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Community views on factors affecting medicines resource allocation: cross-sectional survey of 3080 adults in Australia

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Abstract

Objective. The aim of the present study was to determine Australian community views on factors that influence the distribution of health spending in relation to medicines.

Methods. A cross-sectional web-based survey was performed of 3080 adults aged ≥18 years. Participants were asked to rank, in order of importance, 12 criteria according to which medicines funding decisions may be made.

Results. Of all respondents, 1213 (39.4%) considered disease severity to be the most important prioritisation criterion for funding a new medicine. This was followed by medicines treating a disease affecting children (13.2%) and medicines for cancer patients (9.1%). Medicines targeting a disease for which there is no alternative treatment available received highest priority from 8.6% of respondents. The remaining eight prioritisation criteria were each assigned a top ranking from 6.6% to 1.7% of respondents. Medicines targeting a disease for which there is no alternative treatment available were ranked least important by 7.7% of respondents, compared with 2.4%, 1.9% and 1.0% for medicines treating severe diseases, diseases affecting children and cancer respectively. 'End-of-life treatments' and 'rare disease therapies' received the least number of highest priority rankings (2.0% and 1.7% respectively).

Conclusions. These results provide useful information about public preferences for government spending on prescribed medicines. Understanding of public preferences on the funding of new medicines will help the Pharmaceutical Benefits Advisory Committee and government determine circumstances where greater emphasis on equity is required and help inform medicines funding policy that best meets the needs of the Australian population.

What is known about this topic? There is increased recognition of the importance of taking into account public preferences in the heath technology assessment (HTA) decision-making process.

What does this paper add? The Australian public view the severity of disease to be the most important funding prioritisation criterion for medicines, followed by medicines used to treat children or to treat cancer.

What are the implications for practitioners? The general public are capable of giving opinions on distributional preferences. This information can help inform medicines funding policy and ensure that it is consistent with the values of the Australian population.

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Introduction

In Australia, the Pharmaceutical Benefits Advisory Committee (PBAC) is responsible for advising the government as to which medicines should be subsidised on the Pharmaceutical Benefits Scheme (PBS). In fulfilling this role, the PBAC is the steward of a large sum of public money: for example, for the 12 months ending 30 June 2015, total PBS spending amounted to A$9.07 billion.[1]

The PBAC makes its recommendations primarily on the basis of evidence of clinical effectiveness, safety and cost-effectiveness derived from clinical trials and population-based observational studies. The PBAC also takes into account other factors, such as equity, in its consideration of what does, or does not, constitute 'value for money'. In this context, the term 'equity' refers to access to PBS-listed drugs in a manner that takes into account the distribution of benefits and potential harms based on factors such as prognosis, disease severity, age, distributional effect, context (e.g. emergency or prevention), socioeconomic and geographical status and other issues not typically considered as part of quality of life measurements.[2]

Previous analyses of PBAC recommendations demonstrated that the PBAC has been broadly consistent in its use of economic efficiency as a key criterion for decision making.[3,4] The probability of a positive recommendation does increase with lower incremental cost-effectiveness ratios, but there is no evidence of a fixed threshold for the value of a life year or a quality adjusted life year (QALY).[4] Importantly, the PBAC has been found to actualise equity considerations by accepting a higher incremental cost-effectiveness ratio (ICER) for medicines addressing a high unmet clinical need[5–7] and/or greater uncertainty in the available clinical evidence for rare diseases.[8]

Further, the PBAC in its deliberation may consider the 'rule of rescue' (RoR). The consideration and application of RoR allows the PBAC to potentially reverse a decision not to recommend listing on the PBS because of its consideration of comparative cost-effectiveness (and any other relevant factors). However, evidence (based on the published public summary documents (PSDs) for past PBAC recommendations [9]) indicates that the RoR has been applied infrequently by the PBAC and that there were few documented examples where application of the RoR has led to a positive PBAC recommendation.[9] PBAC consideration of the RoR requires the following four factors to be met: (1) no alternatives exist in Australia; (2) the medical condition is severe, progressive and expected to lead to premature death; (3) the medical condition applies to only a very small number of patients; and (4) the proposed medicine provides a worthwhile clinical improvement sufficient to qualify as a rescue from the medical condition.[2] However, the relative influence or weight of the RoR factors is not quantitatively predefined. Importantly, the RoR, as with other relevant factors, supplements rather than substitutes for evidence-based consideration of cost-effectiveness.[2]

