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Can Severity Outweigh Smaller Numbers? A Deliberative Perspective from Canada

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ABSTRACT

Objectives: To use structured deliberation to elicit and describe the values of the public in Alberta, Canada, on the question of whether the severity of a rare condition can justify it being given priority in funding over common conditions affecting larger numbers of patients, and what aspects of a condition drive this judgment. **Methods:** Thematic analysis of transcripts of a group deliberative exercise carried out as part of two citizens' juries. The exercise was designed to elicit participants' conception of disease severity, and trade-offs between helping small groups with severe conditions and larger groups with less severe conditions. **Results:** In trading off severity and numbers, all groups were willing to choose a more severely ill but smaller group of patients over a less severely ill but larger group of patients, although how much of a severity differential was required varied between groups. Pain that could not be relieved by alternative means was the strongest motivator for choosing the smaller group. Other symptoms with no alternative means of relief were strong motivators as well. **Conclusions:** These findings indicate that, all else being equal, the public would support giving priority to a smaller but more severely ill group of patients over a larger group when prioritizing the needs of the few is life-saving, extends life enough to give hope of future improvement, and relieves otherwise intractable symptoms, especially pain.

Keywords: citizens' jury, deliberative methods, health policy, qualitative research, rare diseases, resource allocation.

Introduction

Rare diseases are usually genetic, often chronic and severe. Their rarity limits the potential size of the market for treatments. As a result, absent special incentives, anticipated returns on investment may be insufficient to stimulate development of treatments, and those that reach the market may have very high prices. To counteract these tendencies, many countries have successfully adopted policies to stimulate development of treatments for rare diseases, help these treatments reach the market, and promote access to them [1,2]. In the absence of such a policy, by 2012, 74% of the orphan drugs approved in the United States were available in the Canadian market. Canadian approvals often happened later [3], and provinces adopted informal mechanisms for providing access to these drugs [4]. In October 2012, the federal government started to develop an orphan drug regulatory framework.

Because drugs for rare diseases are rarely considered cost-effective by usual criteria [5], decisions to cover these drugs require departing from usual cost-effectiveness requirements, treating these diseases as priority cases for which society is willing to pay more than it would pay to treat common diseases. Studies find that the public is unwilling to pay more for the same health gains because of a disease's rarity [6–8], but supports prioritizing the more severely ill, to some extent: a person trade-off study in Norway finds no preference for severity, all else being equal [8]. A person trade-off study in the United States [9] finds preference for severity even at the expense of some aggregate gain, whereas one in the United Kingdom [10] finds no preference for rarity but preference for severity only if health gain is substantial. A deliberative study in the United Kingdom finds that the "rule of rescue" was one of three rationing principles chosen by deliberators [11]. Reviews of preference studies find a consistent preference for severity, although its strength varies [12,13]. A review of social value arguments also finds support for valuing severity but not rarity [14].

Orphan drugs treat diseases that are not only rare, but also severe. Because there is no preference for rarity but there is preference for severity, it becomes necessary to investigate whether severity can outweigh smaller numbers in public preferences for funding allocation, and, if so, what aspects of severity can do so. The exercise described here aims to elicit considered judgments from citizens of Alberta regarding this question, as well as the rationales for these judgments.

Methods

Citizens' juries are a method for engaging the public in structured deliberation. Deliberation allows citizens to articulate and justify

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their views in terms that should be acceptable to others, to be exposed to the views of others, and to broaden and revise their own views [15]. Research on the effects of citizens' juries shows that participation increases jurors' openness to changing their views, influences jurors' opinions, and has long-term effects on their level of information about the policy issue in question and positive effects on trust in health technology assessment processes [16–19].

Two citizens' juries were involved in this study. Each consisted of 16 members of the public who were compensated for their time to make participation accessible for all socioeconomic strata. To minimize selection bias due to inability to get time off of work, juries were held on a Friday evening, Saturday, and Sunday. The first jury took place from April 17 to 19, 2015, and the second from June 5 to 7, 2015, both in Edmonton, Alberta.

