April 2025

Medicines Australia Submission

Strategic Examination of R&D

Department of Industry, Science and Resources

Medicines Australia

Introduction

The number of submissions to the PBAC, and the number of new and expanded listings on the PBS, have grown steadily each year over the past 10 years. The emergence of personalised medicines, such as gene and gene-modified cell therapies, means that the number and complexity of applications needing HTA assessment will continue to grow. For example, Australia's HTA capability and processes must prepare for, and be able to respond to, the volume of gene-modified cell therapies in the pipeline. The following numbers indicate the scale of the HTA tasks ahead. As of January 2024, 7 cell and ribonucleic acid (RNA) therapies were registered in Australia; 46 were registered in other comparable jurisdictions; and 3,951 were in clinical development.

Case study: Australian scientific discovery Omjjara (momelotinib)'s long journey to patients

Omjjara (momelotinib), discovered by Melbourne scientists Professor Andrew Wilks and Dr Chris Burns in 2005 and sponsored by GSK, became available on the Pharmaceutical Benefits Scheme (PBS) on 1 April 2025 to treat the rare bone marrow cancer myelofibrosis.[1]

The PBS listing marked the culmination of 20-year journey from initial discovery to finally reaching Australian patients. However, the journey to PBS listing was not straightforward, involving multiple acquisitions, unsuccessful clinical trials and millions of dollars in R&D investment.

Cytopia Research was founded by Professor Wilks and Dr Burns and listed on the ASX in 2004. After successful in-vitro studies, the company was unable to secure funding for further clinical trials for Omjjara. Cytopia Research was acquired by a Canadian company in 2009.

Had Australia's R&D ecosystem been better equipped to support the translation of discoveries into patient impact with a viable domestic market and stronger reimbursement pathways, Omjjara might have been commercialised earlier.

Omjjara's journey demonstrates the significant obstacles in translating biomedical discoveries in Australia and underscores the need for an end-to-end R&D ecosystem that prioritises innovation and values patient outcomes.

When considering investment in health and medical R&D, the Government must therefore take a holistic view of the whole ecosystem. The life science ecosystem encompasses a range of stakeholders, such as researchers, entrepreneurs, investors, industry, regulatory bodies, healthcare providers, and patients, who collaborate to bring new therapies, diagnostics, and medical technologies to market. The significant challenge – and opportunity – for the Australian life sciences ecosystem is to translate research into clinical and commercial outcomes that benefit patients and the Australian economy. In the Australian

[1] https://au.gsk.com/en-au/media/press-releases/gsk-brings-australian-research-discovery-omjjara-momelotinib-home-new-treatment-pbs-listed-for-rare-bone-marrow-cancer/

context, effective commercialisation is when an innovative medicine reaches the patient via the Pharmaceutical Benefits Scheme.[2]

As the Discussion paper notes, while Australia has a strong foundational research system, including biomedical and clinical sciences, we have a poor history of effective commercialisation because of low alignment between industry and academia which leads to poor collaboration and research translation[3]. Similar problems are seen in the start-up sector with weak connections between start-ups and universities. It is only through commercialisation of scientific advancements that their full benefits can be achieved. This is even more imperative as Australia transitions away from its resource-based economy towards a knowledge-based one.

In the World Intellectual Property Organization (WIPO)'s Global Innovation Index 2024,[4] Australia ranked as the 23rd most innovative economy in the world, however its performance in innovation outputs has deteriorated from last year. This ranking demonstrates the long-standing gap between Australia's cutting-edge research and the commercialisation of this research into treatments that tackle the burden of disease by benefitting patients and the wider economy.

Medicines Australia's submission aims to address a number of the consultation questions, particularly questions 1,2,5 and 9. Its submission focusses on 5 key areas where improvements are needed, or where barriers exist.

1. Create a viable market for biomedical innovation by reforming the Health Technology Assessment (HTA) system

Reforming Australia's HTA system is a vital aspect of growing a sustainable life sciences ecosystem because companies make investment decisions based on a country's willingness to invest in bringing innovative technologies to their populations in a timely manner. Without a reliable and predictable market access pathway in Australia, global companies are unlikely to invest in new, or upgrade existing, advanced manufacturing facilities.