The PBAC also provides advice on the inclusion of medicines on the Life Savings Drugs Program (LSDP).[9] The LSDP sits outside the PBS to provide an alternative funding arrangement for access to medicines that are not eligible for funding under the PBS due to unacceptable cost-effectiveness. While those making submissions to the PBAC occasionally include population survey data on community preferences, assessments of equity are most commonly based on assumptions about community priorities. Given the central role that the general public has in funding publicly subsidised health technologies through taxes, and as beneficiaries of these technologies, it is increasingly recognised that this is inadequate and that more information is needed about public preferences when making decisions about the funding of new medicines.[10–12]

Around the world, government agencies responsible for the selection and reimbursement of prescribed medicines and other health technologies are increasingly concerned with how best to incorporate community preferences into their decision making.[13,14] In Australia, the PBAC currently considers patients and the public views through consumer representation on the Committee, via an online consumer input process and through consumer hearings convened by the PBAC for selected submissions. Recent examples of such hearings include those for lymphoma (brentuximab vedotin, bendamustine, idelalisib and obinutuzumab), which were considered at the March 2015 PBAC meeting,[15] and for ovarian cancer and Morquio A syndrome (olarparib and elosulfase alfa respectively), which were considered at the March 2016 PBAC meeting.[16]

Another important approach to eliciting consumer preferences, which supplements more direct forms of consumer engagement, is to conduct surveys of representative samples of the community. These have been used previously to support policy concerning the funding of cancer drugs in the UK,[17] to assess the preferences for the funding of orphan drugs[18] and to understand public agreement with policies aimed at facilitating access to life-extending drugs used at the end of patients' lives.[19] To date, however, no representative community survey has explored how members of the Australian community rank various criteria according to their importance to funding decisions for prescribed medicines. Therefore, we conducted an online survey of 3080 Australians aged ≥18 years in order to measure community preferences for the distribution of the benefits and costs of PBS-listed drugs.

Methods

A cross-sectional web-based survey was performed of 3080 adult Australians aged ≥18 years. This paper focuses on the findings from the ranking exercise conducted as part of that survey.

SSI (Sydney, NSW, Australia), a market research company with a large online panel (~409 000 registered members) was

used to recruit survey participants. Recruitment was controlled by gender, age and geographical area (state of residence) in order to ensure that the sample was representative of the general adult Australian population. Participants were compensated for their time and received 'reward points' averaging A$1.40 from the panel provider. Selection of the 12 prioritisation criteria was informed by both the published literature and criteria currently used by the PBAC when assessing new medicines for public subsidy.[5–10] The 12 prioritisation criteria were as follows: (1) severity of disease; (2) availability of alternative medicine; (3) significant innovation; (4) carer burden; (5) disadvantaged populations; (6) children; (7) end-of-life treatments; (8) cancer treatments; (9) rare disease therapies; (10) cost to the PBS and savings to patient; (11) medicines that help patients return to work; (12) lifestyle related diseases and individual responsibility.

The survey asked respondents which criteria they believed were the most important in health care spending and resource allocation. Respondents were asked to rank the 12 prioritisation criteria from 1 to 12, with 1 being the most important criterion. The survey was pilot tested with 111 participants in August 2015. An additional question regarding the state of residence was added after pilot testing. The full survey was administered during October 2015 and was closed when our target of 3000 complete responses was achieved. Sociodemographic data were collected to test associations between respondents' views on the prioritisation criteria and demographic characteristics.

Ethics approval

This study was approved by the Human Ethics Research Committee at Sydney University (Protocol no. 2014/906).

Statistical analysis

Descriptive statistics were used to summarise demographic variables. Empirical studies have found that willingness to pay and funding preferences are influenced by respondents' personal circumstances, such as age, income, health status and household composition.[17,20] Multinomial logistic regression modelling was used to assess whether gender, age, marital status, education, health status, cancer history, country of birth, private health insurance, employment status, household income and dependent children were associated with the top ranking of the 12 prioritisation criteria. The model included all explanatory variables listed.

Results

In all, 3080 adult members of the general public in Australia completed the online survey. The 3080 respondents broadly reflected the Australian population in terms of age, gender and geographical area (Table 1). Of the respondents, 39.4% considered disease severity to be the most important prioritisation criterion (Table 2). This was followed by medicines for diseases affecting children (13.2%). Cancer medicines came third and were ranked most important by 9.1% of respondents, whereas medicines targeting a disease for which there is no alternative treatment available received highest priority from 8.6% of respondents. The remaining eight prioritisation criteria were each assigned a top ranking by 6.6–1.7% of respondents.

