Brief Overview of Patient Preference Information (PPI)

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(Disclosures on file)

Food and Drug Administration (FDA) Committee, March 24, 2021

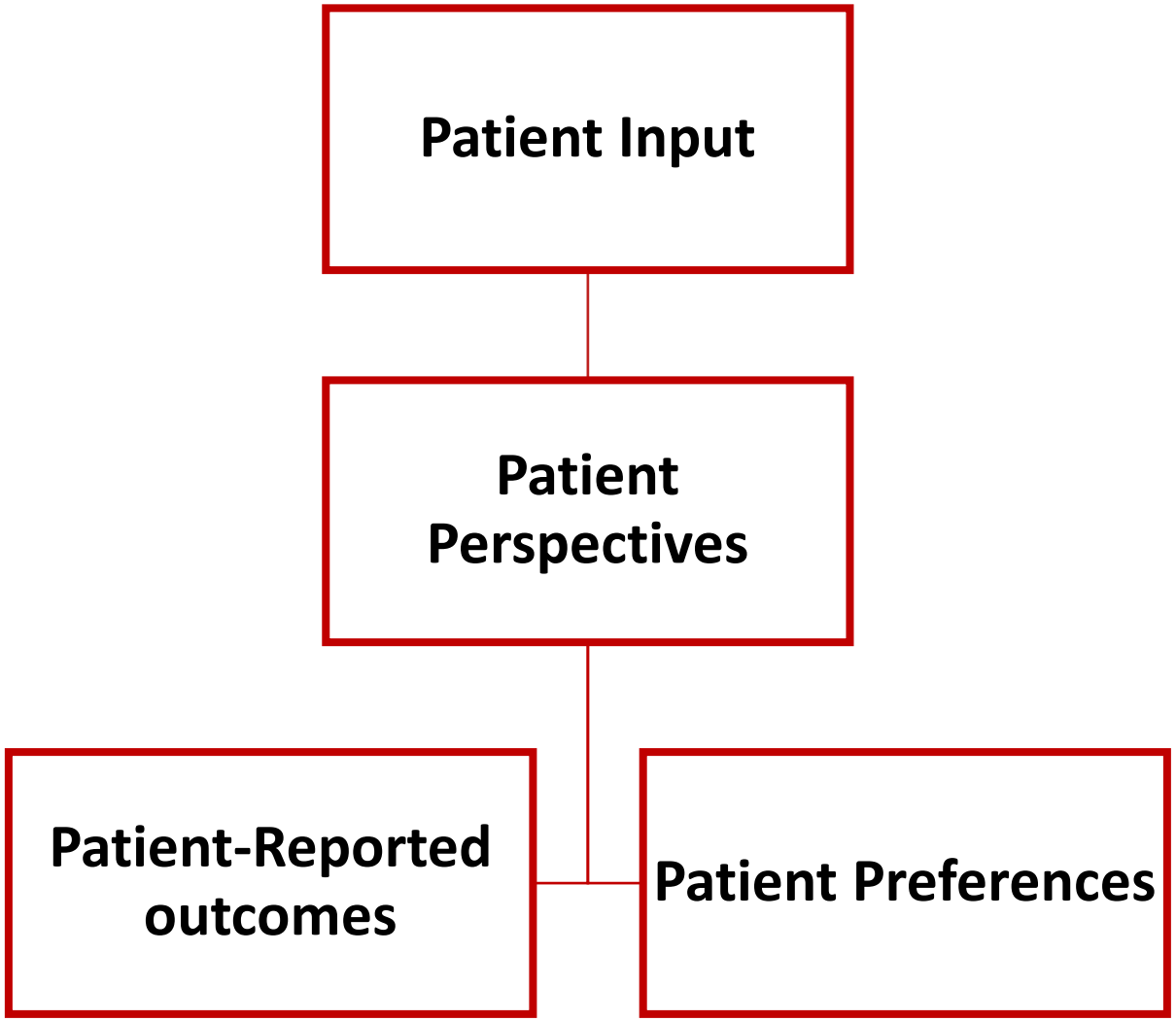
Overview

1) Why Patient Preference Information (PPI)?
2) What is PPI, What Does PPI Measure and Methods for PPI?
   - Best Worst Scaling (BWS) Object Case 1 for Prioritisation
   - Discrete Choice Experiments (DCEs) (forced choice) for Trade-offs
3) Analysis and Interpretation of Results from Preferences Studies
4) Good Research Practices For Preference-Based Methods

Why Patient Preferences?
Patient-Centeredness Through Patient Input

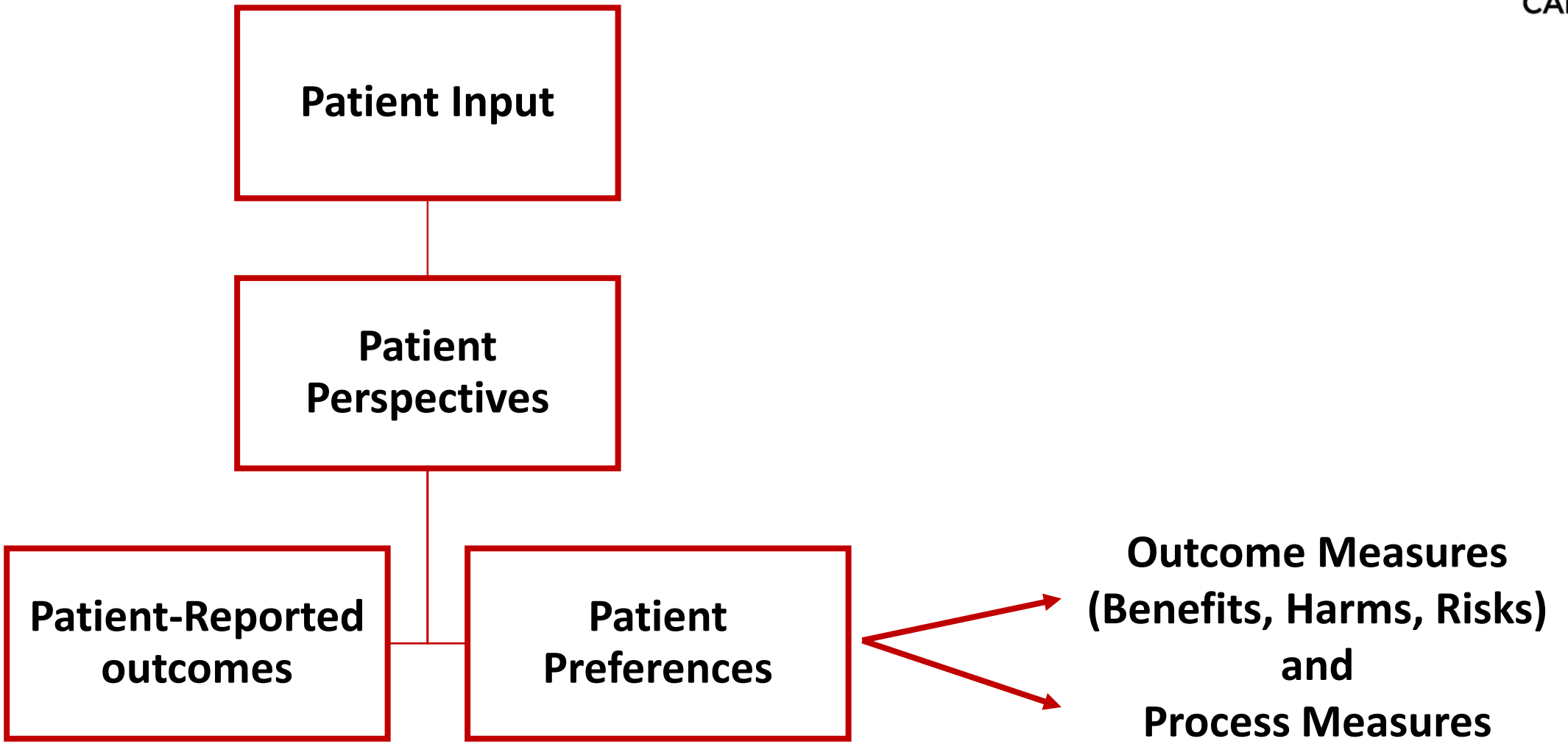
- …committed to hearing, understanding and integrating patients' perspective in regulatory decision making as appropriate

