

**INDEPENDENT REVIEW OF THE
IMPACT OF PHARMACEUTICAL
BENEFITS SCHEME (PBS)
COST RECOVERY**

NOVEMBER 2011

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Glossary

Term	Definition
Complex – Pricing (Regulation 3.3)	Complex pricing negotiations are those that involve an increase in the cost to government or require calculation of dose relativity to be undertaken during the course of pricing negotiations, or where the Commonwealth and the applicant enter into a deed or deeds of agreement relating to the supply of the proposed or currently listed drug, special pharmaceutical product or designated vaccine subject to the application.
Department	The Department of Health and Ageing.
Generic Listing of a new brand medicine (Regulation 2.14)	Generic applications occur where a new product is listed on the PBS because it is bioequivalent or biosimilar and the price is already determined by an existing item. However, if the application is in respect of a product listed in Schedule 2 of the Regulations (currently somatropin and glucose indicators), it is deemed to be an exception under regs 2.9 and 2.13 and is classified as minor or PBAC Secretariat Listing.
LSDP	Life Saving Drugs Program. Through the LSDP, the government provides subsidised access, for eligible patients, to expensive life-saving drugs for very rare life-threatening conditions. Before a drug is made available on the LSDP, it must generally be accepted by the PBAC as clinically necessary and effective but not recommended for inclusion on the PBS due to unacceptable cost effectiveness.
NIP	National Immunisation Program. The current NIP schedule started on 1 July 2007 and outlines the recommended vaccines by age group.
Orphan drug	An orphan drug is defined as a medicine, vaccine or in vivo diagnostic agent that will be administered to not more than 2000 people in Australia in each year after it is registered for use for the disease or condition, and is intended to treat, prevent or diagnose a rare disease, or is not commercially viable to supply to treat, prevent or diagnose another disease or condition.
PBPA	Pharmaceutical Benefits Pricing Authority. The PBPA provides advice to assist in negotiations on the initial price of a medicine, taking into account PBAC recommendations on the cost effectiveness of the medicine. Applications submitted to PBPA attract a fee according to category.

Term	Definition
PBAC	<p>Pharmaceutical Benefits Advisory Committee.</p> <p>The PBAC is an independent statutory body that makes recommendations and provides advice to the Minister about which drugs and medicinal preparations should be made available as pharmaceutical benefits. Since 1 January 2010, applications considered by the PBAC attract a fee according to category.</p>
PBAC Evaluation—Major (Regulations 2.3–2.6)	<p>In general, a major application seeks to list new drugs or medicinal preparations for PBS subsidy or to make substantial changes to current listings.</p> <p>An application for a variation to an existing listing may also be major if it requires the PBAC to apply a health advantage test (as defined in reg. 2.4).</p> <p>Major evaluations are complex evaluations of drugs for which PBS listing may have significant financial implications.</p>
PBAC Evaluation—Minor (Regulations 2.7–2.11)	<p>In general, minor applications include those for new forms of an already listed drug or medicinal preparation or changes to the conditions of their prescription or supply.</p> <p>These applications involve changes to existing items that do not have significant cost implications but do require consideration by PBAC for clinical effectiveness and/or potential impact on the PBS.</p> <p>An otherwise major application may be deemed minor if it involves a resubmission (reg. 2.10) or a medicinal food (reg. 2.11).</p>
PBAC Secretariat Listing (Regulations 2.12, 2.13)	<p>A secretariat listing is a minor application that is straightforward and not considered as a separate agenda item at a meeting of PBAC. PBAC still decides the merit of each application. Secretariat listings may be considered in or out of session by PBAC.</p>
Regulations	<p>National Health (Pharmaceutical and Vaccines—Cost Recovery) Regulations 2009.</p>
Secretariat—Pricing (Regulation 3.5)	<p>A ‘Secretariat—Pricing’ application is considered as a PBAC Secretariat Listing and is not considered by the PBPA.</p>
Simple—Pricing (Regulation 3.4)	<p>Simple pricing negotiations are those that require consideration by the PBPA and will not involve an increase in the cost to the Commonwealth in relation to the supply of pharmaceutical benefits.</p>
Sponsor	<p>A sponsor is the entity that is lodging an application for consideration.</p>

1 Executive summary

In July 2011 a Committee was established to review the available evidence within the terms of reference legislated under the *National Health Act 1953* to ascertain what impacts cost recovery has had on the Pharmaceutical Benefits Scheme (PBS). While the Committee did make some findings and recommendations, particularly around the cost recovery model, it found that overall there is insufficient data available at this time to ascertain the impact of cost recovery on the PBS.

In the 2008–09 budget, the government announced that costs associated with listing medicines on the PBS and designating vaccines for the National Immunisation Program (NIP) would be recovered from the sponsors of submissions to the Pharmaceutical Benefits Advisory Committee (PBAC). These fees were intended to reflect the costs involved in evaluating submissions that seek to make a new listing, or amend an existing listing, on the PBS or NIP.

Not all applications are subject to cost recovery. An application may receive a fee waiver if it is considered to be in the public interest and that payment of the fee would make the application financially non-viable. Some applications are also exempted from fees. These include designated orphan drugs, drugs considered necessary for a public health event of national significance, drugs required for public health emergencies under the *Quarantine Act 1908* and other circumstances outlined in Part 5 of the National Health (Pharmaceutical and Vaccines—Cost Recovery) Regulations 2009 (the Regulations).

Cost recovery commenced on 1 January 2010. Under s. 99YBC of the National Health Act, an independent review of the impact of PBS cost recovery must commence as soon as possible after the second anniversary of the amendments coming into force—22 July 2011—and be completed within four months of that date. As provided for under the legislation, the review must be conducted by a Committee against the terms of reference prescribed in the legislation. This report represents the views and considerations of the Committee.

Given the lead times for applications, the review only considered data from the July 2010, November 2010, March 2011 and July 2011 PBAC meetings, noting that there was insufficient time to confirm final outcomes from the July 2011 PBAC meeting. The dataset was closed and considered final for the review as of the first Committee meeting, which was on 31 August 2011.

The Committee extended to Medicines Australia, Generic Medicines Industry Association of Australia (GMiA), AusBiotech and the Consumers Health Forum of Australia (CHF) the opportunity to provide a submission. These four stakeholders were identified because they are directly subject to PBS cost recovery and are representatives for professional and consumer groups. All four organisations lodged a submission for the Committee's consideration.

The Committee noted that the current cost recovery model was developed between 2006 and 2008 and does not capture a number of activities that currently attract operative costs, such as the cost of formal consumer input for PBAC consideration or requests for early dialogue by sponsors apart from standard pre-submission meetings. The current model also does not include the cost of monitoring risk-share arrangements between the Commonwealth and pharmaceutical companies or the activities associated with requests for price increases.

1.1 Findings

Table 1 gives a summary of the Committee findings.

TABLE 1 FINDINGS

Terms of reference	Finding
<p>(a) the average number of times a submission is presented before gaining approval and the reasons provided for requiring applicants to resubmit</p>	<p>Finding 1: Despite the limited number of resubmissions since the commencement of cost recovery, the Committee noted that the reasons most commonly cited by the PBAC for not recommending an application were inadequate clinical and/or cost-effectiveness evidence provided in the submission.</p> <p>Finding 2: The Committee concluded that, because of the limited dataset available, it was unable to provide a meaningful conclusion with regard to terms of reference item (a).</p>
<p>(b) the average fee for submissions by type of submission (major/minor/generic according to Department of Health and Ageing classifications)</p>	<p>Finding 3: The Committee noted that the number of fee exemptions and waivers will significantly impact on the total fees collected for lodgement and therefore the average fee paid for each classification.</p> <p>Finding 4: It has not been possible to present the pricing fee data in the same way as the lodgement fees data due to a difference in categorisation. The Committee noted that there were 82 pricing applications (including 15 fee waivers and exemptions) with total pricing fees of \$435 000, equating to an average of \$5304 per application.</p> <p>Finding 5: The Committee noted that cost recovery revenue is significantly lower than was anticipated when the measure was introduced.</p>