The four prioritisation criteria that were assigned the highest priority also received the largest number of top 3 rankings: disease severity ($n = 1966$; 21.3%), medicines for children ($n = 1260$; 13.6%), cancer medicines ($n = 1112$; 12.0%) and medicines targeting a disease for which no other medicine is available ($n = 957$; 10.4%).

Medicines targeting a disease for which there is no alternative treatment available were ranked least important (i.e. with a respondent's assigned rank order of 12) by 7.7% of respondents compared with 2.4%, 1.9% and 1.0% for medicines treating severe or life-threatening diseases, treating a disease affecting children and medicines for cancer patients respectively.

'End-of-life treatments' and 'rare disease therapies' received the least number of highest priority rankings (2.0% and 1.7% respectively).

Relationship between respondent characteristics and prioritisation preferences

Country of birth ($P = 0.04$), employment status ($P = 0.04$) and having dependent children ($P = 0.0001$) were associated with funding preferences (see Table S1, available as Supplementary Material to this paper). Respondents who were born overseas were significantly more likely to assign a top priority to medicines that help patients return to work (odds ratio (OR) 1.57; 95% confidence interval (CI) 1.06–2.32; $P = 0.02$) and to medicines targeting lifestyle-unrelated diseases (OR 1.57; 95% CI 1.01–2.42; $P = 0.04$) than to prioritise disease severity compared with respondents born in Australia. Respondents with dependent children were significantly more likely to assign a top ranking to medicines targeting diseases affecting children (OR 2.04; 95% CI 1.52–2.78; $P < 0.0001$) and to cancer medicines (OR 1.45; 95% CI 1.01–2.04; $P = 0.04$). Respondents who were in part-time employment were significantly less likely to assign a top finding priority to medicines targeting rare diseases than those working full-time (OR 0.19; 95% CI 0.05–0.66; $P = 0.01$). Compared with respondents who were in full-time employment, respondents who were neither in employment nor unemployed (i.e. 'other' category; e.g. those who were looking after a home or studying full time) were significantly more likely to assign a top ranking to medicines targeting diseases that affect patients who are not financially well-off (OR 1.72; 95% CI 1.02–2.87; $P = 0.04$). Further, these respondents were significantly less likely to allocate the highest funding priority to medicines targeting lifestyle-unrelated diseases (OR 0.15; 95% CI 0.03–0.63; $P = 0.01$) compared with those in full-time employment.

There was also some evidence that health status ($P = 0.06$) and private health insurance ($P = 0.06$) were associated with funding preferences. Compared with respondents rating themselves as in very good health, respondents who rated themselves as in good, average, or poor or very poor health were significantly more likely to assign a top ranking to medicines targeting diseases that affect patients who are not financially well-off (OR 1.90 (95% CI 1.13–3.20; $P = 0.02$), OR 2.33 (95% CI 1.35–4.01; $P = 0.002$) and OR 2.40 (95% CI 1.20–4.79; $P = 0.01$) respectively) and to medicines that cost the government more and thereby save patients more in out-of-pocket costs (OR 2.25 (95% CI 1.19–4.26; $P = 0.01$), OR 2.18 (95% CI 1.11–4.28; $P = 0.02$) and OR 3.12 (95% CI 1.39–7.02; $P = 0.006$) respectively). Respondents who did not have private health insurance were significantly more likely to allocate the highest funding priority to medicines

Table 1. Characteristics of survey respondents ($n = 3080$)
TAFE, Technical and Further Education

