

MTPConnect
MedTech and Pharma Growth Centre

# Australia's Clinical Trials Sector

Advancing innovative healthcare and powering economic growth

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# Acknowledgments

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## Suggested citation

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# Contents

# Executive summary

Clinical trials are a critical step in the research and development process for new drugs, vaccines, medical devices and diagnostics. They can involve patients as well as healthy volunteers.[1] Patients in clinical trials get early access to these potentially life-saving new treatments or medical interventions, while at the same time advancing medical knowledge. More broadly, clinical trial activity contributes to the development of a thriving research culture within Australia's healthcare system and promotes Australia's international research profile.

A global reputation for clinical trials excellence has made Australia a 'go to' destination for companies wanting to conduct clinical trials and seen the clinical trials sector develop into one of the country's most important and valuable services exports.

This report, developed through extensive and whole-of-sector stakeholder engagement, provides a snapshot of the size and scope of Australia's clinical trials sector. It examines how the sector is performing and identifies opportunities for future growth.

**The clinical trials sector employs 8,000 Australians. More than 95,000 Australians participated in clinical trials in 2019, which saw around 1,880 trials started.**

**This activity saw $1.4 billion spent on clinical trials in Australia in 2019. The sector now generates more export revenue than construction, intellectual property charges and government services.**[2]

Over the reporting period, Australia has increased its competitive standing in early stage trials (Phase I and Phase II) and in oncology, pneumology, neurology and ophthalmology trials. Australia has maintained its share of late stage trials (Phase III and Phase IV).

| Metric | 2015* | 2019** | CAGR (2015–19) |
|---|---|---|---|
| Expenditure | $1.1 billion | $1.4 billion | 5% |
| Employment | 6,900 employees | 8,000 employees | 4% |
| Patient participation | Not reported | 95,000 | N/A |
| Number of trials started | c.1,360 | c.1,880 | 7% |
| Share of global industry sponsored trials | c.5% | c.5% | Nil |

Note: * As calculated in *Clinical Trials in Australia* (2017) report; ** As calculated in this report

[1] The term patient is used throughout the document for simplicity

[2] Department of Foreign Affairs and Trade, Trade and Investment at a Glance, 2020

COVID-19 lockdown measures and social distancing restrictions in 2020 posed significant challenges for clinical trials in Australia. Up to 90 per cent of trials run by some companies were put on hold and patients were unwilling to participate in trials during the pandemic period.[3] As a result, the number of clinical trials started in Australia contracted by 13 per cent; from 1,880 to 1,640 in early 2020.

However, Australia's effective pandemic response saw the sector rebound strongly from as early as May 2020, with a number of trials starting for COVID-19 vaccine candidates including Oxford-AstraZeneca, University of Queensland and Novavax.

Australia remains one of the few countries in the world with a sophisticated healthcare system where many COVID-19 related restrictions have been lifted or substantially eased. This has presented an opportunity to attract greater numbers of clinical trials to Australia that may have been conducted in the US, Canada or Europe where COVID-19 is more prevalent. International companies have viewed Australia's robust health and medical research infrastructure, skilled workforce and stability as an opportunity to relocate parts of their business to conduct clinical trials and other related R&D activities.

The strong growth of Australia's clinical trials sector over the past four years has been underpinned by five key drivers:

- **Medical experts and research staff of global standing:** Australia's investigators and clinical trial networks are among the best in the world.
- **Quality of research and data:** Australia has an excellent global reputation in science and research that is evidenced by high quality publications. Compliance with Good Clinical Practice (GCP) guidelines and high standards of data collection means results from Australian trials are regularly used to support submissions to international regulators including the United States' Food and Drug Administration (FDA) and the European Medicines Agency (EMA).
- **Specialised and dedicated infrastructure:** Australia's Phase I specialised service providers and sites are highly regarded. Significant new investment by government and other sector organisations over the last four years will continue to strengthen clinical trials infrastructure in Australia.
- **Streamlined regulatory and ethics approval:** The convenience and speed of the Therapeutic Goods Administration (TGA) – Clinical Trial Notification (CTN) scheme is an advantage for Phase I trials relative to other countries. Reforms, including the rollout of the National Mutual Acceptance (NMA) scheme, are driving efficient ethics processes that are globally competitive.
- **R&D Tax Incentives:** The tax relief provided by the Research and Development Tax Incentives (R&DTI) enhances Australia's cost competitiveness as a destination for clinical trials, particularly for small and medium sized entities.

These drivers were first identified in MTPConnect's *Clinical Trials in Australia* (2017) report and have been further strengthened and enhanced.

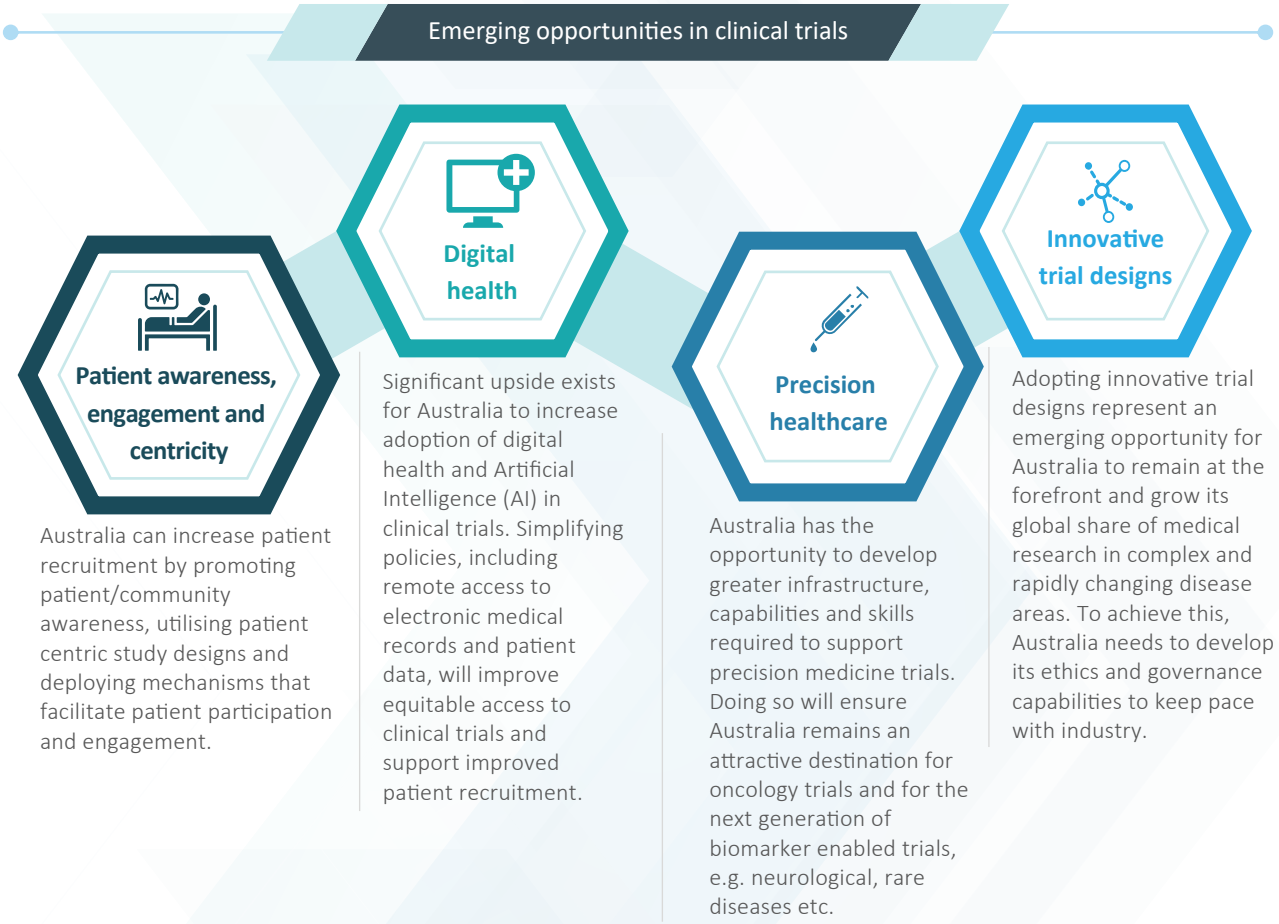
Over the next 10 years, four emerging opportunities have been identified for Australia. These opportunities relate to innovations in therapies and clinical trial design and conduct and include:

- focusing on patient awareness, engagement and centricity to increase recruitment

[3] MTPConnect, COVID-19 Impact Report, June 2020

- applying digital health to achieve efficiencies and enhanced patient recruitment
- further building our capability in precision healthcare in clinical trials
- investing in capability to undertake innovative clinical trial designs.

Being at the forefront of these innovations will enable Australia to capture a greater share of global clinical trials and grow the sector more rapidly.




In light of the above emerging opportunities identified through the stakeholder consultations, there are four key priorities for Australia's clinical trial sector. Addressing these priorities will be critical to enable Australia to maintain and further strengthen the growth trajectory of its clinical trials sector over the next five to 10 years.

## Key priorities for Australia's clinical trial sector

1

### Continue to optimise efficiency in ethics and governance processes

NMA has contributed to some improvement in ethics processes however there is scope for further progress towards a true, single ethical review. Site governance approval processes can still be lengthy and highly variable from site to site and study to study. The implementation of the Clinical Trials Governance Framework (CTGF) offers hope for streamlining these processes, decreasing start-up time and embedding clinical trials as a core part of service delivery for hospitals.

2

### Grow the clinical trials workforce and develop its capabilities

There is a need to address the shortage of experienced Clinical Research Associates (CRAs) and Clinical Trial Coordinators (CTCs) across the sector. These highly skilled staff are crucial to the day-to-day management and operation of clinical trials and if unaddressed, the shortages have the potential to severely constrain the sector's ability to expand.

3

### Enhance patient recruitment per site to improve site economics for clinical trials

There is a need to improve the ease and scale of patient recruitment at each site to help manage the cost of conducting clinical trials on a per patient basis and remain globally competitive. Patient awareness and engagement as well as patient centric approaches, including tele-trials and the adoption of digital health technologies will help improve patient recruitment per site.

4

### Enhance sector metric and reporting

Whilst there have been various initiatives to improve the collection of clinical trials data over the past four years, there is still a need to improve the quality and level of detail of data captured and to systematise the reporting and tracking of key performance indicators.

Australia's clinical trials sector is large and vibrant. It contributes to health and medical research and better health outcomes. It sustains thousands of jobs and drives economic growth – and the outlook for the sector is overwhelmingly positive.

The COVID-19 pandemic has provided a platform upon which Australia can further strengthen its reputation as a clinical trials destination of choice. In addition to industry investment, government funded investments through the National Health and Medical Research Council (NHMRC), Medical Research Future Fund (MRFF) and the Modern Manufacturing Strategy (MMS) will continue to help grow the clinical trials sector over the coming years.

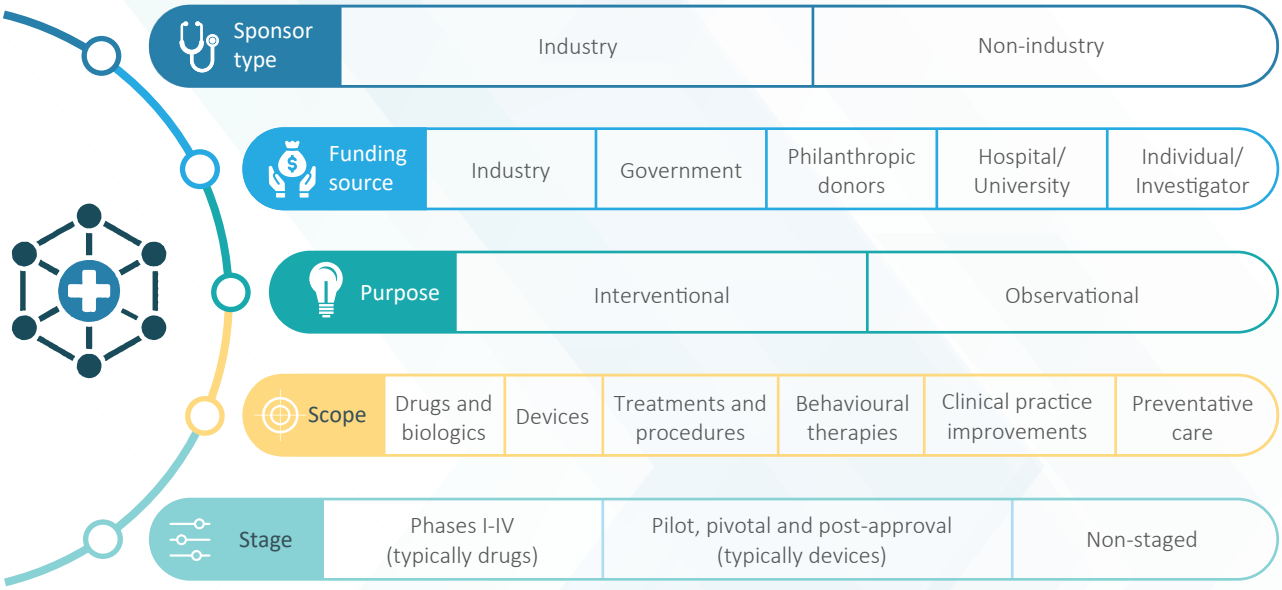
With a steadfast focus on the four priority areas outlined in this report, Australia is well placed to leverage its competitive advantages and continue growing its clinical trials sector, outcompete other countries for foreign investment, create skilled jobs and deliver sustained health benefits for Australians.

# 1. Introduction and context

## Introduction to clinical trials

Clinical trials are an integral part of the innovation process in healthcare. They generate evidence to inform stakeholders about the safety, efficacy and cost-effectiveness of the investigated strategy on health outcomes. Clinical trials are a regulatory necessity prior to a treatment or medical intervention being made available to patients in settings outside of clinical trials.

Clinical trials can take many forms, with widely varying features and characteristics depending on the unique circumstance of each study. The clinical trials defined in this report can be segmented based on five key parameters as illustrated in the figure below.




They are sponsored and funded by a wide range of stakeholders including industry, healthcare professionals, government, hospitals, philanthropic donors and patients. Trials span interventional through to observational studies and a wide range of treatment strategies, from medical devices to drugs to behavioural therapy. Clinical trials are also typically categorised into stages. Most drug trials are classified into one of four phases (I-IV) that indicate the stage of development. Medical device trials sometimes fall within one of the four trial phases, but often go through "stages" instead. These stages might include a First Time In Human (FTIH) study, Traditional Feasibility study (similar to combined Phase I and II trials), Pivotal study (to confirm clinical efficacy and risks, similar to traditional Phase III trials) and Post-approval studies (to determine long term effectiveness and risks, similar to traditional Phase IV trials).

Further background information on these key parameters can be found in Appendix 2.

## Sector participants

A range of sector participants play key roles in the conduct of clinical trials in Australia. These participants include medical technology, biotechnology and pharmaceutical (MTP) companies, contract research organisations (CROs), medical research institutes (MRIs), trial sites/units, universities and clinical trial networks as illustrated in the figure below. Representatives from each of these types of organisations contributed to this report through sector consultations and by providing data not available in the public domain. Their input provided a broad and representative range of perspectives on key issues and opportunities for Australia to strengthen and grow the clinical trials sector. These issues and opportunities are outlined in this report in subsequent chapters.

| Sector organisation | Description of role |
|---|---|
| **MTP companies (industry)** | MTP companies are the main sponsors of clinical trials in Australia in value terms. While most medical technology companies typically manage clinical trials in-house, biotechnology and pharmaceutical companies typically use a combination of in-house and outsourced CROs to manage trials. Collectively, MTP companies and CROs spend the most on clinical trials. |
| **Contract Research Organisations (CROs)** | CROs are service providers that design, plan and manage clinical trials on behalf of sponsors (typically MTP companies). They can range from small, niche local providers to large, full service multinational companies. |
| **Medical Research Institutes (MRIs)** | MRIs undertake medical research (including the conduct of clinical trials) focused on one or more therapeutic or research areas. MRIs are often intertwined with hospitals, universities and clinical trial networks. As of 2020, the Association of Australian Medical Research Institutes (AAMRI) reported that 57 of its member organisations (42 independent MRIs and 15 university- and hospital-based institutes) were involved in 1,329 clinical trials.[4,5] Despite contributing lower expenditure on clinical trials, MRIs contribute the highest number of trials. |
| **Trial sites/units (public/private hospitals, clinics, specialised units, GP practices)** | The main role of a trial site is to host trials and provide clinical staff for the conduct of trials on site. Hospitals are involved in clinical trials both as sponsors and in recruiting, treating and monitoring patients in trials on behalf of other sponsors. Private clinics are less likely to sponsor trials, however, they are involved in recruiting patients and conducting trials. |
| **Universities** | While not usually trial sites for commercial studies, universities are typically involved in early-stage trials where the financial trade-offs are too great for MTP companies or the level of investment is not prohibitive, or in trials relating to clinical practice, behavioural therapies and preventative care (rather than new drugs/devices or discoveries). |
| **Clinical trials networks** | A clinical trial network is a group of researchers, clinicians and academics who share infrastructure to conduct multi-centre clinical trials and facilitate knowledge-sharing between researchers in a field. They are typically virtual and do not have any physical infrastructure. For example, the Australian Clinical Trial Alliance (ACTA) currently has 250 members.[6] |

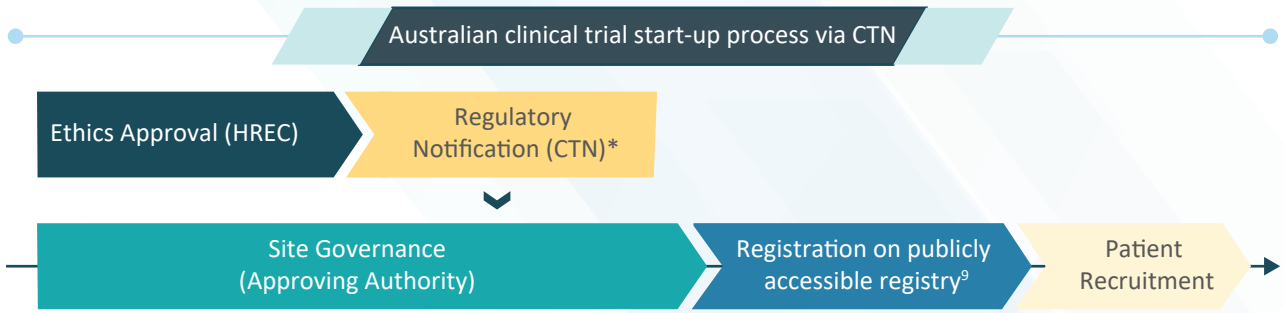
[4] Reflects industry sponsored and non-industry sponsored clinical trials

[5] AAMRI, Australian Medical Research Institutes – The AAMRI Report 2020, October 2020

[6] ACTA, About ACTA website, accessed April 2021

## Overview of Clinical Trial Start-Up Process

In Australia, a series of steps and procedures must be undertaken before a clinical trial can be initiated. There are two TGA schemes under which clinical trials involving unapproved therapeutic goods may be conducted, the Clinical Trial Notification (CTN) Scheme and the Clinical Trial Approval (CTA) Scheme. The CTA Scheme is an approval process however almost all clinical trial sponsors that supply unapproved drugs and devices for human use go through the CTN scheme[7] (see figure below). The scheme defers the review of each clinical trial to the relevant Human Research Ethics Committee (HREC).[8] With advice from the HREC, and after regulatory notification of the intent to sponsor a clinical trial is submitted to the TGA, the institute or organisation which is responsible for the conduct of the trial becomes the ultimate approving authority. In public health organisations, site governance review occurs through the Site-Specific Assessment (SSA) process. While the Therapeutic Goods Administration (TGA) will accept the CTN form submission while the sponsor is obtaining the necessary endorsements, typically in practice the CTN is submitted after HREC approval and before governance approval. It is the responsibility of the sponsor to ensure that all relevant approvals are in place before commencement of the trial.[10]




Note: * The TGA encourages all parties to be in agreement as to when the CTN form should be submitted. Clinical trials that do not involve 'unapproved' therapeutic goods are not subject to requirements of the CTN or CTA schemes

## Context for the 2021 report

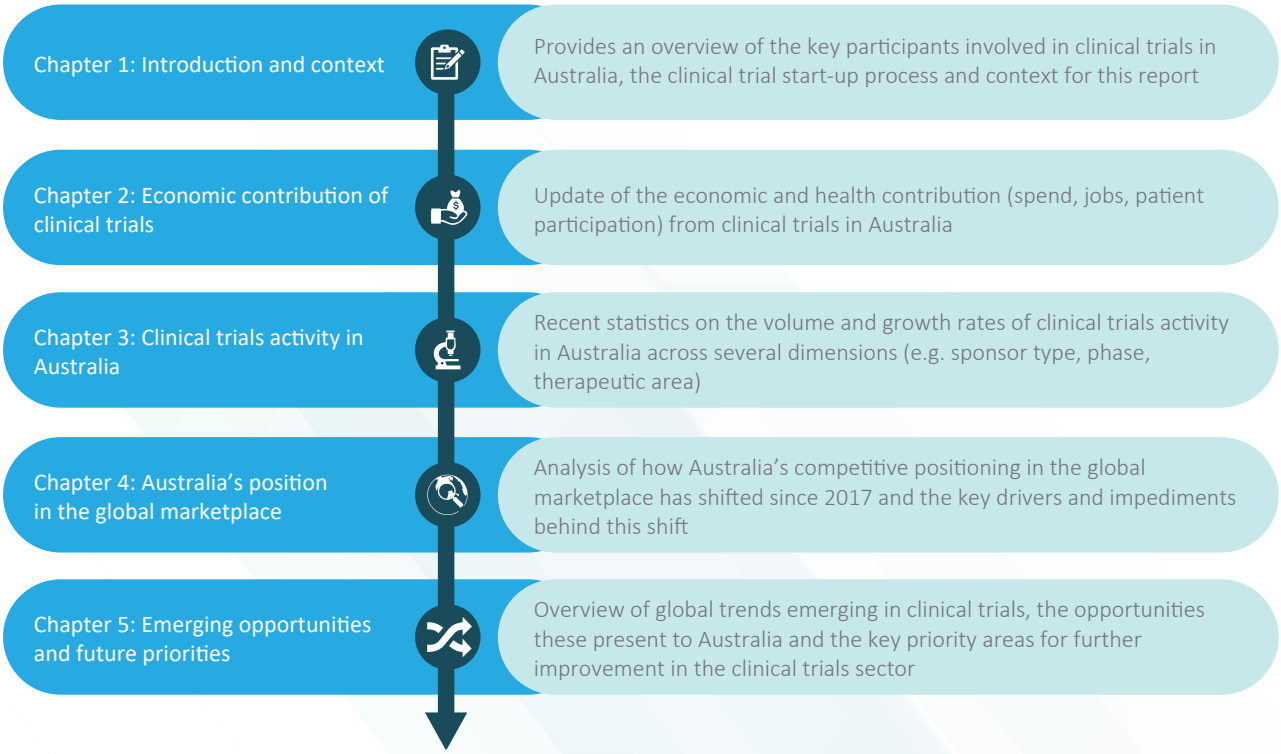
In 2017, MTPConnect carried out a holistic and comprehensive review of the state of the clinical trials sector. Four years on, this report builds on the *Clinical Trials in Australia* (2017) report to understand how the sector has performed and take stock of initiatives and programs rolled out in the intervening years to identify emerging opportunities and issues that should serve as priorities for future activities. The report is structured as follows:

[7] The remaining unapproved drugs and devices must pass through the CTA scheme which requires TGA to evaluate summary information about the product including relevant scientific data. For this reason the CTA scheme is a slower than the the CTN scheme. This CTA scheme is relevant for certain Class 4 biologicals, which typically contribute fewer than five trials annually. For this reason, it is not discussed at length in this report

[8] NHMRC, National Statement on Ethical Conduct in Human Research, 2018

[9] National Statement on Ethical Conduct in Human Research 2007 (updated in 2018) requires clinical trials are registered on a publicly available registry (e.g. Australia and New Zealand Clinical Trials Registry [ANZCTR], ClinicalTrials.gov) before the recruitment of the first participant.

[10] Department of Health, Therapeutic Goods Administration, Clinical Trials website, November 2020




## Methodology

This report has drawn on a wide range of information sources, both quantitative and qualitative in nature to create a comprehensive view of the sector. Publicly available information sources have been supplemented with surveys and consultations to obtain important insights (such as the economic contribution of clinical trials). The key information sources by topic are outlined in the table below.

| Topic | Information source |
|---|---|
| **Economic contribution of clinical trials** | • MTPConnect industry survey<br>• Desktop research and analysis |
| **Clinical trials activity in Australia** | • ANZCTR, which includes daily data feeds from ClinicalTrials.gov |
| **Australia's position in the global market** | • ClinicalTrials.gov |
| **Emerging opportunities and future priorities** | • Stakeholder consultations<br>• Desktop secondary research |

The economic contribution of the clinical trial sector was primarily drawn from a MTPConnect industry survey. Respondent companies reported starting 237 trials in 2019, a number that represents 39 per cent of the total 601 industry-sponsored clinical trials started in that year. The respondents included MTP companies, CROs and members of the RDTF, the majority of whom are members of AusBiotech, the Medical Technology Association of Australia (MTAA), Medicines Australia (MA). A detailed overview of

the methodology used to calculate clinical trial expenditure, employment and participation can be found in Appendix 3.

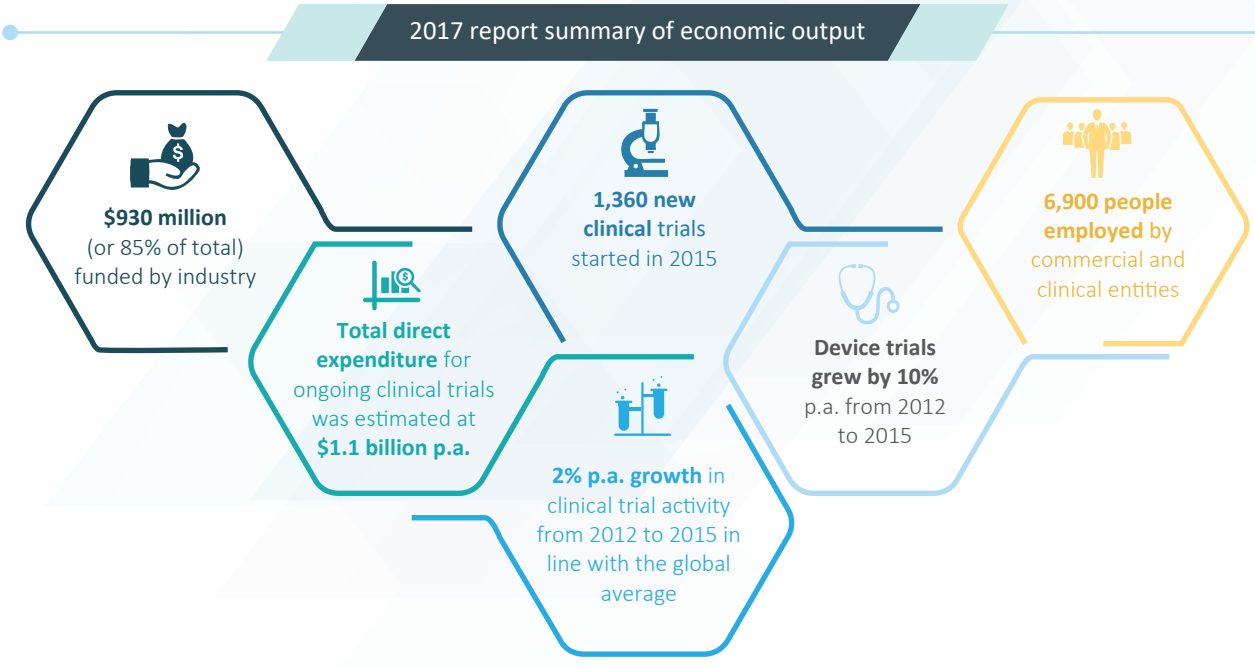
The clinical trial metrics are based on data provided by the ANZCTR, Australia and New Zealand's registry for clinical trials. In 2017, ANZCTR staff estimated that the registry captured 75-80 per cent of clinical trial activity in Australia. As the number of trials that are never registered is unknown, an estimate has not been provided in 2021. However, for trials that have been registered, the ANZCTR captures around 96 per cent of studies recruiting in Australia.

