White Paper

Access to Orphan Drugs in Australia

IQVIA Strategic Insights and Analytics

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Introduction

IQVIA in Australia and New Zealand provide an overview of the Orphan Drug program in Australia. Our experts have analysed trends in registration and reimbursement of orphan drugs following reforms to the program in July 2017. This report helps understand access pathways in Australia and timelines for patient access to therapies for rare diseases.

Orphan Drug program background

Australia's drug regulator, the Therapeutics Drug Administration (TGA) introduced its Orphan Drug Program in 1997.[1] The objective of the program is to facilitate development and access to drugs for small populations, which would otherwise not be financially viable for a sponsor.[1]

The program was overhauled in 2017, with key changes being a more generous prevalence threshold for rare diseases (< 2,000 individuals in Australia to < 5 in 10,000 individuals).[2,3] In addition, the designation validity was shortened from lifetime of the product to 6 months from approval, with an option to apply for extension for another six months.[4]

The key incentive for rare disease drug sponsors in Australia is a waiver of TGA evaluation and registration fees, which are $217,598 for a New Chemical Entity and $129,091 for the Extension of Indication in Financial Year 2023/24.[4,5]

The sponsor is also eligible for a fee waiver from the Pharmaceutical Benefits Advisory Committee (PBAC) for the first Health Technology Assessment (HTA) submission for reimbursement via the Pharmaceutical Benefits Scheme (PBS) or the Life Saving Drug Program for ultra-orphan drugs ($252,540 for Category 1 to $197,930 for Category 2 submissions in 2023/24).[6] Reimbursement applications for highly specialised therapies for rare diseases can also be assessed by the Medical Services Advisory Committee (MSAC) for funding via the National Blood Authority (NBA) or National Health Reform Agreement (NHRA), which currently does not operate on a cost recovery model (but is under consideration).[7]

Objective and Methodology

This review provides an insight into how orphan drug access in Australia has performed in the past 5 years in light of the reform and incentives provided to sponsors looking at the designation, registration and reimbursement of orphan drugs from January 2018 to June 2023.

IQVIA tracked the prescription medicine designation and approval timelines published by the TGA.[8,9] This was followed by looking at HTA submissions and their outcomes followed by tracking of press releases/ decisions for product reimbursement by various HTA bodies or funding schemes.[10,11,12]

