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Mapping non-preference onto preference-based PROMs

Patient-reported outcomes measures (PROMs) in health economics

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RSS Primary Health Care Special Interest Group
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Outline of seminar

- What is meant by "Mapping"?
- Mapping studies in the literature and usage in health technology assessment
- Statistical methods to map non-preference to preference-based PROMs
  - Statistical modelling (direct vs indirect mapping)
  - Three case empirical mapping studies
- The MAPS reporting statement

What is meant by "Mapping"?

Non preference-based PROMs

(e.g. disease specific or generic questionnaire)

Algorithm

Preference-based PROMs

(e.g. EQ-5D-3L, EQ-5D-5L)

Source measure

Target measure

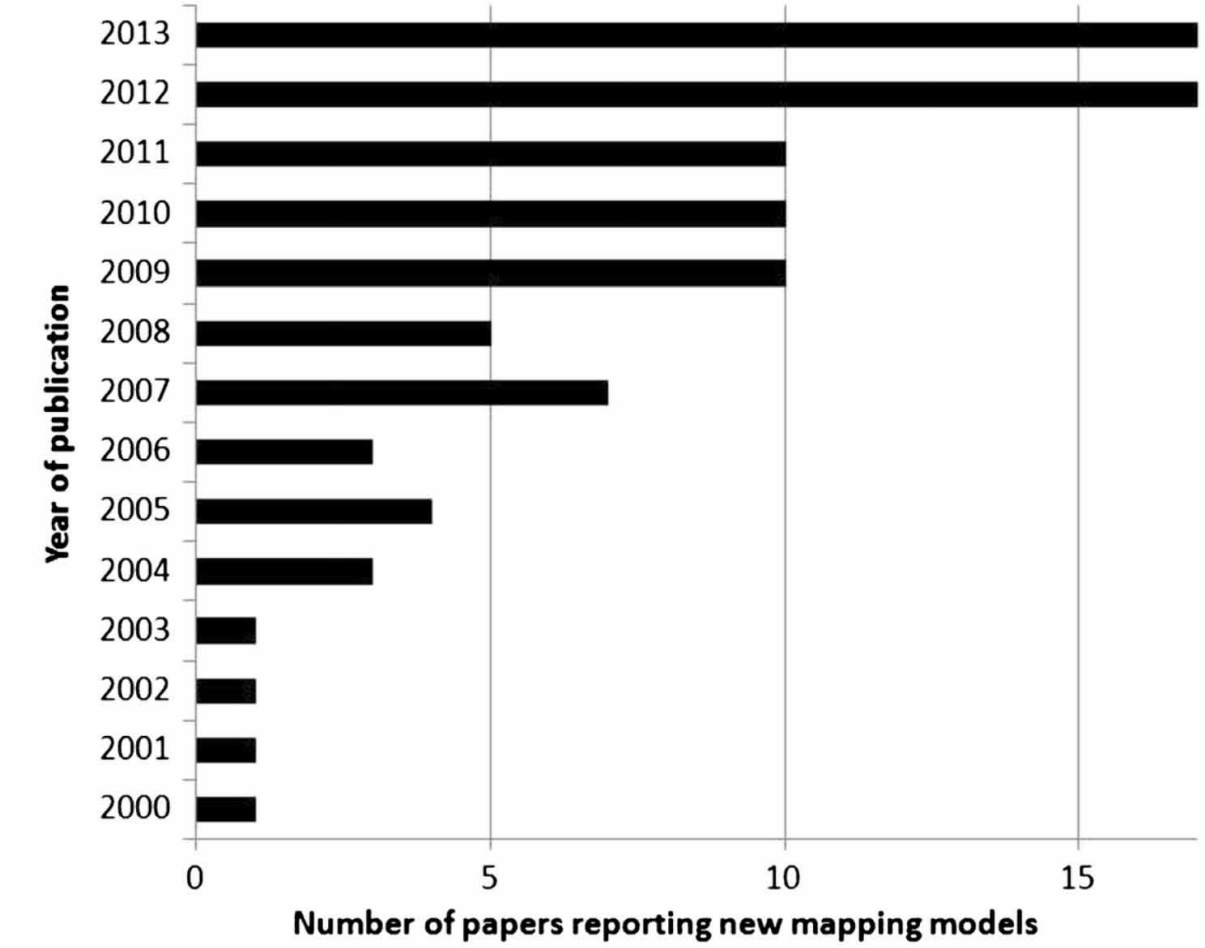
Algorithm: statistical association or more complex series of operations

Mapping in the published literature

- Brazier, J. E., Yang, Y., Tsuchiya, A. and Rowen, D. L. (2010). A review of studies mapping (or cross walking) non-preference based measures of health to generic preference-based measures. Eur J Health Econ; 11(2): 215-225.
  - Searches conducted from 1996-2007
  - Identified 30 studies.
  - Most common target measure was the EQ-5D-3L.
  - Comparisons across studies limited.