Australia's industry-sponsored clinical trial activity has been compared to global data from ClinicalTrials.gov, as it is widely considered the most globally complete clinical trial registry.[11] This report compares the industry-sponsored clinical trial activity across 15 countries, segmented by phase and therapeutic area. Detailed data definitions of ANZCTR and ClinicalTrials.gov and their scope can be found in Appendix 2.

This report has also been informed and enriched through consultations with 61 sector stakeholders across the MTP sector (details can be found in Appendix 1).

## Overview of the *Clinical Trials in Australia* (2017) report

The *Clinical Trials in Australia* (2017) report has served as an important reference for the sector. It quantified the significant contribution the conduct of clinical trials makes to the Australian economy and outlined Australia's competitive position in the global clinical trial sector, including highlighting key priorities for sector action. The key findings of that report are summarised below to provide context for sector performance and priorities and to enable comparisons to more recent performance.




[11] Tony Tse, et al., How to avoid common problems when using ClinicalTrials.gov in research: 10 issues to consider, *British Medical Journal*, March 2018

## Global competitive position

Australia was recognised as having a strong competitive position in conducting industry-sponsored clinical trials in complex therapeutic areas (e.g. oncology), in trials with complex design (e.g. adaptive trial design) and in early stage trials (e.g. Phase I and II drug trials and feasibility/FTIH medical device trials). The key drivers for this strong position were identified as:

- high standards of internationally recognised research
- leading medical experts with global standing
- high quality infrastructure
- comparable cost of conducting trials relative to global markets.

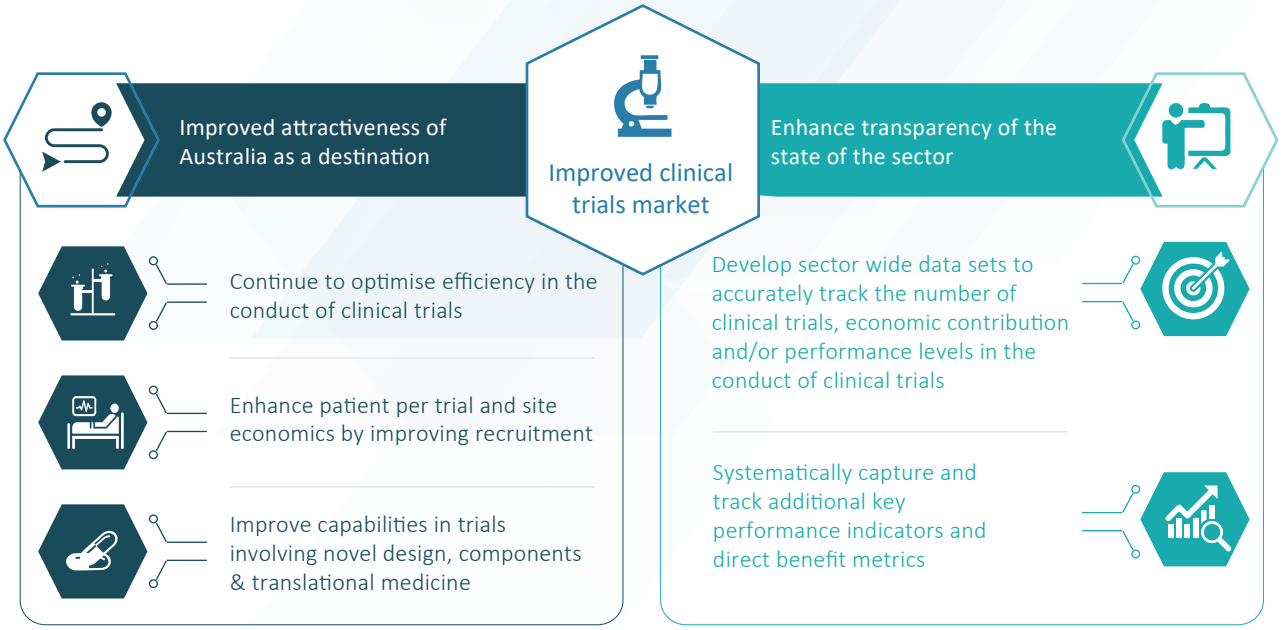
In trials with lower complexity in design, or that required features such as large patient pools, Australia's position was weaker due to increasing competition from countries in Asia Pacific, Eastern Europe or Latin America with better access to larger patient pools and lower cost bases.

In 2017, the main impediments or threats to Australia's competitive position in clinical trials were:

- complexity and variability in site governance approvals that resulted in less predictable trial start-up times
- low participant recruitment numbers per site which in turn drives higher cost per patient
- a need to invest to develop capability in specialised, high risk and innovative trials to remain a leading trial destination.

## Priority areas for improvement

While Australia enjoyed a strong position in the global clinical trials market in 2017, the report also identified a number of key priority areas for further improvement under two overarching themes as summarised in the figure below.



# 2. Value derived from clinical trials in Australia

## Overview of value derived from clinical trials

Clinical trials make a significant contribution to Australia, delivering an array of direct and indirect health and economic benefits, including job creation, patient health benefits and sector skills development as highlighted by the figure below.


The economic activity generated by clinical trials includes investment (expenditure on clinical trials), jobs created and avoided cost to the healthcare system. Avoided healthcare costs come in the form of treatment costs that are typically borne by the trial sponsor that would have otherwise been borne by Australia's healthcare system or patients had they not participated in a clinical trial.

Trial activity triggers a range of flow-on benefits. Patients benefit through early access to new treatments and better care outcomes. The sector benefits through a strengthened research ecosystem and culture, improved standards of care and a more highly skilled workforce.

The wider economy also experiences a variety of multiplier effects, including increased personal spending by healthier trial patients and those employed in the many jobs supported by the sector. Increased personal spending is funded in part by increased workforce participation rates of healthier clinical trial patients. This in turn supports government expenditure through the tax revenue associated with personal income.

In parallel to these economic multiplier effects, clinical trials activity also contributes to the development of a thriving and innovative research culture within Australia's healthcare system and further elevates Australia's international research profile. National clinical trials activity also supports and encourages the development of more local MTP companies, which in turn increases clinical trials activity and attracts more research and associated research expenditure to Australia. This national benefit compounds the quantum of direct economic activity, benefits to patients and the sector, as well as multiplier effects to the wider economy, completing the virtuous cycle.

This chapter analyses the economic and health benefits of clinical trials derived by Australia in 2019 and reveals how these have changed since the publication of the *Clinical Trials in Australia* (2017) report. 2019 was chosen as the most recent annual datapoint not effected by COVID-19 to enable conclusions to be drawn about long-term trends in performance. A discussion of the impact of COVID-19 on clinical trials activity in 2020 has been included in *Chapter 3 – Clinical Trials Activity in Australia*.

## Economic Activity

### Clinical trials expenditure and funding

Clinical trials contributed approximately $1.4 billion to the Australian economy through direct expenditure or investment in 2019, up 6.5 per cent p.a. from $1.1 billion in 2015.[12] This level of expenditure represents approximately 25 per cent of total spending on health research – this relative percentage has remained relatively constant across the period.[13, 14]

The expenditure on industry-sponsored clinical trials has increased 4.8 per cent p.a. since 2015 to approximately $1.1 billion in 2019, up from $0.9 billion. This growth in economic value can be explained predominantly by the growth in the number of industry-sponsored trials, which grew at 6 per cent p.a. from 2015 to 2019 as outlined in *Chapter 3 – Clinical Trials Activity in Australia*.

Expenditure by non-industry organisations, such as universities, hospitals and MRIs was estimated as $288 million in 2019, up from $165 million in 2015. Most of the non-industry expenditure came from government sources, including:

- $157 million of expenditure funded by competitive grants from the NHMRC, the Australian Government's primary health and medical research funding agency. Expenditure funded by the NHMRC grew at 12 per cent p.a. between 2015 and 2019.
- $35 million of expenditure funded by the MRFF directly through its 'Clinical Trials Activity' initiative. The MRFF, established in 2015, is a $20 billion long-term initiative supporting Australian health and medical research. It aims to transform health and medical research and innovation to improve lives, build the economy and contribute to health system sustainability.[15] The estimated $35 million represents the minimum MRFF funding going to support clinical trials. The MRFF also funds R&D and commercialisation initiatives where clinical trial expenditure cannot be easily

[12] This estimate of the economic contribution of clinical trials includes expenditure on conducting the trials but excludes expenditure on supporting infrastructure and overheads (e.g. capital equipment, specialised environments or trial network support services). This estimate also excludes the economic value of flow-on benefits or multiplier effects

[13] Health research spending was $5.6 billion in Australia in 2017–18

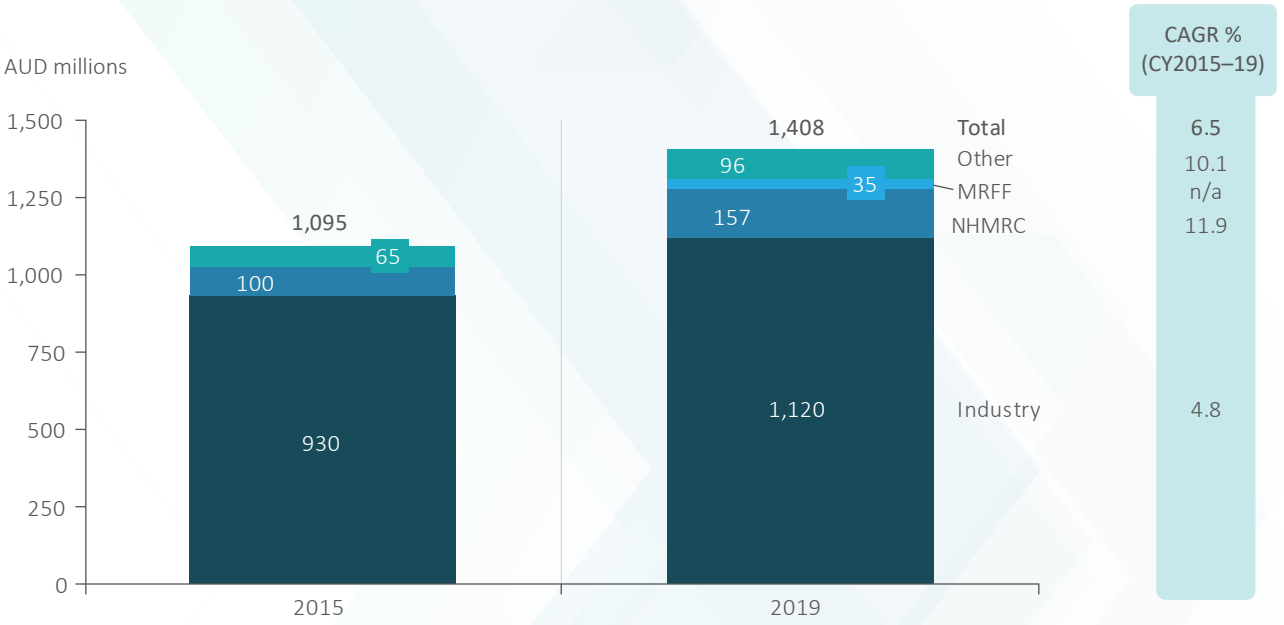
[14] AIHW, Health expenditure Australia 2017–18, September 2019

[15] Department of Health, Medical Research Future Fund website, accessed April 2021

apportioned. Examples of these MRFF initiatives include the BioMedTech Horizons initiative,[16] Biomedical Translation Bridge initiative,[17] Targeted Translation Research Accelerator initiative[18] and the eight MRFF research missions, each of which funds clinical trials directly and indirectly.[19]

- $96 million of expenditure by universities, hospitals and MRIs came from philanthropic sources (bequests, donations and fundraising), other government sources and grants (e.g. state governments, trusts and foundations) and investment income (e.g. interest). This funding grew at approximately 10 per cent p.a. since 2015.

**Clinical trial expenditure (2015 vs 2019)**




Source: MTPConnect industry survey; NHMRC; MRFF; AAMRI; L.E.K. analysis

## Avoided healthcare and drug costs

Industry-sponsored clinical trials typically pay for the treatment of participating patients on the experimental arm (and sometimes the comparator arm) during the trial as part of their overall clinical trial expenditure. The healthcare system therefore reduces the costs incurred for treating patients participating in clinical trials. These costs include payment for items such as pathology, pharmacy, imaging (X-ray, magnetic resonance imaging), lab tests and drug costs. This report has not quantified the specific avoided healthcare and drug costs associated with clinical trials as it would involve estimating the specifics trial by trial, taking into account the therapy area and the specific commercial arrangements of trials sponsors. However, the $1.4 billion estimated expenditure on clinical trials does include the costs of investigational drugs and devices provided by sponsors in clinical trials.

[16] Department of Health, BioMedTech Horizons initiative, December 2019
[17] Department of Health, Biomedical Translation Bridge initiative, December 2019
[18] Department of Health, Targeted Translation Research Accelerator initiative, December 2019
[19] Department of Health, Research Missions, January 2020

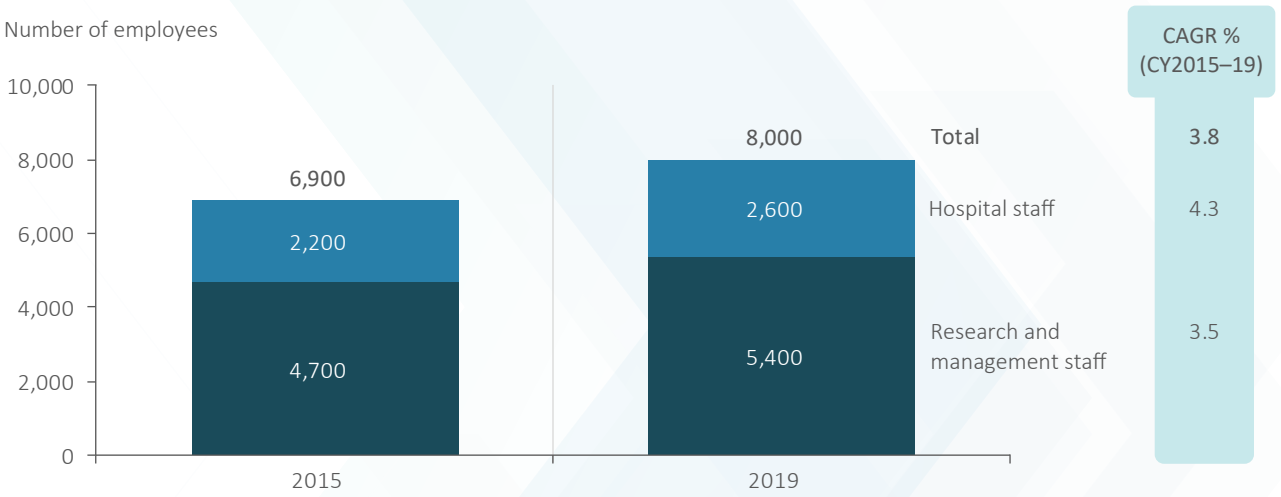
### Clinical trials employment

Wages and salaries account for a large proportion of clinical trial expenditure, directly supporting high value add jobs. At least 8,000 people were directly employed by the clinical trials sector in 2019, comprised of two categories:

- clinical research and management staff employed at MTP companies, service providers such as CROs, MRIs and academic trial centres (5,400 employees[20])
- clinical staff employed within hospitals, clinics and other trial sites (2,600 employees[21])

Employment within the clinical trials sector has expanded at a rate of almost 4 per cent p.a. since 2015 as shown by the figure below.

**Clinical trial employment (2015 vs 2019)**




Source: L.E.K. Survey and Analysis; L.E.K. and MTPConnect interviews with sector participants

It is worth noting that the total employment figure may be even higher because the employment estimate does not consider independent/solo contractors or clinical staff employed within hospitals, clinics and other trial sites that may have an indirect role in clinical trials (e.g. pharmacy, pathology, hospital staff at imaging facilities, etc.).

## Flow-on benefits

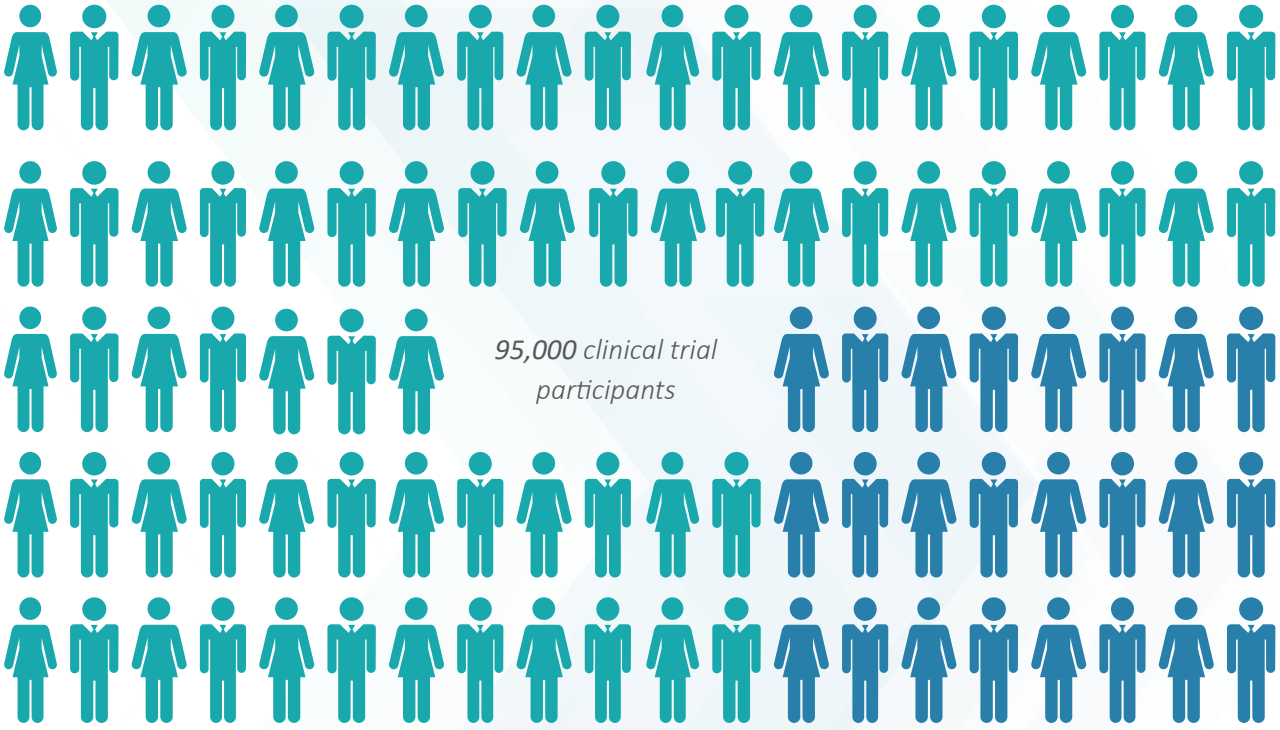
### Participant/Patient benefits

Patients are the main beneficiaries of clinical trials. This is particularly the case for patients that have already received standard treatments with unsuccessful outcomes and who may benefit from new treatments that are otherwise unavailable in Australia. Publicly available data on the number of

[20] MTPConnect industry survey, 2021
[21] Based on L.E.K. and MTPConnect interviews with sector participants

Australians who participate in clinical trials each year is limited. An estimate of total patient participation in clinical trials has been derived by combining MTPConnect industry survey results with an analysis of patient numbers by trial phase and therapy area. Using this method, an estimated 95,000 patients participated in clinical trials in Australia in 2019.[22] While global and Australian patient participation datasets are not directly comparable, it is apparent that there is an opportunity to significantly increase patient participation in Australia. For instance, there are at least four times as many patients participating in clinical trials per capita in the United Kingdom compared to Australia.[23,24]

**Clinical trial patient participation (2019)**




*71,000 non-industry-sponsored clinical trial participants*

*24,000 industry-sponsored clinical trial participants*

Patients involved in clinical trials may receive enhanced or innovative treatments under development that typically lead to better patient outcomes. Specifically, clinical trial patients can often expect to gain additional QALY, a metric used to evaluate healthcare interventions.[25] QALY, a measure of both the quality and quantity of a patient's life, is extended for clinical trial participants in two ways:

- For some patients, receiving early access to new interventions results in better clinical outcomes with fewer side effects.

[22] MTPConnect industry survey, 2021

[23] National Institute for Health Research (NIHR), Annual Report 2018/19, March 2020

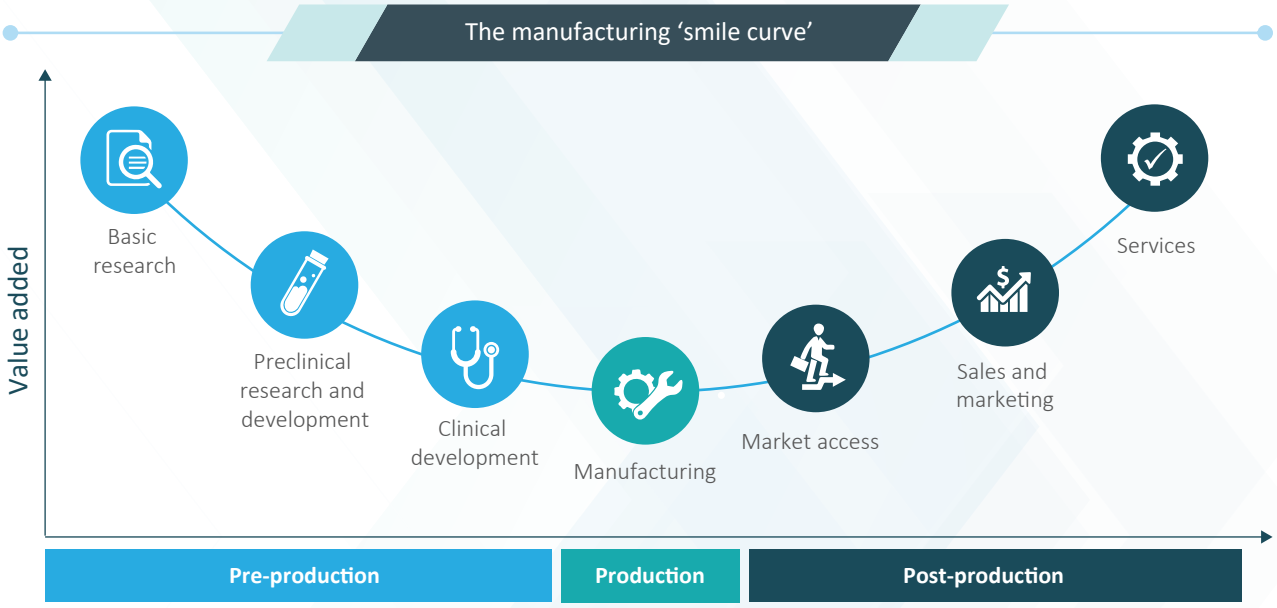
[24] The NIHR reports 1,015,487 people took part in NIHR-supported health and social care research studies and were recruited by the Clinical Research Network (a sub-set of all UK clinical trials), while the population of the United Kingdom is roughly 2.6x that of Australia

[25] Morro Touray, Estimation of Quality-adjusted Life Years alongside clinical trials: the impact of 'time-effects' on trial results, *Journal of Pharmaceutical Health Services Research*, 2018

- Many patients also receive closer clinical surveillance and better clinician adherence with evidence-based care, which ultimately leads to better health outcomes.[26]

## Sector benefits

Clinical trials are a critical part of the broader MTP 'manufacturing ecosystem' which spans basic research through to clinical development, production and the services required to provide access and administer appropriate therapies and solutions to patients. The Australian Government's MMS, particularly the supporting Medical Products National Manufacturing Priority road map, emphasises that value can be achieved across the full ecosystem by building strength in the pre-production (including clinical trials) and post-production phases as highlighted in the 'manufacturing smile' figure[27] below.




A strong and globally competitive clinical trial sector generates broader benefits for the healthcare ecosystem, including:

- **Enhancing clinical capability and clinical practice development –** clinical trials produce new treatments and improvements to standards of care that deliver enduring benefits to Australia's healthcare system. The clinical staff involved in trials gain experience with innovative therapies that will become the future standard of care. This could lead to faster adoption and application of the latest R&D in clinical practice.
- **Elevating research capability –** the funding of trials contributes to infrastructure availability at clinical sites and supports further R&D in healthcare. In parallel, Investigator Initiated Trials (IITs) and high-quality academic research build further capability and capacity, elevate care standards in Australia and contribute to the international reputation of Australian medical experts, investigators and research staff.

[26] Sumit Majumdar, Better outcomes for patients treated at hospitals that participate in clinical trials, *Archives of Internal Medicine,* March 2008

[27] Adapted from Australian Government, Make it Happen, The Australian Government's Modern Manufacturing Strategy, 2020; and the Medical Product National Manufacturing Priority road map, 2021

- **Supporting manufacturing of medical products –** Australia is growing the medical product manufacturing industry, as one among six National Manufacturing Priorities within the $1.5 billion MMS. Clinical trials are an important part of the commercialisation pathway for medical products. Clinical trials provide an avenue to test and validate Australia's competitiveness in scaling up manufacturing capabilities in areas of research such as vaccine production, stem cell and gene therapies. Large-scale production can be anchored to the location where clinical trials take place. Providing products to local clinical trials can also be an important source of income for Australian medical manufacturers. The Queensland Government recently announced a partnership with Vaxxas to establish a facility at Northshore Hamilton in Brisbane for the manufacture of devices for Phase II and III clinical studies.[28] The Vaxxas-Queensland partnership is an example of utilising clinical trials as a stepping stone to develop commercial-scale manufacturing capability. Vaxxas has plans to leverage this facility to build a commercial scale production line over the next few years working with global pharmaceutical, and production and packaging companies.

A strong clinical trials sector enhances the reputation of Australia's clinical capability, medical research and manufacturing expertise. This in turn will help attract more clinical trial activity and investment, creating a virtuous cycle where economic and sector benefits are compounded over time.

## Multiplier effects

The increase in economic activity and flow-on benefits of conducting clinical trials ripple through the broader economy, multiplying the direct economic impact of these trials. Clinical trials galvanise the economy through the multiplier effect in two main ways:

- Private consumption expenditure by the 8,000 people employed in clinical trials. Not only would many of these people not be employed without the foreign investment in clinical trials, but they would also not be spending their income on discretionary goods and services. In addition, the income taxes they pay and expenditure of those tax dollars would be lost.
- Consumption by trial patients who subsequently live and work longer (and take fewer sick days). Incremental to the private consumption by those employed in the industry, trial patients who live and work longer will spend more on discretionary and non-discretionary goods and services.

Previous studies on the economic multiplier of spend on clinical trials range in their approach. Some studies base their calculations on the benefits from healthcare, while others focus on employment. Across these studies, the multiplier estimated could be as high as 1 to 6 times the direct costs incurred.[29,30,31] However, getting an accurate estimate of the multiplier effect across economic activity and flow-on benefits for clinical trials is challenging. Therefore, multiplier effects are excluded from the overall estimate of clinical trial economic activity in this report, as was the case in the *Clinical Trials in Australia* (2017) report.

