Medicines Australia

Bitter pill

How Australian patients are missing out on the latest medical breakthroughs

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Executive summary

Australia's current policy and medicines funding environment is contributing to a growing number of missing medicines, innovative therapies available in other high-income countries but delayed, restricted, or inaccessible for Australian patients.

At the core is a lack of investment in innovative medicines in the PBS, compounded by structural problems within Australia's health technology assessment (HTA) and funding system, which increasingly shape global launch decisions.

First, investment in innovative medicines through the Pharmaceutical Benefits Scheme (PBS) has declined over the last decade in real terms, both as a share of GDP and the health budget, despite the development of medicines that can significantly improve or save lives. This has been driven by policies that set low pricing targets and strong cost-containment measures, such as reference pricing and mandated price reductions. As a result, funding for truly innovative products has diminished, and the Australian market is no longer commercially attractive, or in some cases viable, for companies to launch new medicines.

Second, reimbursement has become increasingly complex, slow, and uncertain. Securing public funding often involves multiple assessment bodies, costly evidence submissions, extended negotiations, and long delays, even after regulatory approval. The likelihood of medicines being reimbursed within two years of international approval has fallen, and once medicines are approved, it is often through narrow conditions for restricted patient populations. As a result, HTA outcomes do not consistently reflect the full value of modern therapies.

The result is a widening access gap for Australians.

Being denied access to effective treatments is a bitter pill to swallow for patients and clinicians. Patients are missing treatments that improve survival, reduce disease burden, and enhance quality of life. Although Australia is a high-income country, our clinicians are often limited to prescribing older or less effective therapies, while access to some newer medicines depends on a patient's ability to pay higher out-of-pocket costs, creating a widening gap in health equity.

This report highlights examples of these '*missing medicines*' and the challenges patients face accessing them in Australia. The list is not exhaustive, but what is more concerning is it continues to grow.

These examples reinforce the need to reform funding for innovative medicines and vaccines. Changing HTA methods has the potential to remove redundancies and duplication, to simplify the evaluation of medicines and deliver access faster. But real change can only happen if underpinned by an overarching commitment to value innovation and big picture thinking about Australia's long-term health. New therapies are transforming care globally, and Australian patients deserve access to them.

Why are Australian patients missing out?

For the context of this report 'missing medicines' refers to innovative therapies, newer medicines that represent a meaningful advancement over existing treatments, delivering improved patient outcomes compared to older treatments.

These are clinically available and accessible to patients in comparable countries but are not available in Australia.

How does this happen?

It is the result of slowly evolving changes within Australia's policy, PBS funding, and health technology assessment (HTA) environment over the last twenty years, which has meant that Australia has slipped from being a global leader in funding new medicines to being a laggard.

This has arisen when:

- **HTA processes are slow or uncertain, delaying funding decisions:** Securing public funding for new medicines is often more bureaucratic and complex in Australia than other countries. It can require navigating multiple decision-making bodies, iterative negotiations, and extended timelines, even long after a medicine has been deemed safe and effective by Australia's regulator, the Therapeutic Goods Administration (the TGA). In some cases, even if a positive decision is reached, these processes can take several years, delaying patient access well beyond that seen in comparable countries. Ultimately, manufacturers may also opt not to progress an application, due to strict criteria or a lack of perceived value for their innovation.
- **HTA evaluation frameworks do not fully capture the value of modern therapies:** Broader benefits of medicines to individuals, their families and society, such as improvements in quality of life, reductions in treatment burden, productivity gains, or long-term system savings, are often not adequately considered or excluded by decision-makers despite their importance. This results in reimbursement recommendations that do not align with how innovation is recognised internationally.
- **Budget constraints and cost-containment mechanisms affect price:** Budget constraints and cost-containment measures have long been central to the design of the PBS helping to ensure affordability and sustainability of public spending. However, over time, these mechanisms have also contributed to delays in patient access to new medicines and, in some cases, decisions not to launch at all.

These dynamics have several flow-on effects:

- **Prolonged negotiations:** Pharmaceutical companies and government take longer to agree on the patient population, the conditions of listing, and price assigned to the medicine, extending the time to PBS listing.
- **Resubmissions and reassessments:** Where initial HTA recommendations include strict criteria that the manufacturer is unable to meet, further resubmissions are required, adding months or years to the process.
- **Deferral or withdrawal decisions:** If pricing expectations are too low, companies may delay submission, deprioritise Australia in their launch sequence, or decide not to proceed altogether.
- **Narrower listings:** To manage costs, reimbursement may be approved only for smaller patient groups, which can further complicate and prolong negotiations.

Why does this matter?

There is clear and growing evidence that Australia is becoming a lower priority market for the launch of new medicines. In a recent survey, 84 per cent of Medicines Australia member companies indicated that Australia has deteriorated, or is expected to deteriorate, in their global launch sequence.[1] A key reason is that it takes longer in Australia to secure funding for medicines that have already been assessed as safe and effective by the Australian regulator, the TGA, than in comparable countries.

Australia funds a smaller share of new medicines than similar countries.[2] Only around 25 per cent of medicines that have been launched globally over the past decade have been reimbursed in Australia, compared to 88 per cent in the United States and 46 per cent in the United Kingdom. Even where medicines are funded, access is often restricted, only a minority are reimbursed for their full intended patient population for which the medicine has been found effective and safe. [3,4]

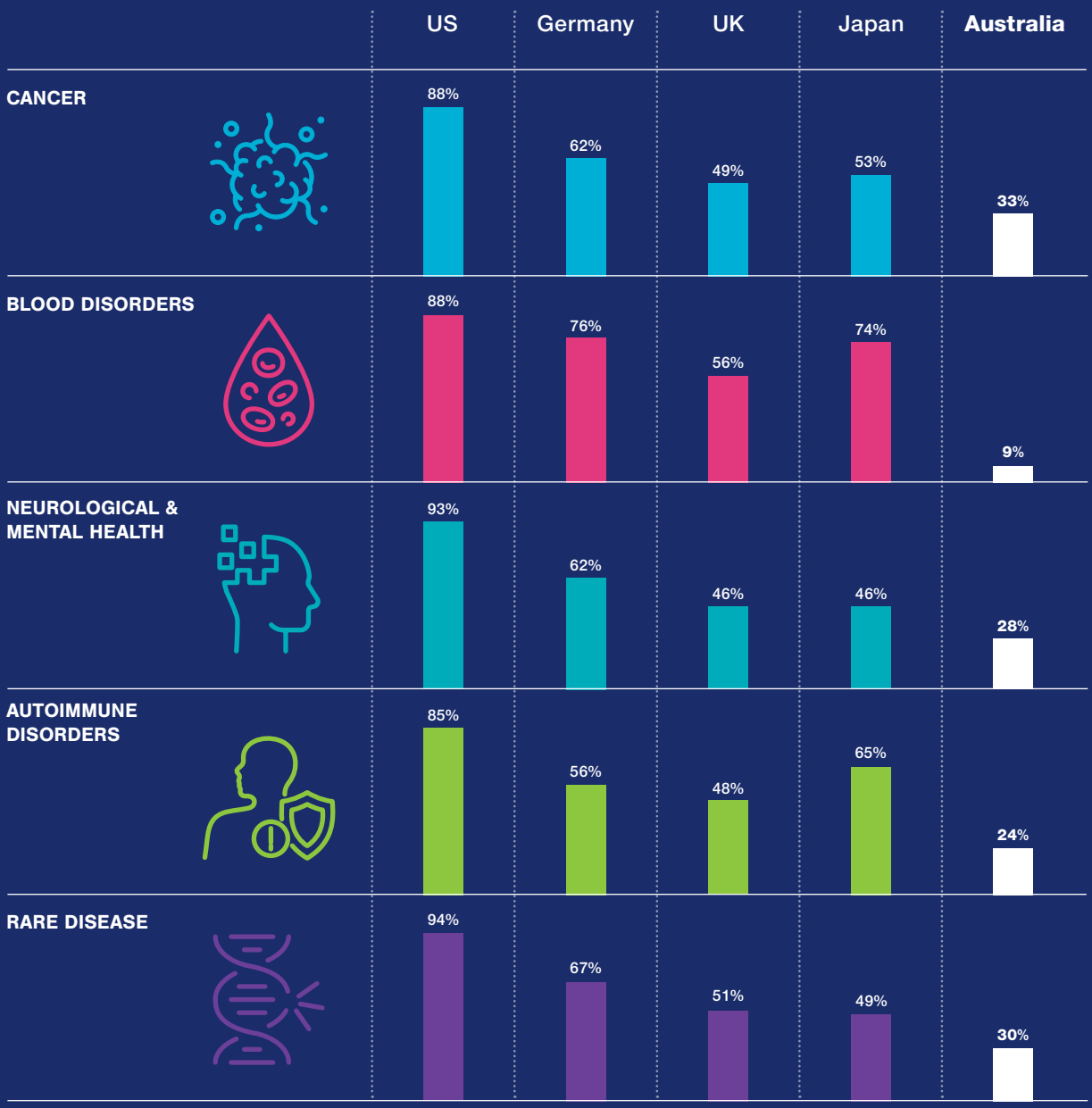
Bringing a medicine to market requires significant, long-term investment and risk. Only a small percentage of medicines that commence the development process successfully complete clinical trials and obtain regulatory approval. If a country's policy settings, through pricing, funding decisions, or delays, do not provide a viable return or recognise the value of innovation, companies may choose to launch later, limit access, or not launch at all.

The consequence is the benefits of new medicines are deferred or may be forgone entirely in Australia. This means Australia misses out on health gains, improvements in broader quality of life for patients and their families, and system and productivity gains that can reduce the number of procedures, strain on healthcare resources and longer-term cost offsets through better disease management.

This impact is also felt by clinicians, who have fewer treatment options available to them and patients, who may experience higher treatment burden, worse outcomes and reduced quality of life. And in cases where patients don't want to be left behind, they may need to self-fund treatment or go without, creating financial strain and inequities in access.

A recent survey by the McKell Institute found almost half of Australians surveyed had been offered or prescribed medicines by their doctor that were not on the PBS (43 per cent); and in the past three years, more than a quarter (29 per cent) have had to purchase a prescription medicine that was not listed on the PBS creating additional financial hardship.[5]

Share of New Medicines Reimbursed by Public Insurance by Therapy Area
(of all new medicines first launched globally within the past 10 years)



How Australia's access environment is compounding the problem

Funding of innovative medicines on the PBS and the reimbursement process for new medicines is not keeping pace with technological advancement.

