White Paper

All Too Rare

The PBS access gap for the two million Australians with a rare disease

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Executive summary

The *All Too Rare* report examines patient access to reimbursed treatment for rare diseases in Australia between 2018 and 2025. These are defined as orphan drugs, medicines for treating rare conditions.

Drawing on Department of Health, Disability and Ageing data on the registration and reimbursement status of medicines, this report tracks the evolution of regulatory designations and approvals, and the reimbursement status of rare disease medicines in Australia.

In addition, the analysis is supplemented by benchmarking Australia against fourteen comparator countries to understand access challenges Australians face, as well as three case studies of rare disease medicines.

The trends observed:

- Only 39% of medicines granted orphan drug designation over the analysis period have progressed to reimbursement
- The average time from registration to reimbursement was 22 months
- 78% of reimbursed rare disease medicines required more than one HTA submission before a positive recommendation, and 35% required more than two
- Australia ranks 14 out of 15 comparator countries by number of rare disease medicines funded over the last 10 years

Between January 2018 and December 2025

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Background

In Australia, a rare disease is defined as a condition that affects less than five in 10,000 people.[2] However, with an estimated 7,000 rare diseases, collectively they have a significant impact, with estimates suggesting approximately 2 million Australians — 8% of the total population — live with one.[2] Only 5% of rare diseases have an effective treatment.[13]

Australia has a dedicated framework for medicines that treat rare disease. The TGA's Orphan Drug Program was first introduced in 1997 and reformed in 2017.[1,3] It supports the development and registration of medicines for small populations that would otherwise not be commercially viable without a waiver of the registration fee. The Pharmaceutical Benefits Advisory Committee (PBAC) also subsidises the first submission to achieve a PBS listing with a fee waiver provided the submission is made within 12 months of TGA approval.[4]

Scope and methodology

This report is an update of IQVIA's 2023 report "*Access to Orphan Drugs in Australia*" and includes:

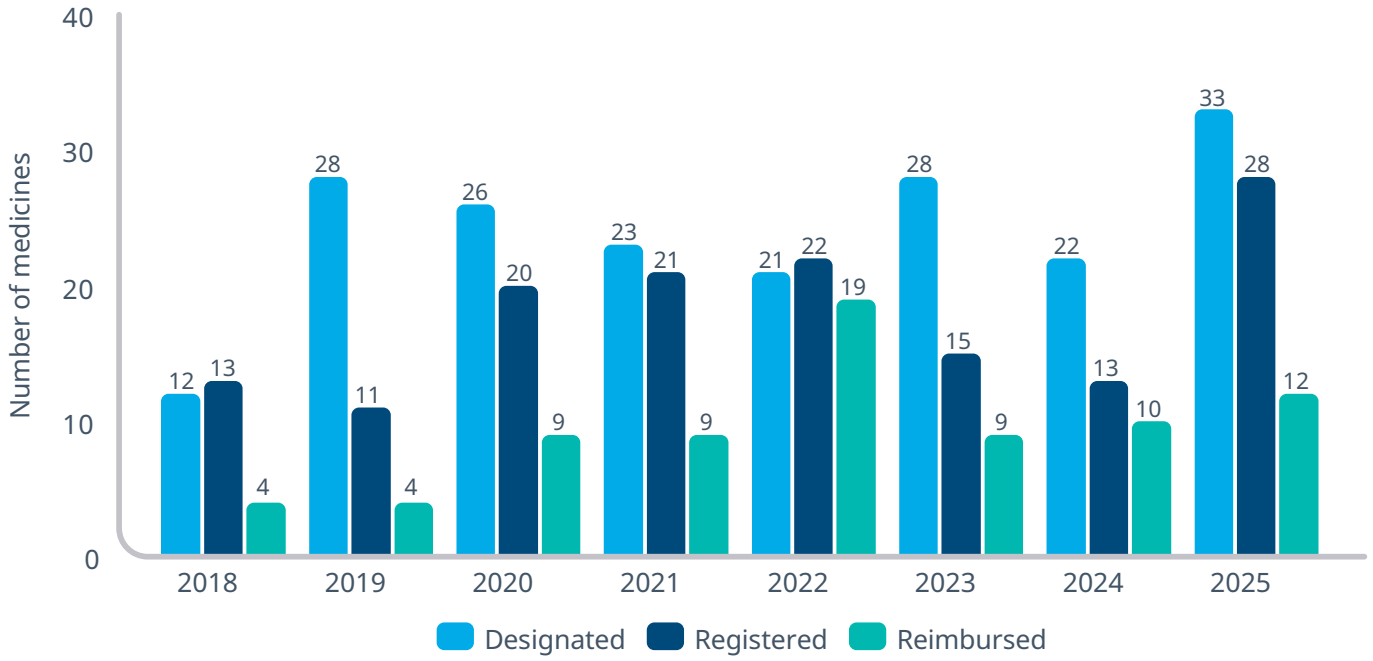
- Tracking of prescription medicine designation, approval and reimbursement timelines as published by TGA and PBS between 2018 and 2025
  - » Medicines funded via the PBS, Life Savings Drugs Program (LSDP), the Medicare Benefits Schedule (MBS), Highly Specialised Therapies (HST) Program under the National Health Reform Program (NHRA) are considered within the scope of this analysis[5]
  - » For medicines with multiple indications, each indication designated as orphan was tracked for the purpose of this analysis
- Benchmarking Australia
  - » Tracking of reimbursement status for New Molecular Entities (NME) reimbursed in 14 comparator countries between 2016-2025: Australia, Austria, Belgium, Canada, France, Germany, Ireland, Italy, Japan, Netherlands, Norway, Spain, Sweden, Switzerland, and the United Kingdom
  - » An NME is defined as an innovative pharmaceutical medicine that contains a new moiety or molecule not previously approved in any country
  - » Only the first indication is considered for registration and public funding
  - » To account for variations in orphan designation for a medicine by country, the Food and Drug Administration (FDA) designation from the United States of America was applied as an international standard for consistency
  - » Provinces in Canada reimburse therapies individually, and a medicine was considered reimbursed if funded for at least 50% of the population

