Inštitut za ekonomska raziskovanja
*Institute for Economic Research*

# WP4: HTA and QoL measurement

# Whose preferences should we use in decision making in health care?

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# Objectives of WP4

1. To improve the robustness of evidence on elicitation of preferences by deriving these in more realistic settings, by drawing on the wider EU citizenship and from within the patient community.
2. To create new data that will incorporate patient relevant values into widely used tools for quality measurement, such as EQ-5D.

# Who was involved?

**Institute for Economic Research**
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- Marko Ogorevc
- Nika Murovec

**London School of Economics**
- Panos Kanavos
- Hala Hourani
- Davina Nauth

**Spanish Patients Forum**
- Maria D. Navarro

**European Brain Council**
- Jean Mossman

**GEPAC, the Spanish Group for Cancer Patients**
- Begoña Barragan
- Natacha Bolaños

**EuroQol Group**
- Juan Manuel Ramos Goñi
- Mark Oppe
- Bernhard Slaap
- Arnd Jan Prause

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# Background

Health state preference values, or 'utilities', are required to calculate the quality-adjusted life-years (QALYs) gained from therapy

The ICER is particularly sensitive to the utility estimates when the treatment impacts primarily on quality of life, or when the life-years gained are not lived at full health

# Experienced Valuations Versus Stated Preferences

- Health state valuations can be obtained from individuals who are either living in the health state, or who have **experienced** it in the past (e.g. patients)

- **Stated** preferences are often obtained from individuals with no direct experience of the health state (e.g. the general public)

- Recent review of guidelines for health economic evaluations (Heintz et al, 2015): 51 guidelines from 25 countries were studied.

# Extracts from the NICE Methods Guide

5.3.4 The valuation of health-related quality of life measured in patients (or by their carers) should be based on a valuation of public preferences from a representative sample of the UK population using a choice-based method. This valuation leads to the calculation of utility values.

5.3.5 Different methods used to measure health-related quality of life produce different utility values; therefore, results from different methods or instruments cannot always be compared. Given the need for consistency across appraisals, one measurement method, the EQ-5D, is preferred for the measurement of health-related quality of life in adults.

National Institute for Health and Care Excellence. *Guide to the methods of technology appraisal*. London, NICE, April 2013. http://publications.nice.org.uk/pmg9

# GENERAL POPULATION VS. PATIENT PREFERENCES

**Arguments for using general population for preference elicitation:**

1. better represent taxpayers and potential patients
2. „veil of ignorance“, blind to their own self-interest (patients in conflict of interest)
3. Not enough patients
4. Patients unable to answer
5. Patients preferences cannot be used for comparisons across interventions

**Arguments for using patients for preference elicitation:**

1. experience: patients have a better understanding of what life is like in worse health states, while general population lack experience and their valuations are not theoretically justified.
2. The preference elicitation in general population is seen as more expensive, more difficult and time consuming

**Source of differences:** adaptation to disability and disease

# Hypothesis

- There **are** differences between patients and population preferences due to adaptation of patients to health states.

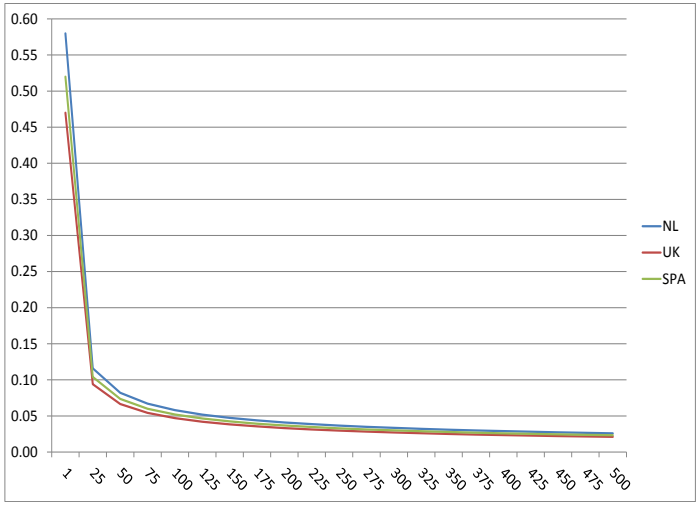
- Those who adapt to the health state (e.g. disease) attach higher utilities to the surrounding hypothetical health states compared to the general public.