Mapping in the published literature

- Dakin, H. (2013). Review of studies mapping from quality of life or clinical measures to EQ-5D: an online database. Health Qual Life Outcomes; 11: 151.



Identified 90 studies reporting 121 mapping algorithms

The use of mapping in NICE technology appraisals

- Longworth, L. and Rowen, D. (2013). Mapping to obtain EQ-5D utility values for use in NICE health technology assessments. Value Health; 16(1): 202-210.

2004-2010	2004-2008	2008-2010
90 submissions	46 submissions	44 submissions
23 using mapping	19 using mapping	4 using mapping
25%	41%	9%

Steps to develop mapping algorithms

1. Rationale for the mapping study
2. Identification of source and target measures
3. Identification of estimation and external validation sample
4. Exploratory data analysis
5. Statistical modelling
6. Estimation of predicted scores or utilities
7. Validation methods
8. Measures of model performance
9. Dealing with uncertainty

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Statistical Modelling

Direct mapping

Indirect or response mapping

Statistical Modelling
Direct mapping

- Dependent variable using a preference-based score
  - EQ-5D-3L index has been widely used in direct mapping studies

Statistical Modelling
Direct mapping

Dependent variable
Vector of observations:
Overall score (e.g. EQ-5D-3L index)

Vector of parameters to be estimated

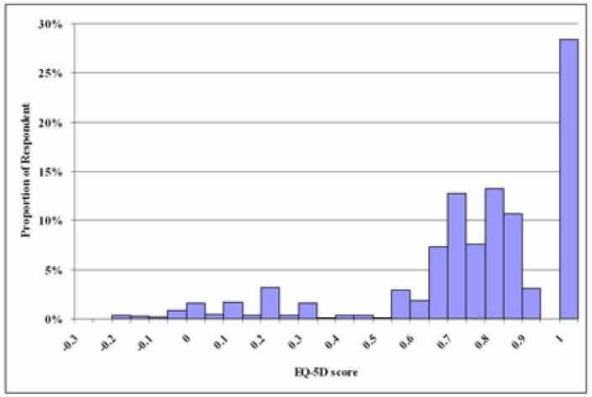
$$Y = X\beta + \epsilon$$

Matrix of predictor variables:
Condition-specific measures
Generic measures
Clinical measures
Sociodemographic variables
Other relevant data

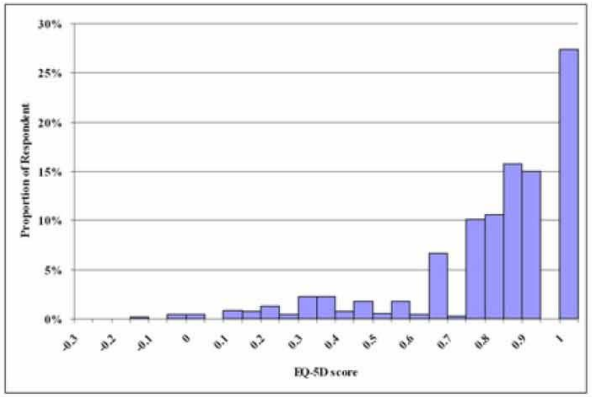
Vector of errors

Distribution of EQ-5D-3L values

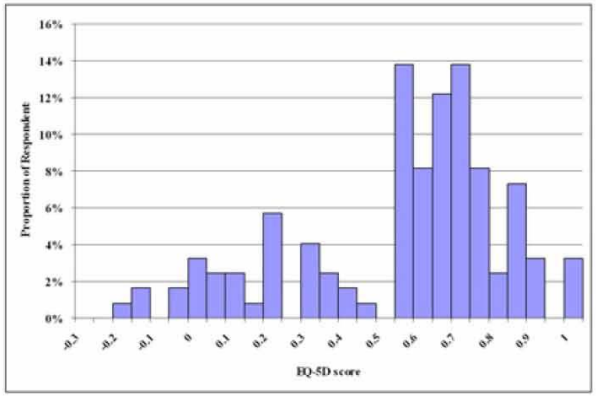
Asthma (n=2,935)



