Attributes of Health Care Interventions

that drive Social Willingness-To-Pay (S-WTP): Focus on “Rarity”

Insights from a Discrete Choice Experiment (DCE) in Switzerland

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Starting Points

SwissHTA: Multi-Stakeholder Consensus on HTA

Drivers of Social Value (beyond individual health gain[1])

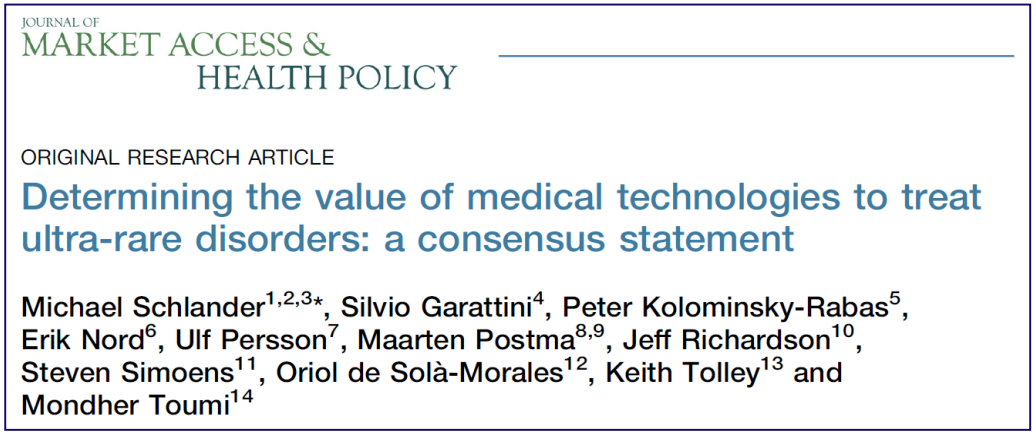
- **Severity and Urgency**
  of initial health problem
- **"*Fair Innings*"**
  interventions for children and young people who have not had an opportunity to pursue their individual life plans (a decent minimum of health as a *"conditional good"*)
- **Nondiscrimination or Fairness**
  fair chance of access to effective health care
  even if condition is rare or intervention is expensive
- **"*Bagatellen*"**
  exclusion of or low priority for minor self-limiting health problems
  and 'affordable' interventions[2]
- **Fast Access to Real Innovation**[3]

[1]Hypotheses, based on literature review and expert consensus; SwissHTA identified a major **research need**;
[2]'affordability' determined from a patient's out-of-pocket perspective; [3]'innovation' to be defined appropriately

Starting Points

How to Evaluate Interventions for URDs?

- **Agreement on Key Challenges (2012)**
- **Agreement on Way Forward (2014/2016)**



- specific challenges that arise when applying conventional HTA methodologies to the evaluation of rare and ultra-rare disorders (URDs) / orphan products
- promising ways forward (notably, MCDA and social cost value analysis), overcoming the loopholes of currently prevailing evaluation paradigms
- need for more empirical research into “social preferences” – notably wrt “rarity”
- development of **European Social Preference Measurement (ESPM)** project

Background

Social Preferences in the Economic Literature

“The taste
for improving the health of others
appears to be stronger
than for improving other aspects
of their welfare.”[1]

[1]Kenneth Arrow (1921-2017)
Uncertainty and the Welfare Economics of Medical Care (1963; p. 954)

Background

Valuation of Health: A Framing Issue?

1. **Use value** (consumer perspective)
2. **Option value** (due to uncertainty and risk averse citizens)
3. **Externalities** (caring externalities and altruistic behaviors)

Perspective on incremental costs and WTP:

1. direct out-of-pocket payments
2. private (voluntary) health insurance premiums
3. public (compulsory) health insurance premiums (or tax)

$$WTP_{direct\_oop} \leq WTP_{private\_ins} \leq WTP_{public\_tax}$$

¬ But – can we expect this additive relationship[1] to be (always) true?

[1] cf. D. Gyrd-Hansen (2013)

Background

Economic Literature: Preferences for Health

Are (Many) Stated Preference Studies Misspecified?

- Restricted to individual “use value”
  (health state, duration, probability)?
- Comparators and cost attribute included?
- “Given that CV studies in health care are overwhelmingly constructed to elicit use-value alone, the question that arises therefore is whether CV studies in health are misspecified.
- Empirical research suggests that […] most CV studies in health care may indeed be misspecified, as a significant element of the value of the good in question is not being captured (Smith, 2007).”[1]

[1]cf. R.D. Smith, T.C. Sach, *Health Economics, Policy and Law* 2010; 5: 91-111

Background

A Rapidly Growing Economic Literature …

… on a broad range of characteristics[1] contributing to **Social Value Judgments,** *including*

- **Attributes of the Health Condition**
  - individual valuation of health conditions
  - severity of the condition
  - urgency of an intervention
  - unmet medical need
  - capacity to benefit from an intervention (to lesser extent than assumed in CEA)
- **Attributes of the Persons Afflicted**
  - non-discrimination (and claims-based approaches)
  - age (and fair innings)
  - other patient attributes
  - fairness objectives; aversion against *all-or-nothing* decisions

[1]cf., for a review, see M. Schlander, S. Garattini, S. Holm, et al., *Journal of Comparative Effectiveness Research* 2014; 3 (4): 399-422.

Objectives

Social Preferences: Research Need

- **Limitations of the literature**
  - many studies limited in size and / or scope
  - many studies likely to be impaired by framing effects
  - sometimes questionable methodology (not choice-based)
  - zero sum assumption in many studies
  - *ex ante* severity probably best documented attribute
    – but distinct difficulties to quantify impact
  - role of prevalence (“rarity”) controversial

- **Cost attribute** (i.e., payment vehicle in most studies)
  - typically reflecting an individual (selfish) health state valuation (or “out-of-pocket” willingness-to-pay) perspective
  - *whereas citizens’ social willingness-to-pay for coverage of health care programs under a collectively financed health scheme would appear more relevant to health care policy makers in the context of Health Technology Assessments (HTAs)*

Objectives

Objectives of Project

Social Preferences for Health Care Interventions (SoPHI) Study

Phase I
Pilot Study
[Switzerland]

- To investigate how Swiss citizens valuate selected characteristics (“attributes”) of health care interventions, with special emphasis on the implications of rarity.
- To assess the sensitivity of weights to the level of information offered to respondents and to potential framing effects.
- To assess feasibility of comparing the valuation results obtained in the study with those based on the logic of cost effectiveness by means of a utility comparator.