From a sampling frame provided by the postal service covering northern Alberta, 3000 potential participants were randomly selected and sent information packages about the juries. The 224 package recipients who manifested a willingness to participate were screened by telephone interviews through which researchers collected demographic information. Health care workers, current patients, individuals involved in advocacy, and others with a preexisting interest in the health care system were excluded from the pool of potential participants. From those remaining, a sample stratified by age, ethnicity, socioeconomic status, and occupation was drawn, with random selection within groups (Table 1).

Each jury started with the testimony of expert witnesses, whose role was to familiarize participants with issues relating to rare diseases, from various perspectives. Among the information provided to the group by the witnesses, decision makers from provincial bodies gave an overview of the system for approval and reimbursement of therapies in Alberta and of the current issues they were most concerned with at the time. A physician who treated patients with rare diseases gave general facts about rare disease patients in Alberta and the issues they face, and the options open to a clinician to obtain treatments that are not usually covered. A patient with rare disease and a mother and caregiver of such a patient both told their personal histories, which touched on some of the themes the clinician and decision makers had talked about. A representative of the pharmaceutical industry discussed the high costs and difficulties of doing research with a small number of patients, the need to apply to health authorities for coverage, and the influence of public payers on private manufacturers' investment decisions.

For the following day and a half, jurors were asked to complete a series of deliberative exercises.

For this exercise, each jury was divided into three groups, two with five members and one with six members. Each group was assisted by two researchers working as facilitators. First, the group was presented with a set of cards, each containing a health state profile described by five attributes, with each attribute having three possible levels (Table 2). Profiles described real

Table 1 – Sociodemographic profile of jury participants.

Characteristic	Jury 1	Jury 2	Alberta	Source of Alberta data
Sex				
Male	8 (50%)	8 (50%)	50%	2016 data [20]
Female	8 (50%)	8 (50%)	50%	
Age (y)				
18–24	2 (13%)	2 (13%)	6% (age 20–24 years)	2016 data [20]
25–34	2 (13%)	2 (13%)	16%	
35–44	3 (19%)	3 (19%)	14%	
45–54	3 (19%)	3 (19%)	14%	
55–64	2 (13%)	2 (13%)	12%	
65–74	2 (13%)	2 (13%)	7%	
>74	2 (13%)	2 (13%)	7%	
Education (highest level)				
Less than high school	1 (6%)	1 (6%)	11%	2016 data [20]
High school	7 (44%)	5 (31%)	25%	Population 25–64 years
Postsecondary diploma	4 (25%)	5 (31%)	36%	
Undergraduate degree	4 (25%)	3 (19%)	22%	
Graduate degree	0 (0%)	2 (13%)	6%	
Annual household income (Can$, before taxes)				
<25,000	3 (19%)	2 (13%)	9%	2016 data [20]
25,000–45,000	2 (13%)	4 (25%)	11%	Refers to income in the year 2015
46,000–70,000	5 (31%)	4 (25%)	16%	
71,000–100,000	3 (19%)	3 (19%)	17%	
>100,000	3 (19%)	3 (19%)	47%	
Employment status				
Employed	7 (44%)	9 (56%)	65% (employed)	2016 data [20]
Unemployed	4 (25%)	3 (19%)	6% (unemployed)	Population 15 years and older
Retired	5 (31%)	4 (25%)	28% (not in labor force)	
Ethnicity				
Asian	1 (6%)	1 (6%)	19%	2016 data [20]
White	14 (87%)	14 (87%)	89%	Total is >100% because multiple answers were allowed
First Nations (Aboriginal)	1 (6%)	1 (6%)	8%	
Other	0	0	4%	
Geographical location				
Urban	11 (69%)	13 (81%)	83%	2011 data [21]
Rural	5 (31%)	3 (19%)	17%	