The prioritization preferences of pCODR members and the Canadian public: a stated preferences comparison

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Abstract

The pan-Canadian Oncology Drug Review (pCODR) is responsible for making coverage recommendations to provincial and territorial drug plans about cancer drugs. Within the pCODR process, small groups of experts (including public representatives) consider the characteristics of each drug and make a funding recommendation. It is important to understand how the values and preferences of these decision-makers compare with the citizens on whose behalf they are acting. This study used stated preference methods to elicit prioritization preferences from a representative sample of the Canadian public and a small convenience sample of pCODR committee members. The results suggested that neither group sought to strictly maximize quality-adjusted life year (QALY) gains and were willing to sacrifice some efficiency to prioritize particular patient characteristics. Both groups had a significant aversion to prioritising older patients, patients in good pre-treatment health, and patients in poor post-treatment health. This result is reassuring in that it suggests pCODR decision-maker preferences are consistent with those of the Canadian public, but it also implies that like the larger public, decision-makers may value health gains to some patients more or less highly than the same gains to others. The implicit nature of pCODR decision criteria means that the acceptability or limits of such differential valuations are unclear. Likewise, there is no guidance as to which potential equity factors (e.g. age, initial severity, etc.) are legitimate and which are not. This vagueness could have negative consequences for the consistency and transparency of recommendations and, in turn, the legitimacy of the pCODR process.

**Keywords**: Priority-setting; stated preferences; pCODR

# 1.0 Background

The pan-Canadian Oncology Drug Review (pCODR), part of the Canadian Agency for Drugs and Technology in Health (CADTH), is responsible for making coverage recommendations to provincial and territorial drug plans about cancer drugs. The pCODR review process is designed “to bring consistency and clarity to the assessment of cancer drugs” and emphasizes four dimensions of value in their decision criteria: clinical benefit, economic evaluations, patient-based values, and adoption feasibility (1). pCODR guidelines state that there is no weighting scheme for these criteria, and that there is no threshold that must be met for any single element of the review. Rather, decisions should be made on the basis of the individual drug, disease and context (1). In this regard, pCODR may be described as taking an implicit approach to decision making. Proponents of implicit approaches to decision-making argue that some ambiguity is necessary to address the inherent complexity of priority-setting, allowing for individual decision-makers to exercise appropriate contextual judgement (2–4). Advocates of more explicit processes, however, argue that being vague or implicit can lead to inconsistency in decision-making, and has the potential to create an invisible class of ‘others,’ who may be victims of injustice or discrimination without knowing it (5,6).

pCODR, like many other decision-making bodies, relies on a process of ‘procedural objectivity’, whereby societal decision-making is delegated to small groups of experts (and often including members of the lay public) appointed on the basis of their knowledge, expertise and professionalism (7,8). In this context, pCODR decision-makers can be seen to be acting as ‘agents’ on behalf of society, ensuring that limited societal resources – in this case, funding for cancer drugs – are put to their most valuable uses (9). This approach, though, concentrates decision-making authority in the hands of a relatively small number of experts. Arguably, the responsibility of societal decision-makers is not always to reflect what citizens *would* do, but rather what they *ought* *to* do (10). That is, the knowledge and expertise of the decision-makers may in some cases lead to decisions at odds with public opinion. However, the allocation of societal resources is, even within a putatively objective process, an unavoidably subjective issue, and therefore the preferences of decision-makers should be broadly consistent with the values and preferences of society (11).

In this light it was of interest to understand how the preferences of pCODR decision-makers compare with those of the Canadian public over the allocation of scarce healthcare resources. Within this overall objective, the first aim of the study was to understand the degree to which respondents’ allocation choices may be driven by the principles of quality-adjusted life year (QALY) maximization. The QALY maximisation framework requires that resources be allocated in the way that produces the greatest aggregate QALY gains, which combine years-of-life , quality-of-life, and the total number of beneficiaries into a single summary measure of health gains. Critically, the QALY maximization framework presumes ‘distributive neutrality’, or that society is indifferent to how health gains are distributed between different individuals or groups (12,13). QALY maximisation is a dominant paradigm in healthcare priority-setting and has been shown to play an important role in drug review processes in the UK and Australia (14,15). This framework is also reflected in pCODR’s consideration of relative cost-effectiveness, although it is important to note that pCODR does not define an acceptable cost-effectiveness threshold.

