Societal preferences for funding orphan drugs in the United Kingdom

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Introduction

This study aims to identify features of Orphan Drugs (OD) that may influence reimbursement decisions, and design a survey of the general population to evaluate their preferences for OD funding.

Previous studies have been conducted to explore the preferences of society; however there are few studies of whether society would endorse special funding for orphan drugs.

The only UK study was a cross-sectional study of 4,118 participants which found no evidence of a societal preference for treating rare diseases over common diseases (Linley and Hughes, 2013). While this study focused mainly on the rarity of the disease, there is a possibility that this is not the only characteristic of orphan drugs that society values (Linley and Hughes, 2013).

The increased numbers of orphan drugs combined with constrained health service budgets has led to a growth of interest and concern in the health technology appraisal of orphan drugs.

This study aims to examine society's preferences for funding these high cost drugs

Methods

Features the National Health Service (NHS) should consider important in making recommendations for funding OD were identified from using a multi-phased, mixed-method approach including; a quantitative ranking of features, and qualitative surveys of key stakeholder groups.

Identification of key attributes

Identification through the literature

Attribute survey

The survey of key stakeholders (clinicians, policy-makers, patients) will be used to determine attributes of greatest importance.

Focus Group

Are the attributes correct and relevant?	Determine the levels to be used in the survey.	Refining the language used

Discrete choice experiment (DCE) / National Survey

The national survey will elicit the preferences of society on the funding and resource allocation for orphan drugs and rare diseases.

Figure 1. Key aims and objectives of the multi-phase study design.

Attribute Survey

Results

A total of 114 participants were recruited into the study, consisting of 70 patients/carers, 27 policy makers and 17 healthcare professionals. Patients and carers were recruited from patient support groups, clinicians with a special interest in rare diseases via Orphanet, and UK policy makers from membership of health technology assessment organisations.

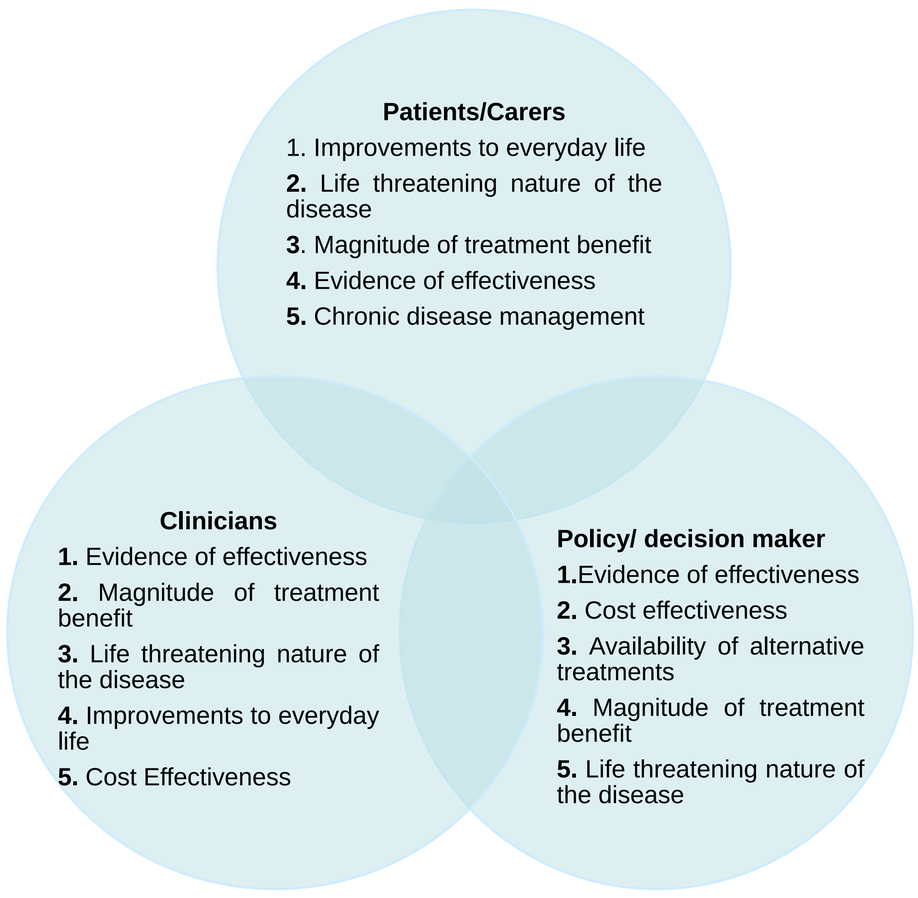

Figure 2. Results of the ranking exercise by participant group.

