likely way to address this is by accessing international VC funding as opposed to growing the local VC sector. To do this there is need for a more focused set of services for Australian RM companies to access international VC funding. The Canadian Government's Boston/Cambridge Life Sciences Technology Accelerator is exemplary of this, as is the European Network of Research and Innovation Centers and Hubs (ENRICH) USA.[37+38]

In addition, we need to provide incentives for VC funding to come to Australia. One way to address this is by providing investors a suite of targeted financing mechanisms that can at once mitigate the downside risk while increasing the upside return for investors. Several ideas have been floated and some have been trialed globally. For example, in the USA the Rare Disease Fund Act of 2018 was introduced to "establish a rare disease therapeutics corporation to encourage the development of high-risk, high-return therapies for rare diseases, and for other purposes."[39] While such novel financial mechanisms are not yet pervasive, there are a range of smaller measures that have been introduced in various jurisdictions to incentivise investment. For example, in the UK, schemes such as Seed Enterprise Investment Scheme (SEIS) and Enterprise Investment Scheme (EIS) aim to attract investment into potential high-growth UK small and medium startups, respectively.

Both programs effectively minimise downside risk and are now routinely used by VC funds with biotech and health tech focus.[40]

Such programs do not have to be in place forever, but providing investors with a suite of financial mechanisms to build the RM sector in the coming ~five years will assist in creating a vibrant market that can then continue with existing support mechanisms (e.g. R&D Tax incentives, Accelerating Commercialisation grants, etc.). The role of governments at this stage is paramount in building the conditions for attracting greater investment – be it local or global – into the RM sector.

Collaboration

Many of the issues discussed in this paper can be partially addressed through greater coordination and collaboration in Australia across the RM development pathway. For example, there is little feedback that occurs between researchers and individuals who have successfully developed RM products. Facilitating formal mentoring and networking opportunities across these two competencies would likely go some of the way towards embedding later-stage development considerations into early-stage research.

Similarly, there is still significant siloing of cell and gene research for the purposes of research funding, which leads to atomisation of the RM research efforts. Coordination of these under the banner of RM would likely assist in driving forward the RM industry in Australia. More fundamentally, the competitive allocation mechanisms of funding for research and development (i.e. grants) fosters competition and replication of efforts rather than cooperation and leveraging of existing capabilities.

Competitive mechanisms also negatively impact our clinical trial capabilities – states compete to attract clinical trials, which means that Australia has sites wherever they have been attracted rather than where they make sense for patient access. Again, coordination and collaboration between states and territories, federal government, hospitals and RM providers would assist with this.

Another area where this kind of systemic collaboration may be useful is in the provision of access schemes to fill the gap between TGA approval and reimbursement. This requires significant risk-sharing between hospitals, government, and RM providers.

These are just some examples of where coordination and collaboration across the sector will drive increasing commercial opportunities as well as increased patient access and outcomes. Without a high degree of integration, the Australian RM sector will miss out on significant commercial and health outcomes.

Appendix A: Sub Report on Investment and Talent

Executive Summary

This sub-report was prepared to supplement the *Catalysing Regenerative Medicine in Australia, Strategic Roadmap* (*Strategic Roadmap*). This sub report is a further investigation of skills and talent specific to the sector and a plan to attract long term investment in the sector and the role of Australian biotech companies partnering with global companies. The findings in this sub-report have informed and been incorporated into the *Strategic Roadmap*. This report was prepared through desktop research and consultation with key stakeholders across the regenerative medicine (RM) sector. A summary of the recommended actions based on the findings of this sub report can be seen in Table 2.

Table 2 Priority actions for addressing skill and talent and investment in the RM sector in Australia

Action Area	Sub Area	Priority action
Increasing access to capital	Increase and evolve the local funding sector	1. Investor education in collaboration with institutional investors (superannuation funds) and retail investors (via ASX)
	Create greater access to international VC funding	1. Developing a pathway for Australian RM companies to land in Boston and other locations 2. Provide investors a suite of targeted financing mechanisms
Health System Capabilities	Training	1. Work with Pharmacy Board of Australia and Medical Board of Australia to create and disseminate standards and guidelines, and a RM medical specialty, respectively
	Organisational change	1. Propagate and support the Centre of Excellence model currently being used by RPA
Commercialisation	Attract	1. Identify and target international talent
Manufacturing	Training	1. Re-skill existing workforce via short-term postgraduate qualifications in GMP in collaboration with a range of tertiary providers (under the Job Ready Graduates reforms). 2. Create additional workforce by embedding GMP skills and know-how in undergraduate programs in collaboration with universities.

Investment

The barriers to investment in Regenerative Medicine (RM) in Australia are in many ways no different from those associated with life sciences investing in Australia in general. Low levels of venture capital (VC) funding, geographical isolation from international investors and gaps across the research translation pathway[41] are all well-accepted constraints.

However, there is a range of challenges that RM companies face in raising funding that are unique to this sector. Amongst them, the costs associated with GMP-quality products in the pre-clinical phases, regulatory uncertainty, and questions around the health economics are all acknowledged[42] as barriers to increasing investment into Australian RM.

