May 2023

Medicines Australia Whitepaper

Funding Innovative Medicines

Medicines Australia

The report has been commissioned by Medicines Australia and prepared by Biointelect and Shawview Consulting.

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Executive summary

Australia has a unique opportunity to reform the way it evaluates and funds medicines and vaccines, to ensure that Australians are among the first in the world to access new technologies. This is a policy objective of the Australian Government that is shared by the medicines industry[1]. Reforms to Australia's Pharmaceutical Benefits Scheme (PBS) and National Immunisation Program (NIP) are needed so that Australian patients can access medicines and vaccines like other similar countries.

Through the COVID-19 pandemic, Australians understand the importance of being first in line to gain access to medicines and vaccines. A renewed commitment to invest in new medicines and vaccines, in line with these expectations, will help to improve the health outcomes of Australians, underpin the post-pandemic development of Australia's healthcare system, and drive productivity and growth in the economy in the 21st century.

Australia is at an inflection point

Australia's system of evaluating and funding new medicines and vaccines through health technology assessment (HTA) is at a critical juncture, or an inflection point. The 2022 update of the Australian Government's National Medicines Policy (NMP) confirmed a commitment that: *All Australians, regardless of where they live, who they are and the health condition they have, must have timely, safe and reliable access to effective medicines and medicines-related services to maintain their health and wellbeing at a world class level.*[2] Today, we have a unique opportunity to redesign our healthcare system for the adoption of new, cost-effective medicines and vaccines, now and into the future.

A unique set of circumstances currently provides an opportunity to expand the scale, scope and effectiveness of the PBS and NIP for future generations:

- Australia is on the cusp of adopting a range of new medical technologies that give new opportunities to provide 21st century health care to Australians. Medical technologies such as personalised medicine, cell and gene therapies and mRNA technologies have the potential to provide new treatments, cures and preventative strategies for a range of diseases for the Australian community.
- The product of ground-breaking scientific research over several decades, Australia will need to ensure that the PBS and NIP are ready to accommodate and embrace such new medical technologies in the future.
- The Australian Government, working constructively with the pharmaceutical industry over the past two decades, has reformed the PBS and NIP to ensure their financial sustainability. These reforms have been effective in constraining growth and have resulted in little or no

[1] *Under the Commonwealth of Australia – Medicines Australia Strategic Agreement in relation to reimbursement, health technology assessment and other matters (p. 10), the Australian Government and Medicines Australia have reiterated their shared policy goal of "maintaining Australia as a first-launch country to build on Australia's status as a world leader in providing patients access to affordable healthcare" (https://www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2021/09/Medicines-Australia-Strategic-Agreement-2022-2027.pdf).*

[2] *Department of Health and Aged Care. 2022. National Medicines Policy 2022, https://www.health.gov.au/sites/default/files/2022-12/national-medicines-policy.pdf, accessed 22 February 2023.*

real growth in pharmaceutical expenditure over the last decade or more, despite an ageing population and growth in other areas of the health budget.

- For some time, the financial pressures on both schemes have led to a suite of emerging problems in how Australian governments evaluate and fund medicines and vaccines. These problems have grown to the point where today Australians wait much longer than their overseas counterparts for these technologies, or have to pay for them themselves out of pocket.

- Finally, the COVID-19 pandemic has demonstrably proven the broader social and economic value of medicines and vaccines. A recent analysis found that the rollout of COVID-19 vaccines had a positive impact on the Australian economy of AUD 181 billion, with the largest impacts in tourism (AUD 28 billion) and education (AUD 26 billion) exports and employment (142,000 jobs).[3] If there is one lesson from the pandemic, it is how critical it is to ensure that the Australian Government appropriately values and invests sufficiently in new medicines and vaccines. The pandemic proved that countries ignore the broader social and economic value of medicines, therapies and vaccines at their peril. Currently, Australia's evaluation and funding systems do not place sufficient weight on these broader benefits of medical technologies, resulting in undervaluing and, potentially, underinvesting.

The case for change

Throughout the pandemic, Australians knew the importance of being first in the queue to obtain new medicines and vaccines. They have shown they do not want to be last in the queue and do not want to wait more than their counterparts in other countries.[4]

There are many constraints in how Australia evaluates and funds new medicines and vaccines that have meant that Australians wait too long, compared to people in similar countries. Australians have either had to do without or, for those that can afford it, pay for these themselves through the private market while waiting for a government decision on funding.

Other countries have found ways to manage costs while ensuring that their people do not have to wait long periods of time to benefit from new medical technologies.

- Japanese policies aim to balance expenditure control with promoting innovation. All medicines are subject to price erosion during the patent protection period, although the Price Maintenance Premium (PMP), which is awarded to products that offer greater value to patients and the healthcare system, defers any price reductions until after patent expiry.

- Germany and Japan, among the fastest adopters of new pharmaceuticals in the world, both introduced HTA with the explicit intention of not allowing this to delay patient access. Funded access to new technologies is provided while HTA is being conducted. New

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[3] *Fox N, Adams P, Grainger D, Herz J, Austin C. The Value of Vaccines: A Tale of Two Parts. Vaccines. 2022; 10(12):2057. https://doi.org/10.3390/vaccines10122057.*

[4] *BioPharmaDispatch. 2021. "Exclusive: PM was forced to intervene on vaccine after months of inaction", 27 July, https://pharmadispatch.com/news/exclusive-pm-intervened-to-get-action-on-vaccine-procurement, accessed 25/9/2022; ABC News. 2021. "Shadow Health Minister Mark Butler criticises Australia's COVID-19 vaccine rollout, saying more options should have been secured", 11 April, https://www.abc.net.au/news/2021-04-11/labor-mark-butler-covid-vaccine-rollout/100061644, accessed 25/9/2022; ABC. 2021. "Shadow Health Minister Mark Butler on Insiders", Insiders, Video interview, 29 August, https://www.abc.net.au/news/2021-08-29/shadow-health-minister-mark-butler-on-including-12/13518880, accessed 25/9/2022.*

products are “automatically listed” in Germany upon receipt of marketing authorisation and are listed within 60-90 days of marketing authorisation in Japan.

- France provides early funded and access to new medicines before they receive marketing authorisation, in areas of high unmet need, including life-threatening and disabling conditions.
- England’s Cancer Drugs Fund and Innovative Medicines Fund provide funded access to new therapies under managed access agreements, where there is uncertainty about cost-effectiveness at the time of market entry. Products are typically re-reviewed after two years and may be re-priced or de-listed at that point.
- Data collection requirements are set out by HTA agencies in early and managed access in France and England, respectively, so that this may be utilised when the full evaluations of new therapies are conducted for routine funding in national health services.

A unique opportunity

While the reform of Australia’s PBS and NIP has been ongoing, a critical part of this work has not yet been completed. Today, Australia has a real opportunity to lock-in the benefits of two decades of PBS and NIP reforms and ensure that Australians do not have to wait any more for new medicines and vaccines. Cementing Australia’s position among first launch countries for new medicines and vaccines will require a detailed and strategic consideration of HTA policy and methods and, critically, an examination of how Australia compares to the world’s leading countries for patient access to medical technologies. Appropriate consideration of these aspects must aim to ensure that the PBS and NIP continue to align with the values and expectations of the Australian community.

The HTA Review is an opportunity to review and improve the policy, methods, assumptions and financial constraints used in the Government’s evaluation processes, to ensure that Australians are among the first in the world to access new technologies. As the PBS and NIP budgets are structurally stable, there is now scope to expand these programs with strategic policies that prioritise the early availability of new medicines and vaccines to Australians, while maintaining robust fiscal discipline in the programs.

Today, the Australian Government, industry, patient groups, other health stakeholders and the broader community have a unique opportunity to work together to secure the Government’s stated policy objective of ensuring that Australia is a “first-launch country” for new medicines and vaccines.[5]

The HTA Review provides a real opportunity to undertake reforms to the PBS and NIP, to ensure that Australians are at the front of the queue for future medicines, treatments and vaccines.

[5] *Department of Health. 2021. Strategic Agreement in relation to reimbursement, health technology assessment and other matters: Commonwealth of Australia, Medicines Australia, p. 10, https://www.pbs.gov.au/general/medicines-industry-strategic-agreement-files/MA-Strategic-Agreement-Signed.pdf, accessed 25/9/2022.*

Introduction

"A healthy economy depends on healthy people."

- *Anthony Albanese, Address to the National Press Club upon 100 days in government, 29 August 2022*[6]

Australia's HTA system is at a critical juncture for reform to set our healthcare system up for the adoption of cost-effective technologies, now and into the future. This paper aims to open a conversation about our HTA and funding policy settings; in particular, the value placed on medicines and vaccines by Australians. As we emerge from the COVID-19 pandemic and look to the future of precision medicine, it is timely to consider the role that HTA plays in funding decisions and policies that will ensure that Australian patients will have access to new technologies.

Australia's people are our most important asset, and the performance of our health system has a critical role in improving the health and ultimately, the lives, of Australians. Investments in healthcare represent investments in the wellbeing and prosperity of a nation, now and into the future. The COVID-19 pandemic demonstrated the obvious point that the health of a country's citizens fundamentally underpins the health of the economy and society.

The PBS and NIP are critical pillars of Australia's universal health system. Through these programs, millions of Australians access medicines and vaccines that improve life expectancy and quality of life, enable patients to be treated in the community and remain working for longer. Medicines and vaccines may prevent serious illness, slow the progression of disease and avoid costly hospital admissions.

HTA, the process that is used by the Pharmaceutical Benefits Advisory Committee (PBAC) to value and, hence, recommend a new medicine or vaccine for funding, is at a critical juncture. New technologies, in particular the rapid advance of precision, or personalised, medicine require us to think differently about HTA. Our policies and processes for HTA must evolve with this step-change in technology, to ensure that Australia is at the front of the queue for innovation.

Over the last 30 years, Australians have not directly considered how we should value the benefits that medicines and vaccines provide to society. This means that critical funding decisions are being made without proper consideration of what matters for Australians. Addressing these basic questions is vital to a future where Australian patients have timely access to new medicines and vaccines that not only contribute to better health outcomes, but the continued wellbeing and prosperity of our economy and society.

Earlier reports have highlighted fundamental problems in the way the Australian Government conducts HTA. Many of their recommendations have not been implemented or seriously followed up on. These include reports by the Productivity Commission (2005 and 2015) and the 2009 Australian Government HTA Review. The report of the most recent Parliamentary Inquiry into approval processes for new drugs and novel medical technologies in Australia (2021) has not yet seen a formal government response.

The COVID-19 pandemic, a once-in-a-century event, profoundly changed Australia. The value that we place on the health and wellbeing of Australians was highlighted by the willingness and urgency of

[6] *Prime Minister of Australia.2022. Speech to the National Press Club, 29 August 2022, https://www.pm.gov.au/media/building-better-future-national-press-club.*

Australian governments to respond rapidly. The urgent development and procurement of vaccines and medicines against COVID-19 were among the pivotal tools in saving lives and in protecting the Australian economy from major recession and further disruption. Although it is an extreme case, the pandemic emphasises the imperative of being a first launch country, as well as taking a holistic approach to valuing and, ultimately, deciding whether to fund medicines and vaccines.

Now is an opportune time to reform and enhance HTA in Australia. This paper provides an overview of the role that HTA plays in funding decisions in Australia, and a comparison against other countries. It considers:

- The role of medicines and vaccines in supporting a healthy and productive Australian population
- The growth of expenditure across the healthcare system in the last 20 years, while PBS expenditure has stabilised and is effectively shrinking as a proportion of healthcare expenditure (with stable growth projected over the next ten years)
- Evidence of the patient access gap in Australia, or the time between regulatory approval and PBS listing, which reflects the delay in Australian patients accessing new medicines and vaccines due to protracted HTA and pricing negotiations
- Australia's HTA approach to valuing new medicines and vaccines, in international comparison
- Strategic policies adopted in other countries to enhance patient access to new medicines and vaccines.

These issues are above and beyond the efficiency of HTA and listing processes which, though important, are only one aspect of the overall picture. Recently implemented reforms to the PBS application process (as a result of the 2017-21 Strategic Agreement between Medicines Australia and the Australian Government) are expected to generate some efficiencies, but do not address the underlying issues that are discussed in this paper.

This paper demonstrates that, due to concerted fiscal management efforts by the Australian Government and the pharmaceutical industry over the last 20 years, Australia has the capacity to improve patient access to new and innovative medicines, without jeopardising the continuing financial sustainability of the PBS. In doing so, it aims to open a conversation about how Australian society values medicines and vaccines, so that Australians our healthcare system may continue to adopt new and innovative technologies, now and into the future.

Medicines, vaccines and the health and wellbeing of Australians

"For wellbeing measures to start making a real difference to people's lives, they have to be explicitly brought into the policy-making process. Bridging the gap between wellbeing metrics and policy intervention is a challenge." -OECD[7]

Medicines and vaccines benefit not only those who receive them, but also the families and loved ones of patients, our economy and society. This broad perspective is reflected in the Australian Treasury's Wellbeing Framework. HTA, which informs funding decisions for new medicines and vaccines, however, takes a much narrower perspective. The quantitative cost-effectiveness assessment at the core of the HTA recommendation considers only direct patient health outcomes and resourcing implications for the healthcare system (although PBAC Guidelines do also allow sponsors to submit an economic evaluation considering wider benefits and costs). Furthermore, HTA decision making uses different thresholds than those used by other areas of government spending. These factors place limitations on how medicines and vaccines are valued, and potentially undervalue their true impact on the wellbeing of Australians, which risks our system underinvesting in innovative medical technologies.

Key points

- Medicines and vaccines are an investment in the wellbeing of Australians, our society and the productivity of our economy.
- The need to consider the broader social and economic impacts of public spending is an increasingly important policy goal for governments, with the OECD, along with Australia, Canada, New Zealand and the UK making progress on embedding a "wellbeing mindset" into decisions about where to invest public funds.
- The quantitative cost-effectiveness assessment at the core of the HTA recommendation considers only direct patient health outcomes and resourcing implications for the healthcare system, excluding other impacts.
- Adopting a broader perspective, such as a societal perspective, on the benefits and costs of a new technology in HTA would require sponsors to provide strong evidence of these impacts to enable them to be seriously considered in an economic evaluation.
- HTA decisions are guided by a threshold value that differs from the thresholds used in other areas of government spending, such as transport and environment.
- Flexibility in HTA decision making is important to enable a wider scope of benefits and costs to be taken into account, i.e. avoiding the strict application of threshold values to recommending or not recommending a new medicine or vaccine for funding on the PBS.

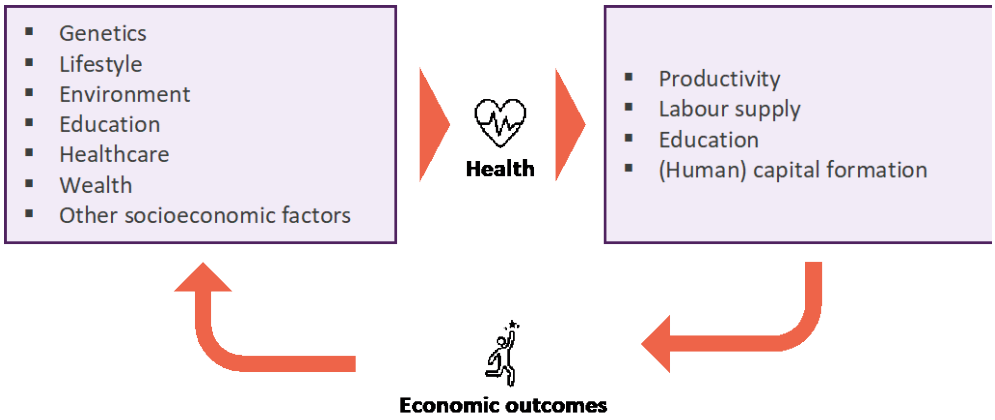
[7] *OECD. 2022. "Measuring Well-being and Progress: Well-being Research", Paris, https://www.oecd.org/wise/measuring-well-being-and-progress.htm.*

Healthcare and wellbeing

Australia's healthcare system has had a profoundly positive impact on the lives of Australians, consistently rating as one of the best performing among advanced Organisation for Economic Cooperation and Development (OECD) countries. Australia leads the world in key population health outcomes, including life expectancy, infant mortality and preventable mortality.[8] Importantly, we have been able to achieve these outcomes while spending a similar proportion of GDP on healthcare as other OECD nations.[9]

Expenditure on medicines and vaccines that leads to better health outcomes may be viewed as an investment in the wellbeing of individuals and Australian society overall. A healthy population, as a strong and productive labour force, also contributes to economic outcomes (Figure 1). Healthcare expenditure may therefore be seen as an investment in human capital. [10]

Figure 1: Factors contributing to population health, human capital and economic outcomes



Source: European Commission 2005[11].

