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Swiss Social Preferences

Briefing Document: Essential Insights from the SoPHI Study

INNOVAL HC
Institute for Innovation & Valuation in Health Care

Michael Schlander and Harry Telser

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THE EUROPEAN SOCIAL PREFERENCES MEASUREMENT ("ESPM") PROJECT – PHASE I:

THE SWISS SOCIAL PREFERENCES FOR HEALTH CARE INTERVENTIONS ("SOPHI") STUDY

BRIEFING ON ESSENTIAL STUDY INSIGHTS & POLICY IMPLICATIONS

Michael Schlander and Harry Telser

in cooperation with
Barbara Fischer, Tobias von Rechenberg, Diego Hernandez, and Ramon Schaefer – on behalf of the ESPM Project Group

Wiesbaden, Heidelberg und Olten, January 2021

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Governance & Conflict of Interest Disclosure

Following-up on the SwissHTA project, an expert panel had been created by Michael Schlander and InnoVal[HC] to serve as the **Scientific Steering Committee (SSC)** for the ESPM project:

- **Silvio Garattini**
  (Mario Negri Institute, Milan / Italy);
- **Søren Holm**
  (University of Manchester / England; University of Oslo / Norway);
- **Peter Kolominsky-Rabas**
  (University of Erlangen / Germany);
- **Deborah Marshall**
  (University of Calgary / Canada);
- **Erik Nord**
  (University of Oslo / Norway);
- **Ulf Persson**
  (IHE, Lund / Sweden);
- **Maarten Postma**
  (University of Groningen / Netherlands);
- **Jeff Richardson**
  (Monash University, Melbourne, Victoria / Australia);
- **Michael Schlander**
  (German Cancer Research Center, DKFZ & University of Heidelberg; InnoVal[HC], Wiesbaden / Germany);
- **Steven Simoens**
  (University of Leuven / Belgium);
- **Oriol de Solà-Morales**
  (IISPV, Barcelona / Spain);
- **Keith Tolley**
  (Tolley HE, Buxton / England);

- **Mondher Toumi**
  (University Aix-Marseille / France).

The Scientific Steering Committee (SSC) contributed to design, implementation, and interpretation of the ESPM project and its first phase, the Swiss SoPHI study. For conduct of the SoPHI study in Switzerland, **Harry Telser** and his team from Polynomics in Olten, Switzerland, were invited to join the group as experts in the design, conduct, and evaluation of discrete choice experiments (DCEs). Commencing with a project team meeting in Amsterdam in November 2014, Harry Telser participated in all subsequent meetings of the SSC to date.

After having secured funding for the first phase of the ESPM project (cf. below, «International Advisory Board»), and following deliberations in Heidelberg and Milan during 2015, the SSC met for an expert workshop in Heidelberg, September 2016, and decided the on attributes to be tested and on the basic design of the SoPHI study. Subsequently, **Michael Schlander** and **Harry Telser** acted as co-leaders of the study.

After each stage of the SoPHI study (cf. Methods, below), i.e., in particular after completion of the cognitive (qualitative) pre-test, after completion of the quantitative pre-test, and upon availability of the first econometric results and the draft study report, the SSC convened – either in person or by means of teleconferencing – and discussed interpretation, conclusions, and next steps.

Further to this, **Michael Schlander** and **Harry Telser** presented and discussed the project design at various international scientific meetings with external peer review.

In addition, stakeholders funding project phase I were represented in an **International Advisory Board (IAB)**, who had the opportunity to comment on each step before final decisions were taken by the study leaders in cooperation with the SSC:

- BioMarin
  Mohit Jain, **Thomas Butt** (London, England);
- Curafutura / CSS
  Christian Affolter (Berne / Lucerne, Switzerland);
- Galenica
  K. Christian Köpe, **Pius Gyger** (Berne, Switzerland);
- Interpharma
  Heiner Sandmeier, **Ansgar Hebborn** (Basle, Switzerland);
- Sanofi / Genzyme
  Vinciane Pirard (Naarden, Netherlands);
- Schweizerischer Versicherungsverband (SVV)
  Ann-Karin Wicki (Zurich, Switzerland).

