White Paper

Accelerated Access and Early-Phase Evidence: Lessons from 15 Years of PBAC Submissions

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Executive summary

This report analyses the use of early-phase clinical trial data in submissions to the Pharmaceutical Benefits Advisory Committee (PBAC) for the funding of medicines in Australia.

Between 2010 and 2025, only 3.8% of PBAC submissions relied on early-phase clinical trial data, yet these achieved notable success when supported by robust evidence and high unmet needs. Of 57 submissions across 33 molecules, most were supported by Phase II data with an 81% recommendation rate, while Phase I/II and Phase II/III submissions had mixed outcomes.

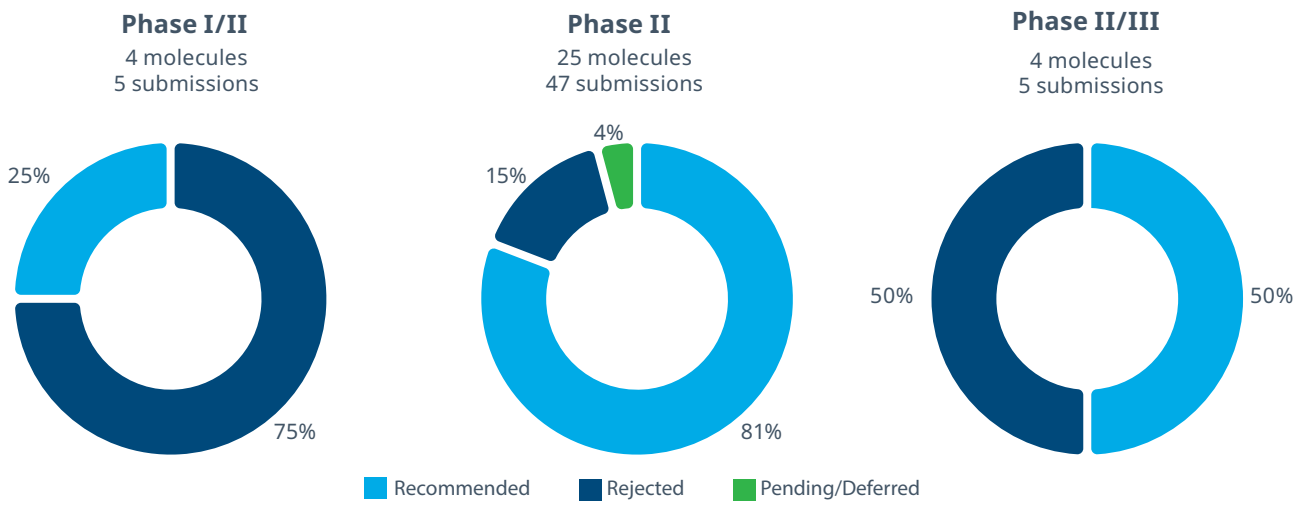
Activity increased from 2016 onward, peaking during 2020–2023 amid global shifts toward accelerated access. Oncology dominated (76% of submissions), reflecting challenges in conducting large-scale trials for rare cancers. Economic evaluations primarily used cost-effectiveness or cost-utility approaches, underscoring PBAC's flexibility in considering earlier-stage evidence when justified by clinical and economic context. Most of the submissions tracked in this report were eventually successful, highlighting the willingness of PBAC to approve medications with earlystage data in the right circumstances and with continuous engagement.

Methodology

We extracted data from IQVIA Health Technology Assessment data assets to find all submissions made to PBAC from 2010 onwards, filtered by the latest phase (e.g. Phase III is higher than Phase II) of clinical trial data used. Our report examines medicines recommended by PBAC for funding that had been submitted using early-phase clinical trial data. This includes data from Phase I, I/II, II, and II/III clinical research studies as the trial for the primary results of the medicine being submitted. Submissions that included Phase III trials for the comparator medicines were still included if the asset of the submission used early-phase evidence. Medicines that failed in their first submissions with early-phase data but were submitted later with Phase III data were not included.

