

What is next for patient preference studies in cancer drug development?

An Overview, Challenges & Opportunities

Who are we?

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- Academic perspective
- Disclaimer

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Agenda

- Definition of patient preference studies
- Patient preference methods
- Importance and relative occurrence
- Cancer specific examples
- Challenges and opportunities

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What is a patient preference study?

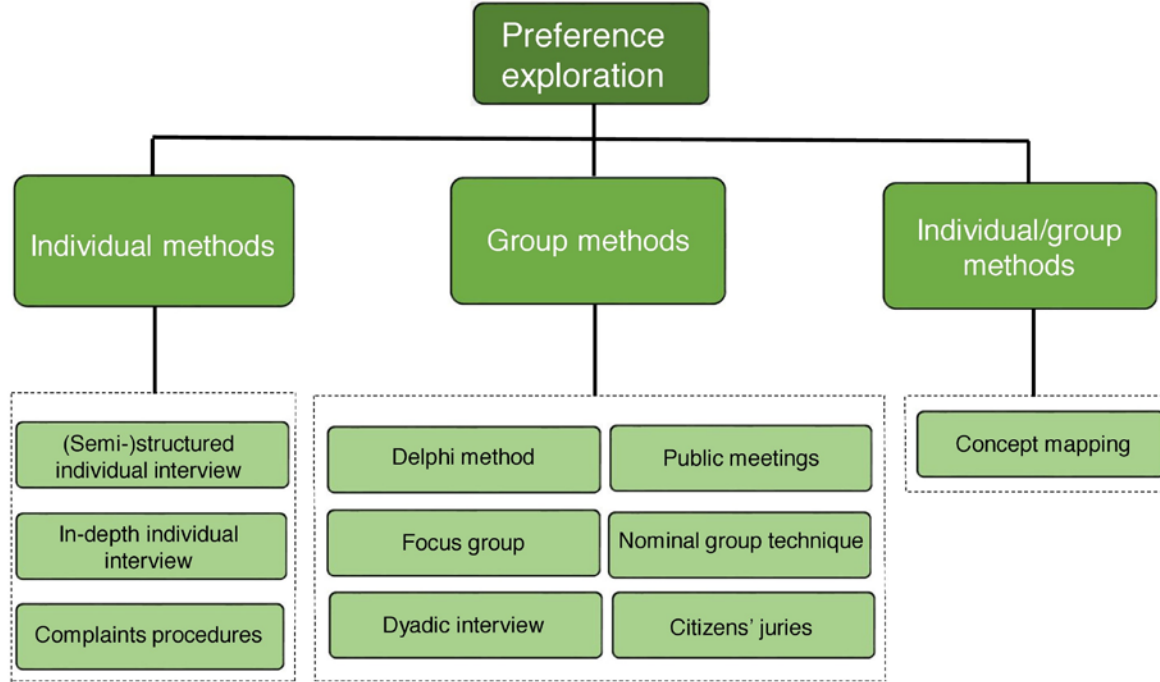
- Broad definition (FDA, 2016):

‘qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions’

- Why important?
 - Patient perspective
 - Shared decision-making
 - Drug regulation
- How to gain insights into preferences?