Health has a profound effect on the ability of individuals to continue working, thereby directly contributing to the tax base and spending money in the economy. The McKell Institute (2018) found that people reporting excellent, very good or good health had approximately 30 percentage points' higher workforce participation rates than those reporting fair to poor health (Figure 2). The economic loss due to early retirement due to health-related reasons in Australia was $45.3 billion in 2017, or 2.5% of GDP. This was expected to increase to $53.4 billion by 2025, or 2.7% of GDP.

Retiring early also means that the individual retires with a lower superannuation balance than those who work until the formal retirement age. People who retired at age 50-54 lost up to $142,100, compared with those retiring at 65, as a result of lost income at a more senior level, and early withdrawals of funds.[12]

[8] *Eric C. Schneider et al. 2021. Mirror, Mirror 2021 — Reflecting Poorly: Health Care in the U.S. Compared to Other High-Income Countries (Commonwealth Fund). https://doi.org/10.26099/01DV-H208.*

[9] *OECD. 2022. Health spending (indicator). doi: 10.1787/8643de7e-en.*

10 PC. 2015. Efficiency in Health. Productivity Commission Research Paper (April 2015), https://www.pc.gov.au/research/completed/efficiency-health/efficiency-health.pdf.

[11] *European Commission, Health & Consumer Protection Directorate-General.. 2005. The contribution of health to the economy in the European Union, https://ec.europa.eu/health/archive/ph_overview/documents/health_economy_en.pdf, accessed 13 September 2022*

[12] *McKell Institute. 2018. Our Health Our Wealth: The impact of ill health on retirement savings in Australia, https://mckellinstitute.org.au/wp-content/uploads/2022/02/McKell_Early-Retirement_WEB.pdf..*

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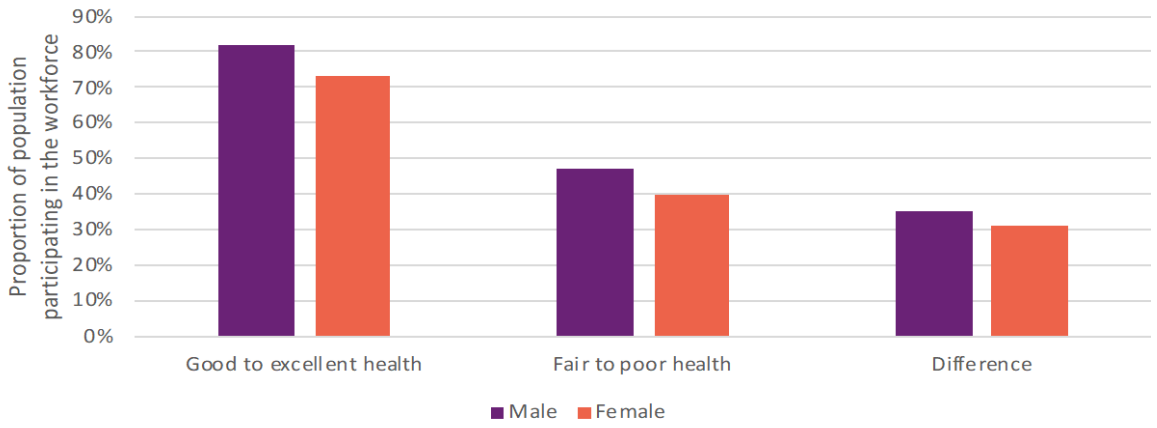
Figure 2: 50–64-year-olds who report excellent, very good or good health have higher participation rates (2017)



Source: McKell Institute 2018.

The McKell Institute's work built upon earlier work conducted by researchers at the University of Sydney regarding the economic costs of early retirement in 45-64 year-olds in 2009 associated with several conditions, including:

- Cardiovascular disease, estimated at approximately $1.1 billion in lost income, $225 million in lost income taxation revenue, $85 million in additional government benefit payments, and $748 million in lost GDP, on an annual basis in 2009 AUD[13]
- Spinal disorders, estimated at approximately $4.8 billion in lost individual earnings, $497 million in lost income taxation revenue, $622 in additional welfare payments, and $2.9 million in lost GDP, on an annual basis in 2009 AUD[14].

These substantial impacts affect not only the patients, their health and quality of life, but also the Australian economy and society. The authors found that this would justify greater expenditure on healthcare interventions that could prevent or ameliorate the effects of these conditions.[15]

Addressing Australia's disease burden

Investments in healthcare aim to address the burden of illness and injury. It is estimated that 50% of Australians lives with a chronic disease.[16] The AIHW's *2018 Australian Burden of Disease* study identifies therapeutic areas associated with premature loss of life and impacts on quality of life (measured as disability adjusted life years, DALYs). The top five areas of disease burden are aligned to the top five areas of healthcare expenditure in Australia, of which PBS expenditure makes up a small proportion (Figure 3).

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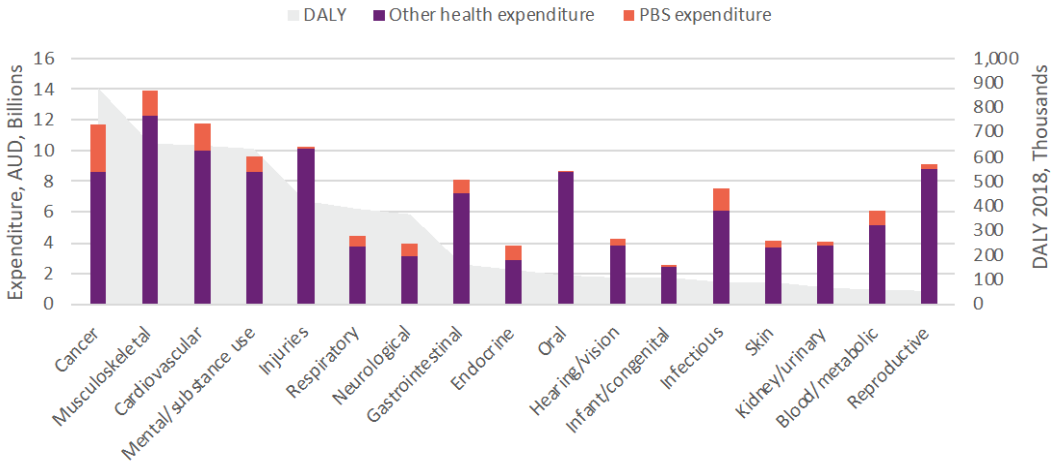
[13] *Schofield, D, Kelly, S, Shrestha, R, Passey, M, Callander, E & Percival, R. 2012. The long-term financial impacts of CVD: living standards in retirement', International Journal of Cardiology vol. 155, no. 3, pp 406-408.*

[14] *Schofield, D, Shrestha, RN, Percival, R, Passey,ME, Callander, EJ & Kelly, SJ. 2012. 'The personal and national costs of early retirement because of spinal disorders: impacts on income, taxes, and government support payments', The Spine Journal, vol. 12, no. 12, pp. 1111–1118.*

[15] *McKell Institute 2018.*

[16] *Australian Bureau of Statistics. 2018. National Health Survey: First results (2017-18), https://www.abs.gov.au/statistics/health/health-conditions-and-risks/national-health-survey-first-results/latest-release.*

Figure 3: Australian Burden of Disease, by disease areas (disability adjusted life years, DALY), 2018



Source: AIHW (2021)[17]. Areas of expenditure are summed across: Allied health and other services; General practitioner services; Medical imaging; Pathology; PBS; Private hospital services; Public hospital admitted patient; Public hospital emergency department; Public hospital outpatient; Specialist services; Dental expenditure. DALY=disability adjusted life years; PBS=Pharmaceutical Benefits Scheme.

Wellbeing policy frameworks

The need to consider the broader social and economic impacts of public spending is an increasingly important policy goal for governments. The October 2022 Australian Government Budget included *Measuring What Matters (Statement 4)* that, alongside standard financial estimates of government expenditure, proposed that federal budgets should also report on social, environmental and other measures of wellbeing[18]. This builds upon the Australian Treasury's *Wellbeing Framework* published over a decade ago (see Box).

These policy developments in Australia have not occurred in isolation; rather, they reflect a growing trend in response to changing policy goals and expectations triggered by issues such as environmental protection, climate change and wellness and mental health impacts of the COVID-19 pandemic. Such considerations feature to a greater degree in national and international economic policy thinking in many OECD countries.

- New Zealand Treasury has developed a Living Standards Framework to help guide its economic policy development and advice to the New Zealand government, which includes a recognition of the links between health and broader economic productivity[19]
- The Scottish Government's National Performance Framework which helps guide Scotland's decisions policy, services and spending explicitly recognises the link between society's health and its socio-economic status[20]

[17] *AIHW. 2021. Australian Burden of Disease Study: Impact and causes of illness and death in Australia 2018, https://www.aihw.gov.au/getmedia/5ef18dc9-414f-4899-bb35-08e239417694/aihw-bod-29.pdf.aspx?inline=true, accessed 13 September 2023.*

[18] *Australian Treasury. 2022. Measuring What Matters (Statement 4). https://budget.gov.au/2022-23-october/content/bp1/download/bp1_bs-4.pdf, accessed 20 February 2023.*

[19] *New Zealand Treasury/Te Tai Ohanga. 2021. The Living Standards Framework. https://www.treasury.govt.nz/sites/default/files/2021-10/tp-living-standards-framework-2021.pdf, accessed 13 September 2023.*

[20] *Scottish Government. 2019. Scotland's Wellbeing: Delivering the National Outcomes, May, https://nationalperformance.gov.scot/sites/default/files/documents/NPF_Scotland%27s_Wellbeing_May2019.pdf, accessed 13 September 2023.*

- Canada's Department of Finance is leading the Canadian Government's economic policy work to better incorporate quality of life measurements into government decision-making and budgeting[21].

The OECD has also had an extensive program over at least the last decade developing metrics to measure for wellbeing and progress in society[22]. The OECD work is, in part, focussed on operationalising such measurements of societal wellbeing to bring rigour and credibility to economic policy development.

Australian Treasury *Wellbeing Framework*[23]

"In undertaking its mission Treasury takes a broad view of wellbeing as primarily reflecting a person's substantive freedom to lead a life they have reason to value."

The Australian Treasury's *Wellbeing Framework*[24], published in 2011, emphasises an "economy-wide" perspective on wellbeing. This encompasses both individual preferences and scope for broader social actions and choices.

Five dimensions that directly or indirectly have important implications for wellbeing have been identified in the *Framework.* Optimising wellbeing across the population (or whole-of-economy) may require trade-offs between these dimensions.

- The **set of opportunities available to people**. This includes not only the level of goods and services that can be consumed, but good health and environmental amenity, leisure and intangibles such as personal and social activities, community participation and political rights and freedoms.
- The **distribution of opportunities across the Australian people**. In particular, that all Australians have the opportunity to lead a fulfilling life and participate meaningfully in society.
- The **sustainability of opportunities available over time**. In particular, whether our human, physical, social and natural assets are maintained or enhanced for current and future generations.
- The **overall level and allocation of risk borne by individuals and, in aggregate, the community**. This includes a concern for the ability, and inability, of individuals to manage the level and nature of the risks they face.
- The **complexity of the choices facing people and the community**. Of concern is not only the costs of dealing with unwanted complexity, but also t their preferences.

[21] *Department of Finance Canada. 2021. Towards a Quality of Life Strategy for Canada, 19 April, Ottawa, mwmtqlsc-mqivsqvc-en.pdf, accessed 13 September 2023.*

[22] *OECD. "Measuring Well-being and Progress: Well-being Research", Paris, https://www.oecd.org/wise/measuring-well-being-and-progress.htm, accessed 13 September 2023.*

23 https://treasury.gov.au/publication/economic-roundup-issue-3-2012-2/economic-roundup-issue-3-2012/treasurys-wellbeing-framework

[24] *Gorecki, S. & Kelly, J. 2012. "Treasury's Wellbeing Framework", Economic Roundup, Australian Treasury, Issue 3, 8 November, https://treasury.gov.au/publication/economic-roundup-issue-3-2012-2/economic-roundup-issue-3-2012/treasurys-wellbeing-framework, accessed 13 September 2023; Gruen, D. 2011. "Wellbeing, living standards and their distribution", Australian Treasury, Speech, 9 September, https://treasury.gov.au/speech/wellbeing-living-standards-and-their-distribution, accessed 13 September 2023.*

How Australians access new medicines and vaccines: HTA and reimbursement

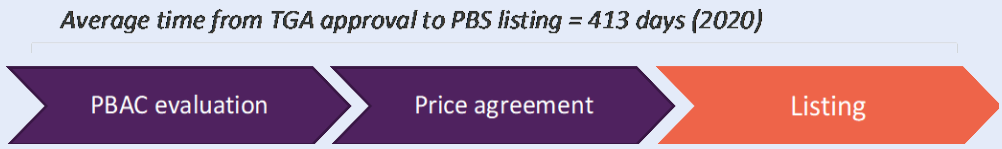
There are three main steps to funded access to new medicines and vaccines in Australia:

1. **Marketing approval** by the regulator, the Therapeutic Goods Administration (TGA), which assesses safety, clinical efficacy, and quality
2. **HTA**, conducted by the PBAC, which typically occurs after marketing approval and involves appraisal of **clinical, economic, and financial impacts, in comparison with already available treatments**
3. **Listing on the PBS. For vaccines, there are additional steps; pre-HTA advice from the Australian Technical Advisory Group on Immunisation (ATAGI), and tendering, which occurs only once every three to four years, to list on the NIP.**

No new medicine can be listed on the PBS (or the NIP, for vaccines) unless the PBAC makes a positive recommendation to the Minister for Health. Efficient pathways through HTA are therefore vital to ensuring timely patient access to new medicines and vaccines.

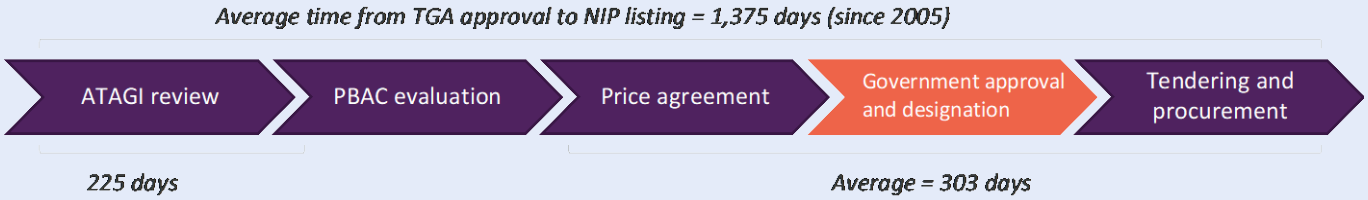
Figure 4: Australian HTA and reimbursement listing process for medicines and vaccines

Medicines



Source: Medicines Australia 2023[25].

