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Analysis of sponsor hearings on health technology assessment decision making

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Abstract

Objective. The aim of this study was to get a better understanding of the frequency of Pharmaceutical Benefits Advisory Committee (PBAC) hearings, the factors that influence a sponsor's decision to proceed with a hearing and to assess the impact hearings may have had on PBAC decision making.

Methods. All public summary documents (PSDs) from March 2014 to November 2016 PBAC meetings, obtained from the Pharmaceutical Benefits Scheme (PBS) website, were examined to identify major submissions for which sponsor hearings were conducted. Each PSD was analysed to determine the topics discussed at the sponsor hearing and the 'usefulness' of a sponsor hearing from the PBAC's perspective.

Results. During the study period there were 472 PSDs. 74 sponsor hearings (28% of major submissions) were conducted during the study period. A clinician external to the sponsor presented at the majority of the hearings (78%) and accordingly, the main topics presented related to clinical positioning/use and clinical benefit/use.

Conclusion. The PBAC considered approximately 45% of sponsor hearings to be informative or moderately informative whereas 18% were classed as uninformative.

What is known about the topic? Although the sponsors of medicines being considered by the Pharmaceutical Benefits Advisory Committee (PBAC) for public subsidy have been able to give a 10 min presentation to the Committee at the time of decision making for several years, it is unknown whether these hearings are beneficial.
What does this paper add? We present what is believed to be the results of the first analysis of PBAC sponsor hearings.
What are the implications for practitioners? All stakeholders should consider the findings of our research and associated recommendations to ensure that future sponsor hearings enhance PBAC decision making and promote good public health policy.

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Introduction

The Australian Pharmaceutical Benefits Scheme (PBS) is a federally funded national program that subsidises the cost of prescription medicines for Australians. The PBS accounts for approximately 80% of the cost of prescription medicines in Australia. Submissions for listing a medicine or vaccine on the PBS are typically prepared by a sponsor, usually a pharmaceutical company, and assessed by the Pharmaceutical Benefits Advisory Committee (PBAC), which provides advice to the Minister for Health regarding the funding of new pharmaceuticals on the PBS. This advice results from a formal evaluation of the clinical and economic evidence relating to the medicine under consideration. No new medicine may be made available on the PBS unless recommended by the PBAC.[1]

The PBAC meets three times a year to consider submissions from sponsors. Submissions to the PBAC are broadly classified as either major (for applications relating to new medicines or indications) or minor (for small changes, such as pack sizes, price changes or new strengths). The PBAC can recommend PBS/National Immunisation Program (NIP) listing, reject the submission or defer a decision pending further information.[1]

Historically, PBAC meetings have been held behind closed doors with no opportunity for the sponsor to engage in any direct dialogue with the PBAC members at the time of decision making. However, as a condition of the Free Trade Agreement signed by Australia and the US (AUSFTA) on 1 January 2005, sponsors or their representatives now have the opportunity to

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make an oral presentation during the PBAC meeting, before the PBAC's deliberation.[2,3] This presentation is referred to as a 'sponsor hearing' and is only allowed for major submissions. The content of a sponsor hearing is limited to matters raised in the submission or during the evaluation of the submission, and no new evidence can be presented. Members of the sponsor company and/or one of its representatives may deliver the presentation, with each hearing lasting up to 10 min. Hearings are requested by the sponsor, and although it is anticipated that most or all of those requests are accepted, it is unknown whether any have been rejected. A hearing provides the sponsor with the opportunity to address outstanding issues that have not been resolved during the submission evaluation process and provide PBAC members with an opportunity to ask the applicant questions about the content of the submission, clinical practice or quality use of medicine issues.[3]

An additional initiative from the AUSFTA has been the publication of public summary documents (PSDs) on the PBS website.[2] PSDs provide public information about PBAC decisions and the basis and rationale for each decision. A PSD is published for each submission, except for those where only pricing matters are considered. PSDs are downloadable from the Department of Health (DoH) website (http://www.pbs.gov.au/info/industry/listing/elements/pbac-meetings/psd, accessed 23 October 2017) and are published approximately 3 months after the PBAC meeting. The content of a PSD closely reflects the PBAC's meeting minutes. As such, PSDs provide a comprehensive overview of the PBAC submission, including information related to the clinical evidence and economic analysis[2] and information on sponsor hearings. Although PSDs have been in the public domain since 2005, their content has evolved considerably over time. Details relating to sponsor hearings have only been consistently included in the PSDs since 2014. In our study, PSDs were used as the source for capturing data relating to sponsor hearings.