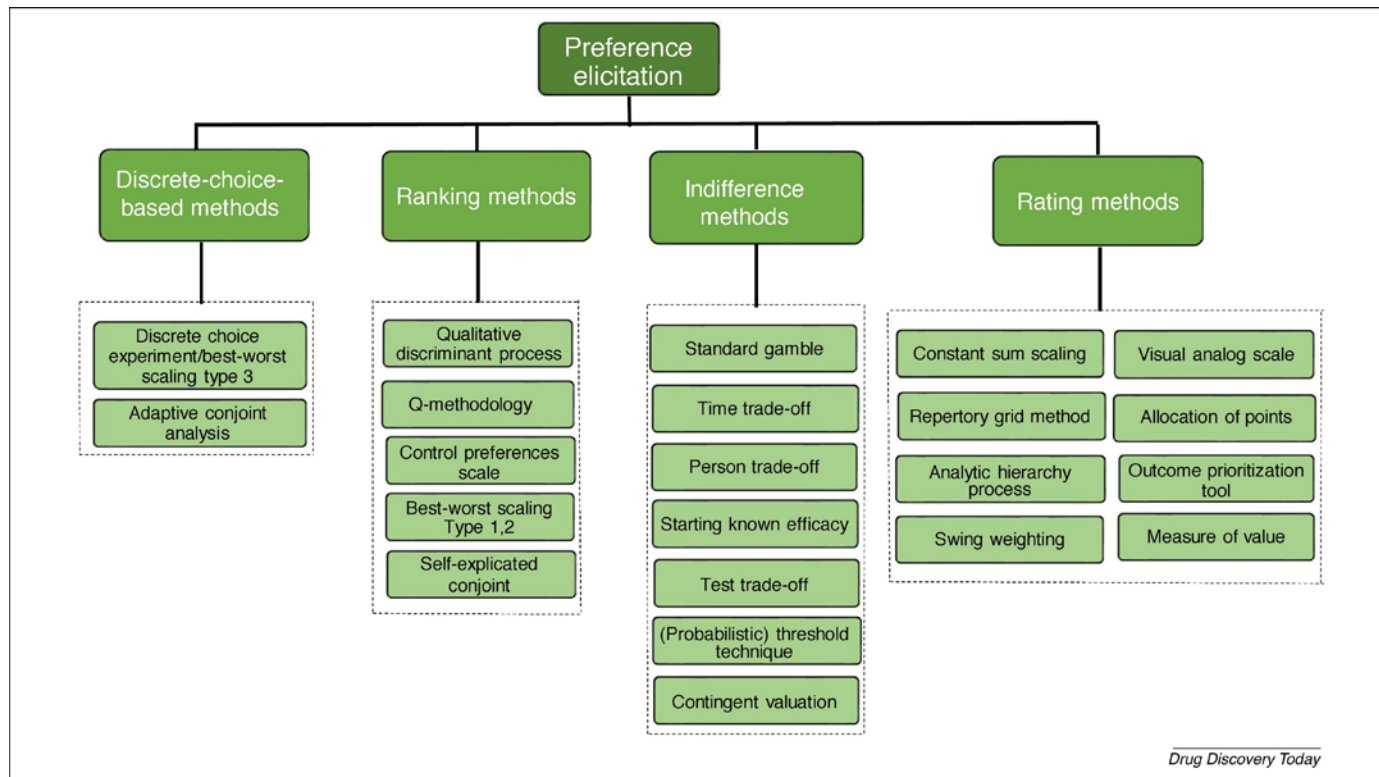
A stylized, handwritten-style logo for Erasmus, featuring a large, flowing 'E' followed by the word 'Erasmus' in a cursive script.

Preference exploration methods (qualitative)



Drug Discovery Today

Preference elicitation methods (quantitative)



Importance of patient preferences

- EUPATI

- www.eupati.eu



- MDIC

- www.mdic.org



- PARADIGM

- www.imi-paradigm.eu



- PREFER

- www.imi-prefer.eu

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How often are preference studies conducted?