Project funding was arranged under an unrestricted educational grant policy and was equally shared between BioMarin and Genzyme for the international pharmaceutical project sponsors, and between the Swiss research-based pharmaceutical industry and Swiss health insurance.

IV

Background

Health care policy makers strive to ensure «value for money», i.e., fair access to and efficient provision of effective health services for populations covered by collectively financed health schemes. It is widely agreed that decisions on coverage, reimbursement, and provision of health technologies should be ethically defensible and consistent with the values of the majority of the public.

In Switzerland, the health insurance law (*Kranken-versicherungsgesetz*, KVG) enacted in 1996 stipulates an evaluation framework reflecting the criteria of effectiveness, appropriateness, and economic viability (*Wirksamkeit*, *Zweckmässigkeit*, *Wirtschaftlichkeit*; the latter sometimes also being translated as "efficiency").

Conventional approaches to the economic evaluation of health services (typically based on the logic of cost effectiveness, i.e., the computation of incremental costs per quality-adjusted life year, QALY, gained), however, rest on restrictive definitions of value and lead to problematic conclusions, which raise reflective equilibrium issues. One reason for this is that they rely on purely selfish preferences for health states only and do not take into account social (and non-selfish) preferences. Any extensions of, or alternatives to, the conventional model require, in addition to rigorous normative analysis and deliberation among «fair-minded» stakeholders, robust information on relevant social preferences as a point of reference.

A comprehensive review of the literature on social preferences with regard to the allocation of health care resources indicated empirical support for a number of characteristics or «attributes» (beyond clinical effectiveness) of interventions, in particular,

- for giving priority to the worst-off (severity, related to the *ex ante* health state),
- for prioritizing urgent interventions (urgency, because of the risk of major irreversible consequences without intervention, which may be conceptualized as an effectiveness),
- for non-discrimination against persons in «double-jeopardy» (or, more generally, persons with comorbid conditions),
- for prioritizing health care for younger over older patients (age or the «fair innings» argument),
  and
- for a wish to share resources with patients even if their treatment costs are high, in order not to disenfranchise them from a fair chance of access to effective care,
- and, albeit generally to a lesser extent, a number of further attributes.

Critical questions remained due to the small size of many studies, heterogeneity of experimental settings, potential framing effects, and the level of information among respondents regarding the implications of their choices. Further issues were identified in relation to the stability of observed preferences and the validity of the resource constraint imposed in some of the studies.

Objectives

Against this background, the «Social Preferences for Health Care Interventions» (or «SoPHI») study was initiated

- to investigate how Swiss citizens valuate selected characteristics (or «attributes», see below) of health care interventions, and how they weigh them against each other;
- to compare the valuation results obtained in the study with those based on the conventional logic of cost effectiveness;
- to assess the sensitivity of weights to the level of reflection and information offered to respondents and thus to potential framing effects;
- to generate learnings and provide a basis for subsequent work, ultimately paving the way towards an exploration of international similarities and differences with regard to the valuation of the attributes tested, including an external validation of results by testing the agreement of respondents between their choices in the experimental setting, their policy implications, and their social preferences.

The approach of the study was a willingness to pay (WTP) design using discrete choice experiment (DCE) methodology. WTP was estimated by introducing a **cost attribute** from the perspective of the members of mandatory health insurance (*Obligatorische Krankenpflegeversicherung*, OKP, in Switzerland), i.e., using as payment vehicle (or «cost attribute») the extra premium in exchange for a new intervention added to the package of services covered by OKP.