PBS investment constraints

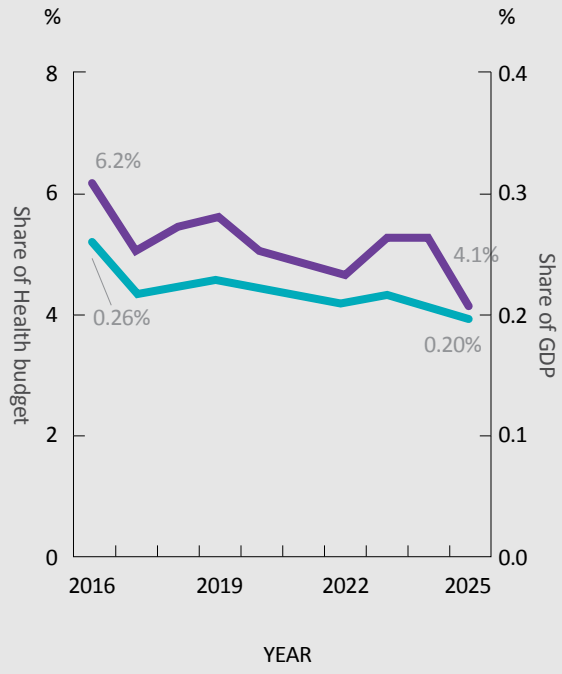
The Pharmaceutical Benefits Scheme (PBS) is the vehicle through which Australians access innovative medicines. It is a cornerstone of Australia's health system because it makes key medicines accessible, affordable, and equitable for the population. Since its inception in 1948 the PBS has delivered great value to Government.[6]

However, increasingly the PBS and the reimbursement process for new medicines is not keeping pace with technological advancement. This is a process problem but also a budgetary problem. Despite the 2022–27 Strategic Agreement stating an expectation of increased, "…real expenditure on new medicines", estimated net spend on innovative medicines has fallen in real terms in recent years.[7]

Since 2015–16 net investment in innovative (F1) medicines via the PBS has declined as a share of GDP from an estimated 0.26 per cent to 0.20 per cent, and from an estimated 6.2 per cent to 4.1 per cent as a share of the health budget.[8]

Even more concerning is that the likelihood that a globally supplied new molecular entity is reimbursed in Australia (listed on PBS or LSDP) within 2 years of approval in the United States has fallen from better than 1 in 5 to only around 1 in 10.

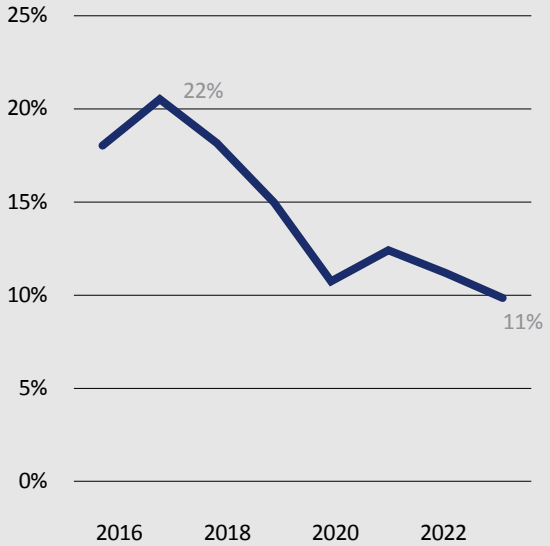
Estimated Net investment in F1 (innovative) medicines via the PBS



Source: MA analysis of PBS Expenditure report

Share of GDP
Share of Health Budget

Probability NME is reimbursed in AUS., within 2 years of FDA approval*



**2 year moving average; NME is New Molecular Entity; Source: MA Analysis of PhRMA database*

Cost containment mechanisms

Cost-containment mechanisms are a long-standing Australian policy and have effectively been applied as a handbrake to the growth of the PBS. With every cycle they are applied, they are having unintended consequences for medicines development, investment, and innovation in Australia.[9]

Policies and other mechanisms such as reference pricing, lowest cost comparator, price disclosure, mandatory price reductions and restrictive risk-sharing agreements systematically drive down the value recognised for innovative medicines over time. While these mechanisms are intended to contain public expenditure, they can also operate as blunt tools that drive down price, limit funded patient numbers, shift financial risk to sponsors, or impose conditions that make reimbursement commercially unviable. These approaches reduce the value ascribed to innovative medicines, despite the additional benefits they may offer to patients, clinicians and the broader health system. Furthermore, because many countries use international reference pricing, a lower price in Australia can influence pricing outcomes elsewhere. As a result, companies may delay launching in Australia to avoid triggering lower prices in other markets and some are having to make difficult decisions to not bring products to Australia at all.[10]

Australia has developed a reputation internationally for its aggressive approach to pharmaceutical pricing.[11] Since the early 2000s this has been positioned as necessary for PBS sustainability, but the real impact of this aggressive approach is starting to be realised. Australia is falling further behind our international partners with fewer innovative medicines being made available at an affordable price.

"A phone is just for calling"

A 25-year-old flip phone and the latest smartphone can both make a call. If you judged them on that single function alone, you might conclude they are essentially the same, and should cost the same.

But that would miss what matters.

Today's smartphone is also a camera, a map, a translator, and a gateway to countless services. Its value lies in the range of benefits it delivers beyond the basic call-making function, built through decades of innovation.

Yet if smartphones were priced by benchmarking them only against the cheapest device that can make a call, their broader value would be ignored.

This is how price referencing and lowest cost comparator approaches operate in medicines policy.

Modern therapies are assessed against older, lower-cost treatments that deliver only part of the benefit of the new treatment. These comparisons can focus on a narrow clinical outcome, such as survival, while undervaluing improvements in precision, safety, convenience, quality of life, and long-term system benefits.

Expecting modern medicine to cost the same as its past equivalents is like expecting cutting-edge technology at yesterday's price: it overlooks how much more it now delivers.

Clinical trials as a form of early access to innovative medicines

Clinical trials and early access programs often provide the first opportunity for patients to receive cutting-edge therapies, particularly for cancer, rare diseases, and advanced chronic conditions.[12]

For patients:

- Trials and early access pathways help bridge the gap between regulatory approval and funded access
- They may gain access to treatments years earlier than would otherwise be possible
- For some, particularly those with limited alternatives, these programs may represent the only pathway to innovative care[13]

For clinicians:

- Clinical trials and early access programs offer additional treatment options to patients who have not responded to the medicines that are otherwise available in Australia
- The treatments they can prescribe can stay aligned with newer international standards of practice
- They gain early experience with new therapies, supporting future uptake and integration into care

A strong clinical trials environment also attracts investment in the life sciences sector in Australia, positions Australia as an early adopter of new medicines and provides patients with access to pipeline therapies before global launch.

However, without PBS access to innovative medicines, Australia risks becoming a less attractive destination for clinical trials, jeopardising both patient access and one of the country's most valuable life sciences industries.

The added complication is patients who gain access to a new therapy through a clinical trial often experience significant improvement in their condition and sometimes a clinical trial offers hope after all other treatment options have been exhausted. However, when that medicine is not subsequently reimbursed, these patients can face considerable uncertainty and distress. At the end of the trial, continued access is not guaranteed, and patients may be forced to discontinue a treatment that was effectively managing their disease. This can lead to deterioration in health, loss of stability, and difficult decisions about whether to self-fund treatment at substantial personal cost. Clinicians are also placed in challenging positions, unable to offer a therapy they know is beneficial.

How the global access environment is compounding the problem

Australia's low willingness to pay for innovative medicines has weakened the incentives for global pharmaceutical companies to launch new therapies in Australia.

The evolving global pricing environment, particularly the US Administration's push to rebalance international prices of pharmaceutical products, places Australia at a critical juncture, with the risk of delayed access to, or absence of, new medicines likely to increase significantly.

For decades, the global pharmaceutical system rested on predictable norms — the US accepted higher prices in exchange for early access and a strong industry base, while Europe, the UK and other developed nations relied on external reference pricing (ERP) and HTA to negotiate favourable visible list prices. Trade policy reinforced this equilibrium through the *"zero-for-zero"* framework, which enabled tariff-free movement of medicines and allowed pricing strategies and supply chains to develop largely independent of trade frictions.[14]

However recently, the United States (US) has turned to a number of different trade levers to try and bring the price of innovative medicines down in the US. The Most Favoured Nation (MFN) policy has become the centrepiece of the drug pricing agenda, both in scale and in political prominence.

MFN pricing requires manufacturers participating in Medicare to offer prices that match, or are lower than, the lowest prices available in comparable countries.[15] Given the size and strategic importance of the US market, manufacturers may rationally delay or avoid launching products in lower-priced markets such as Australia to minimise the risk of setting a lower global reference price.

Statements from the White House frame MFN not as a reduction in total pharmaceutical revenue but as a rebalancing of who contributes to global research and development costs that is designed to exert upward pricing pressure on reference countries and '*push foreign governments to pay their fair share.*'[16]

MFN accelerates the transition toward a globally interdependent pricing system. In this environment, Australia's optimal pricing strategy becomes strategically constrained. Aggressively driving price reductions no longer delivers purely domestic impacts but instead contributes to global price erosion, prompting supply-side responses that reduce availability and threaten the ongoing sustainability of the PBS.

The effects of MFN are already starting to show. Analysis from market research company GlobalData found that European launches of new medicines have fallen by 35% in the 10 months following the US policy change.[17]

Health Minister Mark Butler has recognised this concern in a keynote address, saying that reducing US prices creates price pressure in every other market, including Australia:

"We are concerned, as I think is every other country around the world, that it could have a chilling effect on pharmaceutical companies' willingness to bring new medicines to a market like ours".

International Case Study — The US – UK Pharmaceuticals Deal and Innovation Incentives

The 2025 UK–US pharmaceuticals trade agreement provides a clear example of how governments are intervening in policy settings to address concerns about access to innovation and investment in medicines.

The UK has committed to a series of policy shifts designed to improve access to innovative therapies and strengthen incentives for industry[18]:

- Increased investment in new medicines, with a significant uplift in spending on innovative treatments for the first time in decades.
- Reforms to HTA and pricing settings, allowing more medicines to be approved even where they may previously have been rejected on cost-effectiveness grounds.
- Adjustments to cost-effectiveness thresholds and rebate mechanisms, to better reflect the value and development cost of innovative therapies.
- Commitments to faster patient access, with the explicit aim of bringing new treatments to patients more quickly.

At the same time, the deal included measures to strengthen the commercial environment for life sciences companies, including zero tariffs on pharmaceutical exports and policy settings designed to encourage companies to prioritise the UK for early launches.[19]

The deal reflects a shared recognition by both governments that pricing, reimbursement, and investment settings directly influence whether patients can access new medicines—and whether companies choose to develop and launch them in a given market.

The deal was explicitly framed around the principle that all countries should contribute fairly to the cost of pharmaceutical innovation and that improving the operating environment for companies is essential to ensuring timely patient access.[20]

Evidence and Impact

Australia's growing challenge with access to innovative medicines is increasingly visible across multiple disease areas.

Despite being a high-income country, Australia has reimbursed only a small number of new medicines launched globally over the past decade.[21]

This issue is particularly important in the context of broader health system performance. As the Productivity Commission has found, improvements in healthcare outcomes relative to cost have been driven in part by the adoption and diffusion of new technologies, including medicines. However, it also notes that more timely access to new treatments is essential to sustain these gains, warning that delays in approval and reimbursement risk slowing the contribution of innovation to health and productivity.[22]

This raises a critical question: which treatments are not coming to our shores, and what are the implications for care? The following case studies are examples of medicines that have not come forward, it is not exhaustive and does not preclude other medicines being impacted by the same challenges.