The need for reform is evidenced by the patient access gap for medicines. Recent data shows patients have to wait an average 466 days before a product is reimbursed, well behind other OECD countries, and the top four OECD countries reimbursed more than 70% of new medicines in less than 6 months from registration, while only 17% of NMEs were reimbursed in Australia in less than 6 months.[5]

A key part of the Strategic Agreement between Medicines Australia and the Commonwealth[6] was the independent Health Technology Assessment (HTA) Policy and

[2] Prescription medicines accessed via the PBS constitute the vast majority of prescription medicines dispensed in Australia. See Medicines in the Health system 2022, available at https://www.aihw.gov.au/reports/medicines/medicines-in-the-health-system

[3] P.19 of the Discussion Paper highlights the fact that Australia's substantially lower experimental development levels can be attributed to our lower BERD

[4] https://www.wipo.int/web-publications/global-innovation-index-2024/en/gii-2024-results.html

[5] Medicines Matter 2022 Australia's Access to Medicines 2016-2021

[6] Strategic Agreement in relation to reimbursement, health technology assessment and other matters" Commonwealth of Australia and Medicines Australia, 2022-2027

Methods Review, the first of its kind in nearly 30 years. The Review has now reported[7] and implementation of its recommendations are critical if Australia is to remain amongst the group of countries prioritised for first wave launch of healthcare innovations. The package of reforms recommended by the Review will result in faster access to safe and effective innovative medicines for Australian patients. Such reforms must be accompanied by government policy which demonstrates a willingness to invest in new and innovative medicines, biotherapeutics and vaccines and to maintain a viable and sustainable market for healthcare innovation.

2. Apply a patient-centric approach to R&D investment

Shifting the focus from viewing academic publications as a measure of success to prioritising tangible patient outcomes is critical. This involves fostering a mindset that values and rewards real-world impact, R&D commercialisation and industry-academia collaboration and encouragement of career mobility between the sectors.

By emphasising patient outcomes, the R&D investment process becomes more aligned with the needs and experiences of patients. This means that research efforts are directed towards developing treatments and therapies that have a direct and meaningful impact on patients' lives. It also encourages collaboration between various stakeholders, including researchers, healthcare providers, and patients, to ensure that the research is relevant and beneficial.

3. Establish an Australian Life Sciences Council

The life sciences Industry is already identified as a national priority, but co-ordination is required**.** Given its strategic importance to sovereign wealth and national security, many OECD nations have already established whole-of-government national life science councils to drive innovation, investment, jobs creation and competitiveness. Australia must act now to capitalise on our strengths, lest we fall further behind our international peers. The sector would greatly benefit from a whole-of-government strategic focus, deliberate co-ordination, and an enduring forum to foster partnership between industry, government and other key stakeholders across the value chain. A Life Sciences Council will do this.

The Council could inform a whole-of-government approach to health innovation across the lifecycle – from early-stage research through to clinical trials, translation, development and commercialisation – in partnership with industry. Drawing on international best practice, it could help local innovators overcome existing challenges that make it difficult to bring new health innovations from early-stage discovery through to clinical trials, commercialisation and domestic manufacturing.

Without change to the status quo in Australia, where multiple departments and policies are intersecting with the life sciences industry, sub-optimal outcomes for the nation will continue.

[7] "Accelerating Access to the Best Medicines for Australians Now and into the Future: A review of Australia's health technology assessment policies and methods for the Australian Government", September 2024

4. Bring to fruition the National One Stop Shop for clinical trials

There is an opportunity to attract more international trials to Australia. This would enable patients to access new promising treatments years before they are widely available, contribute to the knowledge economy, and support the growth of a stronger life sciences ecosystem. These benefits were demonstrated by an Australian study which showed that for every $1 invested in clinician-driven clinical trials in Australia, benefits of $5.80 can be realised.[8]

The Government has recognised this opportunity through its commitment of $62 million to support clinical trials, and a further $18.8 million to progress the National One Stop Shop for clinical trials (NOSS) in the 2024–25 Federal Budget. The NOSS is a digital platform designed to streamline approval and reporting processes for human research across Australia.

The Government needs to allocate ongoing and sustainable funding for the NOSS for clinical trials. Ongoing investment will ensure this vital initiative achieves its full potential, ensuring Australia remains globally competitive in clinical research and development (R&D), benefiting patients, industry, and the broader economy.

Since the allocation of funding in the 2024–25 Federal Budget, significant progress has been made under the leadership of Professor Ian Chubb and the Inter-Governmental Policy Reform Group (IGPRG) for health and medical research. The various elements of the platform currently being tested, trialled, and refined mark a critical step toward realising the long-term vision of the NOSS. To sustain the current momentum, continued funding is vital to move the NOSS from concept to full implementation. Building on the progress achieved will cement Australia's position as a leader in clinical research, attract global investment, and deliver faster access to life-changing therapies. Now is the time to ensure these efforts translate into lasting impact for patients and the broader health system.

A lapse in funding would jeopardise Australia's standing as a preferred destination for clinical research and disrupt the delivery of life-saving innovations to patients. Failure to sustain this work also poses the risk of further fragmenting the clinical trials landscape. Without a national platform, jurisdictions will continue to develop disparate systems and processes that are not interoperable. This could lead to longer wait times for patients or, worse, deny them access to cutting-edge therapies

5. Incentivise the life-sciences eco-system in key areas such as intellectual property, public-private partnerships and advanced manufacturing, to drive growing business R&D investment

Successful innovation incentives are founded on creating a sustainable and growing ecosystem of domestic and international partnerships for research and development and

[8] https://www.safetyandquality.gov.au/about-us/latest-news/media-releases/clinical-trials-pay-safety-quality-and-economy

subsequent commercialisation. This also opens advanced manufacturing and export opportunities. R&D is key. Trusted partnerships are essential.

