Social Science & Medicine 94 (2013) 56–62

Contents lists available at SciVerse ScienceDirect

Social Science & Medicine

journal homepage: www.elsevier.com/locate/socscimed

Prioritizing treatment of rare diseases: A survey of preferences of Norwegian doctors

Arna S. Desser [a,b,*]

[a] Department of Health Management and Health Economics, University of Oslo, P.O. Box 1089, Blindern, 0373 Oslo, Norway
[b] Norwegian Knowledge Centre for the Health Services, P.O. Box 7004, St. Olavs plass, N-0130 Oslo, Norway

ARTICLE INFO

Article history:
Available online 26 June 2013

Keywords:
Norway
Rare diseases
Priority setting
Doctor preferences
Societal preferences
Orphan drugs

ABSTRACT

Understanding doctors' preferences for prioritizing treatment of rare diseases can provide an important context for policy makers who must decide whether to exempt rare disease treatments, which are often quite expensive, from standard cost-effectiveness criteria. We surveyed a random sample of 551 Norwegian doctors in November 2011 and compared results to a similar survey of the Norwegian population. Respondents chose whether to prioritize treatment of patients with rare versus common diseases and then decided how to allocate funds between the two groups for each of two scenarios: (1) equal costs per person and (2) higher costs for the rare disease. Respondents were randomized to treatment costs for the rare disease in the second scenario that were either 8 or 25 times higher than treating the common disease. Except for different prevalence, the diseases were described identically. Doctors displayed no general preference for prioritizing treatment of rare diseases, but a large number favored the principle of reserving a small share of funds for rare disease patients. Doctors' responses differed significantly from those of the general population when the rare disease was more costly to treat. A larger share of doctors prioritized the common disease group for treatment while a smaller share expressed indifference. When dividing funds between the two patient groups, doctors allocated a smaller share of funds to the rare disease. Doctors were much less likely than the general population to divide funds equally between the groups. This study indicates that there is little support among Norwegian doctors for prioritizing the treatment of rare diseases.

Introduction

Heightened awareness of the potential demands that medications for extremely rare diseases (orphan drugs) place on health resources has spurred efforts to gain a better understanding of preferences about how such drugs should be prioritized in health care systems. Incentives provided by the U.S. Orphan Drug Act (1983) and the European Commission Regulation on orphan medicinal products (2000) have succeeded in increasing the number of medications available for orphan diseases. However, many of these treatments are so expensive that they fail to meet standard cost-effectiveness criteria for public reimbursement, leading to concerns that patients with rare diseases are disadvantaged by the system and suggestions that orphan drugs be exempt from standard cost-effectiveness criteria. The debate about whether to provide an orphan drug exemption (Drummond et al., 2007; Hughes, Tunnage & Yeo, 2005; McCabe, Claxton & Tsuchiya, 2005) may hinge on the existence of a societal preference for prioritizing rarity (McCabe, Tsuchiya, Claxton & Raftery, 2006).

To date, only a small body of evidence exists concerning societal preferences for rarity. The final report of qualitative discussions by a thirty-person Citizens Council from the National Institute for Clinical Excellence (NICE, 2004) indicates that few participants favored unconditional reimbursement of medications for rare diseases, although about half felt special reimbursement could be justified based on disease severity or documented treatment benefits. Broader based population surveys by Desser et al. (2010) and Mentzakis, Stefanowska & Hurley (2011) of Norwegians and Canadians, respectively, provide little evidence that a specific societal preference for prioritizing rare diseases exists. A follow-up study (Desser, Olsen & Grepperud, submitted for publication) suggests that evidence pointing to a more general societal preference for fairness in resource allocation may, in part, reflect respondents' reluctance to make difficult choices about which patient groups should be prioritized: respondents continued to express indifference between patient

* Norwegian Knowledge Centre for the Health Services, P.O. Box 7004 St Olavs plass, N-0130 Oslo, Norway. Tel.: +47 464 00 441.
E-mail addresses: ade@nokc.no, arna.desser@gmail.com.

0277-9536/$ – see front matter © 2013 Elsevier Ltd. All rights reserved.
http://dx.doi.org/10.1016/j.socscimed.2013.06.019

groups despite an increasing opportunity cost of treating rare disease patients.

Our primary goal in this paper is to examine Norwegian doctors' preferences for prioritizing rarity in the allocation of health resources. Expert opinions can provide an important context for determining public policy. In Norway, for example, doctors comprised a large part of two governmental committees charged with developing official guidelines for setting priorities in use of resources in the health sector (Lønning-I, 1987; Lønning-II, 1997).

