

## **Stakeholder perceptions study**

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# **Cancer Drugs Alliance**

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**GAR Reference:** GAR1408003

**ABN:** 22 003 287 643

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## **Note to the Reader**

*In preparing this report we have presented and interpreted information that we believe to be relevant for completing the agreed task in a professional manner. Where we have made assumptions as a part of interpreting the data incorporated in this report, we have sought to make those assumptions clear. Similarly, we have sought to make clear where we are expressing our professional opinion rather than reporting findings. Please ensure that you take these assumptions into account when using our report as the basis for your decision-making. We are more than happy to discuss the analysis and recommendations with you.*

*This project was conducted in compliance with AS: ISO20252:2012 guidelines.*

# 1. Executive Summary

The Cancer Drugs Alliance (CDA) Stakeholder Forum held in March 2014 highlighted the need for stronger involvement of individual patients and consumer organisations in the Pharmaceutical Benefits Advisory Committee (PBAC) decision-making process, to ensure that consumer needs are both understood and foremost in the minds of those determining what treatments should be funded.

In order to ensure the important issues and themes raised in the Forum are used to develop practical improvements to PBAC processes, the CDA commissioned GA Research to conduct research with patient organisations to capture the perceptions, attitudes and sentiment towards issues around access to cancer drugs and treatments. The findings will be used to make the case for increased public funding for cancer patients and cancer drugs.

A confidential online survey was used to capture the perceptions and opinions of the target audience. A total of 18 representatives from different consumer organisations took part in the survey. This report outlines the key themes that emerged from the survey showing both the quantitative results from rating scale questions as well as de-identified verbatim responses for the open-ended sections. The Appendix contains a copy of the questionnaire used in the survey.

## Key findings summary

The findings from the survey showed that consumer organisations have a strong need for significant changes to the way the PBAC operates, makes decisions and involves consumers in the decision-making process.

In summary, consumer organisations call for:

- **Less focus on economics and more on individuals' quality of life**

A constant theme throughout the survey responses was the need for the PBAC to shift its focus away from economics and the cost-effectiveness of drugs and instead base assessments primarily on the impacts that medicines can have on improving a consumer's quality of life. While many respondents acknowledged the importance of cost-effectiveness in the assessment process, it was argued that this didn't have to impede considerations of livelihood, wellbeing and additional economic benefits that come with a consumer returning to the workforce and contributing to society.

In direct relation to this, respondents called for greater focus on consumers with rare diseases, citing the unfairness of the PBAC in basing assessments on market incentives and consequently creating inequitable access to medicines for consumers with rare diseases.

### ■ **More visibility and transparency**

The PBAC is by and large seen to operate beneath a 'cloak' of obscurity and complexity, where consumers and consumer organisations feel at a loss as to how to infiltrate and make themselves be heard. This perception has resulted from a number of Departmental policies and procedures that are thought to be outdated, rigid and impenetrable.

There are a broad range of consequences that stem from this, namely that consumer representatives are feeling disenfranchised and deterred from making submissions, as well as feeling an overall sense of disempowerment.

Survey respondents made repeated calls for the PBAC to be more inclusive and transparent in the way they operate. Key suggestions made included:

- Greater communication with consumers and consumer groups (including through social media).
- Improved guidance on how to make submissions, how submissions are used and how decisions are made

### ■ **Improvements to the PBAC submission process**

Respondents made repeated calls for improvements to the way the PBAC submission process operates. A key issue with the submission process is that it is felt to be a 'limiting' platform from which to present a case to the PBAC due to timing restrictions and complicated procedures to follow. It was also said that following a submission there is no acknowledgement of receipt or indication as to the outcome of their submission in the PBAC meeting. As a result, several respondents felt this was a 'tokenistic', 'tick box' process.

Suggested improvements to submission process included:

- Detailed follow up advice on submission outcomes
- Using different evaluation criteria for different cancers and diseases

### ■ **Greater consumer involvement in the PBAC assessment process**

A central argument made throughout the survey was the need for more meaningful engagement with consumer groups by the PBAC. Consumers and their representative bodies feel significantly underrepresented and detached from the PBAC assessment process, despite being the actual end-users of the medicines and being best placed to make judgements on the impacts of medicines.

Suggestions made to strengthen the impact and influence of the consumer voice included:

- Having at least one another consumer representative on the PBAC, preferably with a cancer background
- Establishing a consumer sub-committee the PBAC can call on for information regarding specific conditions

- Foster greater collaboration between consumer groups and pharmaceutical companies

- **Greater efficiency and updated processes**

There was a significant amount of reference to the fact that the Australian PBAC system is too rigid, slow to react and out-dated in comparison to other comparable processes overseas (e.g. the UK). It was argued that the system needs to keep pace with rapid medical advancements and efficiencies adopted in other countries. Key suggestions to improve efficiency included:

- More frequent revisions to PBAC system and processes to accommodate medical advancements
- Meetings to be held more frequently to fast-track decisions
- Establish a national data collection on treatment outcomes