Note: A medicine which received an orphan drug designation extension has been combined into one single designation

How has Orphan Drug registration and reimbursement evolved?

Between 2018 and 2025, on average, 24 medicines per year were designated orphan, with 18 moving towards TGA registration and 10 receiving reimbursement (Figure 1). This highlights the significant challenges faced by pharmaceutical sponsors with only 39% of medicines eventually reaching patients.

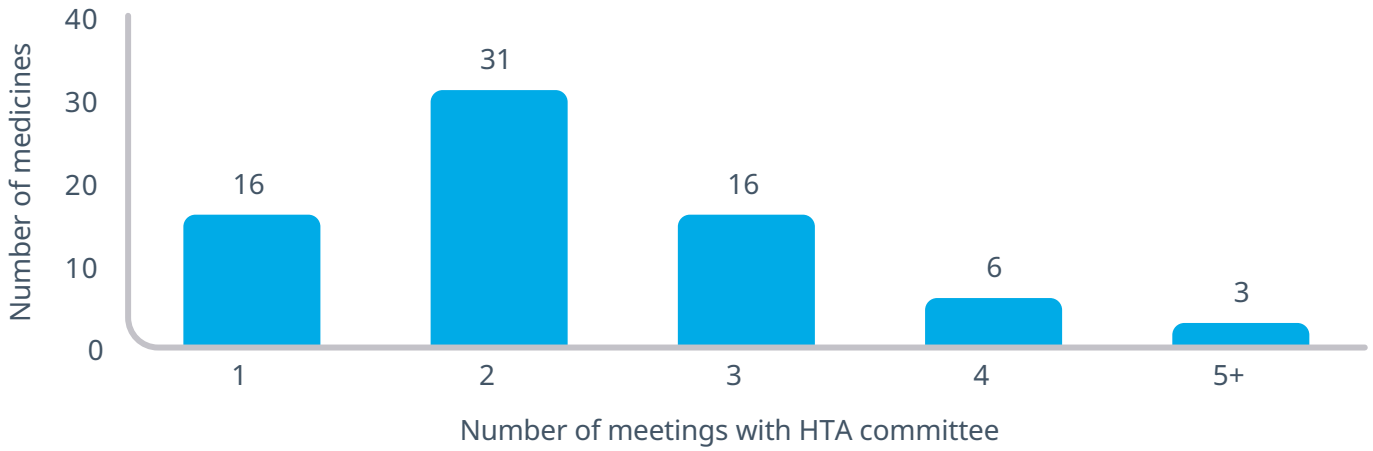
Figure 1. Rare Disease Medicine designation, registration and reimbursement by year



How many HTA meetings before a positive recommendation?

Only 16 (22%) medicines were recommended upon first submission, with 43% receiving a positive recommendation at 2nd meeting (Figure 2). Therefore, 78% of medicines could not take advantage of the fee waiver for HTA submission as they required resubmissions based on a negative outcome at the first meeting.*

Figure 2. Reimbursed Rare Disease Medicines by number of submissions before positive recommendation



*Includes 7 medicines funded via the LSDP where a condition for funding is rejection by the PBAC on first submission for lack of cost-effectiveness
Excludes 4 medicines assessed via MSAC

How long from registration to reimbursement?

The timeline from registration to reimbursement can be lengthy with only 13% of medicines being reimbursed within 6 months of registration (Figure 3). 17% of medicines took 7-12 months and another 20% took 13-18 months from registration for reimbursement. The average time to reimbursement for the analysis period was 22 months.