Phase II
Main Study
[Europe]

- To systematically assess how the general public in key European jurisdictions valuate selected attributes of health care interventions, and how they weigh them against each other, including an assessment of potential intercactions.
- To identify international similarities and differences with regard to the valuation of the attributes tested.
- To explore the agreement of respondents between their choices in the experimental setting, their policy implications, and their policy preferences.

Methods

Key Design Elements

1. Representative population sample(s)
2. Phase I (Swiss pilot study): online survey with 1,501 respondents in 2017
3. Discrete Choice Experiment (DCE) design
4. 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
5. 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
6. Utility comparator
   - ¬ generic health state vignettes, descriptions derived from three dimensions of EQ-5D-5L
7. Survey including an initial “preference formation phase”
   - ¬ reflection phase for respondents, in order to obtain informed and stable preferences
8. Testing for potential cognitive overload
   - ¬ extensive pre-tests (qualitative, quantitative); learnings from phase I (Swiss pilot study)
   - ¬ partial profiles, random design strategy; tests for internal consistency and theoretical validity
9. Econometric evaluation
   - ¬ linear conditional logit as base model; testing for interactions and non-linearities of attributes
   - ¬ analyzing subsamples; preference heterogeneity, random coefficient and latent class models

Methods

ESPM Project: Attributes Selected for Study[1]

1. **Severity** of the initial health state: lost life expectancy
   (i.e., *ex ante*, before / without an intervention)
2. **Severity** of the initial health state: lost quality of life
   (i.e., *ex ante*, before / without an intervention)
3. **Effectiveness** of an intervention: life expectancy gained
4. **Effectiveness** of an intervention: quality of life gained
5. **Age** of patients (or “fair innings”)
6. **Rarity** of disorder
   (i.e., prevalence or number of persons benefitting)
7. **Cost** of intervention:
   perspective of a compulsory health scheme (“OKP”);
   payment vehicle = citizens’ or “social willingness-to-pay”

[1]Not all of the attributes were addressed in Study Phase I

Methods

Study Phase I (Switzerland): Attributes Investigated

Attribute	Status Quo	With (new) Treatment
Age of Patients	mainly children, on average 10 years old mainly young adults, on average 40 years old mainly elderly, on average 70 years old	
Prevalence	1 in 20, i.e. about 400,000 persons in Switzerland 1 in 200, i.e. about 40,000 persons in Switzerland 1 in 2,000, i.e. about 4,000 persons in Switzerland 1 in 50,000, i.e. about 160 persons in Switzerland	
Health State	very good good good fair / impaired fair / impaired fair / impaired low / severely impaired low / severely impaired low / severely impaired low / severely impaired	very good very good good very good good fair / impaired very good good fair / impaired low / severely impaired
Life Expectancy (depending on age of patients)	45 (10), 60 (40), 75 (70) 45 (10), 60 (40), 75 (70) 45 (10), 60 (40), 75 (70)	52 (10), 64 (40), 76 (70) 66 (10), 72 (40), 78 (70) 80 (10), 80 (40), 80 (70)
Cost	no extra cost	60 CHF per year (= 5 CHF per month) 120 CHF per year (= 10 CHF per month) 360 CHF per year (= 30 CHF per month) 600 CHF per year (= 50 CHF per month)

Methods

Measuring Informed, Reflected, and Stable Preferences …

Preference Formation Phase (PFP):

[Introduction (3 Questions)]

Open questions exploring agreement
with proposed statements regarding attitudes towards

- Costs / Insurance Premiums (3); Cost of Interventions (2)
- Age and Quality of Life (3)
- Age and Life Expectancy (3)
- Severity of Disease (2)
- Treatment Effectiveness (2)
- Implications of Rarity / Prevalence (3) – *randomized subgroup only*
- Health Insurance: Premium Policy (4)