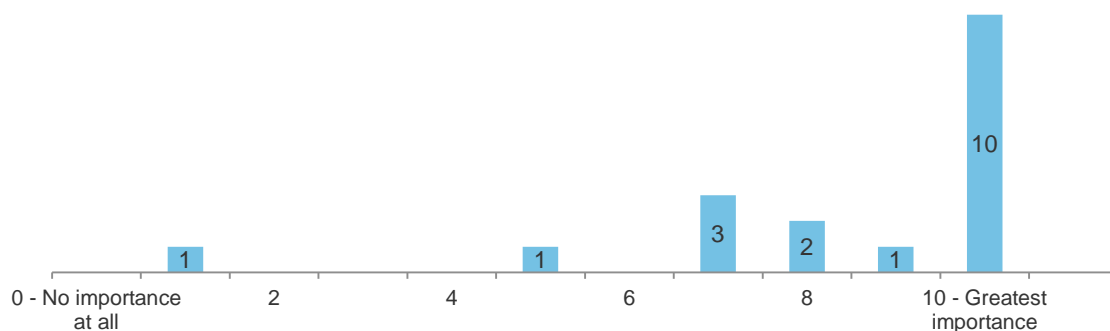
## 2. Research findings

### Voice of the consumer

#### Importance of health consumer input

Respondents were first asked to rate out of ten how important they believe input from health consumers should be in the PBAC approval process. The majority of respondents (n= 16, or 88%) rated the level of importance as 7 or more, with over half (n=10) rating consumer input as having “greatest importance” in the PBAC approval. Only one respondent believed health consumer input had little importance to the PBAC approval process.

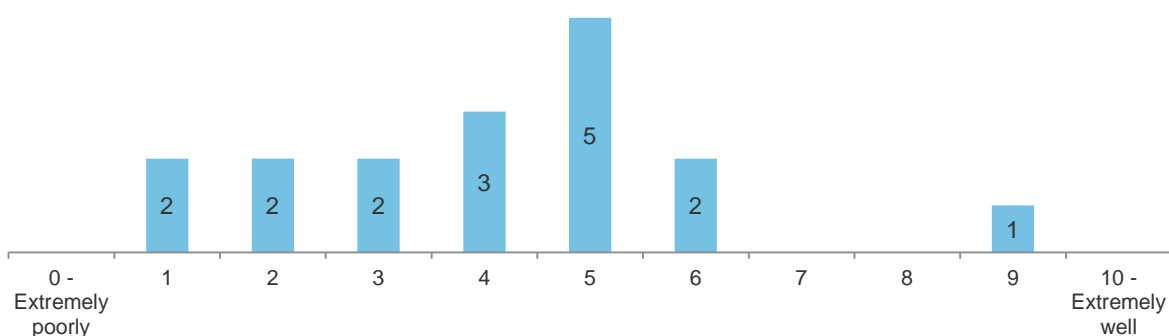
*Q: In your opinion, how important should input from health consumers be in the PBAC approval process for the recommendation of new cancer medicines? (n=18)*



#### Current representation of consumer voice

When asked to rate how well they think the voice of the health consumer is currently represented in the PBAC approval process for the recommendation of new cancer medicines, half of the respondents (n=9) gave it a rating of four or less. Over a quarter (n=5) were on the fence on this issue, giving the current representation of the consumer voice a 5 out of 10. One respondent believed the consumer voice is well represented.

*Q: How well do you think the voice of the health consumer is currently represented in the PBAC approval process for the recommendation of new cancer medicines. (n=18)*



## Voice of the consumer: challenges explained

When asked to explain the reasons for their responses, respondents cited a range of negative issues and concerns with the PBAC.

### ■ Minimal consumer representation on the PBAC

A key issue for many respondents was that consumers are significantly underrepresented in the PBAC decision-making process with just one consumer representative on the committee when there should be at least two, including at least one who comes from a cancer background. Specifically respondents pointed to the comparisons between the composition of equivalent overseas pharmaceutical benefits bodies which they saw as preferable:

*“In Australia there is only one consumer representative in the total PBAC membership of 17. In comparable bodies to the PBAC in UK and Canada there are three times as many consumer representatives. It is not possible for 1 consumer representative to adequately advocate the wide-ranging interests of all consumers”*

It was also said there needs to be consumer representation for different types of drugs and conditions, not just a general consumer representative - as occurs with other health professionals.

*“We believe that having a greater pool of trained consumer advocates to review submissions from across various disease types (depending on the drug up for review) would help in making sure that the best decision is made.”*

### ■ Clinical and economic impact more important than consumer impacts

There is a strong perception that the PBAC places greater value on the opinion of physicians rather than those of consumers and carers in making their decisions about which drugs to approve. Several respondents stressed the value that consumer input can have on the decision-making process by analysing and assessing cancer drugs holistically and in terms of ‘real world’ benefits, as opposed to mere clinical and economic outcomes.