- …considers "valid scientific evidence" when conducting benefit-risk assessment, including nonclinical and clinical investigations **and Patient Preference Information (PPI) studies"**



FDA Final Patient Preference Guidance Document. August 24, 2016. https://www.fda.gov/media/92593/download;
Benz H, Saha A, Tarver M. Integrating the Voice of the Patient Into the Medical Device Regulatory Process…Value Health. 2020 Mar;23(3):294-297

What Matters to Patients?



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1) Why Patient Preference Information (PPI)?
2) **What is PPI, What Does PPI Measure and Methods for PPI?**
   - **Best Worst Scaling (BWS) Object Case 1 for Prioritisation**
   - **Discrete Choice Experiments (DCEs) for Trade-offs**
3) Analysis and Interpretation of Results from Preferences Studies
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What is Patient Preference Information (PPI)?

"Qualitative or quantitative assessments of the relative desirability or acceptability to patients, of features that differ among alternative health states, health interventions, or health services."

Desirability: *preferences for positive outcomes or features (benefits)*

Acceptability: *aversion to negative outcomes or features (harms or risks)*

FDA Final Patient Preference Guidance Document. August 24, 2016. https://www.fda.gov/media/92593/download

What Patient Preference Information (PPI) is Not?

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"Qualitative or quantitative assessments of the relative desirability or acceptability to patients of features that differ among alternative health states, health interventions, or health services."

PPI ≠ Patient Reported Outcomes (perception of health status)

PPI ≠ Shared Decision Making (collaborative decision process considering scientific evidence and patient values and preferences)

FDA Final Patient Preference Guidance Document. August 24, 2016. https://www.fda.gov/media/92593/download

Types of PPI and What PPI Measures

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Attributes	Relative Importance	Trade-offs Relative Importance
What matters	Order of what matters	How much it matters
Qualitative methods	Quantitative methods	Quantitative methods designed explicitly to capture trade-offs
Patient Priorities	Patient Priorities	Benefit-Risk Assessment Preference Heterogeneity

Study Design Complexity

http://mdic.org/mdicx/#archive;
Medical Device Innovation Consortium (MDIC). 2015 http://mdic.org/wpcontent/uploads/2015/05/MDIC_PCBR_Framework_Web.pdf

Multiple Methods to Elicit and Measure PPI

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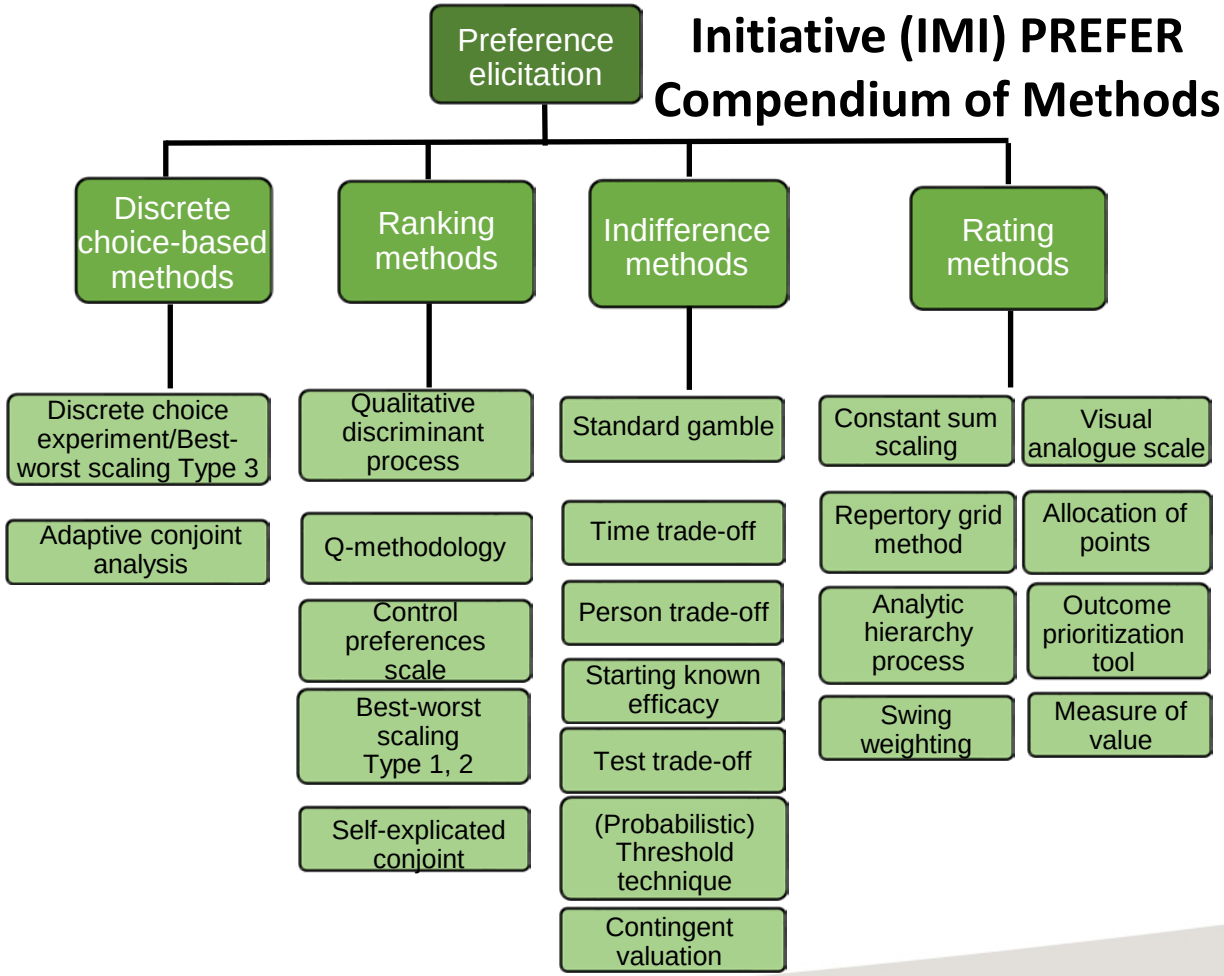
MDIC Catalogue of Methods