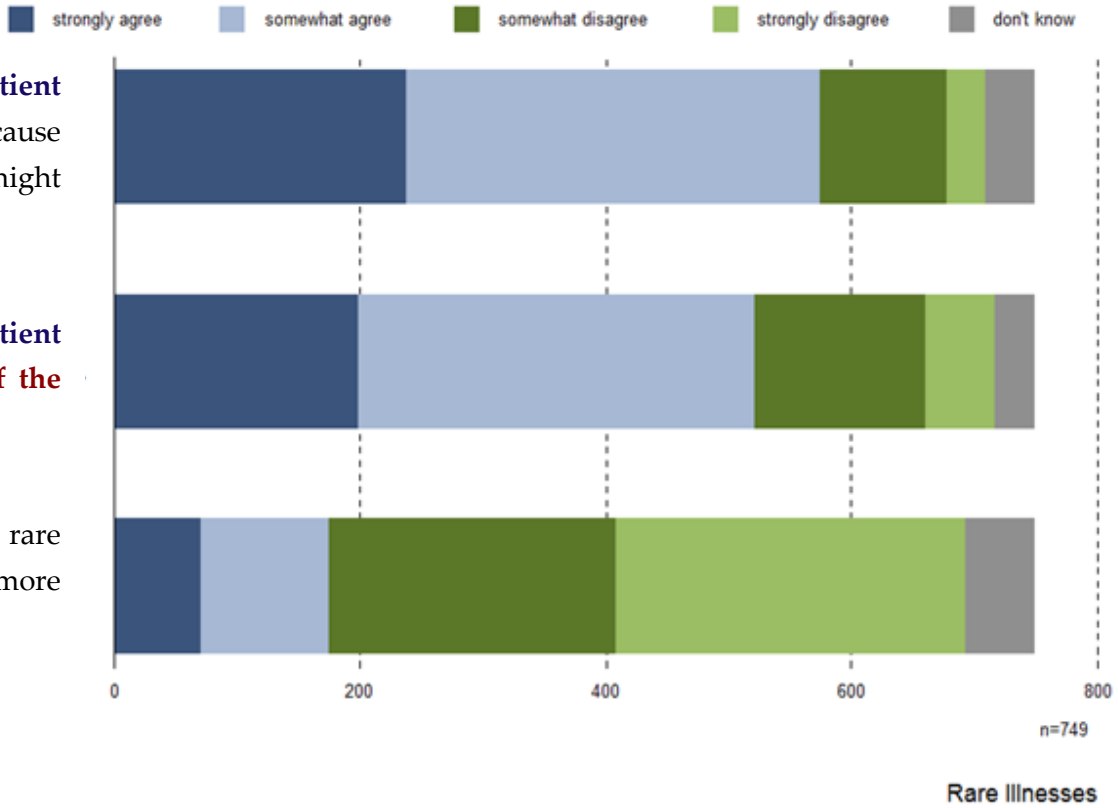
Results

Preference Formation Phase: Attitudes towards Rarity

We should be prepared to accept higher cost per patient for interventions / for treatments of rare disorders, because patients with rare and very rare disorders otherwise might be left without effective treatment.

We should be prepared to accept higher cost per patient for interventions / for treatments of rare disorders, **if the impact on insurance premiums remains low**.

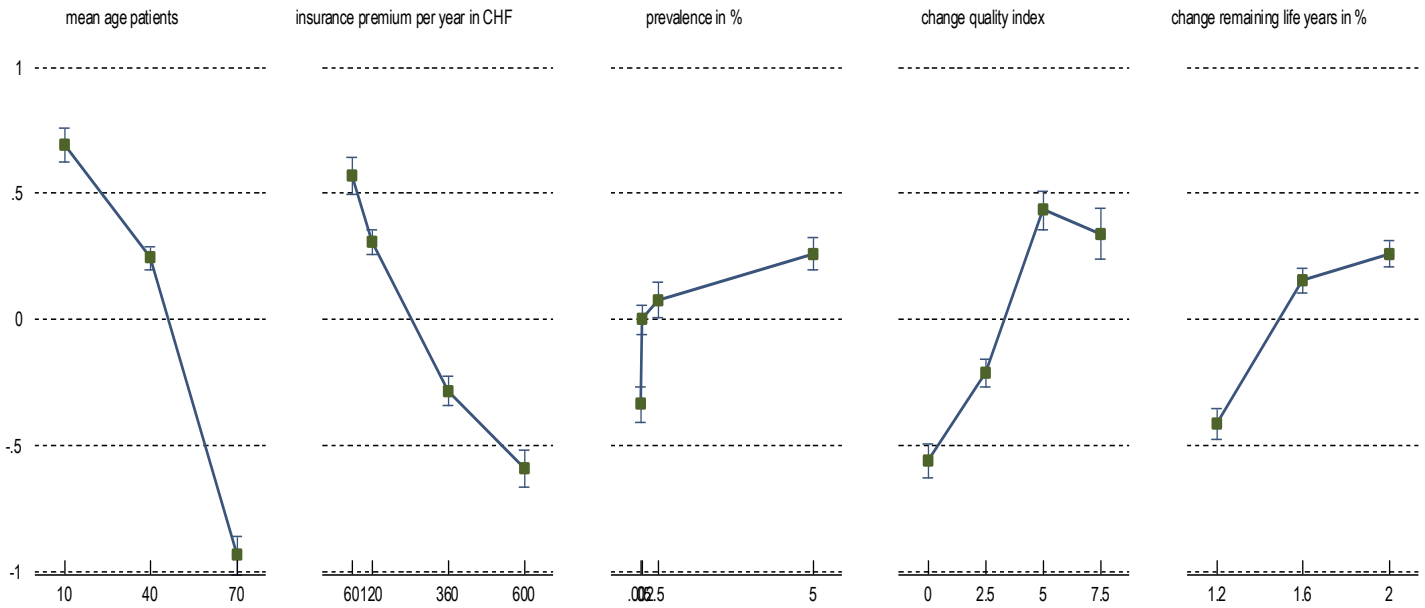
We should not accept higher cost per patient for rare diseases, because we could use this money to help more patients with diseases that are more common instead.



Results

Discrete Choice Experiment: Main Model Selection

Flexible Specification with Dummy Variables: Functional Form



All attributes are specified as indicator variables (without requirements for functional form)

Mean Age of Patients	Prevalence (in %)	Quality of Life Index[1] (0-10)	Change remaining life years (in %)	Insurance premium (per year in CHF)
10 years	5%	2.5 - low/ severely impaired	20%	+60 CHF
40 years	0.5%	5 - fair/ impaired	60%	+120 CHF
70 years	0.05%	7.5 - good	100%	+360 CHF
	0.002%	10 - very good		+600 CHF