[28] Translational Research Institute, Aussie invention propelling needle-free vaccine delivery, September 2020

[29] Medicines Australia and PwC, The economic contribution of the innovative pharmaceutical industry to Australia: Economic footprint of the innovative pharmaceutical industry, February 2018

[30] The Pharmaceutical Research and Manufacturers of America (PhRMA) and TEConomy Partners, LLC, The Economic Impact of The U.S. Biopharmaceutical Industry: 2017 National and State Estimates, December 2019

[31] ACTA and Quantium Health Outcomes, Economic evaluation of investigator-initiated clinical trials conducted by networks, July 2017

# 3. Clinical trials activity in Australia

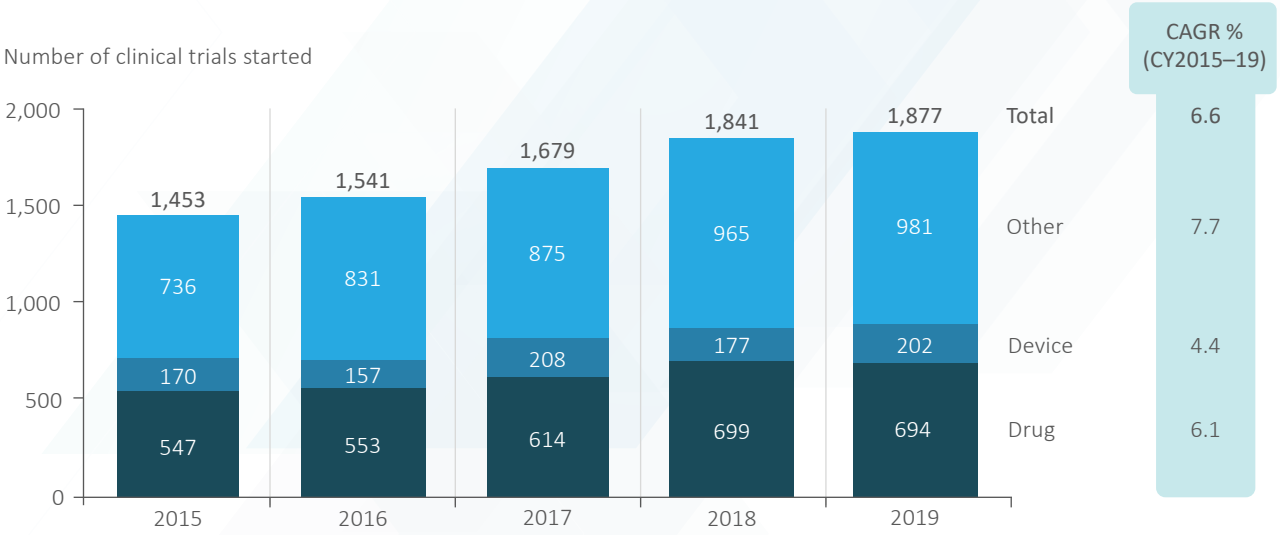
This chapter presents statistics and trends related to the volume and growth of clinical trials activity in Australia over the period between 2015 and 2020. The ensuing analysis is based on ANZCTR data, the most complete data set of clinical trial activity in Australia. Additionally, the global data source, ClinicalTrials.gov, has been used to undertake international comparisons which can be found in *Chapter 4 – Australia's Position in the Global Marketplace.* An overview of data definitions, methodology and limitations can be found in Appendix 2.

This report predominantly uses the number of clinical trials starting, rather than the overall number of clinical trials operating. The two differ as many trials run longer than one year. While the number of clinical trials ongoing is a more accurate measure of economic value derived from clinical trials, the number of clinical trials starting is a reasonable proxy indicator. In the interests of understanding underlying trends, data from 2020 has been excluded from the analysis to isolate the short-term impact of COVID-19. A separate discussion of the impact of COVID-19 on 2020 clinical trials activity is included later in this chapter.

## Overview of clinical trials activity (2015–2019)

In 2019, ANZCTR recorded 1,877 clinical trial commencements in Australia following a period of solid and steady growth. The number of clinical trials grew by 6.6 per cent p.a. between 2015 and 2019, reflecting across the board growth in the various types of clinical trials. In the same 2015–19 period, drug trials were up 6.1 per cent p.a., device trials were up 4.4 per cent p.a. and 'Other' trials registered growth of 7.7 per cent p.a..[32]

**Clinical trials, by type (CY2015–19)**




Note: 'Other' includes behavioural, procedure, genetic and radiation trials, trials where no intervention type is specified and a small amount of trials with both drug and device (typically around 1% of total trials)

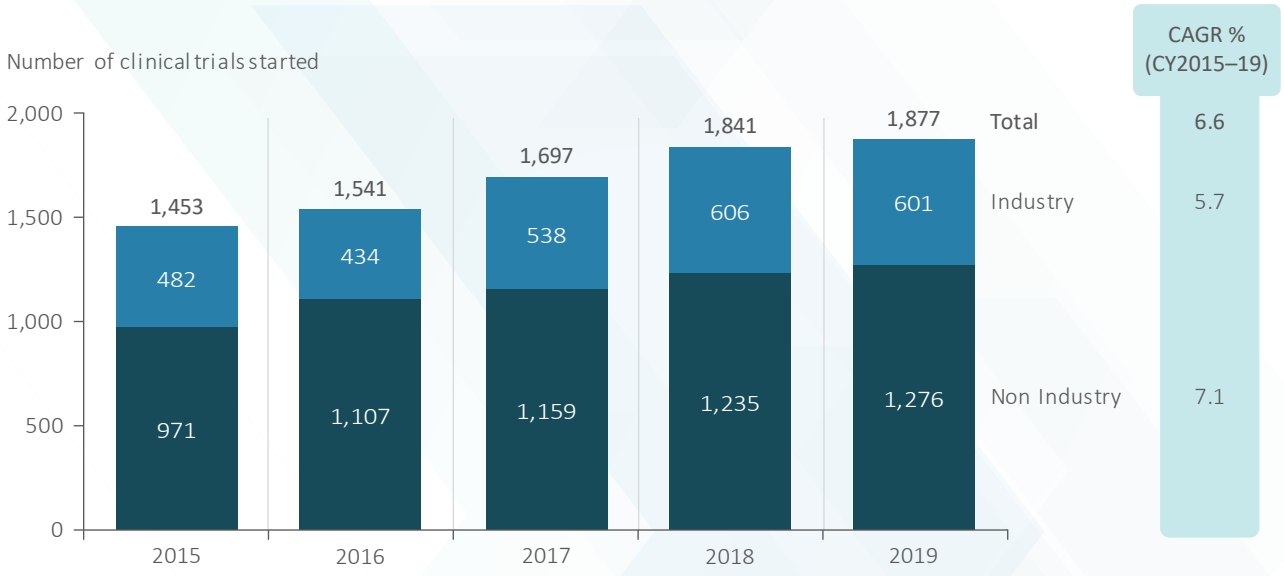
Source: ANZCTR Data (18 Mar 2021); L.E.K. analysis

[32] 'Other' trials includes observational studies and interventional trials listed as 'Behaviour', 'Lifestyle', 'Prevention' research, as well as other treatments that do not involve a drug or medical device

## Clinical trials activity by sponsor type

Industry-sponsored clinical trials accounted for almost a third of all clinical trials started in the period 2015 to 2019. This proportion has remained largely unchanged since 2010 as reported in the *Clinical Trials in Australia* (2017) report. The remaining 68 per cent of trials were sponsored by a combination of non-industry organisations, including government, universities and individuals (this includes IITs).

**Clinical trials, by sponsor type (CY2015–19)**




Source: ANZCTR Data (18 Mar 2021); L.E.K. analysis

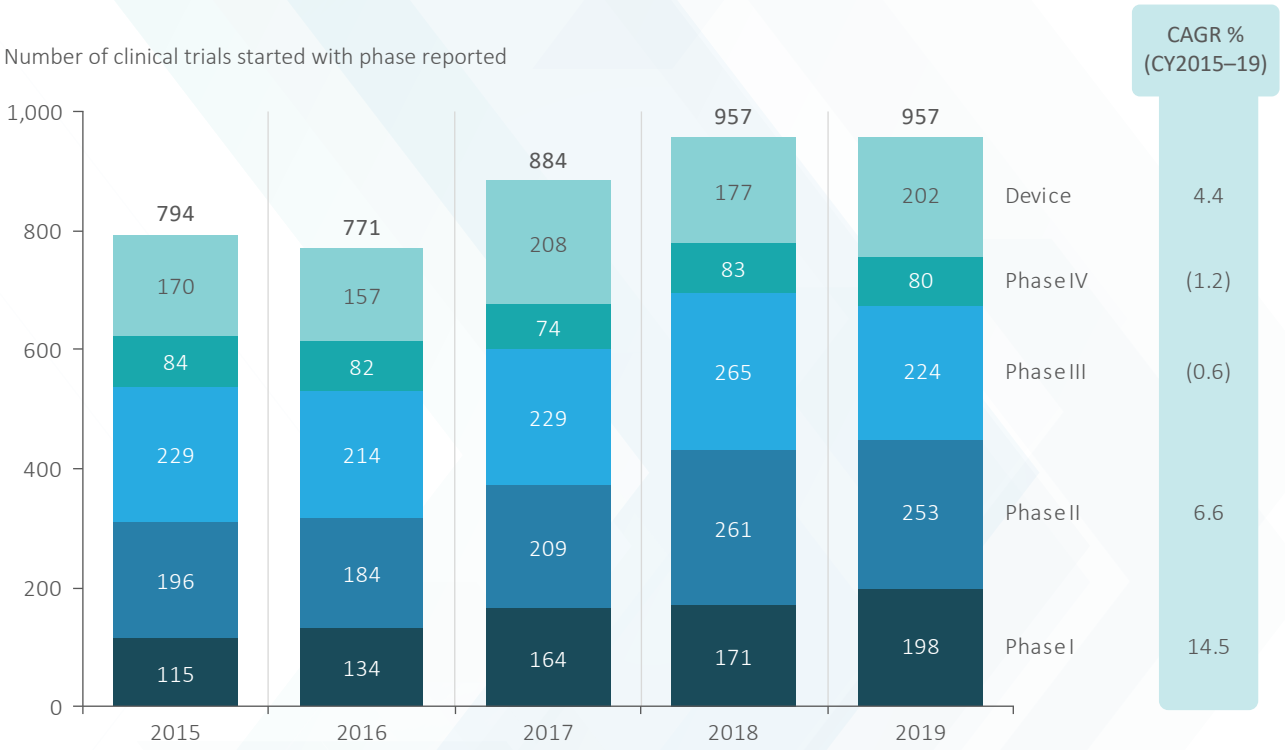
Industry-sponsored trials aim to commercialise the underlying intellectual property (drugs and/or devices). These trials are typically conducted to satisfy regulatory and reimbursement requirements for approval of new interventions and products. Industry sponsors include large pharmaceutical and medical technology companies, CROs as well as smaller start-up and scale-up biotechnology companies. A large proportion of industry-sponsored trials represent a significant 'service export' for Australia as they are funded by foreign companies, often through their local subsidiaries. This foreign investment makes a valuable contribution to R&D, patients and the broader economy in Australia as described in *Chapter 2 – Value Derived from Clinical Trials in Australia.*

Non-industry sponsored trials or IITs encompass a range of trials, including academic trials and collaborative group trials. These trials often investigate clinically relevant research questions that may have less commercial value, including the evaluation of existing treatments and clinical processes in the health system for innovative applications. While non-industry sponsored projects do not bring the foreign investment that many industry-sponsored trials bring, they greatly benefit the sector by supporting local research, improving clinical outcomes for patients as well as developing both the clinical trials workforce and infrastructure. In turn, these sector benefits promote greater industry sponsorship of clinical trials, economic activity and creates jobs. The size and growth of non-industry sponsored trials is primarily dependent on grants provided by the government (see Chapter 2).

## Clinical trials activity by phase

Clinical trials can be segmented into four phases that indicate the stage of development of the drug, device or other form of treatment. Over the last 5 years, Phase I trials have grown the fastest (up 14.5 per cent p.a.), easily outpacing all other categories.[33] Phase II trials have grown in line with the overall growth in clinical trials, up 6.6 per cent p.a. (2015–19), followed by growth in device trials, up 4.4 per cent p.a. (2015–19). The number of Phase III and IV trials has remained flat over this same period. It should be noted that roughly half of the trials registered on ANZCTR do not report their phase.[34]

**Clinical trials, by phase* (CY2015–19)**




Note: A large proportion of clinical trials are not required to go through the four phases and therefore may not have a phase nomenclature to report. This is particularly common for non-device and/or non-drug trials. Data limitations are discussed further in Appendix 2.
Source: ANZCTR Data (18 Mar 2021); L.E.K. analysis

## Clinical trials activity by therapeutic area

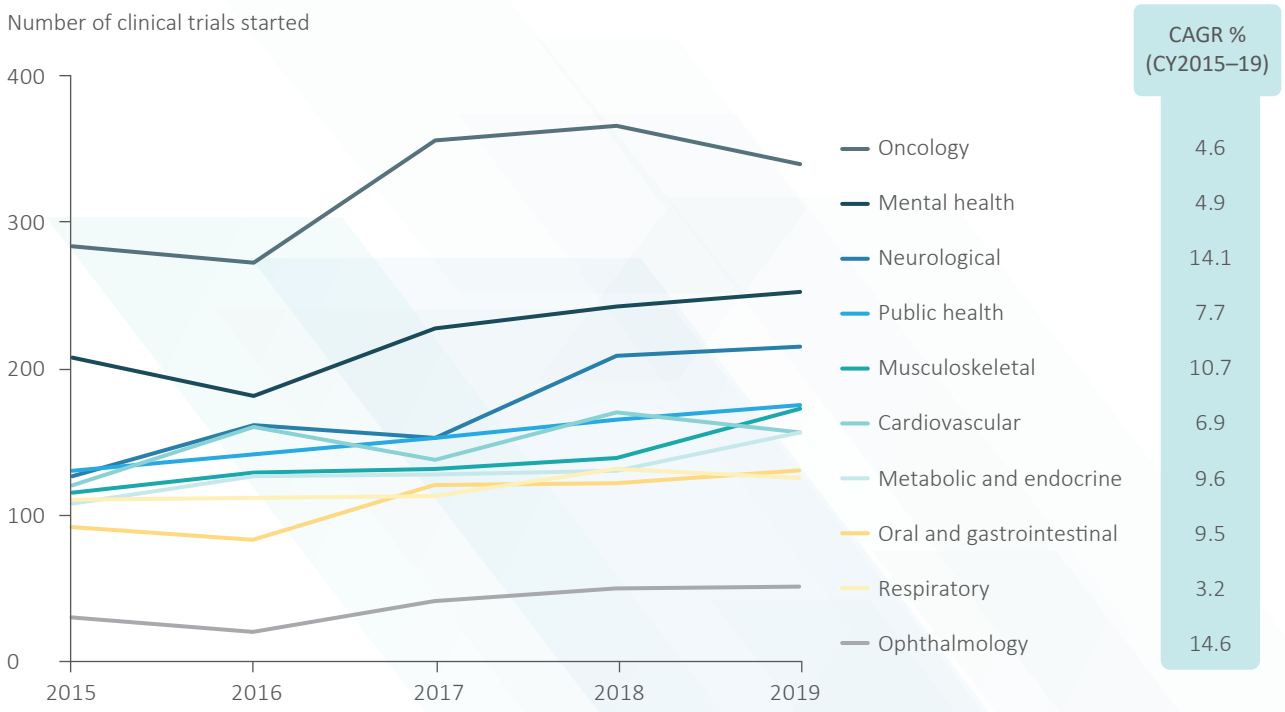
The level of clinical trials activity also varies significantly by therapeutic area, reflecting areas of unmet need and R&D intensity, as well as Australia's relative strengths versus other clinical trials countries. The figure below shows how clinical trials activity in eight leading therapeutic areas has grown since 2015. The eight therapeutic areas align with the most serious causes of death and disease burden in Australia as defined by the Australian Institute of Health and Welfare (AIHW).[35]

[33] Phase I-IV trials include drug and observational trials and exclude device trials

[34] A large proportion of clinical trials are not required to go through the four phases and therefore may not have a phase nomenclature to report to ANZCTR or ClinicalTrials.gov. This is particularly common for non-device and/or non-drug trials. Data limitations are discussed further in Appendix 2

[35] AIHW, Burden of Disease, July 2020

**Number of clinical trials started, by therapy area* (CY2015–19)**




Note: * Select therapy areas shown
Source: ANZCTR Data (18 Mar 2021); L.E.K. analysis

Oncology is the most frequently studied condition in Australian clinical trials. As measured by AIHW's disability-adjusted life years (DALYs), cancer and other neoplasms contribute the largest burden of disease in Australia.[36] Although oncology remains the most studied condition, the number of oncology trials started annually has plateaued in recent years.

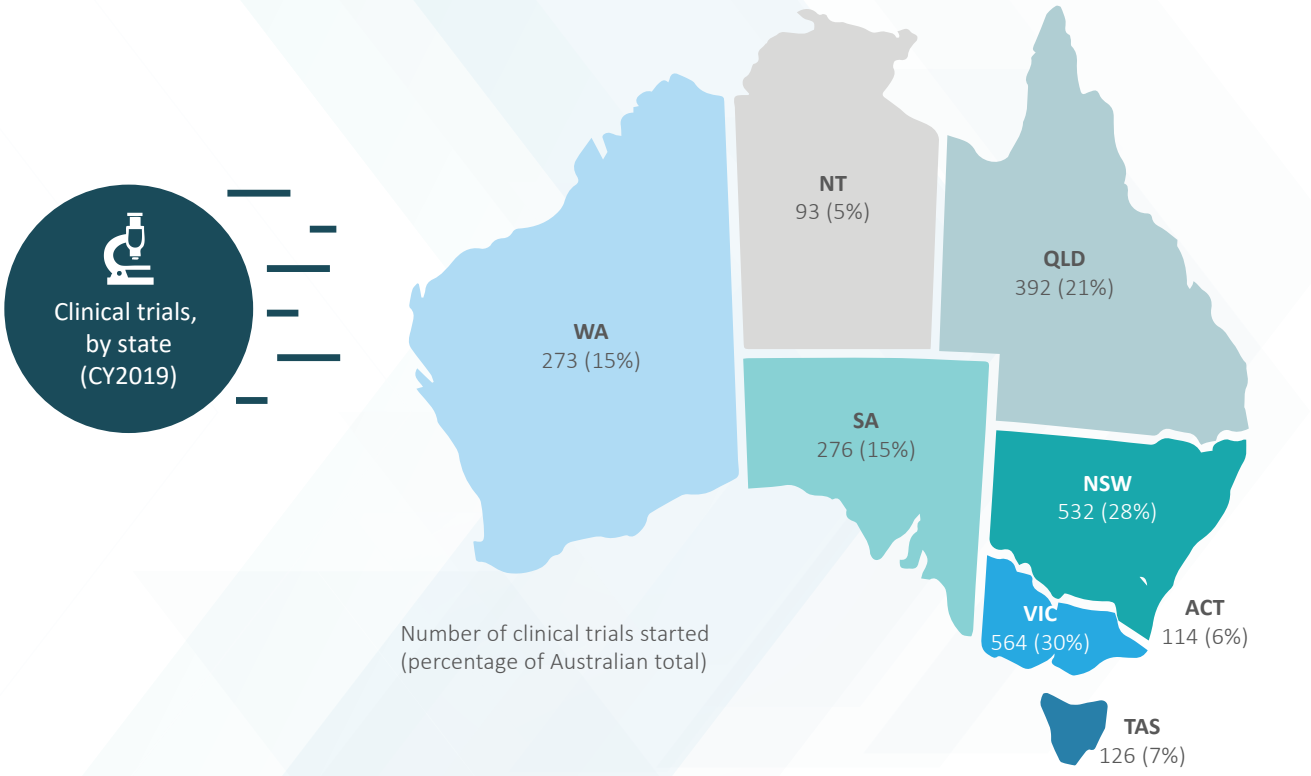
Neurological trials are the fastest growing therapy area among the top 5 therapy areas studied in Australia, up by 14 per cent p.a. since 2015. Neurological conditions include dementias such as Alzheimer's, now the second-highest cause of death in Australia.[37] The growing focus on neurological studies is broadly consistent across industry and non-industry sponsored trials.[38] The MRFF's priorities in Dementia, Ageing and Aged Care and neurological and mental health research through its Clinical Trials Activity initiative will continue to fund trials in this area.[39] The MRFF recognises the enormity of the task ahead, noting that older Australian's physical and cognitive health and wellbeing is one of society's greatest challenges.[40] Significant research capabilities have been built in this therapeutic area and the trajectory is set to continue following the establishment of the Turner Institute for Brain and Mental Health in 2019, which has become one of Australia's largest medical research organisations for brain and mental health.

[36] AIHW, Burden of Disease, July 2020
[37] AIHW, Causes of Death, July 2018
[38] L.E.K. analysis of ClincalTrial.gov data as of February 2021
[39] The MRFF will invest $614.2 million over 10 years in its Clinical Trial Activity initiative to directly fund Australian researchers and patients to participate in clinical trials
[40] Department of Health, Australian Medical Research and Innovation Priorities, 2020

Musculoskeletal conditions contribute the third largest burden of disease in Australia in terms of DALYs and represent the second largest growth area for Australian clinical trials. The number of Musculoskeletal trials has increased by 11 per cent p.a. since 2015. Although Musculoskeletal disease is a Knowledge Priority (KP) for the MTP sector[41], it is not an MRFF priority. This suggests that the growth impetus is mainly coming from industry sponsors.

## Clinical trials activity by state

In order to understand the relative intensity of clinical trial activity across states, ANZCTR data has been analysed to determine the proportion of total trials in Australia that have one or more trial sites in each state. This measurement is a proxy for total clinical trial activity across all participants and all sites in each state. The figure below shows that roughly 30 per cent of clinical trials commenced in 2019 in Australia have sites in Victoria and a similar proportion of trials have sites in New South Wales (NSW). 21 per cent of trials have sites in Queensland and 15 per cent of trials have sites in Western Australia and South Australia.




Note: The sum of the percentages of each state are greater than 100% because each clinical trial could occur in multiple states, in which case it would be counted more than once in the figure above
Source: ANZCTR Data (18 Mar 2021); L.E.K. analysis

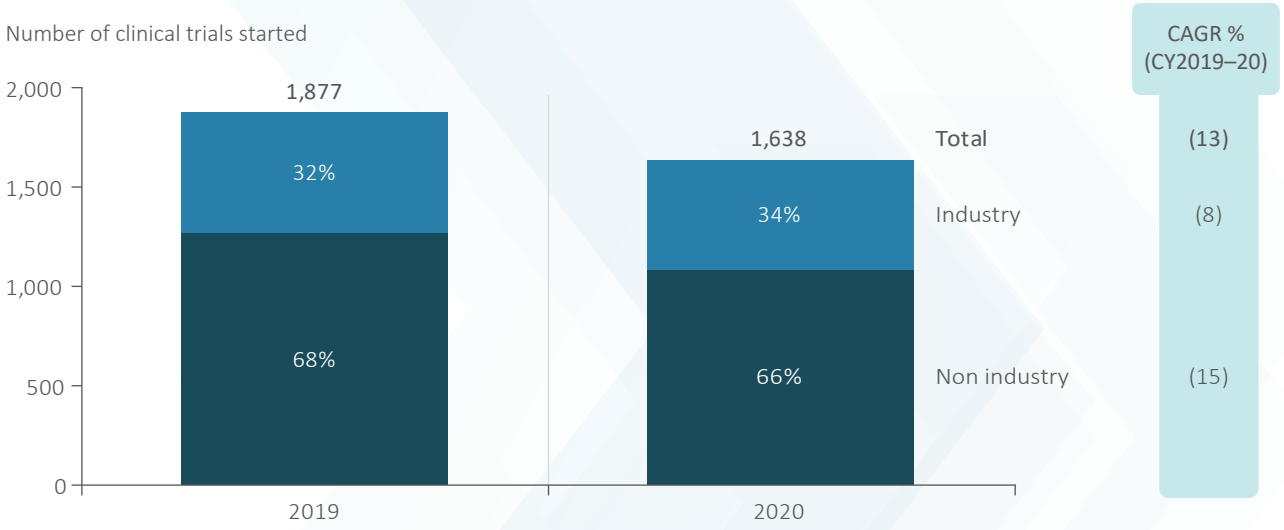
[41] MTPConnect, Sector Competitiveness Plan 2020, April 2020

The observed distribution of clinical trials by state can be explained by the fact that Victoria and NSW have the largest proportions of Australia's population and traditionally have the strongest clinical trials infrastructure, including some of Australia's biggest teaching hospitals and specialist cancer treatment centres.[42]

## Impact of COVID-19 on clinical trial activity

COVID-19 has significantly disrupted clinical trials in Australia and around the world. The number of clinical trials started in Australia in 2020 decreased by approximately 13 per cent when compared to 2019. This reduction was led by a larger decline in the number of non-industry trials started, which decreased by 15 per cent in line with the findings of *MTPConnect COVID-19 Impact Report* (June 2020).

**Clinical trials, by sponsor (CY2019–20)**




Source: ANZCTR Data (18 Mar 2021); L.E.K. analysis

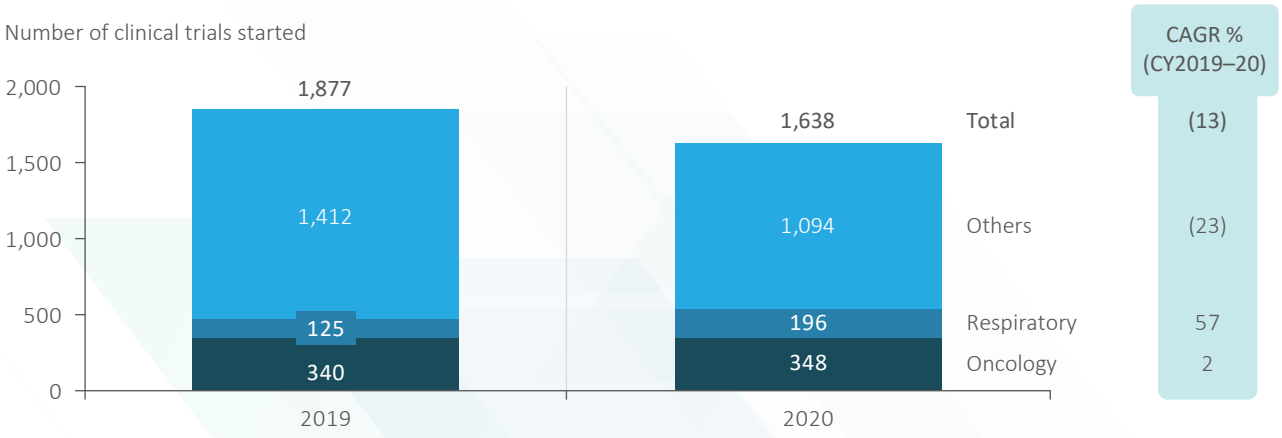
Despite the sharp decline in the number of clinical trials overall, Australia continued to grow the number of oncology trials in 2020 by approximately 2 per cent. Critical clinical trials, for example in oncology, were able to continue via virtual settings; however, many clinical trials were paused or postponed due to concerns regarding patient safety or as a result of the diversion of healthcare infrastructure towards treating COVID-19.[43] Clinical trial activity began to recover as early as May 2020, only for a "second wave" of infections to cause further disruption in August, particularly in Melbourne, Victoria. Unsurprisingly, respiratory clinical trials grew by 57 per cent in support of the search for COVID-19 vaccines and treatments.[44]

[42] It should be noted that calculating the number of trials per capita does not fully explain the distribution as the current metric does not reflect the number of sites and the number of participants per trial in each state

[43] MTPConnect, COVID-19 Impact Report, June 2020

[44] MTPConnect, COVID-19 Impact Report 2nd edition, October 2020

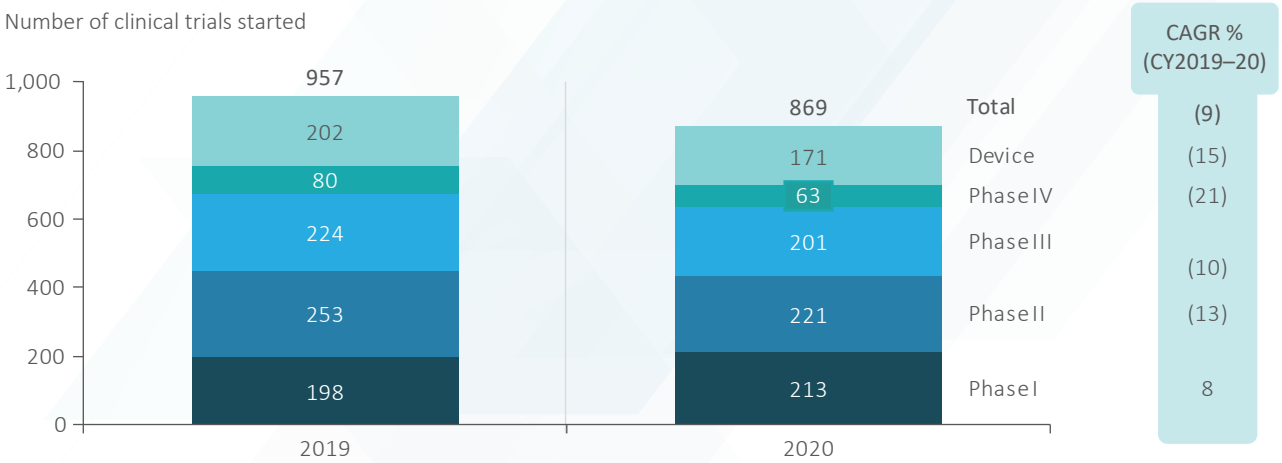
## Clinical trials, by therapy area* (CY2019–20)




Note: *Clinical trials can have multiple therapy areas assigned to them so there may be some double counting between respiratory and oncology trials. 'Others' include all other therapy areas which are not oncology or respiratory (e.g. mental health, neurological, etc)
Source: ANZCTR Data (18 Mar 2021); L.E.K. analysis

The 2020 growth in oncology and respiratory trials coincided with a continued shift towards Phase I trials observed in the period from 2015 to 2019. As shown in the below figure, the number of Phase I trials grew on an absolute basis, while all other trials declined in number. Many of the new Phase I trials were related to COVID-19 vaccine candidates and there was also 20 per cent growth in Phase I industry-sponsored oncology trials.[45]

## Clinical trials, by phase* (CY2019–20)




Note: * Excludes 'Not Applicable' or 'n/a', and trials with no phase indicated
Source: ANZCTR Data (18 Mar 2021); L.E.K. analysis

[45] L.E.K. analysis of ClinicalTrials.gov data as of February 2021

Australia's ability to grow Phase I trials and oncology and respiratory trials during COVID-19 is an indication of the strength of its position in the global clinical trials sector clinical trials sector globally. There is an opportunity to further strengthen this reputation and continue to grow the number of clinical trials in Australia.

