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The Use of Conjoint Analysis to Elicit Patient Preferences in Selecting Treatment Endpoints

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Integrating Stakeholder Preferences in
Comparative Effectiveness Research

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Comparative Effectiveness Research

- Compares the benefits and harms of alternative interventions
- Assists patients, physicians, and regulators to make informed decisions

Institute of Medicine, 2009

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Comparisons for whom?

- Comparing benefits and harms and making informed decisions requires identifying relevant endpoints
- Increased concern about patient involvement in protocol development
- *"When asking the public to assist in determining health priorities, we should use techniques that allow people to reveal their true preferences. If not, why bother asking them at all?"* Gafni, Social Science and Medicine, 1995

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Types of Self-Reported Data

	Patient-Reported Outcomes	Health-State Utilities	Stated Preferences

	Patient-Reported Outcomes	Health-State Utilities	Stated Preferences
Elicitation Formats	Likert Scale	Standard Gamble/Time Tradeoff	Discrete Choice
Example Instruments	SF-36	EQ-5D Tariffs	Tailored
Metrics	HRQoL Scores	QALYs	Preference Weights, HTE, MAR, MAB, WTP
Uses	CEA, licensing	CEA, reimbursement	Preference Weights, HTE, MAR, MAB, WTP

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Health-State Utility versus Preference Utility: Determinants

HEALTH-STATE UTILITY

- Clinical outcomes
- Duration

PREFERENCE UTILITY

- Clinical Outcomes
- Duration
- Treatment factors
  - Side Effects/Tolerability
  - Dosage Method/Frequency
  - Cost
- Process factors
  - Health-Care Setting
  - Physician interactions

- Personal factors
  - Age, gender, education, etc.
  - Health history
  - Financial circumstances

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Labels

- Conjoint (**con**sider **joint**ly) analysis
- Discrete-choice experiments
- Stated-choice surveys

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Choice-Experiment Methods

- Treatment alternatives consist of combinations of features.
- Preferences among treatment alternatives depend on the relative importance of features.
- Respondents state preferences for series of constructed, hypothetical treatment alternatives.
- Statistical model estimates preference weights consistent with observed choices.
- Preference weights quantify relative importance as the willingness to accept tradeoffs.

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Example Benefit-Risk Tradeoff Question
Osteoarthritis

Which treatment would you choose if these were the only options available?

Feature	Treatment A	Treatment B
Efficacy--PAIN	Image of a line scale with a	Image of a line scale with a

Feature	Treatment A	Treatment B
	range of no pain to extreme pain—a red arrow marks the scale at roughly 3 out of 10.	range of no pain to extreme pain—a red arrow marks the scale at 0.
Efficacy--STIFFNESS	Image of a line scale with a range of no stiffness to extreme stiffness—a red arrow marks the scale at 0.	Image of a line scale with a range of no pain to extreme pain—a red arrow marks the scale at roughly 7 out of 10.
Mild-Moderate Side Effects--STOMACH PROBLEMS	Occasional mild symptoms. Treat with over-the-counter medications.	Frequent moderate symptoms. Treat with prescription medications.
Serious Side-Effect Risks--RISK OF BLEEDING ULCER	1 patient out of 100 (1%) will have a bleeding ulcer.	5 patients out of 100 (5%) will have a bleeding ulcer.
Serious Side-Effect Risks--RISK OF HEART ATTACK or STROKE	5 patients out of 100 (5%) will have a stroke.	15 patients out of 100 (15%) will have a heart attack.

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Why are T2DM patients inadherent?

Glucose Control	Base Model	Full model
"Best"	1.000	1
"Satisfactory"	0.734	0.721

Δ = +0.28

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Why are T2DM patients inadherent?

	Base Model	Full model
Glucose Control--"Best"	1.000	1
Glucose Control--"Satisfactory"	0.734	0.721
Number of Injections--1/day	0.599	0.885
Number of Injections--2/day	0.255	0.281

Glucose control--Δ = +0.28
Number of injections--Δ = -0.61

Hauber AB, Mohamed AF, Johnson FR, Falvey H. Treatment preferences and medication adherence of people with type 2 diabetes using oral glucose-lowering agents. Diabet Med. 2009;26:416-24.