MDIC MEDICAL DEVICE INNOVATION CONSORTIUM

Group	Method
Structured-weighting	• Simple direct weighting • Ranking exercises • Swing weighting • Point allocation • Analytic hierarchy process • Outranking methods
Health-state utility	• Time tradeoff • Standard gamble
Stated-preference	• Direct-assessment questions • Threshold technique • Conjoint analysis and discrete-choice experiments • Best-worst scaling exercises
Revealed-preference	• Patient-preference trials • Direct questions in clinical trials

Innovative Medicines Initiative (IMI) PREFER Compendium of Methods



Medical Device Innovation Consortium (MDIC). 2015 http://mdic.org/wpcontent/uploads/2015/05/MDIC_PCBR_Framework_Web.pdf and http://mdic.org/mdicx/#archive;
Soekhai V, Shichello C, Levitan B, et al. Methods for exploring and eliciting patient preferences in the medical product lifecycle... Drug Discov Today, 2019;24(7):1324-31

Focus on Two Stated Preferences Methods: BWS Object Case 1 (Ranking) and DCE

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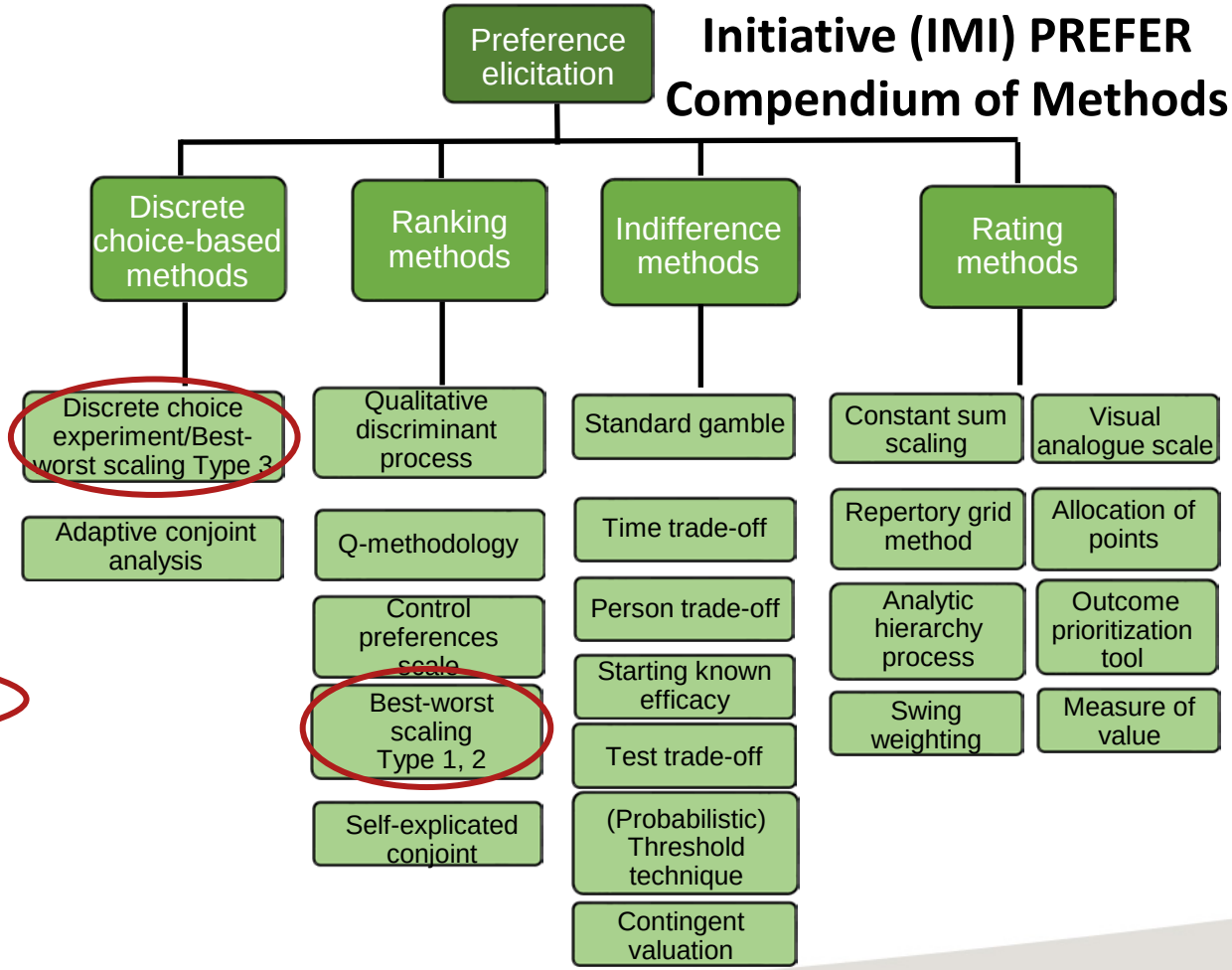

Medical Device Innovation Consortium (MDIC). 2015 http://mdic.org/wpcontent/uploads/2015/05/MDIC_PCBR_Framework_Web.pdf and http://mdic.org/mdicx/#archive;
Soekhai V, Shichello C, Levitan B, et al. Methods for exploring and eliciting patient preferences in the medical product lifecycle... Drug Discov Today, 2019;24(7):1324-31

What do BWS Object Case 1 (Ranking) and DCE Measure?