	Disease	Condition	Medicine	Patient population	Access challenge
	**Mental Health**	Schizophrenia	xanomeline/ trospium chloride	1 in 100 Australians	Comparator pricing
	**Neurology**	Migraine	Rimegepant, erenumab	21% of Australians	Pricing, patient utilisation, budget impact and financial risk to Government
	**Cardiology**	Pulmonary Arterial Hypertension	Sotatercept	3000 Australians	Comparator pricing and budget impact
	**Oncology**	HER2 positive gastric cancer	Trastuzumab deruxtecan	Approx 400 new cases every year	Direct comparator not available in Australia
	**Haemotology**	Haemophilia B	Marstacimab	1 in 25,000 men	Duplication of review and approvals
	**Neurology/ Sleep disorder**	Narcolepsy (type 1)	Oveporexton	3,500–6,000	Lack of innovation and system challenges
	**Rare Disease**	Congenital Adrenal Hyperplasia (CAH)	Hydrocortisone granules	21–26 babies every year	Comparator price erosion
	**Oncology**	Lymphoma	Polatuzumab vedotin	2000 new cases/year	Low ICER threshold
	**Dermatology**	Atopic dermatitis	Lebrikuzumab, abrocitinib	Approx 560,000	Pricing, patient numbers, budget impact and financial risk to Government
	**Immunology**	Crohn's disease	Risankizumab, Guselkumab	Approx 93,000	Comparator price erosion
		Ulcerative Colitis	Mirikizumab	> 80,000	Lowest cost comparator
	**Vaccines**	Meningococcal B	Men B vaccine	> 100 cases in 2024	Lack of national harmonisation
		RSV	multiple	> 27,000 hospital admissions/ year	Not all at-risk groups covered by immunisation
	**Oncology**	Prostate cancer	Lu-PSMA-617 radiopharmaceutical	24,000 new cases/year	Generic medicines bypassing TGA assessment and partial MBS subsidy only
	**Infectious disease**	Antimicrobial resistance	Multiple new antibiotics	5,200 deaths each year	Need for a new reimbursement model

Mental Health:
First new schizophrenia therapy for a generation not accessible in Australia

Schizophrenia is a chronic and disabling mental illness affecting approximately 1 in 100 Australians, around 250,000 to 260,000 people, with an estimated economic burden of $2.6 billion annually.[23] Symptoms span multiple domains, including positive symptoms (hallucinations, delusions), negative symptoms (social withdrawal, reduced motivation), and cognitive impairments (impairments in memory, attention and executive function). Together, these symptoms can significantly disrupt education, employment, relationships, and overall functioning. Schizophrenia is also associated with substantial long-term health consequences, including persistent disability and a markedly reduced life expectancy.[24]

The current standard of care in Australia relies predominantly on antipsychotic medications that act through dopamine D2 receptor signalling, a pharmacological approach that has underpinned schizophrenia treatment for more than 60 years.[25] While these can be effective for the treatment of positive symptoms, significant unmet need remains, particularly for negative symptoms and cognitive impairment. Treatment is also often complicated by adverse effects that can impact adherence and long-term outcomes, including extrapyramidal symptoms such as muscle stiffness, painful spasms and involuntary uncontrollable body movements and hyperprolactinaemia, as well as weight gain, metabolic complications and sedation. In addition, approximately one-third of people with schizophrenia develop treatment-resistant disease. As a result, there remains a significant unmet need.

1 in 100

Schizophrenia affects 1 in 100 Australians, around 250,000 to 260,000 people, with an estimated economic burden of $2.6 billion annually

The development of xanomeline/trospium chloride represents the first new approach in decades. With a distinct mechanism of action xanomeline/trospium chloride activates muscarinic receptors in the brain and does not block dopamine receptors.[26]

The treatment is commercially available to millions of patients in the United States but is not available in Australia.

The PBS comparator framework is designed to ensure cost-effectiveness, but when it defaults to the lowest-cost option, it can systematically undervalue first-in-class therapies. For a medicine with a novel pharmacological approach, this creates a structural barrier to access where current comparator frameworks may not fully capture their differentiation.

Global pricing dynamics (including MFN policies) dictate that the broader international consequences of the reimbursed price in Australia must be considered. The price as prescribed by application of the PBS comparator framework may make listing in Australia infeasible.

For individuals living with schizophrenia—particularly those who have not responded to or cannot tolerate existing therapies—this demonstrates that rigidity of Australia's reimbursement process is directly harming their health.

Neurology:
CGRP inhibitors for the treatment of episodic migraine

Migraine is associated with structural and functional changes in the brain that may lead to increased pain sensitivity, sleep disorders and neurohormonal conditions. People living with migraine can have increased rates of depression, anxiety, obesity, diabetes and cardiovascular conditions and during attacks, and many patients cite productivity issues such as an inability to work or go to school, reduced earning capacity, and ability to contribute to family and caring. [27]

A recent report from the Australian Institute Health and Welfare conducting a health survey on a sample of 13,000 Australian households deemed migraine the most prevalent neurological condition in Australia with approximately 1 in 15, or 1.7 million, Australians living with long-term migraine. However, a report from Deloitte Access Economics suggests that the prevalence of migraine could affect as many as 4.9 million people in Australia (21% of the population). Of the 4.9 million, it is estimated 400,000 experience ≥15 headache days per month, often referred to as chronic migraine.[28]

Calcitonin gene–related peptide (CGRP) plays a central role in the chain of biological events that trigger a migraine attack. Unlike many traditional migraine medications, which were originally developed for other conditions such as epilepsy, high blood pressure, or depression, CGRPtargeted therapies were designed specifically with migraine biology in mind. They are used to prevent migraine attacks and reduce the frequency, duration and severity of migraine attacks.[29] As a result, they represent the first preventive treatments developed specifically for migraine, marking a shift in how the condition is managed.[30]

However, despite the significant clinical burden of migraine in Australia and advances in understanding its underlying biology, access to newer, targeted therapies remains limited. Currently, there are three injectable CRGP receptor antagonist treatments available, limited to patients defined by PBS restrictions as high episodic and chronic migraine.

Rimegepant, an oral CRGP, represents one of the first acute and preventive treatments developed specifically to interrupt the biological pathways driving migraine attacks. For breakthrough migraine attacks it offers an important alternative for patients who do not respond to or have contraindications to therapies such as NSAIDS or triptans.[31]

What patients are saying:

Chloe Cifelli, Chairperson, Migraine Australia

"For people living with migraine, one treatment strategy will never fit everyone. Every patient experience is unique, and many people need access to multiple treatment options to find what actually works for them.

When newer evidence-based treatments are delayed, limited or not subsidised, people are left behind. They are forced into either/or decisions, rather than being able to work with their clinician on the best treatment strategy for their circumstances.

At Migraine Australia, we hear from people who say, "I've tried everything," or "this is my last attempt." That is heartbreaking. Delayed access does not just mean waiting for a medicine. It can mean years of pain, lost work, lost family moments, and people losing hope.

Australians living with migraine deserve timely access to a broader range of evidence-based treatment options, so they have a fair chance at getting part of their life back."

It is estimated 400,000 Australians experience

≥15

headache days per month

Often referred to as chronic migraine

Rimegepant has been widely approved internationally for reimbursement for the treatment of acute migraine and the prevention of migraine, including in the UK, Ireland, Canada and Japan. In Australia, Rimegepant was considered by the PBAC in July 2023 and again in March 2024, and there has been no further application to date. The PBAC assessment of the second submission raised concerns about the cost effectiveness of rimegepant, the budget impact and financial risk to government and the estimated patient numbers. [32] That is despite the PBAC acknowledging that there is clinical need for new, oral treatments for the acute treatment of migraine as well as strong support for the treatment from leading patient organisations including Migraine & Headache Australia, the Australian and New Zealand Headache Society and Pain Australia.

Another innovative, targeted CGRP therapy is erenumab, which is administered as a once-monthly subcutaneous injection. The manufacturer has sought PBS listing for erenumab since July 2018, for patients with chronic migraine who had not responded to at least three prior preventive treatments, those with the greatest unmet clinical need, on three separate occasions. The most recent submission, considered in November 2024 received strong support from clinicians, patients and advocacy groups, and achieved a positive recommendation. However, due to the application of lowest cost comparator, the price available for the medicine does not make it viable to proceed.

The lack of reimbursement for these innovative treatments means many patients face significant outofpocket costs or are unable to access the medicine at all. This can result in continued reliance on less effective or poorly tolerated treatments, prolonging the frequency and severity of migraine attacks and their associated impacts on daily life.

Given that migraine already contributes substantially to lost productivity, reduced workforce participation and diminished quality of life in Australia, restricted access to newer targeted therapies may exacerbate these burdens. For individuals, this can mean ongoing disruption to employment, education and family roles, as well as increased psychological stress and reduced ability to plan and participate in everyday activities.

A win for patients or the end of a frustrating delay?

In June this year, access to galcanezumab, a preventive CGRP therapy for migraine, was expanded on the PBS, to include patients with eight or more migraines a month (episodic migraine) which has been heralded as a significant step forward that could help thousands of migraine sufferers.[33]

Galcanezumab was first PBS listed in June 2021 for the treatment of chronic migraine, (15 or more headache days per month with less than 8). At the time, the sponsor had applied to the PBAC to include treatment for episodic patients as well, but that application was rejected. This effectively kept affordable access to CGRP inhibitors out of reach of thousands of migraine sufferers for more than four years. Over those five years, a patient suffering from migraine could have had to endure more than 380 debilitating attacks without access to an affordable innovative treatment.

Speaking to media following the expanded listing Eli Lilly Managing Director Manny Simons spoke of the time taken to deliver this outcome.

"*We've spent the past year working to improve access to this class of medicines, and we're pleased this has resulted in a better outcome for those eligible for this treatment option.*" *Mr Simons said.*

"*The unfortunate reality is that Australians living with severe migraine have fewer affordable treatment options than patients in other countries like the UK and Canada. This expanded listing is a meaningful step forward, but it took too long to achieve. This is another example of the urgent need to reform the PBS to improve access to innovative medicines.*"

What patients are saying:

Carl Cincinnato, Lead, Migraine & Headache Australia, A division of the Brain Foundation

"For Australians living with frequent or disabling migraine, timely funded access to innovative treatments can be the difference between participating in work, study, parenting, caring and community life, or losing days each month to an often invisible neurological disease. Many people have already cycled through older preventive medicines that were not developed for migraine and can be ineffective or poorly tolerated. Newer CGRP-targeted treatments, including oral options such as rimegepant and preventive options such as erenumab, are important because they are designed specifically for migraine and may offer meaningful relief for people who have exhausted other options. Recent PBS progress is welcome, but too many Australians still face delays, restrictions or out-of-pocket costs that put effective care out of reach. Funded access matters because migraine is treatable, and Australians deserve timely, equitable access to medical breakthroughs available in comparable countries."