Incentivising an eco-system of partnerships for research and development through to commercialisation in the innovative pharmaceutical industry must be underpinned by recognition that Australia is part of a growing global network. A network in which Australia can play a leading global and, especially, regional role.

Key areas where Australia can better incentivise the life sciences sector are through strong and reliable Intellectual Property protections. Intellectual property (IP) rights, particularly patents, are a universally accepted mechanism for the recognition of the value of innovation. Australia is lagging behind comparable countries with regard to Regulatory Data Protection (data exclusivity), which creates a disincentive for innovative IP to be retained in Australia where data exclusivity is only 5 years. Canada, Japan and the EU have a minimum of 8 years data exclusivity while the United States has up to 12 years for biologic medicines. Australia's uncompetitive data exclusivity provisions create a barrier to growth in research, development and commercialisation of innovative biotech sector for human health in Australia.

Public-private partnerships

Public-private partnerships (PPPs) play a crucial role in advancing the life sciences sector. PPPs bring together the strengths of both sectors—government resources and private sector innovation. This collaboration can speed up the development of new drugs, therapies, and technologies. For example, the rapid development of COVID-19 vaccines was a result of such partnerships. These partnerships allow for the pooling of resources, including funding, expertise, and infrastructure. This can help overcome the high costs and risks associated with life sciences research and development. PPPs are also essential in tackling large-scale health issues that require coordinated efforts. They enable the development and distribution of treatments for diseases that might not be profitable for private companies to pursue alone

Case study: Public-private partnership expands access to clinical trials for Australians with cancer

PrOSPeCT (*Precision Oncology Screening Platform Enabling Clinical Trials)* is Australia's largest cancer genomics initiative, enabled by a landmark public–private partnership designed to accelerate the use of precision oncology, improve outcomes for people with cancer, and position Australia as a global hub for clinical trials.

Led by Omico, a national not-for-profit organisation, PrOSPeCT brings together the collective expertise of government, industry, research, clinical and community partners. Founding partners include Roche Australia, National Computational Infrastructure and Children's Cancer Institute. The initiative was launched with over $185 million in funding, including $61.2 million from the Australian Government's Modern Manufacturing Initiative and $25 million from the NSW Government.

The goal: to provide 23,000 Australians with advanced and incurable cancers access to free comprehensive genomic profiling (CGP) and match them to biomarker-led clinical trials that could provide personalised, cutting-edge treatment options not available through standard care.

Results so far include:

From Omico's MoST* and PrOSPeCT initiatives combined:

- Over 23,000 people with advanced cancer have been referred, by over 1,350 clinicians
- More than 16,400 patients with advanced cancers have received comprehensive genomic profiling (CGP); 68.5% (11,260+) received a matched treatment recommendation.
- Over 2,000 patients have been enrolled in clinical trials; 416 received matched therapy outside a trial; another 5,000 accessed other therapies.
- 36% of participants live in rural, regional or remote areas.
- Omico has supported 98 clinical trials—16 of which would not have occurred in Australia without this infrastructure- from 22 industry partners.

From PrOSPeCT alone:

- Omico's clinical trial network has more than doubled in two years—from 34 to 69 sites nationally, including 22 in regional areas.
- PrOSPeCT has driven $176 million in foreign direct investment for clinical trials, created 225 direct and 1,223 indirect jobs, and delivered 39 traineeships.
- The initiative has supported national growth in genomic pathology, with seven laboratories now NATA-accredited for clinical-grade CGP.

Summary

PrOSPeCT demonstrates the true potential of public–private partnerships to deliver health system innovation, economic return, and—most importantly—hope.

By focusing on people with advanced and incurable cancers, PrOSPeCT prioritises those most in need and least likely to benefit from standard care. Through national genomic screening and trial matching, Omico has opened up access to personalised treatments, including in regional areas where options have been historically limited.

This work showcases the value of coordinated, cross-sector collaboration—combining scientific excellence with health equity, economic growth, and real-world patient impact.

**Molecular Screening and Therapeutics Study*

https://www.omico.com.au/

Investments in advanced manufacturing are long-term and therefore require a stable and globally competitive policy environment. This includes political and economic stability, strong and reliable trade relations, a strong legal and regulatory system that supports innovation, and an internationally competitive tax environment. To realise the benefits of advanced manufacturing and build its knowledge economy, Australia should leverage knowledge creation and collaborative partnerships in areas of the value chain that do not require traditional production.