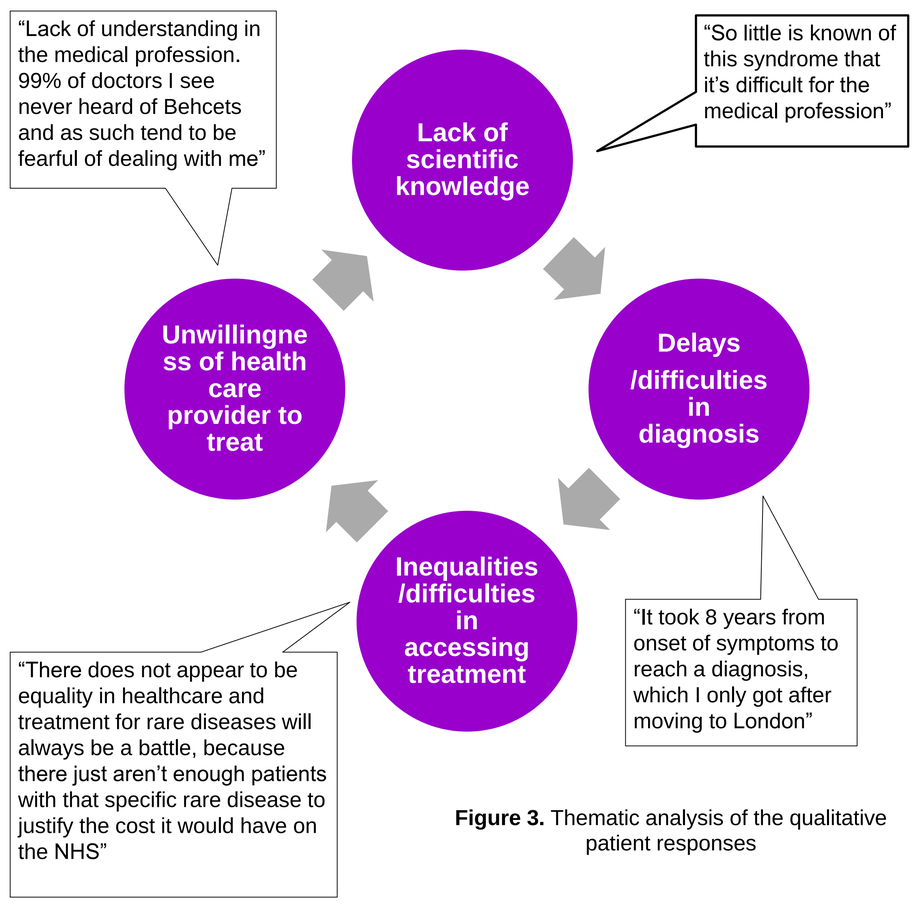

Figure 3. Thematic analysis of the qualitative patient responses

Individual group analysis found slight differences in the ranking of importance of these features between groups. Patients and carers was the only group to prioritise a feature that was not included in the other groups. Overall, however, there was little disagreement of which features were most important.

References:

LINLEY, W. G. & HUGHES, D. A. 2013. Societal views on NICE, cancer drugs fund and value-based pricing criteria for prioritising medicines: A cross-sectional survey of 4118 adults in Great Britain. *Health economics,* 22, 948-964.

RYAN, M. 1996. *Using consumer preferences in health care decision making: the application of conjoint analysis*, Office of Health Economics London.

Focus group – Results

Prior to the focus groups two decisions were made by the researchers:

i) Cost effectiveness would be replaced by cost per year or total cost. This decision was made to simplify the choice task for participants.

ii) Only 5 of the attributes brought to the focus group would be included in the national survey

Attribute	Definition	Level to be used
Debilitating and life threatening	The disease affects patients' everyday life OR the patient could die if they do not receive treatment.	Y- the disease is debilitating life threatening N- The disease is not debilitating and life threatening
Treatment benefit	The extent to which the drug increases survival	Increases survival by 1 year or MORE Increases survival by LESS than 1 year
Availability of other drug treatments	Other drug treatments are available to treat the disease	Y- a drug is available to treat the cause of the disease N- but the patients symptoms are treated
Improvements to everyday life	The drug improves the well-being of patients and their families e.g. school or work, social activities, and self care i.e. dressing oneself etc.	No improvements to everyday life Some improvements to everyday life Normal everyday life
Cost per patient per year	Cost of treatment on the NHS per patient per year	Levels for common: £1,000, £8,000 , £16,000 Levels for rare £15,000, £62,000 , £378,000

Table 1. Attributes/levels used in the DCE

Discrete Choice Experiment

A DCE is a utility-based preference elicitation technique in which individuals are presented with choices comprised of different hypothetical combinations of goods and services (choice sets) are asked to choose their preferred combination (scenario)(Ryan, 1996). A DCE allows for the trade-off between different attributes to be determined quantitatively, so that (for instance) respondents were willing for the NHS to pay more for the treatment of children with rare disease, and adults with a common disease.

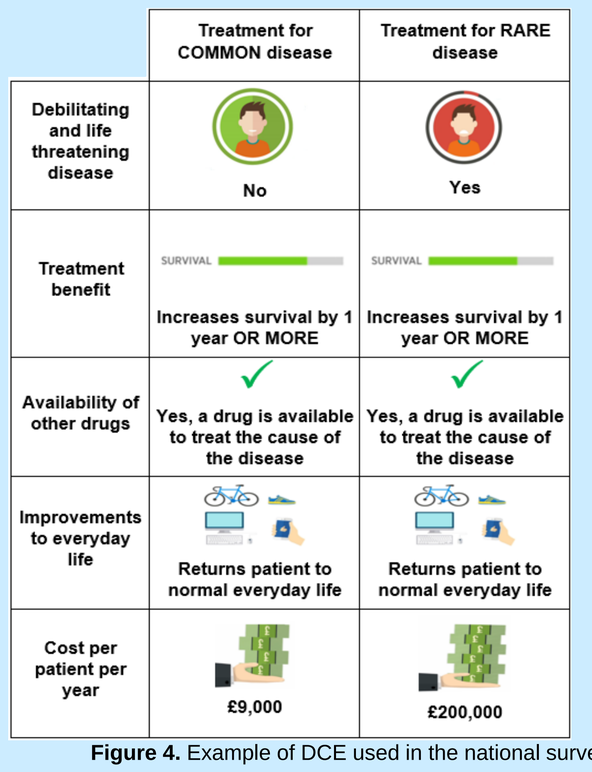

Figure 4. Example of DCE used in the national survey.

Implications

Our study is the first comprehensive attempt to gather public preferences for the funding of orphan drugs, in order to estimate the societal value of OD and to test whether there is support for the prioritisation of OD funding within the NHS.