Terms of reference	Finding
<p>(c) the number of applications where the population is likely to be small and utilisation of the drug, medicinal preparation or vaccine is likely to be highly targeted</p>	<p>Finding 6: The Committee noted that from the total number of applications submitted, approximately one-third of Sponsors have predicted an estimated use of less than 2000 prescriptions in the first 12 months of listing. They also noted that a number of these products had their fees waived or exempted under existing cost recovery criteria.</p> <p>Finding 7: The Committee noted that, even though the estimated use in the first 12 months may be fewer than 2000 prescriptions and/or the product may be exempted because it has been designated an orphan drug or considered for the Life Saving Drugs Program (LSDP), the cost to government may still be significant due to the high unit cost.</p>
<p>(d) the number of reviews requested by applicants</p>	<p>Finding 8: The Committee concluded that there is insufficient data to determine that cost recovery has impacted on the number of reviews, as no independent reviews have been requested.</p>
<p>(e) the number of fee waivers given to applicants and the reasons why waivers were given</p>	<p>Finding 9: The Committee found the majority of requests for lodgement fee waivers and exemptions were granted. Those that were not granted were not able to satisfy the requirements for financial non-viability or were not able to provide adequate supporting documentation.</p> <p>Finding 10: The Committee noted that, for the period 1 January 2010 to 31 August 2011, \$2 743 500 in lodgement fees were waived or exempted.</p> <p>Finding 11: The Committee noted that approximately one in five of all categories were granted a lodgement fee exemption or waiver.</p>
<p>(f) the length of time taken for submissions to be approved</p>	<p>Finding 12: The Committee noted that there is insufficient data available at this stage to conclude whether cost recovery impacted on the average time frames to listing.</p> <p>Finding 13: The Committee noted the median number of days from receipt by the Department of Health and Ageing (the department) to the publishing of a positive PBAC outcome online is 127 days. This is in accord with the agreed time frames. The median number of days from receipt by the department to listing is 199 days.</p> <p>Finding 14: The Committee noted that the median time frame of two to three months from publication of the PBAC meeting outcomes to listing demonstrates reasonable efficiency given the legislative and administrative requirements that must be complied with.</p>

Terms of reference	Finding
<p>(g) the number of applications that fail to gain a listing, the reasons why and the types of drugs concerned</p>	<p>Finding 15: The Committee noted there is insufficient data available at this stage to determine whether cost recovery had impacted on the number of applications that fail to gain a listing.</p> <p>Finding 16: The most common reason that an application fails to gain a positive PBAC recommendation is inadequate clinical and/or cost-effectiveness evidence that was satisfactory to the PBAC.</p> <p>Finding 17: The Committee noted the PBAC sought clarification of issues for the eight products it deferred. The issues were in regard to wording of the restriction or reconsideration of price in order to achieve an acceptable incremental cost-effectiveness ratio.</p>
<p>(h) any increase in operating costs of the Pharmaceutical Benefits Advisory Committee</p>	<p>Finding 18: The Committee noted that the current initiatives to improve consumer input are not included in the cost model. Further, the Committee noted that enhancement of consumer input by the introduction of a systematic approach would result in substantial costs.</p> <p>Finding 19: The Committee noted there has been an increase in the administrative burden and associated costs due to the development and monitoring of a greater number of special listing arrangements.</p> <p>Finding 20: The Committee noted that if pre-submission meetings were formalised it would provide an opportunity for early dialogue with sponsors that would include discussion surrounding clinical trial design. This activity would impact on the total cost of running the program.</p> <p>Finding 21: The Committee noted that there is no evidence that the operating cost of running the PBAC itself has increased as a result of the introduction of cost recovery.</p>
<p>(i) any increase in the cost of pharmaceutical benefits scheme medications to patients</p>	<p>Finding 22: The Committee noted cost recovery does not directly affect the current NIP arrangement whereby the Commonwealth, via States and Territories, provides vaccines free of charge to health providers to administer to eligible patients in the community.</p> <p>Finding 23: The Committee noted that co-payments will not increase as a result of cost recovery.</p>
<p>(j) any other matters considered relevant</p>	<p>Finding 24: The Committee noted that a submission for a life-saving or designated orphan drug is likely to have its fees exempt or waived. However, the criteria for a fee waiver should take into account the combined total cost to government and total projected profit. This would address the issue of highly profitable products being automatically exempt from paying cost recovery fees because of their classification.</p>

1.2 Recommendations

The Committee noted that there is to be a major review of cost recovery after five years from its date of commencement. The Committee is of the opinion that consideration of the recommendations (and actions, as appropriate) should occur before that period.

Notwithstanding the fact that the Committee found that the data available was too immature to determine whether cost recovery had impacted on the PBS or NIP, it wishes to make the following recommendation on the structure of the accounting model that was used to determine the fees payable.

The Committee also noted that there has been an increase in administrative burden and associated costs due to the development and monitoring of a greater number of special listings and that if pre-submission meetings were formalised it would provide an opportunity for early dialogue with sponsors that would include discussion surrounding clinical trial design. The Committee acknowledges that these activities would impact on the total cost of running the program.

RECOMMENDATION 1: The Committee recommends that the Minister consider the re-evaluation of the costing model used in the determination of the fee structure for cost recovery to ensure that all costs associated with the evaluation and listing process are included. These include, but are not limited to, the development and monitoring of special listing arrangements and formalising pre-submission meetings.

The Committee acknowledged the role of fee exemptions and waivers in ensuring that inappropriate cost burdens are not placed on certain applications, particularly those for small numbers of patients. However, the Committee noted that, for some of these applications, the total cost to government was significant due to the high unit cost and in some cases it was higher than the cost for drugs that are not eligible for exemption or waiver. The Committee believes that this has the potential to cause an anomaly within the current fee structure.

RECOMMENDATION 2: The Committee recommends that the Minister consider the inclusion of total government expenditure and total projected profit as factors to be considered in deciding whether an application should be granted a fee exemption or waiver, even where those applications may involve small patient numbers.

Further, the Committee noted the Consumers Health Forum request for a more formalised structure for providing input into PBAC processes. While it supported this development in principle, the Committee noted that the cost of such a process is not currently included in the cost recovery model for the determination of fees.

RECOMMENDATION 3: The Committee recommends that the Minister consider the inclusion of the cost of formalising consumer input into the cost model.

RECOMMENDATION 4: The Committee recommends that the terms of reference for future reviews of the cost recovery program be examined to better align the terms of reference with the data sources available.

1.3 Terms of reference

Under s. 99YBC of the *National Health Act 1953*, an independent review of the impact of PBS cost recovery must commence as soon as possible after the second anniversary of the amendments coming into force—22 July 2011—and be completed within four months of that date.

The review must report on the following legislated terms of reference:

- (a) the average number of times a submission is presented before gaining approval and the reasons provided for requiring applicants to resubmit
- (b) the average fee for submissions by type of submission (major/minor/generic according to Health Department classifications)
- (c) the number of applications where the population is likely to be small and utilisation of the drug, medicinal preparation or vaccine is likely to be highly targeted
- (d) the number of reviews requested by applicants
- (e) the number of fee waivers given to applicants and the reasons why waivers were given
- (f) the length of time taken for submissions to be approved
- (g) the number of applications that fail to gain a listing, the reasons why and the types of drugs concerned
- (h) any increase in operating costs of the Pharmaceutical Benefits Advisory Committee
- (i) any increase in the cost of pharmaceutical benefits scheme medications to patients
- (j) any other matters considered relevant.

As provided under the legislation, the review must be conducted by a panel (Committee), which must comprise not fewer than five persons, including:

- a medical professional nominated by the Minister for Health and Ageing
- a nominee of the Consumers Health Forum of Australia
- three other persons nominated by the Minister, each of whom must have relevant professional qualifications and must not be employed within the pharmaceuticals industry.

The Committee must provide a written report to the Minister which must be tabled in both houses of parliament within 15 sitting days of receipt.

For the review, a ‘medical professional’ is defined as a specialist, general practitioner or clinical pharmacologist. This is based on the PBAC definition as described in the National Health Act.

1.4 Committee members

The names of Committee members are set out in the table below.

Name	Position	Role
Ms Carol Bennett	CEO, Consumers Health Forum of Australia	Member
Ms Paula Cronin	Centre for Health Economics Research and Evaluation (CHERE)	Member
Professor Michael Kidd AM	Executive Dean, Faculty of Health Sciences, Flinders University	Member
Professor Lloyd Sansom AO	Department of Health and Ageing	Member
Dr Lynn Weekes	CEO, NPS, Better Choices, Better Health	Member

1.5 Panel approach

There were five elements of the review:

- Committee meeting 1—agreement on the interpretation of each terms of reference item
- call for submissions
- Committee meeting 2—refinement of the draft report and consideration of the submissions received
- Committee meeting 3—review of draft 2 of the report and insertion of amendments that will lead to report endorsement
- out-of-session opportunities for input and comments.

1.5.1 Committee meeting 1

The first Committee meeting was held on 31 August 2011. The objective was to discuss and interpret the terms of reference. This provided the Committee with clarity regarding the terms of reference data that needed to be provided by the Department of Health and Ageing (the department).

