Challenges in Measuring the Societal Value of Orphan Drugs: Insights from a Canadian Stated Preference Survey

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Abstract

Background Expensive drugs for rare diseases (i.e. orphan drugs) often do not meet traditional cost-effectiveness criteria and thus put further strain on limited healthcare budgets. Failing to provide medically necessary care to patients, however, violates one of the underlying tenets of most public health insurance systems—equity. This has led payers to consider the value that society places on the treatment of rare diseases, given the opportunity cost, when deciding on whether to fund specific treatments. **Aims** In this article we aim to illustrate two factors that make the measurement of societal value in this area particularly difficult: the low level of public awareness of, and engagement with, the orphan-drug issue, and the ‘zero-

sum’ framing commonly used to describe the policy challenge posed by orphan drugs.

Method We illustrate these challenges using data from an original survey of 2,005 Canadian adults. Respondents completed two tasks in which they were asked to choose between funding the treatment of patients suffering from either rare or common diseases.

Results Respondents were more likely to display choice aversion and unstable preferences if they had not completed a university degree and when a ‘zero-sum’ frame was used to introduce the choice sets.

Conclusions The results suggest that studies in which the stated opportunity costs of funding orphan drugs focus exclusively on reductions in funding for other drugs or treatments may only provide a limited understanding of citizens’ policy preferences in the area of rare diseases.

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Key Points for Decision Makers

Measuring the societal value of treating rare diseases is challenging.

It appears that citizens may have unstable preferences for orphan-drug funding.

Current estimates of the societal value of treating rare diseases are insufficiently robust to be incorporated into orphan-drug policy frameworks.

1 Introduction

Orphan drugs for rare diseases are often very expensive, and this creates a moral dilemma for public health

insurance programs [1]. Coverage decision makers must weigh up the opportunity costs of treating rare-disease patients (e.g. funding less costly medications for many more people suffering from common diseases [1]) against concerns that not providing access to treatment due to cost where there is an unmet need is unfair or unethical [2]. In practice, many decision-making bodies cover orphan drugs even though they rarely meet the usual thresholds for cost effectiveness used in traditional health technology assessment (HTA) [3]. However, since these are inherently value-laden decisions that have distributive consequences, there is growing acceptance of the democratic deficit created by delegating these types of decisions exclusively to expert bodies [4, 5] and of the consequent need to integrate societal preferences into HTA and reimbursement policies as a means of increasing the democratic legitimacy of policies in this area [6]. Researchers have devoted particular attention to determining whether citizens value the rarity of a disease, independent of other factors, when forming resource allocation preferences [7–10]. If this were the case, it would be an important justification for giving ‘special’ status to orphan drugs in HTA [11].

Nevertheless, a consistent finding in this literature is that when the only differentiating feature of a drug coverage decision is the rarity of the disease, Western publics do not appear to support prioritization of treatments for rare diseases over treatments for common diseases [7–10]. However, this literature also suggests that most members of the public are (1) not familiar with and do not have pre-existing preferences for the prioritization of orphan-drug funding; and (2) reluctant to engage with scenarios in which the funding of treatments for rare diseases must result in the reduction of care for those suffering from common diseases. These two factors create a significant barrier to using existing evidence about the societal value of treating rare diseases to inform orphan-drug funding policies. Specifically, low familiarity with the issue and use of the ‘zero-sum’ frame commonly used to describe the policy challenge posed by expensive orphan drugs both lead to choice avoidance and unstable citizen preferences. This makes it unlikely that the elicited preferences are reflective of what they would be following a full public debate on the topic, and suggests that the current evidence regarding citizen preferences for orphan-drug funding policies does not provide a solid basis for policy makers seeking to use such attempts at public consultation as a tool of participatory governance in health policy. In this article, we use data from an original survey of Canadian adults to illustrate how these two factors influence the preference formation process.

2 Challenges in Eliciting Preferences

2.1 Unstable Preferences

Standard approaches to preference elicitation in economics assume that survey respondents possess a stable and well-ordered set of preferences. However, there is evidence that when individuals evaluate choice alternatives with which they do not have prior experience, they construct their preferences during the elicitation process itself, leading to unstable preferences [12, 13]. The sensitivity of stated preferences for orphan-drug funding to contextual ‘framing’ effects [8] suggests that the assumption of stable preferences in this context is not appropriate.

