Should Rarity Matter?

Results of a Social Willingness-to-Pay Study using the Discrete Choice Experiment (DCE) Method in Switzerland

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Research for a Life without Cancer

Background

Rarity (or lower prevalence) of a disease has been found to be associated with high(er) **treatment costs per patient**. Some orphan medicinal products (OMPs) rank among the most expensive drugs, which has led to concern with regard to the affordability of this success. Recent **technological advances** (e.g., "personalized medicine"), and the trend that more prevalent diseases may become collections of orphan diseases, raise the need to thoroughly assess the "value for money" offered by OMPs.

Conventional economic evaluation paradigms focus on cost benefit ratios at the level of patient (group). In the context of HTAs, health-related costs and benefits are often analyzed and reported as incremental cost effectiveness ratios (ICERs). It is broadly recognized that the **number of patients** (as a component of both numerator and denominator) cancels out in the calculation of ICERs. By design, a *focus on individual health gains only* (including their subsequent additive aggregation or averaging) will mask any impact of the **size of a program** (or, for that matter, the number of patients concerned) on its *social* valuation, since it implies a linearity assumption. Accordingly, studies designed to measure *selfish* consumer preferences for health gains did not reveal a role for "rarity," thus failing to support the case in favor of expensive technologies for orphan diseases.

It has, however, been argued that the majority of **stated preference or contingent valuation studies** in health care have been misspecified, because they did not capture significant elements of the value of the good in question. These are – beyond **use value** (the consumer perspective) – **option value** (uncertainty; risk averseness) and **externalities** (altruistic behaviors; caring externalities; the will of citizens to share resources with those in need).

In order to capture option value and externalities, the **payment perspective** needs to reflect the *social* willingness-to-pay (*s*WTP) extra compulsory health insurance fees, which implies a shift of the payment vehicle from cost per patient to cost per citizen, i.e., incremental budget impact of a new program divided by the size of the population covered by a health scheme. Against this background, we designed the European Social Preference Measurement (ESPM) project. Here we report key results of the Swiss pilot ("SoPHI," *Social Preferences for Health Care Interventions*) study with respect to the role of rarity.

Objectives

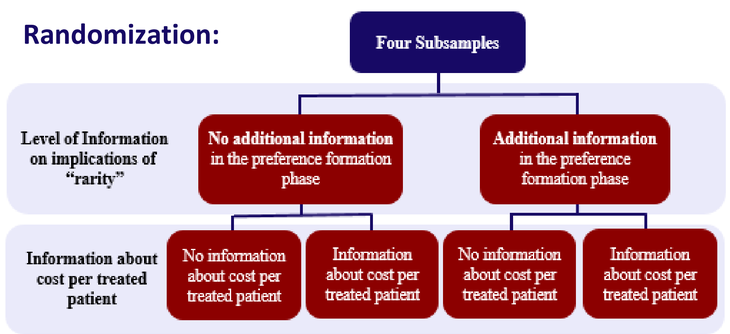
- To investigate how **Swiss citizens** valuate selected attributes of health care interventions, with special emphasis on rarity, from a social willingness-to-pay perspective – i.e., using incremental compulsory health insurance (OKP) premiums resulting from the addition of a new treatment as the cost attribute.
- To assess the **sensitivity** of valuation to the level of information and reflection (on the implications of rarity) offered to respondents and to the impact of information about the implied cost per patient.
- To assess the **feasibility** of a relatively complex discrete choice experiment (DCE) design with a view towards subsequent study extensions with the objective to identify **international similarities and differences** with regard to the valuation of the attributes tested.