# Methods

- Selection of instrument
- Selection of countries
- Selection of conditions/diagnoses
- Ethical approval ( 13 March 2015, Puerta de Hierro Majadahonda Hospital)
- Sample size
- Health states selection
- Mode of administration
- Design of EQ-VT software specifically for ADVANCE-HTA
- Patients recruitment process
- Training of the interviewers (January till August 2015)
- Interviews and database creation (20 August 2015)
- Statistical analysis

# Health state selection

| | STATE | SEVERITY | UK | SPA | NL | | STATE | SEVERITY | UK | SPA | NL |
|---|---|---|---|---|---|---|---|---|---|---|---|
| 1 | 21111 | 6 | 0.86 | 0.88 | 0.92 | 44 | 52215 | 15 | 0.40 | 0.37 | 0.21 |
| 2 | 11121 | 6 | 0.86 | 0.88 | 0.90 | 45 | 22434 | 15 | 0.51 | 0.36 | 0.31 |
| 3 | 11211 | 6 | 0.89 | 0.88 | 0.93 | 46 | 52431 | 15 | 0.54 | 0.35 | 0.48 |
| 4 | 11112 | 6 | 0.83 | 0.88 | 0.91 | 47 | 15151 | 13 | 0.43 | 0.35 | 0.22 |
| 5 | 12121 | 7 | 0.82 | 0.86 | 0.85 | 48 | 12344 | 14 | 0.36 | 0.35 | 0.21 |
| 6 | 12111 | 6 | 0.86 | 0.84 | 0.92 | 49 | 11425 | 13 | 0.51 | 0.35 | 0.24 |
| 7 | 11212 | 7 | 0.81 | 0.83 | 0.88 | 50 | 21444 | 15 | 0.22 | 0.35 | 0.14 |
| 8 | 11221 | 7 | 0.84 | 0.81 | 0.86 | 51 | 35332 | 16 | 0.58 | 0.33 | 0.59 |
| 9 | 12112 | 7 | 0.79 | 0.81 | 0.83 | 52 | 53243 | 17 | 0.27 | 0.33 | 0.19 |
| 10 | 11122 | 7 | 0.81 | 0.80 | 0.93 | 53 | 43315 | 16 | 0.44 | 0.32 | 0.23 |
| 11 | 21112 | 7 | 0.85 | 0.80 | 0.86 | 54 | 21345 | 15 | 0.40 | 0.32 | 0.10 |
| 12 | 13122 | 9 | 0.80 | 0.74 | 0.81 | 55 | 31525 | 16 | 0.46 | 0.32 | 0.31 |
| 13 | 11421 | 9 | 0.67 | 0.70 | 0.63 | 56 | 54231 | 15 | 0.42 | 0.32 | 0.54 |
| 14 | 13313 | 11 | 0.71 | 0.66 | 0.75 | 57 | 12244 | 13 | 0.36 | 0.31 | 0.23 |
| 15 | 14113 | 10 | 0.65 | 0.65 | 0.66 | 58 | 43542 | 18 | 0.29 | 0.31 | 0.08 |
| 16 | 25222 | 13 | 0.58 | 0.62 | 0.61 | 59 | 43514 | 17 | 0.36 | 0.30 | 0.16 |
| 17 | 12334 | 13 | 0.47 | 0.60 | 0.39 | 60 | 45233 | 17 | 0.38 | 0.29 | 0.33 |
| 18 | 23242 | 13 | 0.47 | 0.60 | 0.39 | 61 | 34244 | 17 | 0.28 | 0.27 | 0.02 |
| 19 | 12513 | 12 | 0.65 | 0.59 | 0.62 | 62 | 24443 | 17 | 0.34 | 0.25 | 0.08 |
| 20 | 11414 | 11 | 0.44 | 0.56 | 0.39 | 63 | 42115 | 13 | 0.45 | 0.25 | 0.32 |