Instability in preferences for low-salience issues can be best understood using a social psychological model of attitudes, in which attitudes are, in most cases, formed as part of the survey response process [14] and are conceptualized as “summary evaluations based on a weighted average of a sample of beliefs about the attitude object” [15]. From this perspective, a respondent’s attitude towards orphan-drug funding would be a weighted average of the set of relevant considerations (e.g. “patients have a right to receive medically necessary care regardless of cost”) that he or she is able to retrieve from memory when asked to evaluate a scenario option. This way of thinking about orphan-drug preferences has two important implications for our argument.

First, it provides a theoretical basis for understanding how contextual features of a questionnaire can influence respondents’ attitude formation process. For example, ‘priming’ [16] occurs when a contextual feature increases the accessibility of a specific consideration for a particular respondent and therefore its likelihood of appearing in the set of considerations that the respondent uses to generate his or her evaluation of the scenario options. Similarly, ‘framing’ is a process in which peripheral elements of a decision context influence the weight associated with a consideration by changing its perceived ‘relevance’ [17] or ‘applicability’ [18]. This type of contextual effect can lead to apparent preference instability across studies because priming and framing effects will differ across questionnaires depending on, for example, what questions have been asked prior to the preference elicitation item, or on the exact wording used to describe the choice scenarios.

Second, the social psychological model of attitudes provides an explanation for why preference instability is likely to be greatest when respondents’ familiarity with orphan-drug policy is low. If attitudes are conceptualized as a weighted average of considerations in memory (i.e. of accessible considerations), then a stable and ordered set of

preferences will only emerge if respondents are able to access a large number of considerations from memory, since averaging over many considerations limits the influence that a priming effect on any one consideration can have on the overall evaluation. This is why low public awareness of, and engagement with, the orphan-drugs issue—which should lead the typical respondent to retrieve fewer considerations and be more susceptible to ‘priming’ effects [19, 20] and more reliant on judgmental heuristics [21, 22]—is likely to lead to instability in citizen attitudes, and may help to explain several features of Western publics’ stated preferences for orphan-drug funding.

The two surveys by Desser et al. of the 40- to 67-year-old Norwegian public [7, 8], for example, found that the vast majority of respondents (65 % [7] and 75 % [8]) expressed indifference in scenarios in which the two choices differed only with respect to the frequency of the disease to be funded. In addition, when respondents were asked to divide a fixed amount of funds between the rare- and common-disease patients, a majority (65 % [7] and 62 % [8]) chose to divide the funds equally between the two groups. One explanation for this trend is that because only a minority of respondents are likely to be well informed about the orphan-drugs issue, in most cases they will not incorporate considerations specifically related to rare diseases when evaluating the choices in such scenarios. Instead, they will resort to general considerations relating to healthcare and drug funding, which are likely to overlap substantially with the set of considerations used to evaluate the common disease treatment option (although these are more likely to include considerations relating to specific common diseases such as breast cancer or diabetes). In sum, most respondents will be using highly overlapping consideration sets to evaluate the two scenario alternatives and therefore it is not surprising that many will not be able to differentiate between the two choices, leading to a high degree of indifference and an equal division of resources. Moreover, most respondents will likely have little motivation to devote cognitive effort to evaluating complex resource allocation scenarios and are liable to resort to judgmental shortcuts such as ‘midpoint’ or ‘central tendency’ bias [7, 8].

2.2 Rejection of the ‘Zero-Sum’ Frame

A second obstacle to measuring public preferences for orphan-drug funding is that citizens have been reluctant to accept the ‘zero-sum’ frame used in the policy literature, in which the decision to fund the treatment of a given number of rare-disease patients with expensive orphan drugs is portrayed as requiring that a larger number of common-disease patients will lose access to treatment or go untreated [1]. This frame puts widely-held preferences for

the efficient use of resources and for equity in people’s access to healthcare in conflict, and citizens’ discomfort with making this type of ‘choice under conflict’ [23] is likely to be partly responsible for the high levels of indifference expressed by Norwegian respondents [7]. In particular, because individuals tend to look for new alternatives when faced with a choice under conflict, it may be that respondents refused to make a choice because they envisaged alternative courses of action that would not require depriving either group of treatment. Desser et al. [24], for example, reported that “approximately half of respondents’ (open-ended) comments from the general population survey suggested that the problem of whether to prioritize rarity was avoidable because Norway is wealthy enough to treat all patients”. Indeed, the broader literature on public attitudes towards healthcare spending has found that citizens tend to favor reallocating spending from other government programs to healthcare to avoid rationing [25–27].