Methods in Brief

Key Design Elements

- Extensive cognitive (qualitative) and quantitative pre-tests
- Main survey including representative Swiss population sample (1,501 respondents in 2017)
- Perspective on costs capturing risk aversion and wish to share health care resources
  - costing from a citizen's perspective, i.e., WTP_{public} as payment vehicle
- Testing for framing effects – by way of randomization into subgroups
  - by reflection on implications of rarity (during "preference formation phase"), and
  - by information on cost per patient implied by choice alternatives

Randomization:



Survey Design

1. Initial Preference Formation Phase (PFP)

- 25 open questions to stimulate reflection by respondents
- Random assignment to 3 specific questions related rarity

2. Discrete Choice Experiment (DCE)

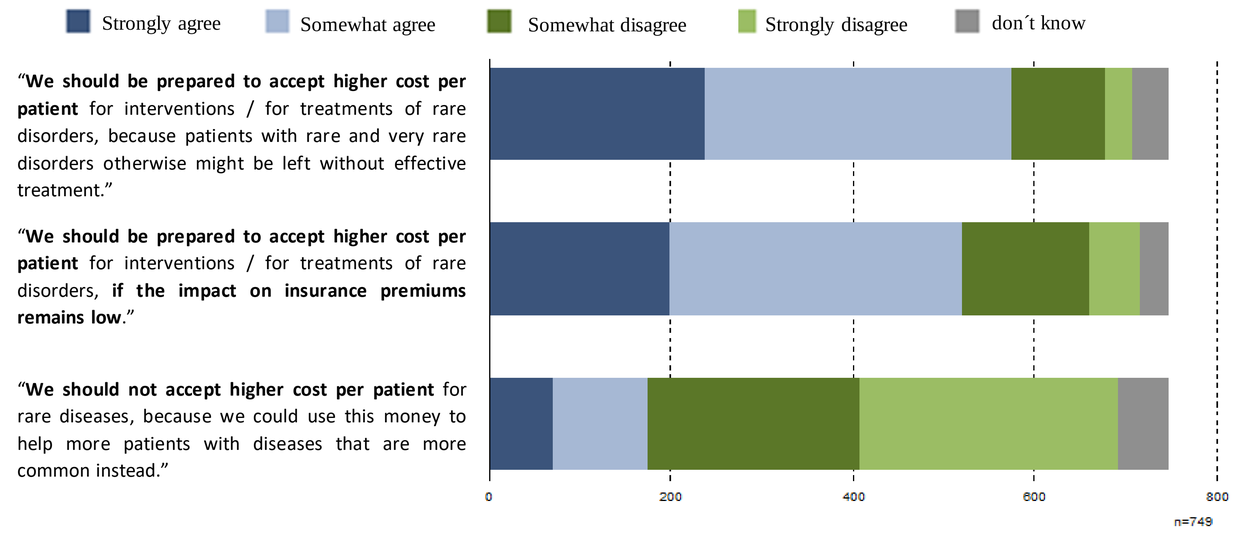
- Decision cards with or without information on cost per patient (random assignment)
- D efficient design; econometric analysis,
  linear conditional logit as base model; testing for interactions and nonlinearities of attributes;
  analyzing subsamples; preference heterogeneity,
  random coefficient and latent class models
- Attributes tested in Swiss study:
  1. **Effectiveness** of new intervention: life expectancy gained
  2. **Effectiveness** of new intervention: health-related quality of life gained
  3. **Age** of patients (or "fair innings")
  4. **Rarity** of disorder (i.e., prevalence)
  5. **Incremental Cost** of new intervention