Recent research, though, has suggested that the QALY maximization framework may not be consistent public preferences, and that the public is not indifferent to how health gains are distributed. Rather, there is evidence of a willingness to forego some QALY gains in order to distribute health gains more fairly, or equitably (12,16–18). If most respondents to this elicitation did not appear to make allocation choices on the basis of strict QALY maximization, the second aim was to compare the degree to which pCODR decision-makers and the Canadian public were willing to forego efficiency for greater equity. This is known as an ‘equity-efficiency trade-off’ and it is based on the idea that equity and efficiency are commensurate concepts, and that more of one can compensate for less of the other (19). That is, society may be willing to prioritise a relatively inefficient program if it is associated with a fairer or more equitable outcome. There are limits to this trade-off, though, and at some point a gain in equity is too minor to justify the sacrifice in efficiency, or alternatively, a gain in efficiency is so great that it justifies some inequity. The second aim, therefore, was to understand how the limits of this equity-efficiency trade-off may differ between the public and their agents.

# 2.0 Methods

The preferences of an age and sex representative sample of the Canadian public and a convenience sample of pCODR committee members were elicited using stated preference (SP) methods. These methods ask respondents to make choices over hypothetical alternatives, each described in terms of sets of attributes and levels. By systematically varying the levels of the attributes across a series of tasks it is possible to infer the weight that respondents gave to different attributes and levels in their choices (20).

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### 2.1 Experimental design

The process of identifying the patient and program attributes used to described each patient group has been described in detail elsewhere (18). Briefly, the attributes were identified through a process of empirical ethics, whereby each attribute had to have empirical evidence of public support, and be consistent with some coherent and defensible theory of justice (21).  An empirical filter ensures that the attributes are relevant to society, while an ethical filter contributes to the legitimacy of those attributes and avoids the “moral relativism” that may result from strictly empirical methods, whereby what is morally right or wrong is reduced to social consensus or a simple majority (22).

A ‘pearl growing’ search strategy was used to identify patient and program characteristics with empirical evidence of societal relevance, building on the references and forward citations of a handful of key articles. The ethical justification for these characteristics was then subjectively assessed on the basis of prominent theories of distributive justice including need, maximising, and egalitarian principles. Through this process, patient age, severity before/without treatment, final health state with/after treatment, and the distribution of health gains were judged to have evidence of public support and a defensible ethical justification:. To distinguish between severity as proximity to death and severity as a poor health state, this factor was decomposed into life expectancy without treatment and initial health state.  Distribution of benefit was considered in terms of the number of patients that could be treated. Finally, duration of benefit was included to facilitate the calculation of QALY gains despite some ambiguity over its interpretation in this context.

It is worth noting that a number of attributes that have been included in similar elicitations were excluded by the process used here.  Most notably, prioritisation on the basis of a patient's lifestyle or culpability for their illness had empirical support but was judged to have little ethical justification, while priority on the basis of social role or productivity had a utilitarian ethical justification but little empirical support.

To balance information on the shape of the utility function with statistical efficiency, each attribute was assigned three levels, evenly spaced across plausible ranges to allow for the identification of nonlinear preferences over a minimal number of scenarios. The attributes and their levels are shown in Table 1. Aggregate QALY gains was calculated as a function of the other attribute levels and included as an additional attribute in each alternative, but was excluded from the statistical models to avoid multicollinearity with the component attributes.