When comparing the average time to reimbursement since 2018 (Figure 4), it is even more evident that orphan medicines face lengthy time to access. The average time from registration to reimbursement was as high as 32 months in 2023, but has fallen to the overall average of 22 months in 2025.

Which therapy areas are seeing the most reimbursements?

Oncology and Haematology made up 43% of designations received from 2018-2025, however, their proportion reduces as medicines move towards reimbursement. Particularly, inherited metabolic disorders demonstrate a higher rate of reimbursement success relative to their orphan designation activity in recent years, with medicines such as Amvuttra™. receiving PBS listing for hATTR amyloidosis.

Figure 3. Distribution of reimbursed medicines by grouped months from registration to reimbursement

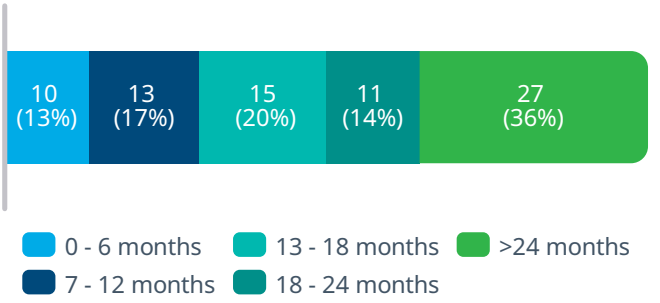


Figure 4. Average time (months) from registration to reimbursement by year

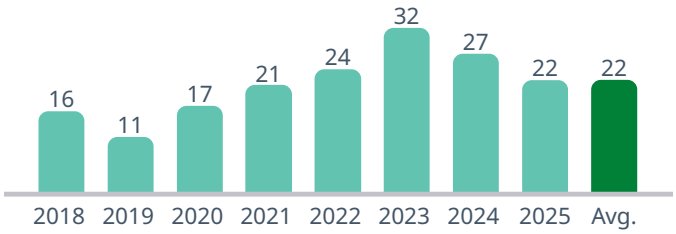
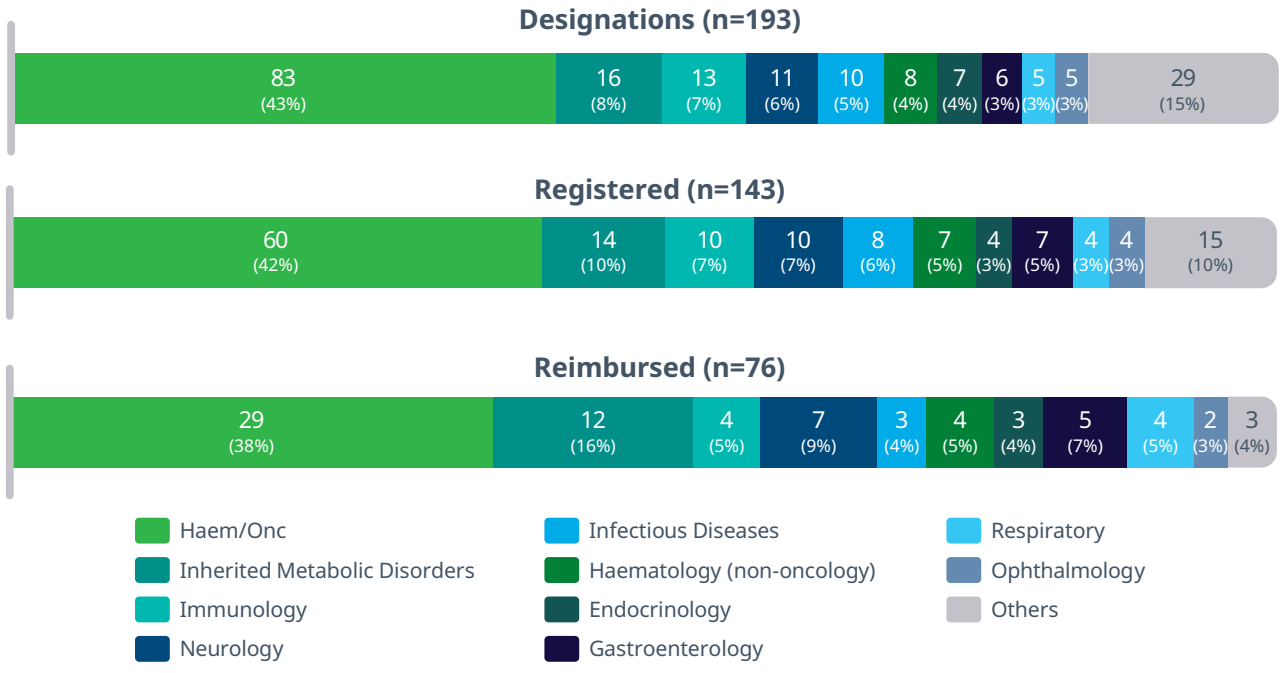