Vaccines



Source: Shawview Consulting 2021[26]. Abbreviations: HTA=health technology assessment; ICER=incremental cost-effectiveness ratio.

[25] *Medicines Matter 2016-2021, https://www.medicinesaustralia.com.au/publications/medicines-matter/, accessed 20 February 2023.*

[26] *https://www.shawview.com/_files/ugd/8a9719_c61751a436ac49638ceed8b75cbf62af.pdf*

How does the PBAC decide whether to recommend a new medicine or vaccine?

HTA allows decision makers to evaluate technologies across different therapeutic areas and conditions, and associated with different benefits for patients and/or the healthcare system; thereby, "comparing apples and oranges". Cost-utility analysis (CUA) estimates the ratio of incremental benefits to costs, compared with existing clinical practices.

The key decision metric is the incremental cost-effectiveness ratio (ICER) (see Box), which is a quantitative assessment of the costs and benefits offered by the new technology. PBAC Guidelines allow for sponsors to submit economic evaluations with different scenarios focusing on direct costs and benefits, as well as the wider societal costs and benefits. Additional, qualitative, factors may also be considered, although it is not clear whether, or to what extent, these have a material impact on the evaluation.

Calculating the incremental cost effectiveness ratio (ICER)

The ICER measures the cost-effectiveness, or value for money, of a new medicine or vaccine. The ICER, or 'cost per QALY', is central to the PBAC's decision to recommend a new medicine or vaccine as cost-effective. If the ICER is too high, the application to list will be rejected.

Health-related impacts of a new medicine or vaccine are estimated using the **quality-adjusted life year (QALY)**. This measures both extensions to life (additional life years) and the quality of those years of life. A QALY value of 0 is equivalent to death, while a QALY value of 1 is a year lived in full health and enjoyment.

The ICER is measured as:

$$ICER = \frac{Incremental\ costs}{Incremental\ benefits}$$

$$ICER = \frac{\left(Total\ cost\ proposed\ (\$) - Total\ cost\ existing\ (\$)\right)}{\left(Total\ effects\ proposed (QALY) - Total\ effects\ existing\ (QALY)\right)}$$

If the ICER that is calculated is too high, the application to list will be rejected by the PBAC. Various analyses have been conducted to estimate the threshold value that is considered acceptable, as this is not formally documented in the PBAC guidelines.

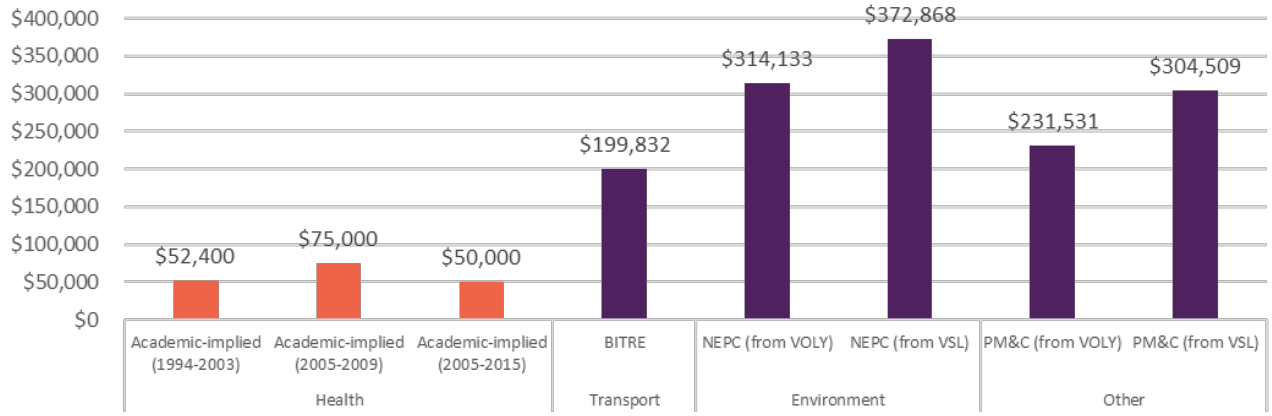
The broad consensus in the literature is that an application with an ICER below approximately A$45,000 – A$75,000 per QALY is more likely to be recommended, although reviews of public summary documents suggest that this is lower for vaccines ($15,000 per QALY) and may be higher for rare disease therapies (up to $150,000-$200,000 per QALY). This threshold value is understood to represent the value that could be generated by spending a similar amount elsewhere in the healthcare system, or the "opportunity cost" of that expenditure, and it does not seek to directly place a value on life, or years of additional life.

These analyses also suggest that the threshold value used by the PBAC has not changed since it was introduced in the early 2000s. By comparison, prices across the economy (the consumer price index) have risen by 65%, and prices in the health sector have risen by 138% between 2002 and 2022.[27]

[27] *ABS. 2022. CPI: Groups, Weighted Average of Eight Capital Cities, Index Numbers and Percentage Changes, Consumer Price Index, June 2022.*

Australian Government funding decisions in other policy areas, such as transport and environment, use different approaches to the PBAC to factor the value of saving lives into funding decisions. The value of a statistical life year (VSLY) used by the Office of Best Practice Regulation (OBPR) to inform funding decisions in areas such as transport and environmental policy is $222,000 in 2021.[28] This is three to four times the value used by the PBAC, and reflects inconsistencies in the underlying philosophy and methodology used to inform funding decisions in different sectors, where these decisions have an impact on the length and quality of the lives of Australians.

Figure 5: Overview of value of life years estimated for Australia and used by Australian Government departments



Source: Cubi-Molla et al. 2021.

There is no universal agreement on the appropriate ICER threshold value, and HTA agencies in other countries use different thresholds than the PBAC (see appendix).[29] The World Health Organisation (WHO) has suggested a rule of thumb that the ICER threshold should reflect approximately one to three times GDP per capita (approximately $60,000-$180,000 in Australia), but that cost-effectiveness analysis should just be one aspect that informs a funding decision.[30]

Perspective adopted in Australian HTA

The perspective adopted in HTA has implications for the valuation of a new medicine or vaccine. In Australia, the ICER calculation focuses on direct impacts for patients and the healthcare system. This essentially excludes consideration of broader economic and social benefits, including those with a direct impact on the Australian Government budget (see Figure 6). Other areas that may be important to consider in HTA include:

- Productivity impacts, as people avoid, or recover from, ill health and are able to return to work or avoid early retirement – it is argued that the QALY measure implicitly captures indirect effects such as the productivity of a patient that returns to work, although this is imprecise[31]

[28] *OBPR. 2021. Best practice regulation note: value of statistical life, https://obpr.pmc.gov.au/sites/default/files/2021-09/value-of-statistical-life-guidance-note-2020-08.pdf, accessed 13 September 2023.*

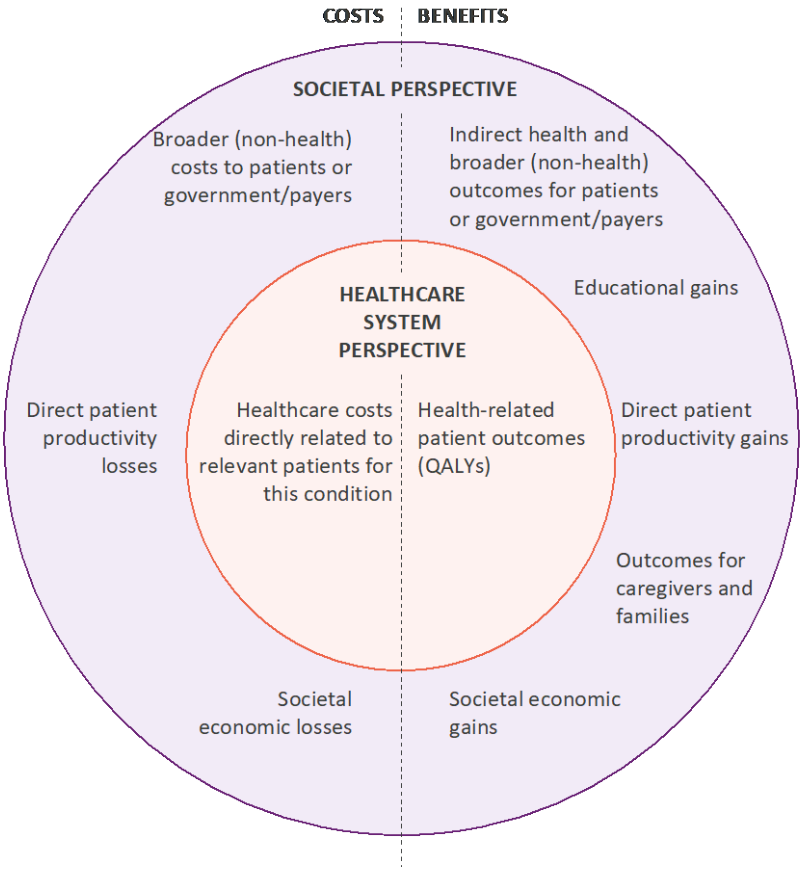
[29] *Sampson C, et al. 2022. Supply-Side Cost-Effectiveness Thresholds: Questions for Evidence-Based Policy. Appl Health Econ Health Policy. Sep;20(5):651-667. doi: 10.1007/s40258-022-00730-3. Epub 2022 Jun 7.*

[30] *Bertram, M., Lauer, J., Stenberg, K., Edejer, T. 2021. 'Methods for the Economic Evaluation of Health Care Interventions for Priority Setting in the Health System: An Update From WHO CHOICE', International Journal of Health Policy and Management, 10(Special Issue on WHO-CHOICE Update), pp. 673-677. doi: 10.34172/ijhpm.2020.244*

[31] *PC. 2005. Impacts of Advances in Medical Technology in Australia, Productivity Commission Research Report, https://www.pc.gov.au/inquiries/completed/medical-technology/report/medicaltechnology.pdf, accessed 13 September 2022.*

- Disability or aged care costs (as these are not direct healthcare costs), which may be avoided or delayed, for example as a result of slowing the progression of conditions such as Parkinson's and Alzheimer's disease
- Impacts on families and loved ones who may provide informal care to patients.

Figure 6: Perspectives in economic evaluation of new medicines and vaccines



Source: Neumann and Sanders 2017[32].

Flexibility in decision thresholds is important for considering a wider perspective

Two factors place restrictions on the valuation of new medicines and vaccines in Australia: (i) PBAC's decision thresholds, which are low by international standards and are not indexed while other prices in the economy rise; and (ii) considering only direct patient outcomes and healthcare costs in the ICER calculation.

These limitations may result in undervaluing new medicines and vaccines, relative to other areas of expenditure, and lead to underinvestment in new medical technologies. Advocates of taking a broader societal perspective in valuing medicines and vaccines include health economists, the pharmaceutical industry, patient groups and independent experts.

At present, PBAC does accept submissions from sponsors considering the societal perspective, including economic evaluations encompassing these broader outcomes (in addition to an economic evaluation from only the

[32] *Neumann PJ, Sanders GD. 2017. Cost-Effectiveness Analysis 2.0. New England Journal of Medicine. 376(3):203-205. doi: 10.1056/NEJMp1612619.*

healthcare system perspective. There is evidence that impacts on patients' quality of life and the lives of families and caregivers have been influential in decisions to list a new therapy.[33]

In order to create meaningful change that will enhance investment decisions in funding new medical technologies, two key areas are important:

1. That the PBAC retains flexibility in the application of ICER thresholds, i.e. that it is not held to a particular ICER value to recommend or not recommend a new medicine or vaccine
2. That sponsors provide robust and reliable evidence regarding the broader impacts of new medicines and vaccines.

Application of the Australian Treasury's Wellbeing Framework, and moves towards nationally consistent HTA across Australian, state and territory governments, may benefit from a more holistic consideration of what matters to Australians.

[33] *PBAC. 2018. Public Summary Document March 2018 PBAC Meeting: nusinersen, https://www.pbs.gov.au/industry/listing/elements/pbac-meetings/psd/2018-03/files/nusinersen-psd-march-2018.pdf.*

Australia's patient access gap

The 'patient access gap', time between registration and reimbursed access to new vaccines and medicines, in Australia exceeds many comparable OECD countries. Process reforms, though important and ongoing, are unlikely to fully address the patient access gap. Multiple resubmissions to the PBAC appear to be a major cause of delays for new PBS listings. Uncertainty around cost effectiveness, which relates to the valuation of the medicine or vaccine, is the main reason why submissions are rejected.

Key points

- Prioritising patient access to new medicines and vaccines is a key priority for both government and the medicines industry – the recent Strategic Agreement states that it is a shared policy goal for Australia to be a first launch country.
- Adoption of new medicines and vaccines is important to maintaining Australia's world class healthcare system, and HTA plays an important role in informing funding decisions.
- Studies across common products have found that the PBAC is more likely to reject applications for listing than HTA agencies in England, Canada, France and Germany. This may result in products not being launched or being sold privately, with out of pocket costs for patients.
- Reimbursement timeframes lag in Australia, compared to the OECD average, and are three to four times longer than world leading countries including Japan, Germany and the UK
- Resubmissions are often required and create delays in patient access to new medicines, which may have real consequences for patients, as illustrated through case studies in spinal muscular atrophy and meningococcal B vaccine where patients waited years for access to treatments that were funded in other countries
- Australia's HTA system will need to be agile to meet the upcoming challenge of evaluating and supporting the introduction of precision medicine and other advanced therapies in Australia.

Reimbursement timeframes lag in Australia, compared to other OECD countries

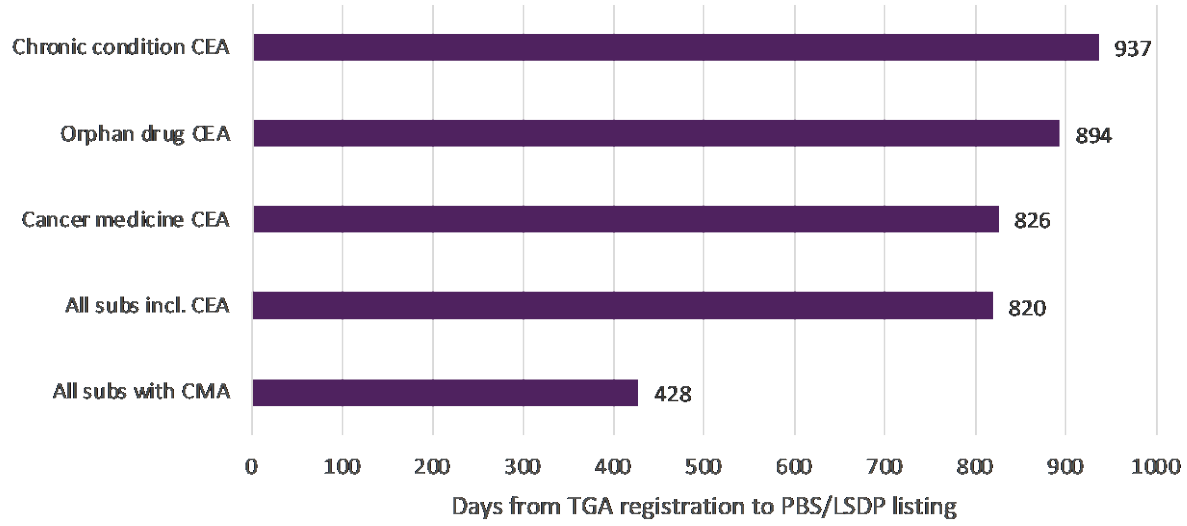
Listing a new medicine on the PBS (and vaccine on the NIP) is a critical factor in achieving equitable access for Australians. The outcome, efficiency and speed of the HTA process that must be satisfied in order to list new products therefore has a direct impact on patient access.