Key Insights

Between January 2018 and June 2023

124 Orphan Drug Designations*

90 Registered

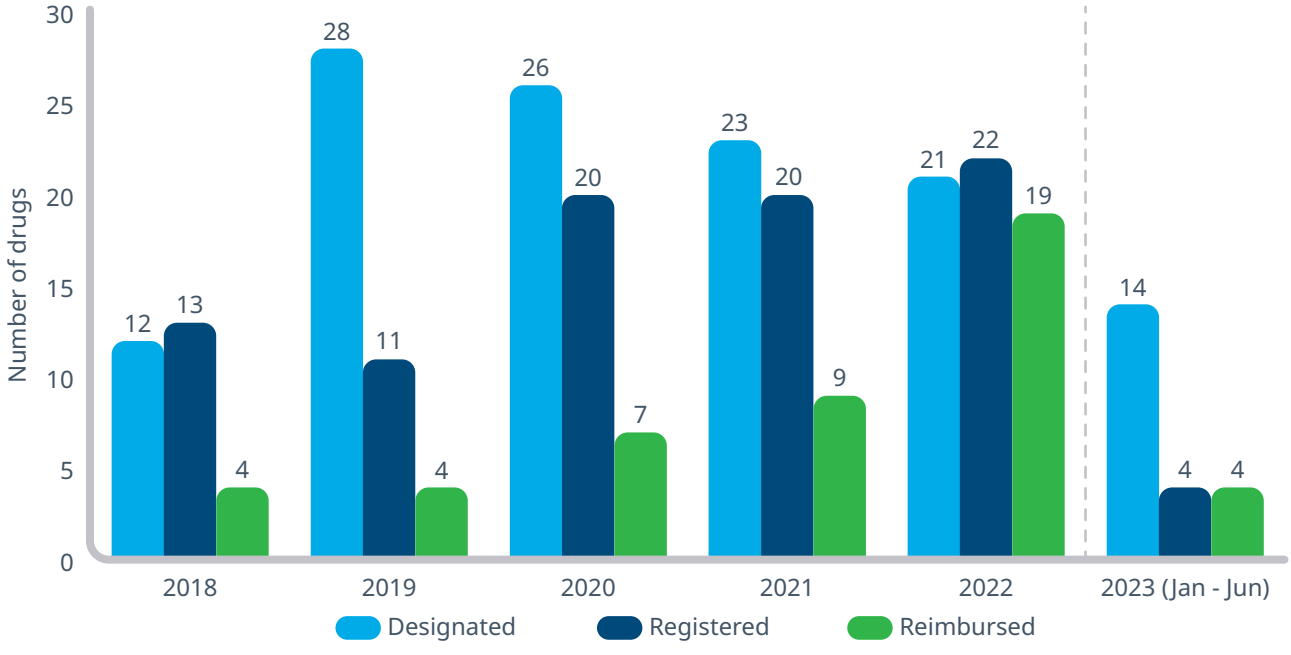
48 Reimbursed

How has Orphan Drug registration and reimbursement evolved?

Between 2018 and June 2023, on average,

21 drugs/therapies per year were designated orphan, with 15 moving towards TGA registration and 8 receiving reimbursement (Figure 1). This highlights the significant challenges faced by Orphan Drug sponsors with only 38% of drugs eventually reaching patients.

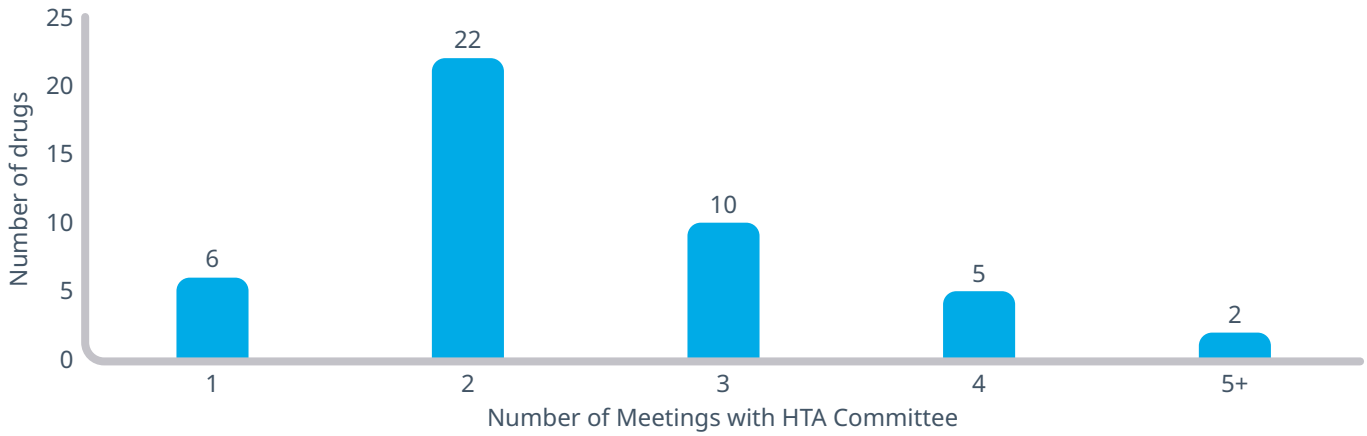
Figure 1. Orphan Drug designation, registration and reimbursement by year



How many HTA meetings before a positive recommendation?

Only 6 (13%) drugs were recommended upon first submission, with 50% receiving a positive recommendation at 2nd meeting (Figure 2). **Therefore, 87% of drugs were not able to take an absolute advantage of the fee waiver for HTA submission** as they required resubmissions based on negative outcome at the first meeting*.

Figure 2. Reimbursed Orphan Drugs by number of submissions before positive recommendation (January 2018 — June 2023)