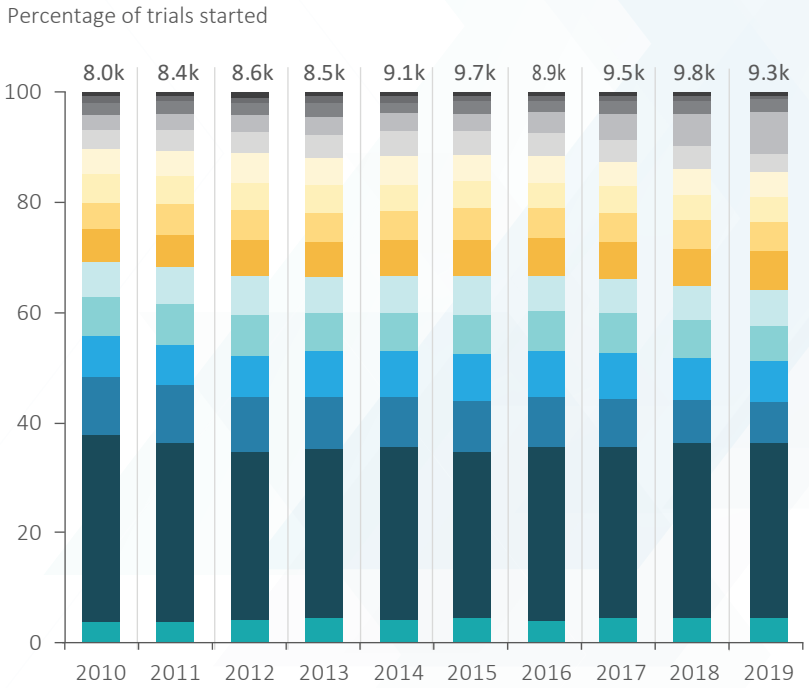
# 4. Australia's position in the global marketplace

While Australia has experienced solid growth in the economic value and level of activity of its clinical trials as outlined in the previous two chapters, it is instructive to assess this growth in a global context. Australia competes in a global market for clinical trials, in particular when it comes to attracting industry-sponsored clinical trials. Industry sponsors have finite resources and will select where to conduct clinical trials based upon a range of factors, including regulatory/market access requirements, the quality of the research personnel and infrastructure, costs per patient, trial start-up time and the ability to recruit patients in an acceptable timeframe. This chapter assesses Australia's competitive position in the global clinical trials market, how it has evolved since the publication of the *Clinical Trials in Australia* (2017) report and re-evaluates the drivers and impediments to future growth.

## Australia's global position in clinical trials

Australia has maintained a consistent position in the global clinical trials marketplace since 2015 as compared to leading competitor destinations. These 15 countries are the same comparator set used to assess Australia's global competitiveness in the *Clinical Trials in Australia* (2017) report. These 15 countries were based on the top 13 countries by clinical trial volume in 2017, plus Singapore and Malaysia (because of their proximity to Australia). It is worth noting that in 2019, Poland and Japan (not shown in this figure) have surpassed Denmark and the Netherlands in their share of global clinical trials.

**Industry sponsored clinical trials, by 15 common countries* (CY2010–19)**




| | Total | Percentage point change (CY2010–15) | Percentage point change (CY2015–19) |
|---|---|---|---|
| Malaysia | | (0.2) | 0.0 |
| Singapore | | (0.4) | (0.1) |
| Denmark | | 0.2 | (0.1) |
| China | | 0.6 | 4.5 |
| Netherlands | | 0.7 | (0.9) |
| South Korea | | 0.1 | (0.2) |
| Belgium | | (0.1) | (0.7) |
| Italy | | 0.8 | (0.3) |
| Spain | | 1.0 | 0.3 |
| France | | 0.5 | (0.4) |
| Canada | | (0.2) | (0.8) |
| United Kingdom | | 1.1 | (1.0) |
| Germany | | (0.8) | (1.7) |
| United States | | (3.9) | 1.5 |
| Australia | | 0.6 | 0.0 |

Note: * Countries shown include the top 13 countries by clinical trial volume in 2017 plus Singapore and Malaysia; excludes 'Withdrawn' trials, and trialswhich do not report a drug or device (e.g. behavioural studies). Trial counts are based on planned recruitment within each country – the same trial may be counted in multiple countries

Source: ClinicalTrials.gov (as at 21/02/2021); L.E.K. analysis

As depicted in the figure above, in the period between 2015 and 2019, clinical trials activity across the 15 countries remained relatively flat with approximately 9,000 to 10,000 trials started each year. The United States and China have increased their share of clinical trials activity, while almost all other comparator countries have experienced share declines or remained relatively constant. The growth in trials in China is being driven by significant local investment in R&D and reforms to the regulatory environment, such as the establishment of an investigative new drug (IND) program and a shortened 60–day clinical trial application approval process.[46,47] In addition, international companies are increasingly seeking to undertake trials in China for regulatory and market access reasons.[48]

Some countries outside the comparative set, for example Japan and Poland, have also increased their share of global clinical trials. While not explicitly considered in this report, it may be worth considering the growth drivers in these countries relative to Australia's competitive advantage.

Overall, Australia has been able to maintain its share of industry-sponsored clinical trials at 5 per cent of the global market over the period of 2015 to 2019. In the context of the global expansion in clinical trials activity, this illustrates that Australia has maintained its reputation as an attractive clinical trials destination.

The rest of this chapter analyses the evolution of Australia's global standing by trial phase and therapeutic area since the publication of the *Clinical Trials in Australia* (2017) report. In addition, it assesses the various drivers of competitive advantage and impediments to growth in clinical trials activity in Australia during this same period.

## Australia's global competitiveness by clinical trial phase

Between CY2013–15 and CY2017–19 Australia increased its share of global industry-sponsored Phase I and II trials.[49] Australia's share of:

- Phase I global trials increased by 2.0 percentage points over the 4–year period, with 246 trials in CY2017–19 (up 62 per cent overall since CY2013–15)
- Phase II global trials increased by 0.5 percentage points over the 4–year period, with 383 trials in CY2017–19 (up 24 per cent overall since CY2013–15).[50]

This gain in Australia's share of Phase I and II clinical trials was echoed by industry sector participants who report that the TGA CTN scheme is a distinct advantage relative to other markets for these trials.

Australia's participation in Phase III and IV trials is usually part of a global trial conducted in multiple

[46] Bayer, Healthy China 2030: China's healthcare journey, 2019

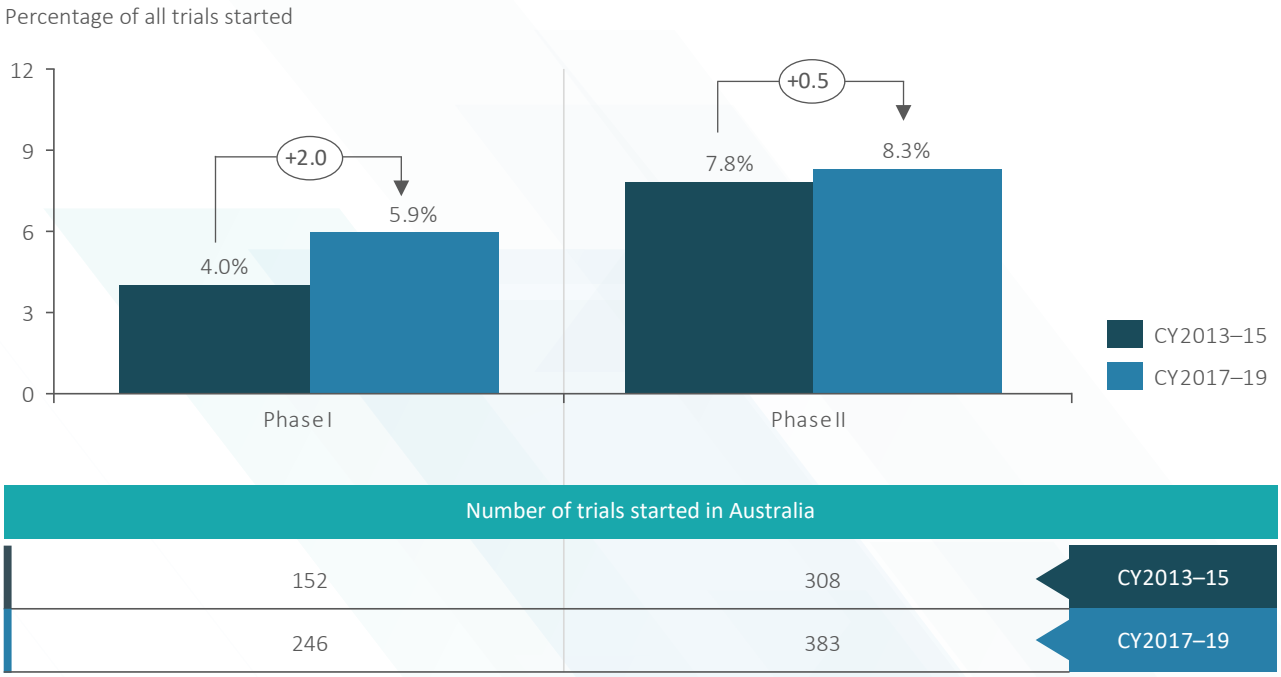
[47] Clinical Leader, Should You Look At China For Your Next Clinical Trial, 2018

[48] Novotech, 5 Key changes accelerate clinical trial and drug approval timelines, 2017

[49] A multi-year approach has been taken to compare Australia's global competitiveness by phase in order to compare like-for-like with the *Clinical Trials in Australia* (2017) report and to reduce year-on-year variability. Phases I and II have a mix of single-country and multi-country trials on the ClinicalTrials.gov registry and therefore 'all trials started' is used as the denominator

[50] The number of trials here is lower than previously shown in Chapter 3 because this analysis is carried out on the ClinicalTrials.gov dataset which includes only industry-sponsored trials. The number of trials here also excludes 'Withdrawn' trials and trials which do not include a drug or device intervention type

**Australia's share of industry-sponsored phase I and II trials* (CY2013–15, CY2017–19)**



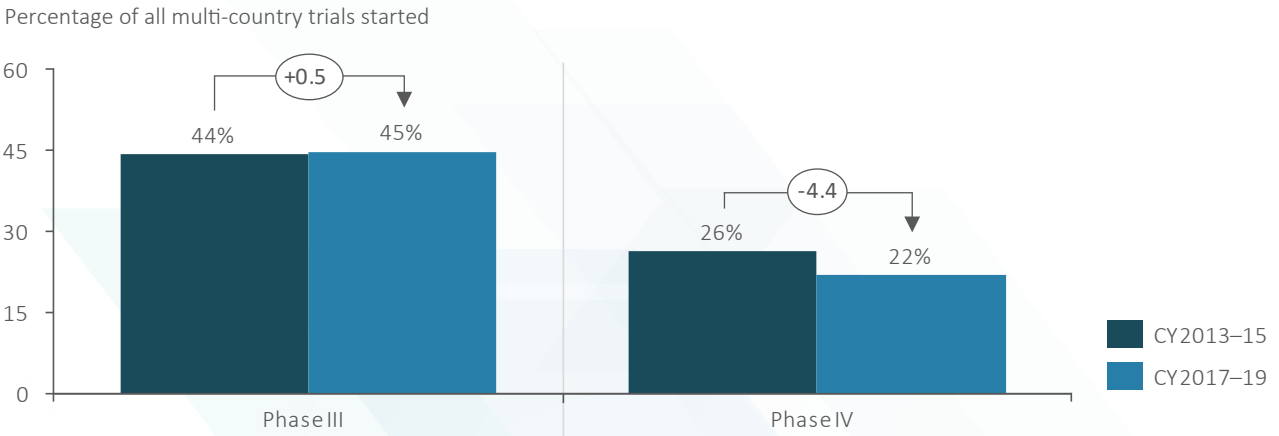

| Number of trials started in Australia | | |
|---|---|---|
| 152 | 308 | CY2013–15 |
| 246 | 383 | CY2017–19 |

Note: * Includes only trials which indicate industry sponsors; excludes 'Withdrawn' trials, and trials which do not report a drug or device (e.g. behavioural studies). Industry sponsored trials are a sub-set of total number of trials presented in *Chapter 3 – Clinical Trial Activity in Australia*
Source: ClinicalTrials.gov (as at 21/02/2021); L.E.K. analysis

countries (as opposed to single-country trials). As shown in the figure below, Australian sites have been included in approximately 45 per cent of Phase III multi-country industry-sponsored trials, a proportion that has remained consistent over the last four to five years. By contrast, Australia's share of Phase IV multi-country industry-sponsored trials has fallen. Only 22 per cent of Phase IV multi-country trials had an Australian site in 2017–19, down 4 percentage points compared to the previous reporting period (2013–15). In the same period, the total number of multi-country, industry-sponsored Phase IV trials fell by approximately 40 per cent.

As measured by its share of global trials, Australia has improved its competitive position in early-stage

**Australia's share of multi-country** industry-sponsored phase III and IV trials* (CY2013–15, CY2017–19)**




| Number of multi-country trials started in Australia** | | |
|---|---|---|
| CY2013–15 | 544 | 34 |
| CY2017–19 | 528 | 18 |

Note: * Includes only trials which indicate Industry sponsors; excludes 'Withdrawn' trials, and trials which do not report a drug or device (e.g. behavioural studies). ** Multi-country trials defined as trials with enrolment in more than three countries. Industry sponsored trials are a sub-set of total number of trials presented in *Chapter 3 – Clinical Trial Activity in Australia*
Source: ClinicalTrials.gov (as at 21/02/2021); L.E.K. analysis

trials (Phase I and Phase II). It has held constant its share of Phase III trials but has seen its relative position in Phase IV trials weaken slightly. The drivers and impediments behind Australia's comparative strengths in clinical trials and the potential causes of these observed trends are discussed in the next section of this chapter.

## Australia's global competitiveness in clinical trials by therapeutic area

Australia has increased its global market share of clinical trials by between 3 and 6 percentage points in several important industry-sponsored therapeutic areas: ophthalmology, oncology, respiratory and neurology. Of these therapeutic areas, oncology and neurology grew the most in absolute terms, continuing a trend first identified in *Clinical Trials in Australia* (2017) whereby Australia is stronger in complex and rapidly changing disease areas.

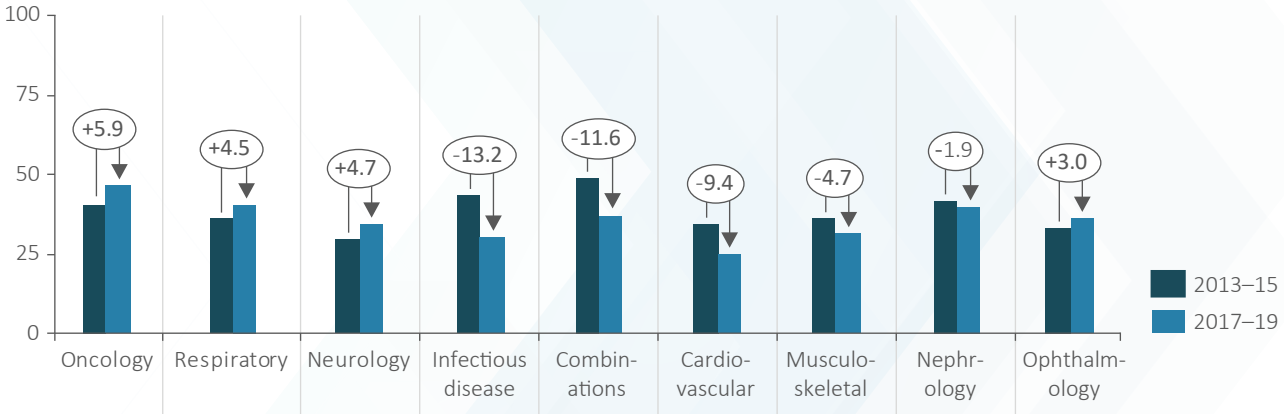
Since 2015, oncology trials have consistently contributed a large proportion of Australia's early phase trials. Australia's strength in the oncology sector is based on the strong reputation of Australian Key Opinion Leaders (KOLs) and its specialised infrastructure. The Australian Government's ongoing focus on funding oncology research, exemplified by the MRFF's Clinical Trials Activity initiative prioritising funding for reproductive, childhood brain and low-survival cancers, has also helped maintain the growth in oncology clinical trials.

Australia's share of industry-sponsored neurology trials has grown by 4.5 percentage points since 2015, in line with the 14.1 per cent increase in the number of neurology trials conducted in Australia during the same time frame (see *Chapter 3 – Clinical Trial Activity in Australia*). In the case of both neurology and oncology, the growth in industry-sponsored trials was accompanied by growth in non-industry-sponsored trials.

By contrast, Australia's share of infectious disease trials and cardiovascular trials has decreased by 13.2 percentage points and 9 percentage points respectively. In both cases, the total number of industry-sponsored trials in these therapeutic areas declined globally; however, trials in Australia declined at a higher rate.[51] It will be important to monitor Australia's clinical trial position in these therapeutic areas over the coming years to understand if this is an ongoing trend or a reflection of the variability in clinical trial activity from period to period. If declines are a reflection of a worsening competitive position in these therapeutic areas, it will be important to identify the drivers behind the declines and take necessary steps to reverse the trend.

**Australia's share of global industry-sponsored multi-country** trials, by therapeutic area* (CY2013–15, CY2017–19)**

Percentage of all multi-country trials started




| Number of multi-country trials started in Australia* | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|
| 316 | 69 | 53 | 82 | 37 | 44 | 44 | 13 | 10 | CY2013–15 |
| 369 | 56 | 57 | 42 | 36 | 30 | 21 | 12 | 12 | CY2017–19 |

Note: * Includes only trials which indicate Industry sponsors; excludes 'Withdrawn' trials, and trials which do not report a drug or device (e.g. behavioural studies). ** Multi-country trials are defined as having planned recruitment in more than three countries. Therapeutic areas were determined using a search of keywords within the 'conditions' field in the clinical trial database – excludes trials where multiple therapeutic areas were found

Source: ClinicalTrials.gov (as at 21/02/2021); L.E.K. analysis

[51] L.E.K. analysis of ClinicalTrials.gov data as of February 2021

## Impact of COVID-19 on Australia's position in the global marketplace

Preliminary data from ClinicalTrials.gov suggests the number of industry-sponsored clinical trials started in 2020 fell considerably worldwide relative to 2019. The magnitude of this decline is subject to revision due to a considerable lag in data available from the ClinicalTrials.gov registry as sponsors often update information in the registry several months after the end of a period. Nonetheless, there have been reports of significant decline in clinical trial activity globally. Almac Group reported that the number of new clinical trials started reduced and that globally patient enrolment slowed through 2020.[52] Evaluate Vantage similarly reported that during the month of April 2020 alone, more than 800 clinical trials were suspended as a result of COVID-19. Some of these trials which were suspended remain on pause, however the majority of trials that remain on pause are observational or involve non-drug interventions.[53]

Australia has not been impacted by COVID-19 to the same extent as many other countries. At the time of writing in May 2021, Australia had experienced fewer than 30,000 cumulative COVID-19 cases, a quantum roughly 80 times fewer per capita than those in the United States and 50 to 60 times fewer than those in France or Spain.[54] Consequently, Australian clinical trials have not been impacted to the same degree as those overseas. Preliminary statistics from ClinicalTrials.gov suggest that Australia has maintained its share of the total number of industry-sponsored clinical trials started in 2020 (relative to the 15 clinical trials comparator countries listed earlier in this chapter).[55]

## Drivers of competitive advantage

As highlighted in the previous sections, Australia has seen strong growth in clinical trials, particularly Phase I and trials in the therapeutic areas of oncology, neurology, respiratory and ophthalmology. Australia's impressive performance can be attributed to five key drivers of Australia's competitiveness. The table below provides a summary of these drivers, a summary of new developments that have strengthened each driver and the resulting advantage of each.

[52] Almac Group, Impact of COVID-19 on the Pharma industry and associated shifts in their outsourcing requirements, 2020

[53] Evaluate Vantage, The pandemic releases its grip on clinical trials, 2021

[54] World Health Organization (WHO), WHO Coronavirus (COVID-19) Dashboard, accessed April 2021

[55] Comparator countries were based on the top 13 countries by clinical trial volume in 2017, plus Singapore and Malaysia

| Driver | Developments since 2017 | Resulting advantage |
|---|---|---|
| **Medical experts and research staff of global standing.** Australia has maintained its strong reputation for the quality and global standing of its investigators and the networks between them. Australia ranks among the top 10 globally in academic and medical research across many MTP disciplines. The combination of this scientific and medical expertise means Australian experts can provide valuable input into the clinical development of protocols and the execution of clinical trials. | Scientific research and healthcare expertise remains a key lever of competitive advantage – according to stakeholder consultations and MTPConnect industry survey responses. | Internationally recognised, respected and accepted research output and capabilities |
| **Quality of research and data.** Australia has an excellent global reputation in science and research evidenced by its many high-quality publications. Compliance with Good Clinical Practice (GCP) guidelines and general high standards of data collection ensures that data collected in Australian trials is often used to support submissions to international regulators, including the FDA and EMA. | The country's high calibre research output is an enduring competitive advantage. Australia continues to rank in the top 10 countries for eminent medical research papers. | Internationally recognised, respected and accepted research output and capabilities |
| **Specialised and dedicated infrastructure.** Australia's Phase I specialised service providers and sites are highly regarded in terms of quality and speed of delivery supported by streamlined processes and private ethics committees.<br><br>Sites with dedicated clinical trial offices and staff are significantly more effective in recruiting patients and delivering high quality data. | New investments from government and sector organisations have continued to strengthen Australia's clinical trials infrastructure especially in oncology. | Fast and efficient trial environment |
| **TGA CTN and ethics approval.** The convenience and speed of the TGA CTN scheme is a competitive advantage relative to other markets. The CTN scheme is a particular advantage for Phase I trials as the process eliminates duplication experienced in other jurisdictions (e.g. FDA's Investigational New Drug [IND] process) whereby the regulator assesses preliminary pre-clinical research in addition to ethics and governance committees. Past reforms have ensured that ethics processes are efficient and competitive globally. | Nationwide rollout of the NMA has simplified efficient ethics approval processes across states. | |
| **R&D Tax Incentives (R&DTI).** The tax relief provided by the R&DTI helps attract clinical trials to Australia, particularly smaller and medium-sized entities, and early phase studies. | The extension of the R&DTI scheme continued to help attract clinical trials to Australia. | Maintained cost competitiveness of Australian trials |

## Key developments since *Clinical Trials in Australia* (2017) report

Three developments since 2017 have helped to maintain and/or strengthen Australia's competitive advantage. The following sub-sections provides more detail regarding these three key developments.

| Progress | Driver impacted | Resulting impact |
|---|---|---|
| **New investments in dedicated clinical trials infrastructure** | Strengthened Australia's specialised and dedicated infrastructure for clinical trials | Will improve the speed and efficiency of the trial environment in Australia as these investments mature |
| **Nationwide rollout of NMA** | Simplified ethics approval across states | Maintained and potentially further improved efficiency of ethics approvals in Australia |
| **Extension of the R&DTI** | Maintained R&DTI for clinical trials | Maintained overall cost competitiveness of conducting trials in Australia |

### New investments in dedicated clinical trials infrastructure

Building upon the existing specialised infrastructure referenced in *Clinical Trials in Australia* (2017), the Australian Government has made further investments in the National Critical Research Infrastructure initiative funded by the MRFF. The enhanced initiative will provide $605 million over ten years to establish and extend the infrastructure needed to conduct world-class health and medical research. Early funding priorities include improving rural, regional and remote (RRR) clinical trial enabling infrastructure.[56] These priority areas (highlighted in the table below) and are incremental to the MRFF funding of clinical trials directly through the Clinical Trials Activity initiative.[57]

*Selected MRFF-funded Clinical Trial Infrastructure Programs and Initiatives since 2017*

Over the past three years, grants for a variety of purposes have been awarded to significantly improve clinical trials infrastructure:

- **The Australian Tele-trial Program – access to clinical trials closer to home** ($75 million), a five–year grant aimed at establishing an interconnected clinical trial system through the Australasian Tele-trial model across all states.[58] The program is led by the Queensland Department of Health and followed learnings from pilot studies across Queensland (e.g. MTPConnect co-funded COSA pilot), New South Wales and Victoria.[59] The program will provide patient access to clinical trials closer to home, enhance rural and regional access to clinical trials, aid patient recruitment and bring patient centricity to trial design. Industry sponsors and investigator groups have shown significant interest in, and support for the tele-trial model.[60]

[56] Department of Health, National Critical Research Infrastructure Initiative website, accessed on 31 March 2021
[57] Department of Health, Clinical Trials Activity Initiative website, accessed on 31 March 2021
[58] Department of Health, Medical Research Future Fund (MRFF) Grant Recipients, November 2020
[59] Healthcare Professionals Group, Tele-trials–Increasing equity for Australian patients accessing clinical trials, 2020
[60] L.E.K./MTPConnect interviews with local MTP sector experts

- **Improving access to innovative healthcare in Rural, Regional and Remote (RRR) NSW and ACT** ($31 million), a project led by NSW Health along with 34 state and national partners. The five–year project will develop decentralised clinical trials capacity and capability to enable delivery of clinical trials directly to RRR communities, support and develop the local clinical trials workforce, raise awareness and engagement regarding clinical trials within communities and establish best practice standards for clinical trial services including governance and coordination.[61]
- **ReViTALISE Project Bridging the metro – regional trials gap by 2025** ($19 million) led by Border Medical Oncology Research Unit[62], the project will add new sites to Regional Trials Network Victoria and introduce seven unique projects across the network. Facilitating equitable clinical trial access for RRR patients will make the latest clinical research and treatments available to an important but historically excluded section of the population.

Together, the three MRFF funded programs outlined above will improve clinical trial access for the 28 per cent of the population that live in RRR Australia.[63] These investments will allow new drugs, devices and enabling therapies to be tested in more diverse patient populations. In addition to the above-mentioned initiatives, several other clinical trial areas have received MRFF funding:

- **Australian Dementia Network (ADNeT)** ($18 million) brings together Australia's leading dementia researchers, clinical trial networks and other stakeholders to explore emerging opportunities such as a national dementia registry over five years. The network creates a sustainable, translational research infrastructure to enable ongoing, high-quality dementia research and clinical care.[64]
- **The Australian Clinical Trials Alliance** ($5.5 million) will provide specialised leadership and support for clinical trial networks to consolidate and strengthen sector capability and collaboration. Over four years, this will strengthen clinical trial networks and help embed evidence-based care in the health system to improve health outcomes.[65]

In addition to the MRFF there are other government investments in clinical trial infrastructure. One such example is the TrialHub initiative ($25 million) a Victorian project led by Alfred Health along with Latrobe Regional Hospital, Bendigo Health and Peninsula Health. The initiative will establish local clinical trial units in regional and remote areas of Victoria to provide patients in these areas better access to prostate, melanoma and rare cancer trials. This initiative is funded under the Community Health and Hospital Program.[66]

### *Impact*

Together, these infrastructure investments have the potential to increase the speed and efficiency of recruiting patients to clinical trials by improving patient access. This will strengthen Australia's competitive advantage as a fast and efficient trial environment for conducting clinical trials.

