AusBiotech
AUSTRALIA'S BIOTECHNOLOGY ORGANISATION

National Cell and Gene Manufacturing Blueprint

July 2023

Supported by

VICTORIA State Government

In partnership with

Biointelect

Contents

Disclaimer

Foreword

AusBiotech is pleased to present the National Blueprint for Cell and Gene (C&G) Manufacturing, outlining how, with a united and coordinated plan, Australia can realise the potential of its cell and gene (C&G) manufacturing future.

C&G products (which include C&G therapies) are a new generation of medical treatments that involve delivering complex biological components. These therapies can address the root cause of diseases, and single treatments are already producing long-lasting and life-changing results for patients with rare diseases and cancer. The global cell and gene industry has developed apace in recent years and is only accelerating. With nine C&G products approved in Australia [1], and the U.S. Food and Drug Administration (FDA) on track to meet its 2019 prediction of 10-20 approvals per year by 2025, the demand for the growing and diversifying suite of therapies is high. While the initial scientific challenges of C&G products have been overcome, the manufacturing and delivery requirements remain complex and diverse, with the increasing number of therapies pushing global manufacturing capabilities and capacity to the limit.

Australia's role in and response to the growth of C&G products has displayed our many strengths and identified our gaps in this complex and interconnected space – especially the opportunities for manufacturing. Without a unified and collaborative approach to expanding C&G manufacturing capabilities, we risk missing out on the benefits of C&G manufacturing to both the Australian economy and healthcare system, including for patients in desperate need of novel therapies. To fully realise the potential of C&G products for Australia, it is therefore imperative to develop a coordinated approach to manufacturing in this rapidly changing and growing space. By working together to develop the necessary infrastructure, workforce and expertise, Australia can position itself as an APAC regional leader in C&G manufacturing.

For industry, this report is an opportunity to shape the field for the future, and to build a sustainable and growing ecosystem that attracts and supports local and international operators. For governments, it's an opportunity to strategically invest and therefore maximise the socioeconomic potential of C&G manufacturing, including job creation and healthcare outcomes. Overall, this report is an opportunity to consider Australia's role in the global C&G ecosystem, and to ensure that we are best-placed as a nation to be involved in and benefit from these life-changing therapeutic approaches now and into the future.

The role, scope, and approach of this report

With the rapid and ongoing global growth of C&G products there is an opportunity for Australia to be a regional C&G hub — providing world-leading manufacturing capabilities, as well as research, clinical trials, and translational know-how. To be a destination of choice for cell and gene manufacturing, Australia requires a significant uplift in our capabilities and capacity to service the commercial opportunities available. It is crucial to develop a coordinated strategy which ensures the Australian C&G ecosystem has the capability, capacity and flexibility to respond to the rapid growth and changes in the industry. This report has been prepared to outline such a strategy.

The Blueprint involved desktop research and a two-phase consultation process involving a wide range of stakeholders from across the industry and across the nation, especially the Manufacturing Taskforce (see Table 18 and Table 19).

Consultations	Consultation commenced with a Manufacturing Taskforce Workshop to refine and develop objectives for each key category.
Objective Development	Through a national survey, the importance of objectives was rated on a 5-point scale by 33 respondents. The sum of the two highest categories 'Absolutely essential' and 'Very important' was taken to represent 'approval' of each objective. Objectives that fell below 50% approval were rejected unless sufficient justification could be provided for their inclusion.
Strategy Development	Derived from desktop research and the above objective development process, these were then refined in workshops held for each category, and through one-on-one consultations.

The *National Blueprint for Cell and Gene Manufacturing* has been undertaken between November 2022 to June 2023. AusBiotech acknowledges and thanks the Australian Medtech Manufacturing Centre (AMMC), an initiative of the Victoria State Government, for funding this important strategy, and for supporting innovation and manufacturing. This work draws on and builds upon the foundational work of the 2020-2021 AusBiotech-led Regenerative Medicines (RM) Consortium Project, and includes contributions from 32 individuals and organisations.

Executive summary

Cell and gene (C&G) products are a new frontier in medicine around the globe. They represent a ground-breaking advancement, offering single treatments that deliver complex preparations of live cells and modified genetic materials. These therapies have already demonstrated life-saving and life-changing results for patients with rare diseases and cancer, and show promise in addressing additional, more common conditions.

Given Australia's advanced manufacturing scene and healthy biotechnology ecosystem, there is an opportunity for Australia to be a regional hub in the Asia-Pacific (APAC) region, and known for leading C&G research, clinical trials, translational know-how and manufacturing capabilities. Unlocking these opportunities requires a much better understanding and coordination, while addressing the gaps in capacity and capabilities.

This *National Blueprint for C&G Manufacturing* outlines an industry-developed strategic approach for leveraging national strengths to overcome key challenges and to coordinate the growth of C&G manufacturing. By delivering critical medications to Australian and regional patients, Australia can be a leader in C&G manufacturing and drive economic growth.

Conducting this detailed research provides: (1) a vision for manufacturing C&G products in Australia; (2) outlines where we are now as a country; and (3) provides a 'blueprint' of implementation strategies for manufacturing C&G therapies locally.

The vision

To foster the development of the Australian ecosystem to a self-sustaining state, and to position Australia as a leader in the APAC region, it is crucial to establish a robust Australian capability in C&G manufacturing, prioritising quality, and reliability. In harnessing Australia's strategic location within the Asia-Pacific (APAC) region, this reach can be expanded to encompass international markets.

Where we are now

Australia possesses the foundational elements for a robust C&G manufacturing ecosystem, including promising research conducted at university and medical research institute level, clinical trial strengths, advanced hospital systems, and government and industry investment. However, further efforts are needed to expand the workforce, enhance capacity and capability for C&G exports (with a focus on the APAC region), develop manufacturing critical mass, streamline market access pathways, and invest in patient delivery.

The Blueprint addresses five key categories outlined in the *Regenerative Medicine in Australia: Strategic Roadmap for the Regenerative Medicine Sector* report from the 2021 Regenerative Medicine Consortium Project [11]: workforce skills development, long-term investment opportunities, strengthened collaboration across the value chain, capability across the value chain, and clear market access pathways aligned with leading global markets.

National objectives within each category were developed, refined, and assessed for support, before a second consultation phase was undertaken to develop tactics for achieving those objectives.

The Australian C&G manufacturing space is highly active. The 2021 *Australia's Regenerative Medicine Manufacturing Capacity & Capability* report [35] identified 11 companies with a total footprint of 3,700 m2 across 49 cleanrooms. This footprint has since grown, with five of those companies reporting facility expansions or increased capabilities and a further three new companies reporting manufacturing space.

Australia's C&G manufacturing strengths includes its high-quality talent across the pipeline, including scientific knowledge and technical skills, strong clinical trial expertise, supply chain and distribution expertise within multinationals, and within our world-class healthcare system. Established government funds for pre-clinical and clinical research and attractive incentives supports access to capital, together with promising levels of private and public investment in CGT manufacturing capability and capacity across multiple states, indicating enthusiasm for the space. C&G manufacturing is also backed by Australia's policy and regulatory strengths including education, IP protection, HREC & CTN pathways, taxation policies, and public health system price policies. A SWOT analysis of the Australian C&G manufacturing ecosystem can be found in Figure 4.

This Blueprint acknowledges the following four key challenges were identified for C&G manufacturing in Australia:

Challenge 1: Addressing critical and growing skills gaps in cell and gene manufacturing

The workforce is a critical limitation to expanding C&G manufacturing output in Australia, and the skills gap is multifaceted, requiring a range of specialised personnel with knowledge of stringent regulatory and quality requirements. The lack of available training programmes and shortage of skilled personnel are identified as barriers impeding the growth of the industry, and a strong workforce pipeline needs to be developed to bridge the skills gap and support the growth of local manufacturing.

Challenge 2: Building critical mass in Australia's cell and gene manufacturing ecosystem

The development and manufacturing of C&G products in Australia presents a significant economic opportunity, and investment across the value chain is needed to optimise the country's role in delivering these therapies efficiently and equitably. Expanding the C&G manufacturing ecosystem can generate economic benefits through job creation, innovation, and export opportunities, but ongoing assessment is required to determine the preparedness of the value chain to deliver the growing pipeline of C&G products.

Challenge 3: Optimising Australia's contributions to the C&G product pipeline

Barriers to C&G research translation include high material costs, high standards of quality required, and limited translational laboratory space and access to industry technology platforms and expertise. To develop C&G products, delivery infrastructure, and expertise, it is crucial to support clinical trial growth in Australia. Our clinical trial sector is a biopharma strength, poised to capitalise on the global rise in C&G products trials.

Challenge 4: Tracking and guiding industry growth

To achieve the sectoral vision, and to inform the scale and scope of the recommendations listed above, Australia needs to proactively monitor industry trends and communicate the implications of these developments to stakeholders. The following actions are recommended.

How we will get there

We see Australia addressing these challenges by implementing the key recommendations outlined in the strategy house (Figure 1) and further contextualised and detailed in subsequent sections of this National Blueprint.

This National Blueprint was developed from the five key categories' objectives outlined in the *Regenerative Medicine in Australia Strategic Roadmap* [11]:

- Workforce skills development, attraction and retention
- Opportunities for secure long-term investment
- Strengthened collaboration across the value chain
- Capability across the value chain
- Creation of a clear market access pathway that is aligned to leading global markets.

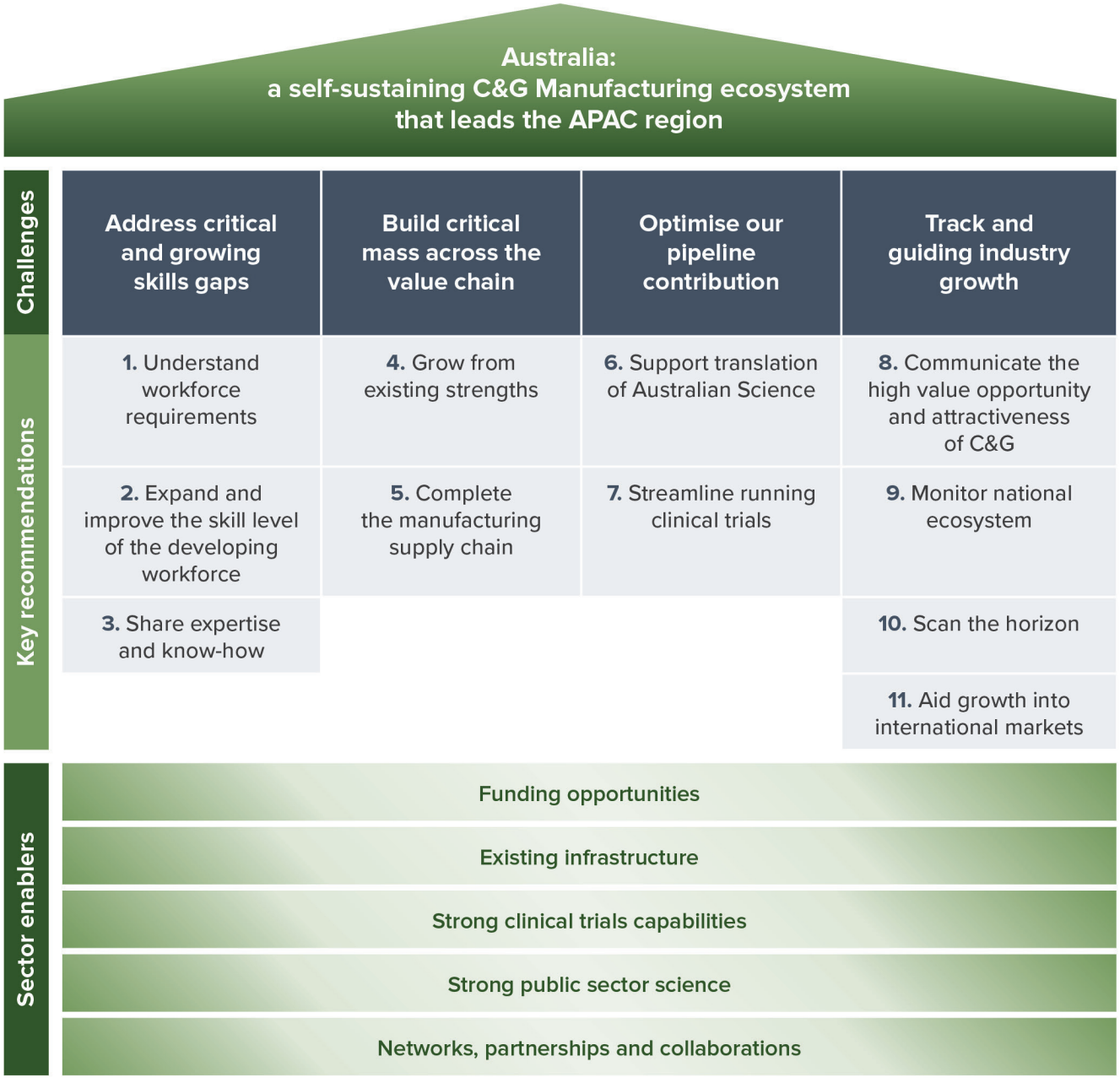


Figure 1: The strategy house offers a graphic representation of the sector enablers, challenges, and recommendations of Australia's C&G manufacturing ecosystem. Adapted from [12].

Key recommendations

The body of this report comprises the strategies and tactics that have been developed, based on the key challenges, to be implemented in the short term (within 12 months), medium term (1-3 years) or long term (3-10 years). Eleven key recommendations that are critical to growing the C&G manufacturing industry are highlighted.

Challenge 1: Addressing critical and growing skills gaps in cell and gene manufacturing

The workforce is a critical limitation to expanding C&G manufacturing output in Australia, and the skills gap is multifaceted, requiring a range of specialised personnel with knowledge of stringent regulatory and quality requirements. The lack of available training programmes and shortage of skilled personnel are identified as barriers impeding the growth of the industry, and a strong workforce pipeline needs to be developed to bridge the skills gap and support the growth of local manufacturing.

Recommendation 1 Understanding workforce requirements

Perform a nationally-funded survey of existing facilities to quantify the shortage of quality assurance (QA)/quality control (QC) and GMP cleanroom staff in Australia to guide immediate workforce development strategies. Deliver the report to State and Federal Health and Industry Ministers and Departments to demonstrate the immediate challenge and call for action for funding of training programmes. Leverage and broaden this report to clarify and quantify the broader skills gaps in the C&G manufacturing ecosystem in Australia on an ongoing basis, using a nationally representative body to establish a methodology and deliver a report that is aligned to international standards.

Recommendation 2 Expand and improve the skill level of the developing workforce

As part of an independent strategy to grow the C&G manufacturing workforce: fund the expansion of fellowships and traineeships in advanced manufacturing for C&G products, and develop an in-depth, collaborative, nationally-accredited training and development programme aligned with Australian and regional regulations – including formalised certification in advanced manufacturing for C&G products aimed at developing an industry-ready workforce pipeline.

Key recommendation 3 Share expertise and know-how

Develop a community of practice with a focus on C&G manufacturing and product development, whose initiatives include networking and educational events aimed at academics, small and large product developers, and CDMOs. A nationally representative body should review local and international communities of practice to identify and support the implementation of ‘gold standard’ approaches to facilitate collaboration and the exchange of knowledge and expertise between stakeholders across the value chain.

Challenge 2: Building critical mass in Australia's cell and gene manufacturing ecosystem

The development and manufacturing of C&G products in Australia presents a significant economic opportunity, and investment across the value chain is needed to optimise the country's role in delivering these therapies efficiently and equitably. Expanding the C&G manufacturing ecosystem can generate economic benefits through job creation, innovation, and export opportunities — but ongoing assessment is required to determine the preparedness of the value chain to deliver the growing pipeline of C&G products.

Recommendation 4 Grow from existing strengths

Leverage Australia's existing C&G manufacturing infrastructure to increase the capacity for manufacturing commercial product. Develop flexible biomanufacturing facilities with open-concept cleanroom layouts to minimise the risk of redundancy. Site selection and expansion considerations should include: (1) prioritise expansion of existing facilities over construction of new ones; (2) leverage state-specific expertise where possible; (3) contribute to strategic and equitable distribution of C&G products around the nation; (4) account for product-specific requirements. In order to avoid duplication, this expansion would require a capabilities audit.

Recommendation 5 Complete the manufacturing supply chain

Optimise nation-wide delivery of C&G products in clinical practice across Australia. Identify and invest in candidate hospitals for concentrated delivery of C&G products and develop optimal logistical solutions to delivering C&G products around Australia and to relevant international markets. Prioritise the expansion and upgrading of C&G-capable warehousing infrastructure, to minimise time-to-treatment for Australian patients and underpin the C&G manufacturing ecosystem.

Challenge 3: Optimising Australia's contributions to the C&G product pipeline

Barriers to C&G research translation include high material costs, high standards of quality required, and limited translational laboratory space and access to industry technology platforms and expertise. To develop C&G products, delivery infrastructure, and expertise, it is crucial to support clinical trial growth in Australia. Our clinical trial sector is a biopharma strength, poised to capitalise on the global rise in C&G products trials.

Recommendation 6 Support translation of Australian science

Create and improve access to local translational capabilities, including (1) GMP-certified clinical-scale laboratories, and (2) NATA GLP-accredited translational laboratories equipped for QA process development and QC testing standardisation and validation; ideally these would be incorporated into the upgraded facilities described in key recommendation 5.

Recommendation 7 Streamline running of clinical trials

Advocate to The Royal Australasian College of Physicians (RACP) and The Royal College of Pathologists of Australasia (RCPA) to establish 'Cell and Gene Therapy' Special Interest Group (SIG). The SIG will develop public health policy position papers to boost the clinical side of manufacturing, including (1) outlining minimum requirements for questions that an ethics committee must ask when reviewing a C&G clinical trial, including GMP requirements, (2) outlining model criteria for GMP manufacturing of C&G products, and (3) the need for a specialised committee experienced in the science of C&G products to support HREC in the assessment of applications and improve clinical trial establishment processes.

Challenge 4: Tracking and guiding industry growth

Australia has the potential to become a C&G manufacturing hub for the Asia-Pacific region. To achieve this, and to inform the scale and scope of the recommendations listed above, Australia needs to proactively monitor industry trends and communicate the implications of these developments to stakeholders. The following actions are recommended:

Recommendation 8 Communicate the high value opportunity and attractiveness of C&G products

Establish and/or extend this project's Manufacturing Taskforce to create a 'Cell and Gene Expert Advisory Manufacturing Group' (CGEAMG) through collaboration between nationally representative bodies across the value chain to develop and deliver tailored communication strategies as a united front to key ecosystem stakeholders. Develop a comprehensive communications strategy to (1) highlight the benefits of expanding C&G manufacturing linked to international practice in Australia to state and federal governments and (2) highlight the attractiveness of undertaking C&G manufacturing in Australia to foreign players, with the aim in each case of acquiring support for investment in ecosystem expansion.