The list of attributes to be tested further comprised

- **severity** of the initial health state (ex ante, i.e., before intervention, reduced life expectancy and – separately - impaired health-related quality of life);
- **clinical effectiveness** (improvements of life expectancy and – separately – health-related quality of life as a result from

adding the new intervention, compared to existing treatment options);

- **age of patients** (to capture the «fair innings» perspective);
- **prevalence of the disorder** (or «rarity»), i.e., the number of persons benefitting from adding the new intervention.

Of note, our experimental design assumed existence of a standard treatment for the condition in question; thus, the elicited preferences relate to an improvement of therapy, not to the existence of therapy (and thus hope) in the absence of any other options.

Framing effects were assessed for the following attributes by way of **randomization** of respondents to one out of four subgroups.

- **Rarity** – with a view towards the specific challenges posed by the evaluation of orphan medicinal products (OMPs), respondents were randomly assigned to subsamples who were offered different levels of information and reflection about the potential implications on cost per patient treated of the fixed (i.e., largely volume-independent) cost for research, development, and certain infrastructure.
- **Cost(s)** – presented from a citizen's perspective, i.e., as cost per member of a collectively financed health scheme (i.e., as an increase in mandatory insurance premiums); randomized subgroup(s) of respondents received additional information on the implied incremental cost per patient treated.

The Swiss SoPHI Study was intended to be a feasibility test for future pan-European study extensions. SoPHI was not designed to fully capture the (independent) impact of the severity attributes, i.e., the initial («ex ante») impairment of health in terms of reduced life expectancy and reduced quality of life.

Rather, its design focused on the attributes age, prevalence («rarity»), health gain (both dimensions, i.e., length and quality of life), as well as cost from a citizen's perspective.

Methods

An international scientific expert panel was created to contribute to (experimental) design and methodology, implementation, and interpretation of the SoPHI study. From the very beginning, an international **Scientific Steering Committee (SSC)** was involved continuously in all study stages, and agreed on key design elements including the following,

- cognitive (qualitative) pre-test to check for comprehensibility and length of the questionnaire as well as cognitive overload;
- a quantitative pre-test using a representative online panel to further test for internal consistency and theoretical validity;
- main survey questionnaire, administered online, including a representative Swiss population sample (n = 1,501 respondents in 2017);
- perspective on costs capturing risk aversion and the wish to share health care resources, by focusing on costs from a citizen's perspective (i.e., using WTP_{public} as a payment vehicle);
- generic health state vignettes used as a utility comparator, with (general) descriptions derived from three dimensions of EQ-5D-5L.

In addition, the **potential for framing effects** was tested by way of randomization of respondents into subsamples (cf. "*Objectives*", above), i.e.,

- firstly, by reflection on implications of rarity (based on different levels of information provided; 1:1 randomization),
  and
- secondly, by information on cost per patient implied by the choice alternatives presented (i.e., information on cost per patient either provided or withheld; 1:2 randomization).

The **main survey** consisted of three main parts – (1) an initial preference formation phase (PFP), (2) the DCE itself, and (3) supplementary general questions related to both experiment and respondent characteristics. By part of the online survey, these were:

(1) Initial Preference Formation Phase (PFP)

- 25 open questions to stimulate reflection by respondents on value judgments, and 1:1 random assignment to a group without any further information on the implications of rarity, or a second group with three additional questions specifically related rarity and its impact.

(2) Discrete Choice Experiment (DCE)

(participants chose repeatedly between two alternatives, i.e., insurance contracts covering the standard treatment only or insurance contracts also covering the new treatment option)

- using decision cards with or without additional information on cost per patient (1:2 random assignment),
- applying fractional factorial design based on the D-efficiency criterion (D-efficient design), designed with 30

choice situations divided in three blocks (i.e., 10 choice situations for each respondent);
- using linear conditional logit as base model,
- testing for interactions and nonlinearities of attributes (or coefficients);
- analyzing subsamples, including to test for preference heterogeneity in different subgroups;
- using random coefficient as well as latent class models.