3. General Questionnaire

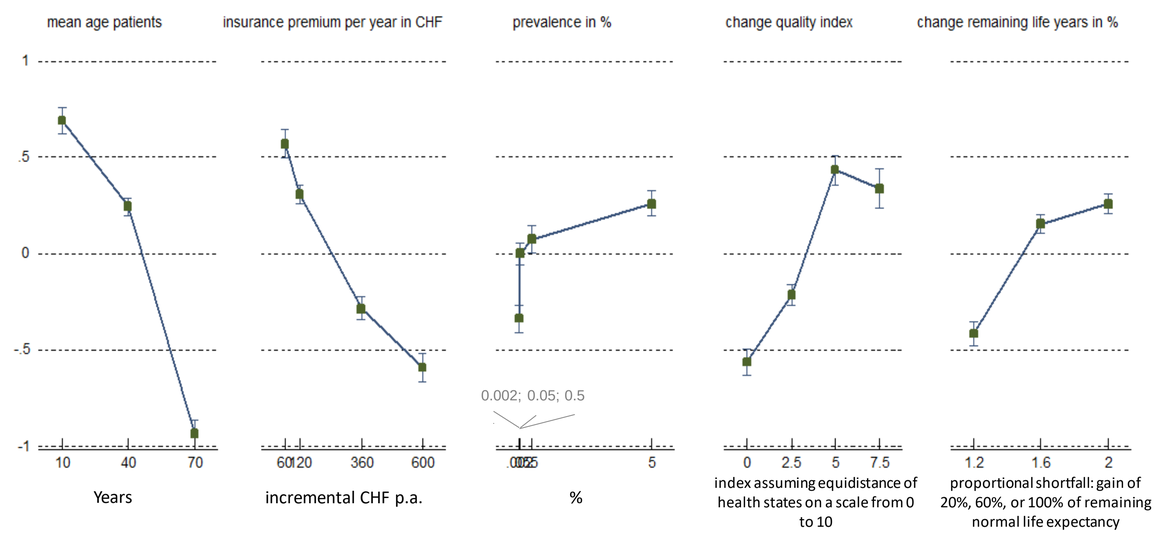
- Health state and health insurance of respondents
- Socioeconomic information and specific feedback

Key Results

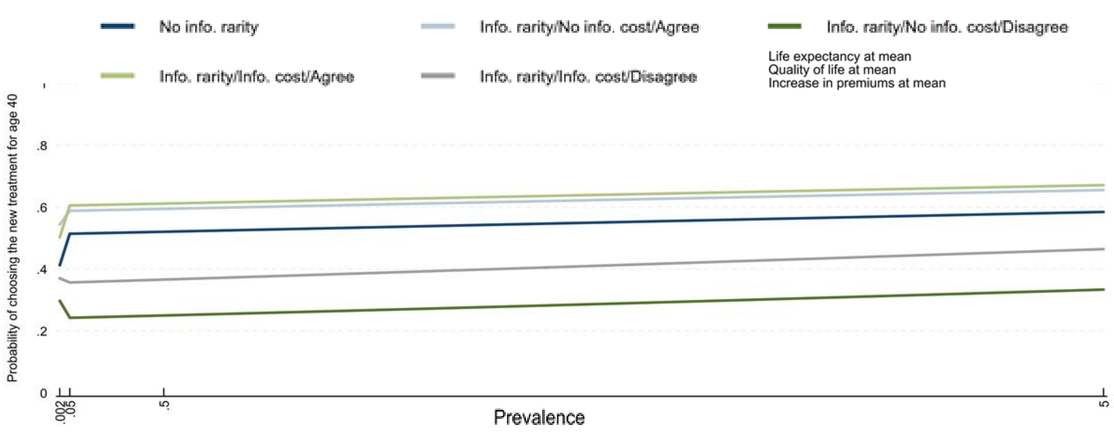
Preference Formation Phase: Stated Attitudes towards Rarity (Subsample, n = 749)



Flexible Specification of Attributes with Dummy Variables: Functional Form



Discrete Choice Experiment Results (Main Model): The Rarity Attribute



Implications

- **All attributes** investigated in the Swiss pilot study had an impact on choice probability and citizens' (*social*) willingness-to-pay.
- The variables with the highest impact on choice probability were change in remaining **life years**, **quality of life**, and **extra insurance premium** per year.
- **The small observed impact of prevalence translates into a profoundly in-creasing implied (*social*) willingness-to-pay per patient (and per life year gained) with decreasing prevalence (or increasing "rarity").**
- The results pass tests of internal consistency, rationality, and theoretical validity.
- The observed impact of rarity on implied (or *social*) willingness-to-pay per patient (and per life year gained, with or without quality adjustment) needs to be confirmed considering numerous questions, including but not limited to (a) deontological preferences ("Should the numbers count?"), (b) a potential role of an availability heuristics in the subgroup randomized to more reflection on rarity, (c) respondents' comprehension of the math behind the numbers, (d) any impact of "insensitivity of WTP to size of the good," (e) the international consistency of observed social preferences.