Recommendation 9 Monitor national ecosystem growth

Conduct regular audits of Australia's C&G manufacturing capability, leveraging the Australia's Regenerative Medicine Manufacturing Capacity & Capability Report and international examples. Outline physical size of facilities (distinguishing between translation capabilities and clinical and commercial manufacturing spaces), the nature of TGA licencing, experience and expertise with technology platforms, and accessibility.

Recommendation 10 Scan the horizon

Thoroughly assess the global C&G product pipeline by regularly updating Australia's Regenerative Medicine Global Pipeline Tracker. To assess the demand for C&G manufacturing, the report should be expanded to include dosage, treatment regimen, and estimates of patient numbers (including progression of therapies through treatment lines) for each indication. This information will identify the need, type, and scale of potential opportunities for investment in local manufacturing, enabling governments and local and multinational product developers to perform market assessments that inform developments in workforce and manufacturing capacity and capability across the value chain.

Recommendation 11 Aid growth into international markets

To inform strategic positioning and potential for export, a nationally representative body should conduct a thorough assessment of key international C&G markets (e.g., Singapore, Japan, Korea). The assessment should consider specific factors such as regulation, reimbursement, healthcare system preparedness, onshore manufacturing capacity, and the competitive landscape in each market.

The vision

The vision to foster the development of the Australian ecosystem to a self-sustaining state, and to position Australia as a leader in the APAC region, it is crucial to establish a robust Australian capability in C&G manufacturing; prioritising quality, and reliability. In harnessing Australia's strategic location within the Asia-Pacific (APAC) region, this reach can be expanded to encompass international markets.

This capability should be actively promoted to two key stakeholders:

1. **International product developers/CDMOs:** Collaborating with these companies can strategically enhance our infrastructure while supporting local research and development (R&D). This can be achieved through government-funded industry-operated facilities with the capability and capacity to support the whole product development pipeline; from translation and first-in-human clinical trials, early to late phase clinical trials, through to commercial product supply.

2. **Foreign markets, particularly in the APAC region:** Sovereign manufacturing capabilities should be scaled to supply regional markets and expertise in accessing and navigating these markets must be developed. This is in recognition that Australia's healthcare population is small (~1% of global) and a larger market opportunity must be realised to ensure a self-sufficient C&G ecosystem.

The complexity and interdependency of the various C&G manufacturing processes highlights the need for a collective national approach.

Cell and gene manufacturing – where are we now?

C&G products have already reached the clinic in Australia, with nine approved therapies and 153 ongoing clinical trials [2]. Significant strides in this space include: regulatory successes of local developers including PYC Therapeutics and CSL [3, 4], commitments to increase manufacturing capability and capacity [5], and the attraction of international investment into the local ecosystem [6, 7], which has been further supported by substantial Federal government investment (Medical Research Future Fund, National Reconstruction Fund, Modern Manufacturing Initiative) [8, 9, 10]. Despite this great progress, these examples constitute individual parts of a finite and interdependent ecosystem. Expanding sovereign C&G manufacturing capabilities and capacity has the potential to cultivate an industry that improves Australian patients' access to these novel therapies, and exports high-value medical goods to regional markets.

This National Blueprint was developed from the five key categories' objectives outlined in the *Regenerative Medicine in Australia Strategic Roadmap* [11]:

- Workforce skills development, attraction and retention
- Opportunities for secure long-term investment
- Strengthened collaboration across the value chain
- Capability across the value chain
- Creation of a clear market access pathway that is aligned to leading global markets.

Cell and gene context

Cell and gene (C&G) products are a rapidly growing field that holds immense potential to revolutionise healthcare. By addressing the limitations of some conventional therapeutic approaches, C&G products offer promising treatments to previously unaddressed conditions including rare genetic diseases, cancers, and chronic diseases.

The transformative power of C&G products can be highlighted by the case of Emily Whitehead:

emily whitehead foundation
activate the cure for childhood cancer

Emily was a paediatric patient who had exhausted all treatment options following her diagnosis with acute lymphoblastic leukaemia (ALL) and was expected to die within months. However, since becoming the first paediatric patient to receive chimeric antigen receptor (CAR) T-cell therapy under a Phase 1 clinical trial, Emily has remained cancer free for over 10 years [98].

In vivo gene therapies also offer incredible results, providing curative treatments for devastating genetic diseases without effective treatments – such as Zolgensma for infants with spinal muscular atrophy (SMA). These treatments are high value and high cost, with Kymriah costing in the order of $460,000 per patient, and Zolgensma around $2.5 million [13]

C&G products have well and truly arrived in the clinic both globally and in Australia, with nine approved by the TGA since 2018. CAR-T cell therapies have experienced significant growth with three CAR-T cell therapies now approved in Australia [2], resulting in treatment of over 100 Australian patients [14]. Extraordinary developments have also been made in mRNA-based technology, which offer great potential for C&G products [15]. This growth is just the beginning, with many more C&G products approved in the EU (19 C&G products) [16] and US (28 C&G products) [17] and a robust global pipeline of products in development – including 2,200 active clinical trials [18] and up to 51 C&G product launches predicted in the USA for 2024 [19]. This growth in novel therapies has led to demand across the C&G value chain outstripping supply in the availability of therapies, with global shortages of crucial raw materials including plasmids and viral vectors [20], and bottlenecks in manufacturing capacity [21].

Australia is poised to reap substantial benefits from developing sovereign cell and gene (C&G) manufacturing capabilities. Beyond ensuring Australian patients have early access to therapies, economic value can be created through the production of therapies, as well as from the creation of jobs both directly (cleanroom personnel) and indirectly (supply chain, logistics, construction, engineers, administrative supports) related to C&G manufacturing [22]. Additionally, expanding C&G manufacturing can strengthen the Australian economy by attracting foreign investment and generating export opportunities. Developing Australia's C&G manufacturing ecosystem is also beneficial to the broader research sector and medicine development industry, including boosting our strong academic and clinical trials systems by fostering innovation and commercialisation, and providing access to capital, infrastructure and expertise.

The global C&G manufacturing market is valued at USD 15.1 billion in 2022 and forecast to grow at a compound annual growth rate (CAGR) of 19.6% between 2023 and 2028 [23]. Australia can leverage this industry growth to provide valuable contributions to both the Australian economy and healthcare system. Sovereign C&G manufacturing can improve and accelerate the delivery of C&G products to Australian patients while building resilience for the complex logistics of the C&G supply chain, distribution and clinical delivery within hospitals, pharmacies, and other clinical settings. This will further support Australian's access to innovative clinical trials and provide Australian patients with faster and more equitable access to life-changing therapies [24]. Expanding domestic manufacturing capabilities will drive job creation both now and, into the future, develop a larger highly skilled workforce, establish new export markets and fuel foreign investment as a manufacturing hub for the Asia-Pacific (APAC) region.

To attain these benefits, we face challenges associated with the technically complex production and delivery of C&G products, whose manufacturing requirements differ starkly from typical pharmaceutical processes [25]. C&G manufacturing requires a highly specialised workforce, state-of-the-art infrastructure

and unique logistical requirements [26]. A concerted effort and considerable government investment is necessary to overcome these challenges and enable Australia to leverage the significant opportunity that C&G manufacturing presents.

Cell and gene definitions

This report considers "cell and gene (C&G) manufacturing" to comprise processes that generate C&G products, which include patient-ready cell and gene therapies and/or key biologic components of C&G therapies, where "C&G therapies" fit the following definitions adapted from the 2021 *Regenerative Medicine Value Chain* report [24].

Gene therapy: involves the introduction, removal, or change in the content of a patient's genetic code, with the goal of treating or curing a disease. "Gene therapy" as used in this report is delivered in vivo (where the genetic modification occurs inside the patient). Gene therapies are delivered to specific cells of interest and include gene transfer (also known as gene replacement) and genome editing.

Cell therapy: is the transfer of intact, live cells into a patient. It may be used to replace cells that are missing or non-functional, or to provide cells that have improved or extended functionality. Cell therapies may include cells that originate from the patient themselves (autologous), or from a human (allogeneic) or animal (xenogeneic) donor. This can include genetically modified cell therapies, which can also accurately be referred to as "ex vivo gene therapies".

C&G manufacturing processes may also be capable of generating a broader range of products that do not necessarily fit within these definitions, such as mRNA vaccines.

Cell and gene manufacturing

C&G products comprise a wide range of products that can involve several components, such as various types of cells, viral vectors, and segments of genetic material (DNA or RNA). This National Blueprint is designed to be responsive to the developing C&G product pipeline and so is largely product-agnostic.

C&G products and their components can be produced by a wide variety of highly complex "manufacturing specialties" that generate different types of products and have been summarised in Figure 2 as:

- Cell donors
- Cell cultures and banking
- Genetically modified (GM) cell manufacturing
- Oligonucleotide manufacturing
- Enzymatic RNA manufacturing
- Plasmid DNA (pDNA) manufacturing
- Viral vector manufacturing

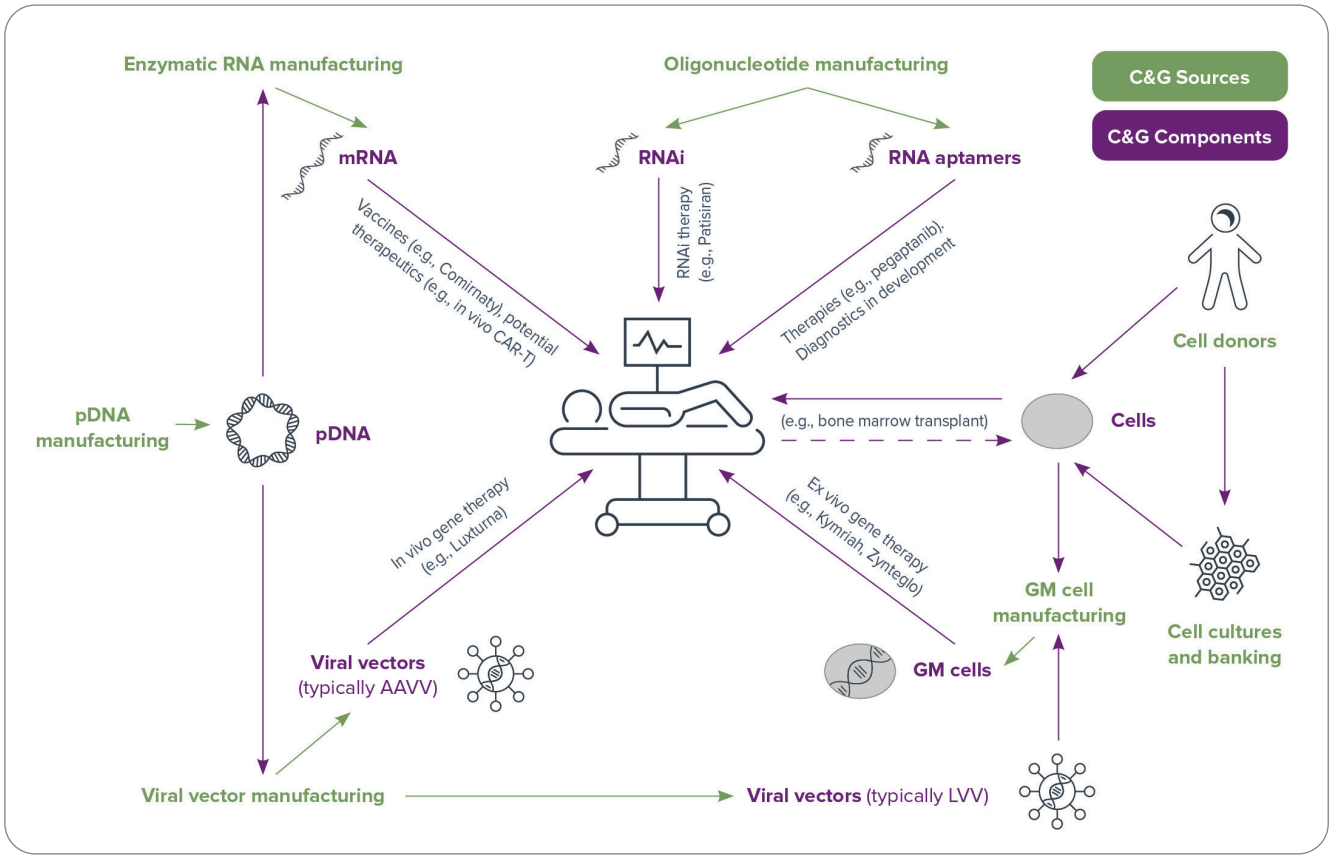


Figure 2: Cell and gene manufacturing approaches (green) that are each individually complex, also form a network of inter-related processes and C&G components (purple), producing therapies (arrows leading to the central symbolic patient) and/or contributing to the manufacture of increasingly complex therapies. Highly complex therapeutic approaches in development (e.g., CRISPR) and various cell, RNA, and viral vector subtypes have been omitted for simplicity. AAVV, adeno-associated viral vectors; C&G, cell and gene; GM, genetically modified; LVV, lentiviral vectors; mRNA, messenger RNA; pDNA, plasmid deoxyribonucleic acid; RNA, ribonucleic acid; RNAi, RNA interference technologies.

The manufacturing processes, the products, and their relationships to each other depicted in Figure 2 have been greatly simplified. Each manufacturing speciality involves deep expertise, and the relationships between the specialities, their outputs, and the range of approved and developing products is highly complex, with many therapies requiring coordination of several manufacturing specialities and complex logistical activities. Furthermore, complex logistics are involved due to the short shelf-life and temperature- and transport-sensitivity of many C&G products and components, especially those containing live cells.

Table 2: Relative challenges of manufacturing autologous CAR-T, in vivo gene therapies, and mRNA

Challenge	CAR-T	In vivo gene therapy	mRNA
Relative manufacturing complexity	High	Moderate	Low
Degree of logistical precision required	High	Low (depending on extent of personalisation)	Moderate (due to unstable nature of product)

Relative complexity of Quality Assurance (QA)/ Quality Control (QC)	High	Moderate	Moderate
Challenge with standardisation	High	Moderate	Low
Batch manufacturing time	High	Low	Low
Requirement of cold chain for storage and delivery	High	Low	High [28]
Challenge with scalability	N/A (for autologous) High (for allogeneic)	Moderate	Low

The complexity and interdependency of the various C&G manufacturing processes highlights the need for a collective national approach. A pressing example of this is the supply chain for the rapidly growing CAR-T sector, which includes the coordination of three manufacturing specialties – cells, viral vectors, and pDNA – the last of which is also a bottleneck for mRNA manufacturing [29] and is being developed into stand-alone therapies [30].

This issue continues to be exacerbated. In April of this year (2023), the Alliance for Regenerative Medicine (ARM) highlighted CRISPR therapies as a "Trend to watch" [18], with the upcoming regulatory review of "exa-cel" (Vertex Pharmaceuticals) the vanguard of 100+ ongoing clinical trials in CRISPR. Exa-cel involves separately manufacturing five components: (1) a Cas9 protein (a conventional biologic not included in Figure 2) and (2) a "guide RNA" (oligonucleotide), are combined in (3) pDNA-derived (4) adeno-associated viral vectors which deliver those components into (5) isolated patient cells [31].

The convergence of these multiple components into a unified product set to reach US clinics later this year, along with the remarkable growth in CAR-T and mRNA sectors, underscores the increasingly interconnected nature of C&G manufacturing. Consequently, there is an urgent need for a comprehensive strategy to address this evolving landscape. Australian patients demand access to novel and increasingly complex life-changing therapies, it is crucial for the local manufacturing ecosystem to possess the necessary capabilities, capacities, and adaptability to meet these demands.

The cell and gene manufacturing ecosystem

C&G product development involves a large investment of time and resources, taking on average 8 to 14 years and USD$0.5-$1 billion per product [32]. C&G product development requires manufacturing at high quality and increasing scale, which involves a wide range of stakeholders. The contributions of these stakeholders along the C&G product development pathway are illustrated in Figure 3 with their roles are summarised below, and provided in more detail throughout The *Regenerative Medicine Value Chain Report* [24].

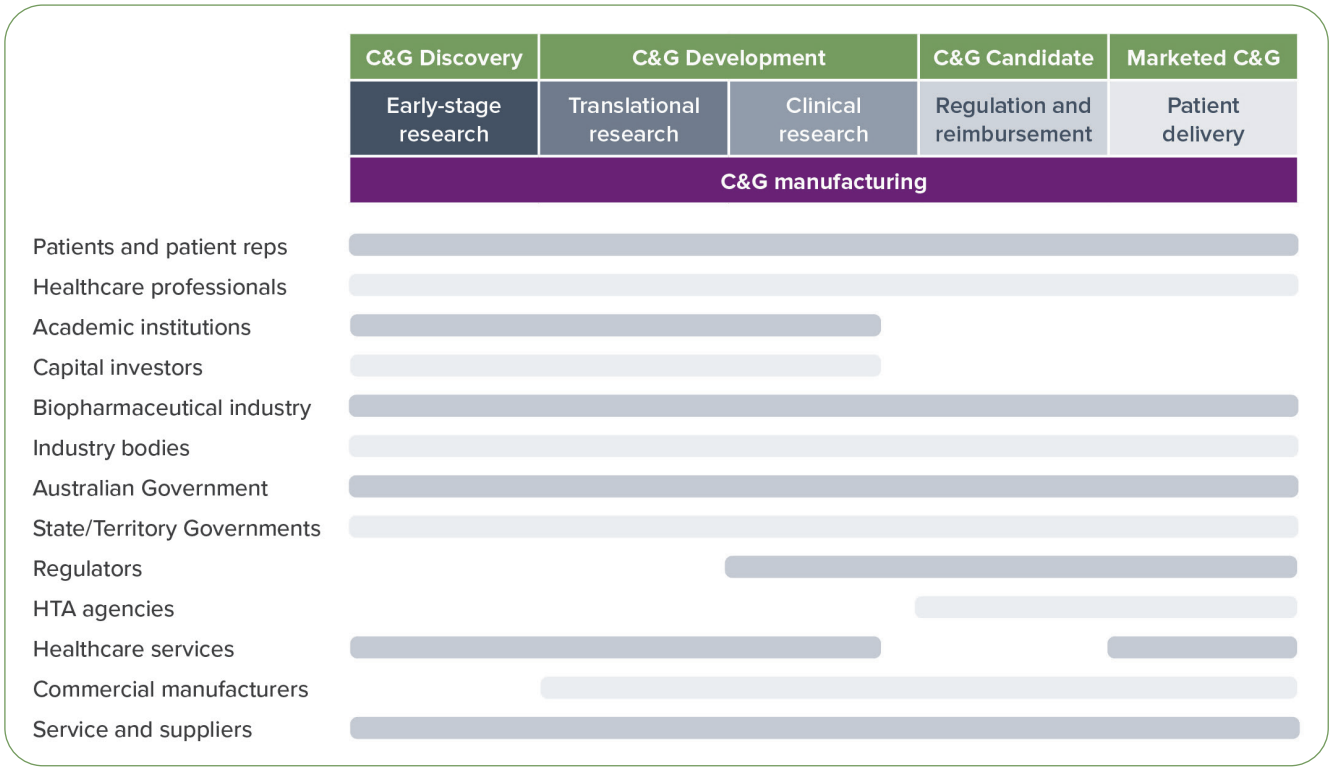


Figure 3: C&G value chain and stakeholder overview, adapted from [24]

Novel C&G products arise from clinician and academic groups innovating at a small-scale. Their primary focus lies in characterising their product and validating their approach. These groups may then sell their intellectual property (IP) or transform into biotechnology companies – pulling in diverse expertise including product development, chemistry manufacturing and controls (CMC), and regulatory as they expand the scale of production and embark on clinical trials. During this transition, their key objectives shift towards achieving rapid market entry, ensuring product consistency and reproducibility, and further amplifying their manufacturing capabilities.