Attributes tested in the DCE (and thus included for econometric evaluation) were

- incremental effectiveness of new intervention in terms of life expectancy gained;
- incremental effectiveness of new intervention in terms of health-related quality of life gained;
- age of patients (to address the idea of a «fair innings»);
- rarity (or prevalence) of disorder;
- incremental cost of new intervention;
- but not the severity of the initial health state (impaired life expectancy) *ex ante*.

(3) General Questionnaire

General questions addressed

- the health state and type of health insurance of respondents,
- basic socioeconomic information on respondents,
- specific feedback (e.g., personal exposure) of respondents.

IV

Results

The respondents who completed the questionnaire (a sample of n=1,501 respondents in 2017) were representative with regard to language (30% French; 70% German), sex (49% men; 51% women), and age (by groups: 18-39 years, 36%; 40-64 years, 42%; 65 years and older, 22%). There was however a slight over-representation of high-income and better-educated groups.

The overall discontinuation rate was 21.5%, but only 6.3% discontinued the questionnaire during the DCE itself, i.e., the majority of discontinuation occurred after the first few questions during the initial PFP.

In the preference formation phase, the majority of the participants in the sample were skeptical about higher insurance premiums and the majority favored to treat all patients equally. However, the majority was also prepared to accept higher costs for treatment of rare disorders.

In the DCE, overall, the new treatment was chosen in about half of the choices. All attributes (or coefficients) tested were statistically significant and showed the expected sign, which means that all of them had an impact on choice probability and citizens´ WTP. The variables showing the highest impact on choice probability were

- change in remaining life years,
  i.e., the positive coefficient "change in remaining life years" indicates that respondents' utility of the new treatment increased with the total number of life years gained;
- quality of life,

i.e., the positive coefficient "quality of life" indicates that respondents' utility of the new treatment increases with health-related quality of life;

- and extra insurance premium per year,
  i.e., the "cost" attribute defined as the increase in the health insurance premium is negative.

The overall impact of prevalence was comparable to the age effect; however, the effect of prevalence remained for all age groups relatively small. By level of reflection on implications of rarity, both subgroups showed a decreasing valuation of an intervention with decreasing prevalence of the disorder. The decrease in valuation was much smaller than the decrease of prevalence, and by implication the accepted cost per patient increased with rarity. In fact, the implied WTP per life year gained revealed a marked increase with decreasing prevalence. While the numerical extremes should be interpreted with caution (cf. "*Limitations*", below), it seems worth mentioning that they reached a maximum WTP estimate per adjusted life-year of more than CHF 800,000 in case of an ultra-rare disorder with a prevalence of 0.002% (equivalent to no more than 160 persons in Switzerland), while decreasing sharply with higher prevalence rates. Providing additional information on implied cost per patient had little impact on valuation only. Interestingly, the negative effect of additional cost on marginal utility was less pronounced for respondents who received information on treatment cost per patient.

As to preference heterogeneity, preferences seemed to vary with the age of the respondents, i.e., the likelihood to choose the insurance contract covering the new treatment was increasing with the age of the respondents, but prevalence, quality of life, and remaining life years became relatively less important. The likelihood of choosing the new treatment also increased among

those who suffered from a rare disease, those who were in favor of organ donation, and those who donate blood. It decreased if respondents were more educated and with a higher deductible. All results from the econometric analysis passed tests of internal consistency, rationality, and theoretical validity.

Finally, answers in the initial PFP were associated with the decision(s) in the DCE, i.e., preferences formed in the PFP were mirrored in the DCE. For example, participants who reported being willing to pay higher insurance premiums to enable treatment of rare diseases were also more likely to choose the new treatment option.

Likewise, more than two third of respondents in the subgroup who were exposed to questions on their attitude towards "rarity" stated that they were willing to accept higher cost per patient with a rare disorder (for wording of questions, cf. Appendix 1, "Subsample information"). Consistently, respondents with a higher WTP per patient also showed a higher WTP in the DCE.