Perhaps equally important is the limited success stories for the RM sector to point to when trying to show the future returns for investors, because of the cutting-edge nature of RM.

In recent years, however, there are increasing signs, particularly overseas, that the barriers are either being overcome, or else seen as less important than they once were.

Globally, investors seem to have now accepted that RM will follow the well-worn trajectory of incremental, iterative development that other biotech technologies have. Progress in areas such as oncology, rare diseases, hematologic diseases and inherited retinal conditions has also paved the way for more optimistic global investor outlook.[43]

Data on the global investment landscape in RM shows that there is increasing investment in the RM sector. The Alliance for Regenerative Medicine (ARM) reports that in 2020 $US19.9b investments was raised by RM companies globally, up from just US$9.8b in 2019, US$13.3b in 2018 and US$7.5b in 2017.[44]. The trend of increasing investment is continuing in 2021 with US$14.4b raised in the first half of the year which is 71% of what was raised in the full year 2020[45].

Investments in Australian RM

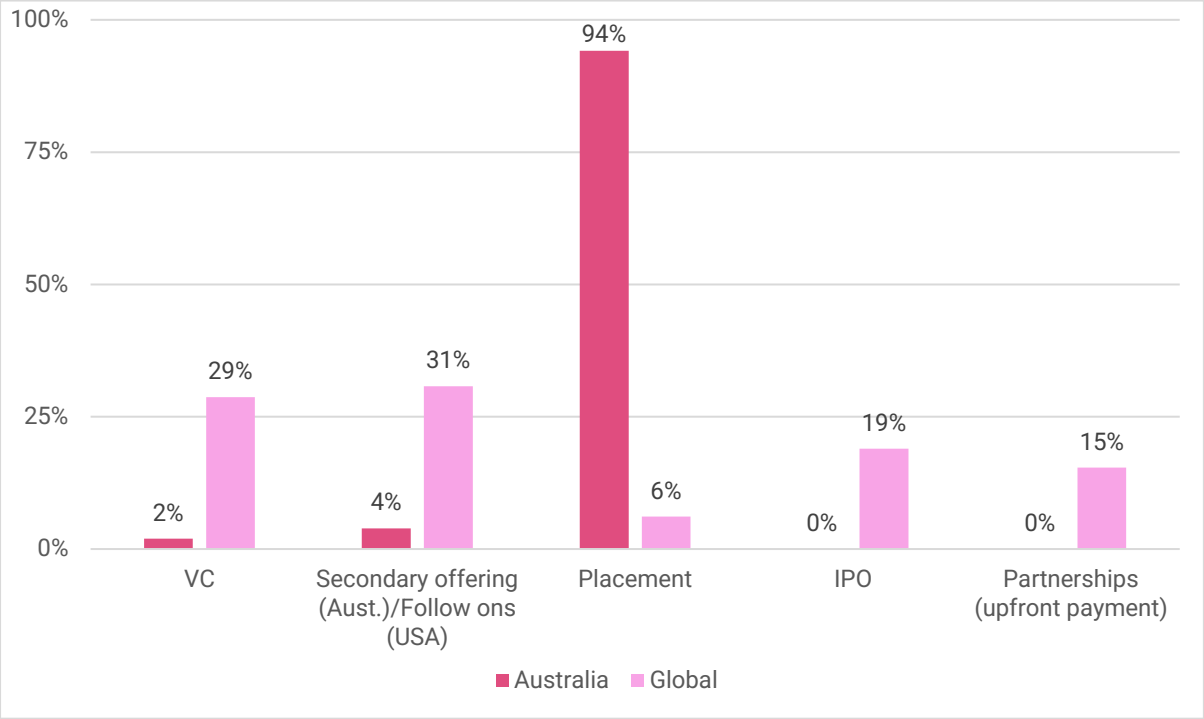
In Australia, the signs point to increasing optimism amongst investors with $394.1m invested in 2020, compared with just $184.7m in 2019.[46]

As a percentage of the global RM investment activity, Australia is doing comparatively well and accounts for ~2 percent of global investment, despite being 1 percent of the global population. What is telling, however, is the extent to which Australia is dependent on placements as a form of raising capital compared with the global norms.

Placements account for 94 per cent of the capital raised by Australian RM companies in 2020 ($371m). This compares with the global picture (Figure 1) which shows a spread of investment mechanisms being employed, led by Follow Ons (31 per cent), VC funding (29 per cent), IPOs (19 per cent), Partnerships (15 per cent) and finally Placements which only account for 6 per cent of investment activity.