*“Physician rated impact isn't given the same values as patient or carer rated impact”*

*“There is scope for significantly increasing and making more meaningful the engagement of consumers in the PBAC approval process. Consumers can provide unique and relevant perspectives to help measure the benefits of new medicines, rather than analysis of clinical outcomes and economic considerations alone. This includes a better analysis of quality of life, and patient-reported outcomes.”*

*“Consumers are people, not just vessels containing disease, living lives in isolation. They are people living lives and interacting with many others, especially immediate family and friends. It would be more meaningful for consumers to be able to rate the impact of their diagnosis on a range of issues that impact their quality of life including*

*social relationships, economic impact - current and future, as well as impact on enjoyment of life.”*

Respondents broadly pointed to a prioritisation of cost-effectiveness at the expense of input from consumer end-users:

*“It is very clear that the overriding consideration is cost-effectiveness and everything else including consumer concerns run a very distant second.”*

#### ■ **Unclear communication channels and input sought at random**

Although PBAC correspondence with consumers and ‘relevant persons’ (i.e. those making a submission) is required to be orderly, timely and transparent (under Section 99YBC of the Act), several respondents felt uncertain about how to communicate with the PBAC other than through the submission process, and that the PBAC’s approach to seeking input is “haphazard” and “opaque”.

For instance, some respondents felt that input is only sought “from time to time”, which makes it difficult for consumers and consumer groups to collate their responses in time. It was also said that the PBAC does not provide feedback following a submission, despite the requirement to do so:

*“No feedback is received from PBAC on submissions received, or even on the outcomes of the meeting. I have no idea whether our submissions are read and what level of importance is placed on them by PBAC members.”*

#### ■ **Restricted timeframe for input**

Another key issue respondents had with the PBAC was the limited time frame for input. The existing practice of allowing a two-week window for consumer participation is insufficient time to disseminate timings and promote the window adequately for full participation from consumers.

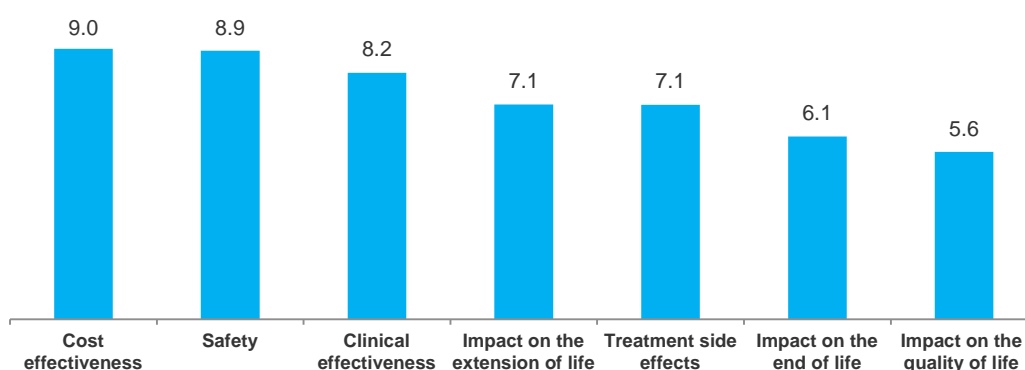


## Current priorities for PBAC decision making

Respondents were asked to consider the level of priority the PBAC places on various matters, ranging from impacts to consumers, economic and clinical effectiveness, and rate these priorities on a scale out of ten. The average ranking (mean) for each is presented in the chart below.

The results show that the vast majority of respondents believe that the PBAC currently places greatest priority on cost effectiveness, followed by safety and clinical effectiveness. Impact on the quality of life is seen to be a low priority for the PBAC.

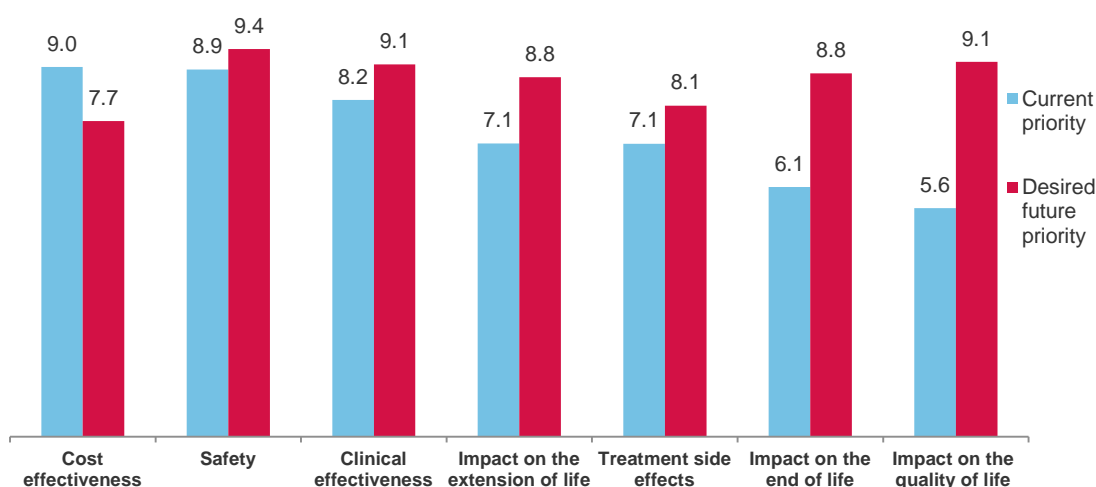
*Q: When making decisions around recommendations for new cancer medicines, what level of priority do you think the PBAC currently places on the following? (n=16)*



## Future priorities for PBAC decision making

Respondents were asked to consider the level of priority they *would like* the PBAC to place on the same issues. The results are presented below, with comparisons drawn between existing priority and desired future priority.