Between 2010 and 2025, only 3.8% of PBAC submissions relied on early-phase clinical trial data, yet these achieved notable success when supported by robust evidence and high unmet needs.

Figure 1: Latest outcome of submissions made with early-phase clinical trial data by phase of data



Outcomes by phase of data

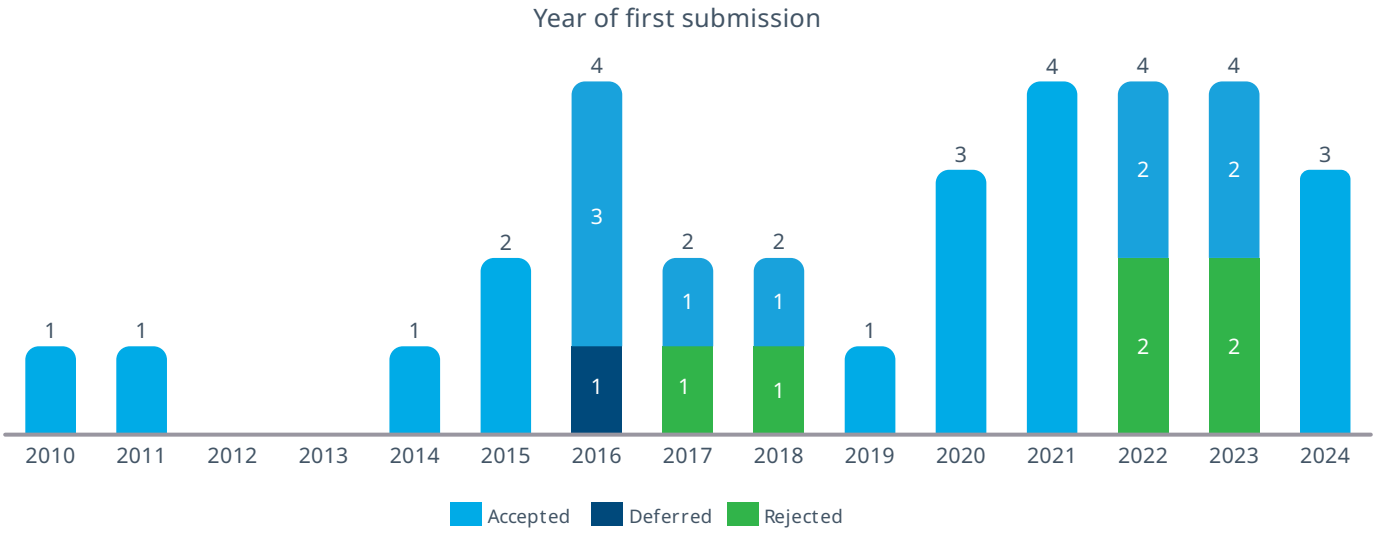
In total, 33 unique molecules were submitted to PBAC with early-phase data during the study period, accounting for 57 individual submissions. This represents 3.8% of the ~2,000 total PBAC submissions made between 2010 and 2025. The relatively small proportion underscores the continued reliance on late-phase data for most reimbursement decisions but also highlights a willingness by PBAC to consider earlier-stage evidence under certain circumstances.

Most early-phase submissions were supported by Phase II data, which accounted for 47 of the 57 submissions. These submissions had a notably high success rate, with 81% receiving a positive recommendation from PBAC. In contrast, submissions based on Phase I/II data were fewer in number (five submissions across four molecules) but still achieved a 75% recommendation rate. Submissions supported by Phase II/III data were evenly split, with half receiving a recommendation and half being rejected.

These findings suggest that while early-phase data are not commonly used in PBAC submissions, they can be effective when the evidence is robust, and the clinical context justifies earlier access. The high success rate of Phase II submissions indicates that PBAC is open to considering such data when it demonstrates meaningful clinical benefit, especially in areas of high unmet need or where traditional Phase III trials may be impractical or ethically challenging.

PBAC demonstrates flexibility when early-phase data is supported by strong evidence and economic rationale.