[61] NSW Health, Rural, Regional & Remote Clinical Trial Program website, accessed on 12 April 2021
[62] Department of Health, Budget 2020-21, Guaranteeing Medicare and Access to Medicines – Rural, Regional and Remote Clinical Trial Enabling Infrastructure Program, 2020
[63] Australian Bureau of Statistics, Regional Population Growth, March 2020
[64] Florey Institute of Neuroscience and Mental Health, Major new Alzheimer's disease initiative announced, 2018
[65] ACTA, Funding and Support, accessed 30 March 2021
[66] Alfred Health, Expanding access to cancer clinical trials, 20 April 2021

## Nationwide rollout of the NMA

The NMA is a national system for mutual acceptance of scientific and ethical review for multi-centre clinical trials conducted in publicly funded health services.[67] Victoria, South Australia, Queensland and New South Wales were the original parties to the NMA. Western Australia, Tasmania, the Australian Capital Territory and the Northern Territory have all signed on since 2017.

The NMA national rollout has helped to provide greater standardisation of the ethics approval processes between states and has reduced duplication of effort for multi-site clinical trials conducted in publicly funded health services. In FY2018–19, approximately 73 per cent of clinical trials are reviewed and approved by HRECs within a 60 day benchmark. This benchmark measures the timeliness of administrators only, and deducts the time taken for investigators/sponsors to respond to queries.[68,69]

Despite process improvements and enhanced collaboration, there is scope for further improvement of ethics approval processes. Areas requiring improvement include:

- Rationalisation of the exemptions from the NMA. For example, projects involving the health and wellbeing of Aboriginal and Torres Strait Islander people and communities require state-specific ethics approvals. ACT and SA also require state-specific ethical review for early phase trials (exploratory/pilot studies [Phase 0], Phase I and FTIH) and NSW requires approval by NSW Health Early Phase Clinical Trial HRECs for early phase trials.[70]
- Further standardisation of HRECs to achieve mutual acceptance without exception between public and private systems to progress to a true single ethical review. Each state has a list of certified HREC committees, however, these are quite often limited to publicly funded service providers.[71] A national list of certified HREC committees across Australia that includes public and private HREC committees would streamline the approvals process further.
- Streamlining of technology platforms used for ethics approvals across states. The use of different systems across states can cause duplication of effort for investigators and industry sponsors (e.g. Research Ethics and Governance Information System (REGIS) used by New South Wales and South Australia, Ethics Review Manager (ERM) used by Queensland and Victoria, Research Governance Service (RGS) used by Western Australia).[72]

A national clinical trials front door that provides national coordination of ethics approvals would help address the above-mentioned concerns and further streamline the approvals process for clinical trials. Such a concept was originally announced in 2018 as part of the FY2018–19 Budget[73] with a further announcement by the Federal Minister for Health in June 2020 relating to the 'one-stop-shop' National Front Door ethics approval initiative.[74] The initiative by the Department of Health, aims to enhance connectivity between jurisdictional platforms to streamline approval and patient recruitment processes.

[67] Department of Industry, Innovation and Science, Australian Clinical Trials website, accessed April 2021
[68] Clinical Trials Project Reference Group, Clinical Trials in Australian Public Health Institutions 2018-19 (NAS 4 report), 2021
[69] It is not possible to provide a meaningful comparison of trial start-up timelines using reported NAS data between FY2018–19 and FY2014–15 due to a shift in mix of trial types (e.g. growth in biologics) and the addition of new states in the FY2018–19 dataset (e.g. Western Australia, Northern Territory). These and other changes in the R&D environment do not make a like-for-like comparison possible.
[70] NSW Health, National Mutual Acceptance webpage, accessed April 2021
[71] NHMRC, HREC Committees registered with the NHMRC, accessed April 2021
[72] Victorian Department of Health & Human Services, National Mutual Acceptance webpage, accessed April 2021
[73] Department of Health, Budget announces new investments in health and medical research, 8 May 2018
[74] The Hon. Greg Hunt MP, Official Opening of the 2020 ACTA Summit, 2020

*Impact*

According to industry stakeholder consultations, the ongoing effort to expand the NMA to cover all states and territories across Australia has supported the nation's competitiveness in trial start-up relative to other markets. The development and eventual implementation of the clinical trials 'one-stop-shop' National Front Door has the potential to further reduce the time taken to start-up clinical trials in Australia relative to other global markets. However there is still a need to expedite research governance approval, as discussed later in this chapter.

## Extension of the R&DTI

The Australian Government subsidises research in Australia with the R&DTI. As a general rule, Phase I, II and III trials meet the criteria for R&DTI, while Phase IV trials are eligible only if they are being carried out for medical research purposes rather than meeting regulatory requirements.[75] Companies currently may be eligible for a 38.5–43.5c tax incentive per $1 of eligible R&D, depending on annual turnover.[76,77]

The Australian Government has acknowledged R&D as an important lever to stimulate foreign investment and has taken steps to strengthen incentives for R&D activities in Australia. The following changes announced in October 2020 as part of the FY2020–21 Federal Budget are likely to positively impact the clinical trials sector, despite being linked to the applicable corporate tax rate[78]:

- Increasing the R&D expenditure threshold from $100 million to $150 million (which secures the benefit of the R&D offset for a larger proportion of businesses, as the benefit is limited to a deduction linked to each company's tax rate above this point).
- Fixing the R&D tax incentive to the corporate tax rates plus an additional 18.5 percentage points.
- Increasing the R&D tax incentive for larger R&D entities with high levels of R&D intensity.[79]

In addition to the extension of the R&DTI, the Australian Federal Government's FY2021–22 Budget has introduced a new "patent box" incentive. This incentive will benefit Australian MTP companies who conduct R&D locally by allowing a 17 per cent effective concessional corporate tax rate on income derived from the patent. Normally corporate income is taxed at 30 per cent or 25 per cent for small and medium companies.[80,81]

*Impact*

A larger set of MTP companies and CROs conducting clinical trials in Australia will benefit from the increase in R&D expenditure threshold proposed. The increase in the amount of the tax incentive will also make it more attractive for those companies carrying out clinical trials in Australia. Together, these changes will help maintain Australia's overall cost competitiveness as a clinical trials destination relative to other global markets in the face of increasing competition from countries with lower labour costs across the Asia Pacific.

[75] Victorian Government, VicTrials website, accessed March 2021
[76] Only companies with aggregated turnover of less than $20 million are eligible for highest tax offset of 45c
[77] Department of Industry, Innovation and Science, Upcoming changes to the R&D Tax Incentive: Overview factsheet, November 2020
[78] AusBiotech, Budget delivers significant news for R&D, October 2020
[79] Parliament of Australia: Treasury Laws Amendment (A Tax Plan for the COVID-19 Economic Recovery) Bill 2020
[80] Australian Government, Tax incentives to support the recovery, May 2021
[81] AusBiotech, Budget delivers for biotech manufacturing, May 2021

## Impediments to clinical trials in Australia

Despite growth in Australia's clinical trials activity and the drivers of competitive advantage listed above, there is room for further improvement. Three key impediments to the sustained growth of clinical trials activity in Australia were highlighted in Clinical Trials in Australia (2017). These are summarised in the table below along with an overview of the recent developments since.

| Driver | Developments since 2017 | Resulting barrier |
|---|---|---|
| **Governance approval process.** Despite improvements in ethics approvals, site governance approval processes are often lengthy and highly variable from site to site and study to study. This erodes some of the advantage achieved in the efficient ethics and CTN process and causes variability in start-up times particularly for multi-site trials. | Remains in need of improvement, although the development of the CTGF provides an opportunity for standardisation. | Variability in start-up time |
| **Patient recruitment and economics.** Australia's small population size relative to other markets such as the UK, US, Eastern Europe and Asia creates a barrier to high patient recruitment levels. Even relative to countries with a similar population density such as Canada, the average number of participants per trial site in Australia is generally low. This issue is compounded by a general lack of effectiveness in referrals between sites and other healthcare entities.<br><br>These factors have negative implications for the economics of a site. Set-up costs are relatively fixed and irrespective of the number of trial participants recruited. Limited numbers of participants per site also implies that in some therapeutic areas the competition for the same patients among trial sponsors is high. | Efforts to raise consumer and clinician awareness have coincided with improved patient recruitment according to responses received to the MTPConnect industry survey. Decentralised trials (including tele-trials) have potential to improve patient recruitment.<br><br>COVID-19 has significantly increased patient/consumer awareness of role and importance of clinical trials in Australia and may lead to increased participation. | High per patient costs |
| **Capabilities and capacity in early-stage high risk innovative trials**. Australia's ability to continue to attract a significant number of early phase trials requires further investment in capabilities and capacity in more novel early-stage trials (e.g. immunotherapy, FTIH, translational medicine, novel biologics). | Strong growth in early phase trials since 2015 suggests Australia has developed capabilities in this space. However, workforce capacity gaps which were noted in 2017 have since emerged as a more significant impediment. | Limited capabilities and capacity for high risk or innovative trials lead to difficulties in establishing a sustainable competitive advantage |

## Key developments since 2017

Since 2017, three developments have successfully removed or mitigated some impediments to Australia's competitive position in clinical trials (see table below). However, the emergence of new workforce capacity gaps has had a negative impact on Australia's competitiveness.

| Key developments | Resulting impact on impediments |
|---|---|
| **Development of the CTGF** | Potential to improve consistency and efficiency of site governance approvals nationally |
| **Consumer and clinician awareness programs** | Enhance patient recruitment |
| **Development of decentralised trials, including tele-trials** | Enhance patient recruitment |
| **Emergence of key workforce capacity gaps** | Limited capabilities and capacity to support further growth of clinical trials conducted in Australia |

## Development of the CTGF

Site governance approval processes were highlighted as an impediment to clinical trials in 2017 and they largely remain lengthy, highly variable and dependent upon the capacity and capability of each Research Governance Office (RGO). As a result, timelines are unpredictable between sites and studies. On average in FY2018–19, SSA authorisation was provided in 118 days from ethics approval date. Approximately 40 per cent of trials received SSA authorisation in less than 60 days. This calculation includes the time taken for investigators/sponsors to respond to queries.[82,83] Proper oversight for clinical trials is critical to ensure the highest standards of quality and safety for patients however some elements of the site approval processes are currently complex. As a result, there are opportunities to streamline site governance processes, without compromising patient safety and quality.

The national CTGF was developed by the Australian Commission on Safety and Quality in Health Care on behalf of all jurisdictions and the Clinical Trials Project Reference Group (CTPRG) as part of the Revitalised Clinical Trials in Australia Agenda and the Encouraging More Clinical Trials in Australia initiative. It is a signature project of CTPRG and offers the opportunity for a more streamlined approach to site governance approvals.[84] The framework aims to:

- strengthen governance arrangements for clinical trials
- provide clarity to those responsible for delivering clinical trials, including government health services, hospital administrators, clinicians, trial sponsors and patients
- reduce duplication and increase efficiency, cohesion and productivity across the clinical trials sector.

Following a national consultation process, a pilot of the framework was undertaken in late 2020 and early 2021. The pilot is expected to release its findings and develop a CTGF later this year to accelerate the improvement of the governance of clinical trials in line with executive governance of other clinical care.

[82] Clinical Trials Project Reference Group, Clinical Trials in Australian Public Health Institutions 2018-19 (NAS 4 report), 2021

[83] It is not possible to provide a meaningful comparison of trial start-up timelines using reported NAS data between FY2018–19 and FY2014–15 due to a shift in mix of trial types (e.g. growth in biologics) and the addition of new states in the FY2018–19 dataset (e.g. Western Australia, Northern Territory). These and other changes in the R&D environment do not make a like-for-like comparison possible.

[84] Australian Commission on Safety and Quality in Health Care, The National Clinical Trials Governance Framework (draft), accessed March 2021

In addition to the development and implementation of the CTGF, site governance approvals can be improved by streamlining processes across hospitals and/or Local Health Districts (LHD)/Local Health Networks (LHN). Providing a consistent process and standards such as standardised SSA forms across multiple hospitals and states will reduce the duplication of effort required on those making the submissions and simplify the evaluation of submissions. Site governance approvals could also be restructured to be streamlined at the level of a LHD/LHN. Doing so would reduce the need for multiple site governance approvals for trials running across multiple sites within a single network of hospitals.[85] Stakeholder consultations have also highlighted that conducting ethics and governance processes in parallel can help speed up clinical trial start-up times, however in come cases this will require an expansion in the capacity of RGOs.

*Impact*

A successful rollout of the national CTGF will drive greater consistency and efficiency in site governance approval processes and help decrease trial start-up times across Australia, reinforcing the benefits being realised by progress in streamlining ethics approvals.

### Consumer and clinician awareness programs

A number of initiatives have been undertaken by government organisations (e.g. the NHMRC 'Helping Our Health' campaign), non-for-profits, and patient advocacy groups since 2017 to raise awareness about the role and value of clinical trials among consumers and clinicians. Organisations such as Research4Me and the White Coats Foundation have run numerous programs and events (the Research4Me Think Tank, Power of One and Thank You sessions) to raise awareness of clinical trials among consumers, including patients. These initiatives reveal that patients are eager to participate in clinical trials and are very engaged when participating.[86] However, finding information and advice about clinical trials that may be applicable and relevant for patients remains an obstacle.

The use of technologies like electronic consent for clinical trials, clinical trials management systems and clinical trial recruitment platforms need to be accelerated to help improve consumer access to information regarding clinical trials. These technologies are discussed further in *Chapter 5 – Emerging Opportunities and Priorities for the Future*. The 'Join Us' register, established by The George Institute in September 2020, is one example of an innovative technological solution aimed at improving patient access to clinical trials. The register aims to recruit a million Australians who pre-consent to be contacted for studies in which they could usefully be involved.[87] The registry would capture personal and health details, information which would be used to match them to relevant clinical trial studies (with appropriate data security measures in place).

On the clinician side, there has been significant growth of the ACTA which now has more than 250 members[88], organisations and individuals who coordinate the efforts of clinical researchers and connect these efforts with government and consumers. In addition to IITs being conducted through clinical trial networks, the CTGF will introduce trials as part of standard healthcare. In support of these efforts, ACTA and Clinical Trials: Impact & Quality (CT:IQ), a consortium initially funded by MTPConnect, has developed a Consumer Involvement and Engagement Toolkit for researchers and research organisations that

[85] L.E.K./MTPConnect interviews with sector participants, 2021
[86] Janelle Bowden and Lisa Briggs, Searching for Clinical Trials: What Patients Want, August 2018
[87] ABC, New registry calls on Australians to join clinical research, September 2020
[88] ABC, New registry calls on Australians to join clinical research, September 2020

provides information on how they can best involve and engage consumers throughout the lifecycle of clinical trials.[89] Such initiatives have helped and will continue to help raise clinician awareness on how to effectively engage and involve their patients in clinical trials.

### *Impact*

The initiatives undertaken over the last few years have helped raise awareness about clinical trials. MTPConnect industry survey responses suggest that two thirds of clinical trials started in 2019 met their recruitment target.[90] Recruitment outcomes are not systematically collected across the industry and this MTPConnect industry survey result will serve as a useful baseline against which to compare future recruitment outcomes.

COVID-19 has helped elevate the conversation regarding clinical trials nationally. There has been much more discussion regarding clinical research in the public domain over the past 12 months as the various vaccine candidates for COVID-19 were being developed. There is always room for improvement and continuing efforts to raise consumer and clinician awareness of the role and value of clinical trials through a sustained and national program will help enhance patient recruitment and help to improve Australia's overall attractiveness as a clinical trials destination.

## Development of decentralised trials, including tele-trials

Decentralised clinical trials enhance patient participation and retention by reducing the burden of travel on patients seeking innovative care. The trials are executed through telemedicine and mobile/local healthcare providers and align with the needs of patients through research design and implementation.[91] As highlighted earlier in this chapter, significant investments have been made in establishing appropriate clinical trials infrastructure for tele-trials and RRR trials.

Tele-trials are one of many mechanisms through which a decentralised trial can be implemented. The development of tele-trials is part of a global trend towards greater patient centricity in healthcare, as discussed further in *Chapter 5 – Emerging Opportunities and Priorities for the Future*. Australia has accelerated the development of operations protocols for tele-trials over the last two years. The development of the National Tele-trials Compendium will also provide national support for tele-trials.

### *Impact*

Developing greater capability for decentralised trials through the implementation of tele-trials and digital technologies will increase the number of patients that can be recruited, managed and retained per site. This in turn will help to drive down the cost per patient over the long term (despite higher initial costs). For example, three Tasmanian patients had their Phase I clinical trial treatment plan transferred from Victoria to Tasmania and have continued their care under a tele-trial model since mid-2020. This was the first time a tele-trial model was used for a Phase I clinical trial in Australia and offers hope for patients in remote and regional locations seeking to participate in trials. It will also be attractive to sponsors seeking to access more patients with fewer physical sites.[92]

[89] ACTA and CT:IQ, Consumer Involvement & Engagement Toolkit website, accessed March 2021

[90] MTPConnect industry survey, 2021

[91] Carsten Sommer, et al., Building clinical trials around patients: Evaluation and comparison of decentralized and conventional site models in patients with low back pain, *Contemporary Clinical Trials Communications*, 2018

[92] Alfred Health, Lifesaving trial moves to Tasmania, 8 May 2020 and input from Tasmanian Department of Health

## Emergence of key workforce capacity gaps

Clinical Research Associates (CRAs) are typically employed by CROs or MTP companies and are the main point of liaison between the study sponsor and the study sites. Clinical Trials Coordinators (CTCs) are employed at the clinical trial sites and are responsible for the day-to-day running of clinical trials, including liaising with and monitoring patients. In the *Clinical Trials in Australia* (2017) report, some stakeholders voiced concern over a shortage of experienced CRAs and CTCs.

MTPConnect's *REDI Initiative Skills Gap Analysis Second Report* concluded that there is now a critical shortage of experienced CRAs and CTCs[93], pinpointing several causes. It takes considerable time to develop CRAs, many of whom join as new university graduates and require between six months and two years before they have enough experience to manage a variety of different trials independently. The CRA role is also demanding in terms of hours and travel requirements. Skills acquired in the first few years are often leveraged to secure senior opportunities elsewhere in the industry, leading to a high turnover of CRAs across the sector.

Experienced CTCs perform a diverse range of tasks that are critical to successful clinical trial operations at the trial site. Any shortage of CTCs is a significant impediment to the volume and quality of trials that can be performed. Unattractive short-term contracts, a general lack of appropriate education/training and competition from within the industry for fully trained CTCs all contribute to the skills deficit.

### *Impact*

The availability of a sufficient number of experienced CRAs and CTCs is critical to ensuring the continued growth of clinical trials in Australia. Addressing skills capacity gaps and short-term unattractive contracts is critical. Skills gaps can be solved through professional traineeships in the short-term and establishing accredited qualifications and training programs in the longer-term is critical. In addition, there needs to be a fundamental shift amongst trial sites from budgeting and resourcing CTCs on a per-trial basis to a more holistic model. If left unaddressed, talent gaps will constrain the growth of clinical trials in Australia.

[93] MTPConnect, REDI Initiative Skills Gap Analysis Second Report, 2021

# 5. Emerging opportunities and future priorities

This final chapter identifies the themes and trends that will shape the future dynamics of clinical trials and determine the scope and nature of the opportunities for the Australian clinical trial sector. It highlights the key priorities for the future that will enable Australia's clinical trial sector to continue to grow. These new priorities reflect the impediments and areas for further development identified in the previous chapter as well as the emerging opportunities described in this chapter.

## Emerging opportunities in clinical trials

Four emerging healthcare trends, each requiring a concerted and decisive response, will create new opportunities for Australia's clinical trials stakeholders:

- **Patient awareness, engagement and centricity**, delivers health and economic benefits and has further potential to enable Australia to grow its patient participation rate. More flexible and decentralised trial designs will be important components of any patient-centric strategy
- **Digital health concepts**, including tele-health, are gaining traction in clinical trial settings, supporting patient recruitment through the use of online portals and AI matching
- **Precision healthcare** trials have been expanding in number and scope, as reflected in the rising use of biomarkers in oncology trials
- **Innovative trial designs**, including platform/umbrella/basket trials and adaptive clinical trials, are expected to grow as companies look to develop new treatment combinations and pathways.

### Patient awareness, engagement and centricity

Patient recruitment remains a global challenge and patient-centric design may provide a solution. In the context of clinical trials, patient centricity means designing and executing trials around the needs and perspectives of the patient. Patient-centric solutions invariably involve incorporating feedback from the patients and their carers during trial design.

Equally, there is opportunity to facilitate greater participation in trials. In Australia, 68 per cent of consumers report receiving support to facilitate involvement in their clinical trial. At the same time, 100 per cent of those who responded on behalf of clinical trials networks and individual clinical trials emphasised the value of consumer involvement.[94]

Patient centricity delivers real benefits to trial sponsors and has been shown to drive faster patient recruitment. A Parexel and The Economist Intelligence Unit (EIU) study of the Trialtrove® database showed that studies mentioning a patient centric approach typically take 40 per cent less time to recruit 100 patients, approximately 3 months less time compared to "all types of trials". Moreover, the study showed that drugs developed using patient-centric designs were 19 per cent more likely to be launched versus the control group.[95]

[94] Janelle Bowden, PhD, Consumers' role in clinical trials research. Where to from here?, November 2018

[95] Parexel and The Economist Intelligence Unit, The Innovation Imperative: The Future of Drug Development, May 2019

**Opportunity**
By utilising flexible trial designs, involving and engaging patients, carers and communities, Australia can achieve better clinical outcomes for patients and increase patient access. The patient reach and efficiencies gained through patient-centric approaches should attract more local and international sponsors of clinical trials.

Australia's recent progress in patient recruitment is a testament to the development of more innovative patient-centric approaches to clinical trials. Examples of initiatives in this regard include:

- ACTA and CT:IQ deepening patient involvement and engagement
- the ongoing development of tele-trials capability in Australia
- stronger engagement with indigenous communities through, for example, NHMRC's "Keeping Research on Track" updated guidelines.

### *ACTA and CT:IQ's Consumer Involvement and Engagement toolkit*

Tools to enhance patient centricity in clinical trials are already available to sponsors. ACTA and CT:IQ, have developed a Consumer Involvement and Engagement toolkit that provides helpful advice for researchers and research organisations conducting patient-centred clinical trials. The toolkit emphasises two principles:

- improving the readability of participant information to make trials more attractive to potential participants
- providing proactive outreach to consumers and the community so that they are better informed about why, how, where and by whom the research is conducted. This open style of engagement may include the sharing of research findings directly with patients as well as public messaging and dialogues using lectures and social media.[96]

In addition, ACTA have been working toward increasing the awareness of the needs of culturally and linguistically diverse (CALD) populations. Developing patient-centric approaches to support the needs to these groups increases equitable access and drives greater diversity in clinical trial patient cohorts.[97]

### *Development of tele-trials capability in Australia*

Tele-trials allow a clinician at a primary site to treat patients at satellite sites, facilitating patient access at a convenient location for the patient. Various pilots and programs that have supported the development of tele-trial capabilities – as highlighted in the previous chapter–have been wholly successful:

- patients have provided uniformly positive feedback about their experience on their tele-trials, suggesting that they would not have participated if not for the tele-trial capability[98]
- sponsors are experiencing higher rates of patient recruitment, which may result in lower costs per patient in the medium- to long-term despite higher initial set-up costs.[99]

[96] ACTA: and CT:IQ, Consumer Involvement and Engagement Toolkit website, accessed April 2021
[97] ACTA, Clinical trial awareness and access amongst culturally and linguistically diverse (CALD) populations: environmental scan, June 2020
[98] Queensland Health, Queensland Health Teletrials Pilot Analysis Report, 2019
[99] Victorian Comprehensive Cancer Centre, Adopting the teletrial model for safe trial delivery, May 2020

Following the completion of the initiatives to date, the CTPRG in collaboration with all state and territory governments has developed a National Tele-trials Compendium to support a national approach to tele-trials. It includes:

- National principles for Tele-trials in Australia based on the International Council for Harmonisation Guideline for Good Clinical Practice
- National Standard Operating Procedures for Clinical Trials agreed to by all states and territories to help organisations standardise their procedures for clinical trials and tele-trials.

However, there remains work to be done to reduce the additional costs incurred and ensure the fullest development and widespread adoption of the tele-trials model for clinical trials. Issues that need to be addressed include:

- Tele-trials can be risky for non-Pharmaceutical Benefits Scheme (PBS) approved drugs, FTIH trials and Phase I exploratory trials due to operational issues such as transporting and administering the drug or device. Hospitals have indicated that this is especially important in the case of oncology trials.
- In addition to the possible risks, the additional logistics for transporting, storing and administering the drug or device, in particular cold chain product, to satellite sites or patient homes can become expensive.
- Enhanced education of regionally based clinicians on the tele-trial process is required to ensure their practices are up to date.
- Infrastructure may need to be developed at satellite sites to enable them to provide adequate patient care and monitoring. For example, this might include the need for a nearby emergency department at a regional/remote location.
- Electronic medical records (EMR) along with remote access is essential to support the full adoption of tele-trials. Currently the management and viewing of data records can be challenging with not all hospitals utilising EMR and where they are, different systems are in use. Adoption of EMR and allowing remote access will be needed to manage and view data.[100]

### *NHMRC's research guidelines for engaging Indigenous Australians*

Clinical trials within Australia's Indigenous communities are typically focused on prevention, behaviour modification, non-drug and non-surgical research.[101] There is currently limited industry involvement in indigenous community trials given the challenges of remoteness, the high degree of comorbidities associated with Indigenous patients, poor health literacy and a high turnover of clinicians and researchers.[102,103] Adopting patient-centric approaches is critical to providing equitable access to clinical trials for these populations.