1.5.2 Call for submissions

The Committee considered whether to seek submissions from stakeholders in response to the terms of reference. Following a detailed discussion, the Committee agreed that four key stakeholders be advised of the review and the process of lodging a submission. These stakeholders were Medicines Australia, Generic Medicines Industry Association of Australia (GMiA), AusBiotech and

the Consumers Health Forum of Australia (CHF). These four stakeholders were identified because they are directly subject to PBS cost recovery and are representatives for professional and consumer groups. All stakeholders lodged a submission for consideration by the Committee.

1.5.3 Committee meeting 2

The second Committee meeting was held on 24 October 2011. The objectives were to review and approve the minutes from meeting 1, review and summarise the submissions received from the stakeholder organisations and discuss the information, content and assumptions in the first draft report.

1.5.4 Committee meeting 3

The third Committee meeting was held on 10 November 2011. The objectives were to review the second draft report, make any amendments required and endorse each section subject to agreed amendments. The Secretariat committed to making the amendments and forwarding the third draft report to Committee members for consideration and endorsement.

1.5.5 Out-of-session opportunities

All agendas, minutes and additional notes and the draft report were circulated at various points in the process for consideration by all Committee members. The Committee had the opportunity to review the report and provide their feedback outside of the Committee meetings.

2 Background

In the 2008–09 budget, the government announced that costs associated with listing medicines on the PBS and designating vaccines for the NIP would be recovered from the sponsors of submissions to the PBAC. In June 2008 the National Health Amendment (Pharmaceutical and Other Benefits—Cost Recovery) Bill failed to pass the Senate.

The Bill was reintroduced into parliament in May 2009 and was passed with amendments in June 2009. The subordinate Regulations were given royal assent by the Governor-General in December 2009 and came into force on 1 January 2010.

The amendments required that an independent review of the impact of PBS cost recovery occur after the second anniversary of passage of the legislation. The legislation requires that the review be conducted by a Committee of not fewer than five persons. Following completion of the review, the panel must provide the Minister with a written report to be tabled in each house of parliament within 15 sitting days of the Minister receiving it.

This report contains the results of the review as provided for by the legislated terms of reference and includes summaries of submissions received.

2.1 PBAC cost recovery reform

Cost recovery fees are collected at two points in the PBS listing process:

- when submissions are lodged for consideration by the PBAC
- following the outcome of pricing negotiations with the Pharmaceutical Benefits Pricing Authority (PBPA) once confirmation is made that the product will be listed on the PBS or NIP.

The intent was that the fees recovered were to reflect the cost involved in the evaluation of submissions, pricing negotiations and the management of PBS or NIP listings.

The current fee structure was developed between 2006 and 2008 using an activity-based costing model. A two-stage process was used to allocate overhead costs to each business unit and then a further step allocated these costs to activities and services. The methodology enabled costs to be allocated to activities and services based on their consumption at each stage of the process through to the final service.

The resource costs associated with the listing of medicines on the PBS were attributed to the identified services on the following basis:

- An estimate of the resource cost base was developed, determined on the internal budgets developed by the department.
- Staff costs and associated overheads were allocated to activities and services based on estimates of resource allocation advised by staff.
- Direct costs were allocated to activities and services to which they relate.
- Overheads were attributed to activities and services in proportion to the staff allocations.

It should be noted that cost recovery fees have not been changed since their development. The first legislated increase, based on the consumer price index (CPI), may occur in July 2012 as provided for under the Regulations.

In accordance with Australian Government Cost Recovery Guidelines, the department was required to prepare a Cost Recovery Impact Statement (CRIS), which included industry consultation. The end date for the current CRIS is July 2012. A new CRIS will be developed on completion of the review. As required by the legislation, the department will also undertake a full review of its current cost recovery arrangements, including the activity-based costing model, within five years of commencement of the measure.

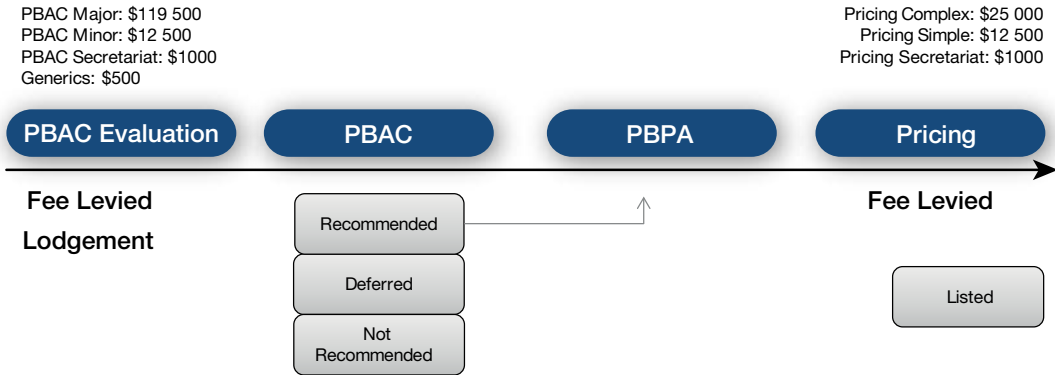
2.2 Process overview

The PBS listing process is managed by the department. Following lodgement, an application will progress through various states of consideration (see Figure 1). These include:

- active consideration (those applications still being considered at various stages of the process)
- recommended by the PBAC
- not recommended by the PBAC
- deferred by the PBAC
- withdrawn by sponsor
- PBPA recommended
- listed on the PBS.

Applications that have been deferred by the government are considered to be still under active consideration.

FIGURE 1 STATUS OF DECISIONS IN CONTEXT OF COST RECOVERY



Note: Sponsor can withdraw at any point in time.

2.2.1 Pharmaceutical Benefits Advisory Committee

Sponsors who wish to list or amend the listing of a product on the PBS or NIP submit an application for consideration by the PBAC. The PBAC is an independent statutory body that makes recommendations and provides advice to the Minister on those products (together with any conditions of listing) that should be considered for listing as a pharmaceutical benefit or on the NIP. Since 1 January 2010, applications considered by the PBAC attract a fee according to categories set out in Table 2.

TABLE 2 EVALUATION FEES AND CATEGORIES

Service	Description	Fee
PBAC Evaluation—Major (Regulations 2.3–2.6)	In general, a major application seeks to list new drugs or medicinal preparations for PBS subsidy, or to make substantial changes to current listings. An application for a variation to an existing listing may also be major if it requires the PBAC to apply a health advantage test (as defined in reg. 2.4). Major evaluations are complex evaluations of drugs for which PBS listing may have significant financial implications.	\$119 500
PBAC Evaluation—Minor (Regulations 2.7–2.11)	In general, minor applications include those for new forms of an already listed drug or medicinal preparation or changes to the conditions of their prescription or supply. These applications involve changes to existing items that do not have significant cost implications but which do require consideration by PBAC for clinical effectiveness and/or potential impact on the PBS. An otherwise major application may be deemed minor if it involves a resubmission (reg. 2.10) or a medicinal food (reg. 2.11).	\$12 500
PBAC Secretariat Listing (Regulations 2.12, 2.13)	A secretariat listing application is a minor application subset that is straightforward and not considered as a separate agenda item at a meeting of PBAC. PBAC still decides the merit of each application. Secretariat listings may be considered in or out of session by PBAC.	\$1000
Generic Listing of a New Brand Medicine (Regulation 2.14)	A generic application is made where a new product is listed on the PBS because it is bioequivalent or biosimilar and the price is already determined by an existing item. However, if the application is for a product listed in Schedule 2 of the Regulations (currently somatropin and glucose indicators), it is deemed to be an exception under regs 2.9 and 2.13, and is classified as a minor or PBAC Secretariat Listing.	\$500

The listing of a generic brand of drug, where bioequivalence has been demonstrated to an already listed product, attracts an administration fee, provided its price is within price parameters and requires no consideration by the PBPA.

For products not recommended by the PBAC for listing on the PBS or NIP, a sponsor can request an independent review or make a new submission addressing the issues raised by the PBAC. The fee for an independent review is \$119 500—the same price as a major submission.

2.2.2 Pharmaceutical Benefits Pricing Authority

Subsequent to receipt of a positive PBAC recommendation, a sponsor can make a pricing application to the PBPA. The PBPA makes recommendations about the price of the product, taking into account PBAC recommendations. Applications submitted to PBPA attract a fee according to the categories set out in Table 3.