Given six attributes of three levels each, there were 729 (36) possible scenarios. As a survey with this many possible combinations would be beyond the capacity of any respondent to complete, a fractional factorial experimental design was developed in SAS (23). The final design had 22 paired choice tasks, which were divided into 2 blocks of 11 tasks each. Each block included a test of dominance or non-satiation, where one alternative was unambiguously better than the other in terms of health gain, and a test of consistency, where respondents were presented with the same alternatives seen in an earlier task. These tests were included for descriptive purposes but were not used to exclude ‘irrational’ respondents (24).

### 2.2 Data collection

Two groups were recruited to participate in the elicitation. First, individuals associated with the pCODR review process, including members of the expert panel and the clinical and economic review committees, were invited to participate via targeted emails. Second, an age-sex representative sample of the Canadian general population was drawn from an online survey panel maintained by Research Nowtm, a market research firm.

The elicitations were administered via the internet. Respondents were asked to imagine themselves as a decision-maker responsible for allocating a fixed budget between two competing healthcare programs. They were told that both programs had the same cost and that the budget was not large enough to fund both of them. To provide a uniform context, respondents were told that the groups each had some form of cancer, but specific diagnoses were not mentioned and the alternatives were presented simply as Program A and Program B. Although labelled alternatives have the advantage of making hypothetical choice tasks more realistic and concrete, respondents may also use such labels to infer information that was not presented or intended as part of the task. At the extreme, respondents might ignore trade-offs between attributes and make their choices based on their perceptions of the labels alone (25).

Participants were randomised to either a discrete choice experiment (DCE) or a constant-sum paired comparison (CSPC) questionnaire. The DCE tasks asked respondents to identify the patient group that they would prefer to prioritise in each of a series of paired alternatives. The CSPC tasks presented the alternatives in the same manner as the DCE but asked respondents to allocate budget percentages between the two programs by moving a slider. Respondents could allocate 100 percent of the budget to program A or program B, or to some combination of the two, including a 50-50 allocation. Examples of the two tasks are shown in the appendix.

### 2.3 Analysis

The two groups were compared in terms of the number of choices out of the 11 tasks that each respondent saw that prioritized the QALY maximising alternative. In each choice task, one alternative was always associated with greater potential aggregate QALY gains than the other. It was anticipated that pCODR decision-makers would be more familiar with the principles of QALY maximisation and therefore would be more likely to make choices on that basis. The number of QALY maximizing choices by respondents in the two groups was compared on the basis of a permutation t-test, and the proportion of respondents in each group who prioritized the QALY maximizing alternative in the majority (≥ 6 out of 11) of their choices was compared using a two sample z-test of proportions.

To maximize the statistical power of the analysis given the small number of pCODR members who could potentially participate, all responses to the CSPC questionnaire were transformed to discrete choices on the basis of which alternative was allocated the majority of the budget. These transformed responses were combined with the DCE responses into a single dataset. Equal 50-50 responses in the CSPC questionnaire were excluded from the analysis as there was no preferred alternative.

Choice responses were modelled using a pooled multinomial logit (MNL) (26), and the strength of preferences for different attribute levels were calculated in terms of compensating variation (CV) (27):



Where *βLYg* was the coefficient on the life years gained attribute, or the constant marginal utility of an additional life year gained, and *v0* and *v1* were the utilities before and after a change in the level of attribute from *x0* to *x1*. CV is a measure of the amount of the numeraire (in this case, life years gained) that a respondent would be willing to forego in order to prioritize a particular level of *x*. A negative CV indicates that respondents prefer the new level (*x1*) over the baseline level (*x0*), while a positive CV indicates a preference for the baseline level (i.e. that a respondent would need to be compensated in order to accept a change to *x1*). Note that these life years accrue to the hypothetical patients in the choice tasks, not the respondents. CV was calculated for upward and downward changes in each attribute holding all other attributes at baseline. Confidence intervals were calculated using the delta method (28). Preferences for a particular attribute level were judged to be significantly different between the two groups if the confidence intervals did not overlap. All statistical analyses were performed using R statistical software, version 2.13.3 (29).