Figure 5. Distribution of rare disease medicines by therapy area

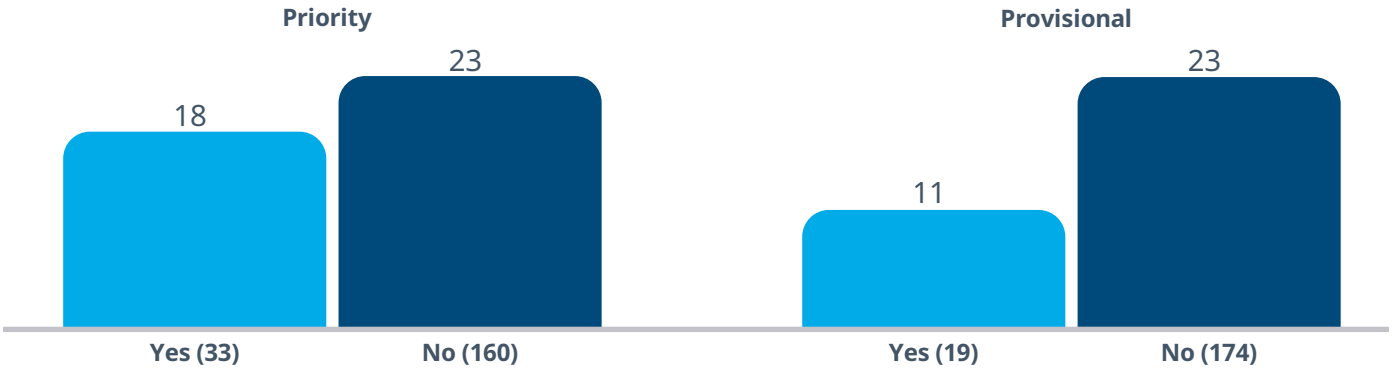


What is the impact of provisional and priority designations by the TGA?

A sponsor can apply for provisional approval and/or priority designation to expedite TGA regulatory review, which are independent of orphan designation. 33 of the 193 orphan designated drugs had priority designation, with 19 achieving reimbursement.

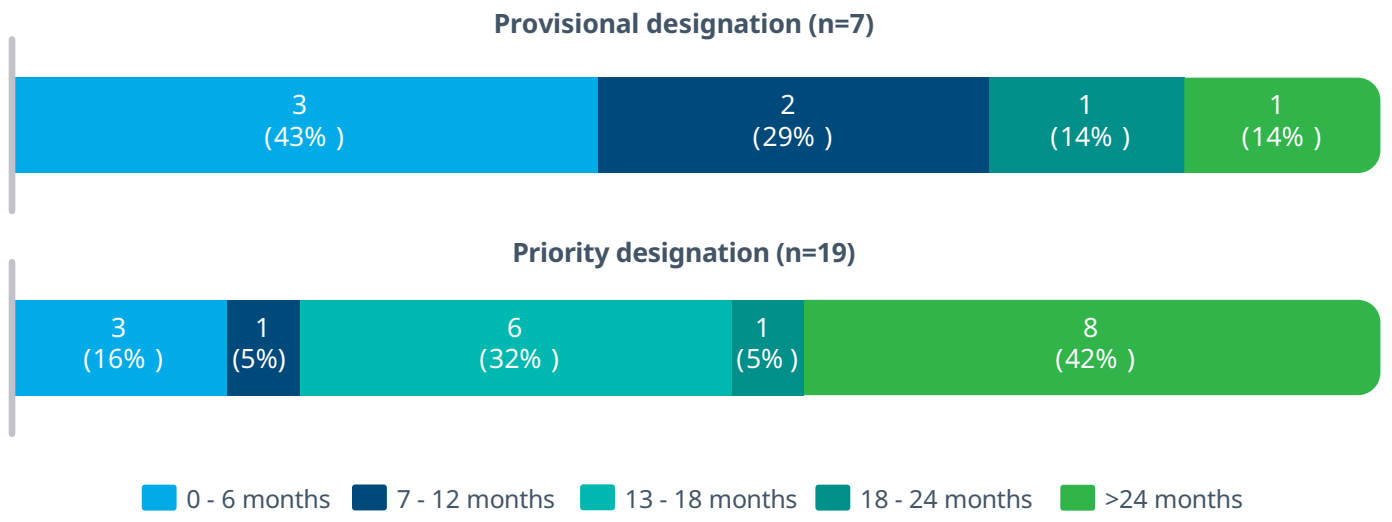
19 of 193 rare disease medicines had provisional approval, with 7 funded. Priority designated medicines took an average of 18 months to achieve reimbursement, faster than non-priority medicines with an average of 23 months. Medicines with provisional approval achieved reimbursement in 11 months, which was significantly quicker than medicines without provisional approval, which took an average of 23 months to get reimbursed.