To our knowledge, sponsor hearings have not been formally evaluated despite being part of the PBAC decision-making process for over a decade. The objectives of the present study were to get a better understanding of the frequency of PBAC hearings, the factors that may influence a sponsor's decision to proceed with a hearing and to assess the effect hearings may have had on PBAC decision making. This analysis may assist applicants in making more informed decisions relating to sponsor hearings. This may also facilitate further dialogue between the pharmaceutical industry, the DoH and the PBAC to ensure that sponsor hearings are optimised to help with consistent and transparent decision making.

Methods

The specific objectives of this study were to: (1) determine the proportion of PBAC major submissions for which sponsor hearings were conducted; (2) classify sponsor hearings according to therapeutic area; (3) assess whether the PBAC considered sponsor hearings to be useful; and (4) determine whether there were any characteristics of sponsor hearings that were particularly relevant to PBAC decision making.

PSDs are the only public data source providing information on sponsor hearings. We examined all PSDs from the March 2014 to November 2016 PBAC meetings, obtained from the PBS website (http://www.pbs.gov.au/info/industry/listing/elements/pbac-meetings/psd, accessed 23 October 2017). March 2014 was chosen as the starting point because this was the first time that the PSDs consistently included a section detailing sponsor hearings. All the commentary included in the PSD that related to sponsor hearings was extracted for further analysis. For each hearing, at a minimum, we identified the sponsor company, whether the submission had been considered by the PBAC previously and the therapeutic area.

Each PSD was analysed to determine the topics discussed at the hearing. The information was classified as follows: (1) clinical need, namely the case for the medicine or vaccine to be listed on the PBS or the NIP; (2) disease or disease course (i.e. the disease or condition and its natural history); (3) clinical positioning or use, namely the target patient population for the medicine or vaccine and how it would be used on the PBS or NIP; (4) comparator/s (for the medicine or vaccine); (5) clinical benefit and/or harm, namely the clinical evidence to support the listing of the medicine or vaccine; (6) economic benefit, namely the economic evidence to support the listing of the medicine or vaccine; and (7) other, namely any other issue that the sponsor or PBAC considered to be relevant.

Sponsor hearings were categorised by therapeutic area according to a prespecified classification system used by the MAESTrO database (https://maestrodatabase.com, accessed 23 October 2017). More than one topic could be discussed at each hearing.

Each PSD was also examined to determine the 'usefulness' of a sponsor hearing from the PBAC's perspective according to descriptions used in the PSD as follows: informative or helpful, moderately informative; not informative or unhelpful; no comment. In addition, for each submission at which a hearing was conducted, the PBAC's decision was classified as either a recommendation, rejection or deferral.

Simple descriptive statistics were used to describe and summarise the extracted data. The data were analysed in Microsoft (Bellevue, WA, USA) Excel 2016.

Results

During the study period, there were 472 PSDs available with an average of 23 major submissions considered at each PBAC meeting. Seventy-four sponsor hearings were conducted during the study period (Table 1). At each PBAC meeting, the percentage of sponsor hearings as a percentage of major submissions varied, ranging from 10% at the July 2014 PBAC meeting to 47% at the November 2016 PBAC meeting.

Across the 74 sponsor hearings analysed, 53 different medicines or vaccines were discussed. More than one sponsor hearing for the same medicine or vaccine was conducted on seven occasions. On several occasions, the same medicine or vaccine was considered at different PBAC meetings. As indicated in Table 2, during the study period most of those companies who sought a hearing presented at one or two hearings; 70% of sponsor hearings were taken up by the sponsor on the first occasion that particular medication or vaccine was considered by the PBAC, whereas 30% of sponsor hearings were

Table 1. Number of sponsor hearings by Pharmaceutical Benefits Advisory Committee (PBAC) meeting date (March 2014–November 2016)
Note, a major submission to the PBAC is an application relating to a new medicine or vaccine or an indication requiring an economic evaluation. PSD, public summary document

	Mar. 2014	July 2014	Nov. 2014	Mar. 2015	July 2015	Nov. 2015	Mar. 2016	July 2016	Nov. 2016	Total	Mean per PBAC meeting
No. PSDs	37	48	52	54	50	61	66	46	58	472	51
No. major submissions	32	29	27	39	28	31	31	20	30	267	23
No. sponsor hearings	7	3	7	11	6	6	14	6	14	74	8
% sponsor hearings/major submissions	22	10	26	28	21	19	45	30	47	28	28

Table 2. Number of sponsor hearings by company

No. hearings per company	No. companies
1	14
2	12
3	4
4	2
5	2
6	1

Table 3. Classification of presenter (external clinician or company employee)

Presenter	No. sponsor hearings (%)
External clinician	58[A] (78)
Company employee	16 (22)

[A]At one of the sponsor hearings the clinician was not present, but a letter from a clinician was tabled.