*Q: What level of priority would you like to see the PBAC place on the following in the future? Use the same scale of 0 to 10 where 0 is the lowest priority and 10 is the highest priority. (n=16)*



The results show discrepancies between the perceived existing priorities of the PBAC and the priorities respondents would like the PBAC to have in the future.

Most notably there appears to be a significant disconnect between the priority level the PBAC is currently placing on quality of life versus the level that consumers and consumer groups would like to see.

A noteworthy finding is also the high level of priority the PBAC is perceived to be placing on cost-effectiveness when on average respondents believe this should be given the least priority out of all the factors. According to the results, greater priority should instead be given to safety, clinical effectiveness and impact on the end of life.

### **Future priorities explained**

When asked to further elaborate on their responses, respondents put forward the following suggestions:

- **Patient impact and quality of life should take priority over cost-effectiveness**

*“Cost effectiveness has to be a consideration but the most importance should be placed on the extension of time with the loved ones of the patient and the treatment side effects, i.e. enhanced quality of life.”*

*“Cancer is unique in that it is many diseases manifesting uniquely in any given patient. We are not looking at a cure but treatments that will manage the disease and improve quality and quantity of life.*

*“Cost effectiveness is important but the current methods for assessing it are inadequate. For example, if a drug is effective in getting a patient back to work but doesn't extend life beyond one which doesn't then the economic contribution of the first drug is not considered.”*

- **PBAC too rigid and slow to react**

*“The PBAC is very rigid in its role and function. Like any government organisation it is slow to react to current changes. Legislation changes are slow if at all, decisions are slow, no real KPI's upon which to base their work.”*

- **Less priority on side effects**

*“I think treatment side effects should not be a particularly high priority. Patients should be able to make decisions about what side effects they are prepared to tolerate.”*

- **Assessments to be made on a case by case basis**

*“It really depends on the therapeutic indication and prognosis. If the condition is not curative, then QOL and impact on end-of-life become all important. If the indication is acute, but curable, then clinical effectiveness and safety are paramount. The clinical effectiveness must always be greater than the sum of the side effects etc.”*

### ■ Greater consideration given to overseas studies

*“Two submissions for an important hormone to be listed have now failed. Australia is the only western country where this hormone is not subsidised. My understanding is that cost efficacy played a role as it is an orphan drug. If the clinical studies were considered biased in Australia why was the overseas literature not taken seriously?”*

### ■ Introduce more medicines for early stage cancer to the PBS

*“The other thing that worries me greatly, is the fact that some drugs are on the PBS for stage iv cancers only. For example Abraxane for Pancreatic cancer. Is this not a case of shutting the stable door after the horse has bolted? If it was on the PBS for stage ii, there might be a greater chance of patient survival.”*

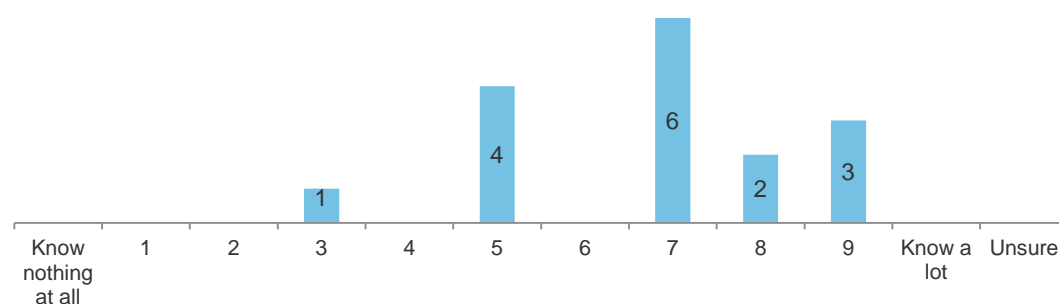
## Knowledge and experience with PBAC submissions

Respondents were asked to rate their level of knowledge of the PBAC submission process, where they sourced information from and their experiences in making a submission.

### Knowledge of the PBAC submission process

On average, respondents rated their knowledge of the PBAC submission process **6.8 out of 10**, suggesting that although respondents have some knowledge of the process, substantial knowledge gaps still exist.

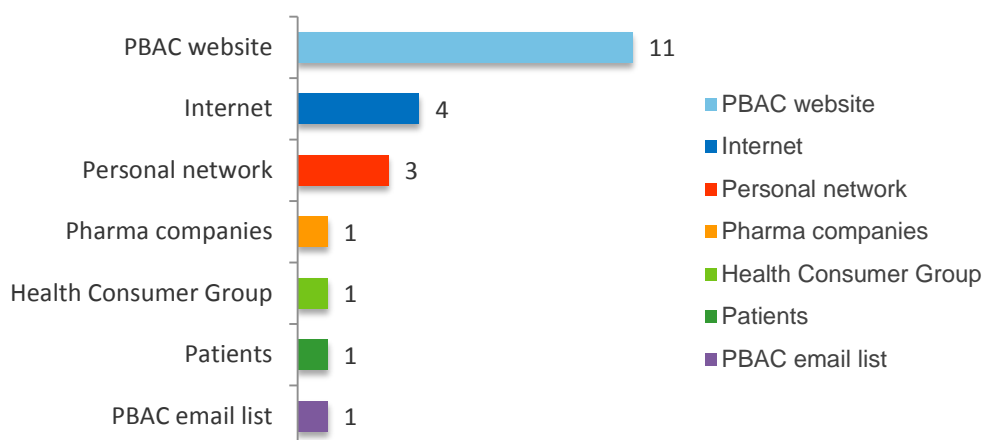
*Q: How much do you know about the PBAC submission process? Using the 0 to 10 scale below where 0 means you 'know nothing at all' and 10 means you 'know a lot'. (n=16)*