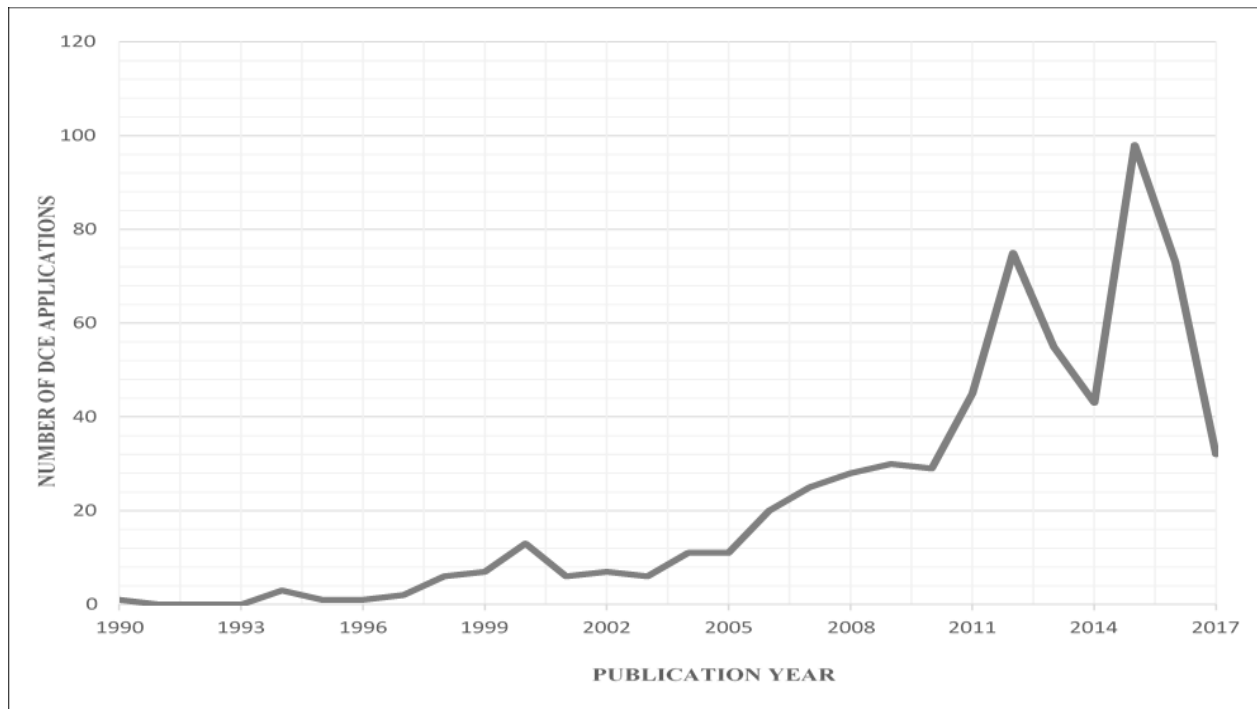
- Focus on discrete choice experiments (DCEs):
 - Available strength of evidence based on existing wealth of literature
 - More and more experience amongst decision-makers
 - DCE method measures trade-offs
 - Suitable method for decisions as taken by industry, regulators and HTA bodies & reimbursement agencies at different stages of the MPLC
 - Different outcome measures

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DCE example: Prostate Cancer Screening

	NO SCREENING	PROGRAMME 1	PROGRAMME 2
Amount of men per 1,000 men that will die from prostate cancer Frequency of a blood test	35 deaths  (0 deaths prevented)	25 deaths  (10 deaths prevented)	18 deaths  (17 deaths prevented)
Amount of men per 1,000 men with an increased PSA that receive an unnecessary biopsy (=no cancer detected, although the blood test suggested that a biopsy was needed)	Not applicable	400 unnecessary biopsies  (600 correct biopsies)	800 unnecessary biopsies  (200 correct biopsies)
Amount of men per 1,000 treated men that receive an unnecessary treatment (=no increase in life expectancy, but there is a risk of remaining urine incontinence and erection problems due to treatment)	Not applicable	0 unnecessary treatments  (1.000 correct treatments)	500 unnecessary treatments  (500 correct treatments)
Out of pocket cost per year	0 euro	100 euro per year 	50 euro per year 
Which alternative would you choose?	☐	☐	☐

More popular in health



Soekhai et al. 2018, Pharmacoeconomics

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Areas of application and outcomes

Category details	Number of papers 1990-2000		Number of papers 2001-2008		Number of papers 2009-2012		Number of papers current: 2013-2017	
	N=34 ^a	(%) ^b	N=114 ^a	(%) ^b	N=179 ^a	(%) ^b	N=301 ^a	(%) ^b
Patient consumer experience	12	(35)	40	(35)	24	(12)	105	(35)
Valuing health outcomes	3	(9)	8	(7)	13	(7)	23	(8)
Investigating trade-offs between health outcomes and patient or consumer experience factors	14	(41)	38	(33)	81	(45)	47	(16)
Estimating utility weights within the QALY framework	0	(0)	2	(2)	4	(2)	13	(4)
Job choices	2	(6)	5	(4)	8	(4)	17	(6)
Developing priority setting frameworks	2	(6)	6	(5)	23	(13)	27	(9)
Health professionals' preferences for treatment or screening options for patients	1	(3)	17	(15)	23	(13)	19	(6)
Other	0	(0)	4	(4)	21	(12)	46	(15)
Not reported	N/C	N/C	N/C	N/C	N/C	N/C	4	(1)