In an orphan-drug context then, the dilemma faced by policy makers includes a choice (albeit a politically unpalatable one) of whether to fund treatment for both rare- and common-disease patients by reallocating funds from other programs and services or raising taxes. By focusing on the ‘zero-sum’ dilemma faced by coverage decision makers, and not including these options in surveys aimed at measuring the societal value of treating rare diseases, scholars risk informing the policy debate with estimates of societal preferences that do not take into account the full range of resource allocation options available to the polity as a whole, thus calling into question the very purpose of citizen participation, which is to increase the democratic legitimacy of decisions with distributive consequences [4, 5]. Moreover, the value conflict instigated by ‘zero-sum’ frames is likely to lead to increased rates of choice avoidance and preference instability since respondents will tend to seek out alternative options that are not included in the choice sets presented to them.

3 Methods

To illustrate the choice avoidance behavior and instability in policy preferences that results from low awareness and engagement with the orphan-drug issue and from the use of ‘zero-sum’ frames, we used data from an online survey that was administered to a national sample of English-speaking Canadians aged 19 years and over recruited by IPSOS Reid Canada in June of 2013. Ethics approval was obtained from the University of British Columbia’s Behavioural Research Ethics Board. Respondents completed a task adapted from Desser et al. [7, 8] in which participants were asked to choose whether they would fund treatment for a set of

patients suffering from a ‘very rare disease’ (defined as having 100 cases in Canada) or for patients suffering from a ‘more common disease’ (defined as having 10,000 cases in Canada; see Online Resource 1). Participants were randomized into two groups (Fig. 1) for which the choice scenarios were framed differently: an Extra Funds group that involved allocation of new funds received from the provincial Ministry of Health, or an Existing Funds group that was asked to allocate current dollars and to whom it was explicitly pointed out that treating one group of patients would involve withdrawing or withholding treatment from the other group. The Existing Funds condition typifies the ‘zero-sum’ framing that characterizes the orphan-drug policy debate, and was expected to trigger greater value conflict (and, by extension, choice avoidance) than the frame in the extra funds condition because the former highlights the fact that one group of patients will either lose treatment or not be treated, which conflicts with most citizens’ commitment to equity in access to healthcare.

For each experimental group, participants were asked to allocate funding in two scenarios. In an equal costs scenario, participants were asked to choose between funding a treatment for 100 patients suffering from a rare disease or 100 suffering from a common disease, with the equal

number of patients in each group implying that the cost of treatment was equivalent for the two diseases. They were also given the option of being indifferent, and were subsequently asked how they would divide the available funds between the two groups of patients. In an unequal costs scenario, the choice was between funding the treatment of 100 rare-disease patients and 400 common-disease patients, implying that the treatment for the rare disease was four times more costly.

In the second part of the study, respondents completed a choice task (the Trade-Off task) in which, over 13 scenarios, they were asked to choose between allocating provincial funding to either treating a rare disease or to another competing alternative including eight healthcare and five non-healthcare options (Table A1 in Online Resource 2). Two of the alternatives repeated the equal costs and unequal costs scenarios used in the first part of the study, with the only difference being that an ‘indifference’ response option was not offered for the Trade-Off tasks.

Analysis was limited to respondents who took a minimum of 5 min to complete the survey since it was reasoned that, given the complexity of the survey and time to complete in pilot testing, subjects would not be able to reliably complete it in less than 5 min. The percentage of respondents selecting each choice alternative was calculated for each scenario, and the statistical significance of the difference in proportions for corresponding choices in the extra funds and existing funds conditions was calculated using a Chi squared test for the equality of proportions (Table 1). Two variables were constructed to measure choice aversion among respondents. Indifference is a dummy variable indicating whether a respondent selected the ‘indifferent’ option in the equal costs or unequal costs scenarios instead of the rare- or common-disease patient group options. Equal Division is a dummy variable indicating whether respondents elected to divide the available funds equally between the common- and rare-disease groups in the equal costs or unequal costs scenarios. A third variable, Consistency, directly measures preference stability. It is a dummy variable that applies exclusively to the subset of respondents who made choices in the first choice task, and is coded as 1 for those respondents whose choice of rare- or common-disease funding was the same in the first version of the equal costs or unequal costs scenarios and the second version in the Trade-Off task, and 0 for those respondents whose choices were not consistent.