Health research involving Indigenous peoples should engage the community, in addition to respecting the individual patient's needs. This is evidenced by the need, in some cases, to involve the whole community in seeking consent. Consent is defined as being voluntary, based on sufficient information and with adequate

[100] Based on L.E.K. and MTPConnect interviews with sector participants

[101] Kylie Hunter, et al., The landscape of clinical trial activity focusing on indigenous health in Australia from 2008 to 2018, October 2019

[102] Alan Cass, Challenges and Successes in Clinical Research with Aboriginal and Torres Strait Islander Australians, November 2018

[103] Joan Cunningham and Gail Garvey, Are there systematic barriers to participation in cancer treatment trials by Aboriginal and Torres Strait Islander cancer patients in Australia?, *Australian and New Zealand Journal of Public Health*, December 2020

understanding. Researchers should aim for mutual understanding between researchers and participants throughout the entirety of the clinical trial and/or health research more broadly.[104]

## Digital health

Digital adoption is a megatrend highlighted in MTPConnect's *Medical Technology, Biotechnology & Pharmaceutical Sector Competitiveness Plan* (2020). The digital evolution will drive a significant shift in data processing, enable greater consumer control and facilitate precision medicine to ultimately improve patient outcomes. In parallel, the efficiency of clinical trials will improve through increased patient recruitment and measurements of treatments.[105]

Digital adoption within the MTP sector has been accelerated during COVID-19 as indicated in *MTPConnect COVID-19 Impact Report 2nd edition* (2020). In Australia, the Australian Government has invested more than $114 million to extend telehealth services until the end of 2021.[106] The CTPRG released guidelines to encourage virtual visits, telehealth and electronic consent within clinical trials. This urgent guidance for the clinical trials community on trial conduct within the COVID-19 restricted environment was developed with the approval and collaboration of all jurisdictions, NHMRC and TGA.[107] This advice was mirrored internationally by regulators such as the FDA and EMA who advocated for greater use of technology within trials, allowing industry sponsors to adopt virtual equivalents for patient consultation, consent and remote site monitoring. [108,109]

Although nascent in their development, digital technologies are being leveraged to improve the efficiency of patient recruitment to clinical trials.[110] Two such innovative technologies are highlighted below.

| | |
|---|---|
| ClinTrial Refer | ClinTrial Refer connects patients, doctors and clinical trial sites through a mobile app with the aim of increasing patient participation in clinical trials via clinical trial networks. The Australian-centric app provides a portal for consumers to search for relevant trials by discipline, therapeutic area and other criteria.[111] |
| Opyl | Opyl also uses AI-assisted technologies to understand and improve clinical trial protocol design and accelerate recruitment. It achieves this by leveraging 300,000 registered clinical trials as well as real world data, including patient reported outcomes (PRO) and the continuous data and dialogue stream embedded in social media.[112] |

AI is proving to be a powerful tool in driving efficiency in patient recruitment. Fuelled by the increasing amounts of medical data from EMR, devices and health apps, MTP researchers are turning to AI to screen and recruit patients for clinical trials. Natural language processing (NLP), a branch of AI that empowers computers to decode written and spoken word, can be applied to doctors' notes, pathology reports and the inclusion and exclusion criteria written in text. Automated AI-powered systems show promise in the screening of large patient cohorts to identify suitable subjects. In Australia, AI matching

[104] NHMRC, Keeping Research on Track II, August 2018
[105] Omer Inan, et al. Digitizing clinical trials, *Nature Partner Journal Digital Medicine*, December 2020
[106] The Hon. Greg Hunt MP, Universal Telehealth extended through 2021, 26 April 2021
[107] Department of Health, COVID-19: Guidance on clinical trials for institutions, HRECs, researchers and sponsors, 2020
[108] Ben Faircloth and Andre Valente, COVID-19 and Clinical Trials: Accelerating the Adoption of eClinical Technology, *L.E.K. Insights*, 2020
[109] Patrick Hughes, Over 300 Clinical Studies Benefit from CluePoints' COVID-19 Risk Management Support, *CluePoints press*, August 2020
[110] Respondents to the industry-wide survey conducted for this report indicated that around 11 per cent of industry sponsored clinical trials reported in the survey leveraged digital technologies
[111] ClinTrial Refer website, accessed March 2020
[112] Opyl Technologies website, accessed March 2020

in lung cancer patients scored approximately 92 per cent for accuracy in overall trial eligibility and 94 per cent in specificity for trial matching criteria.[113]

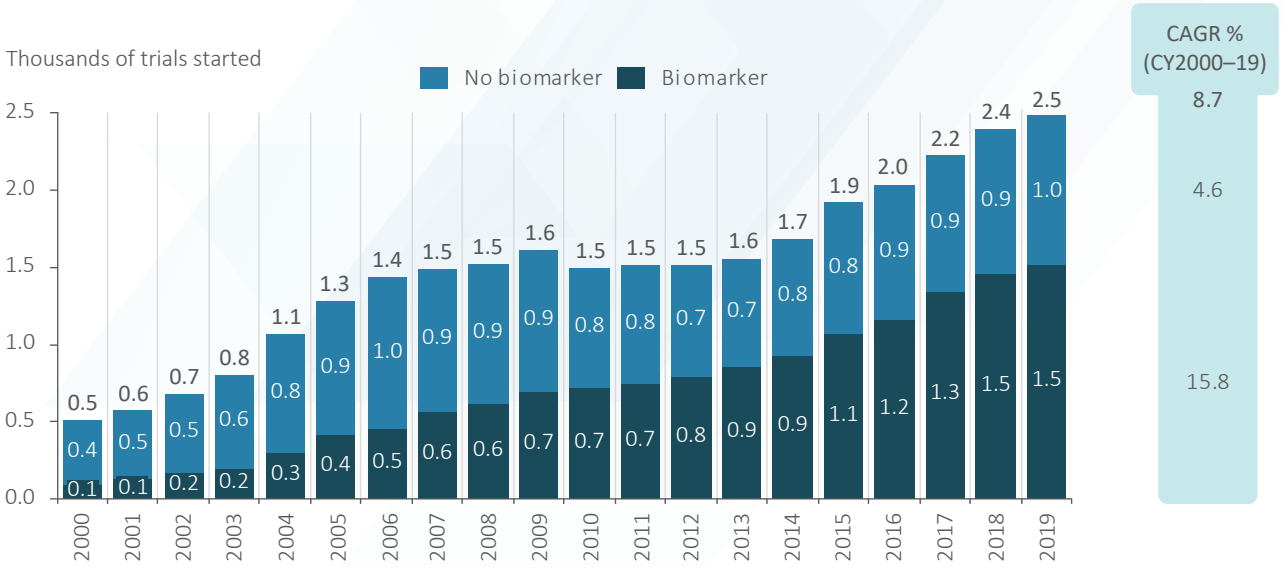
**Opportunity**
Significant upside exists for Australia to increase the adoption of digital health and AI in clinical trials. Simplifying policies, including those related to remote access to EMR and patient data, will improve equitable access to clinical trials, support improved patient recruitment and improve efficiency in trial conduct.

## Precision healthcare

Technological advancements are driving the growth of precision healthcare solutions worldwide, where treatments are tailored to the genetic make-up of individual patients. These bespoke healthcare solutions are enabled through rapid advancements in technology such as comprehensive genomic profiling (CGP) and regenerative medicine, along with the development of diagnostic medical devices and the supporting data and analytics capabilities. There has been significant growth in precision medicine, with the proportion of FDA approved targeted therapies doubling from 21 per cent in 2014 to 42 per cent in 2018.[114] Precision medicine is becoming a central feature of cancer treatments and Australia's continued strength in oncology trials is a direct result of its capabilities relating to CGP and precision medicine.

The use of biomarkers is one example of precision medicine. In oncology, clinical trials with biomarkers have become the norm rather than the exception. In 2019, oncology biomarker trials made up 61 per cent of all oncology trials; 19 years earlier in 2000 they only made up 18 per cent of oncology trials, as shown in the figure below.

**Biomarker utilisation in global oncology trials (CY2000–19)**




Source: L.E.K. Survey and Analysis; AACT

[113] Marliese Alexander, et al., Evaluation of an artificial intelligence clinical trial matching system in Australian lung cancer patients, *Jamia Open*, May 2020

[114] Personalized Medicines Coalition (PMC), Personalized Medicine at FDA – A Progress & Outlook Report, 2019

Several countries have recognised the potential of precision medicine and have begun investing in relevant infrastructure to enable broad-based genomic screening of patients. South Korea now has a national biobank; other elements of a precision medicine ecosystem are also in place, including strong health information systems and national genomics policy priorities.[115] The United Kingdom has committed public funding to sequencing five million whole genomes by 2023 through Genomics England, a company under the Department of Health and Social Care.[116]

In Australia, CGP and gene technology are used in approximately 5 per cent of trials according to respondents to the MTPConnect industry survey. Australia has started to develop its precision medicine infrastructure, with several initiatives underway:

- Omico's Molecular Screening and Therapeutics (MoST) program, which is funded through a combination of public and private funding, is one such example. The purpose of MoST is to screen tumour tissue for DNA or protein markers to identify a biomarker that can be used to guide treatment. The MoST study, about to enter its fifth year, has already screened more than 3,000 late-stage cancer patients and enrolled more than 300 of those patients onto targeted therapy clinical trials. The program has evolved from proof-of-concept screening run only in New South Wales, to a national program delivered through a network of leading cancer treatment and research centres, encompassing every state and territory in Australia.
- The ASPiRATION study, a collaboration between the Federal Government, Roche, the Australasian Lung Cancer Trials Group (ALTG), the Australia Genomic Medicine Centre (AGCMC) and the NHMRC will further grow Australia's precision medicine clinical trials capability. This national, multi-centre prospective observational and interventional cohort study will see 1,000 newly diagnosed lung cancer patients provided with comprehensive genomic profiling, with more than 100 eligible patients enrolled onto sub-studies to test emerging treatments. The ASPiRATION study will also consider how to establish data arrangements that meet the privacy, security and safety needs of patients, clinicians, government and industry.[117]

**Opportunity**
Australia has the opportunity to further develop the infrastructure, capabilities and skills required to support precision medicine trials, e.g. by implementing the broad-based genomic screening capability (as developed in countries such as South Korea) and establishing digital technologies enabling streamlined patient matching onto clinical trials. Doing so will ensure Australia remains an attractive destination for cancer trials and the next generation of biomarker enabled trials (e.g. neurological, rare diseases).

## Innovative trial design

Innovative trial designs include studies with a single common control arm, studies with combinations of multiple investigative drugs (platform/umbrella/basket trials), studies with only one participant (N-of-1 trials) and studies that add and stop treatment arms (adaptive clinical trials).

[115] OECD, OECD Reviews of Public Health: Korea–A Healthier Tomorrow, March 2020
[116] Genomics England, Landmark strategy launched to cement UK's position as global leader in genomics, accessed March 2021
[117] Genomic Cancer Clinical Trials Initiative, February 2020 Workshop Report, April 2020

- Adaptive clinical trials provide more flexibility in their approach than traditional clinical trials. They are already used in approximately 15 per cent of clinical trials in Australia according to industry respondents to the MTPConnect industry survey and this has been growing in recent years.[118] The adaptive trial method is similar to agile project management. Adaptive trials provide an iterative framework for clinicians to make changes to how patients are cared for by observing which patients are reacting most effectively to their treatments. This allows clinicians to eliminate ineffective treatments, assign more patients to the treatment arms that are most effective and determine what treatments are best for different types of patients.[119] Adaptive trials can improve trial efficiency and effectiveness by allowing researchers to answer more complex questions at a faster rate. They are particularly useful for rare diseases where patient recruitment is a challenge.
- N-of-1 trials compare the effectiveness of interventions at the level of a single individual. They are an emerging as an important tool for rare diseases and enable researchers to capture additional data which would otherwise not be possible in traditional clinical trials with a more comprehensive patient participation.[120]
- Platform/umbrella/basket trials require a single clinical trial protocol describing the objectives, design, methodology and safety considerations where multiple treatments are simultaneously evaluated under the one protocol. Utilisation of these trial designs has increased rapidly.[121] This approach is becoming increasingly common.[122] These studies are likely to continue to grow as companies look to develop innovative treatment combinations and pathways. They promise to deliver more efficient treatments with fewer patients, fewer patient failures, in less time and with greater probability of success.

Most of the initial traction for innovative trial design has occurred in oncology. The focus will expand beyond oncology trials, most likely to other therapeutic areas such as neurological disorders and rare diseases.[123]

With complexity comes new challenges. Some MTP companies report difficulty in managing research ethics and governance issues because current processes cannot cope with submissions of such complexity. Ethics and governance processes need to adapt to facilitate the use of more innovative clinical trials. Some committees have begun to accept larger, more complex trial protocols, while others require each sub-study to be submitted separately.[124]

**Opportunity**

Innovative trial design adoption will help Australia remain at the forefront of medical research in complex and rapidly changing disease areas. To continue to be a country of choice for innovative trials, Australia needs to develop its ethics and governance capabilities in order to holistically encompass platform/umbrella/basket, N-of-1 and adaptive trials.

[118] Parexel and The Economist Intelligence Unit, The Innovation Imperative: The Future of Drug Development, May 2019

[119] Adaptive Health Intelligence, What is an adaptive clinical trial?, accessed April 2021

[120] Bethany Percha, et al., Designing Robust N-of-1 Studies for Precision Medicine: Simulation Study and Design Recommendations, *Journal of Medical Internet Research*, October 2018

[121] Jennifer Rogers, The opportunities and challenges of basket studies, *Phastar,* November 2019

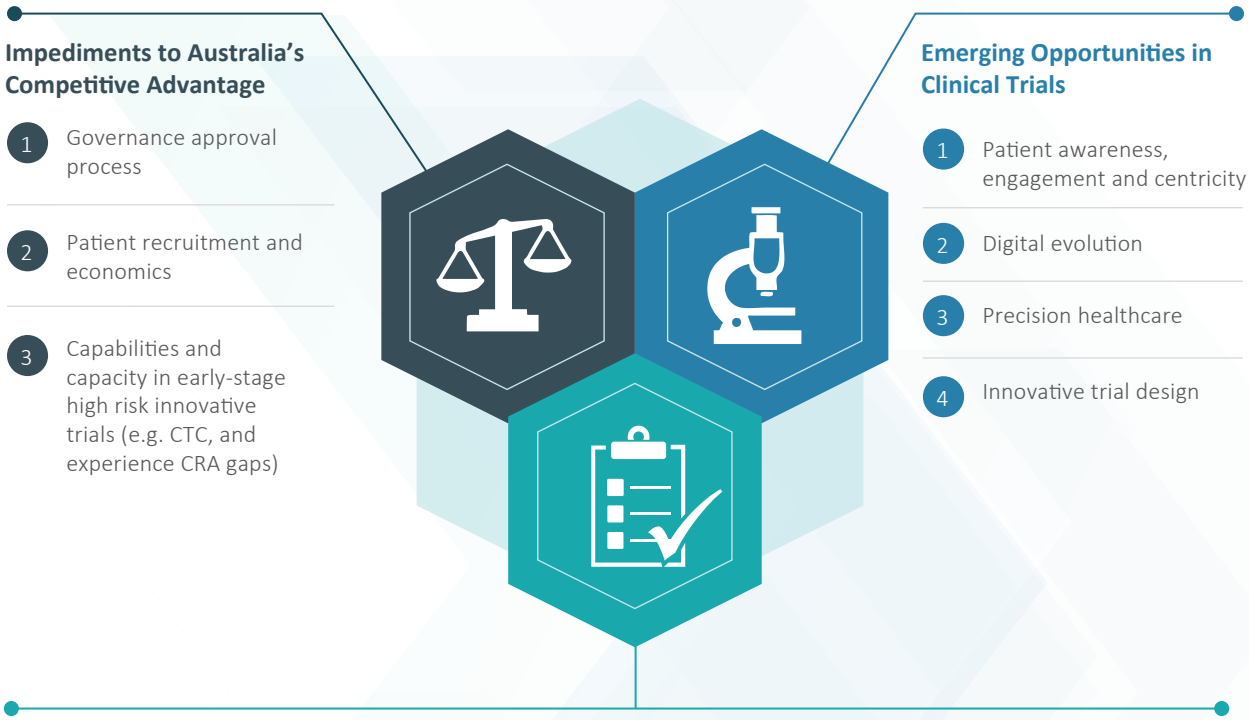
[122] ACTA, Adaptive Multi-Arm Platform Trials: Benefits and Efficiencies website, accessed April 2021

[123] FDA, Master Protocols: Efficient Clinical Trial Design Strategies to Expedite Development of Oncology Drugs and Biologics Guidance for Industry, 2018

[124] L.E.K./MTPConnect interviews with local MTP sector experts

## Priorities for the future

This report has reviewed the progress and achievements of the clinical trials sector in Australia over the past four years. Despite significant success and evident growth, there is a need for continuing improvement for Australia's clinical trials ecosystem to maintain and enhance its global competitiveness. Looking ahead, to take advantage of emerging opportunities and alleviate existing impediments, four priorities for the future have been identified (see figure below).




The following section discusses four priorities for clinical trials sector resilience and growth:

### 1. Continue to optimise efficiency in ethics and governance processes

Streamlining ethics and governance approval processes between each state and territory is critical for Australia's continued success in attracting clinical trials. As discussed earlier in this report, Phase III and IV trials typically involve larger patient cohorts and, as a result, span multiple states and multiple sites. The complexity in site governance processes is consequently amplified for sponsors conducting later stage trials, or Phase I and Phase II trials conducted across multiple states.

Implementation of the CTGF will ensure clinical trials are embedded as a required part of service delivery for hospitals. This will drive efficient and rapid site start-up processes, strengthen governance and provide clarity for roles and responsibilities of investigators, RGOs, CTCs and other clinical staff. This will in turn improve healthcare outcomes for patients. To this same end, process improvement may include the following:

- reconsider the definition of a 'site', pivoting away from the physical location of a hospital to define the site at the LHN/LHD level, where indemnity insurance and sign-off ultimately reside
- standardise the Site-Specific Assessment form and electronic submission platform, such that the information relating to the general clinical trial information and the requirements and format of site-specific information are standardised across sites
- continue to improve the framework and timelines for ethics approvals by delivering the National Front Door platform to connect and modernise the systems used by each of the state bodies in a way that achieves increased efficiency in startup across all states and territories. This should be inclusive of early-phase trials as well as both public and private hospitals.

These efforts to harmonise and streamline processes will minimise duplication of effort for sponsors, investigators, hospitals and HRECs and help improve trial start-up timelines.

### 2. Grow the clinical trials workforce and develop its capabilities

As highlighted in this report and detailed in the *MTPConnect REDI Initiative Skills Gap Analysis Second Report* (2021), Australia has key shortages of experienced CRAs and CTCs.[125] If unaddressed, these shortages will severely constrain the sector's ability to expand. The key skills gap imperatives:

- The shortage of experienced CRAs. In the short-to-medium term, there is a need to expand the pool of junior CRAs and provide them with subsidised training (e.g. through paid traineeships). In addition, there is an opportunity to develop training programs for senior CRAs that develop their coaching/mentoring skills to allow them to train their junior colleagues more efficiently. In the longer term, an accredited training qualification would make it less necessary for organisations to resource training individually. In turn, this will provide greater consistency in training CRAs across the sector. Whilst these solutions may not reduce the high turnover rate of CRAs, they will allow for a larger cohort of experienced CRAs to be developed over the next two to three years.
- The shortage of experienced CTCs. Increasing the profile of the CTC role at the undergraduate level would help, as would feeder programs that raised awareness of clinical trials career paths available. In the medium term, subsidising on-the-job training would encourage science and nursing graduates to take up CTC roles in hospitals and accelerate the development of potential CTCs (e.g. aspiring nurses and clinical staff). In the longer term, there is an opportunity to develop a national competency framework that standardises the training and development of CTCs and pivot budgeting and resourcing to a more holistic model from the current per-trial basis.

[125] MTPConnect, REDI Initiative Skills Gap Analysis Second Report, 2021

### 3. Enhance patient recruitment per site to improve site economics for clinical trials

Australia continues to be disadvantaged by its relatively small population, as noted in the *Clinical Trials in Australia* (2017) report. There is a need to enhance patient participation in clinical trials to remain competitive on the international stage by significantly improving trial economics for industry sponsors. Several levers are available to Australia to increase patient recruitment, including:

- Increasing patient centricity and providing equitable access. As highlighted earlier in this chapter, taking advantage of the emerging opportunities in patient centricity can play a key role in patient recruitment. Patient centricity can be achieved through the wider adoption of tele-trials for the significant segment of Australians living in RRR communities. In parallel, continued support for the use of digital technologies will increase the number of patients that are targeted for clinical trial recruitment and managed at each trial site.
- Implementation of the CTGF. Embedding clinical trials into the routine practices of hospitals will support patient recruitment by increasing executive oversight of clinical trials, reaffirming the roles and responsibilities for clinical trial delivery and strengthening clinician awareness.
- Funding of clinical trials networks will boost clinician awareness through more effective clinical research and improved health outcomes. Doing so promises better patient outcomes through innovative treatments at the forefront of medicine, including precision healthcare solutions, which are becoming central to the treatment of novel cancers, neurological conditions and rare diseases.

### 4. Enhance transparency of the state of the sector

Over the past four years, there has been significant improvement in the collection of clinical trials data. The ANZCTR is now established as the national registry for tracking clinical trial activity. Most states have implemented electronic systems for clinical trial governance that allow tracking of timelines for ethics and site governance approvals. Remaining priorities include the continuous improvement of data collection and reporting to ensure visibility of the progress made.

To further enhance transparency, the quality and granularity of clinical trials data captured could be improved. Immediate opportunities include:

- Mandating the requirement for ANZCTR to be updated annually, in line with updates to ethics bodies. As advised by WHO, these updates should include whether study results have been published and provide relevant details in the registry. There is precedence with similar requirements set by the FDA in the United States for ClinicalTrials.gov. The FDA has indicated that updates benefit the sector by providing a public record of standardised study results, reduce outcome reporting biases, facilitate systemic reviews of research and promote the fulfilment of ethical obligations to trial participants for their contribution to medical knowledge.[126]
- Reviewing and improving data collection and processing through the National Aggregate Statistics (NAS) regarding clinical trials start-up timelines in each of the states.

[126] US National Institutes of Health (NIH), Why Should I Register and Submit Results?, 2020

To make the most out of the available data, it is necessary to systematise the reporting and tracking of key performance indicators of clinical trials. This could be achieved by:

- Establishing regular reporting schedules for clinical trials activity and other Key Performance Indicators (KPIs). This may include regular reporting of clinical trial start-up timelines and potentially also reporting on adherence to the CTGF.
- Expanding reporting to include executive KPIs embedded in organisations to facilitate continuous improvement among sector participants, including state and federal governments, industry sponsors and hospitals.

## Clinical trials sector outlook

In summary, the outlook for the clinical trials sector in Australia is positive. Australia has successfully nurtured core competitive advantages, which it is actively working to defend and enhance:

- improvements in the start-up timelines for clinical trials have been achieved through streamlining ethics approvals and developing new approaches to site governance
- industry and medical researchers in Australia remain world class, a research reputation that is a significant attraction for clinical trials sponsors
- prominent public investment has expanded the reach of clinical trials in Australia (e.g. MRFF-funded National Critical Research Infrastructure initiative)
- innovative solutions to improve the efficiency and reach of patient recruitment are being embraced by clinical trials stakeholders
- the extension of the R&DTI scheme has mitigated the higher cost of clinical trials in Australia (versus more populous lower labour cost countries), particularly for small- and medium-sized entities and early-phase studies.[127]

Despite these advantages, there are further opportunities to improve patient access and retention in the long term through the adoption of consumer involvement and engagement, increased utilisation of decentralised trials and greater inclusion of groups such as Indigenous communities. There are opportunities to improve trial efficiency with the emergence of digital health, particularly through the use of AI in driving patient recruitment. And there are opportunities for Australia to remain at the forefront of clinical trials, through the development of the infrastructure, capabilities and capacity required to support precision medicine trials and innovative trial designs.

COVID-19 has provided a platform from which Australia can further strengthen its reputation as a clinical trials destination. NHMRC, MRFF and MMS government- funded investments will also continue to add momentum to the growth of the clinical trials sector over the coming years. With a steadfast focus on the priority areas outlined in this report, Australia is well placed to continue to grow its clinical trials sector, outcompete other countries for foreign investment, create skilled jobs and deliver health benefits to Australians. An unrelenting focus on continuous improvement will be critical to our success.

[127] MTPConnect industry survey, 2021 and L.E.K./MTPConnect interviews with local MTP sector experts

# Appendices

## Appendix 1: List of senior sector stakeholders consulted and survey respondents

This report was developed with input from 61 senior sector executives, following scoping discussions with the Clinical Trials in Australia Advisory Group and through targeted stakeholder consultations. Many of these executives are also representatives of AusBiotech, Medicines Australia (MA), the Medical Technology Association of Australia (MTAA) and the Research & Development Taskforce (R&DTF). The perspectives shared by these senior stakeholders from industry associations, companies, regulatory bodies, research organisations, government representatives and funders have informed the drivers and impediments of Australia's competitive advantage, emerging opportunities and priorities for the future. MTPConnect would like to thank all those who shared their time and insights through these stakeholder consultations. The list of stakeholders is shown in the table below.

| Name | Organisation | Name | Organisation |
|---|---|---|---|
| Abdul Ekram | Australian Trade Commission | David Wilks | Bristol Myers Squibb |
| Allyson Essex | Department of Health | Dr Deama Amr | Medtronic |
| Amanda Leach | Menzies School of Health Research | Falko Thiele | BIOTRONIK |
| Angie Barba | ANZCTR, University of Sydney | Hank Sciberras | Deloitte |
| Prof Andrew Davidson | The Royal Children's Hospital | Helen Aunedi | Roche |
| Anita van der Meer | NSW Ministry of Health | James Cokayne | NSW Ministry of Health |
| Dr Antonio Penna | NSW Ministry of Health | Jane Kelly | CMAX Clinical Research |
| Brendon Douglas | Menzies School of Health Research | Jane Nelson | Menzies School of Health Research |
| Candice Fitzgerald | Boehringer Ingelheim | Dr Janelle Bowden | AccessCR |
| Carrie Bloomfield | GlaxoSmithKline | Dr Jodi Glading | Tasmanian Department of Health and Human Services |
| Catherine Bourgeois | Abbott | Prof John Zalcberg OAM | Australian Clinical Trials Alliance |
| Prof Christopher Brook PSM | COAG Health Council | Juliana Potulic | AusBiotech |
| Christy Thiel | Edwards Lifesciences | Karen Thompson | Queensland Health |
| Dr Daniela Caiazza | Novotech | Dr Kurt Lackovic | Cancer Trials Australia |
| Dr Darren Gibson | Department of Health, Western Australia | Karen Parr | AusBiotech |
| Dr David Lloyd | Southern Star Research | Kylie Sproston | Bellberry |

| Name | Organisation | Name | Organisation |
|---|---|---|---|
| Laura Ling | Department of Industry, Science, Energy and Resources | Dr Nicole Yu | Johnson & Johnson |
| Lauren Macnaughton | Eli Lilly and Company | Peter Komocki | Medicines Australia |
| Leanne Wells | Consumers Health Forum of Australia | Dr Peter Thomas | Association of Australian Medical Research Institutes |
| Dr Lewis Campbell | Flinders University | Petra Bismire | Therapeutic Goods Administration |
| Linda Cristine | Department of Jobs, Precincts and Regions Victoria | Robert Kent | The Kinghorn Cancer Centre |
| Mandy Crowley | Baxter International | Sabbu Upreti | Therapeutic Goods Administration |
| Marian Lieschke | Parkville Cancer Clinical Trials Unit, Peter MacCallum Cancer Centre | Sebastian Brash | Department of Industry, Science, Energy and Resources |
| Dr Mark Flynn | Global Edge Medtech Consulting | Dr Shanny Dyer | ARCS Australia |
| Dr Mary-Beth Brinson | Cochlear | Sharon Charles | Paradigm Biopharmaceuticals |
| Dr Megan Robertson | St Vincent's Hospital Melbourne | Dr Suzanne Hasthorpe | Department of Jobs, Precincts and Regions Victoria |
| Melissa Hagan | Queensland Health | Terrie O'Brien | Department of Health |
| Dr Melina Willson | ANZCTR University of Sydney | Tracey Mealey | Johnson & Johnson |
| Michelle Hillard | Department of Health | Wendy Keech | Health Translation SA |
| Mitch Kirkman | Novartis | Zoe Armstrong | MSD Australia |
| Neina Fahey | Medical Technology Association of Australia | | |

In addition, MTPConnect would like to specifically acknowledge the assistance of Carrie Bloomfield, Helen Aunedi and Peter Komocki for supporting the MTPConnect industry survey design. Many thanks also to Karen Parr (AusBiotech), Shanny Dyer (ARCS), Peter Komocki (MA), Neina Fahey (MTAA), Carrie Bloomfield and Helen Aunedi (R&DTF) for assisting with distributing and canvassing responses to the industry survey. The following businesses submitted responses to the MTPConnect industry survey. Respondent companies reported starting 237 trials in 2019, a number that represents 39 percent of the total 601 industry-sponsored clinical trials started in that year.

| Company | Company |
|---|---|
| AbbVie | GlaxoSmithKline |
| Amgen | Global Edge Medtech Consulting |
| AstraZeneca | Merck |
| BeiGene | MSD Australia |
| Biogen | Novartis |
| BIOTRONIK | Novo Nordisk |
| Bristol Myers Squibb | Novotech |
| CMAX Clinical Research | Roche Australia |
| Eli Lilly and Company | Southern Star Research |
| Gilead Sciences | |

Finally, MTPConnect would like to acknowledge the assistance of Angie Barba, Mason Aberoumand and Melina Willson (ANZCTR, NHMRC Clinical Trials Centre, University of Sydney) for the provision of ANZCTR data and analysis, as well as Matthew Sammels and Chelsea Stewart (NHMRC) for the provision of NHMRC data.

## Appendix 2: Methodology for analysing clinical trials activity

Two main clinical trials registries were used in analysing data in this report – ANZCTR and ClinicalTrials.gov. Background information regarding each registry and the limitations of data therein are provided below.

### ANZCTR

Clinical trials activity in Australia has been measured in this report using ANZCTR data received 18 March 2021. ANZCTR is a voluntary registry of clinical trials. ANZCTR supplements data relating to clinical trials on its registry with clinical trials registered on ClinicalTrials.gov, which is mandated by the US government for studies initiated after January 2017 and conducted wholly or partly in the United States.[128] As the number of trials that are never registered is unknown, an estimate has not been provided in 2021. However, for trials that have been registered, the ANZCTR captures around 96% of studies recruiting in Australia according to ANZCTR.[129] This is broadly in line with the coverage of data reported in the *Clinical Trials in Australia* (2017) report.