A variety of external service providers can facilitate this progression, especially contract development and manufacturing organisations (CDMOs), who provide expertise, CMC capabilities, and process development services for C&G manufacturing [e.g., AcuraBio, Auspep Holdings, BioCina, Cell Therapies Pty Ltd, Lonza, Westmead Viral Vector Manufacturing Facility], and contract research organisations (CROs), who provide assistance running clinical trials [33]. Other groups may offer services in technical consulting, skills to support product development and commercialisation, or workforce training and skills development (e.g., Biointelect, Centre for Biopharmaceutical Excellence, Cicada Innovations, CCRM Australia, BioCurate, NSW Stem Cell Network).

As C&G product developers progress and broaden their portfolio, they gain valuable experience, resources, and proficiency across the entire product development pipeline. This accumulation of expertise empowers them to potentially establish in-house capabilities for the development and manufacturing of these products. Consequently, these large entities (e.g., Moderna, Novartis, and Kite Pharma), frequently transition towards acquiring assets from smaller developers, ushering them into the market. With this, their primary emphasis shifts towards market expansion, profitability, risk mitigation, and ensuring the security and stability of their supply chain.

Every product developer and manufacturer faces the choice of whether to pursue in-house manufacturing, collaborate with a CDMO, or opt for out-licensing. In some instances, prominent players may even cultivate sufficient manufacturing capabilities to spin-off into a CDMO [34]. Such decisions are multifaceted and hinge on various factors including the product's characteristics, the company's

capabilities, and their strategic approach. Additionally, large product developers might exclusively seek manufacturing partners or, as exemplified by Moderna's collaboration with the State of Victoria [6], invest in ecosystem development. Attracting significant industry players who contribute to ecosystem building becomes indispensable in achieving critical mass and fostering a thriving manufacturing landscape.

Consultations with multinational organisations (MNOs) have unveiled crucial elements that serve as incentives for their investment in local manufacturing. These encompass the presence of transparent reimbursement and regulatory processes, substantial investments in clinical trials, medical science infrastructure, bipartisan governmental support, the opportunity to establish a significant presence in the southern hemisphere, and a thriving and cohesive ecosystem.

This Blueprint therefore focuses on supporting and leveraging those strengths by directly addressing translational capabilities and clinical-scale manufacturing, and building the ecosystem to attract commercial scale manufacturing.

Australian C&G manufacturing activity

The Australian C&G manufacturing space is highly active. The 2021 Catalyst Manufacturing report [35] identified 11 companies with a total footprint of 3,700 m^2 across 49 cleanrooms. This footprint has since grown, with five of those companies reporting facility expansions or increased capabilities and a further three new companies reporting manufacturing space.

The C&G manufacturing sector has also received support from federal and state governments, including:

- The nascent $15 billion National Reconstruction Fund includes dedicated support allocated for investment in medical manufacturing and advanced manufacturing, and has been described as the first step in Prime Minister Albanese's election promise "to revive our ability to make world-class products" [9].
- The $1.3 billion Modern Manufacturing Initiative included "Medical Products" as one of six National Manufacturing Priorities [10].
- The inclusion of "Advanced manufacturing and materials technologies" and "Biotechnologies" on the recently updated Critical Technologies in National Interest list [36]
- The Medical Research Futures Fund (MRFF) [8]
  - Stem Cell Therapies Mission ($150 million)
  - Assessment of High-Cost Gene Treatments and Digital Health Interventions Grant Opportunity
  - Genomics Health Futures Mission ($500.1 million)
  - Medical Research Commercialisation initiative ($450 million)
  - National Critical Research Infrastructure initiative ($650 million)
  - Researcher Exchange and Development within Industry (REDI) initiative ($32 million)

It is encouraging for industry to see the ongoing recognition of medical research and manufacturing in Australia's future, and there is an opportunity to continue to leverage these and upcoming initiatives in our mission to become an APAC leader for C&G manufacturing.

Recent notable C&G manufacturing highlights include:

- As part of the Federal Government investment into the Peter MacCallum Centre of Excellence in

Cellular Immunotherapies [40], an expansion of Cell Therapies Pty Ltd's manufacturing facility included increased translational and early-phase clinical trial manufacturing capacity and three new large-scale manufacturing suits for late-phase and/or commercial product manufacturing with capacity to supply innovative cell-based therapies to Australian and regional (APAC) patients. [41].

- A Viral Vector Manufacturing Facility capable of delivering commercial product is being developed in the Westmead Health and Innovation District as a collaboration between Sydney Children's Hospitals Network, Children's Medical Research Institute, and Western Sydney Local Health District and the NSW Government [5, 42].
- Active involvement of several CDMOs in the field, contributing to product development and manufacturing, including recent progress in pDNA capabilities by companies like BioCina and Acura Bio [43].
- Various investments in the RNA space such as such as partnerships between Victoria and Moderna [6]; NSW and Myeloid Therapeutics [37]; Queensland and Sanofi [38]; and the collaboration between The University of Adelaide, BioCina, and Cytiva [39].

While acknowledging the impressive progress made in these areas, it's important to note that these developments have mainly occurred in a product-specific or institution-specific manner – without a clear link to a broader national strategy. It's crucial to leverage their strengths by integrating them into a comprehensive national framework.

Australia's export market opportunities

The activities outlined above, along with well-recognised strengths in academia and clinical trials [24] highlight that Australia is well-placed to deliver on C&G manufacturing. However, as the industry matures, we are faced with the challenge of building our manufacturing ecosystem in the context of an initially modest local market.

Although C&G products are high-cost, currently approved treatments target relatively rare conditions that have not yet produced a large market from our population of 26.2 million [44, 45]. However, a wide range of C&G products are under development and many of those address increasingly common diseases. Recent modelling has indicated that CAR-T treatment for multiple myeloma may treat 886 Australian patients per year, a significant increase on existing CAR-T options [47]. Furthermore, existing therapies are being made available to increasingly greater numbers of patients as they become progressively accepted into earlier lines of treatment [47]. Despite this massive growth on the horizon, supplying only Australian patients will limit industry growth in the critical short term.

To attract investment from international companies and cultivate regionally competitive capabilities, Australia must proactively expand its onshore capabilities and capacity to facilitate an export market to the APAC region [45]. We have a unique opportunity to tap into larger developing Asian markets like Japan (population over 125 million) [49] and South Korea (population over 50 million) [50]. The APAC region features a rapidly growing C&G clinical trials sector, with notable research initiatives being reported in countries such as Japan and China [51] and the 848 active trials [18] reflecting a region with growing interest and clinical capability in C&G products.

Accessing this opportunity is not without challenges. While one local manufacturer (Cell Therapies Pty Ltd) has regulatory experience with Asian markets [including undergoing audits by international regulators such as the PMDA (Japan), MFDS (South Korea), and HAS (Singapore) [41]], broader

understanding of the intricacies of trade and market access for C&G products will be required to develop a strong export market. The competitive landscape in the APAC region is also poorly understood, despite clear signs of growth – particularly in Singapore as described in the case study below [52, 53, 54, 55].

CASE STUDY: Singapore C&G ecosystem

Singapore has emerged as a key regional player in the CGT field, building a strong ecosystem and positioning itself as a regional hub for the sector. In 2020, Singapore founded the Advanced Cell Therapy and Research Institute (ACTRIS), which undertakes workforce training and regulatory facilitation, while also including process development and manufacturing facilities [115]

1. **Regulatory system:** Through their Health Sciences Authority (HSA), Singapore has recently updated their regulatory approach to C&G products [or "cell, tissue or gene therapy (CTGTP)], through the "Health Products (Cell, Tissue and Gene Therapy Products) Regulations 2021", stipulating provisions related to manufacturing, import and export certifications of CTGTPs [113, 114].
2. **Workforce:** ACTRIS features a training laboratory to allow trainees to learn hands-on skills in GMP manufacturing [115], and also conduct a range of education and training programmes [116].
3. **Manufacturing infrastructure:** ACTRIS's suite of cell therapy services is expected to be fully operational by Q3 2023. While detailed information could not be found, this facility includes a GMP facility with four manufacturing suites and a QC laboratory, and a Good Tissue Practice (GTP) facility, both of which are accredited by a suite of international organisations (PIC/S, HAS, JACIE (Joint Accreditation Committee ISCT-Europe & EBMT), FACT) [115].

   Singapore have also boosted their potential allogeneic cell therapy and biologics manufacturing capacity with the CDMO Lonza expanding their facilities to include 26,400m^2 for biologics [117, 118].

These examples illustrate the active competition in this space and highlight the urgency of investment in Australia if we are to be capture value in the region.

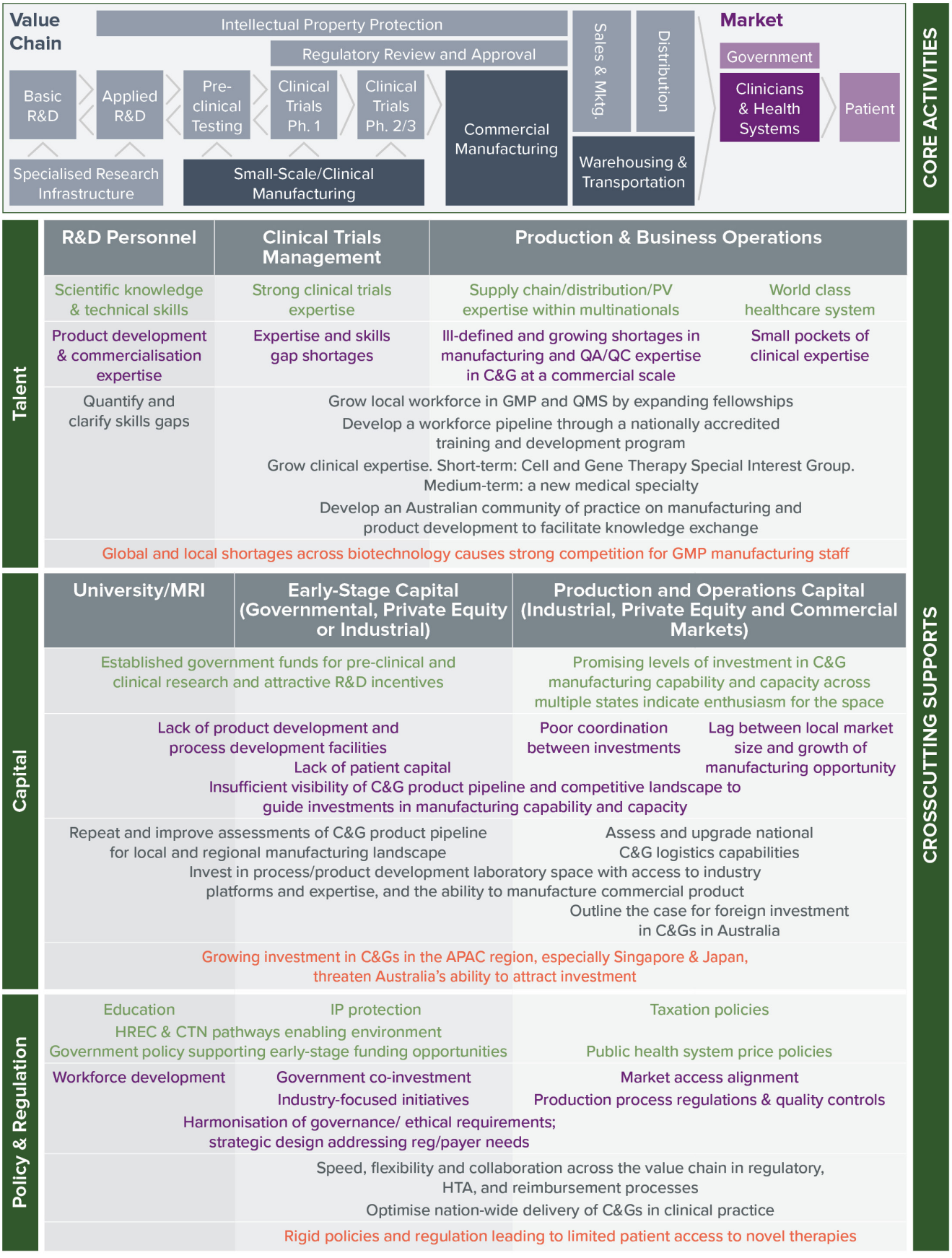


Figure 4: A SWOT analysis of the Australian C&G manufacturing ecosystem. Adapted with permission from Pfizer and TEConomy partners.

Strategy to address key challenges in C&G manufacturing

Challenge 1: Addressing critical and growing skills gaps in cell and gene manufacturing

Workforce has repeatedly been highlighted as a critical and growing limitation to expanding C&G manufacturing output both locally [56] and globally [57, 58]. For example, a report by the Cell and Gene Therapy Catapult predicted that available roles in C&G manufacturing in the UK are expected to more than double to 10,000 by 2026. This critical skills gap is multifaceted and draws parallels with advanced manufacturing more broadly, where skills gaps have presented a persistent barrier to expansion, despite Australia continuing to invest in developing a competitive advantage [59].

C&G manufacturing requires a range of highly-specialised and trained personnel with an understanding of the stringent regulatory and quality requirements which underpin the industry. These GMP standards are of critical importance for C&G manufacturing since these products have limited shelf life, strict temperature requirements, sensitivity to contamination, and require sample collection and distribution due to their personalised nature [60]. The need for cleanroom staff is exacerbated due to this and because C&G manufacturing employs novel processes that rely on manual manufacturing requirements to a greater extent than pharmaceutical manufacturing of small molecules or biologics, which have well established techniques with increasing automation [61].

Developing a sufficient number of GMP-competent experts and workers will be vital to achieving the government's ambition and expanding the production capability of novel medical products in Australia. An appropriately skilled workforce will boost Australia's sovereign supply chain capabilities and reputation in the global market as a manufacturer of advanced medical products [60].

The urgent lack of GMP-qualified cleanroom staff was reinforced in consultations:

> "There is a crisis in Australia... We should be having a very rich pipeline from high school to TAFEs to universities, with training modules across all stages of career to grow into a lifelong space across all of the value chain."

Consulted companies reported that the dearth of cleanroom personnel has seen them resort to piecemeal approaches to finding GMP manufacturing staff from a range of sources, including laboratory scientists with or without PhDs, and pathology staff. However, these areas are themselves not well-staffed, and many recruits lack knowledge and training in fundamental concepts of GMP manufacturing.

Furthermore, a lack of targeted training programmes providing baseline training has led to recruits being trained in-house by industry experts, who may not have a strong background in education or training [60]. This culminates in a variety of sub-optimally developed and delivered training processes being delivered by people with limited experience to recruits that often arrive with low levels of basic experience and education in the core tenets of commercial scale C&G manufacturing. Meanwhile, vocational facilities are restricted from filling the gap as they lack expertise and understanding of the industry staffing requirements, which differ markedly from the requirements of academic laboratories.

Beyond GMP-qualified cleanroom staff, the consultations highlighted the need for C&G experience in supporting workforces including architecture, engineering, construction, maintenance, QC testing, process support, clinical decision-support, therapy delivery, legal, risk-sharing, and patient after-care regulatory, genetic counselling, and logistics.

To fully realise the potential of C&G manufacturing in Australia, it is essential to develop a strong workforce pipeline. The current skills gaps need to be understood and prioritised to ensure workforce needs are aligned with the therapeutic pipeline (see strategy 23), helping to bridge the skills gap and support both the growth of local manufacturing, and the attractiveness for international companies to invest in Australia's C&G manufacturing sector. Additionally, Australia must develop a longer-term workforce strategy, including training and education programmes, to drive sustainable growth in local C&G workforce.

Approach 1: Assess current and future workforce requirements

Issues

Although Australian workforce reports have been released, they have limited suitability to guide workforce expansion. The REDI skills gap reports [60] were qualitative and the relevant Consortium report [56] focused on two types of staff: cleanroom manufacturing staff, and quality control and assessment staff at Australian manufacturing facilities. International reports [1, 58] have been quantitative and more comprehensive: assessing staff in R&D, process development, analytical development, supply chain, regulatory affairs, clinical trials and support services (such as HR/learning & development, finance/payroll, IT, communications/marketing etc).

Significant staffing shortages have been anecdotally reported in a wide range of areas across the Australian cell and gene ecosystem [60] – quantification of those specific shortages would more allow alignment of workforce development with pipeline growth.

Strategy

Prioritise quantification of urgent skills gaps before improving and regularly updating local skills gap analyses based on learnings from international examples, to resolve the most urgent skills gaps in the short-term and guide development of a strong workforce pipeline.

Table 3: Objectives and tactics for assessing current and future workforce requirements

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
1. Urgently quantify the national shortage of QA/QC and GMP cleanroom staff to guide short-term workforce development strategies.	**1.1.** A nationally representative body to fund a survey of existing facilities to quantify the existing challenge and impact to progress of manufacturing plans.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Attract, build and retain world-class talent
	1.2. Deliver the report to state and federal Health and Industry Ministers and their departments, demonstrating immediate challenge with a call for action to fund the training programmes described in the subsequent section.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Attract, build and retain world-class talent
2. Clarify and quantify skills gaps in the cell and gene manufacturing ecosystem on an ongoing basis – expanding on those identified in the MTPConnect REDI Initiative Skills Gap Analysis [60].	**2.1.** Establish and execute a methodology for quantifying the relevant workforce available and required in the Australian industry. Deliver a report that is aligned to international standards (e.g., 2021 UK Cell and Gene Therapy Skills Demand Survey Report and 2023 ARM Workforce Gap Analysis).	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Attract, build and retain world-class talent
	2.2. Repeat skills gap analyses on a regular basis (e.g., every two years) to be determined by the rate of change of positions with the most rapidly changing supply/demand.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Attract, build and retain world-class talent

Approach 2: Mature the cell and gene manufacturing workforce

Issues

A wide range of shortages have been reported across the cell and gene manufacturing workforce, especially in GMP manufacturing, QA and QC. However, the scale and scope of these shortages have not been well-captured in Australia.