Cardiovascular: Pulmonary arterial hypertension (PAH)

Pulmonary arterial hypertension (PAH) is a condition in which the arteries carrying blood from the heart to the lungs become narrowed, increasing pressure and placing strain on the heart, which can lead to life-threatening heart failure. Approximately 3,000 Australians are currently living with PAH. It is most common in women aged 30–60.[34]

PAH can significantly impact daily life, affecting physical activity, employment, relationships, and emotional wellbeing. Many people report difficulty with routine tasks such as walking short distances, climbing stairs, and shopping, as well as feelings of isolation due to the invisibility of the condition.[35]

In Australia, availability and reimbursement of PAH therapies has not evolved in line with contemporary international treatment guidelines. While there is no cure for PAH, current PBS-funded treatments primarily help to relax and widen the blood vessels in the lungs, improving symptoms and quality of life, but not directly addressing the underlying changes in the lung blood vessels that cause the disease to progressively worsen. As a result, many patients continue to deteriorate despite receiving multiple therapies, and there has been no new type of PAH treatment introduced in more than a decade.

3,000

Australians living with Pulmonary arterial hypertension

It is most common in women aged 30–60

Sotatercept is the first approved activin signalling inhibitor for PAH and acts through a different biological pathway from currently available therapies. It is designed to target mechanisms involved in the vascular remodelling that contributes to PAH progression. In Phase III clinical studies, sotatercept demonstrated statistically significant improvements across key clinical outcomes compared with placebo when added to background therapy.[36]

The treatment was first registered by the TGA in Australia in November 2024 and to date is not available through the Pharmaceutical Benefits Scheme (PBS). The PBAC rejected a submission for reimbursement in March 2025, despite recognising high unmet need in this patient population and that the medicine provided added therapeutic value.

The health technology assessment to date has resulted in significant challenges for the reimbursement of sotatercept in Australia. Concerns remain that elements of the economic evaluation may not fully capture the potential value of a therapy that acts through a novel biological pathway and

addresses an area of substantial unmet clinical need. Meanwhile, patients in countries including France, Canada and the United Kingdom already have access to publicly funded sotatercept. As reimbursement discussions continue, there is a risk that Australian patients will experience prolonged delays in accessing the first approved PAH therapy in more than a decade to act through a new mechanism and target the underlying disease process.

What patients are saying:

PAH patients living overseas

"Sotatercept had given me hope and I finally feel optimistic about my future", "Sotatercept has given me my life back", "My heart and pulmonary artery are almost normal, I can live a normal life"

Jenna Mazor, a patient living with PAH in Australia

Meanwhile, here in Australia, our pulmonary hypertension support groups are filled with questions no patient should have to ask: *"What bag fits my pump for my Hickman line?" "How can I manage the nausea from titration so I can keep working?" "Where can I get supplementary oxygen?"*

For a country that prides itself on having one of the world's best healthcare systems, why are Australians with pulmonary hypertension still being left with invasive, decades-old treatments when safer, more effective alternatives already exist?

I am a mother living with PAH. My daughter, Ava, is six years old. Access to this medication wouldn't just change my treatment — it would change our lives. It would mean being able to do activities with her that never felt possible, like run around with her, or go on a bike ride. Ultimately, it would give me the chance to be present for her childhood, to make memories with her instead of watching from the sidelines and hoping I'll still be here for the next milestone.

I am just one patient, and Ava is just one little girl. But access to this medication won't just save lives - it will give families more time together and children more precious moments with the people they love.

It's easy to read a patient's statement like mine as words on a page. I ask you to see the people behind those words. Think of your own daughter, son, grandchild, or someone you love, and imagine if one decision had the power to change their future.

Your decision can change ours.

Tanya Hall, Chief Executive Officer Hearts4Heart

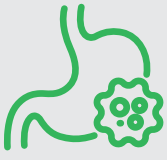
"People living with pulmonary arterial hypertension and their families often face a lifelong journey of uncertainty, complex treatment decisions and the challenges of managing a progressive condition. However, there remains a significant need for new treatment options that can improve quality of life, slow disease progression and support people to live well for longer.

At Hearts4heart, we believe Australians should have timely and equitable access to clinically appropriate, evidence-based treatments, regardless of where they live. Delays in accessing innovative medicines can have a profound impact on patients and families, particularly for those living with serious and progressive heart conditions.

New therapies such as sotatercept represent hope for people living with PAH. Ensuring Australians can access important medical advances through sustainable funding pathways is essential to improving outcomes and addressing unmet need."

2,700

Stomach cancer cases diagnosed in Australia per year



Approximately 13–16% of these are HER 2-positive

Tina Powney – patient

"Hope is incredibly important when you live with a rare disease. Knowing that new treatments exist gives patients hope, but that hope quickly turns to disappointment when those treatments remain out of reach because they aren't funded. Every delay has a real human cost.

"People often take simple things for granted—going for a swim, camping with family, travelling or even leaving the house without carrying life-sustaining equipment. Living with Pulmonary Arterial Hypertension means those simple moments become enormous challenges. Access to innovative medicines isn't just about living longer; it's about giving people the chance to live more freely."

I'm grateful to be alive, but at the moment I'm surviving rather than living. Every decision I make revolves around my IV pump, my medication and whether I'll be well enough tomorrow. Knowing there may be treatments available that could give me back some independence, but that Australians can't readily access, is heartbreaking."

Oncology:
Trastuzumab deruxtecan for the treatment of gastric cancer (HER2 positive)

Around 2,700 new stomach cancer cases per year are diagnosed in Australia and approximately 13–16% of these are HER 2-positive. Gastric cancer is associated with a poor prognosis, particularly in the advanced stages of the disease, with only 5% to 10% of metastatic patients surviving five years.

Trastuzumab deruxtecan delivers chemotherapy directly to cancer cells which is thought to make it more effective than standard chemotherapy alone. Clinical studies show Trastuzumab deruxtecan extends the life of a gastric cancer patient by around 4 months compared to chemotherapy and by around 3 months compared to RAM + PAC.

It is available via reimbursed access for patients in 16 countries including Germany, Singapore, Italy, France, Korea, Canada, Slovenia and Austria.

In most other countries, ramucirumab plus paclitaxel (RAM + PAC) is the Standard of Care in the second line metastatic cancer. In Australia however, although RAM + PAC has been recommended by the PBAC, it is not PBS listed. This means that there is not a direct comparator available to properly assess the medicine in Australia.

Trastuzumab deruxtecan

Is available via reimbursed access for patients in 16 countries including Germany, Singapore, Italy, France, Korea, Canada, Slovenia and Austria

To achieve listing in Australia, Trastuzumab deruxtecan must instead rely on an uncertain comparison with chemotherapy where clinical evidence is limited.

Australia's HTA system is not set up to recognise the value of this therapy where there is an absence of a suitable local comparator it creates a situation where innovative therapies are forced to rely on uncertain comparisons with outdated treatments because innovative therapies are not available on the PBS.

What patients are saying:

Dr Mark Buzza, Head of Research, Innovation and Advocacy (Pancare Foundation)

"Patients with advanced gastric cancer face an extremely challenging diagnosis and treatment path. Compounding this, it is deeply concerning that Australian patients currently do not have access to the latest standard of care therapies that are reimbursed in many comparable countries.

Ramucirumab plus paclitaxel is currently not listed on the PBS, despite being recommended by PBAC. This creates a significant gap in treatment options and makes it more difficult for patients to fairly access innovative therapies like trastuzumab deruxtecan given that there is no direct comparator available in Australia.

From a patient perspective, timely access to affordable, effective and evidence-based treatments can mean more time with loved ones, a better quality of life, and ultimately more hope. Australia risks falling behind international best practice, and this inequity has significant consequences for patients and their families."

We urge decision-makers to consider the clinical reality and urgent unmet need and adopt more flexible reimbursement approaches that ensure Australians can access potentially life-extending therapies, when they need them most.

Haemotology:
Marstacimab for the treatment of haemophilia B

Haemophilia is a rare, X-linked congenital bleeding disorder caused by deficiency of clotting factor VIII (Haemophilia A, HMA) or factor IX (Haemophilia B, HMB), resulting from mutations in the F8 or F9 genes. In Australia, around 1 in 6,000 men have HMA, and 1 in 25,000–30,000 men have HMB.[37]

Marstacimab is approved in many countries for patients aged 12 years and older with severe HMA or HMB without inhibitors. It is administered once weekly via subcutaneous injection, precluding the need for regular intravenous infusions. Intravenous therapy can damage veins over time, and needle phobia is common. Strict adherence is essential to prevent bleeding and long-term complications.

Haemophilia significantly affects patients and their families. Repeated joint bleeds lead to chronic arthropathy, pain, and reduced mobility, limiting independence and daily activities.

Frequent bleeds disrupt work, education, social participation, physical activity, and travel, with flow-on impacts on relationships, mental health, and overall wellbeing. Reduced ability to engage in physical activity can also contribute to poorer fitness and weight management. Many individuals with severe haemophilia develop chronic pain and arthritis early in life, often requiring orthopaedic surgery including joint replacement. Breakthrough bleeds may necessitate hospital visits and ongoing support from carers.

1 in 6,000

Australian men have HMA

1 in 25,000–30,000 men have HMB

If marstacimab was available to Australians via the National Blood Authority (NBA) it would significantly reduce treatment burden and has the potential to improve quality of life, particularly for patients with severe HMB who have limited alternatives. Over time, improved bleed control is expected to support better joint health, a key determinant of long-term quality of life.

Although marstacimab has been approved in over 40 countries, including Australia (TGA approval in January 2025), Australian patients do not have access. This is due in part to the complex reimbursement pathway for blood products, which requires assessment by the National Blood Authority, Medical Services Advisory Committee, Jurisdictional Blood Committee, and completion of a tender process before funding may possibly be granted.

Marstacimab

Approved in over 40 countries, including Australia. However Australian patients do not yet have access

What patients are saying:

Natashia Coco, Executive Director, Haemophilia Foundation Australia

'Marstacimab will alleviate a significant treatment burden for patients with haemophilia B by allowing for a once-weekly, self-administered, subcutaneous injection. Currently treatment is prophylaxis usually with an intravenous replacement clotting factor therapy, ranging from daily to fortnightly. The impact of bleeds and treatment on participating in 'normal' activities with family, friends and peers such as sport, exercise, socialising and travel are consistently priorities for people with haemophilia. Particularly those with moderate and severe forms who require prophylaxis. For young people, benefits may relate to school camp or sharing accommodation and having to explain your medication. Participating in recreational activities with friends and family such as camping – rather than missing out — is also a common desire. Reducing pain is also a high priority.'

Neurology/Sleep disorder: Narcolepsy Type 1

Narcolepsy is a chronic neurological disorder that affects the brain's ability to regulate sleep-wake cycles, resulting in excessive daytime sleepiness, disrupted night-time sleep, sleep paralysis/ hallucinations, cognitive effects such as memory loss, poor concentration and irritability, and comorbidities, such as cardiovascular disease, migraine, weight gain, type 2 diabetes, depression and anxiety.[38]

Narcolepsy type 1 is also associated with cataplexy, a sudden loss of muscle tone, triggered by acute emotions.[39]

All areas of life, including work, school and personal relationships can be affected because of difficulty concentrating, struggling to stay awake during work or school hours and unintentional napping during the day. Feelings of isolation and stigma can lead to depression and anxiety. People living with narcolepsy can incur higher healthcare costs, experience reduced employment rates and lower income levels.[40]

An estimated 12,000 Australians live with narcolepsy, approximately 30–50% of whom have narcolepsy type 1, and are unable to access world-class care.[41]

Newer medicines, such as sodium oxybate (short-acting and extended-release), solriamfetol, and pitolisant, most of which also treat cataplexy in addition to excessive daytime sleepiness, are not registered, reimbursed or widely available in Australia, despite their availability in the USA and major European Union countries.[42]

Only much older medicines are available in Australia, including wakefulness promoting agents modafinil and armodafinil, and stimulants such as dexamfetamine. These medicines only treat excessive daytime sleepiness.[43]

Australian Therapeutic Guidelines recommend the wakefulness promoting agents as first-line therapies and stimulants as alternative agents. However, the PBS only allows access to wakefulness promoting agents if stimulants pose an unacceptable medical risk or have resulted in intolerance necessitating stimulant withdrawal. This means that in clinical practice, stimulants are positioned as first-line and wakefulness promoting agents as second-line treatments.