TABLE 3 PRICING FEES AND CATEGORIES

Service	Description	Fee
Complex—Pricing (Regulation 3.3)	'Complex' negotiations are defined as those which involve an increase in the cost to government or that require calculation of dose relativity to be undertaken during the course of pricing negotiations, or where the Commonwealth and the applicant enter into a deed or deeds of agreement relating to the supply of the proposed or currently listed drug, special pharmaceutical product or designated vaccine subject to the application.	\$25 000
Simple—Pricing (Regulation 3.4)	'Simple' negotiations are defined as those that require consideration by the PBPA and will not involve an increase in the cost to the Commonwealth in relation to the supply of pharmaceutical benefits.	\$6000
Secretariat—Pricing (Regulation 3.5)	A 'Secretariat—Pricing' application is considered as a PBAC Secretariat Listing and is not considered by the PBPA.	\$1000

2.2.3 Fee waivers and exemptions

Not all applications are subject to cost recovery. An application may receive a fee waiver if it is considered to be in the public interest and payment of the fee would make the application financially non-viable. The objective of this approach is to ensure that cost recovery fees do not adversely affect the listing of medicines on the PBS.

Some applications are exempt from fees. These include designated orphan drugs, drugs considered necessary for a public health event of national significance and drugs required for public health emergencies under the *Quarantine Act 1908* and other circumstances as outlined in Part 5 of the Regulations.

An 'orphan drug' is defined as a medicine, vaccine or in vivo diagnostic agent that will be administered in Australia to not more than 2000 people in each year after it is registered for use for the disease or condition. Medicines need to be designated as orphan drugs by the Therapeutic Goods Administration (TGA) before an application to register an orphan drug on the Australian Register of Therapeutic Goods (ARTG) will be accepted.¹

1 <http://www.tga.gov.au/industry/pm-orphan-drugs.htm>

2.2.4 Deferred listing

The PBAC may defer making a recommendation to government in order to seek further information from the sponsor and bring the matter back to a subsequent PBAC meeting. The government may also choose to defer the listing of drugs on the PBS. The Committee noted that, in an announcement made on 30 September 2011, the government, as part of its commitment with the Consumers Health Forum, Generic Medicines Industry Association and Medicines Australia:

- agreed to work together to discuss ways to manage deferrals into the future
- agreed to work together on further savings for the 2012–13 budget and other savings following the expiry of the memorandum of understanding between industry and government
- committed to not deferring any drugs that cost under \$10 million a year for the coming year while it works with all parties to achieve longer-term PBS sustainability.
- Industry has also agreed to legislative amendments to complete price disclosure reforms, which will help to end anomalies in the current pricing system.

It should be noted that a pricing fee is not charged until actual listing occurs.

3 Dataset for this review

3.1 Data interpretation issues

There is considerable uncertainty associated with the cost recovery dataset that is currently available, primarily because of the very short time since cost recovery was introduced. Caution needs to be taken when interpreting the data in this report. In particular, the reader should note the points set out below.

- There are limitations to the dataset given the limited time that has elapsed since the commencement of cost recovery.
- The dataset reflects submissions where the initial submission was lodged after the commencement of cost recovery on 1 January 2010.
- The data is based on one full and two part financial years, limiting the direct comparison with other datasets based on single financial years.
- Applications received by the department are generally tracked by submission, not by the drug; thus, resubmissions are counted anew each time they are submitted.

The following points are relevant to the interpretation of the dataset and the report:

- All data from PBAC meetings held between July 2010 and July 2011 for new applications since cost recovery commenced is included but data from special meetings is not included.
- The information is based on data available as at 31 August 2011.
- Where applications are referenced, they include resubmissions.
- All prices noted in the report are goods and services tax (GST) free.

The following assumptions are relevant to the interpretation of the dataset and the report:

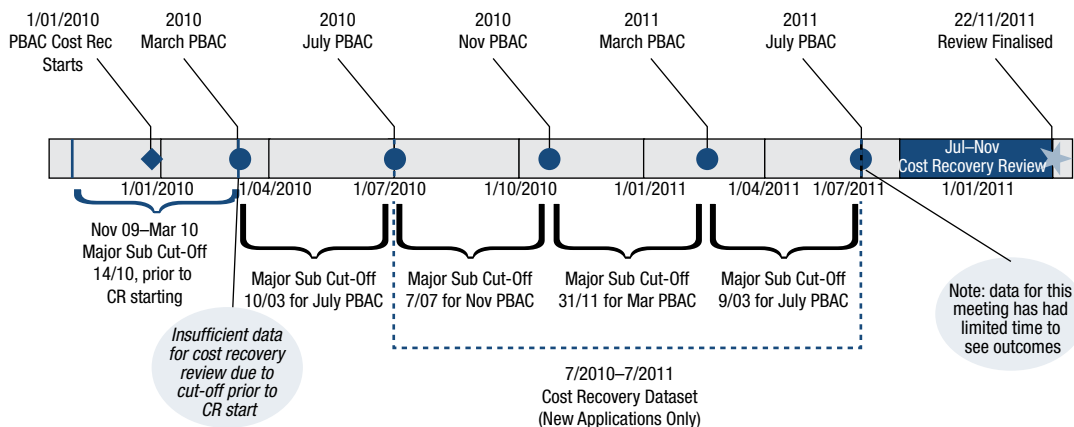
- If the government chooses to defer a listing, this application is considered to be under active consideration.
- If an application is deferred by the PBAC, it is considered closed regardless of outcome.
- Resubmissions addressing reasons for PBAC deferral may be subject to an evaluation fee.

3.2 Data source for this review

PBAC meetings are held in March, July and November each year.

The cut-off date for major submissions is generally 17 weeks before a PBAC meeting (18 weeks over the Christmas to New Year period). For minor submissions it is 11 weeks.

FIGURE 2 PBAC TIMELINE AND DATASET



In setting the scope of data to be analysed, the Committee noted that, due to lead times (cut-off dates) associated with major applications, there would be insufficient data from the March 2010 PBAC meeting.

The Committee also noted that the data from the November 2011 PBAC meeting will not be known in sufficient time for review and inclusion in the report.

Given the start and end date parameters, the Committee decided that data for the March 2010 and November 2011 PBAC meetings should be omitted from the dataset. Therefore, the review has considered data from the July 2010, November 2010, March 2011 and July 2011 PBAC meetings, noting that there may be insufficient time to confirm final outcomes for the July 2011 PBAC meeting. **The dataset was closed and considered final for the review as of the first Committee meeting, which was 31 August 2011.**

The Committee noted that the department's Activity Indicators for the PBS Fourth Report: 2005 to 2010 (KPI) contains useful information on activities of the PBAC and PBPA; however, the comparison of this data with that contained in this report should be undertaken with caution given the different time horizons used in the datasets in each of the reports.

TABLE 4 NUMBER OF SUBMISSIONS PER PBAC MEETING AS DEFINED BY THE EVALUATION FEE CATEGORIES

PBAC meeting	Jul 2010	Nov 2010	Mar 2011	Jul 2011	Total*
PBAC Evaluation – Major applications	15	24	18	31	88
PBAC Evaluation – Minor applications	20	24	20	15	79
PBAC Secretariat Listing	18	7	14	11	50
Subtotal for applications	53	55	52	57	217
All Generic applications					223 [#]
All applications including Generic					440

* All applications (including resubmissions).

[#] The majority of generic applications are not tracked by PBAC meeting. They are therefore aggregated as one figure.

4 Response to the terms of reference

4.1 Terms of reference item (a): the average number of times a submission is presented before gaining approval and the reasons provided for requiring applicants to resubmit

Notes

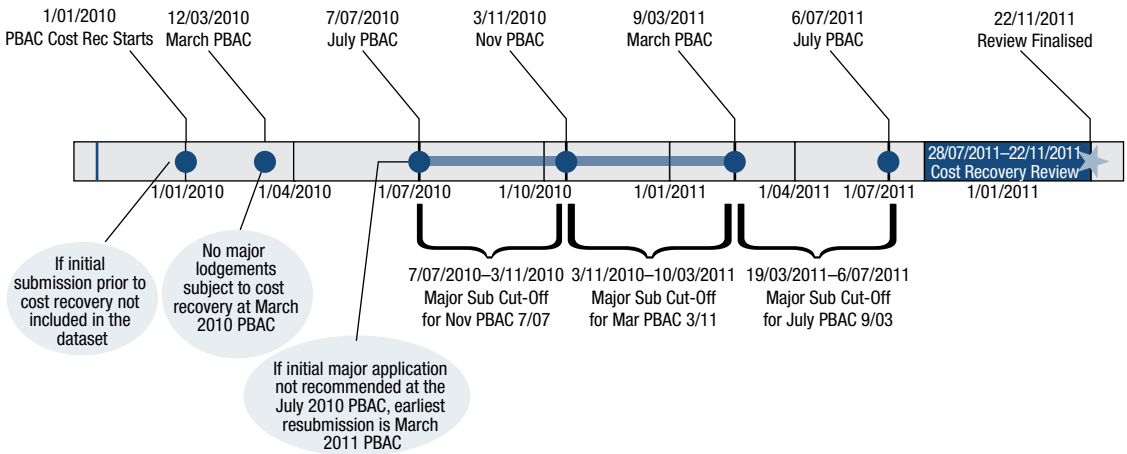
- The word 'approved' is defined as a positive recommendation by the PBAC.
- All data from PBAC meetings held between July 2010 and July 2011 for applications subject to cost recovery are included.
- Any resubmissions where the initial submission was lodged before 1 January 2010 are excluded.
- All lodgement categories (major, minor, secretariat listing) are included, but not generic applications.