Staffing gaps are also expected to expand in both scale and nature across this globally growing industry. Examples of this include the shortage of regulatory staff highlighted by the FDA's newly formed 'super' Office of Therapeutic Products and their plan to increase staffing numbers by 33% over the next five years [62], as well as a shortage of genetic counsellors (with fewer than 8,000 worldwide [63]) and QA and QC technicians [57].

While vocational training institutes are being developed for manufacturing staff [64, 65], they are not expected to meet growing requirements, and without industry input and standardisation of these courses companies will run the risk that potential recruits have been inadequately trained. Furthermore, once staff have been hired and trained, companies report high turnover due to (1) poaching (2) staff leaving the industry due to the lack of career paths and the repetitive and detailed nature of the work.

Critical shortages will be exacerbated by continuing sector growth and must be urgently addressed using multiple approaches while foundations are set for long-term industry growth.

Strategy

Develop a multi-factorial approach to address urgent and growing staffing shortages in the short term, while building a strong staffing pipeline for the future.

Table 4: Objectives and tactics for maturing the cell and gene manufacturing workforce

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
3. Develop and deliver a dedicated strategy to grow the C&G manufacturing workforce that considers both short-term and long-term workforce plans across the range of C&G-related roles.	**3.1.** This strategy should incorporate the below-stated objectives and tactics.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Attract, build and retain world-class talent

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
4. Advocate for contributions (financial and/or in kind) from a range of sources.	**4.1.** Government (state and federal) to provide funding; industry (e.g., product developers and CDMOs) to co-design courses, provide equipment and contribute funding; vocational (e.g., universities, TAFEs, medical research institutes) to host, co-design and run courses; and international organisations (International Society for Cell and Gene Therapy, Foundation for the Accreditation of Cellular Therapy) to contribute to course design.	A national unified group (such as the proposed CGEAMG)	Short	Attract, build and retain world-class talent, Secure long-term investment in the sector
5. Fund expansion of fellowships and traineeships in advanced manufacturing for C&G products.	**5.1.** Leverage existing programme models e.g., REDI fellowship programme.	A nationally representative body (such as the Australian Cell and Gene Catalyst), MTPConnect, REDI partners	Short	Attract, build and retain world-class talent
6. Develop an in-depth, collaborative, nationally accredited training and development programme aligned with Australian and regional regulations – including formalised certification in advanced manufacturing for C&G products aimed at developing an industry-ready workforce pipeline.	**6.1.** Identify appropriate academic organisations to collaborate with industry players and international organisations to drive the development and setup of entry-level workforce training centres.	A nationally representative body (such as the Australian Cell and Gene Catalyst), ISCT ANZ workforce development subcommittee	Medium	Attract, build and retain world-class talent

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
	6.2. Provide baseline training designed to shorten the on-boarding period for mandatory in-house training provided by CDMOs and industry. This should address broad training for three groups: cleanroom technicians, product development scientists and, QA and QC staff. Each course should receive a substantial hands-on component with a focus on the practice of GMP combined with more occupation-specific material.	Select academic organisations, industry players and international organisations	Medium	Attract, build and retain world-class talent
	6.3. Leverage existing training programmes. For example: Spark Oceania; International Society for Cell & Gene Therapy ISCT-ANZ for GMP training; BIF at UTS; ISCT international in Spain; American Society of Hematology (ASH) Education; Cell and Gene Therapy Catapult (UK) Advanced Therapy Skills Training Network (ATSTN) and Advanced Therapies Apprenticeship Community (ATAC); Biomanufacturing Training and Education Centre (BTEC) short courses; UTS's partnership with SeerPharma at the Biologics Innovation Facility; Canadian Advanced Therapies Training Institute (CATTI); the European Advanced Translational Research Infrastructure (EATRIS) Advance course.	A nationally representative body (such as the Australian Cell and Gene Catalyst) in collaboration with select academic organisations, industry players and international organisations	Medium	Attract, build and retain world-class talent

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
7. Develop micro-certifications (flexible, online short courses) to promote the development of C&G skills in supporting industries including architecture, engineering, construction, maintenance, QC testing, process support, clinical decision-support, therapy delivery, legal, risk-sharing, and patient after-care regulatory, genetic counselling, and logistics.	**7.1.** Leverage existing local and international models e.g., Irish International Cleanroom Pass Card, Melbourne MicroCerts.	A nationally representative body (such as the Australian Cell and Gene Catalyst) in collaboration with select academic organisations, industry players and international organisations	Medium	Attract, build and retain world-class talent
8. Streamline re-skilling from other industries in the short-term to make up urgent shortfalls.	**8.1.** Develop tools and approaches to identify prospective aligned industries – such as the ATSTN career converter website in the UK.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Medium	Attract, build and retain world-class talent
	8.2. Identify appropriate academic organisations to collaborate with industry players and international organisations to drive the development and setup of re-skilling short courses.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Medium	Attract, build and retain world-class talent

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
	8.3. Collaboratively develop training modules specific to aligned industries (e.g., automotive and electronics) that account for existing transferrable skills when transitioning into an accredited training and development programme. Use existing examples as models and collaborate with the developers of those examples – e.g., IMNIS programme run by ATSE; Cell and Gene Therapy Catapult in the UK; Canadian Advanced Therapies Training Institute (CATTI); GetReskilled.	A nationally representative body (such as the Australian Cell and Gene Catalyst) in collaboration with select academic organisations, industry players and international organisations	Medium	Attract, build and retain world-class talent
	8.4. Investigate mechanisms to establish and strengthen employee pathways between regulators, academia, and industry – such as secondments, mentorships, and networks.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Medium	Attract, build and retain world-class talent
9. Identify and develop career pathways within industry to improve staff retention.	**9.1.** With industry growth, career pathways may be guided by modular certificates (developed in combination with academic institutes) for standardised technical training programmes to allow 'career laddering' and provide a structure for career path progression.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Long-term	Attract, build and retain world-class talent
	9.2. Leverage the experience of the microelectronics industry, which has faced similar staff retention challenges.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Long-term	Attract, build and retain world-class talent

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
10. Continue to build the staffing pipeline by promoting C&G industry career paths to future talent.	**10.1.** Develop and deliver practical and engaging educational activities for relevant students. Leverage existing examples within Australia for neighbouring fields. E.g., Experience Genetics from the University of Queensland's School of Biological Sciences [66].	A nationally representative body (such as the Australian Cell and Gene Catalyst), in partnership with the state and federal Departments of Education.	Long-term	Attract, build and retain world-class talent

Key metrics for tracking progress

Table 5: Metrics for addressing skills gaps and workforce development in the C&G sector

Metric	Rationale
Number of advertised job vacancies in C&G manufacturing	Advertised vacancies are an important measure of both industry growth and workforce requirements.
Average number of suitable applicants for advertised job vacancies in C&G manufacturing	Assesses the depth of talent in Australia and indicates the state of skills gaps.
Average time-to-fill for advertised vacancies in C&G manufacturing	Time required to fill positions provides a measure of urgent skills gaps.
Number of training programmes and/or micro-certifications specific to aligned industries	Addressing the skills gap through upskilling and retraining in industries with transferrable skills will require the development of high-quality and tailored training modules and micro-certifications.
Value of Federal and State government funds applied to specific C&G manufacturing training programmes	This is an indicator of government commitment to building a strong skills pipeline for the future.
Average industry retention rates	Retention rates can be used to monitor staff retention and can be indicative of career progression and satisfaction.

Challenge 2: Building critical mass in Australia's cell and gene manufacturing ecosystem

The robust pipeline of C&G products in development, coupled with record investment in the sector, presents a significant opportunity for Australia to leverage the benefits of onshore C&G manufacturing, especially in the context of urgent bottlenecks around the supply of lentiviral vectors and plasmid DNA and the capability for commercial C&G product manufacturing.

Developing C&G manufacturing capability and capacity to deliver life-changing C&G products efficiently and equitably to those that need it most will require a substantial investment in capability and capacity building across the supply chain. Timely Government support and funding to develop the C&G ecosystem is pivotal to ensure infrastructure is built ahead of demand to secure Australia's position as a critical hub for C&G.

The approach to these investments should be guided by ongoing quantitative assessment of Australia's C&G supply chain to determine how well it is prepared to deliver the growing local and global C&G pipeline (see strategy 23). The C&G value chain requires infrastructure, services and expertise across product development, clinical trials, manufacturing and delivery of therapies to patients.

Manufacturing infrastructure

Infrastructure support includes cleanrooms, process development laboratories, logistics facilities, and appropriate healthcare facilities (including pharmacies and hospitals). In particular, a high degree of logistical precision is required for manufacturing and delivering C&G products due to their time-criticality and temperature sensitivity [67]. For example, autologous therapies require cells to be isolated from a patient (apheresis, in a clinic or treatment centre), transported to a manufacturing facility for manufacture (genetic modification, cell expansion, significant safety testing and final product release) and then delivered to the clinical setting to administer the therapy to the patient. As such, local manufacturing and building the value chain around the patient can enable a faster turnaround, better scheduling of therapies, and reduced product development costs for manufacturers, which together can improve access for patients and impact equity [24].

Global leadership in C&G manufacturing infrastructure investment

The UK is one of the leading countries in C&G manufacturing, largely due to the activities of their Cell and Gene Therapy Catapult body, which since 2012 has grown from £128 million in core funding to attract £5.5 billion of investment from UK CGT companies. In 2022 Catapult reported a national total of 29 facilities, with a total C&G GMP cleanroom footprint of 16,536m^2 [70].

In 2017, the UK government invested £55 million into the Catapult for construction of the then world-first cell and gene manufacturing facility in the Stevenage Bioscience Catalyst campus [119]. The facility has a total floorplan of 7,700m^2, including 12 segregated cleanroom modules and analytical laboratories and can produce commercial products for UK patients [120].

In 2022, Stevenage was chosen by the UK-based biotechnology company Autolus Therapeutics (founded in 2014) as the site for a £66 million investment into building the UKs first CAR-T cell manufacturing facility to supporting the development and commercial supply of Autolus' "Obe-cel" product [121]. The Catapult facility supported commercial capacity for this product until the Autolus facility opened, with an initial manufacturing capacity of 2,000 batches per year [121].

This is a clear example of how government investment in C&G manufacturing has the potential to attract and support industry investment.

Health system preparedness

To complete the final stages of C&G manufacturing, the healthcare system must be equipped to support product delivery. Delivery of C&G products is novel and complex, performed within Centres of Excellence requiring a variety of well-trained clinical staff, including physicians, nurses, pharmacists and administrative staff [24].

The global healthcare system and its lack of preparedness has been cited as a limiting factor in the development of C&G products, with the ARM emphasising the need to "modernize our approach to healthcare in the US and across the EU" [1]. A recent report by The Economist outlined key elements of the healthcare system for C&G products: regulatory standards, reimbursement environment, clinical settings, and clinical workforce, and highlighted room for improvement in Australia's regulation, guidance and pathways and specialist treatment centres [68].

Building critical mass in Australia's C&G manufacturing will require a collaborative effort, identification and quantification of gaps in the value chain, and strategic investment to address those gaps across manufacturing, infrastructure and the hospital system.

Approach 1: Highlight the potential economic and social value of sovereign C&G manufacturing

Issues

The broader economic and social value of growing C&G manufacturing is often not clearly communicated to the broad range of stakeholders across the value chain. This can involve a lack of awareness of the clinical potential of C&G products, a lack of understanding of the range of their potential benefits, and the limited ways that value is assessed through current Health Technology Assessment processes [69]. This lack of recognition can restrict the development of healthcare preparedness, slowing approvals and reimbursement processes, and inhibiting the growth of cell and gene manufacturing in Australia, potentially to the extent of a failing to build on the nascent examples that are already present in Australia.

Strategy

Develop a compelling communication strategy that highlights the timely opportunity and high value in rapidly expanding cell and gene manufacturing capability in Australia.

Table 6: Objectives and tactics for highlighting the potential economic and social value of cell and gene manufacturing in Australia

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
11. Develop and deliver a communications strategy that highlights to Federal and State governments the value of manufacturing C&G products in Australia, leveraging successful international examples such as Singapore and Texas.	**11.1.** Develop key messages to highlight: (1) the value and benefits to Australian patients in having access to innovative therapies, (2) the creation of highly-skilled and high-value jobs in the biotherapeutics industry, (3) the potential to attract international investment in clinical trials in Australia and (4) the potential for increased commercialisation of C&G-based research out of Australian research institutes and universities.	A national unified group (such as the proposed CGEAMG)	Medium	Secure long-term investment in the sector
	11.2. Identify an opinion leader and a government-based champion in each state with influence at a local level (e.g., Chief Scientific Officer). Use their connections to coordinate the approach.	A national unified group (such as the proposed CGEAMG)	Medium	Secure long-term investment in the sector

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
12. Develop a unified message outlining the case for foreign investment in C&G in Australia to attract greater international investment into the Australian C&G ecosystem.	**12.1.** Highlight government support for investment in capability and capacity building activities. Highlight the value of coordinated capability and capacity building in Australia.	A national unified group (such as the proposed CGEAMG)	Short	Secure long-term investment in the sector

Approach 2: Quantify the capacity and capability of Australia's broader C&G supply chain and its fitness for purpose over time

Issues

The capacity and capability of Australia's C&G supply chain must be adequately monitored to determine how well it meets the demands of the growing local and global C&G pipeline (see Challenge 4) and to ensure investment is properly timed and targeted (see strategies 18 to 20).

While national manufacturing capabilities and capacity were surveyed in 2021 [56], the report's utility would be improved by the collection of additional information about TGA-certification, different uses of cleanroom space [clinical trial product vs commercial/marketed product manufacturing, output capacity (# doses/cleanroom/year)], in-house experience and expertise with technology platforms, and accessibility.

Allowing the report to illustrate which products could be made in Australia and how many of those products could be produced would improve its utility for:

1. assessing the fitness of Australia's cell and gene manufacturing ecosystem for supporting the growing number of clinical trials
2. supporting future growth and investment within the sector, and
3. advertising Australia as a prime location for conducting C&G clinical trials, and a regional hub for manufacturing C&G products for the APAC region.

As part of the current project the 2021 questionnaire was distributed to a wide range of C&G industry stakeholders. Three additional manufacturers were identified, but respondents also expressed uncertainty about the exact nature of the information being sought by several of the questions.

Strategy

Assess Australian capability and capacity in manufacturing, logistics and healthcare.

Table 7: Objectives and tactics for quantifying the capacity and capability of Australia's broader C&G supply chain and its fitness for purpose over time

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
13. Determine capability and capacity requirements across the value chain for C&G manufacturing and delivery.	**13.1.** Conduct and regularly update an audit of Australia's C&G manufacturing capability, leveraging the Australia's Regenerative Medicine Manufacturing Capacity & Capability Report [56].	A nationally representative body (such as the Australian Cell and Gene Catalyst), TIA, National Collaborative Research Infrastructure Strategy (NCRIS)	Short	Build Australian capability across the RM value chain
	13.2. Outline physical size of facilities, experience and expertise with technology platforms, and accessibility. Include process development facilities, manufacturing facilities, and logistics capabilities (in-house, external, and international). Leverage international examples (e.g., Cell and Gene Therapy Catapult (2022) [70]) and adapt them to the Australian environment. The audience and purpose of this information would be two-fold; (1) to the Australian research ecosystem to facilitate access to resources for manufacturing and commercialising local research, and (2) to global C&G developers to promote investment into Australian manufacturing.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Build Australian capability across the RM value chain. Secure long-term investment in the sector

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
	13.3. Review of capabilities and capacity to deliver C&G products in hospitals/healthcare settings. This review should aim to ensure that Australian hospitals can sustainably support clinical trials of C&G products across a range of therapeutic areas and populations, as well as sustainably support the delivery of an increasing number of commercial C&G products in clinical practice.	A nationally representative body (such as the Australian Cell and Gene Catalyst) in collaboration with key stakeholders including HCPs, hospital pharmacies, and state governments.	Short	Build Australian capability across the RM value chain
	13.4. Regular reporting of capacity and capability for C&G manufacturing in Australia would ideally be incorporated into the proposed 'front door' (see strategy 25.1).	A national unified group (such as the proposed CGEAMG)	Short	Build Australian capability across the RM value chain

Approach 3: Invest strategically in manufacturing capability and capacity

Issues

Despite significant recent investments in onshore manufacturing capacity and capabilities (see "Australian C&G manufacturing activity" above), continued growth will be required to deliver the rapidly developing pipeline of therapies at commercial grade and scale [21, 47, 56, 68, 71]. As the industry matures and patient numbers grow, it is essential to strategically develop Australian capabilities to secure onshore manufacturing while ensuring that lower initial demand for certain manufacturing services does not impact the short-to-medium-term sustainability of manufacturing facilities.

The speed of technology development poses a key challenge here. Rapid advances to equipment and processes being updated rapidly highlights the need for future-proofing of facilities and has contributed to a trend towards decentralised single-use technologies and flexible facility design [72]. This is a crucial aspect to strategically approach and consider long-term investments in facility-building (strategy 14).

Strategy

Invest in future-proofed capabilities to support the whole product development pipeline through to commercial product supply.