Attributes	Best Worst Scaling Object Case 1 (Ranking)	Discrete Choice Experiment Forced Choice
	Relative Importance	Trade-offs Relative Importance
What matters	Order of what matters	How much it matters
Qualitative methods	Quantitative methods	Quantitative methods designed explicitly to capture trade-offs
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1) Why Patient Preference Information (PPI)?
2) What is PPI, What Does PPI Measure and Methods for PPI?
3) **Analysis and Interpretation of Results from Preferences Studies**
   - **Best Worst Scaling (BWS) Object Case 1**
   - **Discrete Choice Experiments (DCEs)**
4) Good Research Practices For Preference-Based Methods

BWS Object Case 1 - Ranking

- Starting with list of “objects” (= attributes) (not choice profile with levels like DCEs)
- Choice tasks differ in the subset of attributes shown
- BWS object case is designed to determine the *relative importance* of attributes, such as:
  - Types of outcomes
  - Types of side effects
  - Attributes of treatment
- BWS comparatively easier than rating tasks (Ratcliffe et al.)

- *Ratcliffe J, Kaambwa B, Hutchinson C, et al. Patient. 2020;13(3):307-315; Cheung KL, Wijnen BFM, Hollin IL, et al. PharmacoEconomics 2016; 34:1195–1209;* Mühlbacher, A.C., Zweifel, P., Kaczynski, A. *et al. Health Econ Rev* 2016;**6,** 5; Flynn TN. Expert Rev Pharmacoecon Outcomes Res. 2010 Jun;10(3):259-67.

BWS Object Case 1: Choice Task Example

Question: What are most important attributes of a (non-surgical) management programme for osteoarthritis?

- **Design:** Balanced incomplete block experimental design (BIBD) so each attribute appears a specified number of times, and each pair co-occurs a specified number of times.
- From a set of 9 potential attributes, each choice task includes a subset of 3 attributes

Most important	Attributes of OA Management Programme	Least important
☑	Cost	☐
☐	Type of provider leading program	☐
☐	Travel time	☑

Check marks indicate choice of best (= most important) and worst (= least important)

Analysis and Interpretation of BWS Results

Attribute	Best count	Worst count	Best - Worst count	Rank
Type of provider	1589	195	1394	1
Travel time	1056	381	675	2
Cost	867	373	494	3
Attribute 4	867	442	425	4
Attribute 5	779	799	-20	5
Attribute 6	609	877	-268	6
Attribute 7	525	801	-276	7
Attribute 8	301	1278	-977	8
Attribute 9	139	1586	-1447	9
Example n=500 Respondents				

- Pattern of choices provides data to estimate relative importance for all attributes
  - Count Analysis – (Best count – worst count) gives a ranking of importance implied by the ordering from highest to lowest
  - Conditional logit model where coefficients are interpretated as ranking implied by the ordering
- Can compare importance ranking across respondent groups

- Mühlbacher, A.C., Zweifel, P., Kaczynski, A. *et al. Health Econ Rev* 2016;6**, 5;** Mühlbacher, A.C., Kaczynski, A., Zweifel, P. *et al. Health Econ Rev* **6,** 2 (2016); Flynn TN. Expert Rev Pharmacoecon Outcomes Res. 2010 Jun;10(3):259-67.

Discrete Choice Experiment - Common Method for Eliciting and Quantifying Preferences and Trade-Offs

Attribute	Alternative 1	Alternative 2
Effective Response (Benefit)	9 out of 10 (90%) 	6 out of 10 (60%) 
Side Effects (Risk)	2 out of 10 (20%) 	1 out of 10 (10%) 
Route of Administration	Intravenous	Oral
Which would you prefer?	X	

- Two-alternative forced choice DCE

Discrete Choice Experiment - Common Method for Eliciting and Quantifying Preferences and Trade-Offs

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Attributes

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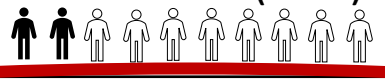
Side Effects (Risk)	2 out of 10 (20%) 	1 out of 10 (10%) 
Route of Administration	Intravenous	Oral
Which would you prefer?	X	

- Two-alternative forced choice DCE with 3 attributes
  - Effective Response (benefit)
  - Side Effects (risk)
  - Route of Administration

Discrete Choice Experiment - Common Method for Eliciting and Quantifying Preferences and Trade-Offs