The clinical trial activity analysed in this report leverages the number of clinical trials started, rather than the total number ongoing. These two metrics differ, as many trials run longer than one year. However, since sponsors are not required to update their trial status and data in ANZCTR, the data quality for ongoing trials is somewhat limited. Therefore, the number of clinical trials started remains a proxy for the level of clinical trial activity in Australia and has been used to analyse trends since 2015.

The data is segmented in this report along the following dimensions to analyse drivers of clinical trial activity:

- Sponsor type – which indicates the individual or organisation responsible for the trial. In this report sponsors have been classified into either industry (capturing 'industry' and 'commercial sector/industry' ANZCTR sponsor types), university, individual, government and hospital (including 'government body', 'NIH', 'US Fed' and 'hospital' ANZCTR sponsor types) and other (including 'other', 'charities/societies/foundations' and where no other sponsor was indicated).
- Study type – which indicates whether a clinical trial was conducted on an interventional or observational basis.
- Intervention type – which may or may not include a drug (including 'medicine' or 'biological'), a device and/or other studies (including 'dietary supplements', 'behavioural' and 'procedural' interventions, etc.).
- Trial phase – which includes phases I to IV of the drug development life cycle. Where combined phase trial designs are used in a registered trial, the higher phase is presented in this report. For example, a combined Phase I and II trial is presented in this report as a Phase II trial.
- Therapeutic area – the largest of which include oncology, mental health, neurology, musculoskeletal and cardiovascular. ANZCTR and ClinicalTrials.gov users can select multiple therapeutic areas when registering a clinical trial. As a result, it is not possible to sum the number

[128] US National Institute of Health, FDAAA 801 and the Final Rule, accessed April 2021

[129] Input provided by ANZCTR

of clinical trials by each therapeutic area. For example, clinical trial relating to the treatment of lunch cancer may be counted in this report under both respiratory and oncology therapeutic areas.

- State – which refers to each of Australia's eight states and territories. A clinical trial is defined as existing within a state when it has at least one trial site within that state. Most clinical trials occur across multiple sites and often in multiple states – in this case the clinical trial is counted more than once in the data presented in this report.

Participation statistics in clinical trials is available on ANZCTR. However, these statistics were not considered in this report because of infrequent updating by sponsors as described above. In addition, the numbers reported are often global patient recruitment/participation numbers rather than Australia-specific.

### ClinicalTrials.gov

For global comparisons, this report exclusively uses industry-sponsored trials on ClinicalTrials.gov extracted 21 February 2021. ClinicalTrials.gov is widely considered to be the most comprehensive registry, with information for approximately 270,000 studies in over 200 countries. Despite this, the registry is reliant on sponsors reporting appropriate information regarding their trials, so there remains limitations around the accuracy, completeness and timeliness of information in the registry.[130] This timeliness issue extends to more than half of trials and, for this reason, the data originally presented in the *Clinical Trials in Australia* (2017) report has been updated to reflect changes in the source registries.[131]

[130] Tony Tse, et al., How to avoid common problems when using ClinicalTrials.gov in research: 10 issues to consider, *British Medical Journal*, March 2018

[131] The Lancet, Fewer than half of US clinical trials have complied with the law on reporting results, despite new regulations, *EurekAlert*, January 2020

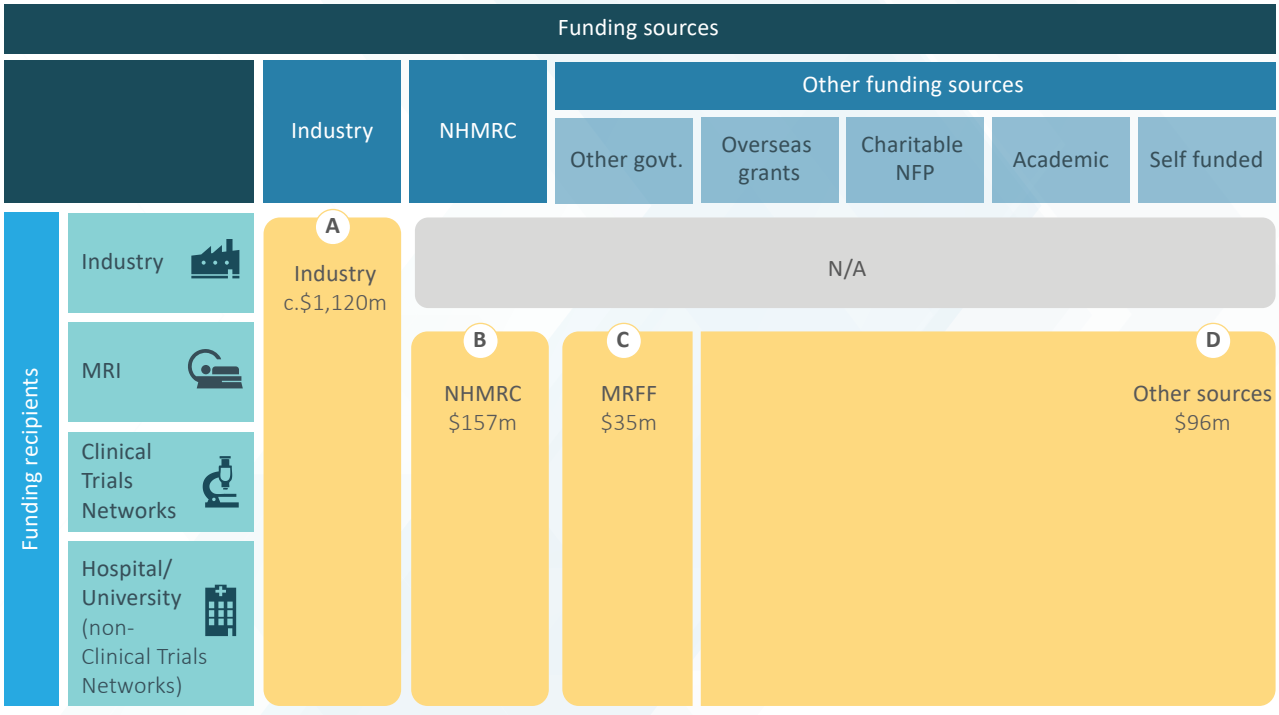
## Appendix 3: Methodology for economic value calculations

The availability of economic data within the clinical trials sector is widely acknowledged to be limited. The value of clinical trials is calculated using a combination of the MTPConnect industry survey, existing secondary data and the application of scale factors to derive a final estimate of the value of clinical trials to the Australian economy.

### Clinical trial expenditure

Total trial expenditure is the total money spent directly on the conduct of clinical trials within Australia. It includes private investment in clinical trials by MTP companies, grants and funding from the NHMRC and MRFF and a range of other funding contributed by various sources which are named within this appendix below D) *Other sources.*

The methodology used to estimate total expenditure on trials is based primarily on the sources of funding (industry expenditure, NHMRC and MRFF), with the residual balance of expenditure estimated based on the expenditure within universities, hospitals and independent MRIs, as illustrated in the figure below.




The methodology represents the minimum expenditure on clinical trials, as some areas of expenditure that exist are difficult to quantify, including:

- additional clinical trials expenditure within clinical trial networks (estimated to be approximately $12 million in 2015)

- additional MRFF funding of R&D and commercialisation initiatives, which include clinical trials, but where the portion for clinical trials is not separately reported. Some but not all of this MRFF funding may be captured within D) Other sources (calculation explained below).

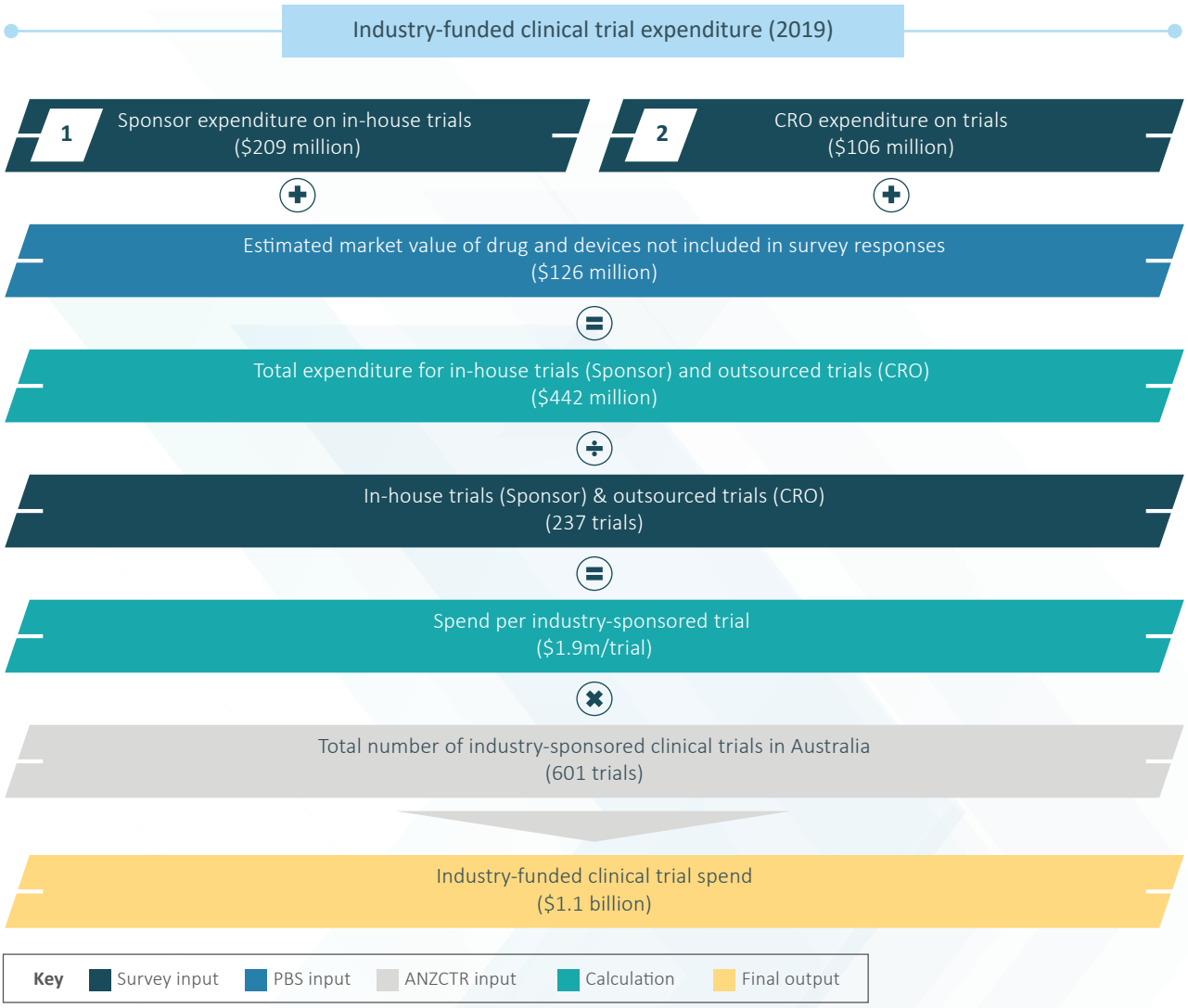
In the *Clinical Trials in Australia* (2017) report, the expenditure from MRI and clinical trial networks were calculated separately. In this report, the data used to calculate MRI-funded expenditure has been expanded to include university-based medical research institutes and hospital-based medical research institutes. This approach does not capture expenditure on, or the funding of, IITs conducted through clinical trial networks, the value of which (estimated to be $12 million in 2015) is negligible compared to overall sector expenditure. Despite the minor methodological deviation in the estimation of trial expenditure, comparisons between the *Clinical Trials in Australia* (2017) report and this report are directionally valid. The following sub-sections describe in detail the methodologies used to estimate each component of the overall clinical trial expenditure estimate.

### *A) Industry expenditure*

Industry expenditure on clinical trials was calculated based on primary research data collected in the MTPConnect industry survey of MTP companies. Respondent companies reported starting 237 trials in 2019, a number that represents 39 percent of the total 601 industry-sponsored clinical trials started in that year. The majority of respondents included members of AusBiotech, MTAA, R&DTF and MA. They were typically larger corporates rather than start-ups/scale-ups and include several multinational corporations.

To ensure total expenditure was captured, analysis was undertaken to determine whether the costs of drugs and devices provided in clinical trials was provided in survey responses. Where there were gaps, this report's analysis leveraged the Pharmaceutial Benefits Scheme (PBS) to determine market estimates for trials that did not report on drug costs. The number of companies that provided information on medical device trials was limited. Where available, the costs of devices used in clinical trials were also included.

The aggregate of the survey responses and drug costs was $442 million – approximately $1.9 million per trial started. With 601 industry-sponsored clinical trials started in 2019, this yields a total estimate of industry-funded trials of approximately $1.1 billion.




There is a possibility of sample bias in the MTPConnect industry survey. For example, it is possible that survey respondents are industry sponsors with similar features. As previously mentioned, this is particularly true when comparing the number of drug sponsors and device sponsors, the latter of which are under-represented by this survey. This risk is mitigated by estimating spend at an aggregate level and then comparing spend by phase and therapy area to stress test the result. Furthermore, as discussed in *Appendix 2 – Methodology for analysing clinical trials activity,* the estimate may be impacted by a possible bias between the distribution of clinical trials started and the total number ongoing.

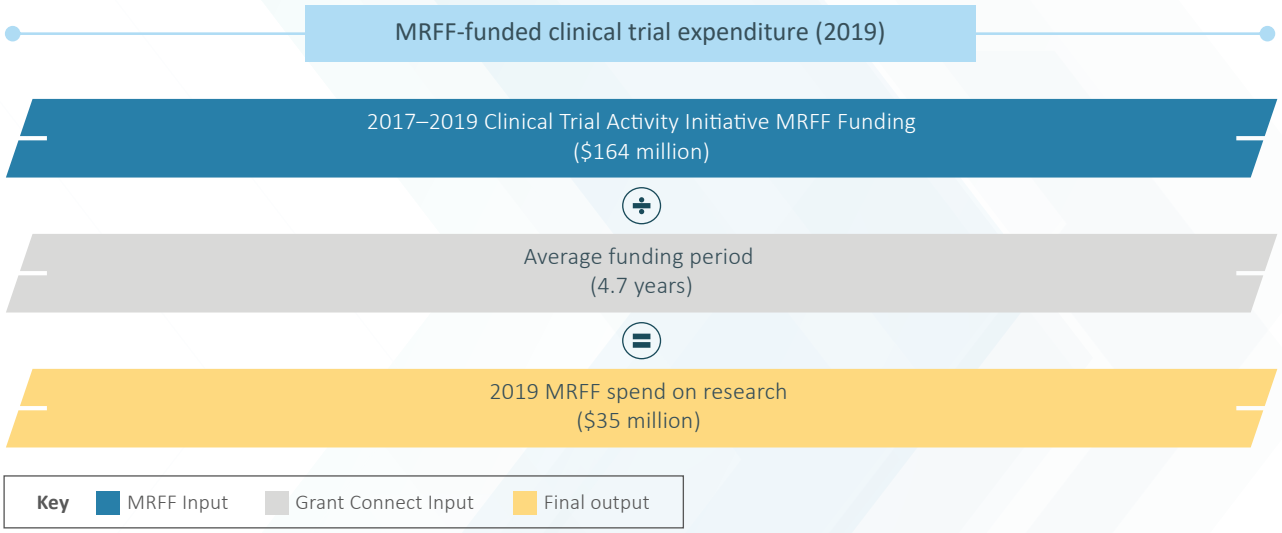
### *B) NHMRC-funded expenditure*

NHMRC-expenditure on clinical trials was sourced directly from the NHMRC. The NHMRC funds clinical trials directly through project grants and indirectly through other grant schemes, including fellowships, scholarships and infrastructure grants. NHMRC funds are typically awarded to universities, MRIs or investigators that utilise clinical trial networks; they are not awarded to commercial companies.

### *C) MRFF Clinical Trial expenditure*

MRFF data has been extracted for projects funded under the Clinical Trial Activity initiative. This initiative helps Australian researchers test new treatments through national and international clinical trials. To support this initiative, the MRFF will invest $614.2 million over 10 years to increase Australia's clinical trial activity. This initiative represents the minimum value of MRFF funding of clinical trials. It does not capture all MRFF funding of clinical trial expenditure, as there are also other MRFF initiatives that fund trials as part of broader medical research and commercialisation efforts. In other MRFF initiatives, the clinical trial portion of these initiatives cannot be easily apportioned. The balance of MRFF funding of clinical trials in other initiatives is in part captured within the calculation below titled D) *Other sources.*

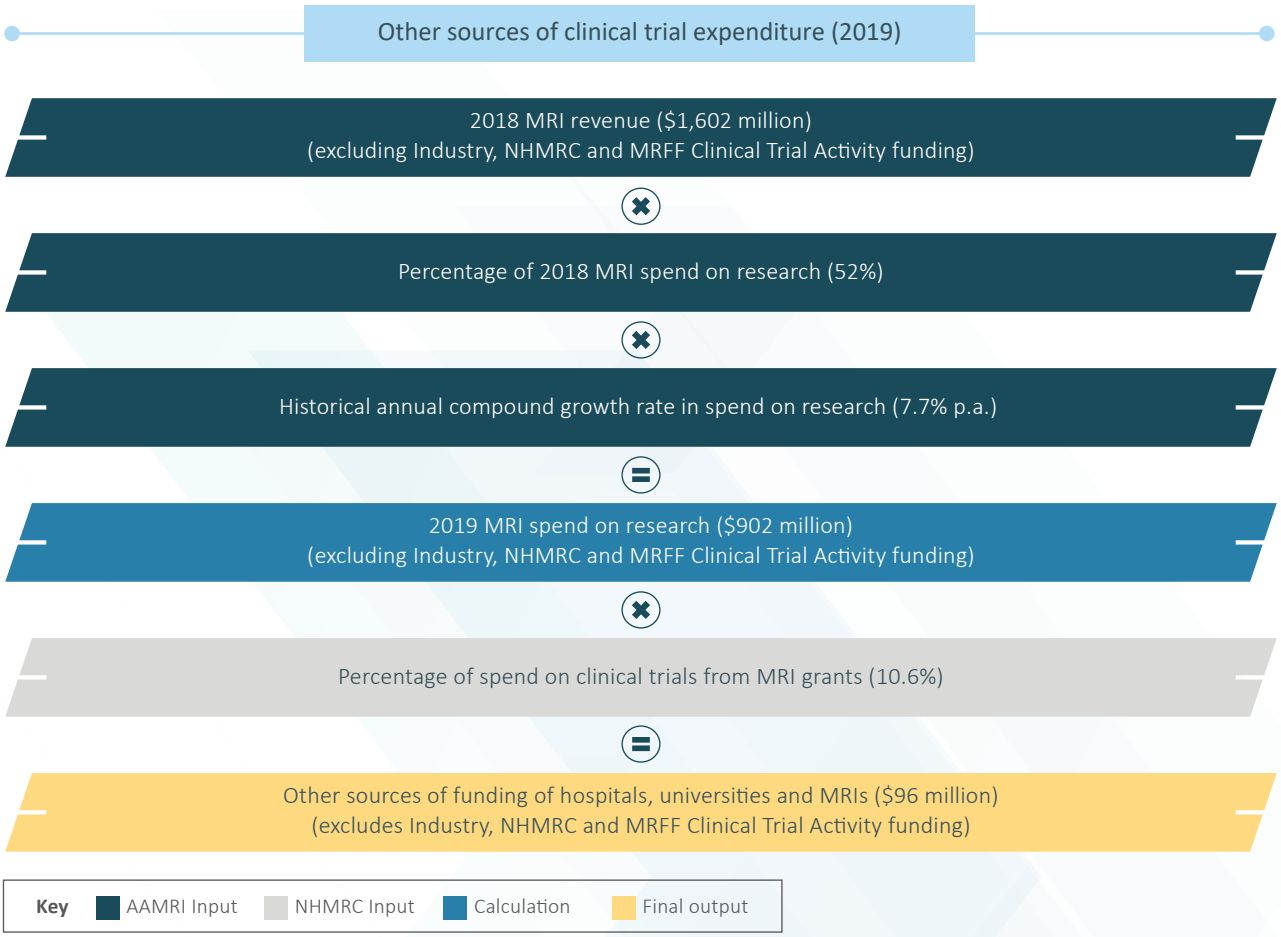
Grant funding is typically committed for several years in one lump sum. To capture the annual impact on expenditure, funding is assumed to be expensed in a linear manner, in line with the total funding period of each grant. The average grant funding period to date is 4.7 years, weighted by spend.




### *D) Other sources*

Neither the AAMRI, nor the individual MRIs, track or publish clinical trial expenditure. MRI expenditure on clinical trials is calculated using a proxy indicator for the average percentage of MRI expenditure specifically for clinical trials (i.e. excluding revenue from industry, NHMRC or MRFF that is captured elsewhere in A, B or C above). The proxy used is the proportion of NHMRC funding provided to MRIs for the purpose of clinical trials. This proxy has been tested and validated by senior industry stakeholders and is broadly unchanged since 2017.

Total revenue and expenditure figures were available in AAMRI's 2020 Member Report. This is the latest report available and the figures in this report are available for 2018. AAMRI is the industry association representing MRIs in Australia. It includes data that represents the vast majority of MRIs (data was garnered from 49 of 54 members).




## Clinical trial employment

Employment in the clinical trial sector has two separate components:

- research and management jobs at MTP companies and comparable roles in MRIs and universities – estimated to be 5,400 persons (1,300 in academia, 4,100 outside academia)
- clinical staff in hospitals, clinics or other trial sites – estimated at 2,600 persons.

Research and management employment estimates have been sourced from survey data. MTPConnect industry survey respondents have indicated they have 1,027 full-time employees. By scaling these numbers up to the whole industry, as shown in the figure below, it has been estimated that there are a total of 4,100 employees in research and management (excluding academia). This estimation is based upon a scale-up of clinical trials started, rather than ongoing, which exposes the calculation to possible sample bias.

The number of clinical staff supported by trials is difficult to estimate due to the part-time nature of many clinical trial roles and incomplete employment data for Australia's healthcare system. The number of full-time employed clinical staff supporting trials was estimated using inputs from clinical trial units at a number of hospitals and LHN/LHDs to be approximately 2,600.


Clinical trial employment (2019)
In-house trials (Sponsor)
Outsourced trials (CROs)
1
People employed full-time by Sponsors for clinical trials (611)
2
People employed full-time by CROs for clinical trials (416)
Number of full-time employees involved in trials from industry survey respondents (1,027 employees)
Number of clinical trials started from industry-sponsor survey respondents (267 trials started)
Number of clinical trials started in Australia (1,063 trials started) (excluding Universities, Government and Hospital sponsors)
Research and Management staff employed outside academia (Approximately 4,100 employees at 3.8 employees per trial started)
People employed full-time by hospitals for clinical trials (120)
Employees per hospital trial (1.4)
Employees per industry trial (3.8)
Number of clinical trials started from hospitals consulted (86 trials started)
Average employees per trial (Approximately 2.6 per trial started)
Number of trials started in Government and Hospital (306 trials started)
Number of trials started in Universities (508 trials started)
Clinical staff employed by Hospitals (Approximately 2,600 employees at 1.4 employees per trial started)
Research and management staff employed by Universities (Approximately 1,300 employees)
Total staff employed in clinical trials in Australia (Approximately 8,000 employees)
Key
Survey/Interview input
ANZCTR input
Calculation
Interim output
Final output

The number of academic staff has not been directly obtained through primary research. It is estimated there are 1,300 clinical trial research and management staff employed by universities. This has been calculated on a per trial basis on the assumption that the number of academic staff involved per university sponsored trial is the average of the number of staff employed to support:

- government and hospital sponsored trials
- the rest of the sector (excluding universities, government and hospital).

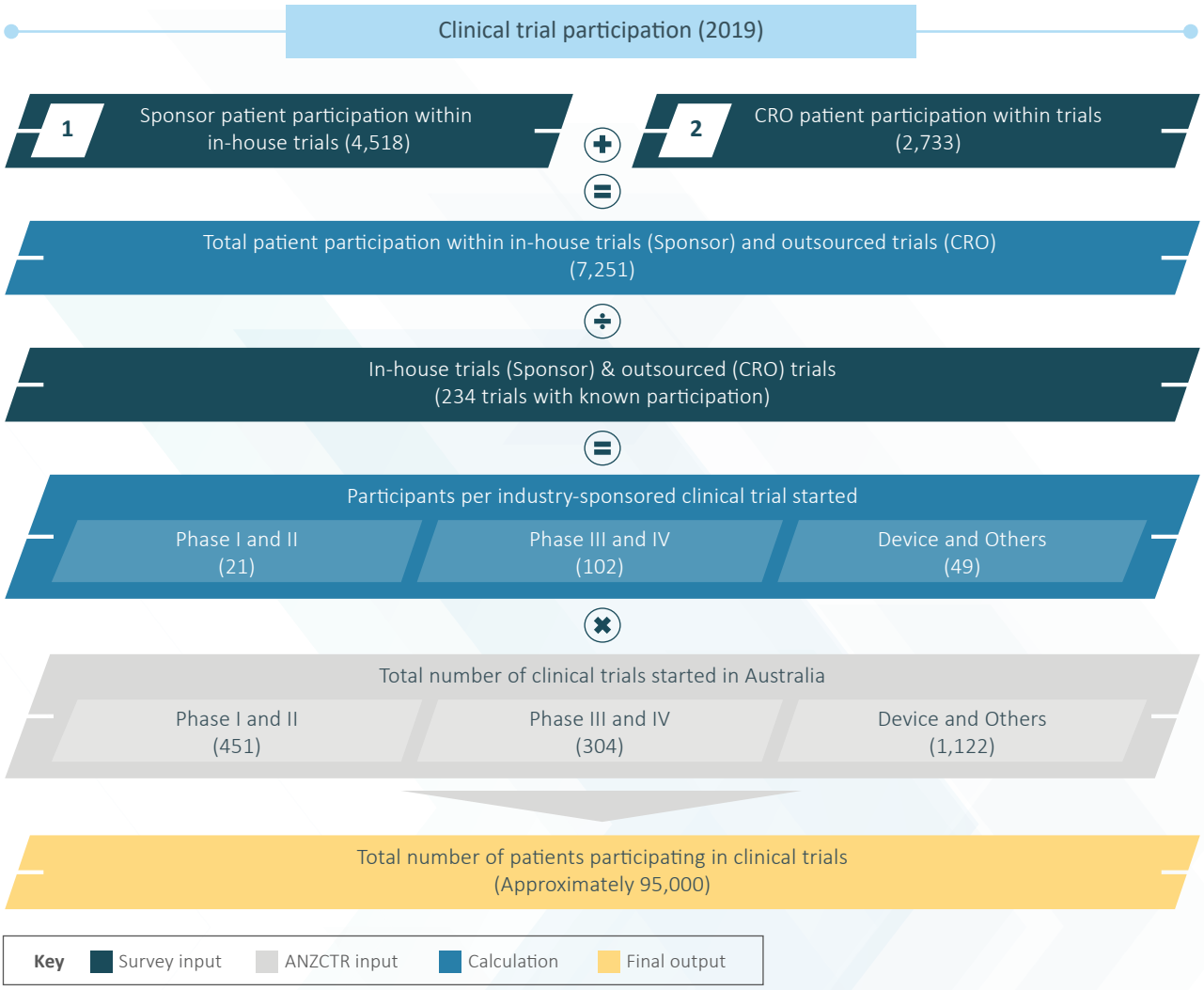
In total, our estimates suggest there are at least 8,000 employees in the clinical trial sector. This includes 2,600 in hospitals, 1,300 in universities and 4,100 in other research and management positions across the sector. There could very well be many more people employed in the sector however, as there are likely to be many people employed either as independent/solo contractors or within roles that partially support clinical trials in an indirect capacity (e.g. clinical staff at imaging facilities).

### Clinical trial participation

Patient participation within industry-sponsored clinical trials was calculated based on primary research data collected in a survey of MTP companies. In the absence of a survey of non-industry participants, clinical trials conducted by hospitals, universities, MRIs and within clinical trial networks are assumed to have similar patient participation rates per trial as each phase of industry-sponsored trials.