Table 8: Objectives and tactics for investing strategically in manufacturing capability and capacity

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
14. Upgrade C&G manufacturing capabilities to support the C&G pipeline, with a focus on boosting Australia's strengths to attract and encourage growth of commercial-scale manufacturing capabilities. Upgrading should encompass the following key tactics:	**14.1.** Addressing the global and local pipelines of therapeutics being developed across the range of modalities (strategy 23.1) in the context of the global supply of C&G products (strategy 23.2).	A nationally representative body (such as the Australian Cell and Gene Catalyst), TIA	Short	Build Australian capability across the RM value chain. Secure long-term investment in the sector
	14.2. Leveraging Australia's existing capability and capacity for manufacturing C&G products to support (1) local translational capabilities (strategy 18.1-18.3, below), (2) clinical-scale manufacturing requirements and, (3) attract commercial manufacturing programs utilising and supporting expansion of existing capabilities where appropriate.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Build Australian capability across the RM value chain. Secure long-term investment in the sector
	14.3. Identifying sources of funding and investment to support development and operations of manufacturing facilities, including federal and state government sources, international industry, and local companies. Ensure that funding is available to support technology transfer processes.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Build Australian capability across the RM value chain. Secure long-term investment in the sector
	14.4. With an initial focus on expanding existing facilities, develop flexible/modular manufacturing capabilities with future-proofed approaches to minimise the risk of redundancy – allowing adaptation to changing requirements and novel modalities and sharing of equipment resources and support staff [73]. Include supporting facilities as described in strategy 18.1 below.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Build Australian capability across the RM value chain. Secure long-term investment in the sector

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
	14.5. Reviewing and identifying potential sites and requirements for investment. Site selection considerations should include: (1) prioritise expansion of existing facilities over construction of new ones; (2) avoid unproductive duplication of capabilities; (3) contribute to strategic and equitable distribution of C&G products around the nation; (4) account for product-specific requirements, for example facilities involved in cell culture will require larger floorplans and scalability (see Table 2 for additional information); (5) ideally, selected sites will be suitable for continued expansion.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Build Australian capability across the RM value chain. Secure long-term investment in the sector
	14.6. Developing approaches to assess the long-term return on government investments into the manufacturing ecosystem. Potential key performance indicators (KPIs) may include access to innovative therapies for Australian patients, the number of new highly-skilled jobs, the number of projects, the progress of projects, the distribution of client types [small-to-medium enterprises (SMEs), researchers, medium companies], and what discounted rates applied/were given based on the investment they had received. However, KPIs should be carefully developed and prudently applied [74].	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Build Australian capability across the RM value chain. Secure long-term investment in the sector

Approach 4: Invest appropriately in national health system preparedness

Issues

The ARM has highlighted global healthcare preparedness as the main limiting factor of C&G product development, emphasising the need to "modernize our approach to healthcare in the US and across the EU". The Australian healthcare system also requires investment to accommodate existing and pipeline C&G products, from regulators and payers, through to logistics, pharmacy capabilities and hospital staff [11].

To ensure that internationally developed C&G products can be manufactured, delivered locally and exported to other markets, and that locally developed C&G products can be available overseas, Australian manufacturing must align with the standards of international healthcare systems. Further, as patients are part of the manufacturing process for C&G technologies, the Australian healthcare system also needs to adapt in order to better deliver future C&G products to Australian patients and support local C&G manufacturers.

Ease of market access was identified in the consultation workshops as a key factor for attracting foreign companies to manufacture locally as well as a reason for Australian companies to dismiss local operations. Market access including regulatory approval, reimbursement, and delivery to patients, was highlighted as challenging in the current Australian market. The key elements of health system preparedness that need addressing to maximise the potential of the Australian market were defined as: coordination of regulatory and reimbursement standards, logistics (especially lack of warehousing capability), equity of access, and collaboration across the health system.

Strategy

Highlight the impact of non-aligned market access processes on C&G manufacturing, fund the development of clinical centres of excellence, and develop hospital-based capabilities with an initial focus on clinical trials and expanding to standard of care.

Table 9: Objectives and tactics for investing appropriately in national health system preparedness

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
15. Communicate to state and federal governments the importance and potential value of coordinated market access standards to C&G manufacturing.	**15.1.** Prepare a white paper exploring the underlying issues that lead to inequities in access to C&G products (need for expansion of newborn screening panels, rural vs urban, differences in state healthcare systems).	A national unified group (such as the proposed CGEAMG)	Short	Creation of a clear market access pathway that is aligned to leading global markets

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
	15.2. Develop unified narratives that highlight the benefits of coordinated market access to C&G manufacturing, and the risks of not having coordinated market access – ensuring that patient and clinician perspectives are included.	A national unified group (such as the proposed CGEAMG)	Short	Creation of a clear market access pathway that is aligned to leading global markets
	15.3. Identify opinion leaders and advocates in each government with influence at a local level (e.g., Chief Scientific Officer). Use their connections to coordinate a unified approach to advocate to state and federal governments for the creation of a more clearly defined reimbursement pathway/ process for equitable access to C&G products as recommended by The House of Representatives Standing Committee on Health, Aged Care and Sport Inquiry into approval processes for new drugs and novel medical technologies in Australia [75].	A national unified group (such as the proposed CGEAMG)	Short-medium	Creation of a clear market access pathway that is aligned to leading global markets
16. Optimise nation-wide delivery of C&G products in clinical practice across Australia.	**16.1.** Work to identify candidate hospitals for concentrated delivery of C&G products and assess the capability and capacity of those centres to deliver C&G products.	A nationally representative body (such as the Australian Cell and Gene Catalyst) in collaboration with state governments	Short	Build Australian capability across the RM value chain.

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
	16.2. Engage with workforce associated with multiple aspects of C&G product delivery (e.g., hospital pharmacy, IBC members, nurses, clinicians) to develop informal networks, and educate staff.	A nationally representative body (such as the Australian Cell and Gene Catalyst) in collaboration with state governments	Short	Build Australian capability across the RM value chain.
	16.3. Work with professional organisations and associations to identify areas where formal upskilling and training is required, and how this should best be done. Leverage international examples of training [76].	A nationally representative body (such as the Australian Cell and Gene Catalyst) in collaboration with state governments	Short	Build Australian capability across the RM value chain.
17. Assess national C&G logistics capabilities based on existing capability and capacity (strategy 13.2), cell and gene product pipeline (strategy 23), and relevant international best practices from sparsely populated countries (e.g., Canada).	**17.1.** Develop optimal logistical solutions to delivering C&G products around Australia and to relevant international markets. Focus on the expansion and upgrading of C&G-capable warehousing infrastructure, to minimise time-to-treatment for Australian patients and underpin the C&G manufacturing ecosystem. Leverage international examples of logistics solutions, such as the Stevenage CryoHub [77].	A nationally representative body (such as the Australian Cell and Gene Catalyst), collaborating with locally active logistics companies (e.g., Amerisource-Bergen World Courier, DHL)	Short	Build Australian capability across the RM value chain.

Key metrics for tracking progress

Table 10: Metrics for achieving critical mass in Australia's C&G manufacturing ecosystem

Metric	Rationale
Percentage of approved C&G products that are manufactured locally	The proportion of C&G products that involve local manufacturing indicates the potential reach of Australia's C&G manufacturing.
Value and proportion of foreign C&G investment in Australia	The scale of foreign investment is a key factor for the industry's sustainability and ability to achieve critical mass.
Scale of manufacturing facilities funded	Investment in developing manufacturing capabilities is a driver of sustainable industry growth and indicator of future critical mass.
Number of jobs created across the C&G value chain	Job creation is a key factor determining government funding decisions. The ability to create jobs will thus drive the industry's sustainability.

Challenge 3: Optimising Australia's contributions to the C&G product pipeline

The Regenerative Medicine Consortium Project outlined Australia's well-recognised strengths in academic research and in its globally competitive clinical trials system, particularly in early phases [35, 12]. However, Australia's broader biotechnology industry has historically struggled to translate these research outcomes into commercially successful products and services [78]. This challenge is amplified for C&G products due to the stringent quality requirements, high cost of materials, highly specialised workforce and lack of expertise in product development and lack of access to product development capabilities, and risk-averse venture capital. Gaps remain in the translational development space regarding access to GMP-grade starting material and product and process development [24], as well as in later phase (II-III) clinical trials [12].

Supporting growth in Australian clinical trials is a key strategy to promote the development of C&G delivery infrastructure and expertise across the value chain. The clinical trials sector is a recognised strength of the Australian biopharma sector [79]. It is seen as globally competitive, due to our reputation for high quality trials, the R&D Tax Incentive, and streamlined regulatory processes via the Clinical Trial Notification (CTN) scheme. Australia's clinical trials sector is ideally placed to leverage the global and regional rise in C&G trials [18], and we must further position ourselves through manufacturing capabilities to take full advantage.

Approach 1: Boost local translational capabilities for C&G product development

Issues

Considering the forecast tsunami of C&G therapies to enter the market in the coming years, and the significant pipeline of therapies progressing through the clinical trial pathway, capacity bottlenecks are expected. These include translational capacity and expertise, and clinical manufacturing capacity [12]. In particular, the cost of access to essential translational capabilities for C&G developers is far higher than that of pharmaceuticals or biologics and is beyond the reach of many local product developers. As the sector matures, and more C&G therapies become standard of care, commercial manufacturing capacity and capability will also need to grow to service local demand, and access export markets.

Strategy

Increasing the capacity and availability of local translational capabilities while supporting communal growth in the local product development knowledge base

Table 11: Objectives and tactics for boosting local translational capabilities for C&G product development

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
18. Create and improve access to local translational capabilities to act as a stepping-stone to local manufacturing and reduce the inefficiencies of local developers using overseas facilities.	**18.1.** Build/support the expansion of existing local facilities including (1) GMP-certified clinical scale laboratories, and (2) NATA GLP-accredited [80] translational laboratories equipped for QA process development and QC testing standardisation and validation (e.g., cellular barcoding). Ideally, these would form part of the government-funded industry-run manufacturing facilities proposed in strategy 14.	A nationally representative body (such as the Australian Cell and Gene Catalyst), Therapeutic Innovation Australia, state and federal governments and large industry partners (product developers and CDMOs)	Short	Build Australian capability across the RM value chain.

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
	18.2. Develop and fund incentives to access local translational capabilities including fee waivers, vouchers, or HELP-style delayed funding for SMEs or low revenue companies developing C&G products, payable on threshold revenue being reached. Any incentive schemes should be developed in consultation with key stakeholders representative of early-stage research and SMEs to ensure they are fit-for-purpose.	A nationally representative body (such as the Australian Cell and Gene Catalyst), state and federal governments and large industry partners (product developers and CDMOs)	Short	Build Australian capability across the RM value chain.
	18.3. Leverage existing resources to promote the proposed product development facilities – such as the TIA, NCRIS, Australian Infrastructure Research Network (ARIN), as well as local scientific and industry conferences.	A nationally representative body (such as the Australian Cell and Gene Catalyst), state and federal governments and large industry partners (product developers and CDMOs)	Short	Build Australian capability across the RM value chain.

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
19. Grow (and found, if necessary) communities of practice between stakeholders across the C&G value chain (academics, small biotech, MNO, CRO, and CDMO) with a focus on manufacturing and facilitation of product development. The community should deliver networking and educational events aimed at facilitating collaboration and the exchange of knowledge, and expertise between stakeholders across the value chain.	**19.1.** Identify and support local networks and communities of practice, providing the support required for them to grow in line with identified best practices, ensuring they are well-resourced and coordinated.	A nationally representative body (such as the Australian Cell and Gene Catalyst).	Short	Build Australian capability across the RM value chain. Attract, build and retain world-class talent
	19.2. Leverage from local (e.g., The BioProcessing Network, TIA's facility network) and international (e.g., 'ATMP Community of Practice' at International Society for Pharmaceutical Engineering (ISPE), International BioPhorum) communities of practice. Identify gold standard approaches to dealing with common issues with these communities such as (1) involving a broad range of stakeholders, (2) how to mitigate inherent bias towards commercial suppliers pushing products, and (3) approaches to sustainable funding and coordination of these communities of practice.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Build Australian capability across the RM value chain. Attract, build and retain world-class talent

Approach 2: Strengthen Australia's clinical trial capability

Issues

Australia's Regenerative Medicine Clinical Trials Database [35] identified 222 RM clinical trials in Australia with start dates between 2015-2021 at a CAGR of 15.5% between 2016 and 2020. However, this report has quickly become outdated, and the effects of pandemic recovery and global sector growth on the Australian C&G clinical trials picture are unclear. Therefore, updated data sets are required to monitor and respond to growth in this space.

Despite being recognised as a leader in clinical trials, Australia's competitiveness is threatened by costs and delays to trial start-up, which can be associated with ethic and governance processes. Doctors play a dual advocacy role in efficiently delivering C&G clinical trials. Firstly, C&G trials are often 'investigator initiated' studies – designed and led by qualified clinicians [81]. Secondly, doctors play key roles in human research ethics committees (HRECs) which must review and approve the proposed clinical trials protocols before the trial can be conducted. Manufacturing processes of C&G products are included in HREC submissions, however a lack of detailed knowledge in this space can lead to delayed and inappropriate approval decisions. Clinician education is therefore key to help more doctors to run and appropriately approve clinical trials.

Strategy

Streamline clinical trials processes by supporting the education of key stakeholders.

Table 12: Objectives and tactics for strengthening Australia's clinical trial capability

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
20. Advocate to RACP and RCPA to establish 'Cell and Gene Therapy' Special Interest Group (SIG). The SIG should enable the sharing of learnings and expertise built up by experience in the trial and clinical delivery of C&G products.	**20.1.** Develop a well-educated clinical workforce able to conduct clinical trials in C&G products through targeted engagements and education sessions with both the proposed CGEAMG and the proposed SIG, including sharing of latest research in the sector.	A national unified group (such as the proposed CGEAMG)	Short	Build Australian capability across the RM value chain. Attract, build and retain world-class talent

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
21. Fund the preparation and submission of proposals to the Medical Board of Australia and Australian Medical Council for recognition of a Cell and Gene Therapist Specialisation [82, 83].	**21.1.** Identify and consult stakeholder groups that will likely be affected by the proposal and describe what differentiates the proposed new specialty from any existing medical specialties that have significant overlap in scope of practice.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Long	Attract, build and retain world-class talent
	21.2. Leverage the results of the recent (April 2023) public consultation held by the Medical Board of Australia for the recognition of genetic pathology as a new field of specialty practice [84].	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Long	Attract, build and retain world-class talent
22. Provide in principle support for the establishment of an Australasian College of Cell and Gene Therapists to train, educate and advocate on behalf of C&G clinicians.	**22.1.** Identify and contact key players from the Royal Australian College of Physicians to drive activity in this space.	A nationally representative body (such as the Australian Cell and Gene Catalyst), clinicians with expertise in C&G	Long	Attract, build and retain world-class talent

Key metrics for tracking progress

Table 13: Metrics for assessing Australia's contributions to C&G product development

Metric	Rationale
Number of C&G clinical trials in Australia	Clinical trials conducted in Australia improve local access to the C&G pipeline and indirectly provide benefits for the healthcare system and C&G research and product development industry.
Number of Australian developed C&G products reaching the clinical trials stage	Progression through to clinical trials improves the probabilistic measure of commercialisation success in the future.
Number of locally developed products that are manufactured locally	Indicator of attractiveness of Australian product development and manufacturing incentives and indirect measure of ecosystem attractiveness.
Number of approved therapies that are manufactured locally	Indicator of global competitiveness of Australian manufacturing capabilities.
Value of therapies manufactured in Australia exported each year	Export value is a measure of the contribution to the Australian economy and the success and competitiveness of local manufacturing.

Challenge 4: Tracking and guiding industry growth

The field of C&G continues to rapidly grow and evolve, with significant R&D and clinical trial activity both in Australia, and globally [18, 2]. C&G has been predicted to grow at a CAGR of 19.6% between 2023 and 2028 [23]. Increased demand for C&G manufacturing capabilities has been fuelled by growth in the C&G pipeline. This pipeline activity is being driven by demand for personalised medicine, advancements in technologies including gene editing, CAR-T cell therapies, synthetic biology and RNA vaccines, and the approval and upcoming regulatory review of several therapies. Significant investments in C&G R&D have also been a key driver of growth, with the ARM reporting $23.1 billion raised in capital in 2021 [18]. Additionally, investment in RM saw a substantial increase, with a 113 percent rise between 2019 and 2020. In 2020 alone, $394.1 million was invested, accounting for approximately 23 percent of the overall capital invested in the Australian biotech sector. C&G ecosystem is well placed to expand capabilities in order to capitalise on this growth from both an economic and social standpoint, where local manufacturing may improve access to these innovative therapies for Australian patients.

To position Australia as a manufacturing hub for the APAC region and leverage the industry growth, we must identify and monitor trends in both pipeline technologies and the manufacturing competitive landscape. This visibility will ensure Australia stays abreast of the industry's evolving needs in order to leverage its strengths to continue developing manufacturing capabilities. Furthermore, these developments and their implications to the field need to be communicated clearly and consistently to ecosystem stakeholders including industry, researchers, clinicians, governments and regulators.

Approach 1: Assess current and future C&G landscape

Issues

A coordinated and strategic approach to the C&G manufacturing market will be effectively delivered when aligned to the variety of C&G products under development, their specific manufacturing requirements (see Figure 2), and the changing nature of regional supply and demand. While pipeline assessments to support industry growth have been performed through several reports [85, 44], the C&G manufacturing ecosystem would benefit from a regularly updated, detailed pipeline assessment and from greater insight into regional markets for Australian-manufactured products.

Pipeline assessments should include sufficient detail to respond appropriately to manufacturing requirements on both the micro- (vastly more cells would be required to treat a liver than an eye) and macro-level (e.g., well-established commercial scale manufacturing of a certain product in Singapore might preclude the need for local manufacturing). Relevant information includes dosage, treatment regimen, and estimates of patient numbers for each indication. These examples highlight the need to use detailed and frequently updated assessments of the C&G pipeline and competitive landscape to guide sector growth.

Strategy

Leverage local and international resources to compile robust, global data about the C&G pipeline (demand), and the competitive landscape of available manufacturing capabilities and capacity (supply), since basing strategic development of a national C&G manufacturing ecosystem on hard data will raise stakeholder confidence.

Table 14: Objectives and tactics for assessing the current and future C&G landscape

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
23. Thoroughly and regularly assess the global (and local) cell and gene product pipeline, and the global manufacturing supply.	**23.1.** Update and upgrade Australia's Regenerative Medicine Global Pipeline Tracker. The report should be expanded to include dosage, treatment regimen, and estimates of patient numbers for each indication.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Build Australian capability
	23.2. Conduct a high-level assessment of global C&G supply to identify relative gaps based on the above-described pipeline assessment to identify potential areas of opportunity for investment in local manufacturing. Compile from news reports and financial reporting of listed companies.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Build Australian capability
	23.3. This pipeline would form the basis for developments in workforce (strategies 3-10) as well as manufacturing capacity and capability (strategy 13 and 14) and patient delivery (strategy 16).	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Build Australian capability
24. Conduct a thorough assessment of key international C&G markets (e.g., Singapore, Japan, Korea) to inform strategic positioning of Australian capabilities and potential for export.	**24.1.** Include considerations specific to each market, including regulation, reimbursement, healthcare system preparedness, and the competitive landscape.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Secure long-term investment in the sector, create a clear market access pathway that is aligned to leading global markets

Approach 2: Unify communications with stakeholder groups

Issues

Despite strong activity promoting and developing Australia's C&G manufacturing capability, the breadth and complexity of the space and inconsistency between the specific motives of each stakeholder group results in a lack of coordination between these approaches, which can send mixed messages to government, potential industry partners, and patients. Lack of clarity across these communications may dilute the strength of the C&G message in terms of social and economic value.