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Physician Versus Patient Preferences
Hepatitis B

Mean relative importance	German Patients	German Physicians	Turkish Patients	Turkish Physicians
How long the medication has been studied (years)	3.3	2.7	10.0	4.0
Probability viral load is undetectable	8.2	10.0	5.6	6.9
5-year treatment – related risk of a fracture	5.0	3.9	3.4	3.8
5-year treatment – related risk of a renal failure	10.0	5.9	6.8	10.0

Lescrauwaet B, Mohamed AF, Johnson FR, Hauber AB. Do patients and physicians have similar preferences for health care decisions involving uncertain outcomes for chronic hepatitis B in Germany and Turkey? Poster presented at the International Society for Pharmacoeconomics and Outcomes Research 16th Annual International Meeting; May 2011. Baltimore, MD.

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Highlighted data: 5-year treatment – related risk of a renal failure	10.0	5.9	6.8	10.0

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How long the medication has been studied (years)	3.3	2.7	10.0	4.0	German patients, German physicians Turkish patients, Turkish physicians
Probability viral load is undetectable	8.2	10.0	5.6	6.9	Most important: Renal toxicity, Efficacy, Weight of evidence, Renal toxicity

Mean relative importance	German Patients	German Physicians	Turkish Patients	Turkish Physicians	Notes:
5-year treatment – related risk of a fracture	5.0	3.9	3.4	3.8	Least important: Weight of evidence Weight of evidence Fracture risk Fracture Risk
5-year treatment – related risk of a renal failure	10.0	5.9	6.8	10.0	

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Table

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Maximum Acceptable Risk Calculation
Renal Cell Carcinoma

Image: Bar chart showing 3 month, 5-month, and 10-month progression-free survival rates and chance of liver failure (no data points).

Wong MK, Mohamed AF, Hauber AB, Yang J-C, Liu Z, Rogerio J, et al. Patients rank toxicity against progression-free survival in second-line treatment of advanced renal cell carcinoma. J Med Econ. 2012 Jul 3. doi: 10.3111/13696998.2012.708689. [Epub ahead of print].

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Maximum Acceptable Risk Calculation
Renal Cell Carcinoma

Image: Bar chart showing 3 month, 5-month, and 10-month progression-free survival rates and chance of liver failure (no data points).

There is a dashed line across 3-months and 10-months with an arrow pointing upward (from 5 months to 10 months) with the equation: $\Delta = +0.84$.

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There is a dashed line across 3-months and 10-months with an arrow pointing upward (from 5 months to 10 months) with the equation: $\Delta = +0.84$.

There is another dashed line from 0.0% to 2.0% with an arrow pointing downward on the 2.0 bar on the chance of liver failure bars with the equation: $\Delta = -0.84$

Wong MK, Mohamed AF, Hauber AB, Yang J-C, Liu Z, Rogerio J, et al. Patients rank toxicity against progression-free survival in second-line treatment of advanced renal cell carcinoma. J Med Econ. 2012 Jul 3. doi: 10.3111/13696998.2012.708689. [Epub ahead of print].

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Maximum Acceptable Breast-Cancer Risk
Vasomotor Symptoms

Image: Bar chart with 3 sets of bars for (1) Severe to moderate symptoms, (2) Severe to mild symptoms, and (3) Severe to no symptoms. Each set has a bar for: absolute risk and relative risk. There are no data points.

Johnson FR, Ozdemir S, Hauber AB, Kauf T. Women's willingness to accept risk for perceived vasomotor symptom relief. J Womens Health. 2007;16(7):1028-40.

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Maximum Acceptable Breast-Cancer Risk
Vasomotor Symptoms

Image: Bar chart with 3 sets of bars for (1) Severe to moderate symptoms, (2) Severe to mild symptoms, and (3) Severe to no symptoms. Each set has a bar for: absolute risk and relative riskThere is a dashed line labeled WHI Risk across all bars. There are no data points.

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- Some Methodological Challenges
  Hypothetical bias
    - Inexperience with condition
    - Socially acceptable responses
    - Stated preference/revealed preference experiments
- Cognitive challenges
    - Effective description of clinical endpoints
    - Surrogate markers
    - Risk concepts
- Consensus among researchers
    - Experimental design

Statistical analysis

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Discussion

- Effective incorporation of patient perspectives in protocol development requires quantification.
- Idea of treating patient-preference measures as evidence is novel for most clinicians.
- DCE methods offer methods for quantifying relative values of health endpoints.

Good validity and reliability for relatively simple trade-off problems. Applications to more difficult problems is an active area of research.