Figure 6. Time from registration to reimbursement for rare disease medicines by priority and provisional designation (total number of orphan designated medicines)



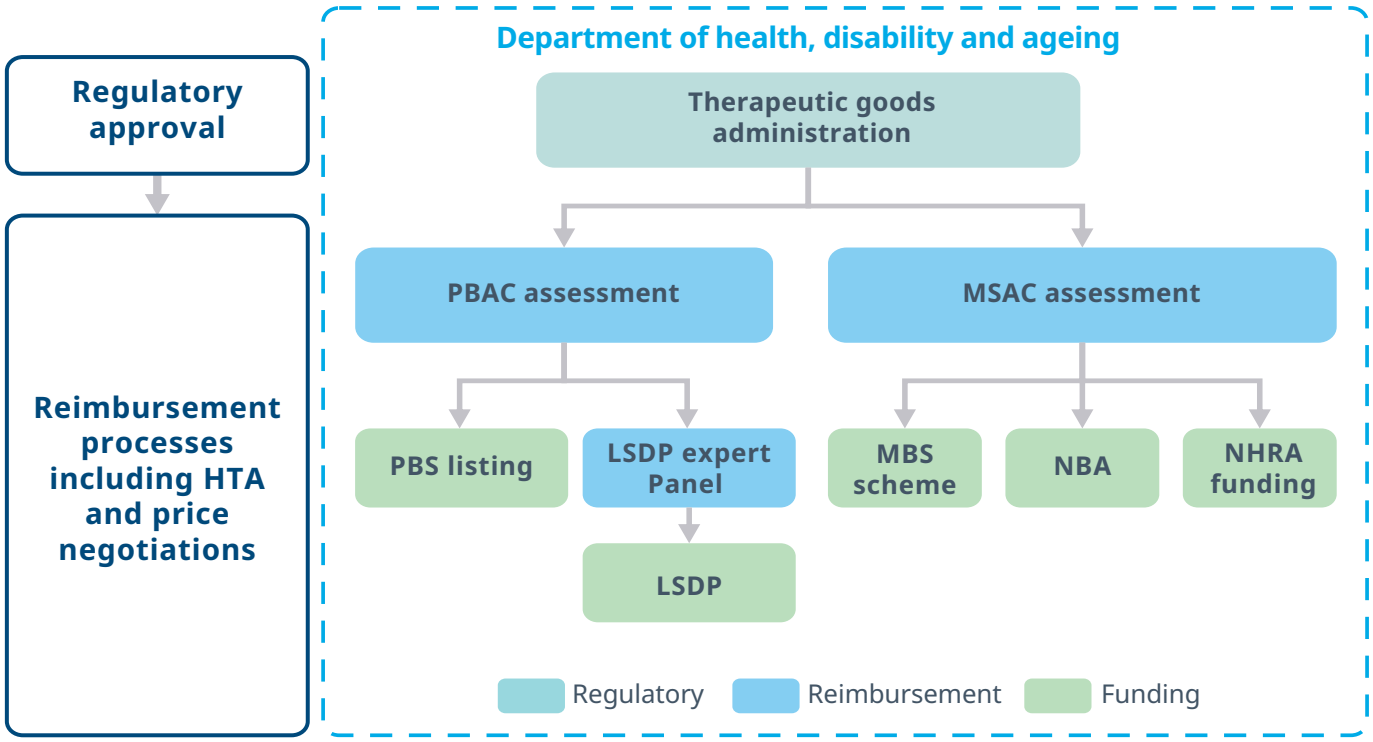
In addition, we explored the impact of the provisional and priority designations on grouped reimbursement timelines. 72% of rare disease medicines with a provisional designation achieved reimbursement within 1 year. In comparison, a priority designation did not expedite the reimbursement process to the same extent, with 37% of priority designated rare disease medicines taking 12-24 months to achieve reimbursement and 42% taking longer than 24 months.

Figure 7. Distribution of reimbursed medicines by grouped months from registration to reimbursement



How are rare disease medicines funded in Australia?