Strategy

Centralise and streamline the communications strategies through a novel body to present a united front to key ecosystem stakeholders, clarifying the strengths and challenges of the C&G Ecosystem.

Table 15: Objectives and tactics for unifying communications with stakeholder groups

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
25. Establish and/ or extend this Manufacturing Taskforce to create a 'Cell and Gene Expert Advisory Manufacturing Group' (CGEAMG) through collaboration between nationally representative bodies across the value chain, which may include the Cell and Gene Catalyst, the MRFF, the NHMRC, the ISCT, Royal Colleges, and The Australian Academy of Science – to develop and deliver tailored communication strategies as a united front to key ecosystem stakeholders.	**25.1.** Establish a comprehensive, up-to-date, and searchable 'front door' service to monitor and share information about the state of the art of Cell and Gene Manufacturing in Australia. This service would be designed to (1) attract foreign companies to Australia, and (2) direct local cell and gene product developers to local resources.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Collaborate across the value chain, Secure long-term investment in the sector

Objectives	Key Tactics	Players	Time Frame	Strategy Alignment
	25.1.1. Learn from, and leverage where possible local and international examples, such as the 'National One Stop Shop', 'National Clinical Trials Front Door', Advanced Therapy Medicinal Products (ATMP) Sweden, and resource portals at Centre for Commercialization of Regenerative Medicine (CCRM) Canada, the Cell and Gene Therapy Catapult (UK), and the National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL). **25.1.2.** Present information about governance, funding, infrastructure, workforce, networks, education, logistics, clinical services and programmes/initiatives.	A nationally representative body (such as the Australian Cell and Gene Catalyst)	Short	Collaborate across the value chain, Secure long-term investment in the sector
	25.2. Advocate for contributions from a range of industry and government sources to C&G training programmes across all levels of education (see strategy 4).	A nationally unified body (such as the proposed CGEAMG)	Short	Secure long-term investment in the sector
	25.3. Advocate the RACP for the establishment of a Cell and Gene Therapy Special Interest Group (see strategy 20).	A nationally unified body (such as the proposed CGEAMG)	Short	Attract, build and retain world-class talent
	25.4. Advocate to state and federal governments to align and improve the market access landscape for C&G products in Australia (see strategy 15).	A nationally unified body (such as the proposed CGEAMG)	Short	Create a clear market access pathway that is aligned to leading global markets

Key metrics for tracking progress

Table 16: Metrics for guiding industry growth

Metric	Rationale
Number of industry membership type organisations that contribute to the development of the global pipeline	The utility and comprehensiveness of an Australian C&G pipeline and assessment of capabilities is dependent on the extent and quality of industry information provided.
Number of stakeholders engaged by the CGEAMG	Extent of industry engagement will impact the depth and breadth of CGEAMG strategies.
Activity at 'front door' website (local vs international users, new and repeat users, organic search vs partners)	This will give a measure of the scale of interest in Australian C&G manufacturing capabilities.
Number of local groups who successfully find resources using the 'front door'	The 'front door' website's utility will be reflected in the number of groups who report being able to effectively interact with it.
Key industry opinion leaders represented on decisive government committees	Development of the sector will require streamlined and effective communications with relevant government committees to drive policy and initiatives.

Implementation and tracking progress

Through this Blueprint, we present a strategic plan to mature cell and gene manufacturing in Australia. This document looks to the future by identifying key barriers and proposing strategic approaches and greater sector coordination needed to overcome them.

Tactics have been developed to collectively deliver on the objectives identified through the consultation process.

Investments into workforce (strategies 1 and 3) and manufacturing capability and capacity (strategies 13 and 14) should be commenced urgently. Meanwhile, detailed information is required to support and refine the development of detailed tactics for growing the C&G manufacturing sector. As such, updated and detailed assessments of the pipeline (strategy 23), workforce (strategies 1-2) and manufacturing capability and capacity (strategy 13) should also be prioritised in parallel.

Due to the complexity and rapidly changing nature of the C&G space, we recommend regularly repeating the aforementioned assessments to monitor the scale and suitability of capability and capacity growth. To ensure appropriate progress is being made, key metrics should be assessed at two years, five years, and seven years.

Australia's Cell and Gene Catalyst led by the Catalyst Steering Group and the General Manager should also integrate this manufacturing blueprint into the upcoming broader strategic plan and foster the collaboration needed to deliver on those plans.

The Cell and Gene Catalyst will also determine timelines for delivering the longer-term elements of this work.

Figure 5: Mapping of objectives to their associated implementation timeframes. The indicative periods for each timeframe are: short term (within 12 months), medium term (1-3 years), long term (3-10 years).

Short term (within 12 months)

- Urgently quantify the national shortage of QA/QC and GMP cleanroom staff to guide short-term workforce development strategies.
- Clarify and quantify skills gaps in the cell and gene manufacturing ecosystem on an ongoing basis – expanding on those identified in the MTPConnect REDI Initiative Skills Gap Analysis [60].
- Develop and deliver a dedicated strategy to grow the C&G manufacturing workforce that considers both short-term and long-term workforce plans across the range of C&G-related roles.
- Advocate for contributions (financial and/or in kind) from a range of sources.
- Fund expansion of fellowships and traineeships in advanced manufacturing for C&G products.
- Develop a unified message outlining the case for foreign investment in C&G products in Australia to attract greater international investment into the Australian C&G ecosystem.
- Determine capability and capacity requirements across the value chain for C&G manufacturing and delivery.
- Upgrade C&G manufacturing capabilities to support the C&G pipeline, with a focus on boosting Australia's strengths to attract and encourage growth of commercial manufacturing capabilities.
- Communicate to state and federal governments the importance and potential value of coordinated market access standards to C&G manufacturing.
- Optimise nation-wide delivery of C&G products into clinical practice across Australia, including standard of care.
- Assess national C&G logistics capabilities based on existing capability and capacity (strategy 13.2), cell and gene product pipeline (strategy 23), and relevant international best practices from sparsely populated countries (e.g., Canada).
- Create and improve access to local translational capabilities to act as a stepping-stone to local manufacturing and reduce the inefficiencies of local developers using overseas facilities.
- Grow (and found, if necessary) communities of practice between stakeholders across the C&G value chain (academics, small biotech, MNO, CRO, and CDMO) with a focus on manufacturing and facilitation of product development. The community should deliver networking and educational events aimed at facilitating collaboration and the exchange of knowledge, and expertise between stakeholders across the value chain.
- Advocate to the RACP and the RCPA to establish 'Cell and Gene Therapy' Special Interest Group. The SIG should enable the sharing of learnings and expertise built up by experience in the trial and clinical delivery of C&G products.
- Thoroughly and regularly assess the global (and local) cell and gene product pipeline, and the global manufacturing supply.
- Conduct a thorough assessment of key international C&G markets (e.g., Singapore, Japan, Korea) to inform strategic positioning of Australian capabilities and potential for export.
- Establish and/or extend this Manufacturing Taskforce to create a CGEAMG through collaboration between nationally representative bodies across the value chain, which may include the Cell and Gene Catalyst, the MRFF, the NHMRC, the ISCT, Royal Colleges, and The Australian Academy of Science – to develop and deliver tailored communication strategies as a united front to key ecosystem stakeholders.

Medium term (1–3 years)

- Develop an in-depth, collaborative, nationally accredited training and development programme aligned with Australian and regional regulations – including formalised certification in advanced manufacturing for C&G products aimed at developing an industry-ready workforce pipeline.
- Develop micro-certifications (flexible, online short courses) to promote the development of C&G skills in supporting industries including architecture, engineering, construction, maintenance, QC testing, process support, clinical decision-support, therapy delivery, legal, risk-sharing, and patient after-care regulatory, genetic counselling, and logistics.
- Streamline re-skilling from other industries in the short-term to make up urgent shortfalls.
- Develop and deliver a communications strategy that highlights to Federal and State governments the value of manufacturing C&G products in Australia, leveraging successful international examples such as Singapore and Texas.

Long term (3–10 years)

- Identify and develop career pathways within industry to improve staff retention.
- Continue to build the staffing pipeline by promoting C&G industry career paths to future talent.
- Fund the preparation and submission of proposals to the Medical Board of Australia and Australian Medical Council for recognition of a Cell and Gene Therapist Specialisation [82, 83].
- Provide in principle support for the establishment of an Australasian College of Cell and Gene Therapists to train, educate and advocate on behalf of C&G clinicians.

Table 17: Mapping of key players and objectives assigned

Players	Objectives
A national unified group (such as the proposed CGEAMG)	Advocate for contributions (financial and/or in kind) from a range of sources. Develop and deliver a communications strategy that highlights to Federal and State governments the value of manufacturing C&G products in Australia, leveraging successful international examples such as Singapore and Texas. Develop a unified message outlining the case for foreign investment in C&G products in Australia to attract greater international investment into the Australian C&G ecosystem. Communicate to state and federal governments the importance and potential value of coordinated market access standards to C&G manufacturing. Advocate to RACP and RCPA to establish 'Cell and Gene Therapy' SIG. The SIG should enable the sharing of learnings and expertise built up by experience in the trial and clinical delivery of C&G products Advocate for clear funding pathways for C&G products in Australia, including considerations of (1) value for these high-cost innovative therapies, (2) innovative funding mechanisms for these often high-cost therapies, (3) use of real-world evidence to support applications for reimbursement, and (4) considerations for state and federal funding mechanisms.
A nationally representative body (such as the Australian Cell and Gene Catalyst)	Urgently quantify the national shortage of QA/QC and GMP cleanroom staff to guide short-term workforce development strategies. Clarify and quantify skills gaps in the cell and gene manufacturing ecosystem on an ongoing basis – expanding on those identified in the MTPConnect REDI Initiative Skills Gap Analysis [60]. Develop and deliver a dedicated strategy to grow the C&G manufacturing workforce that considers both short-term and long-term workforce plans across the range of C&G-related roles. Streamline re-skilling from other industries in the short-term to make up urgent shortfalls. Identify and develop career pathways within industry to improve staff retention. Upgrade C&G manufacturing capabilities to support the C&G pipeline, with a focus on boosting Australia's strengths to attract and encourage growth of commercial manufacturing capabilities. Grow (and found, if necessary) communities of practice between stakeholders across the C&G value chain (academics, small biotech, MNO, CRO, and CDMO) with a focus on manufacturing and facilitation of product development. The community should deliver networking and educational events aimed at facilitating collaboration and the exchange of knowledge, and expertise between stakeholders across the value chain. Fund the preparation and submission of proposals to the Medical Board of Australia and Australian Medical Council for recognition of a Cell and Gene Therapist Specialisation [82, 83]. Thoroughly and regularly assess the global (and local) cell and gene product pipeline, and the global manufacturing supply. Conduct a thorough assessment of key international C&G markets (e.g., Singapore, Japan, Korea) to inform strategic positioning of Australian capabilities and potential for export.

Players	Objectives
A nationally representative body (such as the Australian Cell and Gene Catalyst)	Urgently quantify the national shortage of QA/QC and GMP cleanroom staff to guide short-term workforce development strategies. Clarify and quantify skills gaps in the cell and gene manufacturing ecosystem on an ongoing basis – expanding on those identified in the MTPConnect REDI Initiative Skills Gap Analysis [60]. Develop and deliver a dedicated strategy to grow the C&G manufacturing workforce that considers both short-term and long-term workforce plans across the range of C&G-related roles. Streamline re-skilling from other industries in the short-term to make up urgent shortfalls. Identify and develop career pathways within industry to improve staff retention. Upgrade C&G manufacturing capabilities to support the C&G pipeline, with a focus on boosting Australia's strengths to attract and encourage growth of commercial manufacturing capabilities. Grow (and found, if necessary) communities of practice between stakeholders across the C&G value chain (academics, small biotech, MNO, CRO, and CDMO) with a focus on manufacturing and facilitation of product development. The community should deliver networking and educational events aimed at facilitating collaboration and the exchange of knowledge, and expertise between stakeholders across the value chain. Fund the preparation and submission of proposals to the Medical Board of Australia and Australian Medical Council for recognition of a Cell and Gene Therapist Specialisation [82, 83]. Thoroughly and regularly assess the global (and local) cell and gene product pipeline, and the global manufacturing supply. Conduct a thorough assessment of key international C&G markets (e.g., Singapore, Japan, Korea) to inform strategic positioning of Australian capabilities and potential for export.
A nationally representative body (such as the Australian Cell and Gene Catalyst) in collaboration with...	
A nationally unified body (such as the proposed CGEAMG)	Establish and/or extend this Manufacturing Taskforce to create a "Cell and Gene Expert Advisory Manufacturing Group" (CGEAMG) through collaboration between nationally representative bodies across the value chain, which may include the Cell and Gene Catalyst, the MRFF, the NHMRC, the ISCT, Royal Colleges, and The Australian Academy of Science – to develop and deliver tailored communication strategies as a united front to key ecosystem stakeholders.

Players	Objectives
Select academic organisations, industry players and international organisations	Fund expansion of fellowships and traineeships in advanced manufacturing for C&G products. Develop an in-depth, collaborative, nationally accredited training and development programme aligned with Australian and regional regulations – including formalised certification in advanced manufacturing for C&G products aimed at developing an industry-ready workforce pipeline. Develop micro-certifications (flexible, online short courses) to promote the development of C&G skills in supporting industries including architecture, engineering, construction, maintenance, QC testing, process support, clinical decision-support, therapy delivery, legal, risk-sharing, and patient after-care regulatory, genetic counselling, and logistics.
TIA, NCRIS, HCPs, hospital pharmacies, state governments, and the proposed CGEAMG	Determine capability and capacity requirements across the value chain for C&G manufacturing and delivery.
State governments	Optimise nation-wide delivery of C&G products into clinical practice across Australia, including standard of care.
Locally active logistics companies (e.g., AmerisourceBergen World Courier, DHL) and a national unified group (such as the proposed CGEAMG)	Assess national C&G logistics capabilities based on existing capability and capacity (strategy 13.2), cell and gene product pipeline (strategy 23), and relevant international best practices from sparsely populated countries (e.g., Canada).
State and federal governments and large industry partners (product developers and CDMOs)	Create and improve access to local translational capabilities to act as a stepping-stone to local manufacturing and reduce the inefficiencies of local developers using overseas facilities.
Clinician with expertise in C&G	Provide in principle support for the establishment of an Australasian College of Cell and Gene Therapists to train, educate and advocate on behalf of C&G clinicians.
State and federal Departments of Education	Continue to build the staffing pipeline by promoting C&G industry career paths to future talent.

Acknowledgements

This report was developed with input from a wide range of industry stakeholders through online surveys, consultations, workshops and meetings. Thanks go to these participants for their valuable contributions to the development of this strategy. Special thanks to:

AusBiotech

AusBiotech is Australia's biotechnology organisation, working on behalf of members for more than 37 years to provide representation and services to promote the global growth of the Australian biotechnology industry.

AusBiotech is a well-connected network of over 3,000 members in the biotechnology, including therapeutics, medical technology (devices and diagnostics), digital health and agribiotech sectors.

It has representation in each major Australian state, providing a national network to support members and promote the commercialisation of Australian life science in national and international marketplaces.

AusBiotech is dedicated to the development, growth, and prosperity of the Australian life science industry, by providing initiatives to drive sustainability and growth, outreach and access to markets, and representation and support for members nationally and worldwide.

Biointelect

Biointelect is a strategic planning and commercialisation firm for the biopharmaceutical and medical device sector including commercial, government and not-for-profit organisations.

Its clients include early-stage biotechnology and medical technology companies, pharmaceutical companies, universities, research institutes, government and other not-for-profit organisations.

Biointelect helps clients to develop and drive strategy, identify and evaluate new business opportunities and engage the right partners. It advises clients on the spectrum of therapeutic development from start up through to post launch, with an Australian and a global perspective.

Biointelect partnered with the Regenerative Medicines Consortium Project to deliver the *Regenerative Medicine Value Chain – The Pathway from Discovery to Patient Delivery* report.

AMMC

The Australian Medtech Manufacturing Centre (AMMC) is a $20 million Victorian Government initiative that supports the growth of medical technology manufacturing in Victoria, creating new jobs, enhancing skills and increasing investment. It has funded this important strategy, and supports innovation and manufacturing.

Table 18: The Cell and Gene Manufacturing Taskforce

Organisation	Name and position
ACURABIO	**Guillaume Herry** CEO
BioCina BRIDGEWEST GROUP	**Ian Wisenberg** Executive Chairman (BioCina), Operating Partner (Bridgewest Group)
CCRM AUSTRALIA	**Silvio Tiziani** CEO
cell therapies	**Dr Jennifer Hollands** Government and Academic Liaison
CSIRO	**Professor Susie Nilsson** Research Director, Biomedical Manufacturing
NSW StemCell NETWORK	**Dr Margret Schuller** Board member, NSW Stem Cell Network
THERAPEUTIC INNOVATION AUSTRALIA	**Dr Heather Donaghy** Manager, Scientific Engagement, Cell & Gene Therapies

Table 19: Additional contributors to in-person consultations

Organisation	Name	Role
	Dr Dawn Driscoll	
ARC Training Centre in Cell and Tissue Engineering Technologies **Monash University Dept of Materials Science and Engineering** **SPARK**	Professor Laurence Meagher	Director Director Professor
ATMP Sweden	Dr Heather Main	Project Manager
Bayer	Loredana Esposito	Country Head of Clinical Operations
BMDI Cord Blood Bank, Murdoch Children's Research Institute **Foundation for the Accreditation of Cellular Therapies (FACT)**	Associate Professor Ngaire Elwood	Director Vice President of the international Board of Directors
Cell & Molecular Therapies RPA	Dr Sharon Sagnella	Research and Development Manager
Cell & Tissue Therapies WA, Royal Perth Hospital	Dr Zlatibor Velickovic	Facility Director
Cell and Molecular Therapies Royal Prince Alfred (RPA) Hospital	Professor John Rasko AO	Head of Department
Celleo	David Kneen	Chief Executive Officer, Head of Commercial
Celosia Therapeutics **Macquarie Uni**	Dr Brenton Hamdorf	Chief Executive Officer Director, Strategic Research Initiatives
Centre for Biopharmaceutical Excellence	Andrew Watson	Director
Children's Medical Research Institute and The Sydney Children's Hospitals Network	Professor Ian Alexander	Head, Gene Therapy Research Unit
Cytiva	Dr Janet Macpherson	Business Development Manager

Organisation	Name	Role
Merck Group	Sarah Parsons	Regional Strategy Execution: India, Oceania, SEA and Taiwan — Process Solutions
Miltenyi Biotec	Matt Banfield	Managing Director
Moderna	Sam Develin	Director, External Affairs, Australia & New Zealand
Novartis	Monique Jonson	Head of Cell and Gene Therapy Australia and New Zealand
Paul Fennessy Advisory	Paul Fennessy	Principal
Prescient Therapeutics	Steven Yatomi-Clarke	Chief Executive Officer & Managing Director
QIMR Berghofer Medical Research Institute **Queensland Immunology Research Centre**	Professor Rajiv Khanna	Co-Director Distinguished Scientist
Queensland University of Technology Centre for Biomedical Technologies	Professor Yi-Chin Toh	Director
University of Queensland	Professor Simon Cool	Professor of Bioengineering
World Courier	Brent McPherson	Commercial Manager ANZ

Additional parties were involved in consultations though some have chosen not to be explicitly acknowledged in this report. Additionally, efforts were made to seek input from the TGA, although they do not provide commentary on industry blueprints or strategic documents.