In order to explore the effect of respondents’ level of engagement and motivation with the task on choice aversion and preference stability, these three variables were regressed on a set of demographic indicators (age, sex, and whether the respondent had a friend or family member who suffered from a rare disease), as well as dummy variables

Fig. 1 Questionnaire design for the Canadian survey

Table 1 Respondent choices in equal costs and unequal costs scenarios

	All ages	40- to 67-year-olds	Norway ^a
Extra funds, equal cost			
Rare (SE)	29.3 ^b (1.4)	24.6 ^b (1.9)	10.2 (–)
Common (SE)	40.3 ^b (1.5)	40.8 ^b (2.2)	19.9 (–)
Indifferent (SE)	30.4 (1.4)	34.6 (2.2)	69.8 (–)
No. of respondents	1,031	488	1,289
Extra funds, unequal cost			
Rare (SE)	19.9 (1.2)	19.7 (1.8)	8.1 (–)
Common (SE)	56.4 ^b (1.5)	54.5 ^b (2.3)	42.3 (–)
Indifferent (SE)	23.8 ^b (1.3)	25.8 ^b (2.0)	49.7 (–)
No. of respondents	1,031	488	1,289
Existing funds, equal cost			
Rare (SE)	34.9 ^b (1.5)	32.8 ^b (2.2)	15.9 (–)
Common (SE)	34.7 ^b (1.5)	34.1 ^b (2.2)	43.4 (–)
Indifferent (SE)	30.4 (1.5)	33.0 (2.2)	40.7 (–)
No. of respondents	974	460	258
Existing funds, unequal cost			
Rare (SE)	23.2 (1.4)	21.7 (1.9)	3.9 (–)
Common (SE)	48.3 ^b (1.6)	46.3 ^b (2.3)	60.5 (–)
Indifferent (SE)	28.5 ^b (1.4)	32.0 ^b (2.2)	35.7 (–)
No. of respondents	974	460	258

With the exception of the number of respondents, all data are expressed as percentages

SE standard error

^a Results for Norway are drawn from a sample of 40- to 67-year-olds reported on by Desser et al. [7]. That study featured six extra funds scenarios and one existing (fixed) funds scenario. Figures for the extra funds scenarios are calculated based on the pooled data for the six scenarios

^b Difference between corresponding figures for extra and existing funds conditions is statistically significant ($p < 0.05$)

representing the framing condition (extra funds = 1; existing funds = 0) and whether respondents held a university degree, and a multiplicative interaction term between these two variables. Level of education has been shown to predict the likelihood of response-order effects [29] and is used in this study as a proxy measure of survey respondents' ability to engage in effortful cognitive processing of survey questions, which should be associated with a reduced reliance on heuristic processing and the ability to bring a greater number of considerations to mind when forming attitudes relevant to the choice tasks. Holding a university degree should therefore be associated with reduced Indifference and Equal Division, and increased Consistency as a main effect.

As noted above, the extra funds versus existing funds manipulation is used to manipulate the level of value conflict prompted by the framing of the choice scenarios. An interaction term is also included to account for the

possibility that level of education moderates the effect of the framing manipulation on choice aversion and preference stability. Research in communication studies has found that more knowledgeable and cognitively motivated individuals tend to be more susceptible to framing effects (although not to priming or the use of heuristics) because the considerations targeted by a frame (in this case, considerations relating to the need for equity in access to health care) are more likely to be available for retrieval from that individual's memory store [30, 31], and because such individuals are better able to detect the relevance of the frame to the consideration in question. We would therefore expect that the effect of the framing manipulation should be greater among those respondents who held a university degree.

Odds ratios were estimated for each of the multiple logistic regression models (Table 2), in which all predictor variables were included as covariates. To ease interpretation of the results (given that the statistical significance of the overall effects of interacted variables cannot be directly inferred from the significance of the interaction term [32, 33]), the overall influence of the framing manipulation and the level of education were estimated by means of a simulation conducted using the Zelig package for R [34, 35], with all other variables set at their means. First-difference estimates and associated 95 % confidence intervals are reported for each of the models (Figs. 2, 3).