Attributes and Levels
Coding for Estimation

Results

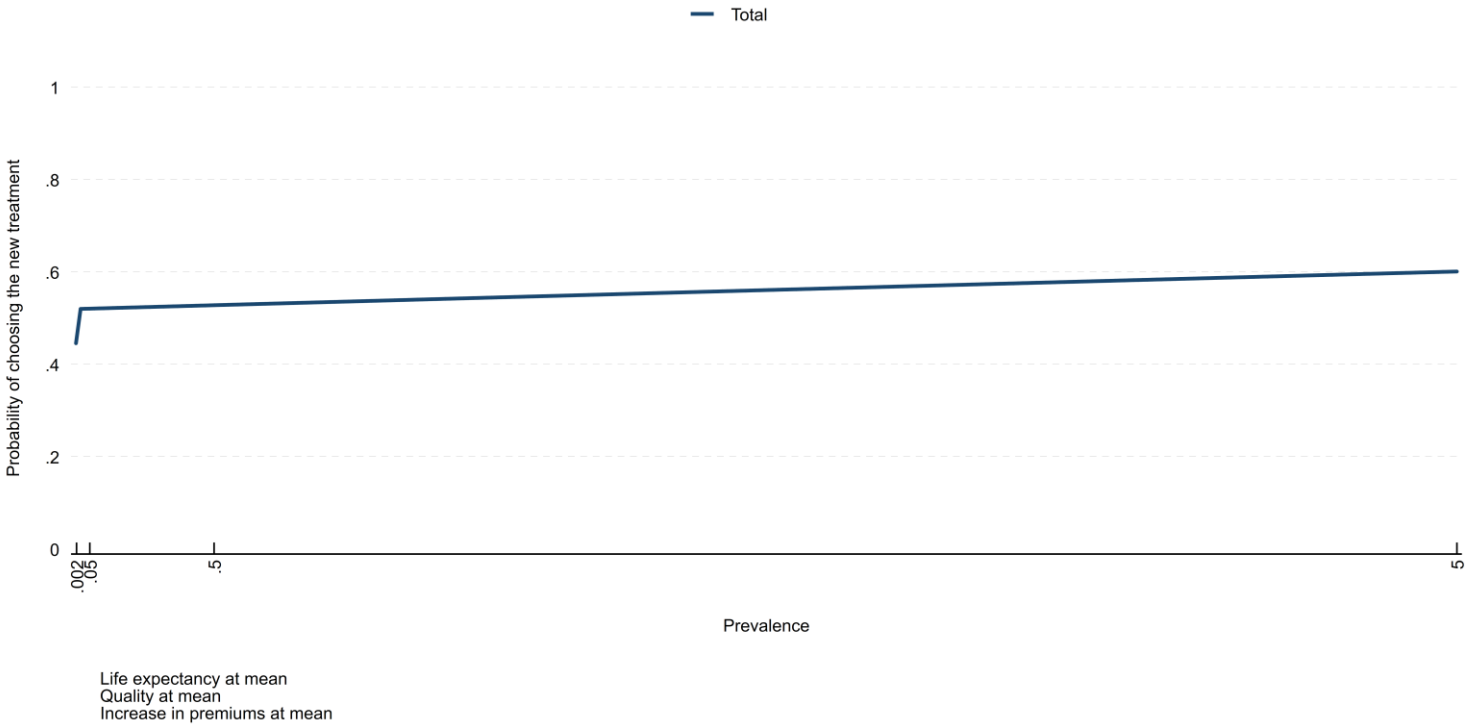
Discrete Choice Experiment: Main Model

- All coefficients show the expected sign
- All coefficients are statistically significant
- Marginal utility of an additional year of life
  is decreasing with increasing total number of years
- Nonlinear relationship for the prevalence attribute:
  modeling using a linear term and a dummy variable
  for the lowest value of the prevalence attribute
  (i.e., the URD qualifier)
- (Approximately) linear relationship for quality of life attribute
- (Approximately) linear relationship for cost attribute

Results

Discrete Choice Experiment: The Rarity Attribute

Probability of choosing the new treatment:



Impact of prevalence (rarity) shown at mean value for all other attributes, i.e., life expectancy at mean, quality of life improvement at mean, increase in mandatory health insurance (OKP) premiums at mean, patients age 40 years (unless specified otherwise.)

Results

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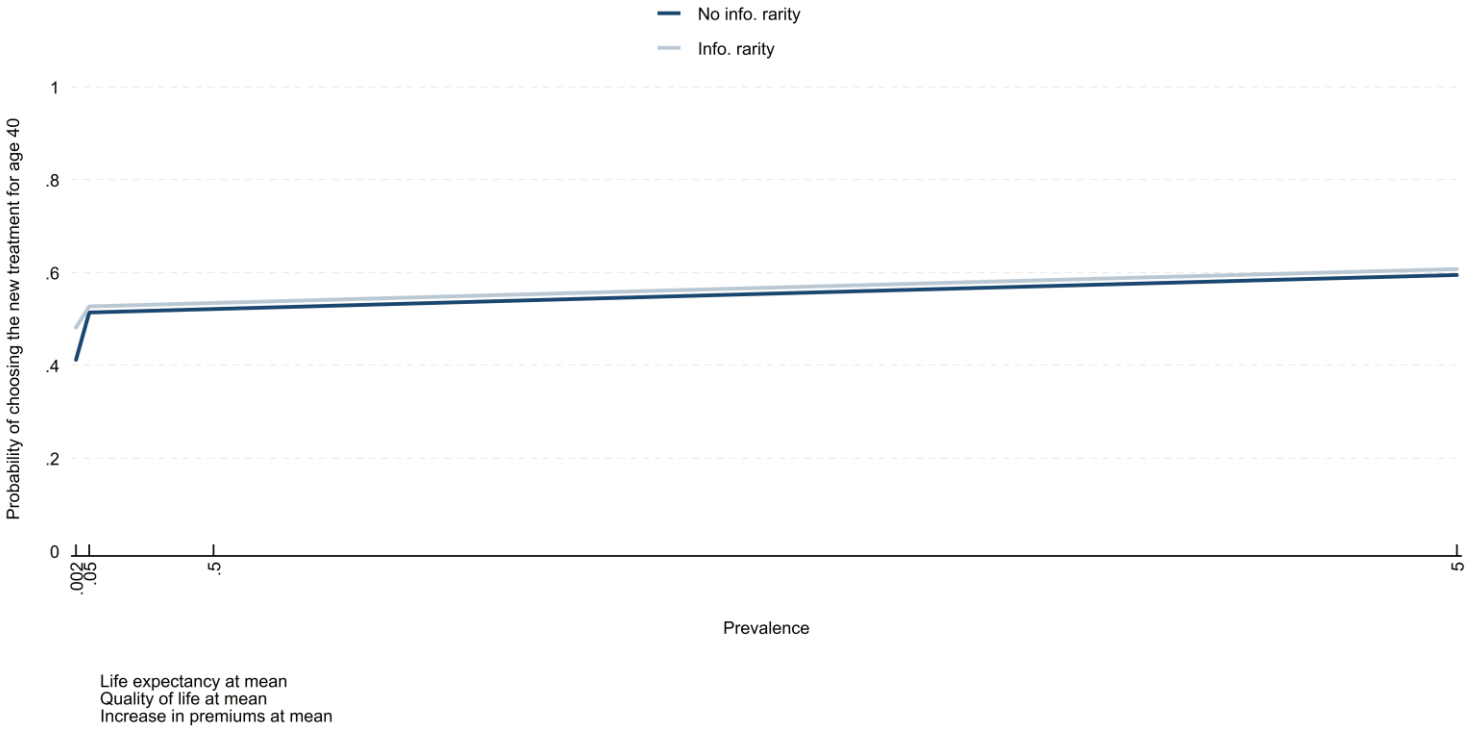