Within the time frame of this review, the Committee noted that the available data does not make it possible to determine the results of this term of reference.

The cut-off date for major submissions is generally 17 weeks before a PBAC meeting (18 weeks over the Christmas to New Year period). For minor submissions it is 11 weeks before a PBAC meeting. Given the lead times associated with the submission of an application, there may be limited opportunity for an application not recommended by the PBAC to be resubmitted in the period from 1 January 2010 to July 2011, particularly a major application. For example, if a major application was not recommended at the July 2010 PBAC meeting, the earliest resubmission date for a major resubmission would be the March 2011 PBAC meeting (see Figure 3). The Committee noted that this limits the conclusions that can be made on the impact of cost recovery on resubmissions.

Where the PBAC does not recommend an application, the sponsor can choose to resubmit. From the commencement of cost recovery until 31 August 2011, a total of 217 applications were subject to cost recovery and considered by the PBAC. Of these, 60 applications (42 major, 17 minor and one secretariat listing) were not recommended and eight were deferred (including resubmissions). During the same period a total of 223 generic applications were submitted.

FIGURE 3 ILLUSTRATION OF RESUBMISSION CYCLE



Within the time frame, only three submissions that were initially rejected have been resubmitted and gained a positive recommendation. The PBAC reasons for not recommending the initial applications are listed in Table 5.

The Committee noted that it is possible that cost recovery could have limited the number of submissions with poorly substantiated claims and/or improved the quality of applications because it provides a financial incentive to minimise the probability of a PBAC rejection. This may also have limited the number of resubmissions. However, the limited time frame does not allow any meaningful conclusions to be drawn at this point in time.

TABLE 5 SUBMISSIONS INITIALLY NOT RECOMMENDED BUT WHICH RECEIVED A POSITIVE RECOMMENDATION AFTER RESUBMISSION²

Ref	Drug	Indication	Reason for PBAC not recommending at initial PBAC Meeting
1	OXYCODONE (as hydrochloride) with naloxone (as hydrochloride) dehydrate, tablet, 5 mg–2.5 mg, 10 mg–5 mg, 20 mg–10 mg and 40 mg–20 mg (controlled release), Targin®	Restricted benefit listing for the treatment of chronic, severe, disabling pain not responding to non-narcotic analgesics.	The PBAC rejected the application on the basis of uncertain clinical efficacy and a very high and uncertain cost-effectiveness ratio.
2	ELTROMBOPAG, tablets, 25 mg and 50 mg (as olamine), Revolade®	Section 100 (Highly Specialised Drugs Program) Public and Private hospital authority required listing for severe thrombocytopenia in adult patients with severe chronic immune (idiopathic) thrombocytopenic purpura (ITP) meeting certain criteria.	The PBAC rejected the submission on the basis of uncertain clinical effectiveness in comparison with Romiplostim.
3	CORIFOLLITROPIN ALFA(rch), solution for injection, 100 micrograms in 0.5 mL pre-filled syringe, 150 micrograms in 0.5 ml pre-filled syringe, Elonva®	Section 100 (IVF/GIFT Program) listing for patients who are receiving medical treatment as described in items 13200, 13201 or 13202 of the Medicare Benefits Schedule.	The PBAC rejected the submission on the basis of uncertainty about the claim that it was non-inferior in terms of comparative effectiveness and safety to its comparator (Follitropin beta), and the uncertainty in the cost-minimisation analysis resulting from this clinical uncertainty and from the pricing structure proposed by the Sponsor.

A further four submissions that were initially not recommended by the PBAC were resubmitted and subsequently not recommended for a second time. The detailed reasons for not recommending listings are summarised in Table 6.

TABLE 6 REASONS FOR PBAC NOT RECOMMENDING INITIAL AND SUBSEQUENT SUBMISSIONS³

Ref	Drug	Indication	Reason for PBAC not recommending at initial PBAC meeting	Reason for PBAC not recommending resubmission
1	AGOMELATINE, tablet, 25 mg, Valdoxan®	Restricted Benefit listing for major depressive disorders.	The PBAC rejected the submission because of uncertainty around the claim that Agomelatine is superior to Venlafaxine and the resultant uncertainty in the economic analysis.	The PBAC rejected the submission on the basis that superior clinical effectiveness and safety over selective Serotonin Reuptake Inhibitors (SSRIs) had not been demonstrated.
2	ICATIBANT, injection, 30 mg in 3 mL (as acetate), single use pre-filled syringe, Firazyr®	Authority Required listing for anticipated emergency treatment of an acute attack hereditary angioedema in a patient with confirmed diagnosis of C1-esterase inhibitor deficiency.	The PBAC rejected the submission because of insufficient evidence in the proposed setting to support the clinical place of the therapy and uncertain cost effectiveness.	The PBAC rejected the submission on the basis that uncertainty remained over the extent of clinical benefit in the self-administration setting and the resultant uncertain and unacceptably high cost-effectiveness ratio.
3	COLISTIMETHATE SODIUM, powder for nebuliser solution, 1 million IU (equivalent to 80 mg colistimethate sodium), Tadim®	Authority required listing for treatment of Pseudomonas aeruginosa infection in patients with cystic fibrosis.	The PBAC rejected the submission on the basis of uncertain clinical benefit, uncertain clinical place in therapy and the resultant uncertain cost effectiveness.	The PBAC rejected the submission on the basis of uncertain clinical benefit and inadequate evidence of cost effectiveness.

TABLE 6 REASONS FOR PBAC NOT RECOMMENDING INITIAL AND SUBSEQUENT SUBMISSIONS³

Ref	Drug	Indication	Reason for PBAC not recommending at initial PBAC meeting	Reason for PBAC not recommending resubmission
4	LIRAGLUTIDE (rys), solution for injection, 3 mL pre-filled injection pen, 6 mg per mL, Victoza®	Resubmission for an Authority required listing for treatment of type 2 diabetes: 1) as triple combination therapy with metformin and a sulphonylurea; 2) as dual combination therapy with metformin or a sulphonylurea in patients for whom a combination of metformin and a sulphonylurea is contraindicated or not tolerated.	The PBAC rejected the submission because of an unacceptably high and uncertain cost-effectiveness ratio.	The PBAC rejected the submission on the basis of uncertain cost effectiveness.

Finding 1: Despite the limited number of resubmissions since the commencement of cost recovery, the Committee noted that the reasons most commonly cited by the PBAC were inadequate clinical and/or cost-effectiveness evidence provided in the submission.

Finding 2: The Committee concluded that, because of the limited dataset available, it was unable to provide a meaningful conclusion with regard to terms of reference item (a).

4.2 Terms of reference item (b): the average fee for submissions by type of submission (major/minor/generic) according to Department of Health and Ageing classifications

Cost recovery fees are collected at two points in the PBS and NIP listing processes: first, when submissions are lodged for consideration by the PBAC; and, secondly, at the point of listing. Over the review period from 1 January 2010 to 31 August 2011, the total fees collected for all lodgements and listings (including generic applications) was \$9.33 million. The Committee noted that this compares to the predicted annual revenue of \$14 million as described in the Portfolio Budget Statements 2010–11.

Some applications may receive a fee waiver or an exemption for lodgement and/or listing. These waivers and exemptions dilute the overall average of fees raised for both lodgement and listing.

The Committee also noted that generic listings attract a fee of \$500, which, because of the number of such listings, further biases the overall average of fees collected on an average basis. Further, the Committee noted that:

- the total amount of lodgement fees (excluding generic submissions) collected between 1 January 2010 and 31 August 2011 was \$8.78 million
- generic submissions attract an administration fee of \$500. There was a total of \$111 500 in fees collected for submissions for generic drug products. Such submissions do not qualify for a fee waiver or exemption. Fees for generic drugs represent 1.2 per cent of the total fee revenue under cost recovery
- the total amount of fees collected for pricing is \$435 000 based on 67 applications where a fee was charged. An additional 15 applications received a pricing fee waiver or exemption. Listing fees represent 4.7 per cent of the total fee revenue under cost recovery.