| 21 | 32314 | 13 | 0.54 | 0.56 | 0.43 | 64 | 52335 | 18 | 0.38 | 0.24 | 0.12 |
| 22 | 21334 | 13 | 0.50 | 0.55 | 0.41 | 65 | 34515 | 18 | 0.34 | 0.24 | 0.10 |
| 23 | 23514 | 15 | 0.46 | 0.55 | 0.31 | 66 | 45413 | 17 | 0.35 | 0.23 | 0.36 |
| 24 | 25122 | 12 | 0.55 | 0.55 | 0.60 | 67 | 34155 | 18 | 0.29 | 0.22 | -0.08 |
| 25 | 25331 | 14 | 0.55 | 0.54 | 0.60 | 68 | 53244 | 18 | 0.18 | 0.21 | 0.06 |
| 26 | 31514 | 14 | 0.42 | 0.54 | 0.34 | 69 | 55225 | 19 | 0.23 | 0.20 | 0.08 |
| 27 | 34232 | 14 | 0.56 | 0.51 | 0.61 | 70 | 51451 | 16 | 0.30 | 0.19 | 0.10 |
| 28 | 13224 | 12 | 0.52 | 0.49 | 0.48 | 71 | 44125 | 16 | 0.39 | 0.17 | 0.14 |
| 29 | 42321 | 12 | 0.59 | 0.49 | 0.69 | 72 | 51152 | 14 | 0.33 | 0.15 | 0.22 |
| 30 | 24342 | 15 | 0.39 | 0.49 | 0.25 | 73 | 24553 | 19 | 0.33 | 0.15 | 0.14 |
| 31 | 35311 | 13 | 0.54 | 0.49 | 0.65 | 74 | 55233 | 18 | 0.33 | 0.14 | 0.27 |
| 32 | 53221 | 13 | 0.62 | 0.48 | 0.60 | 75 | 14554 | 19 | 0.26 | 0.09 | -0.13 |
| 33 | 21315 | 12 | 0.54 | 0.47 | 0.42 | 76 | 45144 | 18 | 0.19 | 0.09 | 0.04 |
| 34 | 12543 | 15 | 0.35 | 0.45 | 0.13 | 77 | 24445 | 19 | 0.21 | 0.09 | -0.14 |
| 35 | 23152 | 13 | 0.40 | 0.42 | 0.28 | 78 | 35245 | 19 | 0.19 | 0.08 | -0.03 |
| 36 | 31524 | 15 | 0.43 | 0.42 | 0.30 | 79 | 54153 | 18 | 0.29 | 0.08 | 0.06 |
| 37 | 53412 | 15 | 0.49 | 0.41 | 0.46 | 80 | 54342 | 18 | 0.28 | 0.07 | 0.07 |
| 38 | 33253 | 16 | 0.43 | 0.41 | 0.27 | 81 | 44345 | 20 | 0.29 | 0.06 | -0.16 |
| 39 | 32443 | 16 | 0.32 | 0.40 | 0.17 | 82 | 44553 | 21 | 0.16 | 0.03 | -0.11 |
| 40 | 12514 | 13 | 0.45 | 0.40 | 0.39 | 83 | 55424 | 20 | 0.26 | 0.03 | -0.04 |
| 41 | 35143 | 16 | 0.30 | 0.39 | 0.32 | 84 | 52455 | 21 | 0.14 | -0.02 | -0.13 |
| 42 | 11235 | 12 | 0.53 | 0.37 | 0.38 | 85 | 43555 | 22 | 0.12 | -0.05 | -0.12 |
| 43 | 45133 | 16 | 0.40 | 0.37 | 0.40 | 86 | 55555 | 25 | 0.00 | -0.16 | -0.29 |




Using a standard error of 0.05 as a threshold, this would imply that as a minimum 125 observations per state are needed. The minimum was slightly raised to **150 observations per state**, which amounts to **300 respondents per disease area**, where each respondent values 10 states. Hence, **19 health states needed to be selected**.