12,000

Australians

Estimated to be living with narcolepsy

30–50% are unable to access world-class care

The Australasian Sleep Association has lodged two PBAC submissions (2008 and 2020) to move modafinil to first line therapy to align with clinical guidelines and minimise the use of dexamphetamine that the PBAC acknowledged has pressing safety concerns. The PBAC agreed in 2022 that modafinil and armodafinil could be used first-line but only if cost minimised to the cheaper dexamfetamine. This change to the listings has not occurred.

Narcolepsy type 1 is caused by a loss of orexin-producing neurons in the brain. Oveporexton is an orexin-2 selective agonist that targets the underlying cause of narcolepsy type 1. It is in the process of seeking regulatory review in several countries. Oveporexton has been designated an orphan drug and received priority determination by the TGA,[44, 45] a Breakthrough Therapy by the FDA and is on track to be first-in-class with the potential to establish a new standard of care.[46]

Listing oveporexton on the PBS in Australia will be highly challenging, as this first in class medicine must be assessed against low-cost comparators in a system that has not recognised high unmet need in narcolepsy. Australia's HTA system rarely accounts for the full societal impact of a therapy, such as increased productivity, which is a key benefit of appropriate treatment of this condition.

What patients are saying:

Dr Aaron Schokman, Research Fellow University of Sydney and patient living with Narcolepsy

"The hardest part of living with narcolepsy isn't any single symptom, but what the condition takes from me each day. From how productive I am, what plans I can keep and what relationships I can maintain. The medicines available to me in Australia help, but only partly. They blunt the overwhelming sleepiness and the paralysis I get from positive emotion (cataplexy), but never completely and never without side effects. Not to mention, there is nothing readily available to treat other symptoms like broken night-time sleep or the constant brain fog. Even on a good day, I have to ration what I do and plan around it all. When I sought newer treatment years ago, most specialists I saw had not heard of most of the medications I asked about, even though they had been standard care overseas for over a decade. People with the same diagnosis in the United States and Europe have had these medicines for years. Why not us? I am not asking for a miracle, only for an equitable, evidence-based standard of care that already exists internationally."

What clinicians are saying:

Dr David Cunnington, Sleep Physician.

"Narcolepsy is more than sleepiness. Alongside excessive daytime sleepiness, it brings cataplexy, disrupted night-time sleep, cognitive difficulty and higher rates of cardiometabolic and mental health disorders, affecting work, study, driving and relationships from a young age. My concern is that these are precisely the impacts that our health technology assessment captures poorly: standard quality-of-life measures are insensitive to sleepiness and sleep disorders, and the productivity and carer costs that dominate the impact of this lifelong condition, which most commonly presents in early adult life, fall outside the assessment's perspective. The effect is to consistently understate the value of treating narcolepsy. This shows in what I can offer: the medicines available to me in Australia are well behind contemporary international guidelines, and several treatments considered standard of care overseas are unavailable to my patients here."

Ass/Prof Clare Ellender, Respiratory and Sleep Physician

"Hypersomnolence disorders like narcolepsy and idiopathic hypersomnia are debilitating sleep conditions. There are evidence-based treatments that have been available overseas for many years, and the lack of access in Australia has left our local patients missing out. Missing out on opportunities to be able to engage with their families, friends, work, earn a reasonable living. Even things many of us take for granted, like driving. Like many clinicians in this area, the limited prescribing options in Australia mean that I'm prescribing therapies that are potentially less effective that internationally available treatments. This sets Sleep Medicine in Australia backwards, whereas we are usually scientific and clinical leaders internationally. We have lots of skilled doctors, sleep scientists and allied health teams in Australia, so the infrastructure is present to deliver excellent healthcare. The missing piece is evidenced based pharmacotherapy."

Rare Disease:
Hydrocortisone for the treatment of paediatric adrenal insufficiency, including congenital adrenal hyperplasia (CAH)

Congenital Adrenal Hyperplasia (CAH) is a rare, lifelong and potentially life-threatening endocrine disorder requiring regular hydrocortisone replacement from infancy. International estimates suggest CAH affects around 1 in 12,000–14,000 births (21–26 babies in Australia each year).[47] Infants and young children often require very small, individualised doses several times daily to avoid adrenal crisis, poor growth and long-term morbidity.

21–26
babies

or 1 in 12,000–14,000 births each year in Australia are affected by CAH

Hydrocortisone tablets (4 mg and 20 mg formulations) are currently reimbursed on the PBS. However, these tablets are not designed for neonates or young children, meaning parents and carers often cut, crush or dissolve adult tablets to approximate paediatric doses. Due to the length of time these adult hydrocortisone tablets have been on the PBS, it has undergone multiple statutory price reductions and is currently available as a generic.

Hydrocortisone is available (and funded) in smaller strength doses in other countries such as the UK, Germany, Austria, Italy, Switzerland and the United States. These enable much more accurate dosing, leading to reduction in adrenal crises, hospitalisations and long-term morbidity.

The PBAC received 30 stakeholder submissions supporting the addition of hydrocortisone capsules to the PBS. Clinicians described it as easier to administer and preferred by families. Although the PBAC recommended the treatment for PBS listing in July 2021, reimbursement negotiations failed. This is because it was determined only a modest premium of approximately 15–20% over the generic hydrocortisone tablets would be acceptable. From the manufacturer's perspective this would not appropriately recoup their R&D and clinical trial costs, manufacturing complexity as well as the supply chain challenges in servicing a small number of patients.

The manufacturer responded by asking for a price equivalent to the lowest international reimbursed prices identified at the time (Austria and Germany), but the revised offer was not accepted. The manufacturer provided detailed financial projections over the first six years of supply, showing that acceptance of the proposed PBAC price would impose sustained and significant financial losses.

This exemplifies how Australia's reimbursement system can limit the availability of new therapies. Not only is innovation being assessed solely against decades-old generic comparators, but rigidity and complexity also prevent the achievement of a suitable compromise.

What patients are saying:

UK CAH Support Group

"Accurate dosing is critical for infants and young children with CAH. The reliance on adult hydrocortisone tablets, which often need to be cut, crushed or dissolved, places an additional burden on families and can make an already complex condition even more difficult to manage. A funded, licensed paediatric formulation would support more precise dosing, reduce stress for carers, and provide families with greater confidence in day-to-day treatment.

We also strongly support the need for equitable access to improved treatment options for people living with CAH in Australia and across the globe. Families should not be disadvantaged simply because an age-appropriate medicine available in comparable countries remains unavailable or unfunded here."

Intergenerational equity

Nearly 2 million Australians live with a rare disease. Around 80 per cent of these diseases are of genetic origin and almost three-quarters of these diseases impact children.[48]

The HTA Review, completed in 2024, found: "access to PBS listed medicines for paediatric patients including children, adolescents and teenagers" needs to be increased.[49] At January 2026, only 15 per cent of highly innovative, US approved cell and gene therapies were registered in Australia. Even where these medicines do come to Australia, the delay for medicines listed on the Life Saving Drugs Program from 2016–2025 was just under 4 years.

The 2026 Federal Budget was characterised by the Government, as strengthening intergenerational wealth,[50] yet its priorities ring hollow for families of children with rare diseases who remain locked out of lifesaving medicines. When access to treatment is determined not by need but by cost or delay, the promise of fairness between generations is undermined at its core. True intergenerational equity should mean ensuring every child has the chance to live, grow, and contribute to the future we claim to be investing in.

Oncology:
The missing new medicines for Diffuse Large B-Cell Lymphoma (DLBCL)

Diffuse large B-cell lymphoma (DLBCL) is a rare, aggressive and life-threatening type of non-Hodgkin lymphoma that develops from the B-cells in the lymphatic system. For the last 20 years, chemoimmunotherapy has been the only treatment option for advanced stage disease with about 40% of patients experiencing a relapse within 2 years.[51, 52]

Optimising initial treatment regimens for patients with DLBCL in settings with curative intent (i.e. first-line) is of critical importance due to the substantial impact on the disease outcome by allowing patients to avoid disease relapse or progression[53] and limiting the psychosocial impact of the disease and fear of recurrence.

Polatuzumab vedotin is a targeted therapy used together with other cancer medicines to treat DLBCL. For certain patients it can reduce the risk of progression and avoid the need for costly subsequent therapies

Polatuzumab vedotin is reimbursed in over 30 countries [54], recommended by multiple overseas guidelines and is considered the standard of care in the United Kingdom, European Union and United States.[55] Notably, patients in the UK have been receiving subsidised access to this drug since September 2020 and in the first-line setting since January 2023.

In Australia, more than 2000 patients are diagnosed with DLBCL each year.[56] Unfortunately, eligible patients are unable to receive subsidised access to polatuzumab vendotin via the PBS. Over the past six years, since it was first registered by the TGA in 2019, three submissions have been made to the PBAC, requesting that polatuzumab vedotin be made available on the PBS for DLBCL patients. While a positive PBS recommendation was finally provided in March 2025 [57], the assigned conditions made a PBS listing unviable for Roche.

The cost-effectivess threshold demanded of Roche is exceptionally low ($15,000 to <$25,000 / quality adjusted life year gained) – about half of what the Australian Government normally accepts for similar cancers and about one-third of what is routinely accepted by NICE in the UK. By setting the bar this low, the system is fundamentally undervaluing the benefits this medicine can offer Australian lymphoma patients.[58]

Without access to polatuzumab vendotin, Australian patients miss out on an important treatment that could prevent relapse and avoid or delay costly later-line treatments.

2,000

Australians diagnosed

With Diffuse Large B-Cell Lymphoma each year

What patients are saying:

Sharon Millman, Lymphoma Australia

"At Lymphoma Australia, we see the devastating impact of relapse every day. Patients tell us they would do almost anything to avoid returning to treatment, extended hospital stays and the uncertainty that follows. When high-quality evidence and international guidelines support a therapy that can reduce the risk of relapse for eligible patients, Australians should have the same opportunity to benefit as patients in comparable healthcare systems."

What clinicians are saying:

Professor Michael Dickinson

"Diffuse Large B-Cell Lymphoma (DLBCL) is the most common aggressive lymphoma, and although about 2/3rds patients can be cured with chemotherapy, if it relapses, treating the relapse is much more difficult, far more intensive for the public health system, and much less likely to be successful than treating the patient properly in the first line.