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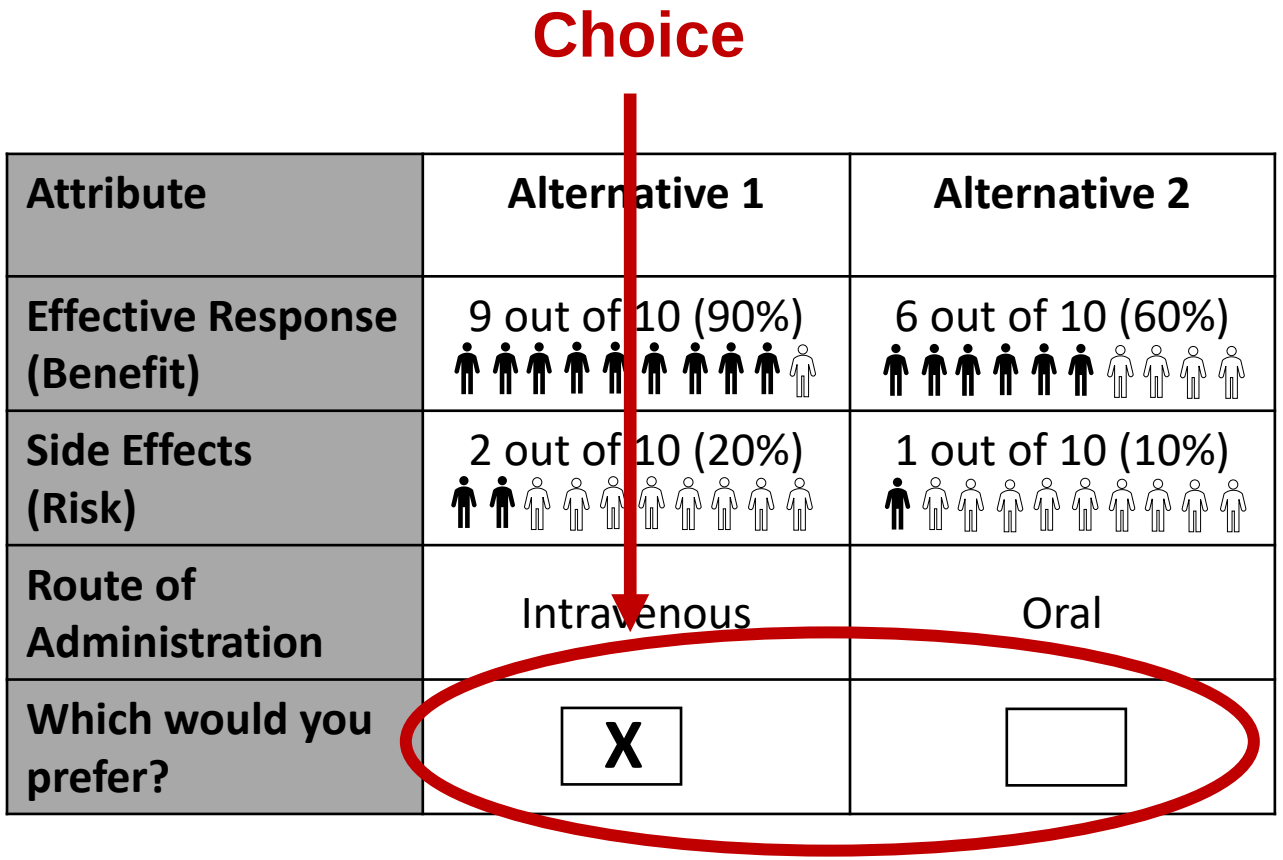
Attributes **Profile**

Attribute	Alternative 1	Alternative 2
Effective Response (Benefit)	9 out of 10 (90%) 	6 out of 10 (60%) 
Side Effects (Risk)	2 out of 10 (20%) 	1 out of 10 (10%) 
Route of Administration	Intravenous	Oral
Which would you prefer?	X	☐

- Profiles are constructed from attributes with varying levels
  - E.g. Effective response rate attribute with 3 levels
    - 9 out of 10
    - 7 out of 10 (not shown in this choice)
    - 6 out of 10
- Profiles combined into choice tasks
- Each choice task has a different set of profiles determined by an experimental design

Discrete Choice Experiment - Common Method for Eliciting and Quantifying Preferences and Trade-Offs



- Respondent asked to chose one alternative (forced choice) in each choice task
  - E.g. Alternative 1 is preferred to 2

Discrete Choice Experiment - Common Method for Eliciting and Quantifying Preferences and Trade-Offs

Attribute	Alternative 1	Alternative 2
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Route of Administration	Intravenous	Oral
Which would you prefer?	X	

Choice Task 3

Choice Task 2

Choice Task 1

- Each respondent completes a series of choice tasks as experimental design

DCE 'Opt-out' and Status-Quo DCE Formats to Reflect Realistic Context and Decisions: Two common formats

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1) Single response opt-out or status-quo

	Scenario A	Scenario B	Common Scenario
Reputation of Surgeon	Excellent reputation	Good reputation	Good reputation
Referral to Surgeon	Surgeon selected by you	Next available surgeon assigned from a list	Surgeon selected by your doctor
Your Wait Time to Surgeon Visit	12 months	1 month	6 months
Your Wait Time to Surgery After Deciding to Have Surgery	18 months	1 month	6 months
Your Travel Time to Hospital for Your Surgery and Follow Up	More than 1 hour	1 hour or less	1 hour or less
I would choose	☐	X	☐

2) Dual response none format: Forced choice task followed by an opt-out

	Scenario A	Scenario B
Reputation of Surgeon	Don't know reputation	Satisfactory reputation
Referral to Surgeon	Next available surgeon assigned from a list	Surgeon selected by you
Your Wait Time to Surgeon Visit	1 month	12 months
Your Wait Time to Surgery After Deciding to Have Surgery	6 months	18 months
Your Travel Time to Hospital for Your Surgery and Follow Up	More than 1 hour	1 hour or less
I would choose	☐	X

Suppose you have the option of choosing the *common situation* (presented in Page 10), would you still select the scenario you chose above?

X Yes, I would still prefer the scenario chosen above

☐ No, I would choose the common situation scenario

Opt-Out

Marshall DA, Deal K, Conner-Spady B, et al. Osteoarthritis and Cartilage 2018; 26:522-530

Deciding on Inclusion of Opt-Out Option and Format

- Why Include Opt-out Option?
  - To capture realistic options where non-participation is an existing alternative
  - Minimise biased estimates of preferences and utilities in analysis and overestimates of participation rates
- Choice for including opt-out or status-quo depends on research objective:
  - Include if related to participation rates (e.g. colorectal cancer screening) or uptake (e.g. new treatment)

- Marshall DA, Johnson FR, Kulin NA, et al. Health Econ 2009;18(12):1420-39; Marshall DA, Johnson FR, Phillips KA, et al. Value in Health 2007;10(5):415-30; Ryan M, Skåtun D. Modelling non-demanders in choice experiments. Health Econ. 2004; 13: 397-402; Determann D, Gyrd-Hansen D, de Wit GA, et al.Medical Decision Making 2019;39(6), 681–692.