When asked where they source information about the PBAC submission process, the most common answers were:

- PBAC website (11 mentions)
- Internet (4 mentions)
- Personal network (3 mentions)

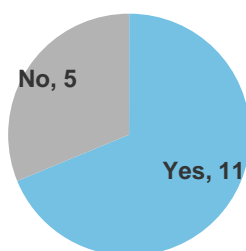
Q: If you need information about the PBAC process, where do you currently go to find it? (n=16)



## Experience in making a submission to the PBAC

The majority of respondents (n=11) have made a submission to the PBAC in the past.

Q: Have you made a submission to the PBAC? (n=16)



When asked to describe the experience, the majority of respondents shared negative feedback on the process. The key issues raised with the submission process included:

### ■ Limited platform to voice representation

Several respondents felt discouraged by the limiting structure of the submission form, saying that it does not allow them to adequately represent their case.

*“It did not give us the opportunity to fully represent the case of our consumers due to the process they have in place e.g. form”*

*“I found the ‘Code of Conduct’ limitations with consumer group and pharmaceutical companies liaison frustrating.”*

*“Web portal - the existing on-line format tends to constrict submitters to the PBAC’s chosen parameters.”*

### ■ A 'tick box' exercise

Some respondents also said they felt the process was tokenistic and simply a perfunctory gesture towards consumers and consumer groups.

*"A PBAC tick box exercise."*

*"An impression was conveyed that it was a 'nominal' process"*

*"Bland.....we write short submissions and email them off in the hope that someone somewhere will read them. We know however, that it is token and the real decision criteria are cost effectiveness based."*

*"Ineffective and disempowering"*

### ■ Tedious and overly complex

*"It is a time consuming but necessary process. Concentration on economies of scale is disheartening when dealing with a less common cancer, and this seems to be the biggest road block"*

### ■ Lack of support from PBAC through the submission process

*"The lack of information from the PBAC to HC groups means we are rushing our submissions. There are no clearly defined measurables from the PBAC to assist the HC with their submission."*

### ■ No acknowledgement of receipt:

*"It's awful, no acknowledgement of receipt, no follow up, nothing re usefulness of information etc.. A black hole!!"*

*"Being a peak consumer body for this hormone, and after putting together a powerful consumer submission, we were not acknowledged by the PBAC for our effort."*

Those who did not make a submission to the PBAC in the past were asked to provide their reasons for not having done so. These included:

- Consumer groups cannot make submissions, only pharmaceutical companies can
- Charities have no need to make submissions
- Unsure how to go about it.

## Improvements to the PBAC submission process

Respondents were presented with a range of potential 'improvements' to the PBAC submission process, and were asked to indicate the extent to which they felt these were ideas worth pursuing.

Q: Below are some ideas others have mentioned in regard to improving the **PBAC submission process** for individuals and cancer consumer organisations. Rate each out of 10, where 0 means you think it is not an idea worth pursuing and 10 means it is an idea definitely worth pursuing. (n=18)

	Improvements to the submission process	Average rating (out of 10)
1	Individuals and consumer organisations are given the opportunity to participate in training about the PBAC submission process	9.3
2	Provide guidelines on the evaluation criteria PBAC use for each submission	9.2
3	The option of providing a Patient Impact Statement and the opportunity to present in person	8.8
4	Notification by the PBAC to consumer organisations of drugs currently being evaluated and a call for submissions	8.8
5	Provide guidelines on how and when to make a submission	8.5
6	PBAC personnel to receive training about the involvement of individuals and consumer organisations in the submission process	8.5
7	Increase the timeframe for consumer input to beyond 6 weeks	8.2
8	Provide a PBAC staff member whose role is to support consumers in their submission process	8.1
9	Provide a web portal access for submissions	8.0

## Further suggested improvements to the PBAC submission process

When asked to elaborate further, respondents raised the following improvements they would like to see in the PBAC submission process:

### ■ Greater consumer involvement

It was suggested that the PBAC should first and foremost accept and acknowledge the integral input of the consumer voice, recognising that consumers can and should have a valid and insightful impact on in PBAC decision-making:

*"I believe that the first thing the PBAC should do is acknowledge that consumers are integral in the process, not an afterthought. We are not just cancer patients, we have knowledge, many are well educated (some better than the members) and we can provide balance, judgement, insight and advice...."*

Another commonly mentioned recommendation was to increase the number of consumer representatives or the amount of consumer input in the PBAC assessment process, particularly those with a cancer background:

*“An improvement would be to giving weighting or defined value to patient rating feedback/submissions - e.g. that consumer input contributed to 1/4 of total evaluation score in assessment process, in addition to 'scores' for drug efficacy, safety and cost effectiveness (the other 3/4 of score)”*

*“I think PBAC needs to have a pool of trained consumers representing different disease areas who can help with the assessment of applications. I think there should be a cancer rep to help assess new cancer drugs.”*