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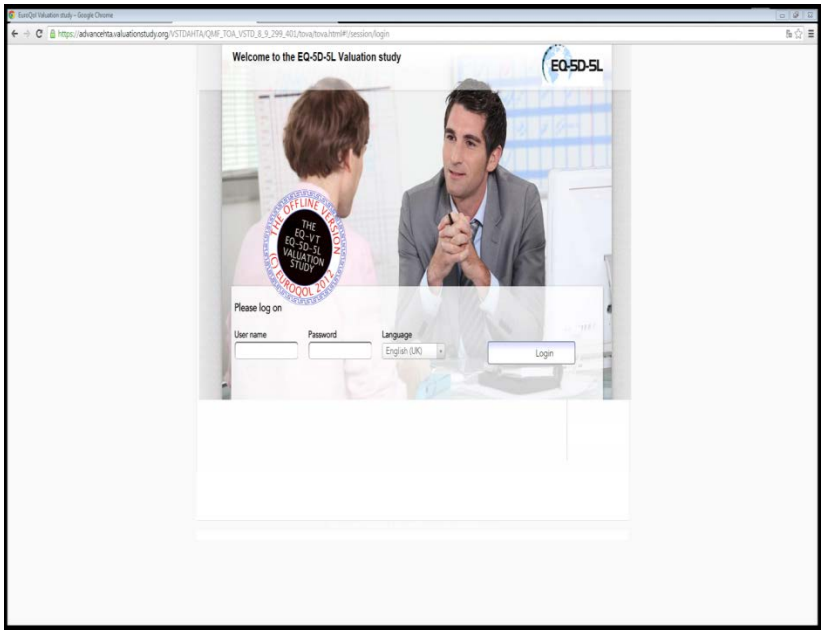
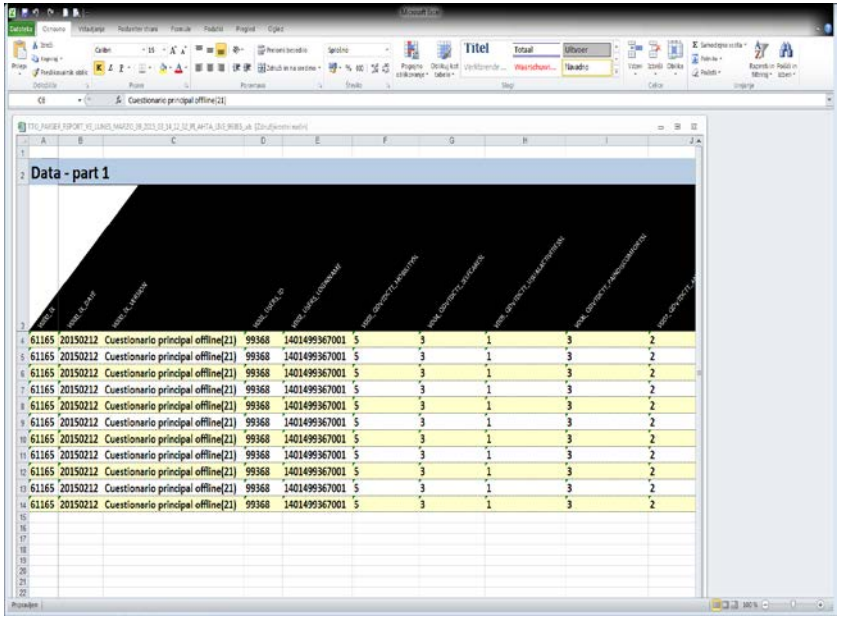
# Software