A secondary goal is to compare doctors' preferences with those previously elicited from the general population (Desser et al., submitted for publication). The earlier results suggest that general population preferences for relatively equal distribution of resources despite large differences in cost might be indicative of a reluctance to make difficult, and potentially unpleasant, decisions. Doctors are likely to have significantly more experience than the general population in making difficult choices, both because of their clinical training and because of their increasing need to balance multiple roles, e.g. patient advocate vs. gatekeeper, so their preferences could provide insights into the extent to which preferences elicited from the general population could reflect a reluctance to decide.

The Norwegian Medical Association's Ethical Guidelines for Doctors (2002) specify that in their practice doctors shall take due account of societal resources and that unnecessary or excessively expensive treatments must be avoided. This is in accordance with national legislation that guarantees patients the right to specialist health services if "the patient can be expected to benefit from the health care and the costs are reasonable in relation to the effect of the measure" (HOD, 1999). The implications of a strict adherence to the guidelines, other things equal, would be a movement towards an allocation of resources that maximizes health gains. We might therefore expect doctors to be more likely than the general public to prioritize common diseases. There is evidence, however, that while Norwegian doctors appreciate the need for gate-keeping, many find it difficult to make rationing decision in the context of their *duty of care* to individual patients (Carlsen & Norheim, 2005). Such concerns could limit the degree to which doctors would choose to allocate resources to maximize health benefits. We formulated the following research questions to examine these issues:

1. Do doctors have a general preference for prioritizing rarity?
2. When the cost of treating a rare disease is significantly higher than that of an equally severe common disease, how do doctors' preferences compare to those of the general population with regard to
   a. the share resources allocated to common disease patients?
   b. the proportion of doctors who support an equal division of resources between rare and common disease patient groups?
   c. the proportion of doctors favoring, in principle, a resource allocation that maximizes the number of patients treated?

Methods

Survey design

To facilitate comparisons with the general population survey results, this survey follows a similar design. The survey introduction explained that we wanted to gain doctors' opinions about how to prioritize use of new medications for rare diseases given that under a constrained health budget an increase in resource use among some patients necessarily means reductions elsewhere. We stressed that there are no "right" answers to these questions, but that a better understanding of preferences was important in policy-making. The survey consisted of five questions.

The first two questions (Q1 and Q2) reflected an *equal-cost scenario*. Respondents were told to assume that some extra funds had been allocated to the health sector and could be used to treat 100 hundred patients from one of two disease groups. The diseases were described, based on the EQ-5D descriptive system (The EuroQol Group, 1990), as equally serious (patients had difficulty moving about normally and were in extreme pain) and equally responsive to treatment (treated patients would be able to move about normally and be pain-free). The only difference between the diseases was prevalence: One was described as rare (100 patients in Norway, population approximately 5 million) and the other as common (10,000 patients in Norway). In Q1 respondents were asked to prioritize either the rare or common disease patients for treatment or could indicate that they were indifferent between the two groups, while in Q2 the task was to divide resources between the two groups by choosing among 11 possible options (100 rare & 0 common disease patients, 90 rare & 10 common, …, 0 rare & 100 common). The opportunity cost of treating an extra rare disease patient in both Q1 and Q2 was one common disease patient, i.e. treatment costs are equal between the two groups (*equal-cost scenario*).

Next, respondents were told that in reality new medications for rare diseases are often much more costly than medications that are equally effective in treating common diseases of equal severity so that the opportunity cost of treating one rare disease patient could be many common disease patients. Question 3 (Q3) asked respondents to choose which of four allocation principles health authorities should use in deciding how to distribute resources between patient groups. These principles were: treat the largest number of patients, treat equal numbers of patients from each group, allocate most of the resources to the common disease patients but retain a small amount for rare disease patients, allocate most of the resources to the rare disease patients.

In questions 4 and 5 (Q4, Q5), which we refer to as the *costly-rare scenario*, respondents repeated the exercises of deciding how to divide resources between the two patient groups (as in Q2) and selecting one group to prioritize for treatment (as in Q1), but now the opportunity cost of treating a rare disease patient was either 8:1 (survey version 1) or 25:1 (survey version 2). This implies a maximum number of common disease patients who could be treated of 800 and 2500, respectively (*costly-rare scenario*). Finally, respondents provided demographic information and had the opportunity to make short, open comments about the survey.

Survey implementation

The Norwegian Medical Association (NMA), with a membership comprising 96% of Norwegian physicians, populated the two survey versions by sending email invitations to two groups of 1000 individual each, randomly selected from its membership roster. The invitation letter, sent on November 1, 2011, provided a basic description of the survey's purpose (priority setting in health care) and the internet address for accessing the relevant survey version. Invitees had six weeks to respond to the survey and received three email reminders. In total, 551 (26%) individuals completed the survey. The final number of respondents was approximately equal across the two versions, with 275 in Version 1 and 276 in Version 2. The NMA also took responsibility for implementing the survey via Questback, a provider of online survey services. The survey received approval from the Norwegian Social Science Data Service, which reviews ethical aspects of surveys that do not directly involve patients.