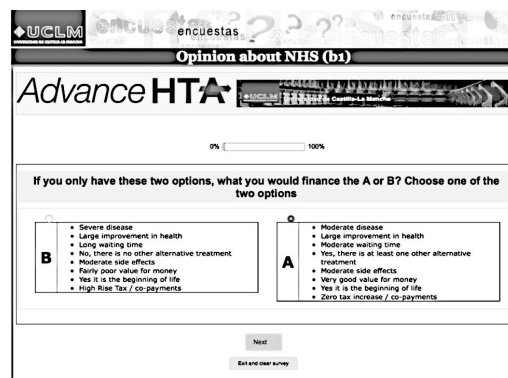


ASSESSING THE SOCIETAL VALUE IN RARE DISEASES: A STATED PREFERENCE DISCRETE CHOICE EXPERIMENT IN DECISION MAKERS

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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. **METHODS:** The DCE survey in this study was conducted replicating the methods used by an already published by Green and Gerard (2009). A systematic review of the empirical literature on distributive preferences informed the attribute selection. The literature review identified attributes for the design of a DCE for rare diseases, in order to develop and validate a framework to support decision-making. 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. 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. A decision makers 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). **RESULTS:** A total of 199 questionnaires (81% of all questionnaires sent out) were completed by and collected from decision makers. Of these, 9 were excluded from the study following the exclusion criteria. Therefore, the valid sample totalled 190 decision makers (36 France, 26 Germany, 35 Italy, 76 Spain and 17 United Kingdom). The five country models (each probit and logit), show preferences for some attributes over others. “Cost of the treatment” (Spain, France, England and Italy), “improvement in health” (Germany, France, England and Italy), “value for money” (England, Germany and France), “availability of other treatment” (England and France), “waiting times” (Germany, Spain and Italy), “side effects” (Italy) and “beginning of life” (Germany) are the attributes receiving greatest attention, while less important are “important of the disease” (France, England and Germany), “value for money” (Spain), “availability of other treatment” (Italy and Spain), “waiting times” (France and England) “beginning of life” (Italy) and “side effects” (France and Spain).



Criteria	Levels	Country				
		England	France	Germany	Italy	Spain
Severity of the disease	Severe disease	0.216	-0.057	-0.055	0.035	0.082
Improvement in health	Moderate	-0.626	0.294	0.153	0.264	0.047
	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
	Very poor	-1.86	0.129	-0.117	0.115	-0.011
Beginning of life	No	-0.537	0.121	0.101	-0.048	-0.027
Cost of treatment*	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

*(measured by tax increase / co-payments)

DISCUSSION: The findings presented in this document provide evidence about how decisions are made in different countries when considering which health technology scenarios are better than others, from the perspective of the decision-makers. The data obtained from the DCE survey was used to estimate the weights associated with each criteria by means of regression models. Logit and probit model coefficients have been tested. 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