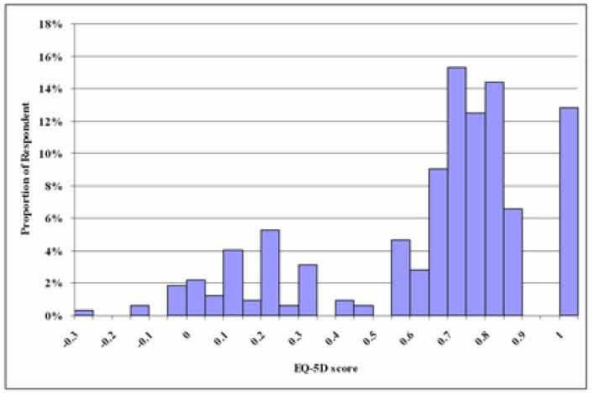
Chest pain (n=679)



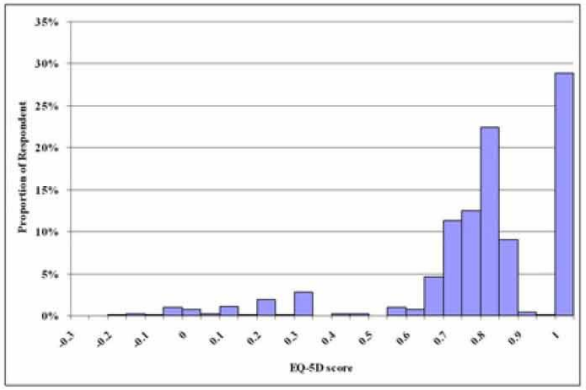
Cronic obstructive pulmonary disease (n=185)



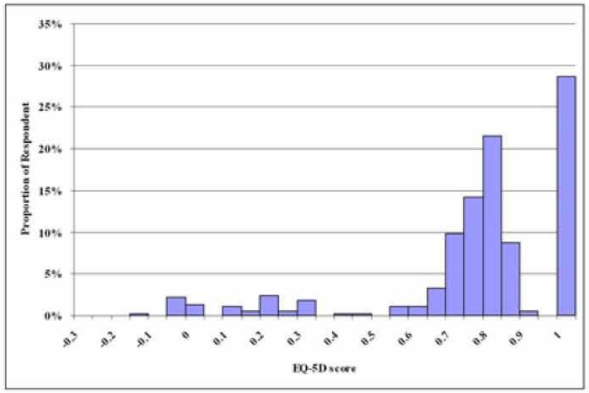
Clodronate (n=320)



Hormone replacement therapy (n=755)



Irritable bowel syndrome (n=374)



Source: Hernandez Alava, M., Wailoo, A. J. and Ara, R. (2012). Tails from the peak district: adjusted limited dependent variable mixture models of EQ-5D questionnaire health state utility values. Value Health; 15(3): 550-561.

Statistical Modelling
Indirect or response mapping

- Dependent variable using response variables rather than overall index
  - EQ-5D-3L responses have been widely used in response mapping
- Ordered and multinomial logit/probit models

Statistical Modelling
Indirect mapping (multinomial logit)

Dependent variable
Categorical variable
(e.g. EQ-5D-3L responses)

Predictor variables

Vector of parameters to be estimated

$$\Pr(y_i = m / x_i) = \frac{\exp(x_i \beta_m)}{\sum_{j=1}^{J} \exp(x_i \beta_j)}$$

Individual participant

Outcome of dependent variable (e.g. 1, 2 and 3 for the EQ-5D-3L

Levels of dependent variable (e.g. 1, 2 and 3 for the EQ-5D-3L

Statistical Modelling
Indirect or response mapping

- Dependent variable using response variables rather than overall index
  - EQ-5D-3L responses have been widely used in response mapping
- Ordered and multinomial logit models
- Probabilistic model and different methods available to calculate utility predictions:
  - Higher or most-likely probability - biased and not recommended
  - Expected value (equivalent to using an infinite number of Monte Carlo draws) – unbiased and recommended

3 case studies

Comparison of direct and indirect methods:

1. Mapping from Health Assessment Questionnaire (HAQ) to EQ-5D-3L
2. Mapping from Parkinson's Disease Questionnaire (PDQ-39) to EQ-5D-3L
3. Mapping from Oxford Hip Score (OHS) to EQ-5D-3L

What will be presented?