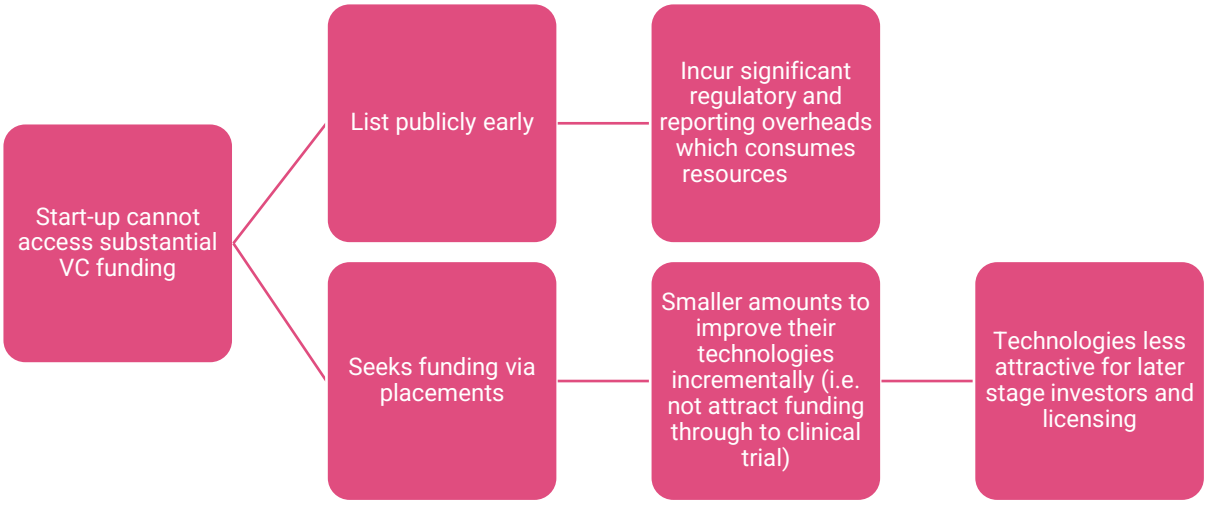
This reveals two things about the investment environment and strategies in the Australian RM sector: first, it highlights the importance of institutional investors to the current Australian RM sector.

Figure 1 Investment by type 2020



Perhaps more importantly, however, it shows how little access Australian RM companies have to VC funding. This is an important distinction to the global picture and something Australia needs to address to progress the sector. The VC funding gap leads to a series of events that will hinder the RM sector if not addressed (Figure 2).

Figure 2 Common fundraising scenarios for Australian RM companies



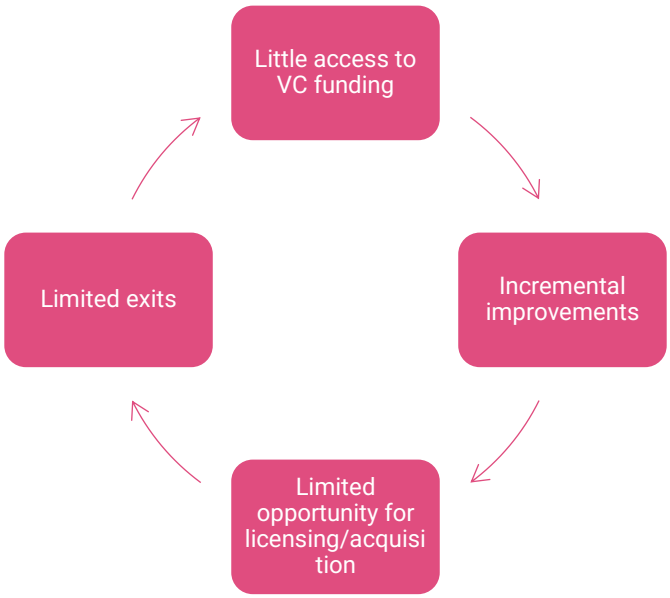
In the absence of VC funding, companies are left with two primary fundraising options: to list publicly on the ASX or to offer private placements.

In the case that a company lists early, they have reported that the regulatory and reporting requirements that come with being publicly listed absorb precious resources and oftentimes see inventor/founders acting in roles that they are not equipped to effectively undertake.

In the case that a company instead seeks private placements, the amounts raised are often only a small proportion of what is required to take a technology through to clinical trial stage. As a result, technologies are improved incrementally. This risks losing ground to competitors in other jurisdictions where VC funding is in higher supply. This approach has the additional consequence that it makes it harder to demonstrate to potential partners (say, large biotechs) a commercial return – as it was put by one interviewee, a start-up that seeks funding with "a mouse model and a few published papers" is not a compelling licensing or acquisition prospect.

Short supply of early VC funding (and the chain of events that follows) therefore creates a vicious cycle for RM companies in Australia – incremental improvements do not make assets attractive targets for licensing or acquisition which in turn limits the exit strategies that would make VC funding interested in the first place, creating further disincentives for VC funding to enter into the Australian sector (Figure 3).

Figure 3 How VC funding constraints perpetuate



Investor Education

The short supply of investment funding to Australian RM is made more acute in the local investment sector which has had little exposure to RM opportunities. There is a lower level of literacy amongst local retail and institutional investors, as well as VC funds of RM, compared to jurisdictions such as USA, excepting those that are specifically set up to invest in the sector such as the MRCF by Brandon Capital. For those investors where there is a greater understanding of RM the barriers associated with life sciences investing in Australia in general, combined with the unique challenges of RM, such as the costs associated with GMP-quality products in the pre-clinical phases, regulatory uncertainty, questions around the health economics and still small numbers of high quality RM investment opportunities in Australia is resulting in the low investment in RM.

In addition, the nascent regulatory and reimbursement landscape globally means that more traditional investing considerations need to be supplemented with detailed analysis that is not widely available across investors.