In additional to stakeholder consultations, a survey was conducted, garnering input from 33 respondents. We would like to thank all survey contributors who shared their perspectives and expertise.

Abbreviations

Term	Definition
AAVV	Adeno-associated Viral Vectors
ACTRIS	Advanced Cell Therapy Research Institute SIngapore
ALL	Acute lymphoblastic leukaemia
APAC	Asia-Pacific
ARM	Alliance for Regenerative Medicine
ATMP	Advanced Therapy Medicinal Products
ATSTN	Advanced Therapies Skills Training Network
C&G	Cell and gene
CAGR	Compound annual growth rate
CAR	Chimeric antigen receptor
CCRM	Centre for Commercialization of Regenerative Medicine
CDM	Contract development and manufacturing organisation
CGEAMG	Cell and Gene Expert Advisory Manufacturing Group
CGT	Cell and gene therapy
CMRI	Children's Medical Research Institute
CR	Contract research organisation
CTGTP	Cell, tissue or gene therapy product
CTN	Clinical Trial Notification
EATRIS	The European Advanced Translational Research Infrastructure
FDA	U.S. Food and Drug Administration
GLP	Good laboratory practice
GM	Genetically modified
GMP	Good manufacturing practice
HAS	Health Sciences Authority

HCP	Healthcare professional
HREC	Human Research Ethics Committee
IP	Intellectual property
KPI	Key performance indicator
MCRI	Murdoch Children's Research Institute
MN	Multinational organisation
MRFF	Medical Research Futures Fund
mRNA	Messenger RNA
NCRIS	National Collaborative Research Infrastructure Strategy
NHMRC	National Health and Medical Research Council
NSW	New South Wales
pDNA	Plasmid DNA
QA	Quality assurance
QC	Quality control
R&D	Research and development
RACP	Royal Australasian College of Physicians
RCPA	Royal College of Pathologists of Australasia
REDI	Researcher Exchange and Development within Industry
RM	Regenerative medicine
SIG	Special Interest Group
SMA	Spinal muscular atrophy
SME	Small-to-medium enterprise
SWOT	Strengths, Weaknesses, Opportunities, and Threats
TGA	Therapeutic Goods Administration
TIA	Therapeutic Innovation Australia
VVMF	Viral vector manufacturing facility

References

[1] Alliance for Regenerative Medicine, "Cell & Gene State of the Industry," Alliance for Regenerative Medicine, San Francisco, 2023.

[2] G. O'Sullivan, J. Philips and J. Rasko, "Clinical gene technology in Australia: building on solid foundations," Medical Journal of Australia, no. 217, pp. 65-70, 18 July 2022.

[3] CSL, "First Gene Therapy for Hemophilia B, CSL's HEMGENIX®, Approved by the European Commission," 20 February 2023. [Online]. Available: https://newsroom.csl.com/2023-02-20-First-Gene-Therapy-for-Hemophilia-B,-CSLs-HEMGENIX-R-,-Approved-by-the-European-Commission.

[4] PYC Therapeutics, "PYC completes Pre-IND engagement with the FDA in the RP11 programme," 23 March 2022. [Online]. Available: https://pyctx.com/wp-content/uploads/2022/03/PYC-COMPLETES-PRE-IND-ENGAGEMENT-WITH-FDA-RP11-Programme.pdf.

[5] Children's Medical Research Institute, "New funding puts Viral Vector Manufacturing Facility in winning position," 21 June 2022. [Online]. Available: https://www.cmrijeansforgenes.org.au/news/new-funding-puts-viral-vector-manufacturing-facility-in-winning-position.

[6] Victoria State Government, The Hon Tim Pallas MP, "Moderna mRNA Vaccines Plant To Deliver Hundreds Of Jobs," 7 December 2022. [Online]. Available: https://www.premier.vic.gov.au/moderna-mrna-vaccines-plant-deliver-hundreds-jobs.

[7] Cell Therapies, "Cell Therapies to manufacture BioNTech's product candidate BNT211 in Australia," 21 September 2022. [Online]. Available: https://celltherapies.com/cell-therapies-cell-therapies-to-manufacture-biontechs-product-bnt211-in-australia/.

[8] Australian Government, Department of Health and Aged Care, "Medical Research Future Fund," 28 November 2022. [Online]. Available: https://www.health.gov.au/our-work/medical-research-future-fund/all-mrff-initiatives. [Accessed 28 April 2023].

[9] Australian Labor Party, "National Reconstruction Fund," Australian Labor Party, [Online]. Available: https://www.alp.org.au/policies/national_reconstruction_fund. [Accessed April 2023].

[10] Australian Government, Department of Industry, Science and Resources, "Modern Manufacturing Initiative and National Manufacturing Priorities announced," Department of Industry, Science and Resources, 1 October 2020. [Online]. Available: https://www.industry.gov.au/news/modern-manufacturing-initiative-and-national-manufacturing-priorities-announced.

[11] AusBiotech, "Regenerative Medicine in Australia," September 2021. [Online]. Available: https://www.ausbiotech.org/documents/item/677. [Accessed April 2023].

[12] AusBiotech, "Biotechnology Blueprint A Decadal Strategy for the Australian Biotechnology Industry," May 2022. [Online]. Available: https://www.ausbiotech.org/.

[13] Pearce IP, "Gene Therapy 22/23 Budget Announcement Big News for Australian Patients, and for Novartis," Lexology, 30 March 2022. [Online]. Available: https://www.lexology.com/library/detail.aspx?g=efeaf9ca-b07a-4f27-b8f8-bffd056927aa. [Accessed 2023 May 22].

[14] Leukaemia Foundation, "Expert Series: A/Prof. Michael Dickinson discusses CAR T-cell therapy," Leukaemia Foundation, 1 June 2021. [Online]. Available: https://www.leukaemia.org.au/stories/expert-series-interview-associate-professor-michael-dickinson/#:~:text=He%20estimates%20that%20more%20than,those%20who%20have%20gone%20overseas. [Accessed 12 May 2023].

[15] Alliance for Regenerative Medicine, "The Pipeline Momentum Builds: H1 2022," September 2022. [Online]. Available: http://alliancerm.org/wp-content/uploads/2022/10/ARM-H1-2022-R13.pdf.

[16] C. Iglesias-Lopez, A. Agustí , A. Vallano and M. Obach, "Current landscape of clinical development and approval of advanced therapies.," Molecular therapy. Methods & clinical development, vol. 23, pp. 606-618, 2021.

[17] U.S. Food and Drug Administration (FDA), "Approved Cellular and Gene Therapy Products," U.S. FDA, 17 April 2023. [Online]. Available: https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products. [Accessed 18 May 2023].

[18] Alliance for Regenerative Medicine, "The Sector Snapshot: April 2023," April 2023. [Online]. Available: https://alliancerm.org/sector-snapshot/.

[19] S. Alfano, A. Gorham, A. Loche and P. Salazar, "Eight imperatives for launching cell and gene therapies," 20 September 2022. [Online]. Available: https://www.mckinsey.com/industries/life-sciences/our-insights/eight-imperatives-for-launching-cell-and-gene-therapies.

[20] A. MacDonald and S. Whelan, "Cell and Gene Therapy: Current Challenges and the Benefits of Automation," 1 November 2022. [Online]. Available: https://www.technologynetworks.com/biopharma/blog/cell-and-gene-therapy-current-challenges-and-the-benefits-of-automation-367167#:~:text=NP%3A%20Some%20of%20the%20challenges%20faced%20by%20those,-Recruitment%20and%20retention%20of%20a%20skilled%20workforce.

[21] L.E.K Consulting, "Gene Therapy Manufacturing Fails to Meet Demand: Implications for Biopharma," 8 March 2021. [Online]. Available: https://www.lek.com/insights/ei/gene-therapy-manufacturing-fails-meet-demand-implications-biopharma.

[22] MTP Connect, "Regenerative Medicine Opportunities for Australia," October 2018. [Online]. Available: https://www.mtpconnect.org.au/images/MTPConnect%20Regenerative%20Medicine%20Report.pdf.

[23] Global Market Estimates, "Cell & Gene Therapy Manufacturing Market," April 2023. [Online]. Available: https://www.globalmarketestimates.com/market-report/cell-gene-therapy-manufacturing-market-3901.

[24] AusBiotech, "The Regenerative Medicine Value Chain," 2021.

[25] K. Elverum and M. Whitman, "Delivering cellular and gene therapies to patients: solutions for realizing the potential of the next generation of medicine," Gene Therapy, vol. 27, pp. 537-544, 2020.

[26] Cell and Gene Therapy Catapult, "National Cell and Gene Therapy Vision for the UK," March 2022. [Online]. Available: https://cgt.ams3.cdn.digitaloceanspaces.com/National-Cell-and-Gene-Therapy-Vision-for-the-UK.pdf.

[27] MTP Connect, "Medical Technology, Biotechnology and Pharmaceutical Sector Competitiveness Plan," April 2022. [Online]. Available: https://www.mtpconnect.org.au/images/2022_MTPConnect_SectorCompetitivenessPlan.pdf. [Accessed April 2023].

[28] Genetic Engineering & Biotechnology News, "Key Insights into Overcoming mRNA Process Challenges," 1 March 2022. [Online]. Available: https://www.genengnews.com/sponsored/key-insights-into-overcoming-mrna-process-challenges/. [Accessed 22 May 2023].

[29] P. Catignol and H. Lim, "Overcoming Bottlenecks in mRNA Manufacturing," 24 October 2022. [Online]. Available: https://www.pharmasalmanac.com/articles/overcoming-bottlenecks-in-mrna-manufacturing.

[30] D. Martínez-Puente, J. Pérez-Trujillo, L. Zavala-Flores, A. García-García, A. Villanueva-Olivo, H. Rodríguez-Rocha, J. Valdés, O. Saucedo-Cárdenas, R. Montes de Oca-Luna and M. Loera-Arias, "Plasmid DNA for Therapeutic Applications in Cancer," Pharmaceutics, vol. 14, no. 9, p. 1861, 3 September 2022.

[31] H. Frangoul, D. Altshuler, D. Cappellini, Y. Chen, J. Domm, B. Eustace, J. Foell, J. de la Fuente, S. Grupp, R. Handgretinger, T. Ho, A. Kattamis, A. Kernytsky, J. Lekstrom-Himes, A. Li, F. Locatelli, M. Mapara, M. de Montalembert, D. Rondelli, A. Sharma, S. Sheth, S. Soni, M. Steinberg, D. Wall, A. Yen and S. Corbacioglu, "Supplementary Appendix to: CRISPR-Cas9 Gene Editing for Sickle Cell Disease and β-Thalassemia," New England Journal of Medicine, pp. 252-260, 21 January 2021.

[32] Z. Kis, C. Kontoravdi, A. Dey, R. Shattock and N. Shah, "Rapid development and deployment of high-volume vaccines for pandemic response," Journal of Advanced Manufacturing and Processing, vol. 2, no. 3, 2020.

[33] P. Van Arnum, "The CDMO/CMO Report: Cell and Gene Therapies," DCAT Value Chain Insights, 9 March 2023. [Online]. Available: https://www.dcatvci.org/features/the-cdmo-cmo-report-cell-and-gene-therapies/.

[34] N. Pagliarulo, "Novartis turns cell therapy skill into contract manufacturing deal," Biopharma Dive, 10 March 2022. [Online]. Available: https://www.biopharmadive.com/news/novartis-carisma-contract-manufacturing-cell-therapy/620180/.

[35] AusBiotech, "Australia's Regenerative Medicine Clinical Trials Database," June 2021. [Online]. Available: https://www.ausbiotech.org/. [Accessed April 2023].

[36] Australian Government, Department of Industry, Science and Resources, "List of Critical Technologies in the National Interest," Australian Government, Department of Industry, Science and Resources, 19 May 2023. [Online]. Available: https://www.industry.gov.au/publications/list-critical-technologies-national-interest. [Accessed 24 May 2023].

[37] New South Wales, Office of the Chief Scientist & Engineer (OCSE), "Global Leader to Operate $96 Million RNA Facility at Macquarie University," NSW Government, 21 February 2023. [Online]. Available: https://www.chiefscientist.nsw.gov.au/news/global-leader-to-operate-$96-million-rna-facility-at-macquarie-university#:~:text=Australia%E2%80%99s%20first-of-its-kind%20%2496%20million%20RNA%20research%20and%20pilot,at%20Macquarie%20University%20and%20opera.

[38] Queensland Government, The Honourable Annastacia Palaszczuk, The Honourable Dr Steven Miles, The Honourable Meaghan Scanlon, "Queensland and Sanofi to establish global vaccine hub1," 5 December 2022. [Online]. Available: https://statements.qld.gov.au/statements/96732.

[39] Cleanroom Technology, "Cytiva supports BioCina with first GMP facility for mRNA in Australia," 27 April 2023. [Online]. Available: https://cleanroomtechnology.com/news/article_page/Cytiva_supports_BioCina_with_first_GMP_facility_for_mRNA_in_Australia/208530.

[40] P. M. C. Centre, "Centre of Excellence in Cellular Immunotherapy," [Online]. Available: https://www.petermac.org/research/centres-of-excellence/centre-of-excellence-in-cellular-immunotherapy. [Accessed May 2023].

[41] Cell Therapies, "Cell Therapies," [Online]. Available: https://celltherapies.com/. [Accessed April 2023].

[42] NSW Government, Health Infrastructure, "A commercial-scale viral vector manufacturing facility located at the Westmead Health and Innovation District, Sydney," NSW Government, Health Infrastructure, 2022.

[43] Cytiva, "AcuraBio expands cGMP plasmid DNA CDMO services with Cytiva's latest single-use purification technology to alleviate supply constraints for mRNA and cell & gene therapies," 2023. [Online]. Available: https://www.cytivalifesciences.com/en/us/news-center/acurabio-expands-cgmp-plasmid-dna-cdmo-services-10001.

[44] Evohealth, "Cell & Gene Therapies: Rising to the challenge," EvoHealth, Canberra, 2021.

[45] L.E.K Consulting, "Regenerative Medicine Opportunities and Challenges in APAC, Special Report," LEK, 2020.

[46] Australian Bureau of Statistics, "Population," Australian Bureau of Statistics, 30 September 2022. [Online]. Available: https://www.abs.gov.au/statistics/people/population. [Accessed 2023 May 23].

[47] Evohealth, "CAR T-Cell Therapy: Ready, Willing and Able?," Evohealth, Canberra, 2023.

[48] United Nations Population Fund (UNFPA), Asia amd the Pacific, "Asia-Pacific: Home to more than half of the world's population," United Nations Population Fund (UNFPA), 11 July 2022. [Online]. Available: https://asiapacific.unfpa.org/en/news/asia-pacific-home-more-half-worlds-population. [Accessed 23 May 2023].

[49] Statistics Bureau of Japan, "Population Estimates," Statistics Bureau of Japan, 1 May 2023. [Online]. Available: https://www.stat.go.jp/english/data/jinsui/tsuki/index.html. [Accessed 23 May 2023].

[50] United States Census Bureau, "Korea, South," United States Census Bureau, 1 July 2022. [Online]. Available: https://www.census.gov/popclock/world/ks. [Accessed 2023 May 23].

[51] BioSpace, "Viral Vector and Plasmid DNA Manufacturing Market Revolutionary Trends in Industry Growth Statistics By 2028," 18 August 2022. [Online]. Available: https://www.biospace.com/article/viral-vector-and-plasmid-dna-manufacturing-market-revolutionary-trends-in-industry-growth-statistics-by-2028/.

[52] J. Keenan, "Charles River inks gene therapy development and manufacturing pact with South Korean biopharma," 18 January 2023. [Online]. Available: https://www.fiercepharma.com/manufacturing/crl-pact-south-korean-biopharma-develop-liver-cancer-therapy.

[53] WuXi Advanced Therapies, "WuXi Advanced Therapies and A*STAR announce partnership to advance cell and gene therapy in the Asia-Pacific region," 28 April 2022. [Online]. Available: https://advancedtherapies.com/wuxi-advanced-therapies-and-astar-announce-partnership-to-advance-cell-and-gene-therapy-in-the-asia-pacific-region/.

[54] FUJIFILM Corporation, "Fujifilm to establish its first Bio-CDMO site in Japan," 6 October 2022. [Online]. Available: https://www.fujifilm.com/am/en/news/hq/8658.

[55] Samsung Biologics, "Samsung Biologics announces construction of Plant 5 to commence Bio Campus II capacity expansion," 17 March 2023. [Online]. Available: https://samsungbiologics.com/media/company-news/samsung-biologics-announces-construction-of-plant-5-to-commence-bio-campus-2-capacity-expansion.

[56] AusBiotech, "Australia's Regenerative Medicine Manufacturing Capacity & Capability," 2021. [Online]. Available: https://www.ausbiotech.org/.

[57] Alliance for Regenerative Medicine, "Workforce Report: Gap Analysis for the Cell and Gene Therapy Sector," March 2023. [Online]. Available: http://alliancerm.org/wp-content/uploads/2023/03/ARM-Workforce-Gap-Analysis.pdf.

[58] Cell and Gene Therapy Catapult, "2021 UK Cell and Gene Therapy Skills Demand Survey Report," 22 November 2021. [Online]. Available: https://cgt.ams3.cdn.digitaloceanspaces.com/2021-Skills-Demand-Survey-Report.pdf.

[59] Engineers Australia, "Developing Australia's advanced manufacturing capability," 2023.

[60] MTP Connect, "MTPConnect REDI Initiative Skills Gap Analysis Third Report," October 2021. [Online]. Available: https://www.mtpconnect.org.au/images/MTPC%20REDI%20Skills%20Gap%20Third%20Report.pdf.

[61] Pharmaceutical Technology, "Cell and gene therapy: the impact of skills gaps in a fast-growing sector," 14 April 2021. [Online]. Available: https://www.pharmaceutical-technology.com/features/cell-and-gene-therapy-skills-gaps-growing-sector/.