Table 4. Presentation topics

Presentation topic	No. sponsor hearings[A] (%)
Clinical need	20 (27)
Disease or disease course	29 (39)
Clinical position or use	44 (59)
Comparator/s	4 (5)
Clinical benefit or harm	32 (43)
Economic benefit	4 (5)
Other[B]	5 (7)

[A]Note, sponsor hearings could be classified as presenting on more than one topic.
[B]Sponsors spoke about the use of the rule of rescue, proposed restrictions and proposed risk-sharing agreement.

Table 5. Usefulness of sponsor hearings as assessed by the Pharmaceutical Benefits Advisory Committee (PBAC)

PBAC comment	No. sponsor hearings (%)
Informative	31 (42)
Moderately informative	2 (3)
Uninformative	13 (18)
No comment	28 (38)

Table 6. Pharmaceutical Benefits Advisory Committee (PBAC) outcomes for PBAC submissions where a sponsor hearing was conducted

PBAC outcome	Number (%)
Recommendation	28 (38)
Rejection	34 (46)
Deferral	12 (16)

first taken up by sponsors after their application had already been considered at least once in a previous PBAC meeting.

Over the course of the study, 35 companies were able to present at a PBAC meeting. Although most companies presented on one or two occasions, one company was reported to have presented six times (Table 2).

The 74 hearings spanned 13 different therapeutic areas, the most common being oncology ($n = 20$) and immunology ($n = 15$). A clinician external to the sponsor presented at most hearings (78%) (Table 3) and, accordingly, the main topics presented related to clinical positioning or use and clinical benefit or use (Table 4).

The results from the assessment of whether the PBAC considered a sponsor hearing to be useful are presented in Table 5. The PBAC stated that 33 of the 74 sponsor hearings (45%) were informative or moderately informative and that 13 sponsor hearings (18%) were not informative. The PBAC did not comment on 28 occasions (38%).

For each submission where a hearing was conducted, we assessed the decision made by the PBAC. As indicated in Table 6, there was a PBAC recommendation for 28 submissions (38%), but, as shown in Fig. 1, no clear relationship between an informative hearing and a favourable PBAC outcome could be discerned from this study.

Discussion

To the best of our knowledge, the present study is the first to analyse and examine the role of sponsor hearings as part of the PBAC submission process. Advice provided by the DoH states that the intention of sponsor hearings is to provide applicants with an opportunity to verbally clarify, explain and reanalyse information provided in a PBAC submission.[3] Throughout the evaluation process, applicants have another two opportunities to provide input, but these are in writing and do not directly involve other parties or stakeholders (i.e. clinicians). Hence, sponsor hearings provide the main opportunity for face-to-face engagement with the decision maker. This is highly valued by sponsor companies.

Our analysis shows that the extent to which sponsors present at a PBAC hearing is highly variable but, overall, sponsors have

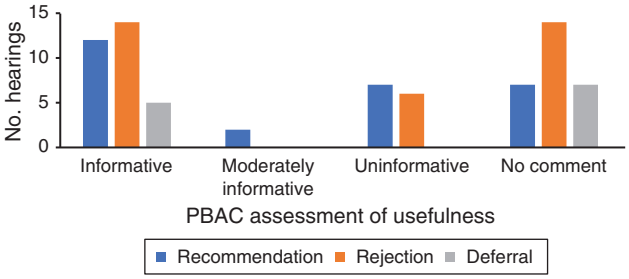

Fig. 1. Pharmaceutical Benefits Advisory Committee (PBAC) outcome according to the PBAC's assessment of the usefulness of the hearing.

chosen not to proceed with a hearing and, on average, only one-third of major submissions considered by the PBAC included a sponsor hearing. This is to be expected because the intention of a hearing is to resolve matters that cannot be resolved using the written opportunities. It is likely that sponsors carefully consider the relevance and value of a PBAC hearing.

Where sponsor hearings were conducted, clinical matters such as clinical need and place in therapy were the main topics discussed, so presenters at hearings tended to include external clinicians (78% of hearings). No details regarding the speciality, research interests or other identifying information for external clinicians presenting on behalf of sponsors were reported in the PSDs. However, we assume that these are generally clinical experts with an interest in the clinical area or treatment under consideration. Of note is that although the PBAC membership includes clinicians across a range of specialties, the areas of clinical expertise represented by members of the committee are not exhaustive.

We found that discussion on other topics such as the choice of the main comparator, the economic benefits of the medicine or vaccine or its likely budget impact occurred less frequently. This may be because sponsors considered that these issues could be adequately resolved through written responses or that sponsors did not believe that addressing these issues at a hearing would be helpful.

Most hearings were conducted for a first-time application. Although permissible, a second sponsor hearing for a resubmission occurred less frequently.