For example, considerations of the interplay between total market opportunity (e.g. addressable market which may be much smaller than for traditional therapies) and reimbursement (e.g. reference pricing may not be available for novel therapies) are relatively unknown for many emerging therapies. This makes the case for investment harder than for the traditional therapies. The competencies to undertake this analysis are not pervasive across the either the Australian retail nor institutional investing sectors.

Investment - Priority Actions

Increasing access to capital

The importance of increasing the pool of funding available to early-stage Australian RM companies cannot be over emphasised. There are two options that need to be pursued: increasing and evolving the local funding sector and gaining greater access to the international funding sector.

Increase and evolve the local funding sector

Growing the local investment sector first and foremost requires increasing the RM-literacy amongst local investors – institutional and retail alike. One proposed way of addressing this is for the RM Catalyst to engage directly with key stakeholders across these sectors, namely the superannuation funds and the ASX, respectively. This should be undertaken in addition to the many activities that groups such as AusBiotech already undertake with investors.

The ASX has a track record of engaging in education activities for particular sectors – its Diggers and Dealers events, for example, are collaborations between ASX and the resources sectors in Australia, targeted at raising awareness of the investment opportunities to retail investors.

With respect to superannuation funds, the need to pool investment opportunities into a portfolio is a fundamental starting point for increasing their participation in RM investing.

Additional ground can likely be made working directly with ultra-high net worth individuals and family offices. Specifically, sharing the know-how to assist these investors with overcoming areas of perceived uncertainty around RM assets (e.g., addressable market, reference pricing, the economics of manufacturing costs etc.) is a fundamental area to address.

In each case a very close collaboration is required to raise the level of understanding required to encourage greater investment.

However, even if these sources of funding are increased substantially, they are unlikely to solve current funding gaps, specifically those at the early stages of development. This is where increased VC funding is fundamental.

In the short term the most likely way to address this is by accessing international VC funding as opposed to growing the local sector. There are two ways that have been proposed for doing this throughout the consultation: Australian companies accessing capital where it resides; and, international capital being attracted to Australia.

The most likely destination for Australian RM companies to be exposed to significant international VC funding is in the USA (Boston, La Jolla, San Francisco, etc.). At present, the Federal Government's Landing Pad program

operates in Singapore, San Francisco, Tel Aviv, Berlin and Shanghai. A dedicated Landing Pad in Boston for RM companies would likely be a welcome addition to the program.

Access to international VC funding

While landing pads provide a useful service (such as dedicated managers, co-working space, access to in-market Entrepreneurs in Residence, connections to local founder communities and introductions to Austrade customer networks, partners and contacts) there is need for a more focused set of services for RM to access VC funding.

The Canadian Government's Boston/Cambridge Life Sciences Technology Accelerator is exemplary of this.[47] Similar programs focused on Boston are offered via the EU through the European Network of Research and Innovation Centers and Hubs (ENRICH) USA,[48] which includes the Boston centre (managed by Temple University) and with a dedicated HealthTech and Biotech hubs being established in Boston. In addition, there are emerging examples partnering government and private providers, as the ENRICH program is partnering with AccelHub, a commercial accelerator focused on bringing international companies into Boston.[49]

Developing a pathway for Australian RM companies to land in Boston is one way to provide greater access to the relevant VC market. Another is to provide additional incentives for VC funding to come to Australia. In interviews there was little noted that would attract VC funds to Australia – while the quality of our science is not disputed, and the potential of our start-ups and RM companies is acknowledged as world class, our geographical isolation and our relatively small-scale sector are unlikely to attract funders away from key markets in the USA. In fact, at the moment unless a VC fund has a specific mandate to operate in this jurisdiction there is a low chance that it will.

Financing mechanisms and incentives

One way to address this is through providing investors a suite of targeted financing mechanisms that can at once mitigate the downside risk while increasing the upside return for investors.

Several ideas have been floated and some have been trialed globally. For example, in the USA the Rare Disease Fund Act of 2018 was introduced to "establish a rare disease therapeutics corporation to encourage the development of high-risk, high-return therapies for rare diseases, and for other purposes."[50] As imagined by Silicon Valley Bank, the aim is to have:

> *"large pools of low-correlation risk assets combined with a more predictable financial return model, attracting huge new sources of capital from investors, even retail investors. This has the promise of fueling new drug development at earlier stages and solving medical challenges faster. …We could be at the very beginning of a new investing strategy that may lead to major disruptions in traditional scientific investing as we know it."*[51]

While such novel financial mechanisms are not yet pervasive, there are a range of smaller measures that have been introduced in various jurisdictions to incentivise investment. For example, in the UK, for example, schemes such as Seed Enterprise Investment Scheme (SEIS) and Enterprise Investment Scheme (EIS) aim to attract investment into potential high-growth UK small and medium startups, respectively.

SEIS allows individual investors (not companies) can invest up to £100,000 in an SEIS business per tax year and claim this as 50 per cent tax break plus additional capital gains relief. Under SEIS a company can accept up to £150,000 total funding.

EIS allows individual or corporate investors to invest up to £1 million per tax year with 30 per cent tax deduction and additional capital gains tax exemptions. Under EIS a company can accept up to £5 million per tax year up to a £12 million funding total. For Knowledge intensive companies (which is where RM companies

would fall) the amounts under EIS are increased to up to £10 million per tax year and up to £20 million total EIS funding.