Australia's application process to the PBAC differs from key international HTA agencies, in that sponsors may be required to resubmit multiple times in order for a product to be listed. As the PBAC only meets once every four months, a deferred or rejected submission is automatically delayed by at least four months before further consideration may take place.

Lybrand and Wonder (2019), conducting analysis of applications for PBS listings over 2010-2018 (35 PBAC meetings) found that, on average, 2.3 for submissions that included a cost-effectiveness analysis and 1.4

submissions were required where only a cost-minimisation analysis was presented.[34] A similar analysis conducted over 2010-2017 (that also included LSDP listings) found that submissions that included a cost-effectiveness analysis took 820 days to be listed (more than two years), while submission with only a cost minimisation analysis took 428 days (over 14 months) to be listed. These results were more pronounced for orphan drugs, which are associated with high unmet patient need (894 days for submissions with a cost-effectiveness analysis) and chronic conditions (937 days).

Figure 7: Mean days from TGA listing to PBS/LSDP listing, 2010-2017



Source: Amgen submission to the Parliamentary Inquiry into Approval Processes for New Drugs and Novel Medical Technologies[35]

Cost-effectiveness analysis is used where the sponsor is claiming that the product has a higher value than existing alternatives on the PBS. This may result in additional costs, but also, potentially, offer greater benefits for patients. Delaying access may have significant consequences for these patients. The authors found that patient access could be sped up by one year for submissions including cost-effectiveness analysis if the number of resubmissions were reduced by 50%.

Analysis over 2016 to 2021 indicated that 44% of New Molecular Entities (NME) that were registered had been reimbursed in Australia. By comparison, 96% of registered NMEs were reimbursed in Japan, 84% in Germany and 80% in the UK. Of the 169 NMEs registered in Australia, 74 were reimbursed and a further 17 had launched on the private market.[36]

Over 2016 to 2021, average time from registration to PBS listing for NMEs in Australia has been estimated at 466 days, three to four times longer than world leaders Japan (102 days), Germany (136 days), Austria (153 days) and Great Britain (156 days) (Figure 8).[37] Over the same period, 17% of medicines were reimbursed within six months in Australia. By comparison, in Japan 65% of medicines were reimbursed within six months, 67% in Germany, 64% in Austria and 53% in Great Britain (Figure 9).[38]

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[34] *Lybrand S, Wonder M. 2020. Analysis of PBAC submissions and outcomes for medicines (2010–2018). International Journal of Technology Assessment in Health Care 36, 224–231. https://doi.org/10.1017/ S026646232000029X*

[35] *https://www.aph.gov.au/DocumentStore.ashx?id=afe9a28b-1684-4afd-8f04-2b53bdbee8a7&subId=693442*

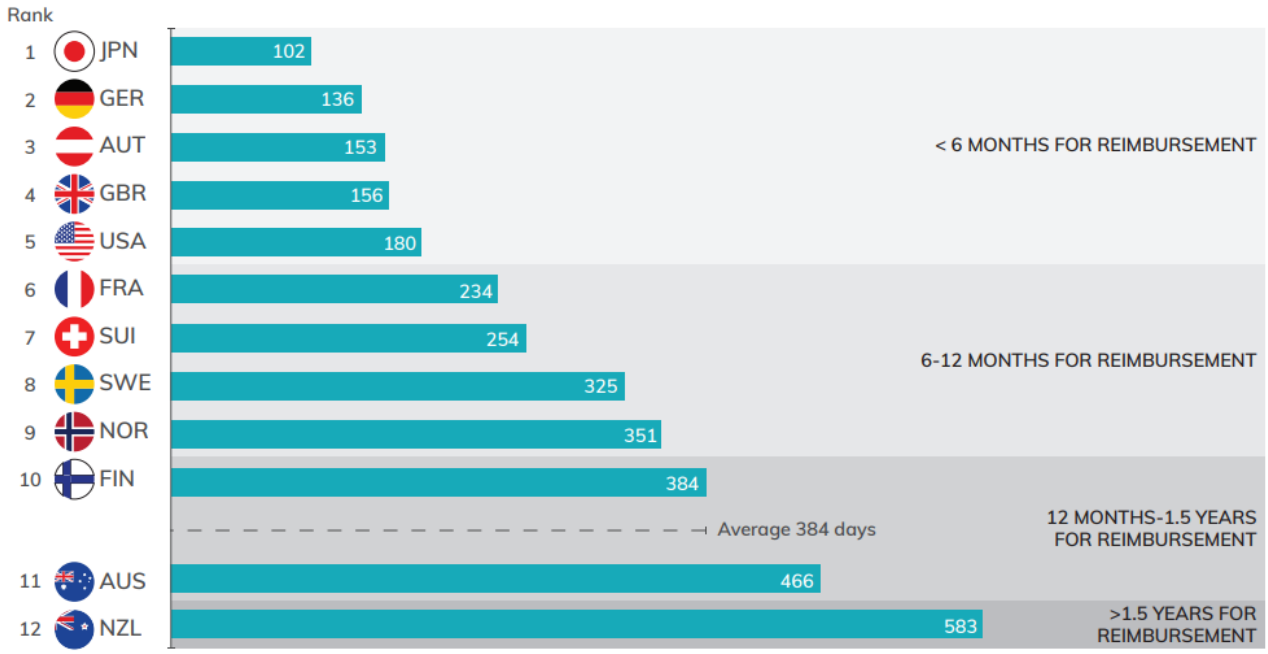
36 Medicines Australia 2023.

[37] *Medicines Australia 2023.*

38 Medicines Australia 2023.

These estimates take into account resubmissions to the PBAC, after a submission is initially deferred or rejected.[39] HTA agencies in other countries, including England's NICE and Germany's G-BA, do not typically involve resubmissions as part of the standard process, although they may engage with the sponsor to request more information or evidence before arriving at a final decision. These differences can lead to some discrepancies in the way that comparative data are reported (discussed in the next section).

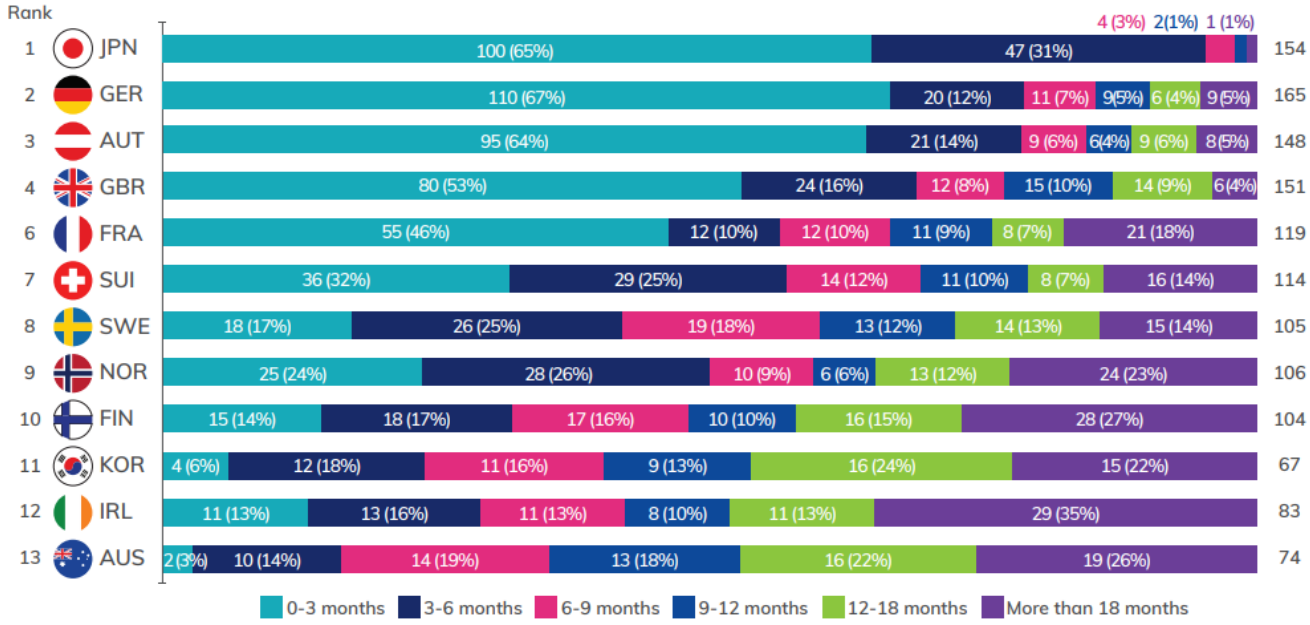
Figure 8: Average time from NME registration to reimbursement, 2016-2021: Fastest 12 OECD countries



Source: Medicines Australia 2023.

[39] *The most common reason for rejection is lack of evidence to satisfy the PBAC that the product is cost-effective. Biointelect 2019, 6 Months in a Leaky Boat, Presentation to the ARCS Conference 2019.*

Figure 9: Time from registration to listing (reimbursement) in selected OECD countries



Source: Medicines Australia 2023.

Analysing time from registration to PBS listing

The Centre for Innovation in Regulatory Science (CIRS) provides an alternative comparison of timing of the overall review cycle across countries. It found that, over 2016-2020, regulatory review, HTA and PBS listing took a median 428 days for products undergoing parallel reviews by the TGA and PBAC, that were recommended in the first submission to the PBAC (60% of submissions underwent parallel review over 2016-2020). This was faster than in comparison countries, including Canada, England, France and Germany. This measure does not take into account resubmissions, however; one resubmission was estimated to add approximately one year to the HTA process.

CIRS analysis of 26 common new active substances reviewed by HTA over 2016-2020 across seven countries found that Australia had the most rejections for listing. Australia rejected 14 (>50%) of the 26, followed by Germany (12). Canada (6), France (1) and England (0) were more likely to approve listing with some restrictions on access than reject outright.[40]

Different methodologies used by reports and data sources to make comparisons between countries on time from registration to reimbursement may not include the impact of resubmissions. Since resubmissions are frequently required for the PBAC, but not in other countries, this masks the true impact of Australia's HTA policies on patient access to medicines.

[40] *Wang T, Sola B, McAuslane N. 2022. R&D Briefing 83: Review of HTA outcomes and timelines in Australia, Canada and Europe 2016 -2020. Centre for Innovation in Regulatory Science. London, UK.*

Precision medicine represents a step-change in technology

Current HTA methodologies for medicines and vaccines were developed in an era when key policy priorities involved controlling expenditure on large scale, population-based small molecule blockbuster medicines such as statins, that were used to treat a high prevalence conditions (e.g. elevated cholesterol levels, high blood pressure). The emerging era of medicine will be increasingly involve personalised medicine, genomics and targeting treatments at the cellular and genetic level bespoke to patient needs.

The nature of precision medicines means that clinical trials involve fewer patients and, potentially, examine different treatment outcomes for specific patient populations. This may mean better treatments for patients, but less clarity on how clinical trial results would apply across the broader Australian patient population.

At the same time, regulators, including the TGA, have introduced pathways that allow provisional approval of new therapies in areas of high unmet need, where the product shows substantial promise. This means that products may receive marketing authorisation with earlier phase clinical evidence.

These factors are likely to result in ambiguity in clinical data at the time a product is launched and, hence, uncertainty regarding cost-effectiveness. Innovative clinical trial designs and earlier phase studies may form the basis for provisional approvals from regulators, but then create challenges in HTA as these evidence packages are associated with greater uncertainty. Under current HTA methodologies, uncertainty in clinical evidence often translates into rejection of funding applications and the need for resubmission to the PBAC, delaying patient access.

These issues are not unique to Australia and must be addressed in order to facilitate the adoption of new technologies into our healthcare system. The PBAC is tightly constrained, as it is bound by the *National Health Act*, which requires that it may only recommend products that it finds to be cost effective. This limits its scope to approve products with uncertainty associated with clinical data.

There is a risk that current approaches to HTA will result in underinvestment in new therapeutics in an era of precision medicine, when optimising value across the healthcare system has never been more important.

Case study: Nusinersen (Spinraza®) for spinal muscular atrophy (SMA)

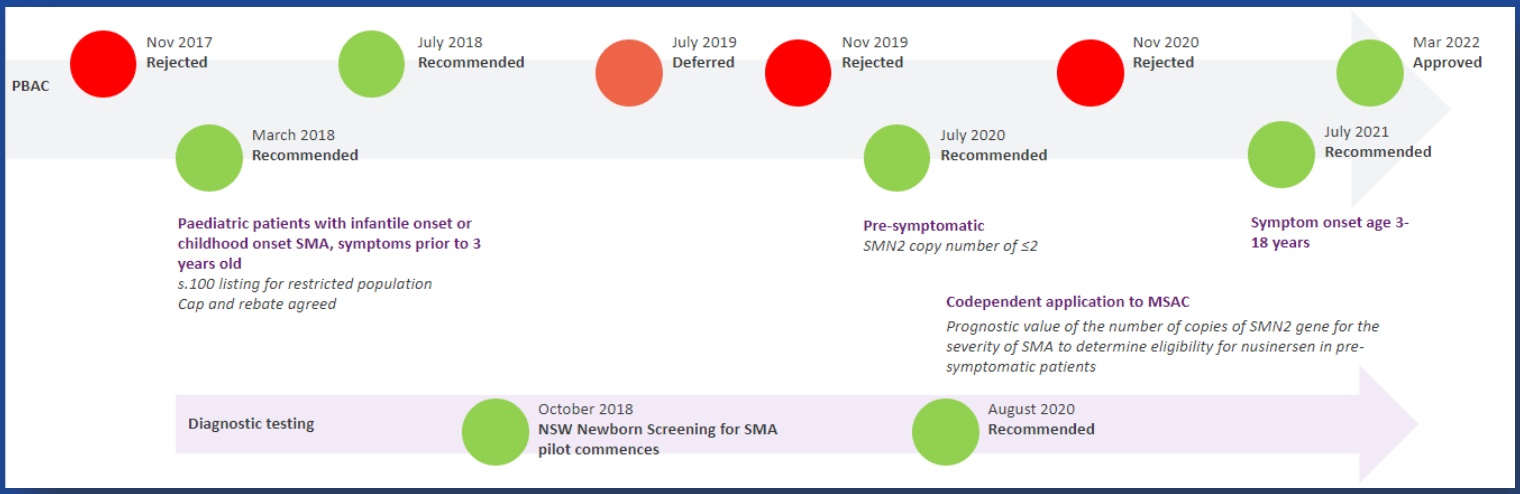
Nusinersen (Spinraza®)is a groundbreaking, disease-modifying treatment for SMA, a rare, genetic neuromuscular disorder that has a profound effect on the lives of patients, their families and loved ones. Some children with SMA may die before age two, while others live with severe disability.

Biogen Australia's submission to the PBAC for the reimbursement of nusinersen was initially made in November 2017. It was rejected on grounds of unclear evidence that nusinersen was cost-effective for all SMA patients. Nusinersen was recommended for only the most severe SMA patients, following resubmission four months later.

The PBAC acknowledged the high and urgent clinical need for treatments for SMA and consumer input was strongly supportive of a PBS listing that covered a broader set of patients.

Over the next five years, a further eight submissions were made to the PBAC. As of 2022, access to nusinersen has now been expanded to a wider population of SMA patients in Australia. Some patients waited more than five years to access this potentially life-changing therapy.

Figure 10: Overview of submissions to the PBAC for nusinersen (Spinraza®), 2017 to 2022



Case study: Meningococcal B vaccine (4CMenB/Bexsero)

Invasive meningococcal disease is a devastating condition that can escalate quickly, leading to death or severe disability. Vaccination against A, C, W and Y strains is part of the standard childhood NIP; however most Australian children remain unprotected against Meningococcal B.