# 3.0 Results

In total, 21 pCODR members responded to the survey; 11 completed a DCE questionnaire and 10 completed a CSPC. In the representative public sample, 656 respondents completed a DCE questionnaire and 662 completed a CSPC. Thirteen out of 231 CSPC allocations (5.6%) among the pCODR sample and 823 out of 14,267 allocations (5.8%) among the public sample were excluded because of an equal 50-50 allocation. No pCODR respondents and only 8 public respondents (1.3%) equalised CSPC allocations in every task, suggesting that most respondents did not hold strict egalitarian preferences.

### 3.1 Consistency with QALY maximization

The distribution of total QALY maximising choices for each respondent, by group, is shown in Figure 1.

*<FIGURE 1 ABOUT HERE>*

On average, pCODR respondents were more likely than the public to choose the QALY maximising alternative (6.7 vs. 5.9 out of 11 tasks), but this difference was not statistically significant (p=0.08). The majority of respondents in both groups chose the QALY maximising alternative in the majority of their choices: 62 per cent of pCODR respondents chose the QALY maximising alternative in 6 or more of their 11 choices compared to 58 per cent of the public (p=0.83). A notably larger proportion of pCODR respondents chose the QALY maximising alternative in all or almost all of their choices: 48 per cent of pCODR respondents made 8 or more QALY maximising choices compared to 23 per cent of the public (p=0.02).

### 3.2 Respondent preferences by attribute

Compensating variations by respondent group are detailed in Table 1 and illustrated in Figure 2 by attribute and level. In the figure, preferences for the high and low levels of each attribute are shown relative to the middle (baseline) level. Note that the y-axis is reversed to show more preferred levels above the baseline and less preferred levels below.

*<FIGURE 2 ABOUT HERE>*

Overall, there was a close correspondence between the preferences of pCODR and public respondents, with no statistically significant differences over any attribute levels. Both groups had a significant aversion to prioritising older patients, patients in a good initial health state or those who would finish treatment in a poor health state, and smaller patient groups. Conversely, there was a significant preference for greater priority for patients in a more severe initial health state and larger patient groups. There were no significant preferences over untreated life expectancy in either group.

# 4.0 Discussion and conclusion

This work addresses the issue of whether the Canadian public, and pCODR committee members acting on behalf of the public, are strict health (QALY) maximizers or appeared willing to forego some degree of efficiency in order to prioritise specific patient characteristics. This is question is important in light of pCODR’s commitment to an implicit decision-making framework, which gives individual committee members considerable latitude in assessing the value of cancer drugs in different patient populations. While this allows decision-makers to exercise context-specific judgements, it could also lead to recommendations at odds with the values and preferences of the broader society they represent.

This question does not address which decision criteria have the greatest impact in predicting pCODR decisions. Such studies have previously been conducted in the UK and Australia (14,15), and a Canadian study is currently ongoing using publicly-available pCODR data. But whereas studies of the impact of different decision criteria focus on the characteristics of the *drugs*, including clinical benefit, economic factors, patient preferences, and adoption feasibility (1), the current study explores differences in the perceived value of health gains to different *patients*. In this sense, drugs with the same clinical and economic characteristics may be valued differently because of the differences in the relative value of health gains to the patients they treat. For example, consideration of a patient’s culpability for their illness was specifically excluded from this analysis, but it is conceivable that society and/or pCODR decision-makers may view the value of health gains to a heavy smoker with lung cancer differently than the same gains to a child with leukaemia.

It is important to acknowledge that the statistical power of this analysis was limited by the very small sample of decision-makers. This was unavoidable, as the centralization of provincial oncology drug review processes within pCODR means that the potential pool of ‘decision-makers’ in Canada is relatively small. The number of responses was also reduced by the exclusion of 50-50 allocations in converting CSPC responses to discrete choices. This would have had the effect of excluding more moderate preferences and emphasizing the extremes. However, these exclusions did not affect the count of QALY maximizing choices, and the relatively small and similar proportions of exclusions among pCODR and public respondents suggests that they should not have had a substantive or differential impact on the observed preferences of the two groups.