Figure 2: Number of submissions with early-phase data by the year of the first submission



Sorted by date of decision of FIRST submission with relevant evidence

Evolution over time

An analysis of the time distribution of PBAC submissions supported by early-phase clinical trial data reveals several important trends. Figure 2 illustrates the number of such submissions by year of first submission. Each bar is segmented to reflect the outcome of the submission. accepted, deferred, or rejected — providing a clear view of both volume and success rates over time.

The data show that early-phase submissions occurred sporadically in the first half of the decade, with isolated accepted submissions in 2010 and 2011, and no recorded activity between 2012 and 2015. A noticeable uptick begins in 2016, with four submissions

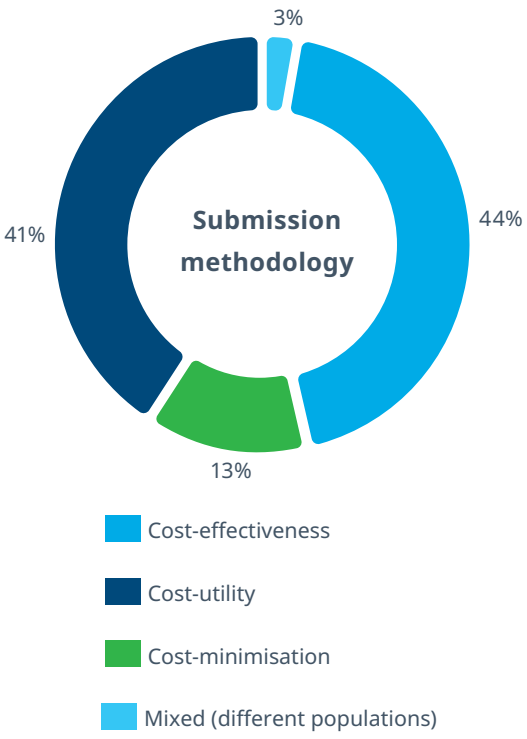
lodged — three accepted and one rejected. This marks the beginning of a more consistent pattern of early-phase submissions, with modest but steady activity continuing through to 2024.

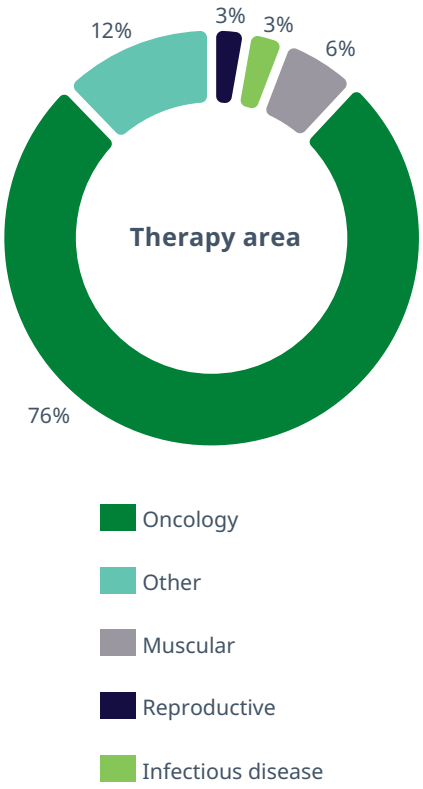
The years 2020 through 2023 show the highest levels of activity, with each recording between four and six submissions. Notably, the majority of these were accepted, while a smaller proportion were deferred. This period coincides with a broader global shift toward accelerated access pathways and increased regulatory flexibility, particularly in response to the COVID-19 pandemic. However, the data also indicate a temporary dip in submissions during the early pandemic years, likely reflecting broader disruptions to clinical trial activity and regulatory processes.

Despite the relatively stable number of early-phase submissions in recent years, this trend must be viewed in the context of the overall growth in PBAC submissions. While the absolute number of early-phase submissions has remained consistent, the total number of PBAC submissions has increased over time. This implies that early-phase submissions may be declining as a proportion of the total, suggesting a potential tightening of evidentiary expectations or a continued reliance on more mature data packages.