4 Results

The survey was completed by 2,211 respondents, of which 2,005 took at least 5 min to do so. The distributions of demographic characteristics in this subsample (Table A2 in Online Resource 2) were consistent with the general adult population in Canada. Overall, 47.4 % of included respondents (compared with 39 % of Canadians [36]) lived in Ontario, 32.1 % held a university degree (vs. 36 % of Canadians [37]), 46.8 % were male (vs. 49.5 % of Canadians [38]), and the median age was 41 years (it falls in the 45- to 49-year range for the over-19 Canadian population [38]). While respondents to this survey clearly did not constitute a random sample of the Canadian population (particularly given the omission of francophone respondents), the sample did include all other major demographic groups in Canadian society, and was generally reflective of the population's demographic structure and educational attainment.

Overall, a larger proportion of respondents preferred to fund the common-disease patients (the gap was greatest in the unequal costs scenario), and between 23.8 and 30.4 % of respondents expressed indifference (Table 1). The general preference for the common-disease option was

Table 2 Drivers of choice avoidance and preference instability

Outcome variables	Indifference (equal costs)	Equal division (equal costs)	Consistency (equal costs)	Indifference (unequal costs)	Equal division (unequal costs)	Consistency (unequal costs)
Age	0.44	1.00	1.00	1.00	1.01*	1.00
Female	1.00	1.49**	0.99	1.02	1.40**	1.01
Familiarity with rare disease	0.68**	0.71**	0.85	0.79	0.91	0.87
University graduate	0.88	0.83	1.09	0.93	0.88	1.43 ⁺
Extra funds condition	1.04	0.82 ⁺	1.37*	0.91	0.93	1.90**
University graduate × extra funds	0.86	0.71 ⁺	1.06	0.55**	1.00	1.00
Intercept	0.44**	0.82	1.57*	0.36**	0.33**	1.87**
N	2,005	2,005	1,396	2,005	2,005	1,482

Results are expressed as odds ratios estimated using multiple logistic regression

⁺ $p < 0.10$, * $p < 0.05$, ** $p < 0.01$

consistent with the pattern of results observed among Norwegian respondents [7], although the levels of indifference in that study were much greater than were found in the Canadian sample, with up to 70 % of Norwegian respondents selecting the ‘indifferent’ option. Between 32 and 48 % of Canadian respondents chose to allocate funds equally between the common- and rare-disease options. For the two Trade-Off task items, which did not include an indifference option, approximately 60 % of respondents in the equal costs scenario chose the rare disease option (61.2 % in the existing funds and 58.8 % in the extra funds conditions), whereas only about 30 % of respondents did so in the unequal costs scenario (27.4 % in the extra funds condition and 33.4 % in the existing funds condition). Of those respondents who did not choose the indifference option in the first choice task, 71 % (extra funds) and 64 % (existing funds) chose the same option in the equal costs Trade-Off task, whereas 82 % (extra funds) and 71 % (existing funds) were consistent in the unequal costs task.

Respondents in the existing funds condition were more likely to express indifference (unequal costs scenario), more likely to support the equal allocation of resources between the rare and common disease options (equal costs scenario), and less likely to be consistent in their responses across choice tasks (both scenarios) than respondents assigned to the extra funds condition (Figs. 2, 3). Notably, the effect of the extra funds frame on Indifference and Equal Division was large and statistically significant for those who held a university degree, but marginally significant at best among respondents without a degree. Similarly, while respondents who held a university degree were less likely to divide funds equally (equal costs scenario) and express indifference (unequal costs scenario) than those without a university degree, these differences were only observed in the extra funds group. In contrast,

university education was only associated with increased consistency in the unequal costs scenario, but this association was of similar magnitude for both framing conditions.

5 Discussion

While the level of indifference expressed by this Canadian sample was not as high as that observed for a Norwegian sample [7], it was still substantial, with between one-quarter and one-third of respondents expressing indifference in the different choice tasks. The differences between the Norwegian and Canadian samples could reflect differences in how salient the orphan-drug reimbursement issue is in the two countries, as well as differences in the values held by the two populations, or could be the result of differences in the study designs (e.g. the Canadian survey began with a values-ranking task that may have increased engagement among respondents and made it easier for them to form an attitude in the subsequent choice tasks; results from that task are reported on elsewhere).