Survey respondents indicated their clinical trials supported 7,251 patients. The total number of clinical trial participants was estimated by calculating the average number of participants per phase and multiplying by the number of clinical trials started in each phase in 2019. In addition, the total number of clinical trial participants was also estimated by using a similar methodology by therapy area. Triangulating the two methodologies resulted in an estimate of 95,000 patients participating in clinical trials in 2019. The estimation of patient participation faces the same data and survey limitations as the estimation of industry trials expenditure (see above).

As discussed in *Appendix 2 – Methodology for analysing clinical trials activity*, clinical trial participation is available on trial registries, but this is not broken down by country. ANZCTR has previously reported roughly 500,000 clinical trial participants in Australian clinical trials. This number represents the global participation in clinical trials that have at least one site in Australia.[124] By contrast, 95,000 represents the number of clinical trial participants in Australia.


Clinical trial participation (2019)
1
Sponsor patient participation within in-house trials (4,518)
2
CRO patient participation within trials (2,733)
Total patient participation within in-house trials (Sponsor) and outsourced trials (CRO) (7,251)
In-house trials (Sponsor) & outsourced (CRO) trials (234 trials with known participation)
Participants per industry-sponsored clinical trial started
Phase I and II (21)
Phase III and IV (102)
Device and Others (49)
Total number of clinical trials started in Australia
Phase I and II (451)
Phase III and IV (304)
Device and Others (1,122)
Total number of patients participating in clinical trials (Approximately 95,000)
Key
Survey input
ANZCTR input
Calculation
Final output

## Appendix 4: Current list of state-based initiatives to improve clinical trials landscape

This appendix provides a list of state-based initiatives that are addressing reforms or generally aiming to improve the conduct and competitiveness of clinical trials in Australia. The initiatives reported below are based on the details that were provided by federal, state and territory bodies. These state-based initiatives tend to impact across the clinical trials landscape (i.e. typically therapy area agnostic) and other initiatives exist beyond what is reported below.

### Review of ethics and research governance processes and technology

Governments across each of Australia's states have had a strong focus on optimising the ethics and governance approval timelines as a key enabler to improve the clinical trials landscape.

| Initiative title | Responsible | Description | Status |
|---|---|---|---|
| **National Mutual Acceptance** | All states | All multi-centre research across Australia now reviewed under National Mutual Acceptance. | Established |
| **Establish ethics and governance IT platform** | New South Wales and South Australia | Implementation of the REGIS system through e-Health and NSW Office for Health and Medical Research, has standardised ethics and governance processes. REGIS transparently records activity, coordinates workflow between sites, provides performance reporting for sites and NSW Health and compiles regulatory reports to both NHMRC and Privacy Commissioner.<br><br>South Australia has also implemented a governance and ethics management system (Research GEMS), leveraging the New South Wales system of REGIS, funded by SA Health. This is part of the 'Encouraging More Clinical Trials' project. | Established |
| | Victoria and Queensland | Victoria has been working towards improved ethics, research governance and data reporting with the 2018 release of the Ethical Review Manager (ERM) system. The system is used by 21 hospitals and supports clinical trials conducted in Victorian public hospitals.<br><br>Queensland Health has also implemented ERM to streamline the ethics, research governance and reporting process. ERM holds information for the entire project lifecycle, including all documents, standard reports and progress reports alerts. | Ongoing |

| Initiative title | Responsible | Description | Status |
|---|---|---|---|
| **Establish ethics and governance IT platform** | Tasmania | Implement research governance ICT system to:<br>• capture information, data and ensure compliance of research projects funded through the public health system<br>• improve Tasmania's ability to externally report on research being conducted within the public health system.<br>Tasmania is likely to partner with a larger jurisdiction to implement their ICT system and use predeveloped forms and collect pertinent data. | To commence |
| | Western Australia | A reporting module for clinical trials is being built into RGS, with standard forms and processes to allow for ethics/governance review in parallel. | Ongoing |
| **Single ethical review model of HREC review** | New South Wales | The state-wide early-phase clinical trial HREC scheme started in April 2019, following the appointment of Bellberry HREC (for adults) and Sydney Children's Hospitals Network (SCHN) HREC (for paediatrics) in November 2018. The implementation of early-phase clinical trial framework allows for the fast and safe review of early-phase clinical trial applications in New South Wales. | Complete |
| | South Australia | Changes to the SA Health ethics policy has allowed the recognition of appropriately certified private ethics committees (Bellberry) within the state-wide single ethical review agreement. | Ongoing |
| | New South Wales | Expanded acceptance of external HREC approval. The NSW Research Ethics and Governance Unit is assessing the expansion of a single ethical review of multi-centre research in New South Wales public healthcare organisations, to include university and private sector HREC review. | Ongoing |

| Initiative title | Responsible | Description | Status |
|---|---|---|---|
| **Pilot and implementation of the CTGF** | All states | The Australian Commission on Safety and Quality in Health Care has been engaged by the Australian Government's Department of Health on behalf of the states and territories to deliver the CTGF. In collaboration with clinicaltrialsNSW, the working group facilitates engagement and collaboration with New South Wales local health districts on the implementation of the governance framework. | Ongoing |
| **Early phase clinical trials accreditation** | Victoria | Victoria accredited seven reviewing ethics committees to review multicentre research from 2009 and has reviewed the accreditation in 2012. In 2019 the accreditation was further expanded for multicentre early phase clinical trials with a focus on expert scientific review. An early phase clinical trial toolkit and expert scientific review proformas were developed with advice from TGA for ethics committees to utilise. | Established |
| **Review of Research Governance (Birch Review)** | South Australia | Report commissioned by the Department for Health and Wellbeing (SA) in 2018. Seventeen recommendations were identified.<br>In late 2019, a high-level steering committee reporting to the Minister for Health was convened to drive the implementation of the 17 recommendations. The project was coordinated by Health Translation SA (HTSA) and implemented by health services across the state. | Report complete<br>Implementation ongoing |
| **Improve partnerships and collaboration with intrastate-based research entities** | Tasmania | Tasmania's research governance unit now has formal connections with the Tasmanian Collaboration for Health Improvement, Menzies Institute for Medical Research, the University of Tasmania's School of Medicine, two local research funding bodies and the state Data Linkage Unit. Tasmania also participates in the NHMRC Targeted Call for Research working group. Tasmania is also in the process of working through how it can participate in the Australian Cardiovascular Alliance (ACvA). | Ongoing |

| Initiative title | Responsible | Description | Status |
|---|---|---|---|
| **Coordination of governance** | Tasmania | Launched a state-wide research strategy and a research governance framework for publicly funded health services and a suite of procedures in 2020, supported by a research governance officer for Southern and North/ North-Western regions of the LHN and research governance project coordinator. | Established |
| | New South Wales | Central coordination of governance tasks through REGIS. This includes single forms for SSA and post-approval reporting. Insurance certificates of currency to receive single review with acceptance across sector. | Ongoing |
| | Victoria and Queensland | Both ethics and governance of research have been integrated and linked in ERM. Standard reports and SSA/research governance reports, specifically for research sites, inform the site governance office. This includes amendment information at ethics informing a site amendment notification. Alerts have been included in ERM to notify a site when reports are due. | Established |

## Supporting infrastructure and capability for clinical trials

The following state-based initiatives are closely interlinked with the ultimate aim of driving reform in the clinical trials policy and process environment to bring more clinical trials to Australia. These initiatives have a particular focus on increasing patient access by enabling RRR communities to have better access to clinical trials.

| Initiative title | Responsible | Description | Status |
|---|---|---|---|
| **Australian tele-trial pilot and program** | Queensland-led, as well as Victoria | Queensland Health has supported the development of the tele-trial standard operating procedures, which have been adopted nationally and are now published on the Australian Department of Health website. Queensland Health is driving the establishment of a nationally accepted template for the tele-trial sub-contract. | Established |
| **Rural, Regional and Remote Clinical Trials Enabling Program** | Queensland, Victoria, Tasmania, South Australia, Western Australia, Northern Territory | Queensland Health has been awarded funding of $75.2 million over five years from the Medical Research Future Fund – 2019 Rural, Regional and Remote Clinical Trials Enabling Infrastructure grant scheme. The funding will support the establishment of the Australian Teletrial Program – bringing clinical trials closer to home for rural, regional and remote communities in Queensland, Victoria, Tasmania, South Australia, Western Australia and the Northern Territory. | Emerging |
| | New South Wales and Australian Capital Territory | NSW Health has been awarded funding of $30.6 million over five years from the Medical Research Future Fund – 2019 Rural, Regional and Remote Clinical Trials Enabling Infrastructure grant scheme. The program includes 34 state and national partners across health, research, private and community sectors to deliver increased and more equitable access to clinical trials for researchers and patients in RRR New South Wales. | Ongoing |

| Initiative title | Responsible | Description | Status |
|---|---|---|---|
| **Improved patient access in RRR communities** | Victoria | Victoria has provided funding to rural and regional hospitals to address administration activities associated with clinical trials and enable hospitals to be 'trial ready' organisation-wide. This initiative has opened up more trial sites and has provided access to clinical trials for patients in rural/regional locations.<br><br>A two-year pilot of the Clinical Trial Research Support Service (CTRSS) model will be evaluated in Q3 and 4 2021. This will inform planned expansion to more rural regional organisations. | Ongoing |
| **Clinical trial management system** | South Australia | All academic institutions (including universities and the South Australian Health and Medical Research Institute) are investing in infrastructure to improve clinical trials management, often in collaboration with local health services. | Ongoing |
| | New South Wales | NSW Health is implementing an international best practice standard clinical trial management system (CTMS) in all public hospitals. A CTMS will provide the foundation for portfolio, financial and resource management of clinical trials at site, LHD and Ministry of Health levels which will underpin performance metrics for clinical trials in NSW. | Ongoing |
| **Development of clinical trial coordination/ concierge units and portals** | Queensland | Queensland Clinical Trials Coordination Unit's (QCTCU) expert team provides a free concierge service to sponsors and researchers (client) by connecting them to clinical trial sites (sites), including thought leaders and key contacts within Queensland hospitals, as well as to service providers to increase clinical trials to Queensland. | Established |
| | New South Wales | clinicaltrialsNSW has been developed as the front door for sponsors, researchers and consumers in NSW. Services provided include Clinical Trial Triage, Connect and Toolkit. | Ongoing |
| | Victoria | Development of the Victorian Clinical Trials Gateway which provides single point of entry for international companies to connect with Victorian clinical trial sites, contract research organisations and professional services | Ongoing |

| Initiative title | Responsible | Description | Status |
|---|---|---|---|
| **Development of clinical trial coordination/ concierge units and portals** | Western Australia | Western Australia has appointed clinical trial liaison officers in the public health system. | Established |
| | Queensland | Development of a comprehensive online data base connecting sponsors to over 100 sites and service providers. This enables quick feasibility evaluations and comprehensive product development. | Established |
| | South Australia | Health Translation SA (HTSA), working with Department for Trade and Investment (DTI) Ministerial Advisory Panel Clinical Trials Cluster, is describing all facets of the South Australian clinical trial ecosystem in preparation for the development of a promotional portal/interface. The existing portal has been developed by SA Health as part of the 'Encouraging More Clinical Trials' project. | Ongoing |
| | Victoria | A new look website is being developed in the Department of Jobs, Precincts and Regions (DJPR) for Clinical Trials and Research, expected August 2021. Regular communication online in the Streamline E-bulletin keeps the sector informed. | Established |
| | Tasmania | Created a research governance website to house information for external and internal researchers to access in one centralised place. | Ongoing |
| **South Eastern Border States (SEBS) contract alignment** | New South Wales, Victoria, Queensland, South Australia and Tasmania | Standardisation of Clinical Trial Research Agreements 'special conditions' (Schedule 7 and 4) for a trial contract. Completed in conjunction with MA and MTAA. | Established |
| | Tasmania | Standardisation of Tasmania's contracts with those of the SEBS approved by MTAA and MA. To facilitate this, Tasmania now participates in SEBS meetings, reviews requests and presents options to SEBS. | Established |

| Initiative title | Responsible | Description | Status |
|---|---|---|---|
| **The Queensland Clinical Trials Consortium** | Queensland | The Queensland Clinical Trials Consortium brings together businesses to cross-promote each other and work closely to develop strategies to bring more trials to Queensland. The consortium has more than 70 national and international companies that have joined to form a one-stop-shop for product development, clinical research and regulatory programs. The consortium brings together multidisciplinary experts, providing everything needed to design, run and include trial data in regulatory applications. | Established |
| **Cost management** | New South Wales | NSW Health is standardising clinical trial costs in the public health system to provide budget consistency for LHDs and Sponsors. | Ongoing |
| **Early-phase clinical trial Quality Recognition Scheme (QRS)** | New South Wales | The QRS facilitates NSW Health assessment and endorsement of public hospital sites conducting early phase clinical trials to best practice standard. | Pilot complete<br>Ongoing |
| **COVID-19 research initiatives** | All states | All states have been actively involved in funding COVID-19 research and development. | Established |

## Enhance transparency of the state of the sector

One of the key priorities for the future articulated within the *Clinical Trials in Australia* (2017) report was to enhance the transparency of the state of the sector and this remains a priority going forward. The following state government initiatives have supported development in this area since 2017.

| Initiative title | Responsible | Description | Status |
|---|---|---|---|
| **All states now contribute to the NAS** | All states | Following the participation of Tasmania and Western Australia, NAS is now more complete than ever and is available for use for benchmarking and continuous improvement.<br>NAS metrics utilise the NMA framework to measure Australia's success relating to overall study start-up timelines, ethics and SSA assessment timelines and site/trial recruitment targets. | Ongoing |
| **Performance metrics and benchmarks** | New South Wales | NSW Research Ethics and Governance Unit has established performance and monitoring measures, defined standards, and facilitated the agreement of these with Chief Executives. Quarterly performance meetings with local health districts to address ethics and governance turnaround times against agreed benchmarks. | Established (measures)<br>Ongoing (monitoring and reporting |
| | Victoria | A 30-working day benchmark has been introduced for single ethics review of multicentre clinical trials and performance reporting to organisation has occurred since 2010. Improvement has occurred in timelines, following reporting to CEO, research executive and offices. | Ongoing |
| **Clinical trial data management** | Western Australia | Western Australia has provided $1.3 million in funding to establish a Clinical Trials and Data Management Centre. | Complete |
| **South Australian Productivity Commission inquiry into Health and Medical Research** | South Australia | A system-wide inquiry was conducted in 2020. Response to the recommendations is pending. | Complete |

### Improvements in workforce capacity and capability

Key workforce capability and capacity gaps have emerged across the clinical trials sector. The following initiatives have commenced to address these gaps.

| Initiative title | Responsible | Description | Status |
|---|---|---|---|
| **Clinical trial workforce training** | Western Australia | Western Australia has provided ongoing funding for the development of research education and online training modules, including GCP. | Established |
| | New South Wales | clinicaltrialsNSW provides Trancelerate-endorsed GCP and ISO-14155 training to clinical trial staff across the public health system through partnership with national education providers. In 2020 over 1,000 clinical trial staff attended GCP training. | Ongoing |
| | South Australia | Coordination of the development of a GCP SOP and training. This is part of the 'Encouraging More Clinical Trials' project. | Ongoing |
| | Tasmania | Surveyed research community and designed educational packages based on needs and wants of health researchers. | Ongoing |
| **Clinical trials workforce analysis** | New South Wales | Analysis of the clinical trials workforce within NSW Health to assess barriers and opportunities in attraction and retention of clinical trials staff, including study coordinators and research nurses. This information will be used to develop a New South Wales workforce strategy to attract and retain staff to support high-quality clinical trials delivery in New South Wales public health organisations. | Analysis complete<br>Ongoing development of strategy |
| **New South Wales Clinical Trial Community of Practice** | New South Wales | Established a Clinical Trial Community of Practice as an essential mechanism for collaborations and professionalisation of clinical trials in New South Wales. Members are responsible for the oversight of clinical trials at their respective local health districts and medical research institutes. | Ongoing |
| **Tasmania Director of Research** | Tasmania | Tasmania is auditing funding and workforce to determine where the state's Director of Research should be based and how this role works with the current project manager for the unit. | Ongoing |

## Appendix 5: References


| Author(s), Organisation | Title | Year |
|---|---|---|
| **AAMRI** | Australian Medical Research Institutes – The AAMRI Report 2020 | 2020 |
| **ABC** | New registry calls on Australians to join clinical research | 2020 |
| **ACTA** | Adaptive Multi-Arm Platform Trials: Benefits and Efficiencies website | 2021 |
| **ACTA** | Clinical trial awareness and access amongst culturally and linguistically diverse (CALD) populations: environmental scan | 2020 |
| **ACTA** | Funding and Support | 2021 |
| **ACTA and CT:IQ** | Consumer Involvement & Engagement Toolkit website | 2021 |
| **ACTA and Quantium Health Outcomes** | Economic evaluation of investigator-initiated clinical trials conducted by networks | 2017 |
| **Adaptive Health Intelligence** | Adaptive Health Intelligence website | 2021 |
| **AIHW** | Health Expenditure Australia 2017–2018 | 2019 |
| **AIHW** | Burden of Disease | 2020 |
| **AIHW** | Causes of Death | 2018 |
| **Alan Cass** | Challenges and Success in Clinical Research with Aboriginal and Torres Strait Islander Australians | 2018 |
| **Alfred Health** | Expanding access to cancer clinical trials | 2021 |
| **Alfred Health** | Lifesaving trial moves to Tasmania | 2020 |
| **Almac Group** | Impact of COVID-19 on the Pharma industry and associated shifts in their outsourcing requirements | 2020 |
| **ANZCTR** | The clinical trials landscape in Australia 2006–2015 | 2017 |
| **AusBiotech** | Budget delivers significant news for R&D | 2020 |
| **Australian Bureau of Statistics** | Regional Population Growth | 2020 |
| **Australian Commission on Safety and Quality in Health Care** | The National Clinical Trials Governance Framework (draft) | 2021 |
| **Australian Government** | Make it happen: the Australian Government's Modern Manufacturing Strategy | 2020 |
| **Australian Government** | Medical Product National Manufacturing Priority road map | 2021 |
| **Bayer** | Healthy China 2030: China's healthcare journey | 2019 |
| **Ben Faircloth and Andre Valente** | COVID-19 and Clinical Trials: Accelerating the adoption of eClinical Technology | 2020 |

| Author(s), Organisation | Title | Year |
|---|---|---|
| **Bethany Percha, et al.** | Designing Robust N-of-1 Studies for Precision Medicine: Simulation Study and Design Recommendations, Journal of Medical Internet Research | 2018 |
| **Carsten Sommer, et al.** | Building clinical trials around patients: Evaluation and comparison of decentralized and conventional site models in patients with low back pain | 2018 |
| **Clinical Leader** | Should You Look at China for Your Next Clinical Trial | 2018 |
| **Clinical Trials Project Reference Group** | Clinical Trials in Australian Public Health Institutions 2018-19 (NAS 4 report) | 2021 |
| **ClinTrial Refer** | ClinTrial Refer website | 2020 |
| **Department of Foreign Affairs and Trade** | Trade and Investment at a Glance 2020 | 2020 |
| **Department of Health** | Australian Medical Research and Innovation Priorities | 2020 |
| **Department of Health** | BioMedTech Horizons initiative | 2019 |
| **Department of Health** | Biomedical Translation Bridge initiative | 2019 |
| **Department of Health** | Budget announces new investments in health and medical research | 2018 |
| **Department of Health** | Budget 2020-21, Guaranteeing Medicare and Access to Medicines – Rural, Regional and Remote Clinical Trial Enabling Infrastructure Program | 2020 |
| **Department of Health** | Clinical Trials Activity Initiative website | 2021 |
| **Department of Health** | COVID-19: Guidance on clinical trials for institutions, HRECs, researchers and sponsors | 2020 |
| **Department of Health** | Medical Research Future Fund Grant Recipients | 2020 |
| **Department of Health** | Medical Research Future Fund website | 2021 |
| **Department of Health** | National Critical Research Infrastructure Initiative website | 2021 |
| **Department of Health** | Research Missions website | 2020 |
| **Department of Health** | Targeted Translation Research Accelerator initiative | 2019 |
| **Department of Health** | Therapeutic Goods Administration, Clinical Trials website | 2020 |
| **Department of Industry, Innovation and Science** | Australian Clinical Trials website | 2021 |
| **Department of Industry, Innovation and Science** | Upcoming changes to the R&D Tax Incentive: Overview factsheet | 2020 |
| **Evaluate Vantage** | The pandemic releases its grip on clinical trials | 2021 |
| **FDA** | Master Protocols: Efficient Clinical Trial Design Strategies to Expedite Development of Oncology Drugs and Biologics Guidance for Industry | 2018 |

| Author(s), Organisation | Title | Year |
|---|---|---|
| **Florey Institute of Neuroscience and Mental Health** | Major new Alzhiemer's disease initiative announced | 2018 |
| **Genomic Cancer Clinical Trials Initiative** | February 2020 Workshop Report | 2020 |
| **Genomics England** | Landmark strategy launched to cement UK's position as global leader in genomics | 2021 |
| **Healthcare Professionals Group** | Tele-trials- Increasing equity for Australian patients accessing clinical trials | 2020 |
| **Janelle Bowden** | Consumers' role in clinical trials research. Where to from here? | 2018 |
| **Janelle Bowden and Lisa Briggs** | Searching for Clinical Trials: What Patients Want | 2018 |
| **Jennifer Rogers** | The opportunities and challenges of basket studies | 2019 |
| **Joan Cunningham, Indigenous Health** | Are there systematic barriers to participation in cancer treatment trials by Aboriginal and Torres Strait Islander cancer patients in Australia | 2020 |
| **Kylie Hunter, et al.** | The landscape of clinical trial activity focusing on indigenous health in Australia from 2008 to 2018 | 2019 |
| **Marliese Alexander, et al.** | Evaluation of an artificial intelligence clinical trial matching system in Australian lung cancer patients. Jamia Open | 2020 |
| **MA and PwC** | The economic contribution of the innovative pharmaceutical industry to Australia: Economic footprint of the innovative pharmaceutical industry | 2018 |
| **Morro Touray** | Estimation of Quality- adjusted Life Years alongside clinical trials: the impact of 'time-effects on trial results | 2018 |
| **MTPConnect** | COVID-19 Impact Report | 2020 |
| **MTPConnect** | COVID-19 Impact Report 2nd Edition | 2020 |
| **MTPConnect** | Sector Competitiveness Plan 2020 | 2020 |
| **MTPConnect** | REDI Initiative Skills Gap Analysis Second Report | 2021 |
| **National Institute for Health Research** | Annual Report 2018/19 | 2020 |
| **NHMRC** | HREC Committees registered with the NGMRC website | 2021 |
| **NHMRC** | Keeping Research on Track II | 2018 |
| **NHMRC** | National Statement on Ethical Conduct in Human Research | 2018 |
| **Novotech** | 5 Key changes accelerate clinical trial and drug approval timelines | 2017 |

| Author(s), Organisation | Title | Year |
|---|---|---|
| NSW Health | Rural, Regional & Remote Clinical Trial Program website | 2021 |
| NSW Health | National Mutual Acceptance webpage | 2021 |
| OECD | Public health genomics in Korea, OECD Reviews of Public Health: Korea: A Healthier Tomorrow | 2020 |
| Omer Inan, et al. | Digitising clinical trials | 2020 |
| Opyl Technologies | Opyl Technologies website | 2020 |
| Parexel and Economist Intelligence Unit | The Innovation Imperative: Future of Drug Development | 2019 |
| Parliament of Australia | Treasury Laws Amendment (A Tax Plan for the COVID-19 Economic Recovery) Bill | 2020 |
| Patrick Hughes | Over 300 Clinical Studies Benefit from CluePoints' COVID-19 Risk Management Support | 2020 |
| Personalized Medicines Coalition (PMC) | Personalized Medicine at FDA – A Progress & Outlook Report | 2019 |
| PhRMA and TEConomy Partners, LL | The Economic Impact of The U.S. Biopharmaceutical Industry: 2017 National and State Estimates | 2019 |
| Queensland Health | Queensland Health Teletrials Pilot Analysis Report | 2019 |
| Sumit Majumdar | Better outcomes for patients treated at hospitals that participate in clinical trials | 2008 |
| The Hon. Greg Hunt MP | Official Opening of the 2020 ACTA Summit | 2020 |
| The Hon. Greg Hunt MP | Universal Telehealth extended through 2021 | 2021 |
| The Lancet | Fewer than half of US clinical trials have complied with the law on reporting results, despite new regulations | 2020 |
| Tony Tse et al. | How to avoid common problems when using ClinicalTrials.gov in research: 10 issues to consider | 2018 |
| Translational Research Institute | Aussie invention propelling needle-free vaccine delivery | 2020 |
| US National Institutes of Health | FDAAA 801 and the Final Rule | 2021 |
| US National Institutes of Health | Why Should I Register and Submit Results? | 2020 |
| Victorian Comprehensive Cancer Centre | Adopting the teletrial model for safe trial delivery | 2020 |
| Victorian Department of Health & Human Services | National Mutual Acceptance webpage | 2021 |
| Victorian Government | VicTrials website | 2021 |
| World Health Organisation | WHO Coronavirus (COVID-19) Dashboard | 2020 |

## Appendix 6: Glossary of terms

| Acronym | Definition | Acronym | Definition |
|---|---|---|---|
| AAMRI | Australian Medical Research Institutes | EMR | Electronic Medical Records |
| ACT | Australian Capital Territory | ERM | Ethics Review Manager |
| ACTA | Australian Clinical Trial Alliance | FDA | Food & Drug Administration |
| ADNeT | Australian Dementia Network | FTIH | First Time In Human |
| AGCMC | Australia Genomic Medicine Centre | GCP | Good Clinical Practice |
| AI | Artificial Intelligence | HREC | Human Research Ethics Committee |
| AIHW | Australian Institute of Health and Welfare | HTSA | Health Translation South Australia |
| ALTG | Australia Lung Cancer Trials Group | IIT | Investigator Initiated Trial |
| ANZCTR | Australia and New Zealand Clinical Trials Registry | IND | Investigative New Drug |
| ASCOT | Australasian COVID-19 Trial | KOL | Key Opinion Leader |
| CGP | Comprehensive Genomic Profiling | KP | Knowledge Priority |
| CRA | Clinical Research Associates | KPI | Key Performance Indicator |
| CRO | Contract Research Organisations | LHD | Local Health District |
| CT:IQ | Clinical Trials: Impact & Quality | LHN | Local Health Network |
| CTA | Clinical Trial Approval | MA | Medicines Australia |
| CTC | Clinical Trial Coordinator | MMS | Modern Manufacturing Strategy |
| CTGF | Clinical Trials Governance Framework | MoST | Molecular Screening and Therapeutics |
| CTMS | Clinical Trial Management System | MRFF | Medical Research Future Fund |
| CTN | Clinical Trial Notification | MRI | Medical Research Institutes |
| CTPRG | Clinical Trials Project Reference Group | MTAA | Medical Technology Association of Australia |
| CTX | Clinical Trial Exemption | MTP | Medical technology, biotechnology and pharmaceutical |
| DALY | Disability-adjusted Life Years | NAS | National Aggregate Statistics |
| DTI | Department for Trade and Investment | NHMRC | National Health and Medical Research Council |
| EIU | Economist Intelligence Unit | NIH | National Institutes of Health |
| EMA | European Medicines Agency | NMA | National Mutual Acceptance |

| Acronym | Definition | Acronym | Definition |
|---|---|---|---|
| NSW | New South Wales | RGS | Research Governance Service |
| NT | Northern Territory | RGO | Research Governance Office |
| PBS | Pharmaceutical Benefits Scheme | RRR | Rural, regional and remote |
| PRO | Patient Reported Outcomes | SA | South Australia |
| QALY | Quality-adjusted Life Years | SCHN | Sydney Children's Hospitals Network |
| QCTCU | Queensland Clinical Trial Coordination Unit | SEBS | SouthEastern Border States |
| QLD | Queensland | SSA | Site-Specific Assessment |
| QRS | Quality Recognition Scheme | TAS | Tasmania |
| R&DTI | Research and Development Tax Incentives | TGA | Therapeutic Goods Administration |
| RDTF | R&D Taskforce | VIC | Victoria |
| REGIS | Research Ethics and Governance Information System | WA | Western Australia |
| REMAP-CAP | Randomised, Embedded, Multi-factorial, Adaptive Platform Trial for Community-Acquired Pneumonia | | |

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