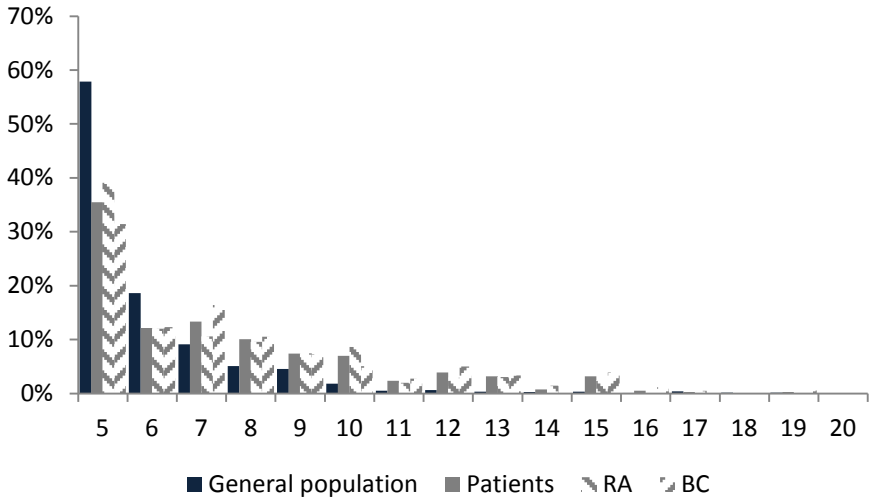


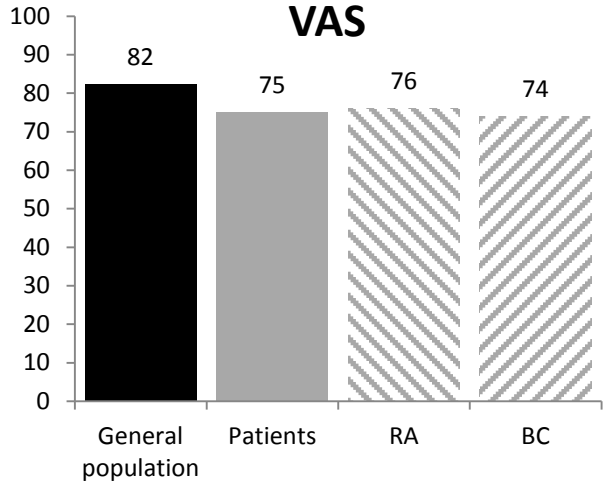




EQ-VT software specifically designed for ADVANCE-HTA.
The data analysis was generated using R (R Development Core Team, 2009)

# Results

| | MOBILITY | SELF-CARE | USUAL ACTIVITIES | PAIN / DISCOMFORT | ANXIETY / DEPRESSION |
|---|---|---|---|---|---|
| No problems | -17.4% | -17.1% | -29.2% | -20.6% | -24.5% |
| Slight | 10.3% | 12.0% | 20.5% | 12.2% | 15.8% |
| Moderate | 7.0% | 5.4% | 8.5% | 9.1% | 8.5% |
| Severe | 0.1% | 0.0% | 0.1% | -0.6% | 0.3% |
| Extreme | 0.1% | -0.2% | 0.1% | 0.0% | -0.1% |