In relation to this, some respondents also said they wished the submission process could be more inclusive and provide equal opportunity to all groups to make a submission. Several respondents said the whole process made them feel segregated, disenfranchised and as a result, disempowered:

*“I felt totally segregated at times. Any assistance from a professional body would have been greatly appreciated.”*

*“For rare diseases relevant consumer groups don't have the opportunity to make PBAC submissions for funding for therapies which are approved overseas where the responsible pharmas choose not to make PBAC submissions for commercial reasons. Patients with rare diseases are unfairly discriminated against by the PBAC processes.”*

*“Ineffective and disempowering”*

#### ■ **Less opaqueness, more transparency**

Another central issue with the current PBAC submission process is a perceived lack of transparency in the way submissions are used and decisions are made:

*“The process needs to change and become more transparent. The PBAC needs to be prepared to discuss and explain decisions. At present, the process is deliberately opaque to the consumer in both lack of information and the way the information is expressed. Transparency is important as ultimately the public are both the payers and the recipients of the treatments.”*

*“While the PBAC process occurs, patients are in the dark about what is happening.”*

It was suggested that a remedy for some of this opaqueness would be more guidance and education in how the submission process works:

*“Most people have no understanding of PBAC so ongoing public education, consumer organisation training and education program are needed and long overdue and would be very valuable to improve understanding and quality of consumer submissions.”*

Lack of communication between the PBAC and consumers was a repeatedly raised issue amongst the respondents. It was suggested that a way to improve this would be to provide more resources and advice to consumer representatives to foster two-way communication:

*“PBAC should provide more resources to consumer rep to facilitate 2 way communication between consumers, consumer organisations and PBAC.”*

#### ■ **Greater efficiency, faster decisions**

Several respondents suggested the turnaround time for the approval of medicines needs to be faster, with meetings held more frequently:

*“We would also like to see more meetings, more rapid turnaround, more disclosure. Meetings are few, decisions take a long time”*

#### ■ **Greater access to subsidies for cancer medicines**

One respondent suggested there be a revision with the way subsidised are accessed by consumers, stating that at present there are too many obstacles in place preventing consumers to obtain subsidies to pay for their medicines, and are paying for their own medicines as a result:

*“Patients are paying for their own medicines or relying on the discretion of the pharma/biotech company to subsidise access. It seems to be more difficult to obtain subsidy than it is to obtain market approval in the first place. This is a disincentive to innovator companies and a hardship to patients. We know of several people who are paying mortgage-sized amounts, without pharma compassionate subsidy, or with very little - this is distressing. It is also **inequitable**. It would be **valuable to explore access and subsidies for such drugs** in these cases.”*



## Improvements to the evaluation process

When presented with a range of potential ‘improvements’ to the PBAC evaluation process, respondents ranked these in the following order of worth being pursued.

*Q: Below are some ideas others have mentioned to ensure individuals and cancer consumer organisations have a stronger voice in the **PBAC evaluation process**. Rate each out of 10, where 0 means you think it is not an idea worth pursuing and 10 means it is an idea definitely worth pursuing*

	Improvements to the evaluation process	Average rating (out of 10)
1	Have a consumer sub-committee the PBAC can call on for information regarding specific conditions	9.5
2	Provide feedback to individuals and consumer organisations about the effectiveness and impact of their submissions on the final approval decision.	9.5
3	A regular review of PBAC consumer engagement by the Department of Health and consumer organisations	9.4
4	Set up a consumer liaison in the Department of Health	8.7
5	Have more consumers on the PBAC	8.6
6	Host public hearings for individuals and consumer organisations	8.3

## Further suggested improvements to the PBAC evaluation process

When asked to elaborate further, respondents raised the following improvements they would like to see in the PBAC evaluation process:

### ■ Change the rules

A key suggestion was that to make improvements to the PBAC evaluation process, change needs to come from the top. A number of respondents called for revisions to the Act and engaging with the Minister directly, in light of the challenges faced with collaborating with the Department.

*“All of those suggestions are great but we need to remember that the **rules will also need to change** to ensure the PBAC can take into account our views as consumers.”*

*“It appears there is a restriction on disclosure of decision making reasons based on legislative constraints due to disclosure of commercial confidential information. **The Act needs to be reviewed and changed** to redress this imbalance.”*

*“The real issue is developing a relationship with the Department - they are wary of consumers, there is little trust and little respect for us. The pendulum needs to swing the other way. They are there to give advice to the Minister - we can more easily get to see the Minister than the Department - so it must come from the top. DOH needs to realise*

*we can give them good advice and insight into policy for cancer consumers that would make their job much easier.”*

■ **More transparency and meaningful engagement with consumers**

Once again, lack of transparency and lack of communication was raised as a key problem within the PBAC evaluation process.

*“We believe that meaningful consumer engagement is important to improving PBAC processes and evaluation”*

*“Current PBAC processes lack transparency on evaluation decisions. There is a real communications problem as only 20% of submissions are successful.”*

One respondent had a more pessimistic view of the potential for the PBAC to engage meaningfully with consumers, and suggested an entirely independent body would be required to collaborate with consumer groups:

*“I think the PBAC have blinkers on and it would take a totally independent person/body to the PBAC to work with consumer groups.”*

## The most important consideration

After having thought about the various ways individuals and consumer organisations can contribute to the decision-making process that determines what treatments should be funded, the last question in the survey invited respondents to share the *one thing more than anything else* they think should be considered in the PBAC system.