Patients with moderate and high risk should have access to polatuzumab vedotin, according to USA, Canadian, British and European guidelines. It reduces the risk of relapse by about a quarter, and in some patients the benefit may be much greater. It is difficult to explain to patients that had they been diagnosed in countries similar to Australia, they would receive this treatment. Many of my patients choose to self-fund, at great expense. It's also upsetting to see patients relapse having not received a global standard treatment, and to know that the personal impact of relapse on patients and the costs of treating with treatments such as CAR-T cell therapy to the community are very high."

Polatuzumab vedotin

is reimbursed in over 30 countries

Application of the lowest cost comparator and its impact in the antirheumatic drugs (bDMARD) class.

The current Pharmaceutical Benefits Advisory Committee guidelines state "Where there is more than one comparator, the main comparator should be the therapy that prescribers would most likely replace with the proposed medicine."[59] Comparator selection is intended to ensure therapies subsidised under the Pharmaceutical Benefit Scheme are assessed against existing available therapies in terms of whether they deliver additional clinical benefit or cost savings for the healthcare system. However, over time, comparator selection has increasingly defaulted to the lowest cost comparator (LCC), regardless of whether this reflects alignment with real-world clinical practice.

The use of LCC in health technology assessment means that, where a new medicine has not been demonstrated to have a significant improvement over existing therapies, the price of that new medicine is benchmarked against the cheapest clinical alternative, rather than the therapy most used in clinical practice. Referring back to the earlier smart phone example, it means a new medicine will be priced in comparison to the cheapest in class, often older, off-patent medicines, rather than the therapy it is most likely to replace – a perverse use of the policy for cost containment purposes.

A recent survey showed 80% of innovative pharmaceutical companies reported experiencing comparator substitution in the past five years, and in 100% of those cases the substitution was to a lower-cost alternative.

The consequences extend beyond pricing. In that survey 100% of sponsors who did not proceed to price negotiations after a positive PBAC recommendation cited the lowest cost comparator as a significant factor, and 95% of respondents agreed that the lowest cost comparator approach undervalues clinical innovation. 90% also said that comparator selection directly affects the timing of Australian launch or commercial decisions.[60]

A prime example of the application of the lowest cost comparator issue is in immunology, and the impact on the biological disease-modifying antirheumatic drugs (bDMARD) class. The bDMARD class — used to treat conditions including rheumatoid arthritis, psoriasis, and ulcerative colitis — are in widespread use. In 2015, infliximab (a bDMARD medicine) moved to the F2 formulary following the entry of the first comparative biosimilar, triggering a substantial price reduction. After this, the PBAC began applying the lowest cost comparator to new bDMARD listings, using the now-cheaper infliximab as a price anchor for the bDMARD class, rather than comparing medicines to the most clinically relevant alternative. This was exacerbated by reference price flow-on reductions, which are applied across entire classes of medicines when new products launch, further eroding value and establishing an even lower floor price.

As the next few examples demonstrate, the consequence of this decision is that manufacturers are deciding to not launch medicines due to price. This leaves patients with immunological conditions such as arthritis, atopic dermatitis or inflammatory bowel disease waiting or unable to access effective therapies that are available overseas. In fact, in inflammatory bowel disease, Australian patients do not have access to an entire class of innovative medicines, the interleukin-23 inhibitors, due to issues relating to comparator price. For many of these patients who have failed multiple prior lines of therapy, the range of available treatment options, and the timeliness of access to those options, matters profoundly to quality of life and disease outcomes.

Comparator selection was explicitly identified as a priority area requiring reform as part of the HTA review in 2024. The reforms proposed include aligning PBAC Guidelines with clinical reality, transitioning to a model where the comparator is agreed early in the submission pathway, and introducing flexible approaches for medicines addressing high unmet clinical need.[61]

Dermatology:
Lebrikizumab and Abrocitinib for the treatment of Atopic Dermatitis

Atopic dermatitis is a chronic inflammatory form of eczema. It is estimated to impact up to 2.8 million Australians.[62] Medicines recently reimbursed for the treatment of severe atopic dermatitis include dupilumab and upadacitinib. However, other countries have funded lebrikizumab, abroctinib and corticosteroids which are effective for patients with symptoms that do not respond to older treatments similar to dupilumab and upadacitinib.

Lebrikizumab is a monoclonal antibody that selectively neutralises IL-13, directly blocking one of the most important pathways for the development of atopic dermatitis.[63] Abrocitinib is a selective JAK-1 inhibitor that blocks signalling inside cells and prevents inflammatory signals from activating disease processes.[64]

Both of these medicines were recommended for listing on the PBS, but the recommended price combined with a restrictive risk sharing agreement did not appropriately value the medical innovation delivered by the treatments and effectively capped the number of patients that would be able to access the treatment. Australian evidence suggests effective treatment of severe atopic dermatitis would likely have significant economic benefits. In a survey of patients, respondents with severe atopic dermatitis reported around 4 to 5 days per month with reduced work hours due to the disease. In addition to this, approximately 70% of respondents reported their skin condition impacted their work or study.[65] However, these productivity impacts are not considered adequately by the PBAC.

What patients are saying:

Angelika Thiew, CEO, Eczema Association Australasia

"For people living with severe atopic dermatitis, access to new, funded treatment options can be life changing. Severe eczema is not "just a skin condition"; it can affect sleep, mental health, school, work, relationships and the wellbeing of the whole family.

The Eczema Association hears every day from people who are exhausted, distressed and searching for options when existing treatments are not enough or are not suitable for them. When effective new medicines are available overseas but remain out of reach for Australian patients, families are left feeling that they are missing out on hope, choice and the opportunity for better quality of life.

Funded access to treatments such as lebrikizumab and abrocitinib would give eligible patients and their clinicians more options to tailor care, reduce disease burden and improve daily functioning. Australians living with severe atopic dermatitis deserve timely and equitable access to medical advances that may help them live, study, work and participate more fully in life."

Melanie Funk, Managing Director, Eczema Support Australia

"Despite recent medical innovations, Australians living with severe eczema continue to face significant unmet need. Access to advanced treatments remains limited, particularly for children, and some innovative eczema medicines have not progressed through the Australian reimbursement pathway.

When effective treatment options are unavailable, the impact extends far beyond the skin, affecting sleep, mental wellbeing, education, employment and overall quality of life. Timely access to appropriate therapies remains an important issue for the eczema community."

The psychosocial impacts of immunological conditions

Chronic skin and immunological conditions (e.g., inflammatory bowel disease, atopic dermatitis) in Australian adults have significant psychosocial impacts beyond physical symptoms.[66,67]

They are linked to high rates of anxiety, depression, and psychological distress. Visible symptoms or unpredictable flare-ups can lead to stigma, embarrassment, low self-esteem, and poor body image.

These conditions often reduce quality of life by limiting work, social participation, and relationships, with many people experiencing isolation and reduced functioning. Overall, psychosocial factors like stress and low social support can worsen both mental wellbeing and disease outcomes, highlighting the need for holistic care.[68]

Australia's current reimbursement framework gives insufficient weighting to psychosocial needs. As a result, medicines that help address these factors are often undervalued, despite clear evidence that psychological distress and social factors significantly affect outcomes and disease management.

Immunology:
Risankizumab for the treatment of Crohn's Disease

Of people under active management for Inflammatory Bowel Disease, approximately half have Crohn's Disease. In 2025, it was estimated IBD imposed a cost on Australian society of $7.5 billion. Australia has among the highest incidence of IBD globally, and this is growing at the fastest rate among peer countries.[69]

Risankizumab selectively inhibits the p19 subunit of interleukin-23, a key immune signalling protein that contributes to chronic gastrointestinal inflammation. It is registered in Australia for the treatment of moderate to severe Crohn's disease in adult patients, who have not responded to or cannot tolerate either conventional or biologic therapy. It is not currently reimbursed in Australia, however is publicly reimbursed in other countries including Canada and the United Kingdom, as well as across Europe and Asia.[70]

In Australia, the PBAC recommended risankizumab be listed on the PBS on the basis that it cost no more that the least costly medicine that was available to treat Crohn's disease.[71]

The least costly medicine was an old, off-patent medicine which had gone through significant price reductions over time. It did not progress to PBS listing and is therefore not available as a reimbursed option for Australians.

Immunology:
Guselkumab for treatment of Crohn's Disease

Crohn's disease is a chronic inflammatory bowel disease that can cause persistent gastrointestinal symptoms, progressive bowel damage, hospitalisation and major impacts on quality of life. Australia has one of the highest rates of inflammatory bowel disease (IBD) globally, with an estimated cost to Australian society of $7.5 billion.[72]

Guselkumab is an interleukin-23 (IL-23) inhibitor for adults with moderately to severely active Crohn's disease who have had an inadequate response, lost response or intolerance to conventional or biologic therapy. It is approved in major international markets and reimbursed in 29 countries. In Australia, guselkumab received a positive PBAC recommendation for PBS listing in 2025, but the recommendation was contingent on pricing linked to a biosimilar medicine that had undergone significant price erosion. It has therefore not progressed to PBS listing. A resubmission for guselkumab is being considered by the PBAC in 2026 as the sponsor continues efforts to secure PBS reimbursement.

This case demonstrates a broader problem with IBD medicines access in Australia. When PBAC reimbursement decisions benchmark new therapies against older biologics that have already lost substantial value through price cuts, it fails to recognise innovation and patient benefit. The result is that medicines funded in comparable countries can remain unavailable to Australian patients, despite demonstrated clinical benefit.

Immunology:
Mirikizumab for the treatment of Ulcerative colitis

Ulcerative colitis (UC) is a chronic inflammatory bowel disease characterised by continuous mucosal inflammation of the colon. The clinical course of the disease consists of periods of exacerbation (flares) and remission.

The estimated prevalence of ulcerative colitis in Australia is between 130 and 300 people per 100,000. This represents one of the highest rates in the world.[73]

Mirikizumab is approved for the treatment of moderate to severe ulcerative colitis in adults who have had an inadequate response to or were intolerant to conventional therapy. It is currently approved in 44 countries.[74]

It is not currently reimbursed in Australia, despite being approved by the TGA for moderate to severe UC in September 2023 and for moderate to severe Crohn's disease in June 2025. More recent studies show that that mirikizumab can both induce and sustain remission over multiple years.[75]

The PBAC recommended mirikizumab be reimbursed in July 2023, provided the price of the medicine match eight other alternative therapies already available, some of which have been available for more than a decade in Australia and now genericised, with a price reduction over time of up to 60%.

As a result the manufacturer decided that this was not commercially viable to proceed and as such, the product is not currently available for use in Australian patients.