The PBAC considered 4CMenB four times between November 2013 and November 2019, rejecting it each time on the grounds of lack of cost-effectiveness. Ultimately, in November 2019, with the provision of additional evidence from the Sponsor, the PBAC approved 4CMenB for a small, high-risk population.[1] It has been listed on the NIP for Aboriginal and Torres Strait Islander children since July 2020.

A key factor in this determination was a highly restrictive threshold value to reflect the opportunity cost of investing in a vaccine or public health intervention. Rather than the $45,000-$75,000 per QALY used for medicines, this threshold was set at only $15,000 per QALY.

[41] *PBAC. 2019. Public Summary Document: MULTICOMPONENT MENINGOCOCCAL GROUP B VACCINE, https://www.pbs.gov.au/industry/listing/elements/pbac-meetings/psd/2019-11/files/multicomponent-meningococcal-b-vaccine-psd-november-2019.pdf, accessed 13 September 2022.*

Levers that stabilise PBS expenditure

Expenditure growth in the PBS has stabilised through reforms to structure and pricing, including through agreements with the Australian pharmaceutical industry. Treasury's projections in the most recent Intergenerational Report indicate that Australian Government expenditure on pharmaceuticals will shrink as a proportion of total health expenditure over the next ten years.

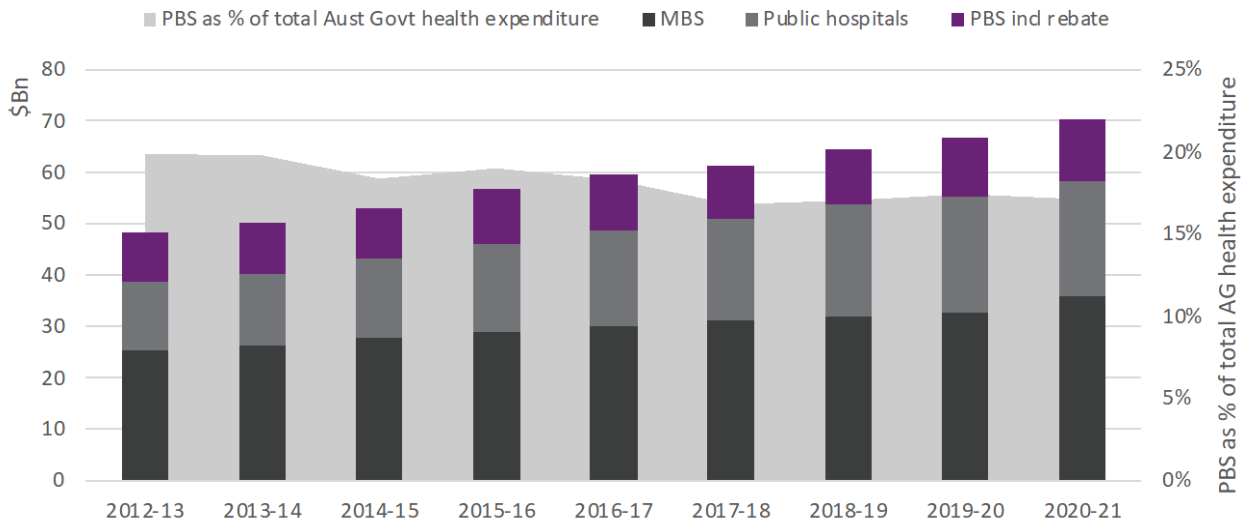
Key points

- With structural and pricing reforms, PBS expenditure has stabilised since the rapid growth of the late 1990s. Price cuts apply once products have been in the market for five or more years, and when generic or biosimilar competitors are listed.
- Including rebates (which reflects actual expenditure), PBS has fallen as a percentage of total Australian Government healthcare expenditure over the last decade.
- Treasury projects that this will shrink further over the next 10 years, as expenditure elsewhere in the system escalates, in particular public hospitals and the Medicare Benefits Schedule (MBS). This may result in a distortion that does not optimise health outcomes across the Australian population.
- There is scope and financial headroom to increase expenditure on the PBS and NIP, with strategic policies that prioritise access to new medicines and vaccines for Australian patients, while maintaining existing cost controls across the PBS.

Contained growth in PBS expenditure

Over the past two decades, reforms to the structure and pricing of PBS medicines have been associated with stabilisation of PBS expenditure. Figure 11 shows that PBS expenditure has grown from $10 billion to $13 billion over the last 10 years in nominal terms, shrinking as a proportion of total Australian Government healthcare expenditure from 20% in 2012-13 to 17% in 2020-21.

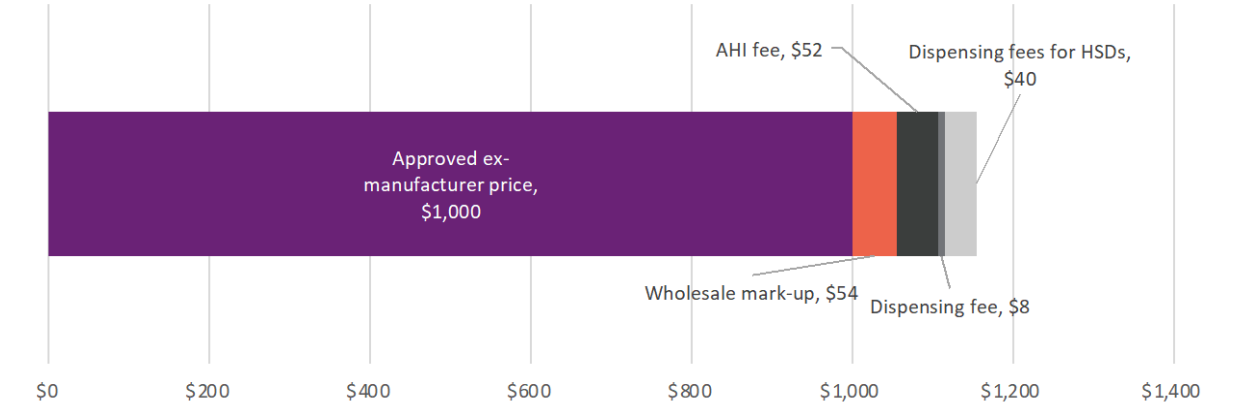
Figure 11: Australian Government expenditure on the PBS and other core healthcare programs



Source: Australian Government Final Budget Outcome 2012-13 to 2020-21; Department of Health Annual Reports 2012 to 2021. AG=Australian Government; MBS=Medicare Benefits Schedule; PBS=Pharmaceutical Benefits Scheme.

Not all PBS expenditure is related to medicine prices. Additional mark-ups and fees are provided to wholesalers and pharmacists to handle and dispense PBS medicines. Figure 12 provides an overview of these fees in 2022, assuming an ex-manufacturer price of $1,000. Mark-ups and fees add approximately 13% to the total cost to the PBS. This may be higher for certain medicines and scenarios where additional fees apply, such as fees for dangerous drug handling and efficient use of chemotherapy medicines.[42]

Figure 12: Components of PBS expenditure (assuming a $1,000 ex-manufacturer drug price), 2022



Source: Services Australia 2022[43]. Wholesale mark-up is capped at $54.14 per dispense for medicines priced above $720; AHI fee is $4.32, plus 5% of the amount where the price to pharmacists exceeds $100.00, per maximum quantity supplied; dispensing fee is set at $7.82 for ready-prepared medicines and dispensing fees for highly specialised drugs (HSDs) are capped at $40.

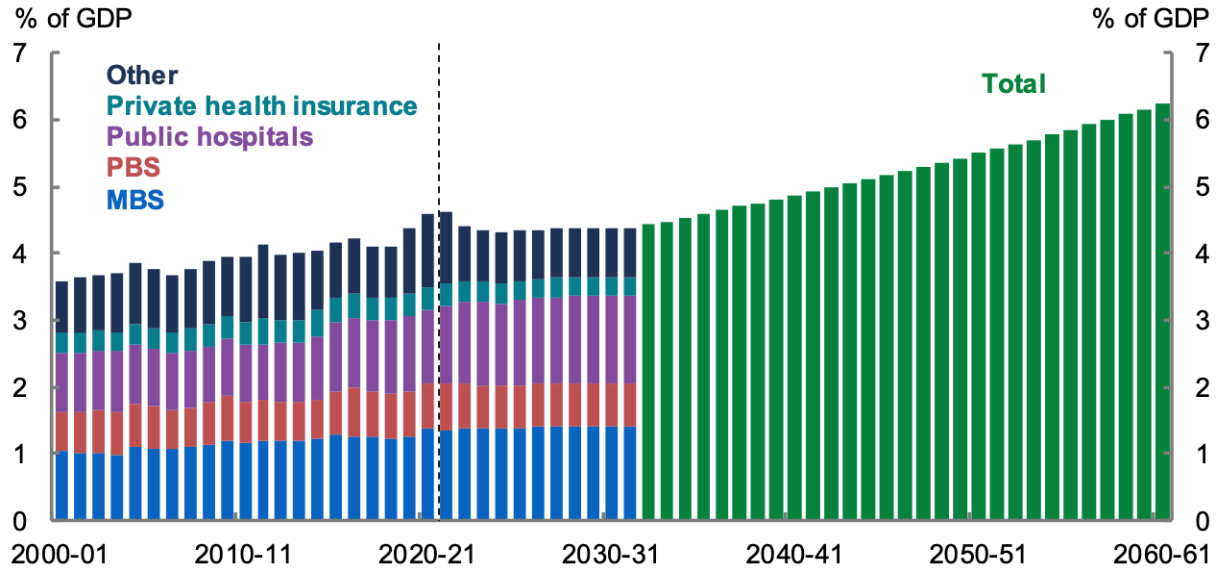
Treasury's first Intergenerational Report (in 2002 highlighted the PBS as "a key priority for ensuring fiscal sustainability", following rapid growth over the previous decade. Treasury (in its 2021 Intergenerational Report) is now more concerned about growth of Australian Government expenditure on public hospitals, projected to almost double in nominal terms (35% real growth) over 2020-21 to 2031-32, and the Medicare Benefits Schedule (MBS), projected to grow by 70% in nominal terms (15% real growth) over the same period. By comparison, PBS expenditure is projected grow by less than 10% (in real terms) over 2020-21 to

[42] *Services Australia. 2022. Detailed information on how prescription medicines are priced under the PBS and RPBS for pharmacists. https://www.servicesaustralia.gov.au/about-pbs-for-pharmacists?context=22861, accessed 13 September 2022.*

[43] *Services Australia 2022.*

2031-32 (Figure 13). The 2023 Australian Government Budget announcement to provide $5.7 billion in additional funding to strengthen Medicare, while important, will exacerbate these discrepancies.

Figure 13: Australian Government health spending by components (2021 Intergenerational Report)



Source: Treasury 2021[44].

The Australian healthcare system is complex and governments employ various strategies to contain costs (Table 1). This means that different rules apply to funding decisions in different areas of the health budget. The Productivity Commission has argued that the same rigour that is applied in HTA should also be applied to expenditure in other areas[45]. Lack of consistency in approaches is likely to be contributing to the reallocation of the health budget seen in Figure 13. As well as reallocating funding, this may not be the most efficient and productive allocation of financial resources in healthcare.

[44] *Australian Government Treasury. 2021. 2021 Intergenerational Report, https://treasury.gov.au/publication/2021-intergenerational-report, accessed 13 September 2022.*

[45] *Productivity Commission 2015.*

Table 1: Cost containment across core areas of Australian Government healthcare expenditure

Area	Model	Cost containment measures
Public hospitals	Activity based funding	• Australian Government growth funding limited to 45% of growth in public hospital expenditure, at the nationally efficient price (as determined by the Independent Health and Aged Care Pricing Authority) • National Funding Cap set at 6.5% growth per year[46]
Medicare	Fee for service schedule	• New technologies (including diagnostic tests) may be subject to HTA • MBS Review (2015-2020) sought to review cost effectiveness and made recommendations to remove some items from the MBS[47]
PBS	Fee per dose	• New medicines and vaccines must be subjected to HTA • New listings may include expenditure caps, rebates, outcome-based agreements • Anniversary price cuts at 5, 10 and 15 years • Statutory price reductions for products with generic/biosimilar competitors and ongoing price disclosure reductions
Private health insurance	% of premiums	• Income tested, eligibility criteria tightened since introduction[48]

PBS reforms over the last two decades[49]

Reforms to the structure and pricing of the PBS have been introduced in order to support the sustainability of the program. The impact of key reforms on containing total expenditure on the PBS is clearly shown in Figure 14.

Statutory price reductions

2005, revised in 2015, and with Strategic Agreements with Medicines Australia in 2017 and 2022

Statutory price reductions apply as:

- First New Brand Statutory Price Reductions (FNB SPR), which have increased from 12.5% in 2005 to 25% on 1 October 2018[50]
- Anniversary price reductions. From 2015, a one-off 5% statutory price reduction is applied to F1 items that had been listed on F1 for five years. From 2018, anniversary discounts were also applied to F1 items that had been listed for ten years and fifteen years, at 10% and 15% respectively.

Other price reductions may also be applied to F1 medicines. If a new medicine demonstrates therapeutic equivalency to an F1 medicine and is of lower price, the government may request that the price of the existing medicine match that of the new medicine.

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[46] *Addendum to the National Health Reform Agreement 2020-2025, https://federalfinancialrelations.gov.au/sites/federalfinancialrelations.gov.au/files/2021-07/NHRA_2020-25_Addendum_consolidated.pdf, accessed 13 September 2022.*

47 Australian Government Department of Health and Aged Care. Medicare Benefits Schedule (MBS) Review, https://www.health.gov.au/initiatives-and-programs/mbs-review, accessed 13 September 2022.

48 Australian Taxation Office. Private health insurance rebate, https://www.ato.gov.au/Individuals/Medicare-and-private-health-insurance/Private-health-insurance-rebate/, accessed 13 September 2022.