Soekhai et al. 2018, PharmacoEconomics

Category	1990-2000		2001-2008		2009-2012		Current: 2013-2017	
	N=34 ^a	(%) ^b	N=114 ^a	(%) ^b	N=179 ^a	(%) ^b	N=301 ^a	(%) ^b
Per WTP unit	10	(29)	44	(39)	54	(30)	80	(27)
Per WTA unit	N/C	N/C	N/C	N/C	N/C	N/C	13	(4)
Per risk unit	3	(9)	2	(2)	4	(2)	9	(3)
Monetary welfare measure	5	(15)	14	(12)	4	(2)	8	(3)
Utility score	8	(24)	18	(16)	14	(8)	50	(17)
Odds ratio	1	(3)	9	(8)	14	(8)	30	(10)
Probability score	1	(3)	15	(13)	14	(8)	38	(13)
Coefficients	N/C	N/C	N/C	N/C	N/C	N/C	169	(56)
Other	N/C	N/C	N/C	N/C	90	(50)	147	(49)

- Specific examples in oncology and drug development: next slides

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Example 1

DCE Review database

- Systematic review
- Cancer screening
- Outcome attributes (e.g. efficacy) , vs. health care delivery (e.g. waiting time)

Table 2

Characteristics of Included Studies, Stated Preference for Cancer Screening, Systematic Review, 1990–2013

Citation	Population and Sample Size	Cancer type	Purpose of Study
Studies with only screening test attributes			
Araña et al, 2006 (12)	60 Students in Gran Canaria, Spain (compared preferences to those of 60 oncologists)	Cervical	Compare the preferences of general population with preferences of subjects with medical expertise.
Basen-Engquist et al, 2007 (13)	Women with (n = 457) and without (n = 449) a history of abnormal Papanicolaou smear who live in Groot-Rijnmond, Netherlands	Cervical	Compare the preferences of women with and without a history of abnormal Papanicolaou smear tests, including a new technology.
de Bekker-Grob et al, 2010 (14)	Adults aged 50–74 years with (n = 649) and without (n = 626) a colorectal cancer screening history in the Netherlands	Colorectal	Compare preference results for a labeled and an unlabeled discrete choice experiment.
Gyrd-Hansen, 2000 (15)	207 Women aged 50 years living in Denmark	Breast	Assess women's preferences for the attributes of breast cancer screening programs.
Gyrd-Hansen and Sogaard, 2001 (16)	483 Adults aged 50 years living in Denmark	Colorectal	Assess women's preferences for the attributes of colorectal cancer screening programs.

Example 2

DCE Review database

- Premarket approval medical device

Table 2 Preference relative to a no-device alternative, minimum acceptable benefit, and maximum acceptable risk of a 243 pound respondent for three weight-loss device profiles

Weight loss device profile	% Respondents who judged better than a no-device alternative	Risk Tolerance	Minimum acceptable TBWL ^a	Maximum acceptable mortality risk ^b
Virtual device A Benefit: 5 % TBWL Risk: 1 % chance of death Type of surgery: laparoscopic surgery Dietary restriction: eat 1/4 cup of food at a time Weight-loss duration: 36 months Minor side-effect duration: 36 months Chance of hospitalization with surgery: 5 % Comorbidity: no improvement	5.0 %	Middle 50 % Upper 25 %	>30 % ^c 15.1 % (11.63–19.05)	0.1 % (0.04–0.26) 3.8 % (1.85–7.77)
Gastric band Benefit: 13 % TBWL Risk: 1 % chance of death	11.6 %	Middle 50 % Upper 25 %	>30 % ^c 13.0 % (9.76–16.90)	0.16 % (0.07–0.38) 7.1 % (3.82–13.50)

Ho et al., 2015 Surgical Endoscopy



Erasmus Choice Modelling Centre

Erasmus Choice Modelling Centre

- Breast cancer
- Colorectal cancer
- Cervical cancer
- Lung cancer
- Oesophageal cancer
- Prostate cancer
- Skin cancer