Table 7 shows the average lodgement fee by submission type, including waivers and exemptions. Over the period reviewed, there were 217 general applications and 223 generic applications.

TABLE 7 AVERAGE LODGEMENT FEE BY TYPE, INCLUDING WAIVERS AND EXEMPTIONS*

	Fees collected	Total applications	Lodgement exemption granted	Lodgement waiver granted	Average fee
PBAC Evaluation— Major applications	\$8 006 500	88	1	20	\$90 983
PBAC Evaluation— Minor applications	\$737 500	79	10	8	\$9335 [#]
PBSC Secretariat Listing	\$39 000	50	3	6	\$780 [^]
Generics	\$111 500	223	—	—	\$500

* Data in Table 7 does not include pricing submissions.

[#] Average includes two minor applications that were withdrawn before the PBAC meeting, where no fee was charged.

[^] Average includes two Secretariat Listing applications that were withdrawn, where no fee charged.

Finding 3: The Committee noted that the number of fee exemptions and waivers will significantly impact on the total fees collected for lodgement and thus the average fee paid for each classification.

Finding 4: It has not been possible to present the pricing fee data in the same way as the lodgement fee data due to a difference in categorisation. The Committee noted that there were 82 pricing applications (including 15 fee waivers and exemptions) with total pricing fees of \$435 000, equating to an average of \$5304 per application.

Finding 5: The Committee noted that cost recovery revenue is significantly lower than was anticipated when the measure was introduced.

4.3 Terms of reference item (c): the number of applications where the population is likely to be small and utilisation of the drug, medicinal preparation or vaccine is likely to be highly targeted

Note

For the purposes of this review, the Committee defined 'small population' as an estimate of 2000 scripts in the first year of operation.

Terms of reference item (c) of the review seeks data on the number of applications where the population is likely to be small and use of the drug, medicinal preparation or vaccine is likely to be highly targeted. There is no universally accepted definition of 'small population'. For the purposes of this review, it has been defined as an anticipated claim by the sponsor in its submission of fewer than an estimated 2000 prescriptions within the first year of listing.

The Committee noted that:

- 2000 prescriptions may not equal 2000 patients—for example, one patient may require a single prescriptions for a once-in-a-lifetime vaccine, while another patient may require multiple prescriptions over a period of time
- while 2000 prescriptions may appear to be a low number, the cost to government may be extremely high—for example, the annual cost per patient of high-cost drugs for the Life Saving Drugs Program (LSDP) may exceed \$500 000
- in the first 12 months of listing, the uptake of new products can be slow, therefore this period may not be an accurate indicator of future use
- not all products with a predicted use of fewer than 2000 prescriptions in the first year are designated as orphan drugs, which are exempt from evaluation and pricing fees under the Regulations.

It is estimated that, from a total of 217 lodgements (excluding applications withdrawn by sponsors), 73 fell into the category of fewer than 2000 prescriptions predicted in the first year of listing. This represents 33.6 per cent of all applications that are estimated to fall into a small or highly targeted population utilisation category (see Table 8).

TABLE 8 NUMBER OF APPLICATIONS WHERE FEWER THAN 2000 PRESCRIPTIONS ARE LIKELY IN FIRST YEAR

PBAC meeting	Jul 2010	Nov 2010	Mar 2011	Jul 2011	Total
PBAC Evaluation—Major application	7	12	12	15	46
PBAC Evaluation—Minor application	5	6	5	8	24
PBAC Secretariat Listing	1	1	0	1	3
Total small population	13	19	17	24	73

Total submissions	53	55	52	57	217
Percentage small population	24.5	34.5	32.6	42	33.6

Finding 6: The Committee noted that, from the total number of applications submitted, approximately one-third of sponsors have predicted an estimated use of fewer than 2000 prescriptions in the first 12 months of listing. They also noted that a number of these products had their fees waived or exempted under existing cost recovery criteria.

Finding 7: The Committee noted that, even though the estimated use in the first 12 months may be fewer than 2000 prescriptions and/or the product may be exempted because it has been designated an orphan drug or considered for the LSDP, the cost to government may still be a significant amount due to the high unit cost.

4.4 Terms of reference item (d): the number of reviews requested by applicants

Under Part 4.3 of the Regulations, an applicant may seek an independent review of a decision by the PBAC not to make a recommendation that the Minister:

- declare a drug or medicinal preparation under s. 85(2) of the National Health Act
- specify further circumstances, requested in an application, in which a prescription for the supply of a pharmaceutical benefit may be written under s. 85(2A) of the National Health Act.

For the period reported, there were no requests for independent reviews of a PBAC decision or requests for a review of a waiver or exemption decision.

Finding 8: The Committee concluded that there is insufficient data to determine that cost recovery has impacted on the number of independent reviews of a PBAC decision, as no reviews have been requested.

4.5 Terms of reference item (e): the number of fee waivers given to applicants and the reasons why waivers were given

4.5.1 Evaluation fee waivers and exemptions requested and granted

Fee waivers and exemptions can be applied at lodgement and/or listing. In accordance with Part 5.2 of the Regulations, an application to the PBAC may receive a fee waiver if it is considered to be in the public interest and payment of the fee would make the application financially non-viable.

Additionally, under reg. 5.1 certain applications receive an exemption from fees. These include designated orphan drugs, products considered necessary for a public health event of national significance and products required for public health emergencies under the *Quarantine Act 1908* and other circumstances as outlined in Part 5 of the Regulations.

Tables 9, 10 and 11 indicate the number of waivers and exemptions requested and granted, the percentage of the total that received a waiver or exemption and the reason they were given.

TABLE 9 TOTAL LODGEMENT FEE WAIVERS AND EXEMPTIONS REQUESTED AND GRANTED AND PERCENTAGE OF THOSE GRANTED

Category	Lodgement waivers					Lodgement exemptions				
	Jul 2010	Nov 2010	Mar 2011	Jul 2011	Total	Jul 2010	Nov 2010	Mar 2011	Jul 2011	Total
Major requested	0	0	0	1	1	5	5	5	5	20
Major granted	0	0	0	1	1	5	5	5	5	20
Minor requested	1	7	1	2	11	3	1	2	3	9
Minor granted	1	6	1	2	10	2	1	2	3	8
Secretariat Listing requested	4	0	0	1	5	5	1	0	1	7
Secretariat Listing granted	2	0	0	1	3	4	1	0	1	6
Total requested	5	7	1	4	17	13	7	7	9	36
Total granted	3	6	1	4	14	11	7	7	9	34
Percentage granted	60	86	100	100	82	85	100	100	100	94

TABLE 10 PERCENTAGE OF SUBMISSIONS THAT RECEIVED A LODGEMENT EXEMPTION OR WAIVER

PBAC meeting	Jul 2010	Nov 2010	Mar 2011	Jul 2011	Total
Number of submissions	53	55	52	57	217
Number of exemptions and waivers granted	14	13	8	13	48
Percentage of submissions that received an exemption or waiver	26	24	15	23	22
Fees forgone for submissions that received an exemption or waiver	\$641 000	\$686 000	\$635 000	\$781 500	\$2 743 500

A total of five requests for a lodgement fee waiver or exemption were not granted.

Within the time frame of this review, 48 requests for waivers and exemptions were granted. This equates to \$2 743 500 of forgone fees.

TABLE 11 REASONS FOR GRANTING / NOT GRANTING EVALUATION FEE WAIVERS AND EXEMPTIONS

Type	Outcome	Number	Reason	Regulation
Waiver	Granted	14	The application involved the public interest and the evaluation fee would make the application financially non-viable.	5.2
Waiver	Not granted	3	The sponsor's claim that the evaluation fee would make the application financially non-viable was not accepted by the department.	5.2
Exemption	Granted	32	Designated orphan drug.	5.1
Exemption	Granted	2	Change in pack size with no price implications.	5.1
Exemption	Not granted	1	The sponsor provided inadequate supporting documentation.	5.1

4.5.2 Pricing fee waivers and exemptions requested and granted

It has not been possible to present the pricing exemption fee waiver data in the same way as the lodgement fees waiver and exemption data due to a difference in process: when an exemption is granted at lodgement, it is automatically granted at the pricing stage; therefore, the exempted pricing applications are not categorised.

However, the Committee noted that, during the period of the review, of those applications not granted submission waivers, only two pricing fee waivers were requested. One was granted and the other was not recommended on the basis that it did not demonstrate that the pricing fee would make the submission financially non-viable. Of those applications that were recommended by the PBAC that were considered by the PBPA, 14 were designated orphan drugs and therefore exempt from fees. Table 12 outlines the reasons for granting pricing exemptions and waivers.