[49] *Viiv Healthcare. The Pharmaceutical Benefits Scheme in Australia, https://au.gsk.com/media/6259/gsk-viiv-the-pbs-in-australia-feb-2018.pdf, accessed 13 September 2022.*

[50] *PBS. First New Brand Price Reductions, https://www.pbs.gov.au/info/industry/pricing/pbs-items/first-new-brand-price-reductions, accessed 13 September 2022.*

Creation of F1 and F2 formularies and introduction of price disclosure requirements

2007, revised in 2010 under the Memorandum of Understanding with Medicines Australia and in 2022 under the Strategic Agreement with Medicines Australia

PBS items were separated into two formularies, F1 and F2. F1 consists of medicines with only one brand listed, and F2 consists of medicines with a variety of brands (i.e. generic or biosimilar competitors). This allows different pricing rules to apply to each formulary. In particular, it allows price reductions to be applied to F2 products, which have lost exclusivity and are subject to competition, without these lower prices flowing on to prices of products on F1. This protects the value of innovative pharmaceuticals on F1.[51]

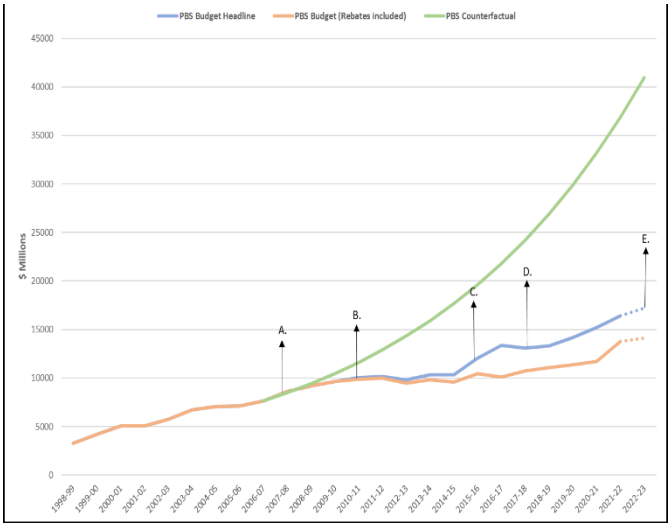
F2 medicines are subject to price disclosure arrangements (with the exemption of a few), to align PBS prices to those being offered in the market. PBS prices are revised to reflect prices offered in retail pharmacy and, since 2022, public hospitals.[52]

Patient co-payments (updated each year)

The PBS introduced patient co-payments in 1960, but these were substantially increased in response to the 2002 Intergenerational Report, which raised concerns over rapid growth in PBS expenditure in the preceding decade. Co-payments (and safety net thresholds) are adjusted every year on 1 January, in line with the Consumer Price Index (CPI). Brand and therapeutic group premiums may also apply if consumers opt for more expensive products that are therapeutically equivalent to a cheaper product, where these are available.[53]

As of 1 January 2022, patients may pay up to $42.50 for medicines and $6.80 if patients have a concession card[54], although these will be reduced as part of an election commitment in the October Budget. The PBS Safety Net, set at to $1,542.10 per year for general patients and at $326.40 per year for concessional patients (January 2022) seeks to maintain affordability for consumers.[55]

Figure 14: Impact of PBS reforms on expenditure, 2007-08 to 2022-23



A. Price Disclosure Requirements introduced on 1 August 2007.
B. 2010 Memorandum of Understanding with Medicines Australia including greater reductions in F2 and all brands of drugs in F2 subject to price disclosure.
C. 2015 Access and Sustainability Package changes to the PBS. The introduction of Anniversary Price Reductions for F1 medicines which included a reduction for medicines listed for over five years.
D. 2017 Strategic Agreement with Medicines Australia which included expanded price reductions for F1 medicines at ten and fifteen years after listing.
E. 2022 Strategic Agreement with Medicines Australia will begin, which will include greater reductions for medicines listed on the PBS for over fifteen years.

[51] *Searles A, Jefferys S, Doran E, Henry DA. Reference pricing, generic drugs and proposed changes to the Pharmaceutical Benefits Scheme. Med J Aust. 2007 Aug 20;187(4):236-9. doi: 10.5694/j.1326-5377.2007.tb01208.x. Epub 2007 Jun 13. PMID: 17564580.*
[52] *PBS. Price disclosure, https://www.pbs.gov.au/info/industry/pricing/price-disclosure-spd, accessed 13 September 2022.*
[53] *PBS. About the PBS, https://www.pbs.gov.au/info/about-the-pbs, accessed 13 September 2022.*
[54] *PBS. Pharmaceutical benefits, https://www.pbs.gov.au/info/healthpro/explanatory-notes/front/fee, accessed 13 September 2022.*
[55] *Services Australia. PBS Safety Net thresholds, https://www.servicesaustralia.gov.au/pbs-safety-net-thresholds?context=22016, accessed 13 September 2022.*

How does Australia compare internationally?

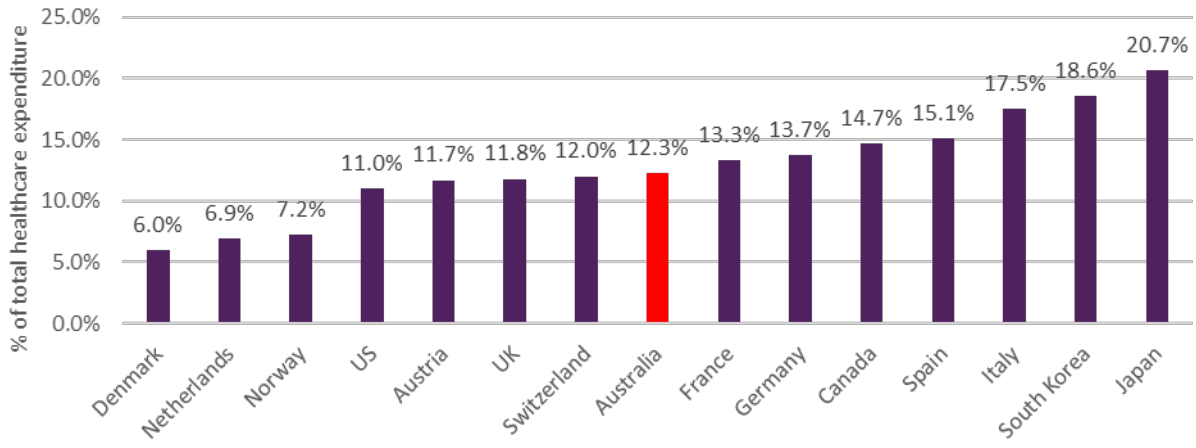
Australia currently spends approximately 12.3% of the total health budget on pharmaceuticals, which is in line with the OECD average (Figure 15). Maintaining expenditure at similar levels to comparable OECD countries is frequently cited as important to remaining among the first launch countries for new medicines and vaccines.

It is difficult to accurately compare launch prices for new medicines and vaccines across countries, as prices are typically confidential. Moreover, the actual prices paid may be obscured by conditions on reimbursement and risk sharing arrangements such as caps and rebates.

Countries, including major global pharmaceutical markets Germany, Japan and the US, have recently implemented policies targeted at reducing spending on pharmaceuticals. Some commentators have argued that Australia's prices for older pharmaceuticals are higher than in other countries, including England and New Zealand.[56] These analyses appear to focus on products that have been listed on the PBS for some time and following loss of exclusivity[57]. More recent changes to statutory price reductions and price disclosure rules (see previous section) has since led to major price reductions in the prices of generic medicines in Australia

While being internationally competitive is a factor in attracting new product launches to Australia, just spending more may not be sustainable or represent good value for money across the PBS. Alone, this is unlikely to cement Australia's position as a first launch country, which requires a more detailed and strategic consideration of policy settings, *vis a vis* the global market.

Figure 15: Pharmaceutical expenditure as a proportion of total health expenditure, 2021 or latest available, selected OECD countries



Source: OECD 2022[58].

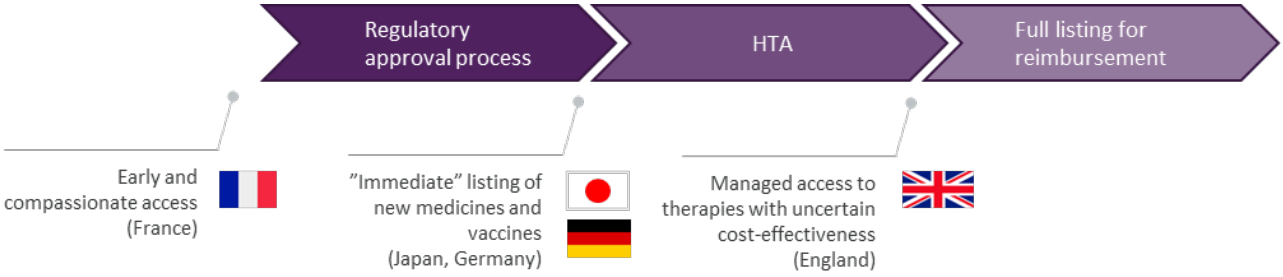
[56] *Duckett 2013; Clarke 2013; NCOA 2014; Mansfield 2014; Duckett and Breadon 2013, cited in PC 2015.*
[57] *PC 2015.*
[58] *OECD. Pharmaceutical spending (indicator). doi: 10.1787/998febf6-en, accessed 22 July 2022.*

Smarter policies to prioritise patient access

Australia may look to the experience of countries such as Japan, France, Germany and England for strategic policies that aim to position them as a first launch country for new medicines and vaccines (see Figure 16). These policies prioritise patient access, while keeping spending under control. Like the majority of OECD countries, they remain actively concerned about constraining expenditure in healthcare as a result of ageing populations and rapidly advancing medical technologies. It is pertinent to ask, what HTA system would we create if we started without our existing HTA system? The goal is to design smart, strategic policies for Australia that will put patient access first.

- Japanese policies aim to balance expenditure control with promoting innovation. All medicines are subject to price erosion during the patent protection period, although the Price Maintenance Premium (PMP), which is awarded to products that offer greater value to patients and the healthcare system, defers any price reductions until after patent expiry.
- Germany and Japan, among the fastest adopters of new pharmaceuticals in the world, both introduced HTA with the explicit intention of not allowing this to delay patient access. Funded access to new technologies is provided while HTA is being conducted. New products are "automatically listed" in Germany upon receipt of marketing authorisation and are listed within 60-90 days of marketing authorisation in Japan.
- France provides early funded and access to new medicines before they receive marketing authorisation, in areas of high unmet need, including life-threatening and disabling conditions.
- England's Cancer Drugs Fund and Innovative Medicines Fund provide funded access to new therapies under managed access agreements, where there is uncertainty about cost-effectiveness at the time of market entry. Products are typically re-reviewed after two years and may be re-priced or de-listed at that point.
- Data collection requirements are set out by HTA agencies in early and managed access in France and England, respectively, so that this may be utilised when the full evaluations of new therapies

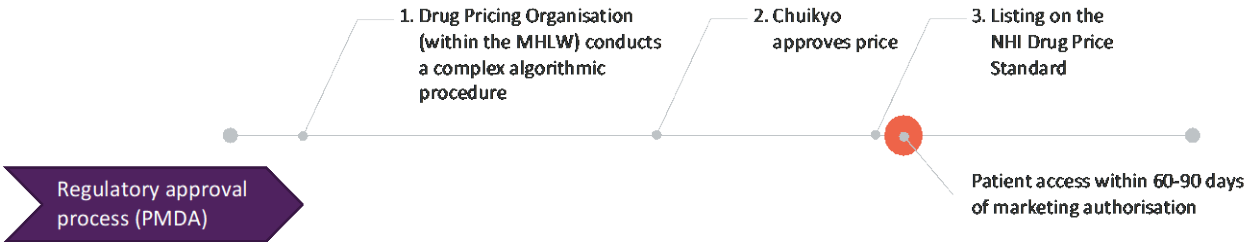
Figure 16: Programs that enable faster patient access to innovative medicines and vaccines in other countries (case studies)



Access and pricing of new medicines in Japan

Japan is the largest pharmaceutical market for innovative medicines outside of the US. The modern pricing system in Japan was developed via a series of reforms approximately 12 years ago in response to public outcry in relation to 'drug lag' - whereby patients had to wait upwards of eight years to access potentially lifesaving drugs[59]. In recent years, there have been notable changes to the pharmaceutical pricing system; however, the concept of avoiding drug lag via maintaining conditions that promote **early access to innovation, while maintaining expenditure controls** remains a key plank of pharmaceutical policy[60] and has direct consequences on the implementation of HTA.

Figure 17: Overview of the Japanese drug pricing system



MHLW= Ministry of Health, Labour and Welfare; NHI=National Health Insurance; PMDA=Pharmaceutical and Medical Devices Agency; Chuikyo=Central Social Insurance Medical Council, composed of payers, physician and public interest representatives and is responsible to set fee schedule revisions for medical services and pharmaceuticals.[61]

Japan has a variety of mechanisms to erode the price of listed pharmaceuticals during their patent protection period.[62] All drugs are subject to a repricing mechanism that aims to reduce the difference between the DPS reimbursement price and its actual market price, with an average reduction of 5-7%.[63] Beginning in 2016, several drug price shocks resulted in the introduction of budget impact-related repricing rules targeted at drugs that have either far exceeded revenue forecasts or that have significant market expansion via quarterly reviews of drugs that have new indications.[64]

Japan tries to balance expenditure control with promotion of innovation and in 2010 established a mechanism called the **Price Maintenance Premium (PMP)** to defer price erosion from products offering added value against the standard of care. PMP eligible products are still repriced during their patent period, however the impact of the repricing is deferred until their loss of exclusivity. The concept aims to enable companies a faster recovery of R&D investment and therefore to promote products that offer additional therapeutic benefits.[65]

Introduction of HTA in Japan

In April 2019, Japan introduced a system of HTA to be applied to technologies that meet certain criteria related to budget impact, as a means of driving greater efficiency in expenditure on medicines. Cost-effectiveness assessment was integrated into the pre-existing pricing system (Figure 17), adjusting prices

[59] *Tsuji K and Tsutani K. 2010. Approval of new drugs 1999-2007: comparison of the US, the EU and Japan situations. J Clin Pharm Ther. 35(3): 289-301.*
[60] *Shibata S et al. 2020 Japanese pharmaceutical industry: recent perspectives and areas for further research. Journal of Regulatory Science 8: 1-13.*
[61] *Shibata S et al. 2020; Mamiya H. 2018. Update of Drug Pricing System in Japan. https://www.mhlw.go.jp/file/04-Houdouhappyou-11123000-Iyakushokuhinkyoku-Shinsakanrika/price.pdf Accessed September, 2022.*
[62] *Shibata et al., 2020.*
[63] *Nagatani T et al (2018) Change in the Japanese pharmaceutical market: cradle of innovation or grave of corporate profits? McKinsery & Company. https://www.mckinsey.com/industries/life-sciences/our-insights/change-in-the-japanese-pharmaceutical-market-cradle-of-innovation-or-grave-of-corporate-profits Accessed September 2022.*
[64] *Mamiya 2018.*
[65] *Shibata et al. 2020.*

after new therapies have already been listed and are available to patients. This aims to avoid delays in patient access to innovative treatments while the HTA process is conducted (a similar rationale to Germany's interim access arrangements).

In Japan, HTA is coordinated by the Centre of Outcomes Research and Economic Evaluation for Health (C2H) within the National Institute of Public Health, with the Chuikyo selecting products for assessment and making the final decision on proposed price adjustments. HTA is limited to products that meet one of five categories that are primarily based around budget impact or high list prices and manufacturers are informed that HTA will be conducted at the time of initial listing.

Eligible products undergo a 15-18 month process that includes nine months for the manufacturer to consult with C2H and develop a CEA submission, three months for independent HTA review with an optional three months if the manufacturer's analysis is deemed to require re-analysis, and three months for evaluation and price determination, including the Chuikyo decision.[66]

Following cost-effectiveness assessment, the ICER is compared against three cost-per-QALY thresholds of ¥5, 7.5 and 10 million yen (approx. AUD $53,000; $80,000; and $106,000) per QALY. The three thresholds result in a stepwise impact to the price premiums above the comparator price that range from no impact in the lowest band (i.e., if the ICER is under the ¥5m threshold) to a 90% premium reduction for the highest threshold.[67]

For pharmaceuticals where there is no direct comparator and that have been priced by the cost calculation method (these are usually new mechanisms of action), the impact to the premium is lower. If companies fail to provide price transparency relating to R&D costs, then the impact can extend beyond the premium into the operating profit component of the price.[68] The total impact of price reduction is limited to 10-15% of the overall product price, to avoid risk of excessive price cuts given the presence of the other price reducing mechanisms in the system.

The system makes allowances for rare or paediatric diseases and for cancer therapy where the ICER might not fully capture the treatment value by shifting the three thresholds by 1.5x to ¥7.5m, ¥11.25m and ¥15m per QALY.[69] In recognition of the fact that the QALY does not capture all aspects of value, broader value determinants such as productivity are currently under investigation.[70]

It took seven years of debate for HTA to be introduced into Japan and care was taken to avoid causing any delay to listing medicines and to ensure that the HTA approach was complementary to the current pricing system.[71] Despite being heavily influenced by the approach used by NICE, the Japanese application is unique. HTA is typically considered to include a combination of methodological assessment with deliberative appraisal. Deliberative appraisal considers ethics, broader societal impacts, patient input and an overall sense-check on the result. Final decision making, or appraisal, considers both the cost-effectiveness assessment and the appropriate threshold for the ICER value.