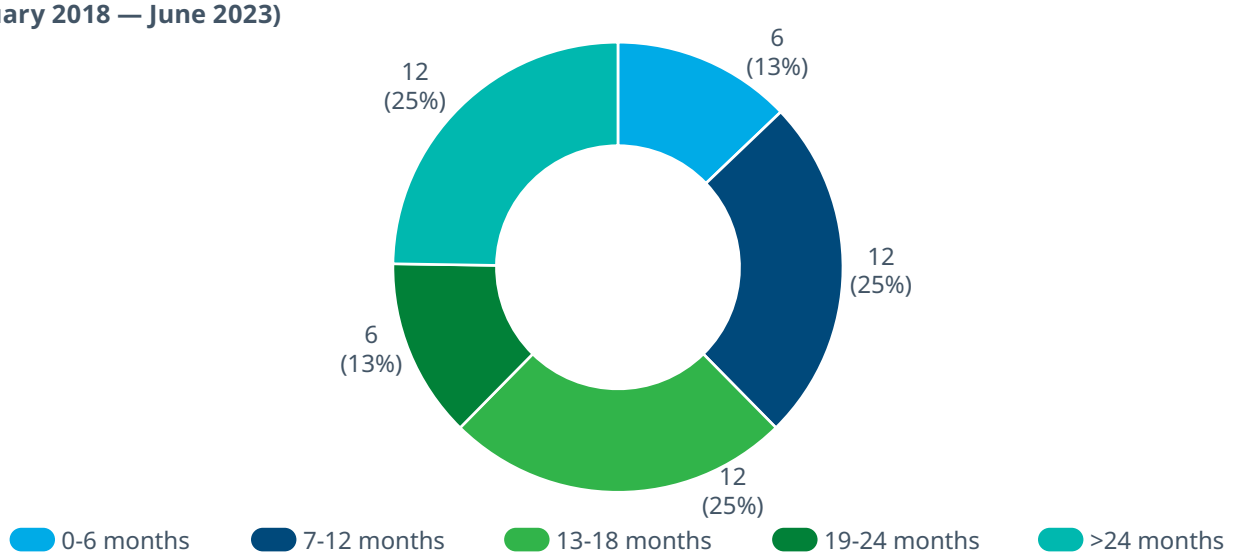
*Includes 4 drugs funded via the LSDP where a condition for funding is rejection by the PBAC on first submission for lack of cost-effectiveness
Excludes 3 products assessed via MSAC

How long from registration to reimbursement?

The timeline from registration to reimbursement can be quite lengthy with only 12.5% of drugs being reimbursed within 6 months of registration.

25% of orphan drugs took 7-12 months and another 25% took 13-18 months from registration to reimbursement.

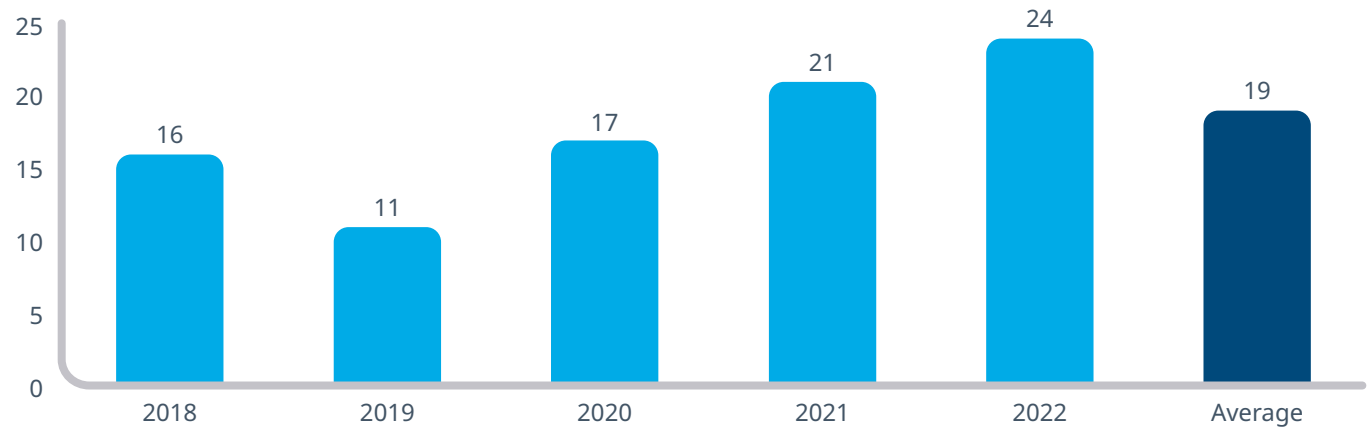
Figure 3. Distribution of reimbursed drugs by grouped months from registration to reimbursement (January 2018 — June 2023)



The average time *from registration* to reimbursement for the analysis period was 19 months.

When comparing the average time to reimbursement since 2018 (Figure 4), it is even more evident that orphan drugs face lengthy time to access.