Overall, the evolution of early-phase submissions over time reflects a dynamic interplay between regulatory openness, industry behaviour, and external factors.

Figure 3: A — Final submissions with early-phase data by the submission methodology.
B — Final submissions with early-phase data by the therapy area of the medicine





Submission methodology and therapy areas

This section explores two key dimensions of PBAC submissions supported by early-phase clinical trial data: the **health economic methodology** used in the submission and the **therapeutic area** of the medicine under review. These factors provide important context for understanding how early-phase data are positioned within the PBAC framework and which types of therapies are most likely to be submitted with such data.

44% of submissions used cost-effectiveness analysis, while 41% used cost-utility analysis.

Figure 3A highlights that most early-phase submissions employed either a cost-effectiveness or cost-utility approach. Specifically, 44% of submissions used cost-effectiveness analysis, while 41% used cost-utility analysis. These methodologies are typically applied when there is a need to demonstrate the value of a new treatment relative to existing alternatives, particularly in cases where clinical outcomes are still evolving or surrogate endpoints are used. They are also used in indications where there are no available comparators, or the standard of care is best supporting care. The relatively even split between these two approaches suggests that sponsors are tailoring their economic arguments to the nature of the available data and the expectations of PBAC.

In contrast, only 13% of submissions used a cost-minimisation approach. This methodology is generally reserved for situations where the new treatment is demonstrably equivalent in clinical outcomes to an existing therapy, and the primary consideration is cost. One submission (3%) employed a mixed methodology, where a cost-effectiveness methodology was used in one patient population and a cost-minimisation methodology was used in another.

Figure 3B provides insight into the therapeutic areas most associated with early-phase submissions. The data reveals a striking concentration in oncology — accounting for 76% of all cases. This dominance likely reflects the urgent need for new treatments in this area, particularly in rare cancer subtypes where performing larger scale Phase III trials might be time and resource intensive.

EXAMPLE CASE 1

A notable example is the submission of Rozlytrek for ROS1-positive Non-Small Cell Lung Cancer (NSCLC), a variant affecting only 1–2% of NSCLC patients. This cost-minimisation submission was accepted based on Phase II data, with PBAC acknowledging the rarity of the condition and the submission's aim to demonstrate non-inferiority to an existing therapy.

Together, the findings indicate that early-phase submissions are most viable in areas where there is a strong unmet need, a clear economic rationale, and a regulatory environment that supports flexibility in evidence requirements.

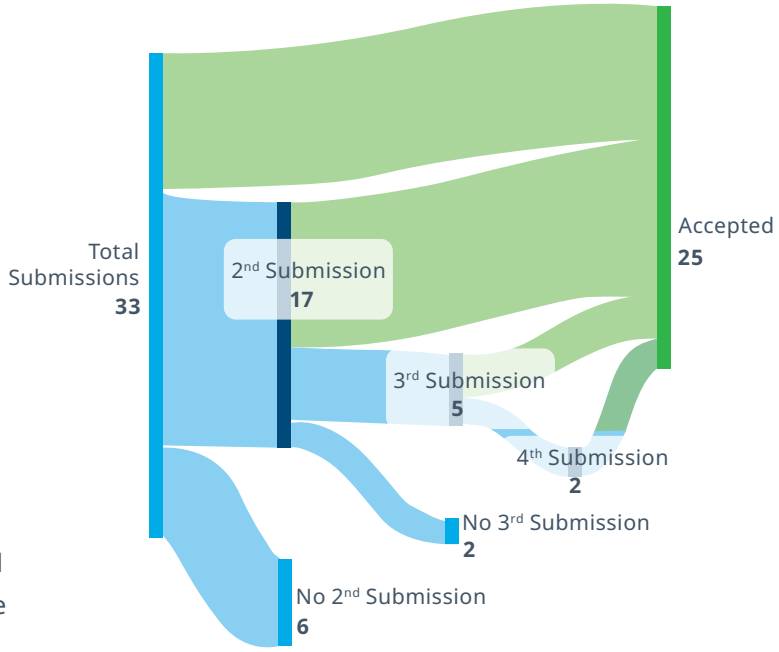
Flow of submissions

The Sankey diagram (Figure 4) maps the journey of early-phase submissions through the PBAC process, from initial submission to eventual recommendation. This visualisation provides a clear picture of how many submissions are successful on their first attempt (10/33), how many require resubmission (17/33), and how many ultimately fail to secure a recommendation (8/33).