Q: *Having thought about the various ways individuals and consumer organisations can contribute to the decision-making process that determines what treatments should be funded, is there one thing more than anything else you think should be considered in the current Australian PBAC system?*

The following considerations were put forward as being most important:

### STRENGTHEN THE CONSUMER VOICE

✓ **Include a consumer representative on the PBAC with cancer background**

*“The inclusion of a consumer representative who comes from the cancer environment.”*

*“Having a range of consumers involved in the process who have specific expertise and experience associated with the condition being considered.”*

*“PBAC needs a panel of consumer reps from different disease areas who can provide considered responses to applications for new drugs in their area of expertise.”*

✓ **Establish consumer sub-committees**

*“Consumer sub-committees for major diseases, like cancer, could be an informed conduit between the cancer consumer groups, PBAC and Dept of Health. It could also advise on best processes etc for consumer impact input, how to advise the consumer members/s of PBAC. Perhaps we recommend that we start this for cancer as a pilot.”*

✓ **Enable greater collaboration between consumer groups and pharmaceutical companies**

*“With the current application fee it would only be Pharmaceutical companies who would have the funds to do a submission. The Code of Conduct limits our participation with them, and what consumer groups have to contribute could enhance their submission.”*

### GREATER FOCUS ON PATIENT IMPACT

✓ **Personal impact**

*“A greater emphasis on patient impact either in person, by a liaison person in the department of health or a greater pool of consumers on which to call on to participate in the decision making process.”*

✓ **Hope and fairness: it is everyone's right to equitable care**

*"Every Australian contributes to the community during their lifetime. When they are stricken with cancer they have a right to expect that their community will care for them, that their doctor will be able to treat them as he/she thinks best. Working out cost effectiveness based on averages and medians ignores both the experience of a clinician in assessing the applicability of a drug and denies a patient reasonable hope that he has the right to have based on his contribution to society."*

*"We live in a community, not an economy, and our funding process should reflect the community's wishes."*

#### UPDATE PBAC PROCESSES TO REFLECT MODERN INNOVATION

✓ **Make legislative changes to the PBAC system**

*"The current PBAC system and processes need to be changed to redress the blatant unfairness to Australians with rare diseases. Australia needs something comparable to the English Cancer Drug Fund so that patients with rare diseases can have access to funding. Currently Australian patients with rare diseases have no access avenue for funding for needed therapies because the current rules preclude applications"*

✓ **Make the PBAC more flexible to keep pace with medical breakthroughs**

*"Overall the PBAC processes need to be reviewed and changed to accommodate the rapidly changing nature of medical treatments such as targeted therapies. **The rigid and inflexible PBAC procedures are outdated** and don't accommodate the new medical treatment breakthroughs. As a result, Australia will fall behind other countries where special breakthrough treatments are recognised and accommodated by fast-track approval procedures."*

#### GREATER SUPPORT FOR RARER DISEASES

✓ **Protect those with rarer diseases, don't just cater to the 'big diseases'**

*"The system caters reasonably well for big diseases and for diseases with organised consumer advocates but tends to neglect rare diseases for which a drug therapy is not a commercially attractive option in Australia's relatively small drug market and diseases/conditions which do not have an organised consumer voice."*

✓ **Use different evaluation criteria**

*"Use of different evaluation criteria for rare indications and indications where prognosis is poor"*

**IMPROVE EFFICIENCY TO CANCER DRUGS ASSESSMENTS****✓ Designate a cancer drugs section of the PBAC**

*“Cancer drugs section of the PBAC. Not because Cancer is more important than any other disease but by segregating this group it could speed up the process for all diseases and allow for cancer drugs to be evaluated by appropriate members of the PBAC.”*

**✓ Speed up access to treatments**

*“Fast track access for treatments that are already proving to be effective at the global level to offset the issues that we are having with a smaller population for rarer cancers and subtypes of some cancers .”*

**✓ Form a shared risk or managed entry/exit schemes**

*“Consideration and formation of shared risk or managed entry/exit schemes to make more therapies available for life threatening cancers when no more treatment options or clinical trials are available.”*

**✓ Develop a mechanism that is sponsor-independent to support therapy**

*“PBAC may need to develop sponsor-independent mechanism to support reimbursement of therapy for some conditions with high medical need but neglect from industry due to lack of market incentive.”*

**IMPROVED COMMUNICATION****✓ Greater PBAC communication via social media platforms**

*“With e-comms and social media, some of these barriers can be addressed - PBAC should be working in social media platforms too”*

**OTHER****✓ Establish a national data collection on treatment outcomes**

*“Having a national data collection on treatment outcomes for PBAC funded therapies would help to review drug value once listed in PBS or made available through a shared risk scheme to justify ongoing public funding of individual therapies.”*

**✓ Stop ‘double blind’ clinical trials**

*“We have to get away from the double blind clinical trial as the real evidence....there are cancer patients who will go on trials to support new treatments, if they don't work then they stop. Crossover must be considered or there is little hope of drugs for small patients ever coming to market.”*

## **APPENDIX A: Questionnaire**

## Introduction

The Cancer Drugs Alliance (CDA) Stakeholder Forum stakeholder forum held in March 2014 highlighted the need for stronger involvement of individual patients and consumer organisations in the Pharmaceutical Benefits Advisory Committee (PBAC) decision-making process, to ensure that consumer needs are both understood and foremost in the minds of those determining what treatments should be funded. To ensure the important issues and themes raised in the Forum are used to develop practical improvements to PBAC processes the CDA has commissioned GA Research to conduct this confidential online survey amongst consumer organisations.