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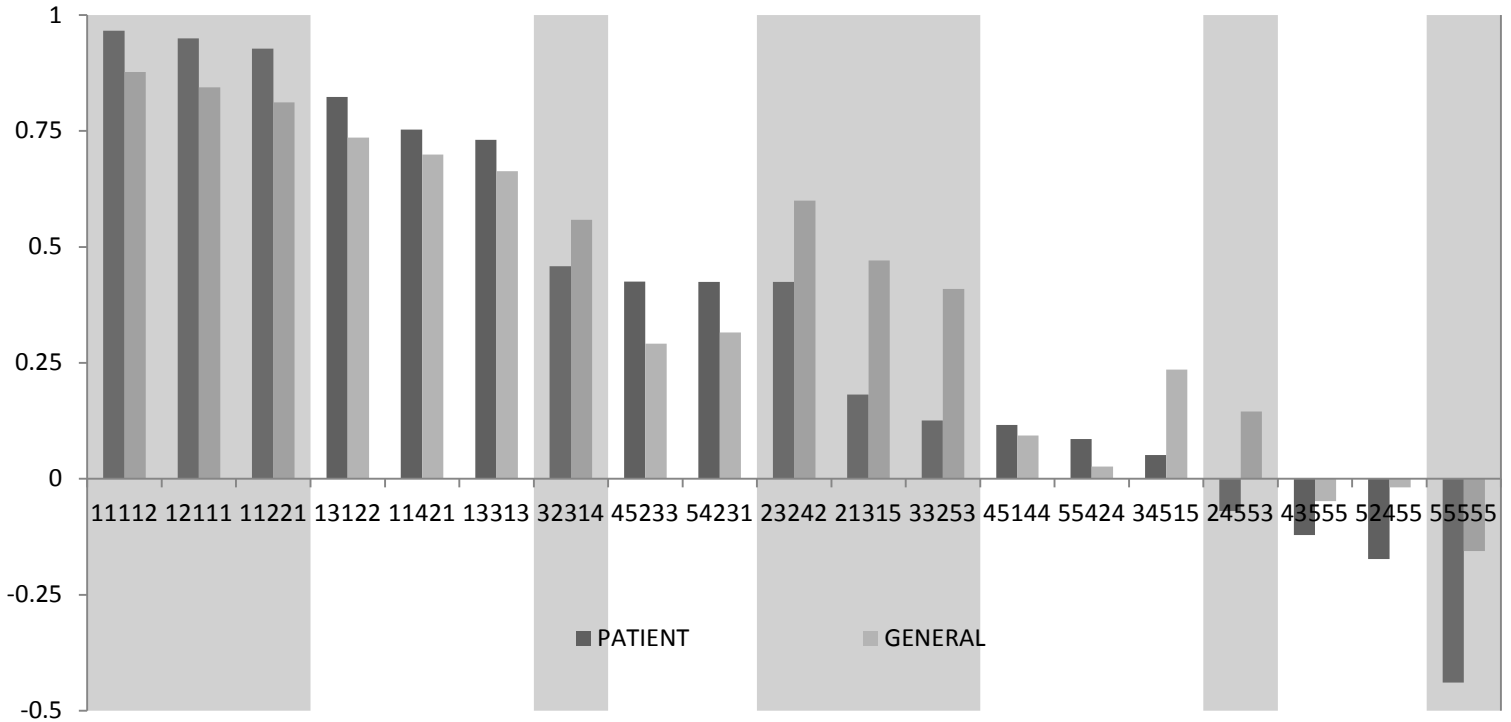
# Are there differences?

| | G : P | Ra : Bc | G : Ra | G : Bc |
|---|---|---|---|---|
| 11112 | ✓ | ● | ✓ | ✓ |
| 12111 | ✓ | ● | ✓ | ✓ |
| 11221 | ✓ | ● | ✓ | ✓ |
| 13122 | ● | ● | ● | ● |
| 11421 | ● | ● | ● | ● |
| 13313 | ● | ● | ● | ● |
| 21315 | ✓ | ● | ✓ | ✓ |
| 23242 | ✓ | ● | ✓ | ✓ |
| 32314 | ✓ | ● | ● | ✓ |
| 54231 | ● | ● | ● | ● |
| 33253 | ✓ | ● | ✓ | ✓ |
| 45233 | ● | ● | ● | ● |
| 34515 | ✓ | ● | ● | ✓ |
| 45144 | ● | ● | ● | ● |
| 24553 | ✓ | ● | ✓ | ✓ |
| 55424 | ● | ● | ● | ● |
| 52455 | ● | ● | ● | ● |
| 43555 | ● | ● | ● | ● |
| 55555 | ✓ | ● | ✓ | ✓ |

Findings:

1. No differences between patient groups
2. (10/19) Statistically significant Differences between general population and patient groups