Both programs effectively minimise downside risk and are now routinely used by VC funds with biotech and health tech focus such as Calculus Capital and o2h Ventures.

Such programs do not have to be in place forever, but providing investors with a suite of financial mechanisms to build the RM sector in the coming ~5 years will assist in creating a vibrant market that can then continue with existing support mechanisms (e.g., R&D Tax incentives, Accelerating Commercialisation grants etc.). The role of government at this stage is paramount in building the conditions for attracting greater investment – be it local or global – into the RM sector.

Talent

Developing Australia's RM sector requires attracting and retaining world-class talent. In addition to previous work[52] throughout the course of consultation for this project, the following capability gaps have been identified or confirmed:

- Readying the Australian health system to distribute, administer and follow up RM therapies;
- Increasing understanding amongst investors (see previous section for discussion);
- Embedding commercial know-how inside of our universities; and
- Manufacturing capacity.

Addressing these will require a mix of strategies, including re-training existing or training additional workforce, attracting talent from outside our current system i.e., overseas, or else monitoring in the understanding that the issue will be addressed organically as our system matures (Figure 4).

Figure 4 Strategies to attract, develop and retain talent

Train	Attract	Monitor
Clinicians Hospital pharmacy Nurses Manufacturing Investors	Commercialisation	Corporate e.g., legal, IP, contracting

Health System

Providing RM therapies in clinical settings in Australia poses a series of challenges. It has been widely observed that there is a mismatch between existing hospital and clinical capabilities and those required for delivering RM therapies. The current delivery of healthcare is designed based on drug delivery, device-based therapies and surgery. These are likely poorly suited for RM therapies.[53]

The related issues range across particular functions of the system with pharmacy capabilities and clinical expertise amongst the most pressing areas where current skillsets will need to be supplemented with additional training.

At present there is no standardised guidance on the handling of RM products provided to hospital pharmacies (particularly in vivo treatments governed by the Gene Technology Act). As a result it was reported that, in some cases, pharmacy departments have refused to receive RM products because of lack of familiarity with guidelines. Additional concerns have been cited by staff around the potential health risks associated with handling genetically modified viruses.

In addition to these issues of understanding, there are simply additional resource imposts associated with delivering new therapies that have significantly different requirements. It is likely that this will require specialised staff in key front-line roles (nurses, clinicians, pharmacy etc.).

Similarly, the current clinical workforce is not trained in RM therapies. In many cases expertise in identifying opportunities for RM therapies as well as the know-how to administer and provide follow up care are limited to individuals or otherwise non-existent.

Commercialisation

The need to increase Australia's know-how in the commercialisation of RM assets has been widely discussed. As part of their earlier reporting on the opportunity for Australia in RM, MTP Connect and LEK noted:

> "There is a shortage of commercialisation experience in the RM sector, which should change as the number of successful therapies brought to market increases. Commercialisation skills include identifying an unmet need, demonstrating proof of concept, creating the appropriate IP strategies, navigating regulatory requirements, clinical trial design and establishing strategic partnerships."

In addition to these skills, the current consultation has identified a range of additional commercial skills that, if more broadly available, would significantly increase Australia's ability to translate its research into new RM assets.

To effectively commercialise RM therapies a number of considerations must be made at the early stages of basic research. This includes understanding the future clinical requirements, production demands, and the full range of associated costs. This is particularly critical given the time intensive nature and high development costs associated with RM technologies.

Consideration of factors critical to translation and commercialisation by RM researchers is made more complicated by the range of actors involved along the RM development pathway from basic research to clinically validated therapy. This includes researchers, clinicians, biotech companies, pharmaceutical companies, manufacturers, regulators, payers, and patients.

Each of these will have a unique set of needs that should be factored in during early-stage research to increase the likelihood of successful translation of research. For example, consideration of GMP and other quality control requirements from the earliest stages increases the likelihood of translational outcomes.

At present, there is very little capability in this respect available to Australian researchers. While there are several success stories that we can point to (for example, Orthocell, Mesoblast and Cynata), the deep understanding of how to plan for commercial success in RM is not widely available. This has been highlighted recently as a broader concern across the Medical Technology, biotechnology, and Pharmaceutical sectors by MTPConnect in the *REDI Initiative Skills Gap Analysis*[54] which has identified the following gaps:

- Shortage of industry professionals with end-to-end commercialisation experience
- Ability to identify unmet market need and understand the clinical context
- Ability to secure investment, funding and/or industry
- Collaboration
- Identification of the payer and understanding of reimbursement pathways and requirements.

While these were general skills gaps across the MTP sector, they are all relevant to the discussion of RM in particular.

Manufacturing

The benefits to having a domestic RM manufacturing capability are numerous. This is true of core manufacturing capability (e.g., manufacturers) as well as for a broader, integrated supply chain and delivery network spanning clinicians, hospitals, supply chain logistics and manufacturing facilities. In the post-COVID world, this kind of sovereign capability in our medical supply chain has become an increasingly acute concern amongst government policy makers.