Analysis and Interpretation of Results from DCE

Indirect Utility (stylized) is a function of the attributes that are being traded:

$$V = \alpha + \beta_1 X_1 + \beta_2 X_2 + \varepsilon \ldots$$

- Pattern of choices provides data for regression analysis to estimate β parameters (relative preference weight) for each attribute level
- Differences in preference weights reveal the impact of a change in attribute levels on utility
- We can estimate a variety of measures conditional on the attributes and range of attribute levels included in the DCE

Analysis and Interpretation of Results from DCE

Direction of preference	Positive/Negative Sign on β parameter (depending on coding)
Ordering of attribute levels	Relative value of β parameters
Relative importance of attribute	Total absolute difference in attribute level β parameters for attribute X / Sum of absolute difference in attribute level β parameters for all attributes
Value of change in attribute levels	Difference between attribute level β parameters ($\beta_1 - \beta_2$)
Trade-offs between changes in attribute levels (Marginal rates of substitution)	e.g. Marginal willingness to wait (WTW): $-\beta_k / \beta_{waiting\ time}$ At least one attribute continuous
Greatest increase in harm/risk for which a patient would accept a given benefit (Maximum acceptable risk - MAR)	Risk equivalent of a given benefit improvement Utility increase for given benefit improvement/ Utility decrease for 1% risk increase

- Ryan M, Gerard K, Amaya-Amaya M, Using Discrete Choice Experiments to Value Health and Health Care, Springer, 2008; Louviere J, Hensher DA, Swait J. Stated Choice Methods. Cambridge University Press, 2000; *Medical Device Innovation Consortium (MDIC). 2015* http://mdic.org/wpcontent/uploads/2015/05/MDIC_PCBR_Framework_Web.pdf

Interpretation of Preferences Results

Attribute	Level	β Parameter	Attribute Range	Relative Attribute Importance	Maximum Acceptable Risk (MAR)
Effectiveness (Positive Response)	10 6 3	0.9 0.1 -1.0	0.9 + 1.0 =1.9	1.9/3.4 *100%= 56%	Utility increase for improvement benefit = 0.9-0.1 = 0.8
Risk of Side Effects	1% 2% 5%	0.7 0.1 -0.8	0.7 +0.8 =1.5	1.5/3.4 *100%= 44%	Utility decrease for 1% risk increase = 0.7-0.1/ (2-1) =0.6

- Ordering: Higher effectiveness (β=0.9) is preferred to lower effectiveness (β=-1.0); Fewer side effects (β=0.7) are preferred to more side effects (β=-0.8)
- Relative importance: Marginal utility of improving effectiveness from 6 to 10 (4 points) = 0.9 – 0.1 = 0.8 = 0.2 per point; Marginal utility of reducing side effects from 2% to 1% = 0.7 – 0.1 = 0.6
- Compare changes in Benefits (effectiveness) and Risks (side effects): Willing to give up 3 points of effectiveness to reduce side effects from 2% to 1%
- Maximum Acceptable Risk (MAR) = Utility increase for given benefit improvement/ Utility decrease for 1% risk increase = 0.8/0.6 = 1.3; Patients would on average be willing to accept 1.3% increase in side effects for improving effectiveness from 6 to 10 (4 points)

Summary Comparison of BWS Object Case 1 and DCE

	Best Worst Scaling (BWS) Object Case 1	Discrete Choice Experiment (DCE)
Possible Outputs and Interpretation	• Rank Attributes • Relative Importance of Attributes	• Rank Attribute Levels • Relative Importance of Attribute Levels • Conditional Relative Importance Weights • Trade-offs amongst attributes • Identify differences in preferences in subgroups
Analysis	• Best worst count difference • Log square root ratio statistic • 'Scores' using normalized count difference or logistic regression	• Conditional logistic regression • Extensions of conditional logit (e.g. random parameters logit) • Latent Class analysis

1) Why Patient Preference Information (PPI)?
2) What is PPI, What Does PPI Measure and Methods for PPI?
3) Analysis and Interpretation of Results from Preferences Studies
4) **Good Research Practices For Preference-Based Methods**
   - **Recommended Qualities of PPI For Valid Scientific Evidence**
   - **Identification and selection of attributes and levels**
   - **Selection of study population – representativeness and generalisability**
   - **Minimising bias**

FDA Recommended Qualities of Patient Preference Studies to Generate Valid Scientific Evidence

- Relevant
- Scientifically valid
- Feasible

General Principals

1. Patient Centeredness
2. **Relevance (Benefits, Risks Harms, Uncertainty)**
3. **Good Research Practices**

Study Design

4. **Representativeness**
5. Logical Soundness
6. **Effective communication of benefit and risk**
7. Minimal cognitive bias

Technical

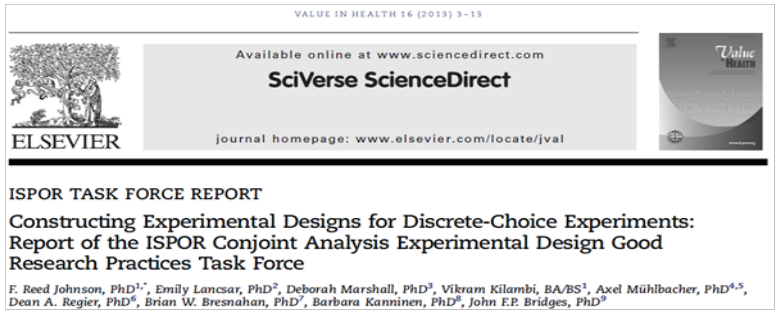
8. Heterogeneity
9. Robustness of analysis

Practical

10. Study Conduct
11. Comprehension by Participants

Adapted from Virtual ISPOR-FDA Summit 2020: Using Patient-Preference Information in Medical Device Regulatory Decisions: Benefit-Risk and Beyond. Patient Preference Information – What It Is and What It Is Not. https://www.ispor.org/conferences-education/conferences/past-conferences/ispor-fda-summit-2020. Modified from FDA Final Patient Preference Guidance Document. August 24, 2016. https://www.fda.gov/media/92593/download

Established Good Research Practices for Preference Preference-Based Studies by Recognized Professional Organizations


VALUE IN HEALTH 16 (2013) 3–13

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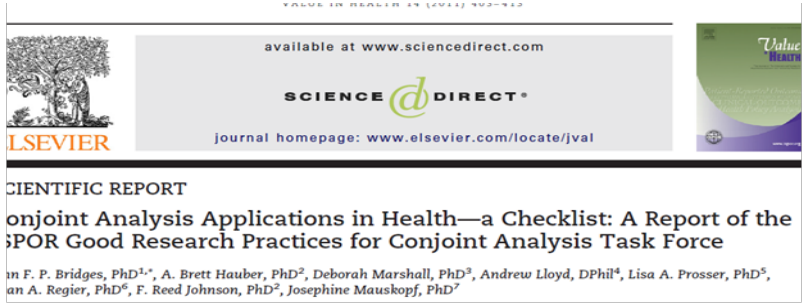
ISPOR TASK FORCE REPORT

Constructing Experimental Designs for Discrete-Choice Experiments: Report of the ISPOR Conjoint Analysis Experimental Design Good Research Practices Task Force