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

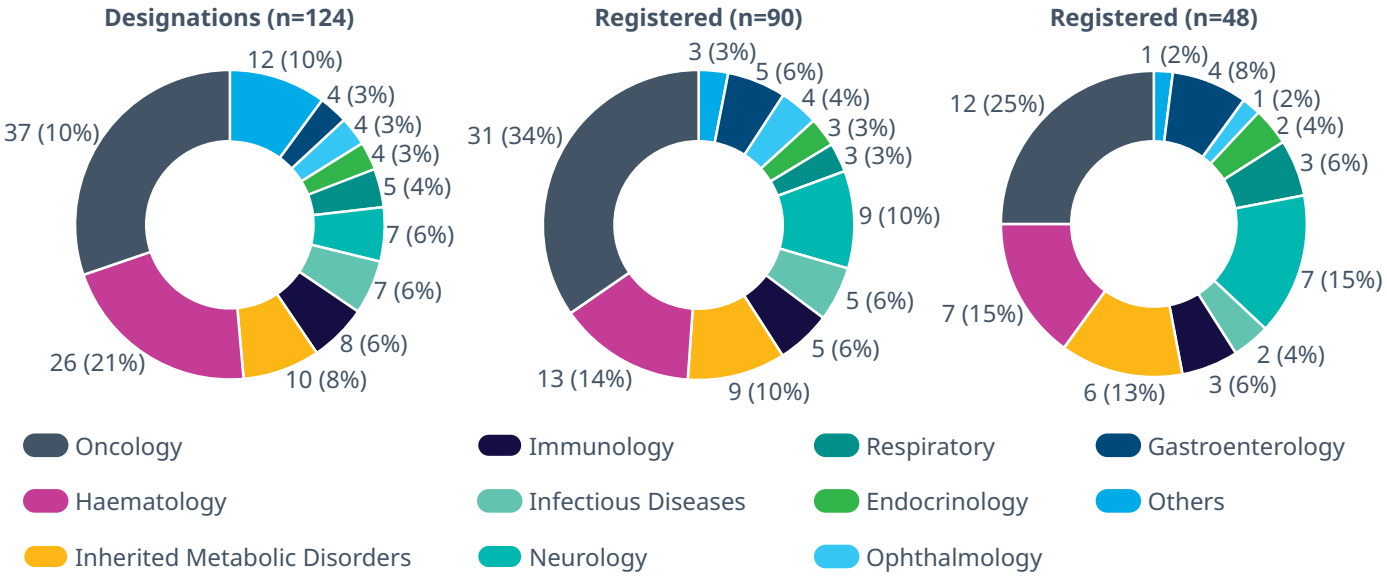


Apart from 2019, the overall time from registration to reimbursement has been on the increase with the average for 2022 being 24 months.

Which therapy areas are seeing the most reimbursements?

Oncology and Haematology make up 50% of designations received within 2018-2023, however, their proportion reduce as drugs move towards reimbursement. Particularly, neurology stands out in reimbursement in recent years with Spinraza, Zolgensma and Evrysdi for Spinal Muscular Atrophy receiving PBS listing.

Figure 5. Distribution of Orphan drugs by Therapy Area (January 2018 — June 2023)



Key Takeaways

- Benefits for the Australian Orphan Drug program are minimal and only include waiver of registration fee and first submission to the PBAC
- 124 drugs have received an Orphan Drug Designation from January 2018 to June 2023, the highest was 29 in 2019, with number of registrations remaining at ~20 in subsequent years
- Time from registration to reimbursement for orphan drugs was highest in 2022 at ~24 months showing there's a large delay in subsidised access following regulatory approval
- Current financial incentives of fee waiver for first submission to the PBAC does not benefit most sponsors with 87% of reimbursed drugs having to undergo assessment at more than one HTA meeting
- Timelines to reimbursement following registration are prolonged with 50% of drugs taking between 6-18 months, and 25% requiring more than 2 years
- While haematology and oncology dominated designations, registrations and reimbursements, success rate is relatively low compared to neurology

How can IQVIA help you?

- Understanding the patient journey including current treatment paradigm, unmet needs and barriers to treatment
- Identifying differentiating factors for therapy such as clinical improvement over standard of care, improvement in burden to patient
- Identifying funding models including innovative funding models to support reimbursement of novel therapies
- Determining addressable patient population, treatment uptake to inform budget impact
- Support with reimbursement submissions including health economic modelling

References

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