[66] *Kamae I et al. 2020. Health technology assessment in Japan: a work in progress. Journal of Medical Economics 23 (4): 317-322.*
[67] *Hasegawa M et al. 2020. Formal implementation of cost-effectiveness evaluations in Japan: a unique health technology assessment system. Value in Health 23(1): 43-51.*
[68] *Center for Outcomes Research and Economic Evaluation for Health (COREEH). 2022. Guideline for preparing cost-effectiveness evaluation to the Central Social Insurance Medical Council. Version 3.0.*
[69] *Hasegawa et al. 2020.*
[70] *COREEH 2022.*
[71] *Kamae et al. 2020.*

Funded pre-registration and compassionate access in France

Reforms to French early access programs in 2021 evolved one of the most comprehensive and longest-standing early access programs in the world. The remit of the Haute Autorité de Sante (HAS; Health Authority) was expanded to encompass decision making regarding early and compassionate access. Prices under both programs are freely set by the manufacturer, although rebates generally apply at the end of the AP coverage period, when full HTA and price negotiations are completed.

The main changes introduced through this reform were to:

- Streamline six pre-existing pathways into two, reducing complexity
- Expand eligibility to include disabling, as well as life-saving, conditions
- Introduce "presumption of innovation" criteria, defined as a novel treatment, likely to be a substantial or major contribution to the evolution of care in France, a minimum level of clinical data depending on the stage of development, and few uncertainties in tolerance – which aligns with, and therefore prioritises, therapies that are more likely to be successful in HTA
- Make the rebates that apply at the end of the coverage period provisional, calculated on a progressive scale of turnover, to enable better manage the impact on the manufacturer and the public budget
- Publish guidelines for the collection of high quality real world evidence in French patients during the early or compassionate access period, which may then be used to inform HTA
- Introduce a target of 80 days for decisions regarding listing on either pathway.[72]

Table 2: Early access (accès précoce) and compassionate access (accès compassionel) in France

	AP (accès précoce; early access)	AC (accès compassionel; compassionate access)	
		Compassionate use	Compassionate prescription
Description	• Early access to drugs for serious, rare and disabling diseases, at the request of the manufacturer	• Access to drugs not intended to be marketed, at the request of the prescriber	• Access to drugs not intended to be marketed *in that indication*, at the request of the regulator (ANSM)
Coverage	• Pre-marketing authorisation • Post- marketing authorisation (for the gap between pre-marketing authorisation and full listing)	• Compassionate use authorisation	• Compassionate prescription scheme
Eligibility criteria	• There is no appropriate treatment • The initiation of the treatment cannot be deferred • The efficacy and safety of the medicinal product are strongly presumed based on the results of clinical trials	• Product not marketed in France • There is no appropriate treatment • Efficacy and safety presumed	• Product not marketed in that indication in France • There is no appropriate treatment • Efficacy and safety presumed

[72] *HAS. 2021. Accès précoce et compassionnel aux médicaments: quels changements pour les industriels ?, https://www.has-sante.fr/upload/docs/application/pdf/2021-07/acces_precoces_-_support_webinaire.pdf, accessed 20 February 2023.*

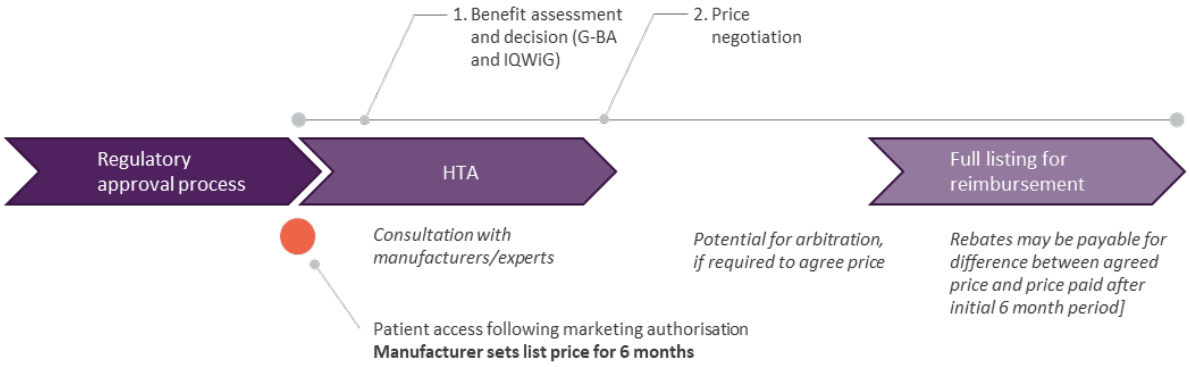
	• This medicinal product is presumed to be innovative, notably compared with a clinically relevant comparator		
Duration	• 1 year, renewable	• 1 year, renewable	• 3 years, renewable
Pricing	• Free pricing set by manufacturer	• Annual flat price per patient or set by manufacturer	• Same price as product for in-market indication
Rebates	• Provisional rebates defined by a progressive scale of turnover • Mandatory rebates may apply if there is a delay in the reimbursement submission	• Price cap may apply (rebates for amounts above cap)	
Data collection	• Manufacturer must agree to fund data collection meeting the specification of the HAS and ANSM		
Decision time	• 80 day target		

Adapted from HAS 2021[73].

Interim access while HTA is conducted in Germany

In Germany, the Arzneimittelmarkt-Neuordnungsgesetz (AMNOG) (Pharmaceuticals Market Reorganisation Act) process enables automatic listing of new therapies, following receipt of marketing authorisation (from the European Medicines Agency, EMA) and while HTA and pricing negotiations are completed. Products are listed at the manufacturer's price for six months. If the listing process takes longer than six months, the manufacturer may have to rebate any amounts paid above the final negotiated price to insurance funds. The AMNOG process affords German patients rapid, reimbursed access to new therapies.

Figure 18: Overview of German approach to negotiating prices for new medicines



Source: Commonwealth Fund[74].

Managing uncertainty and expenditure with England's Cancer Drugs Fund and Innovative Medicines Fund

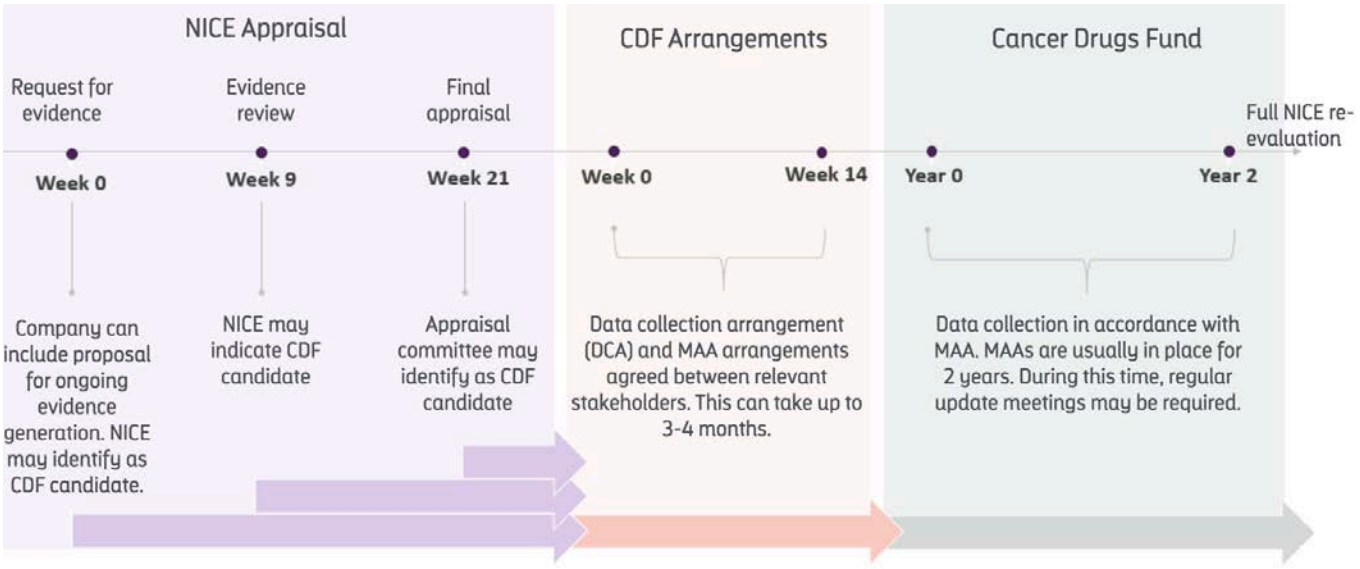
The Cancer Drugs Fund (CDF) and Innovative Medicines Fund (IMF) follow similar principles to accelerate patient access to promising, innovative therapies. In general, new therapies are eligible to be considered for these pathways where a preliminary assessment by the National Institute for Health and Care Excellence (NICE) finds that the therapy could be suitable for routine commissioning, although there is some uncertainty in the available clinical trial evidence. This may occur where a therapy has received marketing authorisation through a provisional approval pathway.

[73] *HAS 2021.*

[74] *Commonwealth Fund. 2019. How Drug Prices are Negotiated in Germany, https://www.commonwealthfund.org/blog/2019/how-drug-prices-are-negotiated-germany, accessed 20 February 2023.*

All therapies are funded under a managed access agreement, which includes a confidential commercial agreement and data collection arrangement. Data collection is designed to address key areas of uncertainty highlighted by NICE in a preliminary appraisal and is then utilised in a full appraisal for routine commissioning. The data may include data being gathered in ongoing clinical trials as well as real world data. The period of the managed access agreement is determined based on the length of time required to gather data to address the uncertainty.75 A full evaluation for routine commissioning is required at the end of the managed access period, generally after two years.

Figure 19: Overview of the CDF listing process



Source: NICE[76].

More than 14,000 patients received treatment via the CDF in 2020-21, at a total cost of £336m.77 The CDF has operated since 2010, although it was substantially reformed in 2016 in response to criticisms regarding sustainability of expenditure, efficient processes and prioritisation of funding to the most promising therapies. Key to the success of this reform was bringing the CDF under the remit of NICE, such that it was no longer perceived as a "work-around" and is now a key component of the subsequent HTA and commissioning processes, generating real world data for consideration in these processes.

The IMF was then established in 2021 to extend funded managed access to non-cancer conditions, thereby enhancing equity across all patients. Funding is targeted to "the most promising medicines for which there is significant remaining uncertainty around the level of clinical benefit and cost-effectiveness".[78]

Total budgets for both the CDF and IMF are each capped at £340m per year. Expenditure is managed using a combination of: (i) confidential discounts in commercial agreements; and (ii) rebates across the entire fund at the end of this year if the budget cap is exceeded.[79] This approach provides scope to continue to list new therapies.

[75] *NICE. 2022. The Innovative Medicines Fund Principles, https://www.england.nhs.uk/wp-content/uploads/2022/06/B1686-the-innovate-medicines-fund-principles-june-2022.pdf, accessed 20 February 2023.*
[76] *NICE 2022. Managed Access, https://www.nice.org.uk/about/what-we-do/our-programmes/managed-access, accessed 20 February 2023.*
[77] *NHS. 2021. Cancer Drugs Fund Activity Update, https://www.england.nhs.uk/wp-content/uploads/2018/01/cdf-q4-20-21-activity-update.pdf, accessed 20 February 2023.*
[78] *NHS. 2016. Appraisal and Funding of Cancer Drugs from July 2016 (including the new Cancer Drugs Fund), https://www.england.nhs.uk/wp-content/uploads/2013/04/cdf-sop.pdf, accessed 20 February 2023.*
[79] *NHS 2016.*

Concluding statements

Reforms to the structure and pricing of the PBS since 2005 have stabilised growth in expenditure. Treasury projections indicate that medicines and vaccines will make up a shrinking share of total health expenditure over the next ten years and into the future. There is a risk that stringent requirements to demonstrate cost-effectiveness will go too far and result in under-investment.

HTA systems internationally face challenges from the step-change in technological advances that have been brought about by precision medicines. New regulatory pathways aim to speed up marketing authorisation of new medicines and vaccines. At the same time, the COVID-19 pandemic has indicated that the Australian population expects to be able to access new medicines and vaccines that have marketing authorisation in a timely manner.

Maintaining our international competitiveness is important. This will require a detailed and strategic consideration of policy settings, and how we compare to some of the world's leading markets for patient access to new technologies. Clearly, it remains critical to consider the sustainability of any new expenditure, but policy makers should have confidence in the now in-built stabilisers (in particular, statutory price reductions and price disclosure) of PBS expenditure.

A renewed commitment to invest in new medicines and vaccines appropriately in the future will help improve Australians' health outcomes and drive productivity and growth in the economy in the post-pandemic development of the health system and the 21st century.

Appendix: Valuation of medicines and vaccines in HTA

- HTA, conducted by the PBAC, is used to inform decisions about funding new medicines and vaccines, via the PBS and NIP, respectively. The PBAC must be convinced that the proposed listing is cost-effective, i.e., represents value for money, in order to make a positive recommendation for listing.
- The incremental cost effectiveness ratio (ICER) is used to derive an objective comparison of the cost-effectiveness of medicines and vaccines, across different therapeutic areas and conditions, and associated with different benefits for patients and/or the healthcare system. The ICER measures value for money in terms of quality-adjusted life years (QALYs).
- Medicines and vaccines with an ICER below an implicit threshold value of $45,000-$75,000 per QALY are more likely to be recommended by the PBAC. The implication is that this represents the opportunity cost of expenditure elsewhere in the healthcare system.[1]
- The PBAC's acceptable ICER threshold value has not changed in more than two decades and is out of step with valuations of life used by other Australian Government departments. By comparison, the value of a statistical life year (VSLY) used by the Office of Best Practice Regulation (OBPR) to inform funding decisions in areas such as transport and environmental policy is $222,000 in 2021.
- There is no universal agreement on the appropriate ICER threshold value, and HTA agencies in other countries use different thresholds than the PBAC.[1] The WHO has suggested a rule of thumb that the ICER threshold should reflect approximately one to three times GDP per capita (approximately $60,000-$180,000 in Australia), but that cost-effectiveness analysis should just be one aspect that informs a funding decision.[1]

The ICER threshold

The acceptable range for the ICER in Australia, in general, is understood to be up to approximately A$45,000 – A$75,000 per QALY, although this is not formally documented in the PBAC guidelines. The PBAC does have discretion to recommend applications with ICERs above this threshold value, with consideration to other factors (Table 3).

Public Summary Documents (PSDs), which provide an overview of the key considerations and drivers for the PBAC's decisions, suggest that an ICER as high as A$200,000 may be acceptable for rare diseases where there is a high and acute unmet need, and as low as A$15,000 for vaccines, due to the uncertainty of the value of broad, preventative public health interventions.

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[80] *Sampson C, et al. 2022. Supply-Side Cost-Effectiveness Thresholds: Questions for Evidence-Based Policy. Appl Health Econ Health Policy. Sep;20(5):651-667. doi: 10.1007/s40258-022-00730-3. Epub 2022 Jun 7.*

[81] *Sampson C, et al. 2022.*

[82] *Bertram, M., Lauer, J., Stenberg, K., Edejer, T. 2021. 'Methods for the Economic Evaluation of Health Care Interventions for Priority Setting in the Health System: An Update From WHO CHOICE', International Journal of Health Policy and Management, 10(Special Issue on WHO-CHOICE Update), pp. 673-677. doi: 10.34172/ijhpm.2020.244*

The PBAC's ICER threshold is intended as a measure of the 'opportunity cost' of spending money elsewhere in the healthcare system. There is a lack of empirical evidence to support this, however, and it has been referred to as a "yardstick" value[83]. In practice, it serves as a pricing benchmark within just the PBS (and NIP), which has been constrained to the same value over the past two decades, while prices elsewhere in the economy have more than doubled.[84,85]

Affordability is also a key consideration for the PBAC, although the PBS is ostensibly an uncapped program, and attention is paid to managing growth in expenditure. PSDs indicate that submissions with an expected budget impact above $10 million in any of the first four years of listing receive more scrutiny.[86] Since the Australian Government has agreed to list products that have been recommended by the PBAC through successive strategic agreements with the pharmaceutical industry, this role for the PBAC has become increasingly pivotal in controlling new expenditure on the PBS.