Australia has one of the highest rates of ulcerative colitis in the world

Between 130 and 300 people per 100,000

What patients are saying:

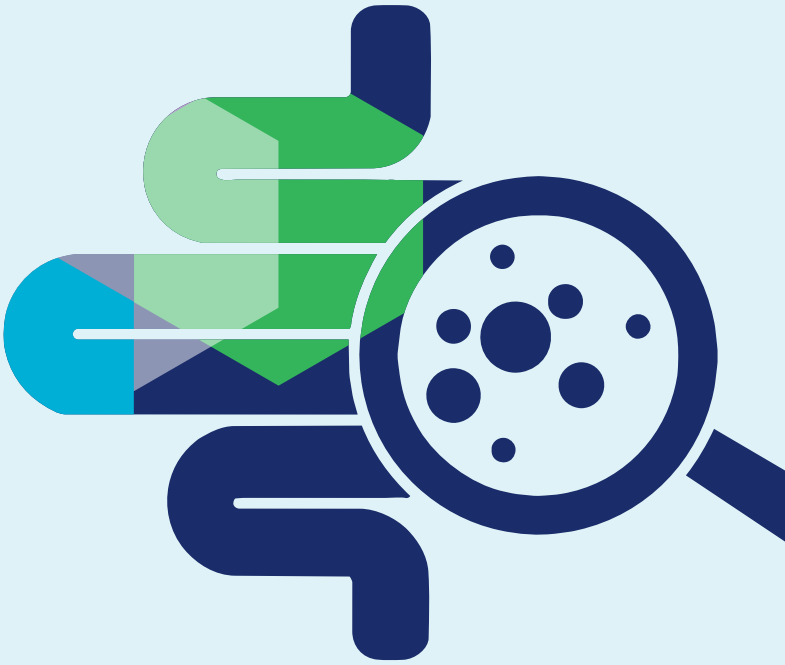
Leanne Raven, CEO, Crohn's and Colitis Australia

"We are increasingly hearing from people who have exhausted all PBS funded therapies and need access to novel therapies to avoid severe symptoms, side effects, or surgery to remove their bowel. It is frustrating that a class of drugs available in other countries is not listed on the PBS for IBD despite positive recommendations.

People awaiting this class of drugs are aware of the disparity, many are struggling to keep up with their work or study, and the exasperation adds to the mental health burden of these diseases and can impact on their ability to work and earn an income."

State of the Nation in Inflammatory Bowel Disease shows Australia's access is behind comparable countries

Crohn's and Colitis Australia's State of the Nation in IBD in Australia shows that despite advancements in the treatment of inflammatory bowel disease with the advent of biologic and JAK inhibitor medicines, a number of new therapies that are available in other developed nation peers are not subsidised in Australia. As a result, patients may have to wait for significant periods before gaining access to affordable treatment options, or they may face high out-of-pocket costs if the medications are not covered by the PBS. For paediatric patients, this situation was even worse with some medications taking more than seven years to be made available for children, following approval for adult populations.[76]



	AUSTRALIA		CANADA		ENGLAND	
Therapy	**CD**	**UC**	**CD**	**UC**	**CD**	**UC**
Adalimumab	✓	✓	✓	✓	✓	✓
Infliximab	✓	✓	✓	✓	✓	✓
Ustekinumab	✓	✓	✓	✓	✓	✓
Vedolizumab	✓	✓	✓	✓	✓	✓
Upadacitinib	✓	✓	✓	✓	✓	✓
Risankizumab	✗	✗	✓	✗	✓	✓
Mirikizumab	✗	✗	✗	✓	In Development	✓
Tofacitinib	✗	✓	✗	✓	✗	✓
Golimumab	✗	✗	✗	✓	✓	✓
Etrasimod	✗	✓	✗	✓	✓	✓
Ozanimod	✗	✓	✗	✓	✗	✓

"More timely approval processes for pharmaceuticals and other medical technologies would help ensure that the diffusion of new treatments remains a positive contributor to productivity growth."[77]

Vaccines

There has been a concerning decline in Australia's childhood vaccination rates since 2020.[78] Australia's adult immunisation schedule is assessed as "very limited"; so expanding this schedule is judged to be a clear opportunity to improve Australia's health.[79] In 2019 Australia had a higher rate of vaccine-preventable deaths than comparable countries – 3.4 per 100,000 population, compared with a significantly higher rate than Canada (2.7 vaccine-preventable deaths per 100,000 people), France (2.9), Italy (1.5), South Korea (3.1) and the UK (2.4).[80]

Currently, there is a 1,375–day delay between a vaccine being deemed "safe and effective" by the TGA and Australians receiving subsidised access to it, almost four years.[81]

Meningococcal B

A good example is limited access to a Meningococcal B Vaccine in Australia. In 2002, there were 210 confirmed cases of Meningococcal B and 162 confirmed cases of Meningococcal C in Australia. In 2003, the Vaccine for Meningococcal C was introduced.[82] By 2022, there were 100 confirmed cases of Meningococcal B and zero confirmed cases of Meningococcal C.[83]

Up to 10 per cent of people with Meningococcal disease may die; around 25 per cent suffer serious long-term disabilities (including brain damage, deafness or loss of limbs).[84] One severe case of Meningococcal B is estimated to create cost to government of $10 million (including: educational assistance, disability support, direct support and lost tax revenue.[85]

ATAGI (Australian Technical advisory Group on Immunisation) strongly recommends Meningococcal B vaccination for a significant share of the population: (1) Infants and children under 2 years, (2) Healthy adolescents aged 15–19 years, (3) Individuals aged 15–24 years living in close quarters and (4) Aboriginal and Torres Strait Islander peoples aged 2 years – 19 months.[86]

Individuals who are able can choose to self-fund the vaccine. The governments of South Australia, Queensland and Northern Territory have delivered their own immunisation programs for infants and adolescents and Tasmania now funds vaccination for infants. Victoria intends to offer immunisation for year 10 students in 2027. This means access is now dependent on Australians' income and postcode.

10%

Up to 10% of people with Meningococcal disease may die

Around 25 per cent suffer serious long-term disabilities

Despite more than four submissions for inclusion of the National Immunisation Program (NIP), the vaccine sponsor has been unsuccessful in securing access for all Australian children to the Meningococcal B vaccine.

What patients are saying:

Catherine Hughes AM, Founder and Executive Director, Immunisation Foundation of Australia

"Every year, Australian families must decide whether to pay hundreds of dollars out of pocket to protect their child from a disease that strikes without warning and can kill within hours. Parents in the UK and New Zealand don't face that choice: meningococcal B vaccination is funded for their children as a matter of course. Australian parents are left navigating a gap that comparable countries closed years ago.

A meningococcal B vaccine has been before the PBAC four times. The evidence hasn't changed. The disease hasn't changed. What has kept this vaccine out of reach for ordinary Australian families is a reimbursement system that uses an outdated methodology to calculate the value of vaccines with long-term population benefit and consistently

arrives at the wrong answer. One in three survivors of meningococcal B disease will live with lifelong impairment. The Immunisation Foundation of Australia has supported families who have lived through this. Funded, universal access would mean fewer of those calls, fewer of those funerals, and fewer families asking why their child died from something that was preventable."

What clinicians are saying:

Karen Quick, Chief Executive Officer—Meningitis Centre Australia

"The inequality of the Meningococcal B vaccine to Australian families due to cost and location is currently at an all-time high. Five states currently provide this vaccination, two only for babies or teens, not both, causing confusion. In WA and NSW families are now forced to choose between food on the table or protecting their children from a disease that kills in under 24 hours while children across their borders are protected. Why?

In our recent survey of 22,000 parents, 97% strongly indicated they wanted their children vaccinated, and 87% indicated while wanting to vaccinate their children, they simply could not afford it. A review has been going on for over five years of our antiquated PBAC system. The PBAC recently showed no flexibility or looked at socio-economic benefits towards introducing the Men B vaccine. Furthermore, the government commits no money to raising awareness – all this leads to even more deaths and disability due to cost, but what cost do you put on even one life? Do the maths, hear the stories, listen to the parents crying out for their kids to be saved from this disease and you'll see it's common sense because every second and every child's life counts – vaccines should be in arms not on shelves!"

Respiratory syncytial virus (RSV)

Respiratory Syncytial Virus (RSV) imposes a significant burden of disease on Australians. In 2022, there were 27,900 hospitalisations and 58 deaths in Australia caused by disease.

Amongst infants and babies (Australians under 1yo) the hospitalisation rate more than doubled from 1999 to above 30,000 per million in 2022.[87]

Since early 2025, all pregnant women in Australia have been eligible for a free maternal RSV vaccine under the NIP.

In May 2026 access to a second RSV vaccination was provided under the National Immunisation Program to cover adults aged 75 and over as well as Aboriginal and Torres Strait Island adults aged 60 and over.

The Australian Immunisation Handbook recommends RSV vaccination for specific groups [88] including:

- pregnant women at 28 to 36 weeks of pregnancy
- people aged 75 years and older
- Aboriginal and Torres Strait Islander people aged 60 years and older
- people with medical risk factors for severe RSV disease aged 60 years and older.

Yet despite this recommendation there are no RSV vaccines funded through the National Immunisation Program for people with medical risk conditions and adults aged 60–74 years.

What patients are saying:

Catherine Hughes AM, Founder and Executive Director, Immunisation Foundation of Australia

"RSV is one of the most common reasons older Australians are hospitalised each winter, and the people who fare worst are usually those already living with heart disease, chronic lung conditions or diabetes. Vaccination is recommended for them from age 60, but funding has not kept pace. Unless someone is aged 75 and over, or an Aboriginal or Torres Strait Islander adult aged 60 and over, the vaccine their doctor recommends is one they pay for themselves. Some cannot. Families have told us they would go without a week's groceries to afford it. The people most at risk of severe RSV are too often the ones asked to fund their own protection. Extending funded access to the older adults already recommended to have it would prevent hospitalisations we can see coming."

RSV

Respiratory Syncytial Virus

In 2022, there were 27,900 hospitalisations and 58 deaths in Australia caused by disease

Is it time for a prevention revolution?

Prevention is an essential component of an effective health system, whether targeted at individuals or populations. Yet only a small fraction of health spending is allocated to prevention activities, currently less than 2% of the total health budget [89]. Despite this, investing in prevention can reduce hospitalisation, tackle chronic disease, and ease pressure on the entire healthcare system. The Productivity Commission, in its fifth Intergenerational Report Delivering quality care more efficiently, recommended establishing a national framework to support government investment in prevention and early intervention.[90]

A modern prevention agenda should encompass a broad mix of interventions—including public health measures, vaccination programs, and the appropriate use of innovative medicines—to reduce both the onset and progression of disease. Vaccines remain one of the most effective and cost-efficient preventative tools available, reducing the burden of infectious disease and increasingly playing a role in preventing complications in at-risk populations. At the same time, advances in medicines are expanding opportunities to intervene earlier in the disease pathway and address key risk factors before they lead to more serious health outcomes.

Chronic diseases are Australia's greatest health challenge, with 61% of Australians (15.4 million people) living with at least one chronic condition and 38% (9.7 million people) living with multiple conditions. These diseases account for 91% of preventable deaths and 85% of years lost due to ill health, placing significant pressure on the healthcare system and economy. Many chronic diseases share underlying risk factors and causal pathways, creating complex multimorbidity patterns that require coordinated and sustained prevention efforts.

Obesity is a key example of a modifiable risk factor that contributes to multiple chronic conditions, including type 2 diabetes and cardiovascular disease. The number of people living with obesity has increased significantly over the past decade, with 6.3 million Australian adults currently affected. Addressing obesity and other risk factors requires a combination of behavioural, public health, and clinical interventions. Emerging therapies, including innovative obesity medications, demonstrate how medicines can support prevention by reducing disease progression and associated complications when used appropriately alongside lifestyle interventions.