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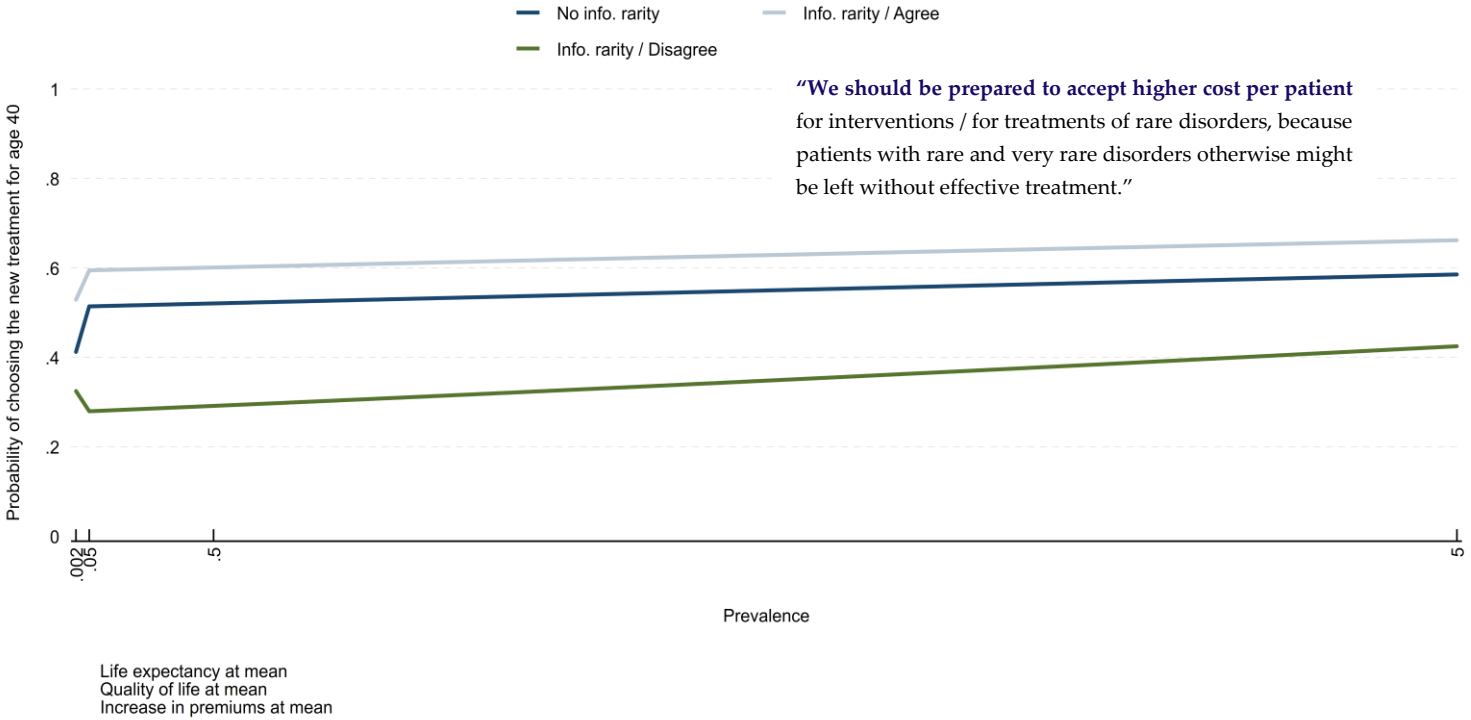