Table 3: Quantitative and qualitative factors that may influence PBAC recommendations

Quantitative	**Qualitative**
• *Comparative health gain* • *Comparative cost-effectiveness* • *Patient affordability in the absence of PBS subsidy* • *Predicted use in practice* • *Financial implications for the PBS* • *Financial implications for the Australian government health budget*	• Overall confidence in evidence and assumptions in submission • Equity • Presence of effective therapeutic alternatives • Severity of medical condition treated • Ability to target therapy with the proposed medicine precisely and effectively to patients likely to benefit most • Public health issues • Any other relevant factor

Source: PBAC[87].

How does Australia's willingness-to-pay (WTP) threshold compare with other countries?

Several international HTA agencies utilise a similar approach to the PBAC that includes the estimation of ICERs as a core decision metric for listing. Though not all countries employ a formal WTP threshold, the valuation of a QALY, when adjusted for inflation and purchasing power parity (PPP) to 2015 USD, can range from as little as 4,419 USD per QALY gained in Thailand to 173,971 USD per QALY gained in Norway.88 England's NICE, a world-leader in development of HTA approaches, states an explicit threshold of £20,000-£30,000/QALY (see box).

There is no universal agreement on the appropriate ICER threshold value, and HTA agencies in other countries use different thresholds than the PBAC.[89] The World Health Organisation (WHO; WHO-CHOICE program) has suggested a rule of thumb that the ICER threshold should reflect approximately one to three

[83] *Abelson. 2008. Establishing a Monetary Value for Lives Saved: Issues and Controversies, https://obpr.pmc.gov.au/sites/default/files/2021-06/Working_paper_2_Peter_Abelson.pdf, accessed 13 September 2022.*
[84] *Cubi-Molla et al. 2021. Resource Allocation in Public Sector Programmes: Does the Value of a Life Differ Between Governmental Departments?, https://www.ohe.org/publications/resource-allocation-public-sector-programmes-does-value-life-differ-between#, accessed 13 September 2022.*
[85] *RBA. 2022. Consumer Price Index. https://www.rba.gov.au/inflation/measures-cpi.html, accessed 13 September 2022.*
[86] *Ghabri, S., & Mauskopf, J. 2018. The use of budget impact analysis in the economic evaluation of new medicines in Australia, England, France and the United States: relationship to cost-effectiveness analysis and methodological challenges. The European Journal of Health Economics, 19(2), 173–175.*
[87] *PBAC 2022. Guidelines, https://pbac.pbs.gov.au/information/about-the-guidelines.html, accessed 13 September 2022.*
88 Schwarzer, R., et al. 2015. Systematic overview of cost-effectiveness thresholds in ten countries across four continents. Journal of comparative effectiveness research, 4(5), 485–504. https://doi.org/10.2217/cer.15.38
[89] *Sampson C, et al. 2022. Supply-Side Cost-Effectiveness Thresholds: Questions for Evidence-Based Policy. Appl Health Econ Health Policy. Sep;20(5):651-667. doi: 10.1007/s40258-022-00730-3. Epub 2022 Jun 7.*

times GDP per capita (approximately $60,000-$180,000 in Australia), but that cost-effectiveness analysis should just be one aspect that informs a funding decision. It warns against using a threshold value as a decision rule.[90]

NICE WTP threshold and modifiers (England, UK)[91]

NICE WTP threshold and modifiers (England, UK)[91]

'NICE's recommendations should not be based on evidence of costs and benefit alone. We must take into account other factors when developing our guidance. We also recognise that decisions about a person's care are often sensitive to their preferences.'

- NICE principle 7 (2020

NICE uses an explicit WTP threshold of £20,000-£30,000 per QALY when considering a recommendation to list a new technology for use in the National Health Service (NHS).[1] This quantitative assessment is considered alongside qualitative factors, as well as patient views and preferences.

Modifiers are factors that may affect NICE's decisions, in the context of the WTP threshold. A factor is considered a modifier if it has not (and cannot) been included in the QALY estimate and is a value judgement. It must therefore reflect societal values regarding priority setting in healthcare decisions.

A quantitative modifier may be applied such that it weights, or increases, the value of the QALYs estimated, or (equivalently) increases the threshold value of the acceptable ICER. This means that the NHS will pay more for the new technology.

In principle, NICE's decisions aim to maximise total health gains to the UK public from investing in new technologies. Therefore, QALYs should be treated as equivalent across different therapeutic areas and patient groups, and the goal of HTA should be to maximise total QALYs across the population.

Modifiers, on the other hand, prioritise specific circumstances over total health gains across the population. These specific circumstances are deemed to justify "deviating from the reference case in which all health-related benefits are valued the same irrespective of any other considerations", as determined from "a moral and ethical reflection supported by reason, coherence and available evidence". This imposes an opportunity cost on the overall NHS budget. It is therefore critical, according to NICE, that the use of modifiers be transparent and informed by the preferences of the UK public wherever possible.

During the recent *NICE Methods Review*, a range of potential modifiers were considered for adoption. The framework used to assess the case for change for each modifier was heavily influenced by the moral case, evidence of importance to the public and the NHS. Disease severity was recommended as a new modifier, and the previously applied "end of life" criteria were removed as a modifier.

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[90] *Bertram, M., Lauer, J., Stenberg, K., Edejer, T. 2021. 'Methods for the Economic Evaluation of Health Care Interventions for Priority Setting in the Health System: An Update From WHO CHOICE', International Journal of Health Policy and Management, 10(Special Issue on WHO-CHOICE Update), pp. 673-677. doi: 10.34172/ijhpm.2020.244*

[91] *NICE 2020, CHTE methods review, Modifiers Task and finish group report, October 2020, https://www.nice.org.uk%2FMedia%2FDefault%2FAbout%2Fwhat-we-do%2Four-programmes%2Fnice-guidance%2Fchte-methods-consultation%2FModifiers-task-and-finish-group-report.docx&usg=AOvVaw3KABVdzXtk8EDLtNDeA4lM, accessed 13 September 2022.*

92 NICE. Our principles, https://www.nice.org.uk/about/who-we-are/our-principles, accessed 13 September 2022.

Table 4: International ICER thresholds[93]

	HTA Agency	ICER range (per QALY)	ICER range in A$[94]	Method for determining acceptable ICER threshold
Australia	PBAC	No explicit ICER threshold, but implied ICER range is A$52,400 - A$75,000	A$52,400 - A$75,000	▪ Not specified
New Zealand	PHARMAC	NZ$33,306	A$32,017	▪ Not specified
England	NICE	£20,000 - £30,000	A$41,467 – A$62,201	▪ Not specified ▪ Modifiers (higher thresholds may be applied for severity and ultra-rare diseases)
Netherlands	ZIN	€20,000 - €80,000	A$36,527 – A$146,109	▪ ZIN assesses new medicine based on cost-effectiveness and takes a societal perspective in their analyses. The threshold depends on the severity of the disease – proportional shortfall (PS) - bound between 0 (no disease-related QALY loss) and 1 (immediate death because of the disease).[95]
Canada	CADTH	No explicit threshold, but implied health ICER range is CA$50,000 – CA$100,000	A$59,693 – A$119,386	▪ Not specified
Japan	Central Social Insurance Medical Council (Chuikyo)	No explicit threshold. Implied health ICER range for normal treatments is ¥5,000,000 - ¥10,000,000 and range for products with special considerations is ¥7,500,000 - ¥15,000,000	A$77,340 – A$154,680 Special considerations: A$116,010 – A$232,020	▪ Chuikyo employs cost-effectiveness analysis with QALYs as the measure of benefit – the results used to adjust prices of pharmaceuticals and medical devices instead of rationale for reimbursement. Special considerations apply to rare diseases products that have insufficient alternatives, paediatric conditions and anti-cancer drugs.
South Korea	HIRA	No explicit threshold. Understood to be around 25 million KRW	A$43,826	▪ ICER threshold is tied to the country's GDP per capita. Up to 50 million KRW (A$87,652) flexibility for threshold for limited number of drugs for cancer and rare diseases.

[93] *Cubi-Molla et al. 2021.*

[94] *CCEMG - EPPI-Centre Cost Converter, http://eppi.ioe.ac.uk/costconversion/default.aspx (Cost converted to 2022 AUD using CCEMG-EPPI-Centre cost converter) , accessed 13 September 2022.*

[95] *Zorginstituut Nederland. Cost-effectiveness in practice, https://english.zorginstituutnederland.nl/publications/reports/2015/06/16/cost-effectiveness-in-practice, accessed 13 September 2022.*

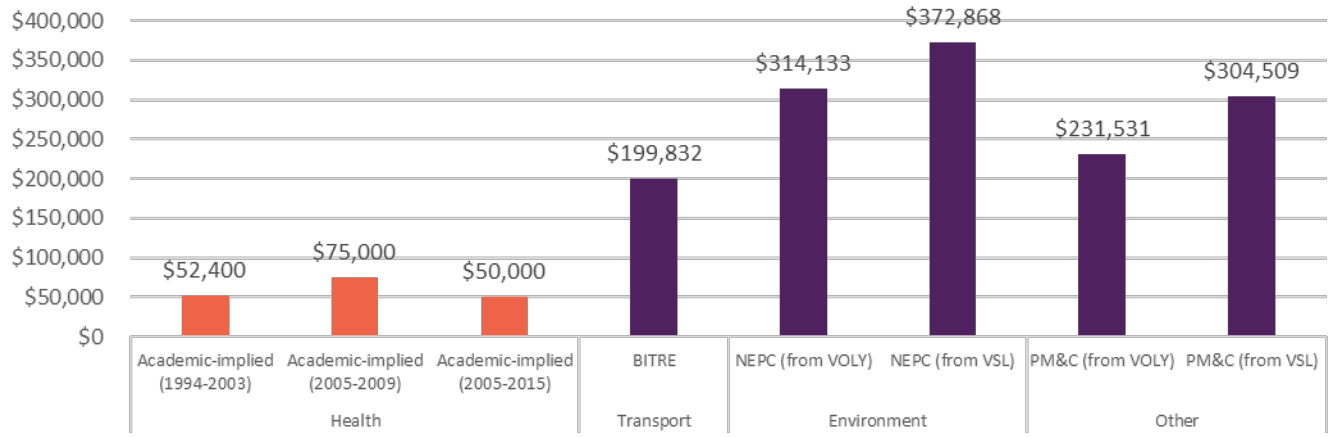
Valuations of life used in other Australian Government decisions

The Office of Best Practice Regulation (OBPR) estimates the value of a statistical life in Australia is A$5.1 million and the value of statistical life year is A$222,000, in 2021 dollars. This estimate underpins key investment decisions in areas such as public health measures, new roads and infrastructure.

VSL is an estimate of the value society places on reducing risks of death and VLY is an estimate of the value society places on a year of life. The OBPR's 2021 valuation is linked to a 2007 estimate (Abelson 2007[96]), which was taken from European studies, converted to Australian dollars and then inflated at the wage price index. While there are important differences in the derivation of the VLY/VSLY reported by the OBPR, compared with the PBAC's ICER threshold, both are now accepted and embedded into decision making to allocate public funds towards saving and improving the lives of Australians.

A recent study found wide variation between valuations of life years used by different Australian Government departments, as shown in Figure 20. It is common across countries, including the UK, Canada, Netherlands, Japan and New Zealand for HTA (and reimbursement funding decisions) to use a much lower value than in areas such as transport and environment. These large discrepancies result from differences in the approach and methodology used to value life (see box).[97]

Figure 20: Overview of value of life years estimated for Australia and used by Australian Government departments



Source: Cubi-Molla et al. 2021.

[96] *Abelson 2008*
[97] *Cubi-Molla et al. 2021.*

Methods for valuing life[98]

The **value of a statistical life** (VSL), is a common approach and may be estimated using different methods

- Human capital approach
- Revealed preferences, e.g., the wage-risk approach (also referred to as the wage differential or labour market method)
- Stated preferences, such as the willingness-to-pay (WTP, also referred to as contingent valuation) approach. This involves asking a sample of participants to each state their WTP for a small risk reduction, which is then translated into an overall VSL estimate. It should not be interpreted as the amount that would be paid to save a life, as this is considered to be priceless.

In HTA, valuation of a quality-adjusted life-year (QALY) may be derived by either estimating opportunity cost (supply-side) or WTP (demand-side) approach. Opportunity costs are frequently represented as a "cost-effectiveness threshold", as utilised by the PBAC. Importantly, PBAC's approach does not reflect society's WTP. One Australian study has actually estimated a lower reference (opportunity) cost (approximately $28,000 in 2018) than the threshold value used by the PBAC.99

Abbreviations

[98] *Cubi-Molla et al. 2021.*
[99] *Edney LC, Haji Ali Afzali H, Cheng TC, Karnon J. 2018. Estimating the Reference Incremental Cost-Effectiveness Ratio for the Australian Health System. Pharmacoeconomics. 36(2):239-252. doi: 10.1007/s40273-017-0585-2.*

AC	Accès Compassionel (France)
AP	Accès Précoce (France)
AMNOG	Arzneimittelmarkt-Neuordnungsgesetz (Germany)
ATAGI	Australian Technical Advisory Group on Immunisation
C2H	Centre of Outcomes Research and Economic Evaluation for Health (Japan)
CADTH	Canadian Agency for Drugs and Technologies in Health
CDF	Cancer Drugs Fund (UK)
CF	Cystic Fibrosis
CHTE	Centre for Health Technology Evaluation (UK)
CIRS	Centre for Innovation in Regulatory Science
CPI	Consumer Price Index
DA	Diagnostics Advisory
EMA	European Medicines Agency
FIC	First-In-Class Medicine
FNB	First New Brand
G-BA	Federal Joint Committee (Germany)
GDP	Gross Domestic Product
HAS	Haute Autorité de Sante (France)
HST	Highly Specialised Technologies
HTA	Health Technology Assessment
ICER	Incremental Cost-Effectiveness Ratio
IMF	Innovative Medicines Fund
IQWiG	Institute for Quality and Efficiency in Health Care (Germany)
MBS	Medicare Benefits Schedule
NHS	National Health Service (UK)
NICE	National Institute for Health and Care Excellence (England)
NME	New Molecular Entity
NMP	National Medicines Policy
OBPR	Office of Best Practice Regulation
OECD	Organisation for Economic Co-operation and Development
PBAC	Pharmaceutical Benefits Advisory Committee
PBS	Pharmaceutical Benefits Scheme
PHARMAC	Pharmaceutical Management Agency (New Zealand)
PMP	Price Maintenance Premium (Japan)
QALY	Quality-adjusted life year
R&D	Research and Development
SMA	Spinal Muscular Atrophy
SPR	Statutory Price Reduction
TA	Technology Appraisal
TGA	Therapeutic Goods Administration
UK	United Kingdom
US	United States
VLY	Value of a Statistical Life Year
VSL	Value of a Statistical Life
WHO	World Health Organisation
WTP	Willingness to Pay
ZIN	National Healthcare Institute (Netherlands)