Access to local manufacturing capabilities will provide equitable access for Australian patients to emerging RM therapies, as well as access to early phase trials.

However, RMs require specialised GMP manufacturing capabilities and infrastructure, a highly-skilled workforce, and complex supply chains that are currently not well developed in Australia.

At present,[55] Australia has limited capability, including:

- 7 RMT GMP manufacturers with TGA licences are manufacturing for commercial supply and for clinical trials.
- 34 cleanrooms; and
- 231 full-time and 45 part-time/casual employees.

While early phase clinical trials are (0-1) do not need to be manufactured in a TGA-licenced GMP facility, these facilities are also in short supply. They are comprised of a total of 15 cleanrooms, employing 24 full-time and 10 part-time/casual employees.

The scale of this workforce pales in comparison to the scale manufacturing capability required to services RM demand across the R&D pipeline for both Australian companies and internationals.

Talent - Priority Actions

Health System Capabilities

The primary mechanism to address the shortfall in health system capabilities and prepare the health system for future RM delivery is training. In the medium term there are scalable ways to address these issues by working with groups such as the Pharmacy Board of Australia to create and disseminate standards and guidelines for the pharmacy profession. Similarly, engaging with the Medical Board of Australia to develop recognition of RM as a medical specialty may certify specialised training programs for RM in clinical settings.

This will also require working with tertiary education providers to create training material related to RM.

In the short term there are opportunities to address this through organisational change. At the Royal Prince Alfred Hospital in Sydney they have taken the approach of establishing Cell and Molecular Therapies group as a centre of excellence to the hospital and across the NSW Health system more broadly. They provide education and training for clinical, scientific and regulatory staff across the hospital and have established a deep expertise in Cell and Molecular therapies.

A centre of excellence-based approach has been shown to work in the short-term to achieve a significant uplift in capability and skills while broader capability is being is developed.

Commercialisation

There is limited exposure in Australia to the end-to-end process of successfully taking a RM product to market. This means that there is a knowledge gap around the commercialisation of RM. It is more than likely, given our relatively small footprint, that this talent at least in the short-term will need to be attracted from overseas where there are more products in market and in late phase clinical trials. This will serve a short-term need while a home-grown capability can be grown.

Manufacturing

In the short term the solution to the shortage of GMP-qualified workforce can be addressed through upskilling of workforce from other related areas of the medical sector, for example graduates with training in pathology or in pharmacy can receive supplemental training in GMP manufacturing requirements. There are already incremental gains being made in this respect through partnerships such as the UTS/SeerPharma training program, though this program does not specifically address the needs of the RM sector.

This program delivers university-accredited graduate programs in GMP which are practice-based. They are offered as postgraduate qualifications as a

- Graduate Certificate in GMP
- Graduate Diploma in GMP
- Master of GMP

The courses have been designed specifically to address unmet industry need, and units of study cover all stages of the product life cycle, from pre-clinical and clinical through to commercial manufacturing.

Developing specific RM postgraduate qualifications like these will, in the short term, provide a boost to the available workforce. However, this approach in the long-term will simply transfer workforce shortages into other areas of the health and medical system and not address the overall burden.

In the long-term, there need to be strategies in place to create additional workforce with these specialised skills. To do this, GMP skills and know-how need to be embedded within a range of relevant undergraduate programs as a core competency. Unlike the reskilling approach, this will create a pipeline of new workers. This will require deep collaboration between universities and other training providers and industry, to address the specific and evolving needs of industry.

With the emergence of government initiatives such as the Job Ready Graduates package – which provides incentives for universities to orient their courses to criteria including employability, and which also provides broader scope for the participation of non-university higher education providers to provide short-courses – there are now more opportunities and financial incentives for universities to participate in this kind of co-design approach. Brokering a coordinated national approach and framework for this should be a high priority action item for the RM Catalyst.

Appendix B – Implementation Considerations for the Strategic Roadmap

Introduction

This strategic roadmap has identified a series of priority action areas that will be the focus of a RM Catalyst Body. This section provides a brief series of options when it comes to implementing this roadmap. It is intended as indicative only, and the final decision about how to implement the plan is deferred to the Catalyst body[56].

In Figure 5 each of the priority action items from the Strategic Roadmap have been categorised against their complexity (Low, Medium and High), the timeframe to realisation (Short term, Medium Term or Long Term) and their likely impact on the Australian RM sector (Low Impact, Moderate Impact and High Impact).

Figures 6-8 propose three options for how the roadmap could be used as the basis for the RM Catalyst Body's initial focus. In Figure 6 the focus would be on the highest impact activities; in Figure 7 the focus would be on the low and medium impact items that can be delivered in a short or medium term; and in Figure 8 those which have the highest strategic value are the focus.

Finally, Table 3 provides a brief comparison of the advantages and disadvantages of the different approaches.