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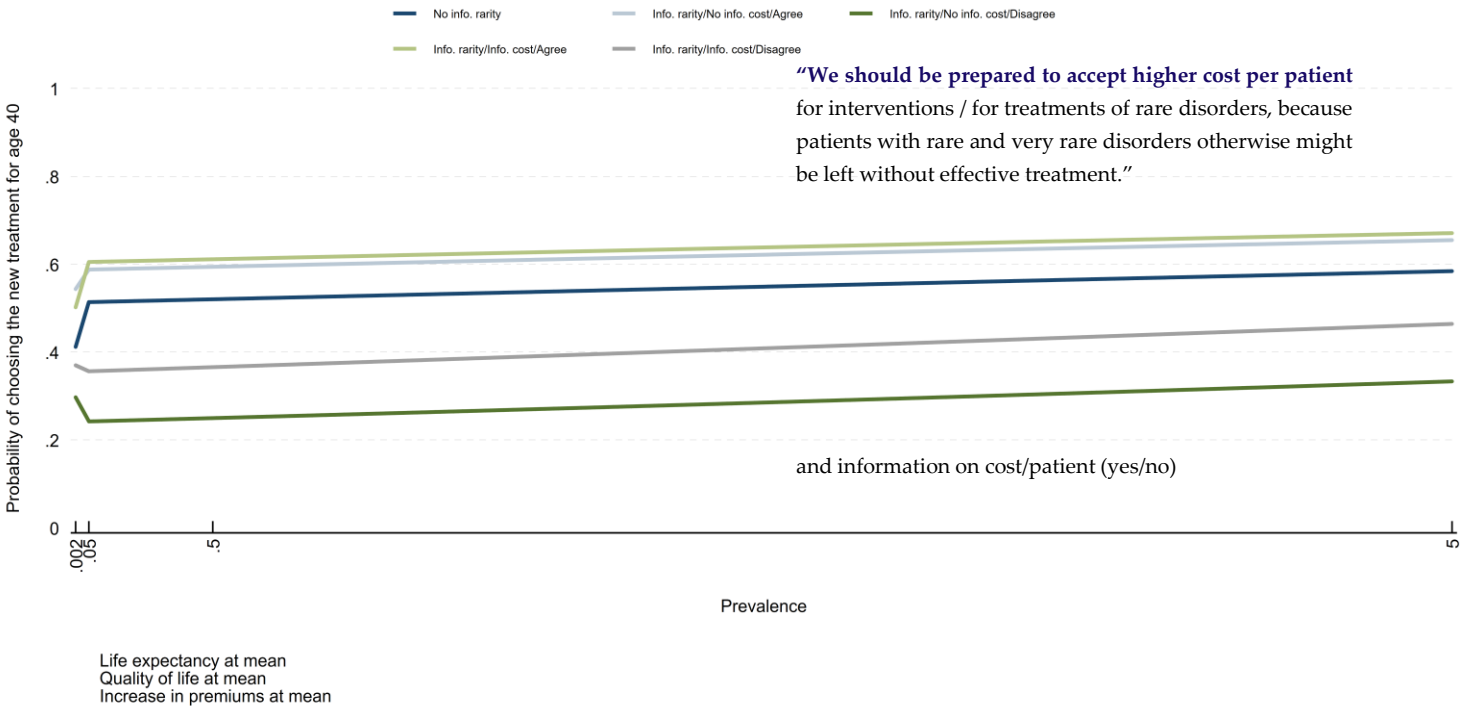

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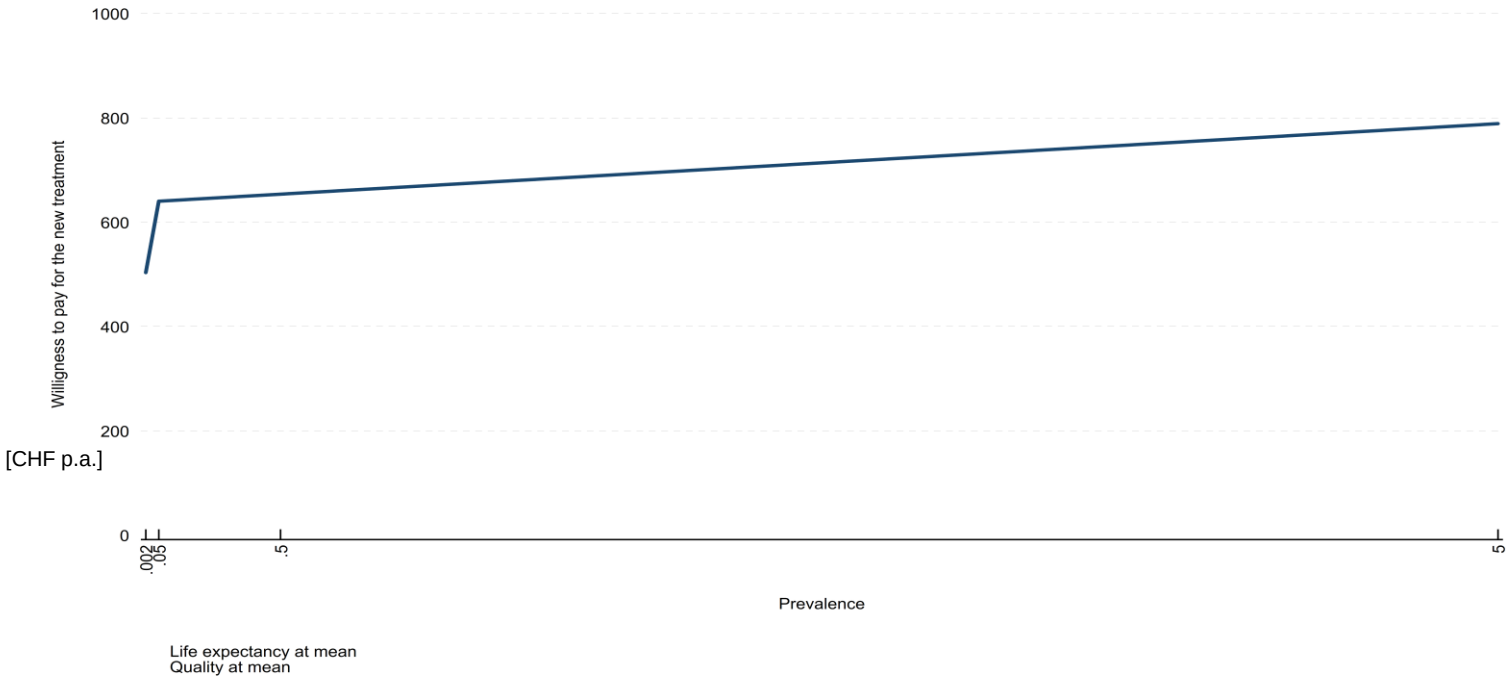
Results

Discrete Choice Experiment: The Rarity Attribute

Citizens' Willingness-to-Pay for the new treatment:



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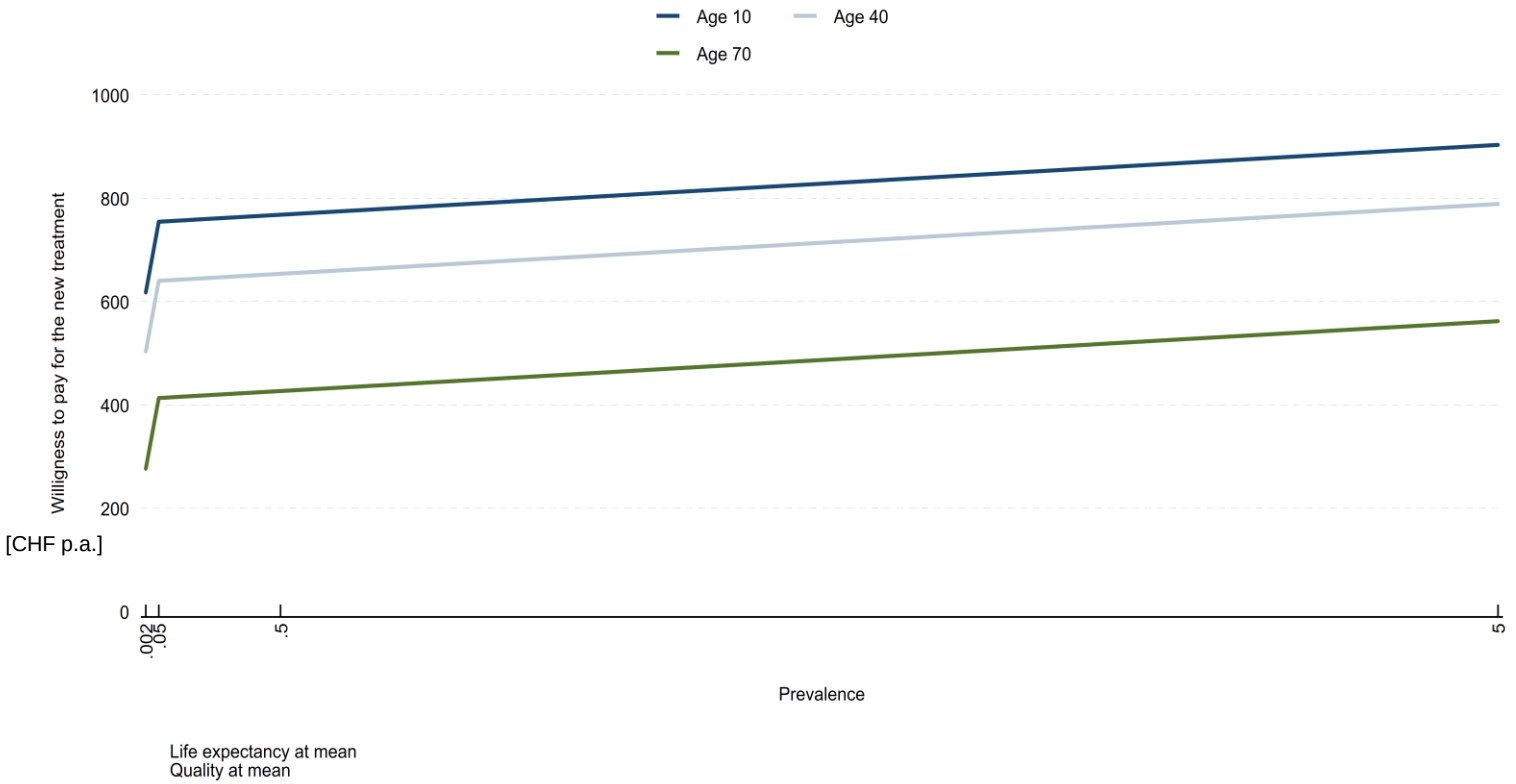

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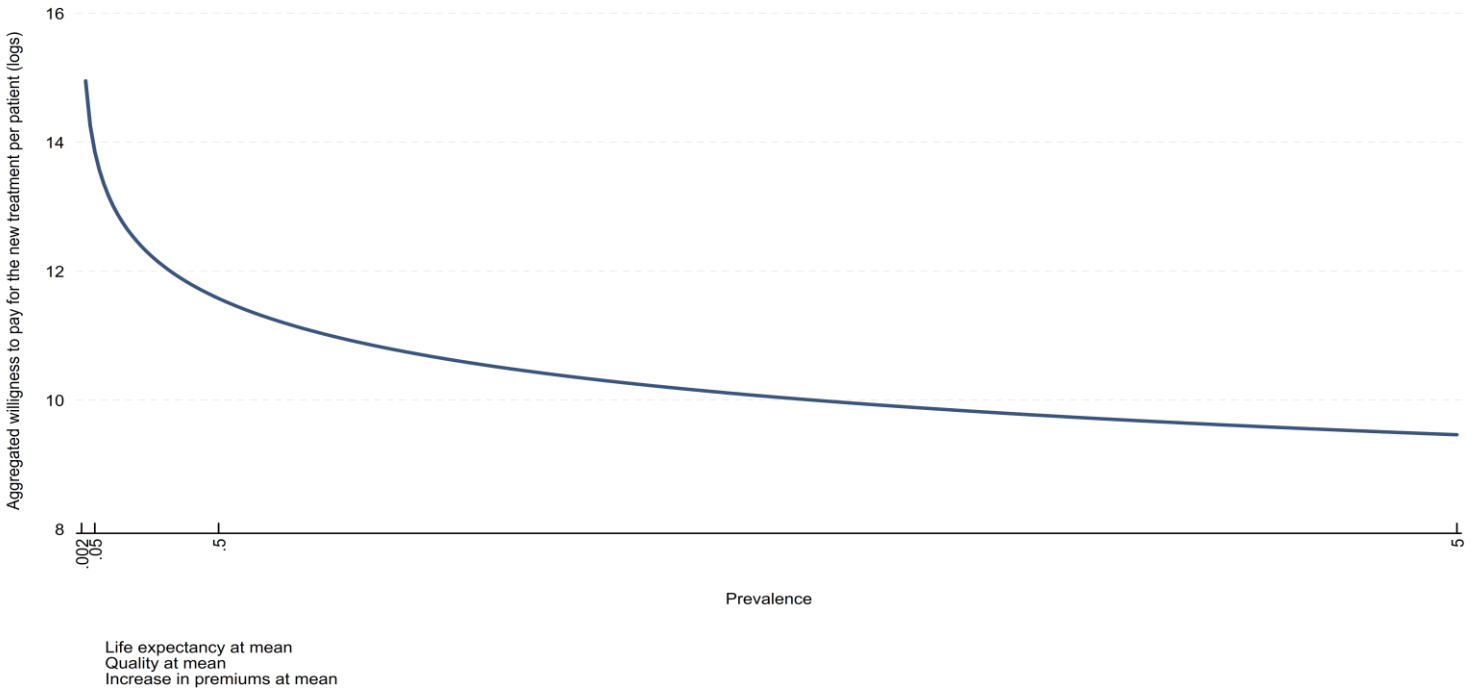
Results