Within these limitations the results suggest that the preferences of pCODR respondents were not substantively different from those of a representative sample of the Canadian general public. pCODR respondents appeared, on average, only slightly more likely than the general public to make their choices on the basis of QALY maximisation, although a significantly greater proportion of pCODR respondents appeared to be relatively strict QALY maximisers. Both groups were willing to forego some potential life year gains in order to give greater priority to particular patient groups, suggesting a willingness to sacrifice some degree of efficiency for greater fairness and equity. This is reassuring as it suggests that the preferences of pCODR decision-makers are consistent with those of the larger Canadian public they represent.

Critically, however, these results also imply that health gains to some patients may be valued more or less highly than the same gains to other patients. The pCODR decision framework does not define which potential equity and fairness considerations are legitimate in funding recommendations, or how much weight should be given to such factors. For example, the results implied that both groups would be willing to pay more for health gains accruing to younger patients than the same gains to patients aged 70. The acceptability and the limits of such differential valuations are not addressed in the pCODR guidelines. Such ambiguity has negative implications for the transparency and consistency of pCODR recommendations, and could jeopardise trust in the pCODR decision-making process (6,30).

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***Table 1: Attributes and levels presented to respondents***

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
| **Level** | **Age** | **Initial utility** | **Initial life expectancy** | **Final utility** | **Gain in life expectancy** | **Patients treated** |
| **1** | 10 | 1/10 | 1 month | 1/10 | 1 year | 100 |
| **2** | 40 | 5/10 | 5 years | 5/10 | 5 years | 2,500 |
| **3** | 70 | 9/10 | 10 years | 9/10 | 10 years | 5,000 |

*The values of aggregate QALYs gained ranged from 10 to 55,000. Utilities were presented on a 10-point scale but analysed as decimal values.*

Table 2: Compensating variations by attribute level and group

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
|  | **pCODR respondents** |  | **Public** |  |  |  |
| **Attribute** | **CV** | **Std Err** | **L95CI** | **U95CI** |  | **CV** | **Std Err** | **L95CI** | **U95CI** |  | **Difference** | **Significant?** |
| Patients aged 10 | 0.59 | 1.02 | -1.41 | 2.59 |  | -1.17 | 0.77 | -2.68 | 0.35 |  | 1.76 | NO |
| Patients aged 70 | 5.72 | 1.27 | 3.22 | 8.22 |  | 5.29 | 1.00 | 3.32 | 7.26 |  | 0.43 | NO |
| Initial utility 0.1 | -3.45 | 1.09 | -5.58 | -1.32 |  | -3.29 | 0.84 | -4.94 | -1.64 |  | -0.16 | NO |
| Initial utility 0.9 | 2.39 | 1.15 | 0.14 | 4.64 |  | 4.07 | 1.01 | 2.09 | 6.04 |  | -1.68 | NO |
| Initial life expectancy 1m | -0.78 | 1.11 | -2.96 | 1.39 |  | 1.40 | 0.92 | -0.41 | 3.22 |  | -2.19 | NO |
| Initial life expectancy 10yrs | 0.42 | 1.15 | -1.83 | 2.68 |  | 0.51 | 0.89 | -1.24 | 2.26 |  | -0.09 | NO |
| Final utility 0.1 | 9.20 | 1.61 | 6.04 | 12.36 |  | 8.45 | 1.21 | 6.08 | 10.83 |  | 0.75 | NO |
| Final utility 0.9 | -2.31 | 1.36 | -4.97 | 0.35 |  | -2.88 | 1.02 | -4.88 | -0.88 |  | 0.57 | NO |
| 100 patients treated | 3.73 | 1.16 | 1.46 | 6.00 |  | 7.18 | 1.27 | 4.70 | 9.67 |  | -3.45 | NO |
| 5000 patients treated | -1.75 | 1.12 | -3.95 | 0.44 |  | -3.21 | 0.88 | -4.94 | -1.48 |  | 1.46 | NO |