The PBAC considered approximately 45% of sponsor hearings to be informative or moderately informative, whereas 18% were classed as uninformative. The usefulness of sponsor hearings as assessed by the PBAC was underreported in the PSDs, with the PBAC not providing any comment on 38% of occasions. Of those sponsor hearings where the PBAC commented on their usefulness, the PBAC considered approximately 70% of sponsor hearings to be at least moderately informative.

No clear relationship between the PBAC outcome and participation at a hearing could be discerned from this study, with 54% of applications where a hearing was conducted resulting in a recommendation or deferral, compared with 46% resulting in a rejection. It is possible that where a sponsor decides to present at a hearing they are more inclined to do so for those submissions that are more complex or where the outcome is more uncertain. Given this, the results may suggest that the success rate in submissions with hearings is reasonably good.

There are several practical considerations associated with presentation at a sponsor hearing. The PBAC meetings are held three times a year and conducted over 3 days. Sponsors are not notified of the exact date and time of their hearing until the week before the PBAC meeting. Limitations imposed upon the hearing schedule mean that it may be challenging for sponsors to secure particular expert clinicians for the PBAC presentation.

Other countries with health technology assessment (HTA) processes similar to Australia include Canada (Canadian Agency for Drugs and Technologies in Health) and England (National Institute for Health and Care Excellence (NICE)).

In Canada, there are two distinct processes for HTA: (1) the Common Drug Review (CDR), which conducts HTA assessments for non-oncology medicines; and (2) the Pan-Canadian Oncology Drug Review (pCODR), which considers cancer therapies. The CDR involves a closed-door deliberation by the Canadian Drug Expert Committee (CDEC), such that the sponsor is not allowed at the meeting and has no opportunity to meet with CDR staff, external reviewers or the CDEC at any point in the process. The pCODR process is somewhat more transparent and, although the sponsor is not allowed at the meeting, a sponsor can meet with pCODR staff and external reviewers at specific checkpoints.[4]

NICE is the most transparent HTA agency in that sponsor representation is mandatory at the meeting and decision makers have direct engagement at the point of deliberation.[5] Throughout the evaluation process there are several opportunities for sponsors to have early and continued direct engagement and consultation with the decision-making committee.

In Australia, the primary opportunity for sponsors to directly address the committee is at a hearing on the day of the PBAC meeting. Additional face-to-face engagement is available to sponsors following a PBAC rejection or deferral, where companies can attend a post-PBAC meeting with the Chair of the PBAC to seek advice on matters that may inform a resubmission.[1] Increased direct engagement between the PBAC and sponsors, more in line with the approach adopted by NICE, may be a possible area of improvement of the existing process.

Our analysis has several limitations. First, we only had data on sponsor hearings since the March 2014 PBAC meeting. Prior to then, details of sponsor hearings were not routinely included in the PSDs.

Second, we were constrained by the lack of detail and standardised reporting in relation to hearings included in the PSDs. Some PSDs provided considerable detail, whereas for others the level of information was minimal. Although the information included in the PSDs is published in a relatively consistent and semistructured manner, there were some data gaps. For example, the PBAC did not comment on the usefulness of sponsor hearings on 38% of occasions. Based on the information provided in the PSDs, it was often not clear what aspects of a hearing the PBAC considered informative. We included in our analysis an assessment of the topics discussed at the hearing, but the way this information was reported in the PSDs was not standardised, thereby requiring a level of interpretation by the authors.

Third, our analysis was limited to an evaluation of the information provided in the PSDs. We did not obtain the views of the sponsors who presented at a hearing as to usefulness

from their perspective. We also did not ascertain from the sponsors the factors influencing their decision to request and proceed with a hearing. None of this information was included in the PSDs.

Sponsor hearings are an important and integral component of the reimbursement process in Australia and, because they provide the main opportunity for sponsors to engage directly with the PBAC, they are highly valued by sponsor companies. Although not assessed directly, we believe that, based on our own experience and the results of our analysis, sponsors value the opportunity for face-to-face engagement at hearings. Sponsors exercise prudence in considering whether a sponsor hearing is going to be helpful to address outstanding issues and help PBAC decision making.

Although sponsor hearings have been available for more than a decade, there has been little formal evaluation. The relatively recent inclusion of information relating to sponsor hearings has been a welcome addition to the information provided in the PSDs. Our analysis has been limited by a lack of standardised reporting of information in the PSDs, so one of our key recommendations is to ensure that the information reported in the PSDs is complete, standardised and consistent.

PBAC members or sponsor companies were not interviewed as part of this analysis. It would be useful to extend this research to a broader range of stakeholders, including sponsor companies, the PBAC, DoH and Medicines Australia, to identify additional areas for improvement in the current process and work towards standardisation and optimisation of the information provided in PSDs.

Competing interests

MF and SL are employees of Amgen Australia Pty Ltd. MW has no competing interests.

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