Discrete Choice Experiment: The Rarity Attribute

Implied [“Social”] Willingness-to-Pay / *per patient* for the new treatment:



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Discrete Choice Experiment: The Rarity Attribute

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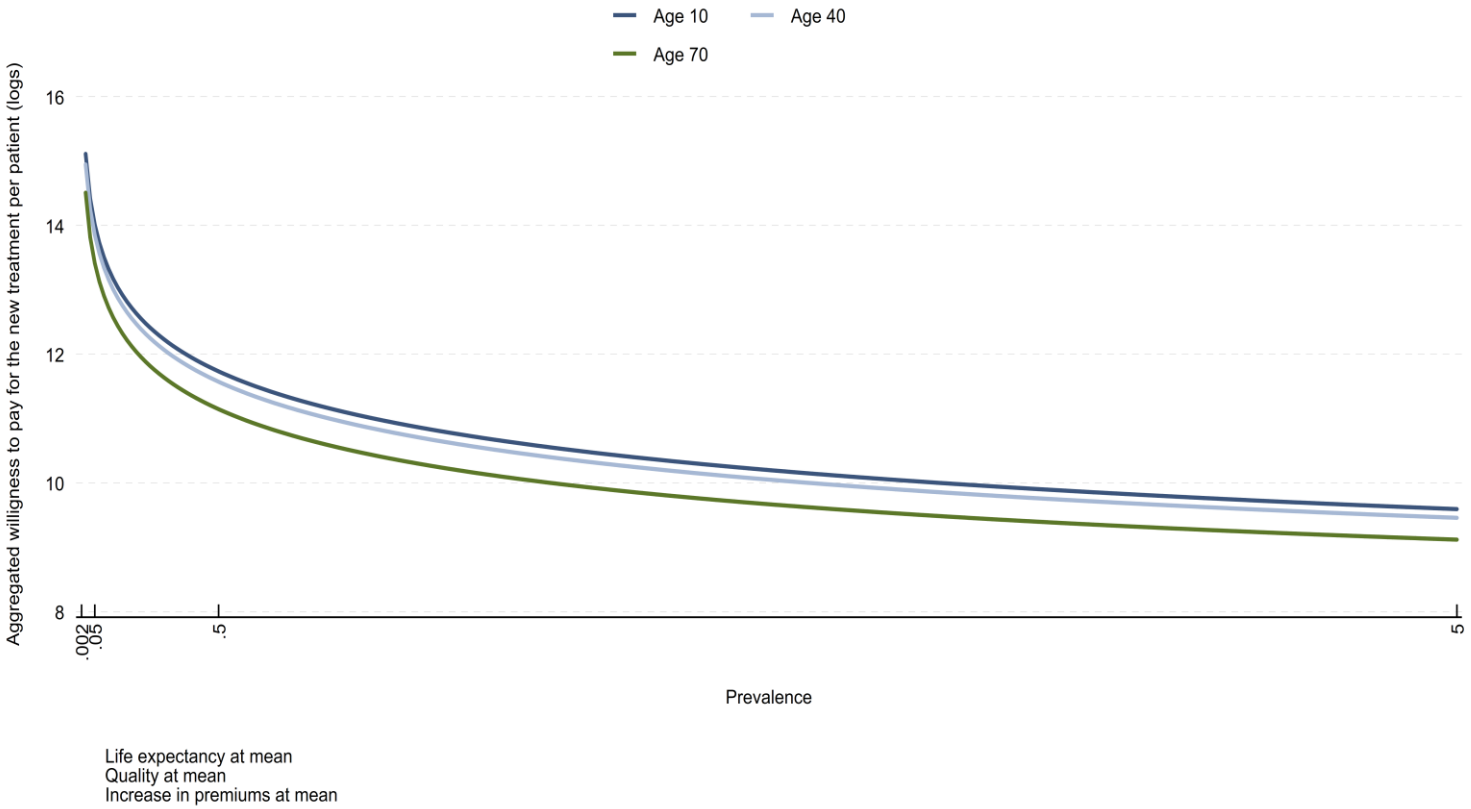

Impact of prevalence (rarity) shown at mean value for all other attributes, i.e., life expectancy at mean, quality of life improvement at mean, increase in mandatory health insurance (OKP) premiums at mean.

Implications

Social Preferences

Observations

- Our Discrete Choice Experiment including a sample of 1,501 Swiss respondents (in year 2017) assessed the relative importance of selected attributes of health care interventions, capturing social preferences from a citizens' perspective (using marginal compulsory health insurance premiums as the payment vehicle).
- All attributes investigated in Study Phase I had an impact on choice probability and citizens' (or "social") willingness-to-pay (S-WTP).
- The variables with the highest impact on choice probability were
  - change in remaining **life years**,
  - **quality of life**,
  - **extra insurance premium** per year.
- The relatively small impact of prevalence translates into **a profoundly increasing implied willingness-to-pay per patient** (and per life year gained) **with decreasing prevalence** (or "**rarity**").
- These results pass tests of internal consistency, rationality, and theoretical validity.

Thank You for Your Attention!

For further information,
please refer to www.dkfz.de
or www.innoval-hc.com
or www.polynomics.ch
Contact: m.schlander@dkfz.de

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