1. Mean (SD) of actual EQ-5D-3L in estimation and external validation dataset (if available)
2. Measures of prediction accuracy: mean squared error (MSE) or root mean squared error (RMSE)

HAQ to EQ-5D-3L

Hernandez-Alava et al 2014

	Estimation dataset (n = 100,398)	External validation dataset (n=n/a)
	Mean	**Mean**
Actual EQ-5D-3L index	0.665	n/a
	RMSE	**RMSE**
Direct mapping		
Simple linear regression	0.175	n/a
Adjusted limited mixture models	0.169	n/a
Indirect mapping		
Generalised ordered probit	0.171	n/a

n/a: not available

Source: Hernández Alava, M., Wailoo, A., Wolfe, F. and Michaud, K. (2014). A Comparison of Direct and Indirect Methods for the Estimation of Health Utilities from Clinical Outcomes. Medical Decision Making; 34(7): 919-930.

PDQ-39 to EQ-5D-3L

Kent et al 2015

	Estimation dataset (n = 9,123)	External validation dataset (n=719)
	Mean (SD)	**Mean (SD)**
Actual EQ-5D-3L index	0.60 (0.27)	0.51 (0.27)
	MSE	**MSE**
Direct mapping		
Simple linear regression	0.031	0.045
Adjusted limited mixture models	0.031	0.044
Indirect mapping		
Multinomial logit model	0.030	0.044

Source: Kent, S., Gray, A., Schlackow, I., Jenkinson, C. and McIntosh, E. (2015). Mapping from the Parkinson's Disease Questionnaire PDQ-39 to the Generic EuroQol EQ-5D-3L: The Value of Mixture Models. Med Decis Making. Online First

OHS to EQ-5D-3L
Work-in-progress (Oxford team)

	Estimation dataset (n = 51,800)	External validation dataset (n=75,322)
	Mean (SD)	**Mean (SD)**
Actual EQ-5D-3L index	0.558 (0.356)	0.561 (0.355)
	MSE	**MSE**
Direct mapping		
Simple linear regression	0.033	0.033
Two-part model	0.033	0.032
Adjusted limited mixture models	0.024	0.035
Indirect mapping		
Multinomial logit model	0.032	0.032

Direct versus indirect mapping

- There is no consensus about which method is preferable
- Evidence seems to suggest that overall both approaches are similar in terms of prediction accuracy
  - Differences observed favouring one method cannot be generalised to all instrument and patient populations
- Indirect mapping has some attractive properties:
  - Preserves logic of utility instruments such as EQ-5D
  - Provides more descriptive information than direct mapping
  - Compatible with different country-specific tariff sets

Additional statistical challenges ahead

- Performance of methods deteriorates as health states decline
- Does using more complex models (e.g. mixture models, Bayesian networks) improve performance of both direct and indirect methods?
- Need of better methods to deal with uncertainty
- Guidance on appropriate validation of mapping algorithms in practice

Overall we need to improve the reporting of these studies

MAPS reporting statement

- **MAPS statement:** MApping onto Preference-based measures reporting Standards
- **Objective:** to develop a checklist to promote complete and transparent reporting by researchers
- **Methods:** two-round Delphi survey with 48 representatives from academia, consultancy, HTA, and journal editors
- **Results:** a set of 23 essential reporting items was developed

MAPS reporting statement

Title and abstract
Item 1: Title
Item 2: Abstract
Introduction
Item 3: Study Rationale
Item 4: Study Objective
Methods
Item 5: Estimation Sample
Item 6: External Validation Sample
Item 7: Source and Target Measures
Item 8: Exploratory Data Analysis
Item 9: Missing Data
Item 10: Modelling Approaches
Item 11: Estimation of Predicted Scores or Utilities
Item 12: Validation Methods
Item 13: Measures of Model Performance

Results
Item 14: Final Sample Size(s)
Item 15: Descriptive Information
Item 16: Model Selection
Item 17: Model Coefficients
Item 18: Uncertainty
Item 19: Model Performance and Face Validity
Discussion
Item 20: Comparisons with Previous Studies
Item 21: Study Limitations
Item 22: Scope of Applications
Other
Item 23: Additional Information

For each item examples of good reporting practice, an explanation and the rationale and relevant evidence is provided

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Conclusions

- Mapping algorithms to translate non preference onto preference-based PROMs are available
  - HOWEVER, collection of primary data with the preferred utility instrument is desirable (mapping as second-best)
- Statistical methods have been evaluated to understand direct and indirect methods
  - No consensus in the literature
  - Additional statistical challenges ahead
- The development of the MAPS statement should improve the reporting (and quality?) of this studies in the future