The questionnaire contains a series of rating questions as well as your opportunity to expand upon your responses to fully capture your views. The survey is likely to take up to 30 minutes to complete depending on the range and depth of information you would like to provide. There is an option to exit the survey at any time and come back to it at a more convenient time.

All your responses will be anonymous and confidential – according to the Australian Privacy Principles set out by the Australian Market & Social Research Society and the Privacy legislation.

The findings will then be collated to develop recommendations, that can be presented to Government by the CDA, with the support of all cancer consumer organisations, to seek changes both in the short and long-term – for a better future for Australians affected by cancer.

If you have any questions about the survey please contact Michelle Kirsznner on [mkirsznner@garesearch.com.au](mailto:mkirsznner@garesearch.com.au) or 02 9552 8996.

## Voice of the health consumer

- 01.** In your opinion, how important **should** input from health consumers be in the PBAC approval process for the recommendation of new cancer medicines? Use the 0 to 10 scale below, where 0 means you think it 'should be of no importance at all' and 10 means it 'should be of greatest importance'. If you are not sure type 11
- 02.** How well do you think the voice of the health consumer is **currently represented** in the PBAC approval process for the recommendation of new cancer medicines, on a scale of 0 to 10 where 0 is extremely poorly and 10 is extremely well. If you are not sure type 11.
- 03.** What makes you say that? OPEN ENDED

**Priorities for PBAC decision making**

- 04. What level of priority do you think the PBAC **currently** places on the following during their decision making around recommendations for new cancer medicines. Use a scale of 0 to 10 where 0 is the lowest priority and 10 is the highest priority. If you are not sure type 11. ROTATE OPTIONS
- 05. Using the same scale what level of priority would you like to see the PBAC place on each **in the future**. If you are not sure type 11. ROTATE OPTIONS

	<b>Current PBAC priority</b>	<b>Priority would like to see in the future</b>
Impact on the quality of life		
Impact on the extension of life		
Impact on the end of life		
Cost effectiveness		
Safety		
Clinical effectiveness		
Treatment side effects		

- 06. Please feel free to provide further explanations of your responses. OPEN ENDED

**Knowledge and experience with PBAC submissions**

- 07. How much do you know about the PBAC submission process out of 10, where 0 means you know nothing at all and 10 means you know a lot about the process?
- 08. If you need information about the PBAC process, where do you currently go to find it? OPEN ENDED
- 09. Have you made a submission to the PBAC?
  - a. Yes
  - b. No
- 10. IF YES, how would you describe the experience? OPEN ENDED
- 11. IF NOT, what has prevented you from doing so? OPEN ENDED

**Improvements to the submission process**

- 12. Below are some ideas others have mentioned in regard to improving the **PBAC submission process** for individuals and cancer consumer organisations. Rate each out of 10, where 0 means you think it is not an idea worth pursuing and 10 means it is an idea definitely worth pursuing.



- a. Notification by the PBAC to consumer organisations of drugs currently being evaluated and a call for submissions
- b. Providing guidelines on how and when to make a submission
- c. Provide guidelines on the evaluation criteria PBAC use for each submission
- d. Providing a web portal access for submissions
- e. Provide a PBAC staff member whose role is to support consumers in their submission process
- f. The option of providing a patient impact statement and the opportunity to present in person
- g. Increase the timeframe for consumer input to beyond 6 weeks
- h. PBAC personnel to receive training about the involvement of individuals and consumer organisations in the submission process.
- i. Individuals and consumer organisations given the opportunity to participate in training about the PBAC submission process

13. Please feel free to provide further explanations of your responses. OPEN ENDED

### Improvements to the evaluation process

14. Below are some ideas others have mentioned to ensure individuals and cancer consumer organisations have a stronger voice in the **PBAC evaluation process**. Rate each out of 10, where 0 means you think it is not an idea worth pursuing and 10 means it is an idea definitely worth pursuing.

- a. Have more consumers on the PBAC
- b. Have a consumer sub-committee the PBAC can call on for information regarding specific conditions
- c. Host public hearings for individuals and consumer organisations
- d. Set up a consumer liaison in the Department of Health
- e. Provide feedback to individuals and consumer organisations about the effectiveness and impact of their submissions on the final approval decision.
- f. A regularly review of PBAC consumer engagement by the Department of Health and consumer organisations

15. Please feel free to provide further explanations of your responses. OPEN ENDED

### The most important consideration

16. Having thought about the various ways individuals and consumer organisations can contribute to the decision-making process that determines what treatments should be funded, is there one thing more than anything else you think should be considered in the current Australian PBAC system?

**Final comments**

17. Are there any other comments you would like to make about the topics and themes contained in this survey?

**Thank you for your time in completing this questionnaire**