F. Reed Johnson, PhD[1,*], Emily Lancsar, PhD[2], Deborah Marshall, PhD[3], Vikram Kilambi, BA/BS[1], Axel Mühlbacher, PhD[4,5], Dean A. Regier, PhD[6], Brian W. Bresnahan, PhD[7], Barbara Kanninen, PhD[8], John F.P. Bridges, PhD[9]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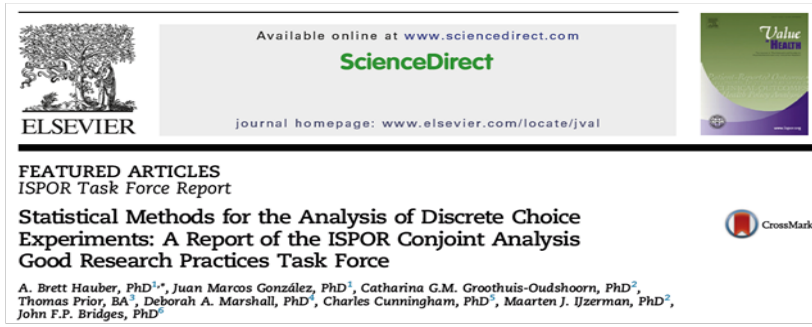
CIENTIFIC REPORT

onjoint Analysis Applications in Health—a Checklist: A Report of the SPOR Good Research Practices for Conjoint Analysis Task Force

n F. P. Bridges, PhD[1,*], A. Brett Hauber, PhD[2], Deborah Marshall, PhD[3], Andrew Lloyd, DPhil[4], Lisa A. Prosser, PhD[5], an A. Regier, PhD[6], F. Reed Johnson, PhD[2], Josephine Mauskopf, PhD[7]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FEATURED ARTICLES

ISPOR Task Force Report

Statistical Methods for the Analysis of Discrete Choice Experiments: A Report of the ISPOR Conjoint Analysis Good Research Practices Task Force

A. Brett Hauber, PhD[1,*], Juan Marcos González, PhD[1], Catharina G.M. Groothuis-Oudshoorn, PhD[2], Thomas Prior, BA[3], Deborah A. Marshall, PhD[4], Charles Cunningham, PhD[5], Maarten J. IJzerman, PhD[2], John F.P. Bridges, PhD[6]

"Aligning health care policy with patient preferences could improve the effectiveness of health care interventions by improving adoption of, satisfaction with, and adherence to clinical treatments."

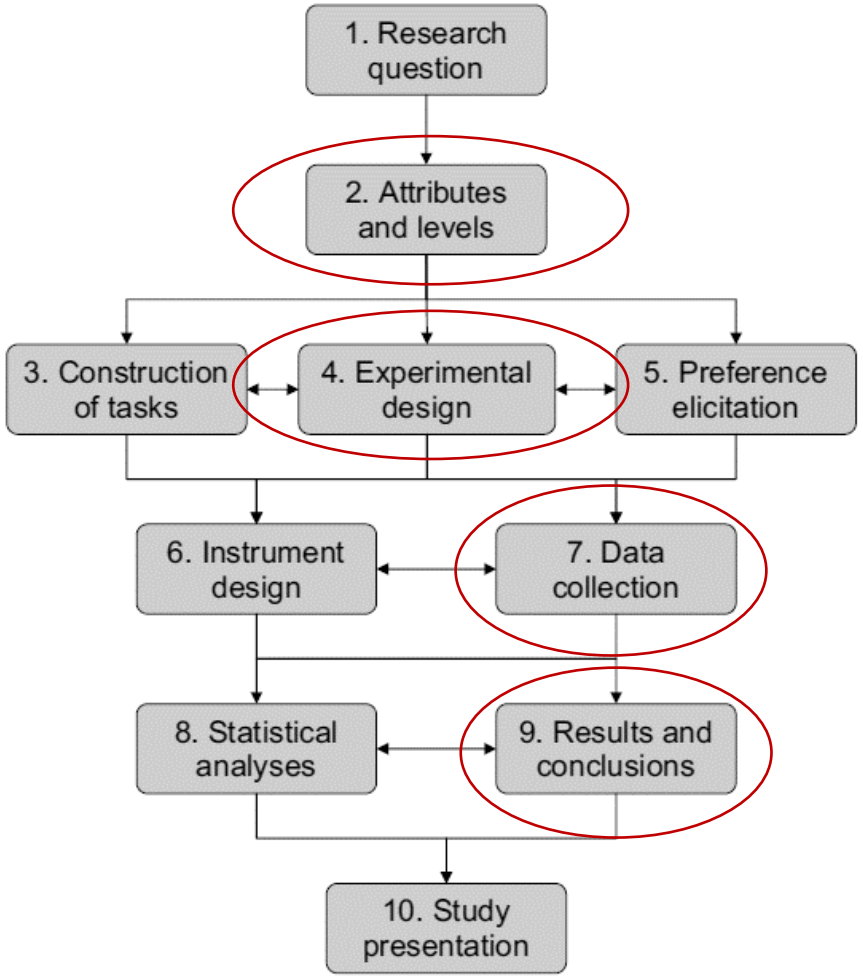
In Progress: ISPOR Task Force #4 (Co-chairs Bridges, Marshall, de Bekker-Grob)
Using Patient Preferences to Inform Healthcare Decisions

- Bridges JFP, Hauber AB, Marshall DA, et al. Conjoint analysis applications in health…Value Health. 2011;14(4):403-13; Johnson FR, Lancsar E, Marshall DA, et al. Constructing experimental designs. Value Health. 2013;16:3–13; Hauber AB, Gonzales JM, Groothuis-Oudshoorn CGM, et al. Statistical methods for the analysis of discrete-choice experiments. Value in Health 2016; 19(4):300-15

Designing and Conducting High Quality Preference Studies
Principals of GRP and Preference Study Design Apply to BWS and DCE

- Relevant
- Scientifically valid
- Feasible

Consult with decision makers and patients early in the process!