Characteristics	No. respondents (%)	Australia[A] (%)
Gender		
Male	1502 (48.8)	48.9
Female	1578 (51.2)	51.1
Age (years)		
18–24	374 (12.1)	12.2
25–34	542 (17.6)	18.0
35–44	596 (19.4)	18.5
45–54	553 (18.0)	17.9
55–64	481 (15.6)	15.2
65+	534 (17.3)	18.2
Marital status		
Married or de facto	1832 (59.5)	
Separated, divorced or widowed	406 (13.2)	
Never married	842 (27.3)	
Education		
Never attended school; primary, some high school; preferred not to answer	444 (14.4)	
Completed high school	627 (20.4)	
University, TAFE etc.	2009 (65.2)	
Cancer history		
Cancer history with death	1175 (38.1)	
Cancer history with no death or death unknown	489 (15.9)	
No cancer history	1376 (44.7)	
Prefer not to answer	40 (1.3)	
General health		
Very good	544 (17.7)	
Good	1481 (48.1)	
Average	842 (27.3)	
Poor or very poor	213 (6.9)	
Country of birth		
Australia	2285 (74.2)	
Overseas	795 (25.8)	
Private health insurance		
Yes	1814 (59)	
No	1266 (41)	
Employment status		
Working full-time	1082 (35.1)	
Working part-time	622 (20.2)	
Currently not working, but looking for work	376 (12.2)	
Retired	669 (21.7)	
Other	331 (10.7)	
Household annual income (A$)		
0–20 000	249 (8.1)	
20 001–40 000	610 (19.8)	
40 001–80 000	863 (28.0)	
≥80 001	1008 (32.7)	
Prefer not to answer	350 (11.4)	
Personal annual income (A$)		
0–20 000	754 (24.5)	
20 001–40 000	711 (23.1)	
40 001–80 000	792 (25.7)	
80 001–180 000	422 (13.7)	
≥180 001	47 (1.5)	
Prefer not to answer	354 (11.5)	
Household composition		
With financially dependent children	927 (30.1)	
Without financially dependent children	2153 (69.9)	
State		
Australian Capital Territory	47 (1.5)	1.7
New South Wales	985 (32.0)	32.2
Northern Territory	10 (0.3)	0.9
Queensland	587 (19.1)	19.9
South Australia	236 (7.7)	7.6
Tasmania	70 (2.3)	2.3
Victoria	745 (24.2)	25.1
Western Australia	289 (9.4)	10.4
Unknown[B]	111 (3.6)	–

[A]Australia demographics (gender, age and state of residence) are for people aged ≥18 years and were sourced from the TableBuilder available from the Australian Bureau of Statistics based on 2011 Census data. (http://www.abs.gov.au/websitedbs/censushome.nsf/home/tablebuilder?opendocument&navpos=240, accessed 17 May 2016).
[B]The pilot survey ($n = 111$) did not include this demographic question.

that cost the government more, thereby saving patients more in out-of-pocket costs compared with those with private health insurance (OR 1.58; 95% CI 1.07–2.31; $P = 0.02$).

Discussion

The results of the present study give a clear picture of public preferences regarding resource allocation for medicines. The targeting of severe or life-threatening diseases is clearly and by far the most important prioritisation criterion, followed by medicines targeting diseases affecting children, cancer medicines and medicines targeting diseases for which no treatment alternative is available. However, the first three top ranking prioritisation criteria were assigned a least important ranking by a small proportion of respondents (1–2.4%). Medicines targeting a disease for which no alternative treatment exists were ranked most and least important by a similar proportion of respondents (8.6% and 7.7% respectively). One possible explanation for this variation is that societal opinion on the use of this as a prioritisation criterion for new medicines funding may be divided and 'polarised'.

Further, findings from the present study resonate with previous studies,[11,17,19,21,22] which have shown that members of the general public give higher priority to medicines used for the treatment of severe illness and for those with no available alternatives. The finding of support for prioritising anticancer medicines is also generally consistent with existing evidence[23,24] and could explain the current focus both in Australia and internationally on achieving timely access to such treatments.[25] However, because cancer medicines were the only disease-specific medicines explored in the present study, this finding should be interpreted with caution. We found no compelling evidence for prioritising end-of-life treatments. This is consistent with the study of Linley and Hughes,[17] who examined the views of the UK general public on the current and proposed medicines

Table 2. Number of times a prioritisation criterion was assigned the top priority, lowest priority (i.e. with a ranking order of 1 and 12 respectively) and a top 3 ranking by respondents