[62] J. Eglovitch, "FDA elevates OTAT to "Super Office" within CBER," Regulatory Focus A RAPS Publication, 30 September 2022. [Online]. Available: https://www.raps.org/news-and-articles/news-articles/2022/9/fda-elevates-otat-to-super-office-within-cber.

[63] Illumina, "Addressing the shortage of genetic counselors with software," Illumina, 10 November 2022. [Online]. Available: https://sapac.illumina.com/company/news-center/feature-articles/addressing-the-shortage-of-genetic-counselors-with-software.html. [Accessed 23 May 2023].

[64] ARC Training Centre for Cell & Tissue Engineering Technologies (CTET), "ARC CTET Training Centre for Cell & Tissue Engineering Technologies," April 2023. [Online]. Available: https://ctet.org.au/about/.

[65] Monash University, "Monash Centre for Advanced mRNA Medicines Manufacturing and Workforce Training," [Online]. Available: https://www.monash.edu/mrna-workforce-training-centre/training. [Accessed April 2023].

[66] The University of Queensland, School of Biological Sciences, "Experience Genetics (individuals)," The University of Queensland, School of Biological Sciences, [Online]. Available: https://biological-sciences.uq.edu.au/event/session/9083. [Accessed 24 May 2023].

[67] A. Loche, N. Paolucci, N. Peters and L. Van der Veken, "A call to action: Opportunities and challenges for CGTs in Europe," 19 January 2021. [Online]. Available: https://www.mckinsey.com/industries/life-sciences/our-insights/a-call-to-action-opportunities-and-challenges-for-cgts-in-europe.

[68] The Economist Group, "Cell and Gene Therapies: Health system progress in moving from cutting edge to common practice," 2022.

[69] A. Gye, S. Goodall and R. De Abreu Lourenco, "A Systematic Review of Health Technology Assessments of Chimeric Antigen Receptor T-Cell Therapies in Young Compared With Older Patients," Value in Health, vol. 25, no. 1, pp. 47-58, 2022.

[70] Cell and Gene Therapy Catapult, "Cell and Gene Therapy GMP Manufacturing in the UK," 16 November 2022. [Online]. Available: https://cgt.ams3.cdn.digitaloceanspaces.com/Manufacturing-Report-Final.pdf.

[71] Genetic Engineering and Biotechnology News , "Australia Needs to Invest to Tap Demand for Cell and Gene Therapy Production," Genetic Engineering and Biotechnology News , 16 November 2021. [Online]. Available: https://www.genengnews.com/topics/bioprocessing/australia-needs-to-invest-to-tap-demand-for-cell-and-gene-therapy-production/#:~:text=Capacity%20for%20cell%20and%20gene%20therapy%20production%20in,employs%20just%20231%20full-time%20and%2045%20part-time%20. [Accessed 23 May 2023].

[72] A. van Hagen, J. Drinkwater and J. Milne, "Advantages of single-use in cell and gene therapy manufacturing platforms," 5 December 2022. [Online]. Available: https://pharmaceuticalmanufacturer.media/pharmaceutical-industry-insights/pharmaceutical-manufacturing-insights/advantages-of-single-use-in-cell-and-gene-therapy-manufactur/.

[73] K. Kloosterman, "Considerations for Open Clean Room Design," 17 December 2019. [Online]. Available: https://angstromtechnology.com/considerations-for-open-cleanroom-design/.

[74] Geckoboard, "The Cobra Effect: How to Avoid Unintended Consequences When Setting Goals.," 16 May 2019. [Online]. Available: https://medium.com/geckoboard-under-the-hood/the-cobra-effect-how-to-avoid-unintended-consequences-when-setting-goals-b5c2864276e1.

[75] Parliament of the Commonwealth of Australia, House of Representatives Standing Committee on Health, Aged Care and Sport, "The New Frontier - Delivering better health for all Australians," Commonwealth of Australia, Canberra, 2021.

[76] The Advanced Therapy Treatment Centres Network, "Pan-UK pharmacy working group for ATMPs," 2021. [Online]. Available: https://www.theattcnetwork.co.uk/readiness_toolkit/pan-uk-pharmacy-working-group-for-atmps.

[77] Cleanroom Technology, "Thermo Fisher Scientific collaborates with UK Cell and Gene Therapy Catapult," Cleanroom Technology, 30 March 2017. [Online]. Available: https://www.cleanroomtechnology.com/news/article_page/Thermo_Fisher_Scientific_collaborates_with_UK_Cell_and_Gene_Therapy_Catapult/127573#:~:text=The%20CryoHub%20is%20a%20complete%20cryogenic%20storage%2C%20distribution,clinical%20trial%2C%20regardless%20. [Accessed 23 May 2023].

[78] T. Brennan, "Science and research," Parliament of Australia, 2023.

[79] National Health and Medical Research Council and Department of Industry and Science, "Why conduct a clinical trial in Australia," 14 April 2023. [Online]. Available: https://www.australianclinicaltrials.gov.au/why-conduct-clinical-trial-australia.

[80] NATA, "Organisation for Economic Cooperation & Development (OECD) Principles of Good Laboratory Practice (GLP)," [Online]. Available: https://nata.com.au/accreditation/oecd-principles-of-good-laboratory-practice/.

[81] Novartis, "Investigator Initiated Trials / Studies (clinical) and Investigator Initiated Research (non-clinical)," Novartis, [Online]. Available: https://www.novartis.com/healthcare-professionals/investigator-initiated-trials-studies-clinical-and-investigator-initiated-research-non-clinical. [Accessed 24 May 2023].

[82] Australian Medical Council, "Recognition of new medical specialties," [Online]. Available: https://www.amc.org.au/accreditation-and-recognition/recognition-of-new-medical-specialties-2/.

[83] Medical Board of Australia, "Recognition of medical specialties," 2021. [Online]. Available: https://www.medicalboard.gov.au/Registration/Recognition-of-medical-specialties.aspx.

[84] Medical Board of Australia, "Current Consultations," 6 April 2023. [Online]. Available: https://www.medicalboard.gov.au/News/Current-Consultations.aspx.

[85] AusBiotech, "Australia's Regenerative Medicine Global Pipeline Tracker," 2021. [Online]. Available: https://www.ausbiotech.org/documents/item/673.

[86] S. B. C. G. &. R. J. E. J. Mallik, "Approved gene therapies in Australia: coming to a store near you.," Internal medicine journal, no. 52, pp. 1313-1321, 2022.

[87] T. Hunt, "The Cell And Gene Therapy Sector In 2023: A Wave Is Coming – Are We Ready?," 9 January 2023. [Online]. Available: https://invivo.pharmaintelligence.informa.com/IV146781/The-Cell-And-Gene-Therapy-Sector-In-2023-A-Wave-Is-Coming--Are-We-Ready.

[88] Phacilitate, "Singapore's Cell Therapy Advanced Manufacturing Programme (STAMP)," 2 January 2020. [Online]. Available: https://www.phacilitate.com/singapores-cell-therapy-advanced-manufacturing-programme-stamp/.

[89] Medicines and Healthcare producys Regulator Agency, Department of Health and Social Care, "MHRA trialling pioneering stem cell robot that could transform the availability of life-saving cell therapies," 16 March 2023. [Online]. Available: https://www.gov.uk/government/news/mhra-trialling-pioneering-stem-cell-robot-that-could-transform-the-availability-of-life-saving-cell-therapies.

[90] AusBiotech, "Australia's regenerative medicine clinical trials database," 2021. [Online]. Available: https://www.ausbiotech.org.

[91] The University of Melbourne, "Melbourne MicroCerts," [Online]. Available: https://study.unimelb.edu.au/study-with-us/professional-development/micro-credentials. [Accessed April 2023].

[92] Therapeutic Goods Administration (TGA), Australian Approved Manufacturer: MI-2015-LI-10786-1, 2023.

[93] Australian College of Nursing, "Education: Graduate Certificates," [Online]. Available: https://www.acn.edu.au/education. [Accessed April 2023].

[94] J. Bulcha, Y. Wang, H. Ma, P. Tai and G. Gao, "Viral vector platforms within the gene therapy landscape," Signal Transduction and Targeted Therapy, vol. 6, p. 53, 2021.

[95] A. El-Kadiry, M. Rafei and R. Shammaa, "Cell Therapy: Types, Regulation, and Clinical Benefits," Frontiers in Medicine, vol. 8, 2021.

[96] Acumen Research and Consulting, "Biopharmaceutical Market Size Will Attain USD 853 Billion by 2030 growing at 11.3% CAGR - Exclusive Report by Acumen Research and Consulting," 28 September 2022. [Online]. Available: https://www.globenewswire.com/en/news-release/2022/09/28/2524510/0/en/Biopharmaceutical-Market-Size-Will-Attain-USD-853-Billion-by-2030-growing-at-11-3-CAGR-Exclusive-Report-by-Acumen-Research-and-Consulting.html.

[97] S. Kemler and A. Lohr, "Cell & Gene Therapy Investment Outlook In 2022 & Beyond," 21 February 2022. [Online]. Available: https://www.cellandgene.com/doc/cell-gene-therapies-investment-outlook-in-beyond-0001.

[98] GlobalData Healthcare, "Viral vector shortage is a problem for AZ's and J&J's Covid-19 vaccines, gene therapies," 19 March 2021. [Online]. Available: https://www.pharmaceutical-technology.com/comment/viral-vector-covid-19-vaccines/.

[99] Emily Whitehead Foundation, "Our Journey," 2023. [Online]. Available: https://emilywhiteheadfoundation.org/our-journey/.

[100] A. Muigai, "Expanding global access to gene therapies," Nature Biotechnology, vol. 40, pp. 20-21, 2022.

[101] Economist Impact, "Cell and Gene Therapies: Health system progress in moving from cutting edge to common practice," The Economist Group, 2022.

[102] Australian Government, Australian Digital Health Agency, "My Health Record," 2023. [Online]. Available: https://www.digitalhealth.gov.au/initiatives-and-programmes/my-health-record.

[103] House of Representatives Standing Committee on Health, Aged Care and Sport, "The New Frontier - Delivering better health for all Australians," Commonwealth of Australia, Canberra, 2021.

[104] Australasian Institute of Digital Health, "Delivering precision health by 2030: Outcomes of the Precision Health Roundtable held in Canberra 2022," Industry Genomics Network Alliance, Canberra, 2022.

[105] C. Iglesias-Lopez, A. Agusti, A. Vallano and M. Obach, "Current landscape of clinical development and approval of advanced therapies," Methods & Clinical development, vol. 23, pp. 606-618, 2021.

[106] U. Blache, G. Popp, A. Dunkel, U. Koehl and S. Fricke, "Potential solutions for manufacture of CAR T cells in cancer immunotherapy.," Nat Commun, vol. 13, p. 5225, 2022.

[107] A. Gajra, A. Zalenski, A. Sannareddy, Y. Jeune-Smith, K. Kapinos and A. Kansagra, "Barriers to Chimeric Antigen Receptor T-Cell (CAR-T) Therapies in Clinical Practice," Pharmaceut Med, vol. 36, no. 3, pp. 163-171, 2022.

[108] M. Lehmicke, "Manufacturing Cures: Infrastructure Challenges Facing Cell and Gene Therapy Developers," In ViVo Pharma Intelligence, 2019.

[109] E. Capra, A. Gennari, A. Loche and C. Temps, "Viral-vector therapies at scale: Today's challenges and future opportunities," 29 March 2022. [Online]. Available: https://www.mckinsey.com/industries/life-sciences/our-insights/viral-vector-therapies-at-scale-todays-challenges-and-future-opportunities.

[110] N. Pardi, M. Hogan, F. Porter and D. Weissman, "mRNA vaccines - a new era in vaccinology," Nature reviews. Drug discovery, vol. 17, no. 4, pp. 261-279, 2018.

[111] N. Ram, "The mRNA Manufacturing Advantage," 6 August 2022. [Online]. Available: https://themedicinemaker.com/manufacture/the-mrna-manufacturing-advantage.

[112] Australian Government, Department of Industry, Science and Resources, "Manufacturing a new future for Australia," 6 October 2020. [Online]. Available: https://www.industry.gov.au/news/manufacturing-new-future-australia.

[113] Government of Singapore, Health Sciences Authority, "Regulatory overview of cell, tissue or gene therapy products," Government of Singapore, Health Sciences Authority, 21 June 2021. [Online]. Available: https://www.hsa.gov.sg/ctgtp/regulatory-overview. [Accessed 23 May 2023].

[114] Pharma to Market, "Singapore Implements New Regulations for Cell, Tissue, and Gene Therapy Products (CTGTP)," Pharma to Market, 22 March 2021. [Online]. Available: https://www.pharmatomarket.com/singapore-implements-new-regulations-for-cell-tissue-and-gene-therapy-products-ctgtp/. [Accessed 23 May 2023].

[115] Advanced Cell Therapy and Research Institute, Singapore (ACTRIS), "Our Facilities, Training Lab," Advanced Cell Therapy and Research Institute, Singapore (ACTRIS), [Online]. Available: https://www.actris.sg/our-facilities/training-lab/. [Accessed 23 May 2023].

[116] Advanced Cell Therapy and Research Institute, Singapore (ACTRIS), "Our Services, Education and Training," Advanced Cell Therapy and Research Institute, Singapore (ACTRIS), [Online]. Available: https://www.actris.sg/our-services/education-and-training/. [Accessed 23 May 2023].

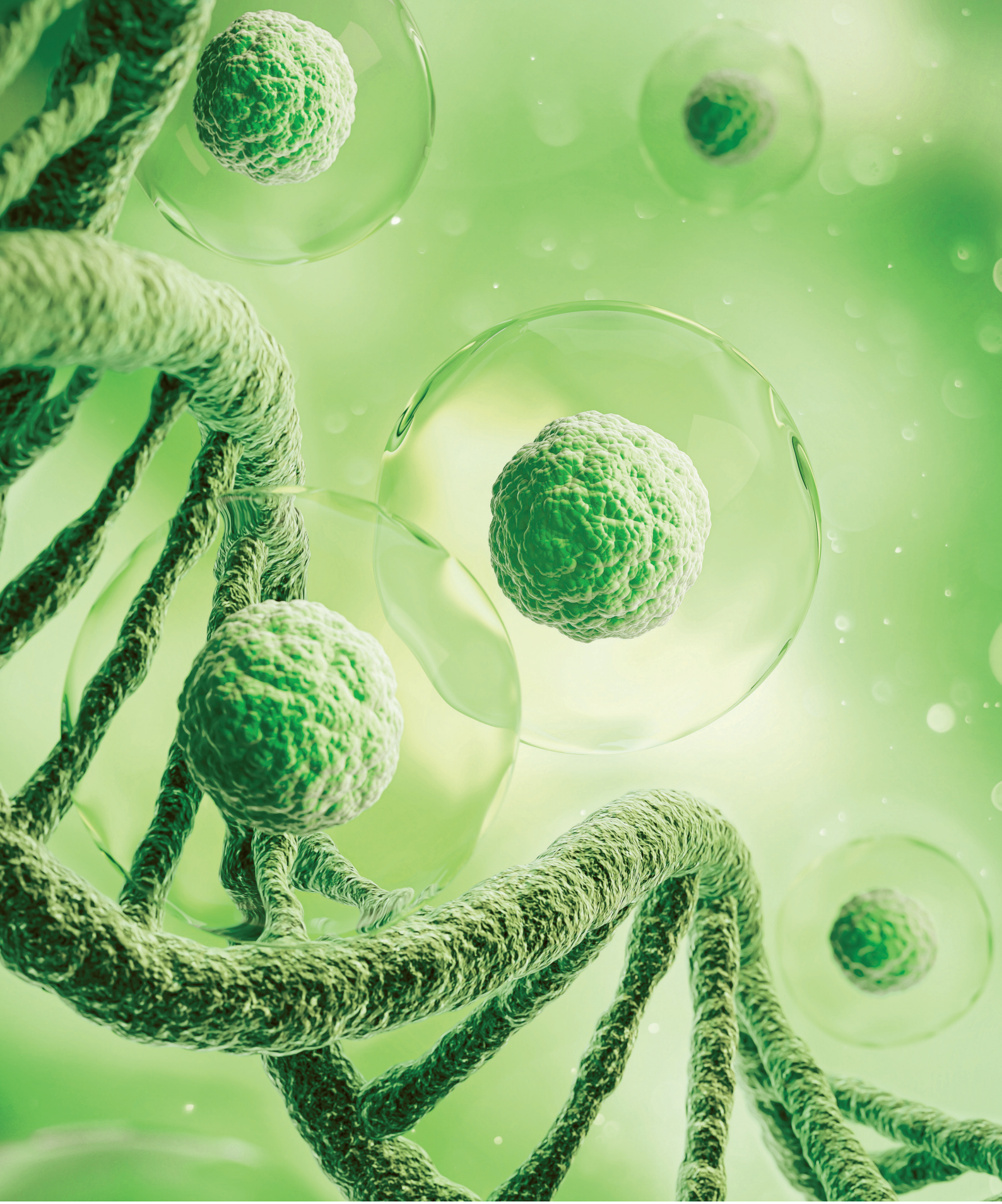
[117] J. Byrne, "Lonza invests in Singapore to support growth in mammalian biologics," BioPharma Reporter, 11 October 2021. [Online]. Available: https://www.biopharma-reporter.com/Article/2021/10/11/Lonza-invests-in-Singapore-to-support-growth-in-mammalian-biologics. [Accessed 23 May 2023].

[118] Lonza, "Singapore, Tuas," Lonza, 2023. [Online]. Available: https://www.lonza.com/about-us/our-locations/tuas-singapore. [Accessed 23 May 2023].

[119] Cell and Gene Therapy Catapult, "Construction initiated on Cell Therapy Catapult's £55 million cell and gene therapy manufacturing centre," Cell and Gene Therapy Catapult, December 2015. [Online]. Available: https://ct.catapult.org.uk/news/construction-initiated-on-cell-therapy-catapults-55-million-cell-and-gene-therapy-manufacturing-centre. [Accessed 23 May 2023].

[120] Cell and Gene Therapy Catapult, J Kerby, "Manufacturing for Advanced Therapy Medicinal Products," 19 May 2021. [Online]. Available: https://www.apsgb.co.uk/wp-content/uploads/2021/06/Jkerby-May-Acad.-Pharm-Sci-2021-v1.0-Slides-1.pdf. [Accessed 23 May 2023].

[121] Pharmaceutical Technology, "Autolus Therapeutics' CAR-T Cell Manufacturing Facility, Stevenage, UK," Pharmaceutical Technolog, 7 January 2022. [Online]. Available: https://www.pharmaceutical-technology.com/projects/autolus-therapeutics-facility-stevenage/. [Accessed 23 May 2023].



AusBiotech
AUSTRALIA'S BIOTECHNOLOGY ORGANISATION

ausbiotech.org
admin@ausbiotech.org