Of the 33 early-phase submissions identified in this analysis, 25 were ultimately accepted, representing a 76% overall success rate. Notably, a substantial proportion of these were successful on their first submission, indicating that early-phase data can be sufficient for PBAC recommendation when the clinical and economic arguments are compelling. An example of this is the submission of Adcetris for systemic anaplastic large cell lymphoma. PBAC noted in the outcome that in the context of this rare disease (only 2% of lymphomas) that the early-stage data was the best available and that the patient group had high clinical need.

Resubmission is a common feature of the early-phase submission pathway. Seventeen submissions proceeded to a second submission, five to a third, and two to a fourth. This suggests that while PBAC may initially withhold recommendation due to uncertainty or insufficient evidence, sponsors often respond by refining their submissions and addressing PBAC's concerns. The fact that most submissions are ultimately successful after one or more resubmissions highlights the iterative nature of the process and the importance of persistence and adaptability. An example of a molecule that was successful after four submissions was Folotyn for peripheral T-Cell lymphoma. The first three submissions were rejected by PBAC due to the earlystage data meaning there was unacceptably high uncertainty of benefit over alternative therapies. In the fourth and final submission the sponsor was successful in achieving a recommendation as they lowered the price of Folotyn to adequately address this uncertainty.

Figure 4: Sankey diagram of the flow of submissions made with early-phase data



EXAMPLE CASE 2

An example of a sponsor halting after one rejected submission is Elzonris for Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN). The submission was made using Phase I/II data on the basis of cost-effectiveness against physicians' choice of chemotherapy. PBAC noted in their response that while BPDCN was a very rare disease with poor outcomes, the data used in the submission was not sufficiently strong to support superiority over chemotherapy, and suggested the sponsor resubmit with stronger data and updated economic evaluations.

Interestingly, only six submissions did not proceed to a second attempt, and just two stopped after a second submission without progressing further.

The trend of success on resubmission indicates that most sponsors are willing to re-engage with PBAC, and that the system is structured to allow for refinement and reconsideration over time, even without resubmitting with more mature data.

Conclusion

Early-phase data submissions to PBAC remain relatively rare, yet they continue to demonstrate strong potential for success — particularly when supported by robust Phase II evidence. The high success rate of these submissions underscores PBAC's openness to considering earlier-stage evidence, especially in areas of high unmet need or where traditional Phase III trials may be impractical. The evolving landscape of early-phase submissions reflects a dynamic interplay between regulatory flexibility, industry behaviour, and external factors such as the COVID-19 pandemic. As the trend towards accelerated access pathways continues, including for multiple new therapy areas with no current treatments, early-phase submissions will likely play an increasingly important role in bringing innovative treatments to market more swiftly.

About IQVIA

Who we are

IQVIA is a global provider of advanced analytics, commercial strategy advisory services, and clinical research services to the life sciences industry. With a footprint in over 100 countries, we have unparalleled expertise in understanding issues faced by the life sciences industry and governments to respond to the challenges as novel therapies and technologies come to market. Leveraging our global expertise, IQVIA provides services based on best-practice case studies to help adapt to a rapidly evolving healthcare landscape.

Our capabilities

IQVIA's team in Australia supports local and global pharmaceutical and biotechnology companies with their pricing and payer strategy needs, including:

- Payer value proposition
- Pricing strategy
- Innovative access strategies
- Support PBAC/MSAC submission

By leveraging our proprietary databases, a variety of data sources and our team's expertise, we can deliver rapid, clear and insightful recommendations on how to effectively navigate Australia's evolving market access environment.

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