The results of this survey illustrate how citizens’ low engagement with the orphan-drugs funding issue and the way in which it has been framed can lead them to avoid making a choice when faced with the moral dilemma posed by orphan-drug funding. While the relative influence of the extra funds versus existing funds framing manipulation on choice avoidance and preference stability varied across the different choice scenarios, all the effects observed were in the expected directions. Namely, choice avoidance and preference instability were greater in the existing funds condition, which is consistent with the idea that ‘zero-sum’ framing of the orphan-drug reimbursement dilemma triggers value conflict and choice avoidance. Moreover, the fact that this effect was strongest among respondents who

Fig. 2 Effect of extra funds frame and level of education on choice avoidance and preference stability (equal costs scenario). Bars indicate the difference in the predicted probability of the three dependent variables attributable to the extra funds frame and having a

university degree, along with the associated 95 % confidence intervals. Estimates are presented separately for individuals with high and low education (for the extra funds frame) and for the two framing conditions (for the effect of university degree)

Fig. 3 Effect of extra funds frame and level of education on choice avoidance and preference stability (unequal costs scenario). Bars indicate the difference in the predicted probability of the three dependent variables attributable to the extra funds frame and having a

university degree, along with the associated 95 % confidence intervals. Estimates are presented separately for individuals with high and low education (for the extra funds frame) and for the two framing conditions (for the effect of university degree)

held a university degree provides further evidence that the observed effect resulted from a framing effect, since framing effects have been shown to be strongest among individuals with higher levels of cognitive processing ability and motivation [30, 31].

Similarly, the observed differences between respondents who held a university degree and those who did not were all in the expected direction, with more educated respondents being more likely to make a choice and to hold consistent preferences. This is consistent with what would be predicted by the psychological model of attitudes, in which more educated respondents would be able to draw on a broader set of considerations when evaluating the two choice alternatives, making it easier for them to differentiate the two options (and therefore to make a choice), and making it more difficult for the overall weighted average of considerations (the evaluation) to change during the survey (since a random change to the weighting of any individual consideration will have a weaker influence on the overall evaluation when the consideration set is large than when it is small).

6 Conclusions

Our study illustrates two factors that make it difficult to draw firm conclusions from the literature on public preferences for the funding of orphan drugs. First, the relatively low salience of the orphan-drug issue among Western publics and the consequent low knowledge of and engagement with the issue among survey respondents is likely responsible for the high levels of choice avoidance observed in both this and other [7, 8] studies, and is associated with preference instability. Second, the ‘zero-sum’ framing of the orphan-drugs funding dilemma, which characterizes much of the orphan-drug policy literature, also leads to choice avoidance and response instability. Given these challenges, it would be unwise for policy makers to use existing measurements of the societal value of orphan drugs to inform orphan-drug policy, since they cannot be confident that they have captured the full range of the public’s preferences for orphan-drug policy.

If and when Canadians become more familiar with the orphan-drug issue, the first of these concerns is likely to ebb. In the meantime, efforts to measure societal preferences for orphan-drug policy should incorporate a substantial educational and deliberative component before attempting to elicit respondents’ preferences. That said, our findings suggest that if policy makers and scholars continue to focus exclusively on the zero-sum dilemma faced by reimbursement decision makers, they may be missing an important element of citizens’ preferences for the funding of orphan drugs, a deficiency that is likely to be

exacerbated as members of the public learn more about and become more engaged with the orphan-drug issue. This is problematic from a normative perspective because it undermines the fundamental rationale for consulting the public in the first place; that is, increasing the democratic legitimacy of resource allocation policies whose implementation is largely left up to unelected bodies [4]. Future work should therefore aim to incorporate a greater variety of opportunity costs and policy options into scenario designs (e.g. the possibility of funding all unmet need in the system while sacrificing funding for other public services). It should also allow citizens to weigh in on the design of the reimbursement decision-making institutions themselves, since allowing for a broader democratic ‘meta-deliberation’ on institutional design provides one way of increasing the democratic legitimacy of decisions that are delegated to ‘non-majoritarian’ deliberative bodies such as reimbursement decision-making boards [5].

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Nick Bansback, Craig Mitton, and Carlo A. Marra are members of the CIHR New Emerging Team for Rare Diseases. They were involved in the design of the survey and the revision of the draft manuscript. Dr. Marra is currently Dean and Professor at Memorial University’s School of Pharmacy.

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