By reducing the incidence, severity, and long-term complications of disease, these interventions not only improve individual health outcomes but also help prevent downstream healthcare costs and demand, reinforcing the critical role of prevention in building a more sustainable health system.[91]

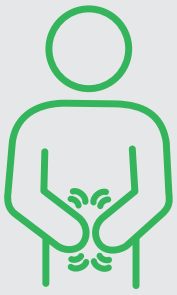
For example, a recent Gallup survey demonstrated the obesity rate in the United States (where innovative medicines are available to treat obesity) had fallen from a high of 39.9% three years ago to 37% late last year.[92] This may not sound like a lot but that equates to 7.5 million fewer Americans classified as overweight. In addition, expanding access to vaccines—through inclusion on the PBS where appropriate, or broader coverage under the National Immunisation Program—offers a highly cost-effective way to prevent illness and reduce future healthcare utilisation. Together, these measures highlight the importance of a balanced prevention strategy that invests across the full spectrum of interventions.

Oncology:
Radiopharmaceutical therapies for the treatment of prostate cancer

Prostate Cancer is the most commonly diagnosed cancer in Australia, with 24,000 males diagnosed in 2022 alone.[93]

Radiopharmaceutical therapies harness radioactive isotopes to detect and destroy cancerous cells while sparing healthy tissue. TGA registered therapies are now available for prostate cancer (lutetium Lu 177 vipivotide tetraxetan (Lu-PSMA-617)) and neuroendocrine tumours (Lutetium (177Lu) oxodotreotide (177Lu]-DOTA-TATE)). Lu-PSMA-617 was approved for use in various countries from 2022 onwards.[94] For example, patients can access the medicine via full public reimbursement in most Canadian provinces, Japan, Germany, France Italy and Belgium.

Most commonly diagnosed cancer in Australia



Prostate Cancer. With 24,000 males diagnosed in 2022 alone

Unfortunately, the experience in Australia has been quite different. While the sponsor was undergoing TGA assessment for Lu-PSMA-617, a process that ensures the quality, safety, and efficacy of medicines made available to Australian patients, an application was made to the Medical Services Advisory Committee (MSAC) seeking public reimbursement for a generic version therapy that had not been assessed by the TGA, and which relied upon Lu-PSMA-617 Phase III trial data, not its own. Despite intellectual property concerns and the absence of regulatory evaluation, MSAC positively recommended and the Government subsequently funded MBS item numbers to subsidise access to the entire class of therapies, including both the generic version and Lu-PSMA-617.

This creates several problems for patients who need to access these medicines:

1. **Significant private costs –** Patients face substantial out of pocket costs, as the reimbursement paid via the MBS is only a small part of actual costs and operates as a rebate after full payment upfront
2. **Geographic access barriers –** Patients can only access the medicine in private clinics or in those public hospitals that allow doctors to charge private costs. This adds to the out-of-pocket costs faced by patients and means patients in many areas cannot access the medicine. This is particularly problematic in States like South Australia, where public hospitals cannot charge patients out of pocket and are restricted from providing private options unless they are also available publicly.

Medicines Australia urges the Government to ensure, as a pre-requisite to reimbursement, Radiopharmaceutical therapies should be subject to TGA assessment.

What patients are saying:

Anne Savage, Chief Executive Officer, Prostate Cancer Foundation of Australia

"Every Australian with advanced prostate cancer deserves timely access to the best available treatment, regardless of where they live or their capacity to pay. Innovative therapies such as Lu-PSMA-617 are changing the outlook for men with advanced disease around the world, yet too many Australians continue to face unnecessary delays, inequitable access, and significant out-of-pocket costs.

For men living with advanced prostate cancer, time matters. Every delay can mean the difference between receiving a treatment that extends life and improves quality of life, or missing that opportunity altogether. Their families should not have to watch effective therapies remain out of reach when they are available to patients in comparable countries.

Australia has a proud history of delivering world-class cancer care, but our systems must keep pace with scientific progress. Patients deserve faster, fairer access to proven innovations so they can benefit from the latest advances in treatment when they need them most."

Infectious disease: Novel antibiotics to combat the threat of anti-microbial resistance (AMR)

Antimicrobial resistance (AMR) is a global health emergency, recognised by the World Health Organization (WHO) as one of the top ten public health threats facing humanity. AMR occurs when microbes such as bacteria and fungi become resistant to the antimicrobial drugs which once killed them. Over 5,200 Australians die from AMR-associated causes each year and this number is only set to increase as the threat of AMR grows.

To combat the threat to AMR, new antimicrobials are needed. However, the current reimbursement system in Australia does not recognise the true value of effective antimicrobials.[95]

Missing novel antibiotics means that in clinical practice; in order to save lives, Australian doctors are having to make applications to access antibiotics not registered for use in Australia via special access schemes.

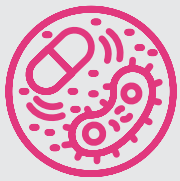
A recent study examined how quickly new antibacterial drugs become available in Australia after international approval and assessed the use of unregistered antimicrobials in clinical practice over a six-year period (2018 to 2024). Using hospital utilisation data and Special Access Scheme (SAS) records, the study found over 36,000 requests for unapproved antimicrobials were made (almost 500 a month), with more than a quarter of these requests used for critically ill patients.[96]

AMR is a global problem. Many countries are investigating how to assess the value of novel antimicrobials to include the broader value they bring to society. In the UK, the Government has partnered with industry to pilot a model of reimbursement that will decouple the revenue of an antimicrobial from the volume sold and base it instead, on the antimicrobial's value to the NHS and wider public health. This means companies will be paid for antimicrobials based on how valuable they are rather than by the quantity being used or sold. This pilot will also help to reduce the financial uncertainty in antimicrobial research and elevate incentives to develop novel anti-biotics.[97]

AMR

Antimicrobial Resistance

Over 5,200 Australians die from AMR-associated causes each year and this number is only set to increase as the threat of AMR grows



This subscription model has also been endorsed by the HTA Review, which included a number of recommendations to incentivise the development of novel antibiotics and to create new pathways for their reimbursement to address the 'broken market' that currently exists. However, action on these recommendations is still outstanding and for consecutive Federal Budgets, there has been no new funding for AMR initiatives. This is cause for concern.

What clinicians are saying:

The Australasian Society for Infectious Diseases

The Australasian Society for Infectious Diseases (ASID) released a statement in 2024 which called for the Government to consider novel reimbursement approaches to new anti-infectives. "The loss of effective antimicrobials due to overuse and misuse is putting lives at risk and threatens to undo decades of progress in modern medicine. ASID calls on governments to adequately invest in interventions that prevent unnecessary use and misuse of antimicrobials, as well as exploring innovative treatments for the superbugs that are already affecting our communities."

The economic incentives for pharmaceutical companies to invest in the development of new antimicrobials are limited, primarily because of the high cost of research and development, and the relatively small market for these drugs once they are approved.

In response to this gap, ASID advocates for the creation of a subscription-based funding model for novel antimicrobials in Australia and New Zealand, where governments would commit to paying a fixed fee rather than pharmaceutical companies relying on traditional volume-based sales. This model, inspired by a successful model already piloted in the UK, would provide a sustainable mechanism for ensuring the availability of new and highly effective antimicrobials to treat drug-resistant infections, while also ensuring fair access for patients who need them most. This approach would help balance the need for innovation with responsible use of new drugs. It would provide reliable access to some of the most needed new antibiotics, where access is currently difficult, delayed in inequitable."

The bitter pill for patients and clinicians to swallow

The absence of newer medicines that can more effectively treat a wider range of conditions from the Australian market is a major problem.

What were once isolated delays are becoming a broader pattern, resulting in a widening gap between the therapies available to Australian patients and those accessible in comparable countries.

Direct comparisons show Australia is funding a smaller and declining share of new medicines, compared to other nations and to complicate matters, even when medicines are eventually subsidised it is after considerable time, or the approval is laden with restrictions and provisions. Patients are missing out or waiting longer for therapies that have become part of standard care elsewhere.

For patients, delayed or restricted access to innovative therapies can result in greater reliance on older, less effective treatments. This can result in a higher burden of disease and major impacts on their length or quality of life.

For clinicians, the absence of new therapies constrains their ability to deliver best-practice care. Treatment decisions are increasingly shaped by limitations rather than clinical evidence, with clinicians relying on older standards of care, suboptimal therapies, or in the most serious cases lacking appropriate options for patients who do not respond to existing treatments.

The fact consequences are now being felt not just at a system level, but in the lived experience of patients and clinicians is a bitter pill to swallow.

For Australia's broader health system, these issues are reducing our attractiveness as a launch destination for new medicines. Companies are increasingly sequencing Australia later in global launches—or not at all. Over time, these factors will also diminish Australia's role in global pharmaceutical innovation, including participation in clinical trials, early access programs, and associated investment.

However, this trajectory is not fixed. By addressing access barriers and modernising the way new medicines are evaluated and funded, Australia has a clear opportunity to reverse this trend. Doing so would not only close the widening gap with comparable countries but also strengthen the health system, restore confidence among clinicians and patients, and re-establish Australia as an attractive environment for innovation, investment, and early access to cutting-edge therapies.

What needs to change?

Addressing the rising gap in treatment availability will require targeted policy action to enable Australians to access newer medicines.

Two priorities emerge as critical. Future budgets need provisions for real growth of the PBS over time, to recognise new and innovative medicines and vaccines as an investment in the health of Australians, and in turn, the health of our economy. Secondly, HTA policies and associated methods should be reconsidered and updated, to ensure they can accommodate the next generation of breakthrough medicines.

Increased net investment in F1 (innovative) medicines in the PBS

Sustained underinvestment in innovative medicines has contributed to Australia's new position as a later-launch market. Medicines Australia has consistently highlighted the need for greater and more predictable investment in innovative therapies through the PBS.

This is not simply a funding question—it is about ensuring the PBS can continue to deliver on its core objective of timely, equitable access to effective treatments. Greater investment would support:

- Faster and broader access to new therapies
- Improved alignment with international standards of care
- A more competitive environment for global launch decisions

Strengthening the PBS through increased investment in F1 innovative medicines is necessary to ensure it remains fit for purpose for the health needs of Australians.

Timely implementation of the HTA Review

The Health Technology Assessment (HTA) Review, completed in 2024, provides a comprehensive roadmap for reform. It found that Australia's HTA system is outdated, overly complex, and contributes to delays in patient access, and made more than 50 recommendations aimed at improving timeliness, equity, and the ability to assess modern therapies.[98]

Medicines Australia and broader stakeholders have emphasised that the value of the Review lies not only in its recommendations, but in their timely and coordinated implementation.

This has not happened.

Given the scale of consultation and the strong public, clinician, and patient interest in this Review, there is also a clear need for greater transparency in how implementation decisions are being progressed. The HTA Review was developed through extensive stakeholder engagement, and its outcomes carry significant implications for patients, clinicians, and the broader health system.

Implementing these reforms in a clear, sequenced, timely and transparent manner is essential to restoring confidence in Australia's access pathway and ensuring patients and clinicians benefit from innovation as it emerges – rather than being confronted with the bitter pill of missing out on breakthroughs that could significantly improve their lives and the lives of their family and friends.

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