Figure 5 Priority actions categorised – all actions

*Note: See next page for numbered Action list

A. Attract, build and retain world-class talent

1. Advocating for increased financial and structural incentives that encourage RM operations in Australia and that can attract individuals and groups with experience in commercialising RM therapies;
2. Building stakeholder engagement and mobilising resources to facilitate training and mentoring programs across the RM value chain;
3. Supporting stakeholders to improve information sharing across RM providers, clinicians, and patients especially around successful clinical models;
4. Building activities aimed at improving health system readiness by working to embed RM in standards and guidelines and as a medical specialty

B. Collaborate across the value chain

5. Building stakeholder engagement and mobilising resources to facilitate mentoring and networking programs across the RM value chain;

6. Supporting efforts amongst stakeholders to ensure that commercial considerations are factored into early-stage research and to manufacturing capability;
7. Advocating for and contributing to policy aimed at establishing a national, coordinated approach to capability development (e.g. clinical trials, site selection, etc.).

C. Secure long-term investment in the sector

8. Encouraging investment from governments and provision of broad economic incentives for private investment including a suite of targeted financing mechanisms;
9. Mobilising private investment, via international and domestic venture capital into Australia's biotechnology sector, including increasing institutional and retail investor understanding of RM;
10. Encouraging multinational companies to engage locally, to market their products in Australia and also to provide expertise and partnering to SMEs located here;
11. Encouraging more public-private partnerships (PPPs).

D. Create a clear market access pathway that is aligned to leading global markets

12. Contributing to policy around expedited approval for RM therapies in Australia;
13. Contributing to policy discussions that seek to create definitional and decision-making alignment across the different regulatory mechanisms;
14. Advocating for Australia to have access to priority RM clinical trials;
15. Contributing to policy discussions on, and advocating for, the standardisation of international quality control processes for RM.

E. Build Australian capability across the RM value chain (early stage, pre-clinical, manufacturing, clinical, market access, patient delivery)

16. Mobilising resources, building stakeholder engagement, and advocating for mechanisms that provide researchers early access to clinical-grade cell lines;
17. Mobilising resources and advocating for a national stem-cell registry for researchers;
18. Advocating for ongoing and increased government investment in domestic manufacturing capability and infrastructure through additional post-graduate training for the current workforce and undergraduate training for future workforce and, through support of current and emerging manufacturing infrastructure;
19. Building stakeholder engagement and supporting activities aimed at improving health system readiness.

Figure 6 High-impact focus

Impact: *High*
Complexity: *Medium-High*
Estimated time to outcomes: *3 to >5yrs*
Focus: *Increasing investment*