Data are given as *n* (%). PBS, Pharmaceutical Benefits Scheme

Prioritisation criteria	Rank 1 (most important; *n* = 3080)	Rank 12 (least important; *n* = 3080)	Top 3 ranking (*n* = 9240)
Severity of disease: Preference for funding should be given to new medicines that treat severe or life-threatening conditions	1213 (39.4)	73 (2.4)	1966 (21.3)
Children: Preference for funding should be given to new medicines targeting diseases that typically affect children	405 (13.1)	57 (1.9)	1260 (13.6)
Cancer treatments: Preference for funding should be given to new medicines targeting cancer patients	280 (9.1)	30 (1.0)	1112 (12.0)
Availability of alternative treatment options: Preference for funding should be given to new medicines that target diseases for which no other treatments are available	266 (8.6)	236 (7.7)	957 (10.4)
Disadvantaged populations: Preference for funding should be given to new medicines targeting diseases that typically affect disadvantaged patients (e.g. low-income families)	204 (6.6)	161 (5.2)	760 (8.2)
Cost to the PBS and savings to patient: Preference for funding should be given to new medicines that cost the government more and thereby save patients more in out-of-pocket costs	139 (4.5)	288 (9.4)	474 (5.1)
Medicines that help patients return to work: Preference for funding should be given to new medicines that help patients return to work	133 (4.3)	200 (6.5)	508 (5.5)
Carer burden: Preference for funding should be given to new medicines targeting diseases that, if untreated, cause patients to be reliant on carers	110 (3.6)	146 (4.7)	594 (6.4)
Lifestyle-related diseases and individual responsibility: Preference for funding should be given to new medicines targeting diseases that are not considered to be a lifestyle-related disease (i.e. diseases that could not be avoided through individual lifestyle changes)	109 (3.5)	1041 (33.8)	296 (3.2)
Significant innovation: Preference for funding should be given to new medicines that work in a new and different way to existing treatments	107 (3.5)	221 (7.2)	569 (6.2)
End-of-life treatments: Preference for funding should be given to new medicines that prolong life (even for a few months) at the end of life (i.e. for patients with a life expectancy of less than 2 years)	63 (2.0)	476 (15.5)	363 (3.9)
Rare diseases: Preference for funding should be given to new medicines targeting rare diseases (i.e. diseases affecting less than 2000 patients in Australia)	51 (1.7)	151 (4.9)	381 (4.1)

prioritisation criteria used by the UK National Institute of Health and Care Excellence (NICE) and government.

Our study suggests that rare disease therapies per se are not a strong driver for public funding preferences. Although this is consistent with other research,[17,18] it is nonetheless a somewhat surprising finding given that rarity of disease is one of the four criteria that form the basis of the RoR PBAC claim.[2] An RoR applies in exceptional circumstances for drugs that provide a worthwhile benefit for a severe and rare condition for which there is no alternative treatment.[2,9,26] The results of the present study suggest that the use of rarity of the disease as an inclusion criterion for LSDP or as a basis for an RoR claim does not appear to be supported by the Australian public. One possible interpretation of this result is that rarity is not a shared prioritisation criterion between the general public and the PBAC. Given that the rarity of the disease is linked to the total number of eligible patients and cost for funding a medicine, it is, and may need to remain, an important prioritisation criterion from the PBAC and government perspective, especially for high-cost medicines.

An important strength of the present study is that it included a large, broadly representative sample of 3080 adult Australians. However, due to the design of the study, non-completion rates and details of non-responders were unavailable for analysis or assessment for potential non-responder bias. Another potential limitation of the present study relates to framing effects. It has been found that the choice of wording in surveys is very important.[27] The results for the prioritisation criterion relating to lifestyle-unrelated diseases appear to be somewhat surprising, with the largest proportion of respondents ranking this criterion last. It is possible that respondents' preferences may have been confounded by the labelling choice used in the survey. Despite these limitations, the present study has important implications for health policy development with regard to the funding of new medicines in Australia.

Further, our research shows that respondents' funding preferences for access to new medicines are influenced by their personal characteristics and circumstances. Therefore, if the general public's views and preferences are to be included in the PBAC decision-making process, a representative sample is required.

In summary, the findings of the present study provide assurance that the Australian public support some of the currently used prioritisation criteria. However, quantification of criteria weights and equity issues relative to other factors will require further research in order to provide guidance to the PBAC

on the cardinality of equity preferences and quantification of ICER increase to account for the specific equity issues and criteria identified.

Conclusions

The reimbursement of prescribed medicines should reflect both evidence of safety and effectiveness, as well as social values.[28] As such, it is important to understand societal views and preferences for the distribution of health care spending. The results of the present study provide useful information on public preferences related to the equity aspects of government spending on prescribed medicines in Australia. Understanding of public preferences on funding of new medicines could help the PBAC and government determine the circumstances under which greater emphasis on equity is required, and how equity may be defined and achieved in a manner that is congruent with the values of the Australian population. To ensure that public preferences are reflected in the PBAC's assessments and recommendations, there is a need for further research to determine the best way to incorporate these preferences into PBAC decision-making processes. This will, in turn, improve alignment between government and societal preferences for the funding of new medicines.[29,30]

Competing interests

Lesley Chim is employed by BioMarin Pharmaceutical Inc. and was enrolled in a PhD degree with the School of Public Health, University of Sydney at time of this study. No personnel within BioMarin read or had input regarding the study or the content of this paper. Professor Glenn Salkeld has received an honorarium from Pfizer for teaching in a short course.

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