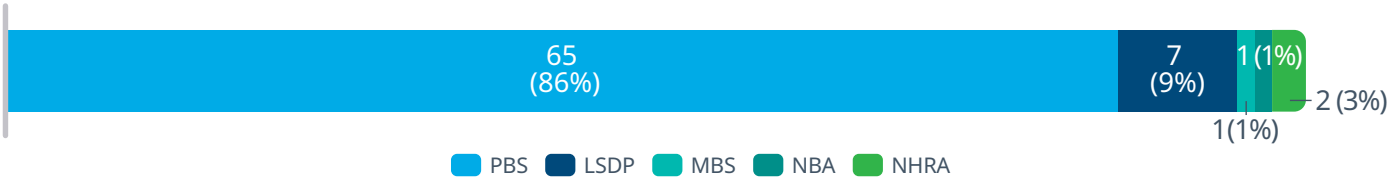
Figure 8. Funding mechanisms for rare disease medicines in Australia



Several funding schemes operate in Australia for the reimbursement of medicines[5]

- **PBS:** Primary funding scheme, under the National Health Act of 1953, the PBS only funds therapies classified as medicines (by TGA) and administered in outpatient settings (100% Commonwealth). The PBS has funded 86% of all reimbursed rare disease medicines (Figure 9)
- **LSDP:** Funds medicines for ultra-rare diseases (1 in 50,000), which have clinical efficacy but are not cost-effective, requires one rejection by PBAC for consideration of funding under the LSDP (100% Commonwealth). The LSDP funds 17 medicines, including 7 rare disease medicines since Jan 2018
- **MBS:** Funds medical services, such as a rare cancer radiopharmaceutical service
- **NBA:** Funds blood products and has been used to fund orphan therapies for haemophilia (Funding mix: 66% Commonwealth, 37% States and Territories)
- **NHRA:** Funds high-cost, highly specialised therapies (HST, >$200,000 per patient), which are either not classified as medicine and/or administered inpatient
  - » Funding Mix: 50% Commonwealth and 50% States and Territories
  - » Jurisdictions can choose not to fund treatment locally due to capability and capacity considerations

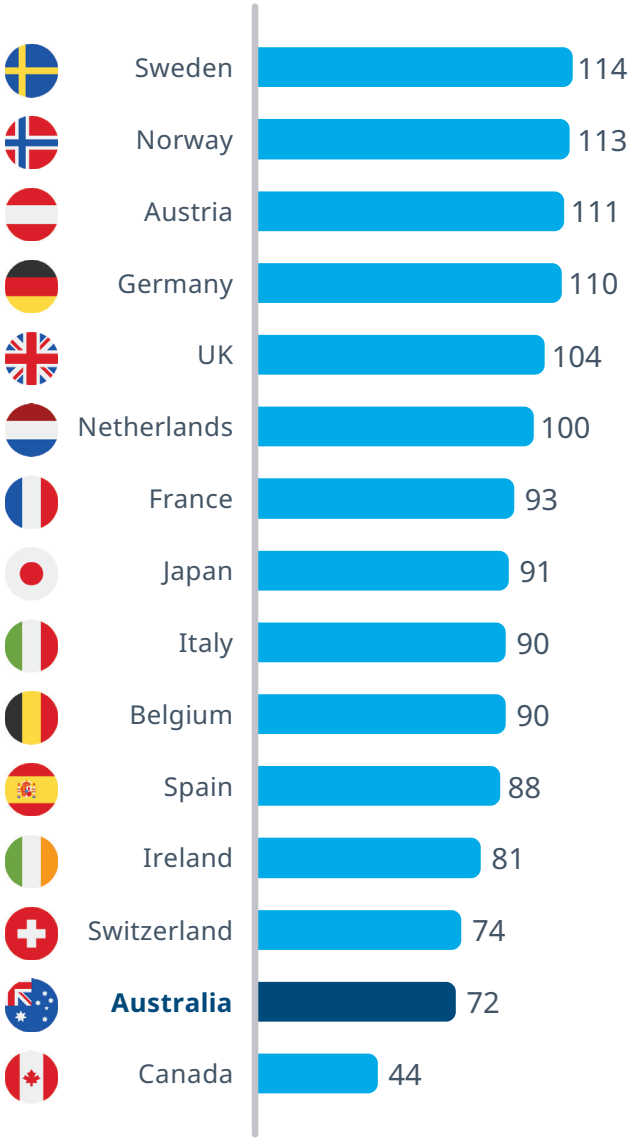
Figure 9. Distribution of funding pathways for orphan designated medicines (2018-2025)



How does Australia compare to other countries for funding rare disease medicines?

The number of rare disease medicines funded in Australia and 14 other comparator countries between 2016-2025 were compared to understand how well Australia is doing in enabling access to these orphan rare disease medicines.

Figure 10. Rare disease medicines funded in each country from 2016-2025



How does Australia compare?

Australia funds fewer rare disease medicines than most comparable countries in this analysis. From 2016–2025, Australia funded 72 orphan medicines, placing it near the bottom of the comparator group. This is well below countries such as the UK, France, Germany and Sweden, which funded between 93 and 114 orphan medicines over the same period.

What this means

This suggests that Australian patients with rare diseases may wait longer, or miss out altogether, on medicines that are publicly funded in similar health systems overseas. The gap is not just about how many medicines are approved by regulators. It reflects differences in how countries assess uncertainty, recognise value, and use special access pathways for rare disease medicines.