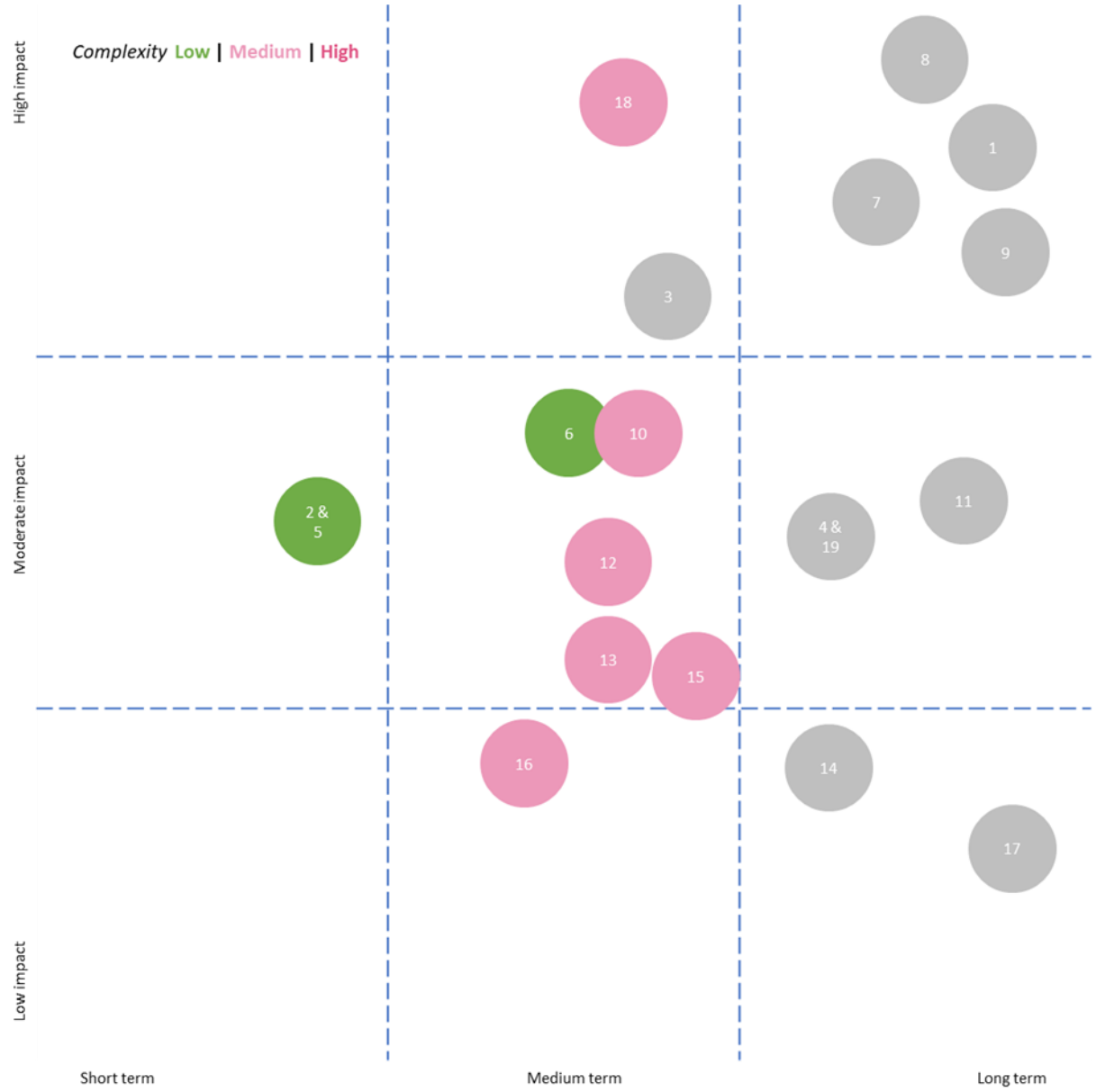
Main activities

A. Attract, build and retain world-class talent

- Advocating for increased financial and structural incentives that encourage RM operations in Australia and that can attract individuals and groups with experience in commercialising RM therapies
- Supporting stakeholders to improve information sharing across RM providers, clinicians, and patients especially around successful clinical models
- Encouraging investment from governments and provision of broad economic incentives for private investment including a suite of targeted financing mechanisms

- Mobilising private investment, via international and domestic venture capital into Australia's biotechnology sector, including increasing institutional and retail investor understanding of RM
- Advocating for ongoing and increased government investment in domestic manufacturing capability and infrastructure through additional post-graduate training for the current workforce and undergraduate training for future workforce and, through support of current and emerging manufacturing infrastructure

Figure 7 Low-hanging fruit focus



Impact: *Low-Moderate*
Complexity: *Low-Medium*
Estimated time to outcomes: *1 to 5yrs*
Focus: *Translation and commercialisation*

Main activities

- Building stakeholder engagement and mobilising resources to facilitate training and mentoring programs across the RM value chain
- Building stakeholder engagement and mobilising resources to facilitate mentoring and networking programs across the RM value chain
- Supporting efforts amongst stakeholders to ensure that commercial considerations are factored into early-stage research and to manufacturing capability
- Encouraging multinational companies to engage locally, to market their products in Australia and also to provide expertise and partnering to SMEs located here
- Encouraging more public-private partnerships (PPPs).

- Contributing to policy around expedited approval for RM therapies in Australia
- Contributing to policy discussions that seek to create definitional and decision-making alignment across the different regulatory mechanisms
- Advocating for Australia to have access to priority RM clinical trials
- Contributing to policy discussions on, and advocating for, the standardisation of international quality control processes for RM.
- Mobilising resources, building stakeholder engagement, and advocating for mechanisms that provide researchers early access to clinical-grade cell lines

Figure 8 Strategic priority focus

Impact: *Moderate-High*

Complexity: *Medium-High*

Estimated time to outcomes: *3 to >5yrs*

Focus: *Talent development and increasing investment*

Main activities

- Advocating for increased financial and structural incentives that encourage RM operations in Australia and that can attract individuals and groups with experience in commercialising RM therapies
- Building stakeholder engagement and mobilising resources to facilitate training and mentoring programs across the RM value chain
- Supporting stakeholders to improve information sharing across RM providers, clinicians, and patients especially around successful clinical models

- Building activities aimed at improving health system readiness by working to embed RM in standards and guidelines and as a medical specialty
- Encouraging investment from governments and provision of broad economic incentives for private investment including a suite of targeted financing mechanisms
- Mobilising private investment, via international and domestic venture capital into Australia's biotechnology sector, including increasing institutional and retail investor understanding of RM
- Encouraging multinational companies to engage locally, to market their products in Australia and also to provide expertise and partnering to SMEs located here
- Encouraging more public-private partnerships (PPPs)

Table 3 Comparison of implementation options

Approach	Pros	Cons
High Impact	• 'Game changing' if it succeeds • Clear objective - to drive increased investment in RM sector • Relatively broad stakeholders • Addresses some mission-critical areas e.g. manufacturing • Could probably be delivered with minimal resourcing	• Long time to demonstrated outcomes • Low control of outcomes i.e. high risk • Doesn't address major hurdles e.g. 'talent' • Most outcomes will require substantial stakeholder buy-in/system level change
Low Hanging Fruit	• Includes some short to medium term outcomes to demonstrate success and build momentum • A large number of moderate impacts • Broad set of activities representing diverse parts of the value chain • Most outcomes are controlled i.e. low risk	• Broad focus and potentially atomised effort • High degree of activity may diminish impact or else become resource intensive • No clear 'BHAGs' e.g. "*Double investment in RM in Australia by 2030*"
Strategic	• Addresses the two areas identified as major blockers and enablers, talent and investment • Mix of short-, medium- and long-term outcomes • Mix of medium and high impact outcomes	• Most outcomes will require substantial stakeholder buy-in/system level change • Low control of outcomes i.e. high risk

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Level 3, 15 Queen Street
Melbourne, VIC, 3000
www.ausbiotech.org

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AUSTRALIA'S BIOTECHNOLOGY ORGANISATION