TABLE 12 REASONS FOR GRANTING PRICING FEE WAIVERS AND EXEMPTIONS

Type	Number	Reason	Regulation
Waiver	1	The sponsor claimed the pricing fee would make the application financially unviable.	5.2
Exemption	14	Designated orphan drug.	5.1

Finding 9: The Committee found the majority of requests for lodgement fee waivers and exemptions were granted. Those that were not granted were not able to satisfy the requirements for financial non-viability or were not able to provide adequate supporting documentation.

Finding 10: The Committee noted that, for the period 1 January 2010 to 31 August 2011, \$2 743 500 of lodgement fees were waived or exempted.

Finding 11: The Committee noted that approximately one in five of all categories were granted a lodgement fee exemption or waiver.

4.6 Terms of reference item (f): the length of time taken for submissions to be approved

4.6.1 Average days from receipt of application to publication of PBAC

Notes

- The word 'approved' is defined as a positive recommendation by the PBAC.
- Generic products are excluded, as they are already deemed to be bioequivalent to an existing listed product and have a different process for listing.
- Applications that are under active consideration, not recommended, withdrawn or PBAC deferred and government deferred are excluded.
- The data used to evaluate this term of reference is based on July 2010, November 2010 and March 2011 PBAC meetings only.

There are a number of steps in the process of gaining listing of a product on the PBS and NIP, including:

- seeking pre-submission advice from the department (optional but recommended)
- making an application to the PBAC
- communicating PBAC evaluation and recommendations
- conducting pricing negotiations
- providing formal advice of listing.

The cut-off date for the receipt of major submissions is generally 17 weeks (119 days) before a PBAC meeting (18 weeks over the Christmas to New Year period). It is 11 weeks (77 days) for minor submissions. The PBAC is required to consider a submission within this agreed time frame. While advice is sent to applicants early, PBAC outcomes are published online and may take a further six to 10 weeks (42–70 days). Thus the maximum time for receipt of an application for public release of a PBAC recommendation is up to 189 days for a major submission.

The Committee noted that a more detailed analysis of submission trends is available in the PBAC key performance indicator (KPI) report, which is available at www.health.gov.au/internet/main/publishing.nsf/Content/health-pbs-activity-indicators

Table 13 shows the average number of days from receipt of an application to the publishing of outcomes on the department website. It shows that for major applications this is around 23 weeks (166 days), while for minor applications the period is around 17 weeks (119 days) and for a secretariat listing it is around 16 weeks (115 days).

TABLE 13 AVERAGE DAYS FROM RECEIPT BY PBAC TO ONLINE PUBLICATION OF OUTCOME

PBAC meeting date	Jul 2010	Nov 2010	Mar 2011	Jul 2011	All	Median
PBAC Evaluation – Major	162	163	169	167	166	163
PBAC Evaluation – Minor	114	117	125	123	119	127
PBAC Secretariat Listing	112	111	108	128	115	127
Overall average	127	137	136	148	137	127

Table 14 shows the average number of days from receipt of an application to listing.

TABLE 14 AVERAGE DAYS FROM RECEIPT TO LISTING*

PBAC meeting date	Jul 2010	Nov 2010	Mar 2011	All ⁺	Median
PBAC Major	318	310	281 [#]	306	269
PBAC Minor	256	190	208	229	198
PBAC Secretariat Listing	229	170	168	194	193
Overall average	252	217	193	227	199

* Includes only positive recommendations made between the date that recommendations are published online and the date that listing occurs.

⁺ The July 2011 PBAC meeting has no outcomes associated with submissions as at the time of this report.

[#] Based on two applications to 31 August 2011.

Finding 12: The Committee noted that there is insufficient data available at this stage to conclude whether cost recovery impacted on the average time frames to listing.

Finding 13: The Committee noted that the median number of days from receipt by the department to the publication of a positive PBAC outcome online is 127 days. This is in accord with the agreed time frames. The median number of days from receipt by the department to listing is 199 days.

Finding 14: The Committee noted the median time frame of two to three months from publication of the PBAC meeting outcomes to listing demonstrates reasonable efficiency given the legislative and administrative requirements that must be complied with.

4.7 Terms of reference item (g): the number of applications that fail to gain a listing, the reasons why and the types of drugs concerned

For the purposes of the review, 'fail to gain a listing' is defined as:

- failing to gain a positive recommendation from the PBAC
- being unable to satisfy the requirements of the PBPA, including agreement on pricing or risk share arrangements
- withdrawal by the sponsor
- PBAC recommendations not being accepted by government.

As at 31 August 2011, a total of 217 applications were subject to cost recovery. Of the 217, 78 were recommended, 57 were still under active consideration and 14 were withdrawn by the sponsor. A total of 68 (including those not recommended or deferred by the PBAC) of the 217 applications failed to gain listing. This represents 31 per cent of the applications.

Of those that failed to gain listing, 60 were not recommended by the PBAC. The Committee noted that these applications can be reconsidered at any stage when the sponsor resubmits. Further details for each application are available in PBAC public summary documents online at www.health.gov.au/internet/main/publishing.nsf/Content/pbac-outcomes-info

The Committee found that it was not possible to ascertain the types of drugs that were not listed, as the data is not available.

TABLE 15 REASONS FOR APPLICATIONS NOT ACHIEVING LISTING AS AT 31 AUGUST 2011

Reasons that applications failed to gain listing	Number
Not Recommended by PBAC—Major	42
Not Recommended by PBAC—Minor	17
Not Recommended by PBAC—Secretariat Listing	1
Withdrawn by applicant	14
PBAC deferred	8

Finding 15: The Committee noted there is insufficient data available at this stage to determine whether cost recovery had impacted on the number of applications that fail to gain a listing.

Finding 16: The most common reason that an application fails to gain a positive PBAC recommendation is inadequate clinical and/or cost-effectiveness evidence that was satisfactory to the PBAC.

Finding 17: The Committee noted the PBAC sought clarification of issues for the eight products it deferred. The issues related to wording of the restriction or reconsideration of price in order to achieve an acceptable incremental cost-effectiveness ratio.

4.8 Terms of reference item (h): any increase in operating costs of the Pharmaceutical Benefits Advisory Committee

The cost recovery model developed between 2006 and 2008 was intended to ensure that the costs of all regulatory activities associated with the listing of products on the PBS or NIP are recovered, whilst ensuring that the arrangements comply with the Australian Government Cost Recovery Guidelines.

The Committee noted that the function of the PBAC is only one of many activities that are cost recovered and is part of the total operating cost of running the entire listing process, from pre-approval engagement to listing. The operating costs of the PBAC, as per terms of reference item (h), serve as a proxy to understand if the costs of administering the program have changed since the introduction of cost recovery.

The direct operational costs of the PBAC during the time frame examined in this review increased by 5.2 per cent⁴ compared with the four PBAC meetings before the introduction of cost recovery. This increase shows that the cost of operating the PBAC itself has not increased significantly beyond the CPI, despite fluctuations in the number of people that attend PBAC meetings and changes in the location and duration of those meetings.

The Committee noted that the current cost recovery model does not take into account the cost associated with a number of current functions and activities associated with the evaluation and listing process. Examples include consumer input, enhanced early dialogue with sponsors and the management of complex listings, including risk-sharing agreements.

There have been recent initiatives to improve consumer input into the decision-making process, but these have been on an ad hoc basis following advice from the PBAC. The Committee also noted that these activities have been fully funded by the government, but the cost is not included in the cost recovery model. The Committee also noted that a more formalised and systematic approach to consumer involvement had been recommended in the recent Health Technology Assessment Review⁵ report. This is consistent with an international trend toward enhanced consumer input. The Committee acknowledged that a more systematic approach to consumer input would be beneficial but notes that this would add additional cost to the process.

4 Comparison is based on the direct operational cost of the November 2008, March 2009, July 2009 and November 2009 meetings with the March 2010, July 2010, November 2010 and March 2011 meetings. Direct operational costs include Committee member salaries, estimated printing, venue hire, catering, accommodation, fares and travel costs.

5 Health Technology Review, <http://www.health.gov.au/htareview>

The requests by industry for more formalised early dialogue for future submissions would also add a significant cost for government. These, and other program enhancement costs, were not included in the original cost recovery model.

Further, the Committee noted that the management of complex listings can involve considerable costs to the department. The use of risk-sharing and other special listing arrangements has increased in recent years. These arrangements often require considerable legal input and the need for regular monitoring of use to inform the management of associated deeds of agreement.