# Do the preferences differ between the general population and defined patient groups?



# What are the causes of the differences?

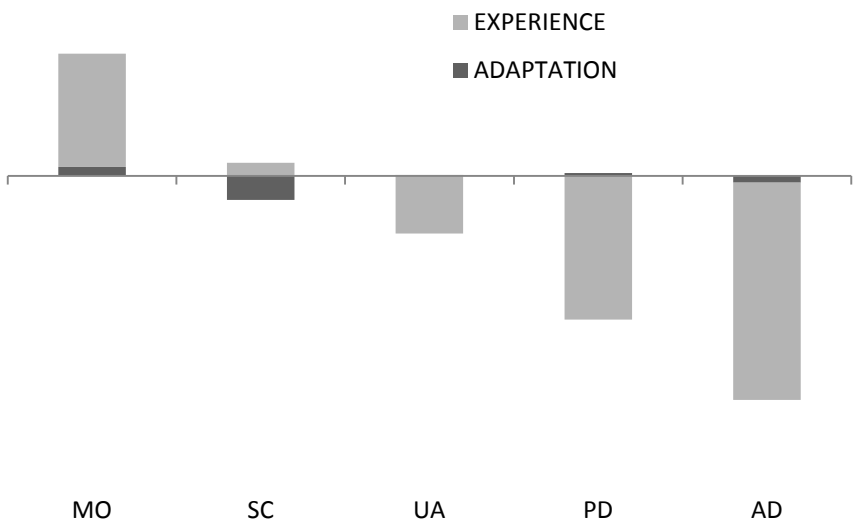
1. Significant differences on three health dimensions:
   - mobility,
   - pain or discomfort,
   - anxiety or depression.

- Mobility ( increase in mobility severity led to a reduction in QoL: this reduction is perceived to be **lower** by patients compared to general population)

- Pain/Discomfort and Anxiety/Depression (increase in PD and AD severity led to a reduction in QoL, which is perceived to be **higher** by patients compared to general population)

- The values that patients attached to hypothetical health states are affected by their own health state, although the explanatory power of this variable explained only additional 1% of the variation in TTO score. The adaptation was only indicated on Self Care and Anxiety/Depression dimensions.

# To sum up: What are the causes of the differences?




1. Differences exist because patients have experience with the health states
2. Significant differences on three health dimensions:
   - mobility,
   - pain or discomfort,
   - anxiety or depression.
3. Effects of adaptation on hypothetical health states valuation are trivial.

# Conclusion: Shall patient preferences be used for QALY calculation?

- **Adaptation** in the case of HRQoL no longer possess a normative problem, as the effects of adaptation on hypothetical health states valuation are **trivial**

- It seems that a "**veil of ignorance**" is **too thick**
  - differences in preferences suggest that patients are able to more accurately imagine **"non-tangible"** dimensions of health states (anxiety or depression and pain or discomfort)

- The arguments to use general population preferences (not enough patients, non comparability across the diseases) seems not to be problematic as lack of differences between patient groups suggests **that patients, regardless of the disease, have similar preferences**, yet different to general population.

- Eliciting patient preferences is **far more expensive and time consuming** than eliciting preferences from general population (ethical approval, patient recruitment, experienced interviewers).

# Limitations of the study

- **More health states need to be valued in** order to estimate the preferences for all EQ-5D-5L health states
- The **quality** of general population preferences elicitation needs to be assured prior to patient preference elicitation exercise
- The study concluded that there are no differences among both disease areas – **more disease areas** should be tested before this conclusion is generalized.
- The study was only carried out in one country; **more countries** need to be included to check the possible differences among countries.
- **Interviewer effect** could not be accounted for as interviewers were assigned interviews according to the disease areas (each interviewer performed interviews in only one disease area).
- Further studies are needed to address whether **mapping from general to patient population** is possible, especially due to the fact that eliciting preferences from patients is a demanding task.

# Policy implications

1. Patient preferences do differ from general population preferences which has an impact on priority setting.

2. A shift from using general population preferences to using patient preferences would result in **lower priority** given to health states/diseases where the problems are connected to

- Mobility dimension

and in **higher priority** given to health states /diseases where the problems are connected to

- Pain / Discomfort,
- Anxiety / Depression

Treatments of conditions that affect mobility would thus receive fewer fund and treatment of conditions that affect pain/discomfort and anxiety/depression would receive more funds.

**Thank you for your attention!**