Conclusions

The DCE included a sample of 1,501 Swiss respondents in 2017 and 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 the SoPHI study had an impact on choice probability and citizens' (or "social") WTP. The variables with the highest impact on choice probability are

- change in remaining life years,
- quality of life,
- extra insurance premium per year, and
- (to some extent) age of patients.

The relatively small impact of prevalence translated into a profoundly increasing implied WTP per patient (and per life year gained) with decreasing prevalence (or rarity).

Study design, and hence our presented data, focused on the "rarity" attribute. Results should therefore not be interpreted as a comprehensive account of all relevant attributes. This also imposes limits on the precision of the quantitative information provided:

Limitations

The Swiss SoPHI Study was not designed to capture initial severity of disease (i.e., the initial impairment of health in terms of reduced life expectancy and reduced quality of life), and / or urgency of an intervention as independent variables.

The observed impact of rarity on implied (or "social") WTP per patient (and per life year gained, with or without quality adjustment) will need to be confirmed considering numerous aspects, including but not limited to the following:

- Does the observed gradient by prevalence reflect true social preferences, i.e., at the extreme, do respondents believe that patient numbers should (almost not) count when it comes to resource allocation, i.e., acceptance of programs for reimbursement?
- Was there an impact of an availability heuristic in the subgroup with more reflection on rarity, which showed a relatively higher willingness-to-pay?
- Did respondents fully understand the math behind the numbers presented, in particular regarding the prevalence attribute, and related to this, was there a potential bias due to "insensitivity to size"?
- Are the observed social preferences, if confirmed as robust by further work, internationally consistent or are social preferences heterogeneous across jurisdictions?

The agreement of respondents between their choices in the experimental setting, their policy implications, and their policy preferences also warrants further study. Future work will have to be designed to address the questions above. It should also be designed to explicitly capture the impact of the initial severity of disease and the urgency of an intervention as independent attributes driving the social valuation of health care interventions.

Policy Implications

While caution should be exercised in the interpretation of some of the numerical values derived from the present study, the findings clearly indicate the relevance of attributes of health care

interventions beyond the improvement of length and quality of life and cost per patient. Perhaps most importantly, the results point to a strong impact of the (decreasing) prevalence of a disorder on the (increasing) implied WTP per patient. Thus, the study provides empirical support for a higher societal WTP per patient treated for rare and ultra-rare disorders, compared to patients with high-prevalence diseases.

IV

Appendices

Appendix 1: Subsample information («*Rarity*»)

Respondents in the subsample with information about implication of «Rarity» received the following text with three statements:

«Another example is the case of rare diseases. Some people believe that we should not pay more for treatment of patients with rare disorders, whereas others believe that we should be prepared to pay more. The reason is that the cost of development of new medicines and the risk of failure of research programs can be very high. Thus, in the absence of acceptance of a higher cost per person treated, many patients with rare and ultra-rare disorders might have no access to effective treatments – simply because of their sometimes high or very high costs in relation to small or very small patient numbers.

In the following, you can see three statements. Please indicate respectively, if you agree strongly, rather agree, rather disagree or disagree strongly with the statement.

- *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.»*

IV

Appendix 2: Hypotheses tested in DCE and study findings

The following hypotheses had been defined ex ante and were assessed in the discrete choice experiment (DCE):

- Importance of Attributes

H1: Social preferences for incorporating a new treatment into the benefit package of mandatory health insurance are not only dependent on duration and quality of life but also on additional aspects like age of patients and prevalence of the disease.

To estimate the preference weights of attributes in the utility function of respondents, we used a conditional logit model. All coefficients show the expected sign and are statistically significant.
H1 cannot be rejected as all analyzed attributes have a statistically significant impact on the choice of the new treatment, not only quality of life and remaining life years.
In the following, the attributes are analyzed in more detail.