Note: Countries have different population sizes, health budgets, HTA pathways, pricing systems and decision-making processes. However, the comparison still shows a clear pattern: Australia's current approach appears more restrictive than many peer countries when it comes to funding orphan rare disease medicines.

Case study 1 — Palynziq™ (pegvaliase) for phenylketonuria

Protracted funding timelines for rare disease medicines

DISEASE OVERVIEW

Phenylketonuria (PKU) is a rare disease that causes the amino acid phenylalanine (Phe) to build up in the body over time.

People with this disease do not have the enzyme to remove Phe like healthy people do.

PKU affects around 1 in 10,000 born in Australia6.

Newborns are routinely screened for the condition a few days after birth.

THERAPY

Palynziq™ is the first enzyme replacement therapy developed for people with PKU, meaning that it adds an artificial version of an enzyme to the body that can break down Phe independent of the body's natural pathways.[6]

For people, whose PKU is not controlled through diet alone, Palynziq™ may help lower harmful Phe levels and improve day-to-day health outcomes.

The Australian journey

- PBAC originally rejected Palynziq™ due to high cost and restrictive patient grouping. The next submission in March 2026 was deferred to allow for more negotiation on pricing; the outcome of the resubmission in July 2026 is still pending

International comparison

COUNTRY	REGISTRATION	REIMBURSEMENT	TIME DIFFERENCE
Germany	May 2019	July 2019	2 months
Japan	March 2023	May 2023	2 months
Australia	July 2021	Pending	Not applicable

- Germany and Japan both had short wait times between registration and reimbursement for Palynziq™, meaning patients had to wait less time between the drug being available and funded
- In Germany, no comparators are needed for rare disease medicines below a certain sales threshold[7] making the process smoother, while Australia still requires comparators

What could be different

Although Palynziq™ has been approved for use in Australia since 2021, it is not publicly funded. As a result, many Australians with PKU face barriers to accessing a treatment that is already funded in several comparable countries. As such, new mechanisms could be considered to reduce the time to reimbursement for rare disease medicines.

Case study 2 — Yorvipath™ (palopegteriparatide) for hypoparathyroidism

Uncertain patient populations can lead to longer wait times

DISEASE OVERVIEW

Hypoparathyroidism (HPTH) is a rare disease where the body doesn't produce enough Parathyroid Hormone (PTH). This helps the body control the balance of calcium and phosphorus.

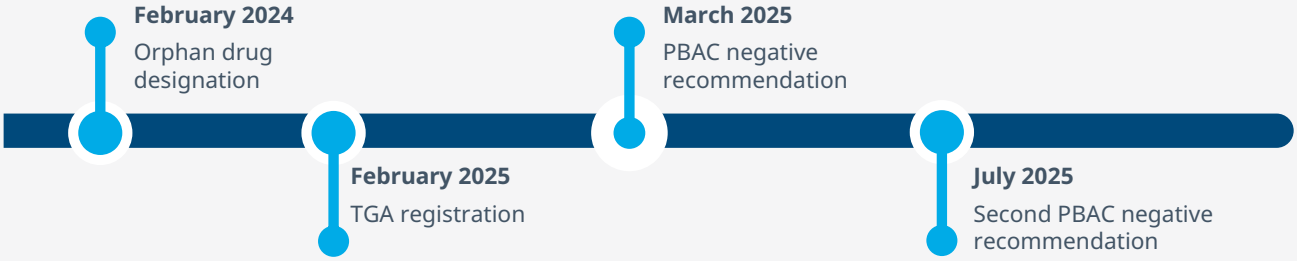
The symptoms can be different for everyone, but will often include painful spasms of the body, burning sensations on the hands and feet, and seizures. HPTH affects 2-4 people per 10,000.[8]

THERAPY

Treatment of HPTH has primarily been with calcium to keep levels normal, but this does not address the underlying disease.

Yorvipath™ is the first medicine that delivers an artificial version of PTH, keeping hormone levels the same as a healthy person.

The Australian journey



- PBAC has rejected funding Yorvipath™ twice due to costs, and concerns that more patients would use it instead of calcium supplementation in first line treatment
- The submission sponsor has advised that they will not be proceeding with a third PBAC submission

International comparison

COUNTRY	REGISTRATION	REIMBURSEMENT	TIME DIFFERENCE
France	November 2023	December 2024	13 months
Italy	November 2023	February 2026	27 months

- France and Italy show that uncertainty in patient populations does not have to result in no access
- France used an early access pathway so eligible patients could receive Yorvipath™ before routine reimbursement was finalised
- Italy reimbursed Yorvipath™ with registry-based monitoring, allowing use to be targeted to the correct population and tracked
- Yorvipath™ highlights a common challenge for rare disease medicines in Australia: regulatory approval does not guarantee public funding. While patients in several comparable countries can access Yorvipath™ through publicly funded healthcare systems, Australian patients continue to face limited access

What could be different

Access for treatments like Yorvipath™ could be supported through a bridging fund: fund Yorvipath™ for the highest-need patients, cap cost and use, and collect real-world evidence on calcium control, reduced supplement burden, kidney function, quality of life and safety. This would directly address PBAC's economic uncertainty while reducing delays for patients.