Example 3

Erasmus Choice Modelling Centre

- Genetic testing for colorectal cancer
- Patient preference heterogeneity

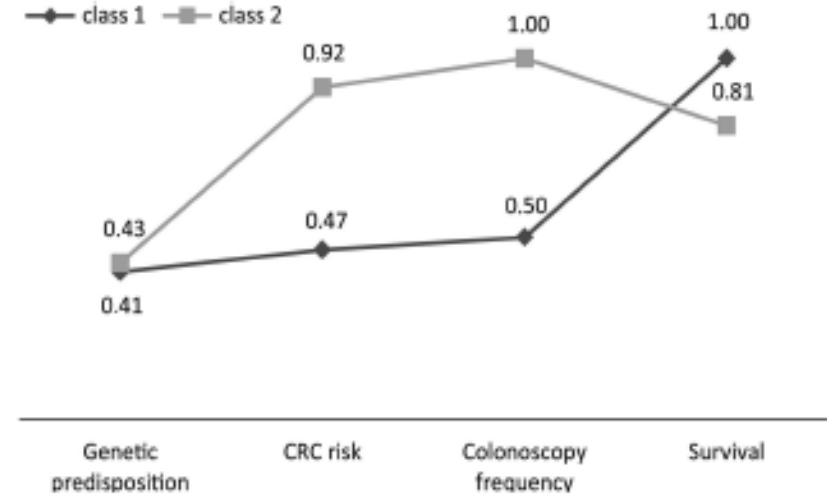


Figure 1 Relative importance of the attributes stratified by class. Values reflect the relative distance of all attributes to the most important attribute on a scale from 0 to 1 (1 indicating the most important attribute).

Veldwijk et al., 2016 Eur J Hum Gen

What is still required to reach the full potential of preference studies?

- Research categories
- Research topics

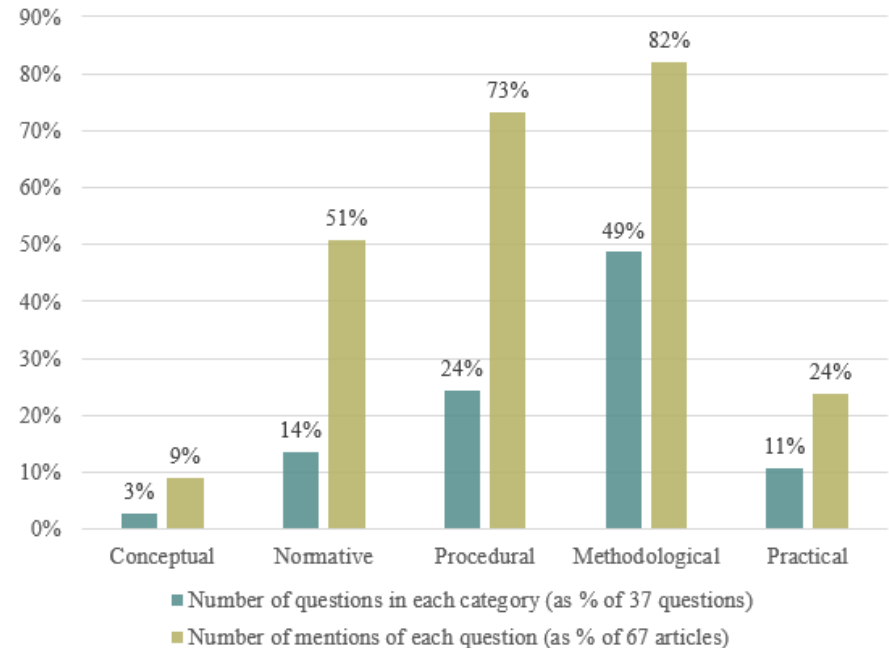
Table 2: Relative occurrence of issues, per category of issues and per topic of issues

Category	Topic	# issues		# mentions	
		N=37 ¹	% ²	N=67 ¹	% ²
Conceptual		1	3%	6	9%
	Taxonomy	1	3%	6	9%
Normative		5	14%	34	51%
	Whose preferences	4	11%	26	39%
	Relevance of preferences	1	3%	11	16%
Procedural		9	24%	49	73%
	Weight	3	8%	21	31%
	Impact	1	3%	21	31%
	Patient education	3	8%	20	30%
	Evidence-based	1	3%	17	25%
	HTA stage	1	3%	16	24%
Methodological		18	49%	55	82%
	Choice of method	3	8%	30	45%
	Internal validity	3	8%	24	36%
	Generalizability	4	11%	19	28%
	Sample selection	1	3%	15	22%
	External validity	2	5%	9	13%
	Patient characteristics	2	5%	8	12%
	Reliability	3	8%	7	10%
Practical		4	11%	16	24%
	Resources	4	11%	16	24%

What is still required to reach the full potential of preference studies?

- Academic perspective of priorities

Figure 3: Relative occurrence of research questions in categories



What is still required to reach the full potential of preference studies?

- Audience's perspective of priorities

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Questions?

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