

Assessing Societal Value from a Stated Preference Discrete Choice Experiment on Decision Makers in Europe. Does it work for Rare Diseases?

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INTRODUCTION: The multicriteria decision analysis (MCDA) approach provides opportunities for evaluation of process effects and non-health outcomes additional to traditional QALY analysis. One MCDA technique, the discrete choice experiment (DCE) has received attention and is a technique for investigating individual preferences. A DCE survey was conducted, using an online questionnaire, in order to explore the preferences of decision makers over health-care scenarios in five different countries in Europe (Spain, United Kingdom, France, Germany and Italy).

METHODS: The DCE survey in this study was conducted replicating the methods used by an already published (1). *Attributes and levels:* A systematic review of the literature on distributive preferences informed the attribute selection (2). The aim of the literature review was to identify attributes for the design of a DCE for rare diseases, in order to develop and validate a framework to support decision-making (see Table 1). *Experimental design:* the design used an orthogonal approach, including 36 pairs of scenarios distributed into two blocks of 18 pairs each. Each scenario described a combination of attribute and levels. *Different scenarios:* respondents were asked to make a series of choices involving two alternative healthcare scenarios (pair comparisons). Figure 1 shows an example of a survey question. *Data collection:* We used an online questionnaire to conduct a DCE survey of decision-makers in five different European countries (England, France, Germany, Italy and Spain) in order to explore their preferences. *Data analysis:* As the data are binary choice data – ‘1’ represents the option being chosen, with ‘0’ where not chosen – the conditional logit model was used for modelling. A series of utility models were fitted. Means and standard errors were used for continuous variables and proportions for dichotomous variables. All statistical analyses were performed on STATA MP.

RESULTS: A total of 199 questionnaires (81% of all the surveys sent out) were completed by and collected from decision-makers. Of these, nine were excluded from the study due to the time employed in fulfilling. Therefore, the valid sample totalled 190 decision-makers, the largest sample coming from Spain: 17 England, 36 France, 26 Germany, 35 Italy and 76 Spain. The five countries logit model showed relative preference for some attributes over others. “The cost of the treatment”, “improvement in health”, “value for money” and “availability of other treatment” were the attributes receiving the greatest attention. However, “disease severity”, “beginning of life”, “waiting times” and “side effects” were important social values that could not be ignored (see table 2).

Table 1: Discrete choice experiment: attributes and levels

Attribute	Description (summary)	Levels
Severity of the disease	Refers to the pre-treatment health state of patients	Moderate Severe
Improvement in health	Refers to the benefits that the patient feels following treatment	Large Moderate Small Very small
Waiting times	Refers to the time a patient must wait for treatment.	Short Moderate Long
Availability of other treatments	Refers to the existence of alternative treatments for the same disease	Yes No
Side effects	Refers to the undesired effects caused by the treatment. Any medical treatment carries risks	Few Moderate Many
Value for money	Refers to how efficiently resources are used	Very good Fairly good Fairly poor Very poor
Beginning of life	Refers to the age patients are diagnosed with this disease, referring to situations in which the patients are younger than 10 years (children)	Yes No
Cost of treatment	Refers to the resources that must be mobilized to ensure the financing of the treatment	Zero Lower Moderate High

Table 2: Logit model coefficients

	Levels	Country				
		England	France	Germany	Italy	Spain
Importance of the disease	Severe disease	0.216	-0.057	-0.055	0.035	0.082
	Moderate	-0.626	0.294	0.153	0.264	0.047
Improvement in health	Small	-1.235	0.422	0.061	0.023	-0.081
	Very small	-2.804	-0.014	0.043	-0.344	0.012
Waiting times	Moderate	0.375	0.103	-0.206	0.158	-0.176
	Long	-0.259	-0.141	0.09	-0.123	-0.081
Availability of other treatment	No	0.766	0.347	0.044	0.06	-0.126
Side effects	Moderate	-0.419	-0.037	-0.039	-0.461	-0.009
	Many	-0.624	0.093	-0.032	0.261	0.043
Value for money	Fairly good	-0.799	-0.303	-0.034	0.053	-0.032
	Fairly poor	-0.663	0.269	0.08	0.161	0.023
Beginning of life	Very poor	-1.86	0.129	-0.117	0.115	-0.011
	No	-0.537	0.121	0.101	-0.048	-0.027
Cost of treatment ^a	Low	0.878	0.207	0.022	0.012	-0.027
	Moderate	0.018	-0.1	-0.023	-0.433	0.122
	High	-0.786	0.2	0.061	-0.029	0.045

(a) Measured by tax increase / copayments

DISCUSSION: The findings presented in this study provide evidence about how decision makers think that decision should be made in Spain, Germany, UK, France and Italy when considering which health technology scenarios are more appropriate to receive funding. The DCE approach is an instrument that allows to measure the preferences of decision makers about all kinds of health care interventions. DCE data can be used to consider the strength of preference over alternative scenarios in a priority-setting context. *Limitations:* This study has a number of limitations. The experimental design is not complex, with a small orthogonal design being used, and the results presented here are based on a simple analytical framework, using the conditional logistic model. The study is open to a certain level of criticism over the presentation and contextual approach adopted. However, the results are useful and indicative of what may be possible in future research of this type. Future research could address many of the limitations highlighted; for example, using interviewers and qualitative methods to investigate the interpretation of attributes and the considerations when respondents make their choices.

CONCLUSIONS: Our results suggest that a “beyond Cost-QALY” framework could be created by using the DCE method. Specifically, the results suggest that such a “new” framework would be potentially beneficial for rare diseases in terms of “fair play” in the decision-making process. The findings presented in this study provide insight about how decisions are made in different countries when considering orphan drugs. This study also provides valuable prior information that could inform future DCE designs in this area.

Figure 1: DCE on-line survey caption

REFERENCES: (1) Green C, Gerard K. Exploring the social value of health-care interventions: a stated preference discrete choice experiment. Health Econ. 2009;18: 951-76. (2) Trapero-Bertran M, Rodríguez-Martín B, López-Bastida J. XXXV Jornadas de Economía de la Salud. Which attributes should include a discrete choice experiment: a systematic literature review. Granada. Junio 2015.