Case study 3 — Winrevair™ (sotatercept) for pulmonary arterial hypertension

Second order benefits can have great impact

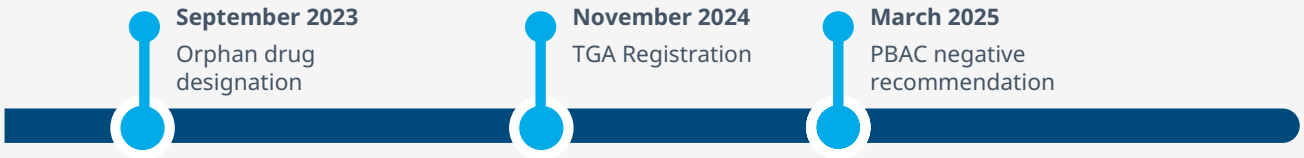
DISEASE OVERVIEW

Pulmonary arterial hypertension (PAH) is a type of high blood pressure that affects the lungs and heart. Damage to blood vessels increases strain on the heart, which can cause it to become weak and fail.
An estimated 15-50 people per million in Australia have PAH.

THERAPY

Winrevair™ is a therapy for PAH that prevents blood vessel narrowing, making it easier for the heart to function without weakening.

The Australian Journey



- Winrevair™ was rejected by PBAC in March 2025 due to the economic analysis presented
- Limited consideration of downstream clinical and economic benefits may have contributed to the negative funding outcome

International comparison

- Winrevair™ is available in the UK, France, and Germany (among other countries), but has not been funded in Australia
- Other countries considered second order benefits in their funding assessments as shown below, which influenced the final funding decisions

COUNTRY	REIMBURSEMENT	SECOND ORDER BENEFIT ASSESSMENT
Australia[9]	Not funded	Limited recognition
UK[10]	June 2026	Acknowledged disease progression reduction and value of delaying deterioration
France[11]	May 2025	Recognition of risk reduction and unmet need
Germany[12]	March 2025	Noted improvements in walking capacity and cardiovascular symptoms

- The main divergence between Australia and the major European HTA systems was not disagreement over the efficacy of Winrevair™, but the extent to which broader downstream benefits were incorporated into value assessment.

What could be different

Greater recognition of second-order benefits, including delayed disease progression and reduced healthcare utilisation, could improve the assessment of medicines' overall value. Incorporating long-term patient outcomes and broader system benefits may align Australia's approach more closely with leading European HTA agencies.

Key takeaways

- Benefits of the Australian Orphan Drug program are minimal and only include waiver of registration fee and first submission to the PBAC
- The current fee waiver financial incentive for the first submission does not cover the full submission cost for most sponsors, as 78% of reimbursed drugs undergo assessment at more than one HTA meeting
- 193 medicines received an Orphan Drug Designation from January 2018 to December 2025, the highest was 33 in 2025, with an average number of registrations at ~24 throughout the sample period
- Time from registration to reimbursement for rare disease medicines was highest in 2023 at ~32 months, and despite a recent decline in reimbursement time, there is still a large delay in subsidised access following regulatory approval with an average of ~22 months in 2025
- Timelines to reimbursement following registration are prolonged with 34% of drugs taking between 12 - 24 months, and 36% requiring more than 2 years
- Compared to other countries, Australia is funding fewer rare disease medicines in the same period, leading to patients waiting for access
- In specific cases such as Palynziq, Australian patients are waiting more than 5 years for access, and the restricted criteria of access for certain other medicines can exclude some patients
- Learnings from comparator countries such as incorporating quality of life, impact on carer, bridging funding can help expedite patient access to life-changing medicines

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Abbreviations

ABBREVIATION	DEFINITION
FDA	Food and Drug Administration
hATTR	Hereditary transthyretin-mediated amyloidosis
HST	Highly Specialised Therapies
HPP	Hypophosphatasia
HPTH	Hypoparathyroidism
HTA	Health Technology Assessment
LSDP	Life Saving Drugs Program
MBS	Medicare Benefits Schedule
NBA	National Blood Authority
NHRA	National Health Reform Agreement
NME	New Molecular Entity
PBAC	Pharmaceutical Benefits Advisory Committee
PBS	Pharmaceutical Benefits Scheme
Phe	Phenylalanine
PKU	Phenylketonuria
PTH	Parathyroid hormone
TGA	Therapeutic Goods Administration

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