The Committee noted the original cost recovery model developed between 2006 and 2008 took into account costs known at the time. However, it has become evident that the current cost recovery model does not include some costs that have subsequently become relevant.

Finding 18: The Committee noted that the current initiatives to improve consumer input are not included in the cost recovery model. Further, the Committee noted that enhancement of input by the introduction of a systematic approach would result in substantial costs.

Finding 19: The Committee noted that there has been an increased administrative burden and associated costs due to the development and monitoring of a greater number of special listing arrangements.

Finding 20: The Committee noted that if pre-submission meetings were formalised it would provide an opportunity for early dialogue with sponsors, which would include discussion surrounding clinical trial design. This activity would impact on the total cost of running the program.

Finding 21: The Committee noted that there is no evidence that the cost of running the PBAC itself has increased as a result of the introduction of cost recovery.

4.9 Terms of reference item (i): any increase in the cost of pharmaceutical benefits scheme medications to patients

4.9.1 Patient co-payments

All Australian residents are eligible to access the PBS and are required to pay a co-payment to access the product as a pharmaceutical benefit. From 1 January 2011, a general patient will pay up to \$34.20 for most medicines listed on the PBS. People with concession cards pay \$5.60. Safety net provisions exist.

The Committee noted that there was no available evidence to determine whether or not sponsors had increased their base price to accommodate cost recovery fees. Data on average additional payment for therapeutic group premium or brand premium is not currently available.

Cost recovery will not affect the current co-payment arrangements.

TABLE 16 CO-PAYMENT OVER PERIOD USED IN THE REVIEW**

Date of change	Concessional beneficiaries co-payment	General beneficiaries co-payment
1 January 2010	\$5.40	\$33.30
1 January 2011	\$5.60	\$34.20

* Co-payments include the costs to both inpatients and outpatients. These are not separately defined and do not impact on the cost recovery fee structure.

+ Safety net provisions apply.

Finding 22: The Committee noted that cost recovery does not directly affect the current NIP arrangement whereby the Commonwealth, via the States and Territories, provides vaccines free of charge to health providers to administer to eligible patients in the community.

Finding 23: The Committee noted that co-payments will not increase as a result of cost recovery.

4.10 Terms of reference item (j): any other matters considered relevant

4.10.1 Cost recovery model

The Committee requested information from the department on the current cost recovery model in order to determine whether costs of other activities currently undertaken but not included in the model should be considered for recovery—for example, more formalised consumer engagement and the recovery of fees waived and exempted for the LSDP and those drugs designated as orphan drugs.

The current cost recovery model was developed to ensure that all regulatory activities associated with the listing of products on the PBS and NIP are recovered and that the cost recovery arrangements comply with the Australian Government Cost Recovery Guidelines.

The activities that are reflected in the fees currently charged include:

- pre-approval activities comprising:
 - initial liaison with sponsors
 - receipt and registration of submissions
 - evaluation of submissions
 - Committee reviews (Economics Sub Committee (ESC), Drug Utilisation Sub Committee (DUSC) and the Pharmaceutical Benefits Advisory Committee (PBAC))
 - PBPA pricing reviews
- entering recommended medicines on the PBS
- monitoring and compliance with restrictions
- tracking and monitoring of submissions
- service to parliament related to PBS listing activities.

The current cost recovery model does not include the cost of formal consumer input or a request for early dialogue by sponsors apart from standard pre-submission meetings. However, the government has on occasion provided funding for consumer input on selected products where it was deemed to provide important information for consideration by the PBAC and for early dialogue exchanges. These were not incorporated into the cost recovery model when the fees and charges were developed between 2006 and 2008 before the introduction of cost recovery.

Further, the current model also does not include the ongoing cost of monitoring risk share arrangements between the Commonwealth and pharmaceutical companies or the activities associated with requests for price increases after a product is listed.

In the original cost recovery model, the volume of submissions where a fee would be waived or exempted was taken into account. This ensured that the fees that were waived or exempted were not being included in the fees payable by sponsors whose submissions were not eligible for a fee waiver or exemption. The cost of evaluating submissions where the fee is waived or exempted is funded by government, which is considered to be consistent with the Australian Government Cost Recovery Guidelines.

The Committee noted that some LSDP drugs and designated orphan drugs are fee exempt under the Regulations and yet can involve substantial cost to government.

Finding 24: The Committee noted that a submission for a life-saving or designated orphan drug is likely to have its fees exempt or waived. However, the criteria for a fee waiver should take into account the combined total cost to government and total projected profit. This would address the issue of highly profitable products being automatically exempt from paying cost recovery fees because of their classification.

4.10.2 Submissions

The Committee extended to Medicines Australia, Generic Medicines Industry Association of Australia (GMiA), AusBiotech and the Consumers Health Forum of Australia (CHF) the opportunity to provide a submission. These four stakeholders were identified because they are directly subject to PBS cost recovery and are representatives for professional and consumer groups. All four organisations lodged a submission for the Committee's consideration. A summary of these submissions is outlined below.

4.10.2.1 Consumers Health Forum of Australia

CHF stated that consumers are a key stakeholder in the PBAC and other medicine and therapeutic goods policy discussions. CHF would like to see greater consistency of involvement of consumers in the PBS process and would like to see this activity funded by industry as part of the cost recovery arrangements. Further, CHF argued that increased funding would allow better balanced, informed and researched responses to therapeutic goods issues.

CHF considers that stronger consumer engagement and involvement, especially in PBAC and other therapeutic goods policy processes, can be better supported through a range of measures such as:

- consumer impact statements
- direct involvement with consumer bodies
- support for PBAC representatives.

COMMITTEE RESPONSE: The Committee agreed with the case for enhancement of consumer engagement. The Committee noted that current consumer engagement is funded by the government. Any future review of the cost recovery model should consider systematic funding of consumer engagement to inform better decision making. Further, the Committee noted that, if formal consumer engagement were to be introduced, this would be an additional cost outside the scope of the current cost recovery model. It was noted that CHF could be one of a number of organisations that could assist with consumer engagement in the future.

4.10.2.2 *Generic Medicines Industry Association*

GMiA submitted that any medicines that trigger a statutory price reduction, by either a fixed percentage rate or price reduction strategy, on the PBS should be exempt from all PBAC fees. It states that the government cost recovery measures for PBS and NIP listings have the potential to create an unnecessary financial barrier to market entry for second and subsequent brands of medicines.

GMiA submitted that the listing of second and subsequent brands of medicines on the PBS delivers important savings to the public. It argued that subsequent brands and medicines may never enter the Australian market due to high costs of registration and PBS listing and thus important public savings to the PBS would be forfeited.

COMMITTEE RESPONSE: The Committee noted that the current fee for generic drug listings is \$500 and that this fee is unlikely to be a genuine barrier to a sponsor requesting listing. The Committee noted that a \$500 fee associated with generic drug applications would not cover the costs of the administration of listing of a generic product.

4.10.2.3 *AusBiotech and Medicines Australia*

AusBiotech and Medicines Australia provided a joint submission. They also provided the data from an online survey of their members on pre- and post-PBAC process and administrative experiences that informed their response.

Medicines Australia and AusBiotech remain opposed to cost recovery arrangements for the listing of medicines on the PBS and designating vaccines on the NIP. The submission states that fees increase the cost of applications, potentially compromising patients' access to new medicines. The submission also cites five instances from the industry survey where they believe submissions were delayed or not lodged due to cost recovery.

Further, they stated in their submission that cost recovery arrangements are inconsistent with the National Medicines Policy and the government's own Cost Recovery Guidelines. In the event that cost recovery continues, the submission requests that government engage Medicines Australia and AusBiotech to improve existing pre-submission meetings by introducing better structure around their preparation to implement quality assurance measures to maintain existing high standards to the benefit of the PBAC, industry and the Australian community.

Medicines Australia and AusBiotech also recommended that quality assurance measures around the evaluation process be introduced and a set of guiding principles be developed with industry to address efficiency and quality concerns.

COMMITTEE RESPONSE: The Committee acknowledged that AusBiotech and Medicines Australia are opposed in principle to cost recovery arrangements for the listing of medicines on the PBS and NIP. However, these concerns are outside of the terms of reference of this review.

The Committee notes that the process for cost recovery arrangements is relatively new and there is always room for improvement. It suggests that any process or policy improvements should be raised with the department independently of the review. Further, the Committee noted that, if formal quality assurance processes were to be introduced, this would be an additional cost outside the scope of the current cost recovery model.

While the submission cites instances from their survey relating to drugs that were delayed or not lodged due to cost recovery, the Committee was not provided with any evidence to support this claim. The Committee notes that fee waivers and exemptions are available for applicants.

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