- Age of Patients

H2: The utility of a new treatment is ceteris paribus higher for young patients compared to middle age patients.
H3: The utility of a new treatment is ceteris paribus higher for middle aged patients compared to old patients.

Both hypotheses *H2* and *H3**H3*** cannot be rejected.

- Costs

H4: The utility of a new treatment decreases with the cost measured via insurance premium per month/year in CHF.

The impact of the cost attribute defined as the increase in the health insurance premium is negative, as expected. This means the higher the additional costs for the new treatment to the respondent, the less frequently it is chosen. The coefficient is statistically significant and therefore, the hypothesis *H4* cannot be rejected.

¬ Clinical Effectiveness

H5: The utility of a new treatment increases with the improvement in quality of life.
H6: The utility of a new treatment increases with an increase in remaining life years.

The positive and statistically significant coefficients "quality of life" and "life expectancy" indicate that respondents' utility of the new treatment increases with quality of life and the total number of life years gained. Therefore, the hypotheses *H5* and *H6* cannot be rejected. However, the marginal utility for an additional life year is decreasing with the number of years (statistically significant).

¬ Prevalence

H7: The utility of a new treatment increases with the number of people affected by the disease.

The utility of respondents for the new treatment is higher the more people are affected by a disease, i.e., the more patients can profit from the new treatment (statistically significant positive estimate for the variable "prevalence"). In addition, the estimate for the lowest prevalence rate of 0.002% is statistically significant negative indicating a mark down for ultra-rare diseases. According to these results, *H7* cannot be rejected.

¬ Relative Importance of Attributes

H8: Attributes like age of patient, prevalence, quality, remaining lifetime and cost determine the utility of a new treatment. The attributes differ in relative importance.

The importance of the attributes depends on the level, since there is a non-linear relationship. Even though all attributes contribute significantly to the utility of respondents, they all do so to varying degrees. Therefore, the hypothesis *H8* cannot be rejected. – For full analysis, please refer to the Final Study Report.

¬ Severity of Illness

H9: The severity of the condition in the standard treatment has a positive effect on the utility of new treatment.

Since the focus of this study was set on rarity, we did not include a specific attribute for severity. The main model only includes the effectiveness of treatment w.r.t. quality and life year gains of the new treatment. However, since we varied the initial health status for quality of life in the standard treatment, we can test whether "severity" (operationalized as quality of life before treatment) has an effect of the choice probability. According to this analysis, the perceived utility of the treatment is higher for healthy patients than for patients with a poor health state. Therefore, following our data, *H9* has to be rejected.

¬ Framing & Nudging

H10: The subgroup who receives the information on rarity has a lower disutility from a smaller number of patients affected by the disease.
H11: The subgroup who receives the information on total cost per patients has a lower utility of (accepting) a new treatment.

H12: There is no clear hypothesis for the subgroup who receives both information.

Respondents are randomly assigned to four different subgroups receiving different levels of information with regard to the impact of rarity on cost per patient and cost per treated patient. We construct these subgroups to analyze potential nudging and framing effects. We investigate the impact of nudging due to the additional information in the preference formation phase regarding rare diseases. By adding the information about treatment cost per patient – besides the increase in own insurance premiums – as additional information to each decision in the DCE, we analyze the impact of framing on the valuation of attributes.

The different subgroups and number of respondents allocated to them are shown in the Table below.

Table: Number of respondents per subsample

number of respondents	no info rarity	info rarity	total
no info cost	505	487	992
info cost	247	262	509
total	752	749	1,501

Since a very low prevalence rate with a given premium increase can result in very high treatment cost per patient, we further study the effect of offering both information on rarity as well as cost. The statistically significant positive effect of ultra-rare disorders can still be observed for the subsample with information on rarity and without information on treatment cost but diminishes when respondents also receive information on treatment cost. Respondents who only receive information on treatment cost show a larger mark down for ultra-rare

disorders. However, this difference is not statistically significant.

For full analysis, please refer to the Final Study Report.

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