Define Objective

Attributes and levels

Design experiment

Design and implement survey instrument

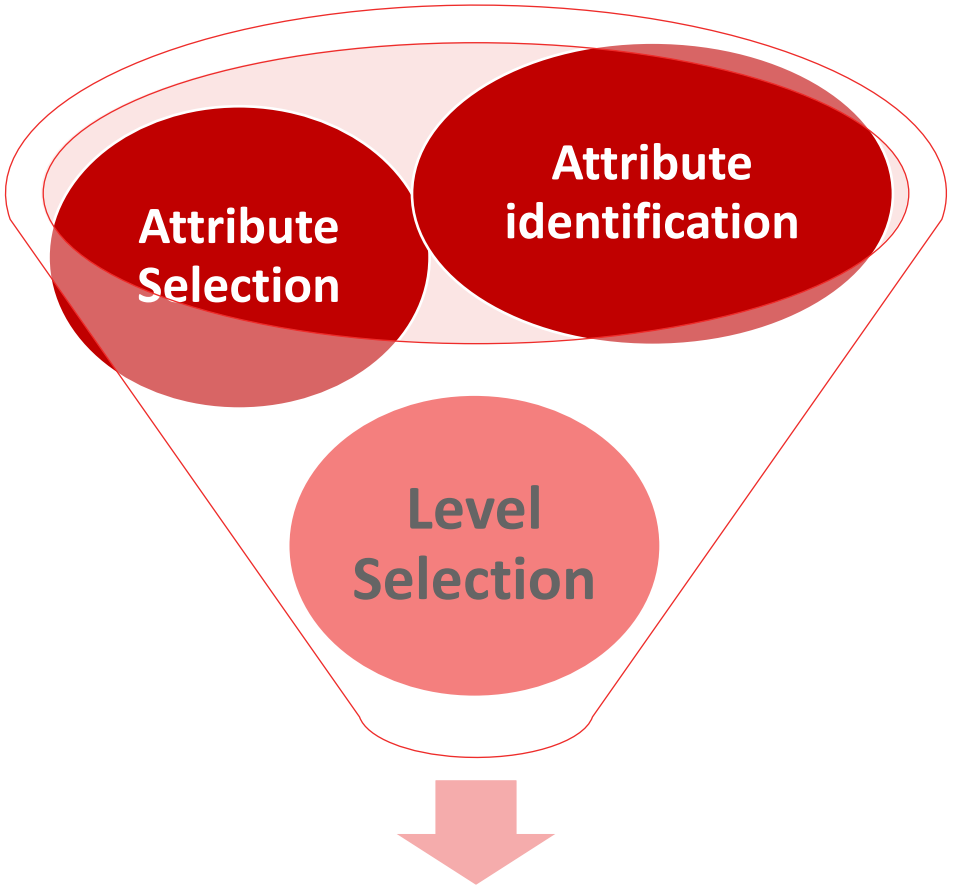
Analyze data

Report Results

Some of these categories require multiple steps

- *Bridges JFP, Hauber AB, Marshall DA, et al. Conjoint analysis applications in health…Value Health. 2011;14(4):403-13*

Identifying and Selecting Important and Relevant Attributes and Levels Using Qualitative Research



Identifying Attributes

- What attributes are important to people
- Number of attributes relevant to research question
  - Omitted attributes adversely affect study quality
- How people discuss attributes - what words?
- Understand relationships between potential attributes (e.g. pain and function)

Selecting Attributes (and Levels)

- **Attributes -** Consider all potential attributes, but balance:
  - Relevant to research question
  - What is important to respondents
  - What is important in the decision-making context
  - Plausibility and feasibility
- **Levels -** Encompass salient range of values

- *Hollin IL, Craig BM, Coast J et al. The Patient 2020;13(1):121–36;* Vass C, Rigby D, Payne K. Med Decis Making 2017;37(3):298-313. *Coast J, Al-Janabi H, Sutton EJ, et al. Health Economics 2012;21(6):730–741; Coast J, Horricks S. J Health Serv Res Policy 2007;12:25-30.*

Study Population – Representativeness of the Sample and Generalizability of Results

"A study should measure the preferences of a representative sample of adequate size so that the study results can be reasonably generalized to the population of interest."

- A function of sample size and the sampling frame
  - Larger sample sizes - but no guarantee!
  - Generalizability on average vs subgroup analysis
- Assess study population in context of the *research question* and how findings will be applied
  - For resource allocation decisions – broader study population
  - For informing specific benefit-risk trade-offs in a subgroup – eligible study population
- Consider *a priori*:
  - Subgroups of interest
  - Size of the population of interest e.g. rare disease

*- FDA Final Patient Preference Guidance Document. August 24, 2016. https://www.fda.gov/media/92593/download; FDA Final Guidance Document. Factors to Consider When Making Benefit-Risk Determinations in Medical Device Premarket Approval and De Novo Classifications. August 30, 2019. https://www.fda.gov/media/99769/download; Tinelli, M., Ryan, M. & Bond, C. What, who and when? Incorporating a discrete choice experiment into an economic evaluation. Health Econ Rev **6,** 31 (2016); https://mdic.org/wp-content/uploads/2018/05/MDIC_PCBR_Framework_Web.pdf*

Minimise Bias - Experimental Design for Statistical Efficiency and Pre-Testing for Response Efficiency

Principle of experiment: Systematically vary attributes and levels to investigate the determinants of choice behaviour

Unbiased parameter estimates for all model parameters

Maximise precision of parameter estimates

Minimise confidence intervals around parameter estimates
Efficient Experimental Design

Minimise measurement error
Relevance based on qualitative Pre-test, Pre-test, Pilot test !

Minimise Bias: How to Communicate Benefit, Risks and Uncertainty Effectively?

Attribute	Alternative 1	Alternative 2
Effective Response (Benefit)	9 out of 10 (90%) 	6 out of 10 (60%) 
Side Effects (Risk)	2 out of 10 (20%) 	1 out of 10 (10%) 

- Graphically using icon arrays (e.g. 9 in black and 1 in white) to reflect part-to-whole relationships **AND**
- Words (e.g. 9 out of 10 people) **AND**
- Numbers (e.g. 90%)

Veldwijk J, Lambooij MS, van Til JA, et al. Patient Educ Couns. 2015 Nov;98(11):1376-84; Ancker JS, Senathirajah Y, Kukafka R, et al. J Am Med Inform Assoc 2006;13:608-18; B. Fischhoff, et al, Communicating Risks and Benefits: An Evidence Based User's Guide, U.S. Food and Drug Administration (2011)

Concluding Comments

- Designing patient preference study is different than most surveys!
  - PPI can be considered *valid scientific evidence if* high quality study that is relevant, scientifically valid and feasible
- Qualitative research is fundamental to identify and select attributes/levels and test the survey
- Experimental design for quantitative to systematically vary attributes and levels to investigate the determinants of choice behaviour and minimise bias
- 11 qualities recommended for a scientifically valid patient preferences study following good research practices by recognized professional organizations
- PPI methods capture different types of preferences and need to be interpretated in the context of the design and research question

Thank you!

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