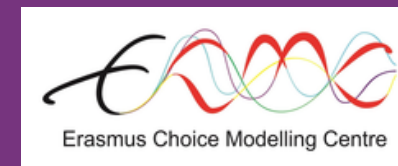**Erasmus School of Health Policy & Management**

How to elicit patients' preferences to generate robust evidence for evidence-based decisions

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Erasmus

Involving patients

- Every patient has wishes, needs, values and preferences

- Involving 1-2 or small group of patients versus perspective of representative sample?

- Observations?
  - Sample size
  - Time and budget restrictions
  - What about future treatment option?

Move to hypothetical situations

Patients' preferences

- Groups of patients have wishes, needs, values and preferences

- Likert-scale survey?

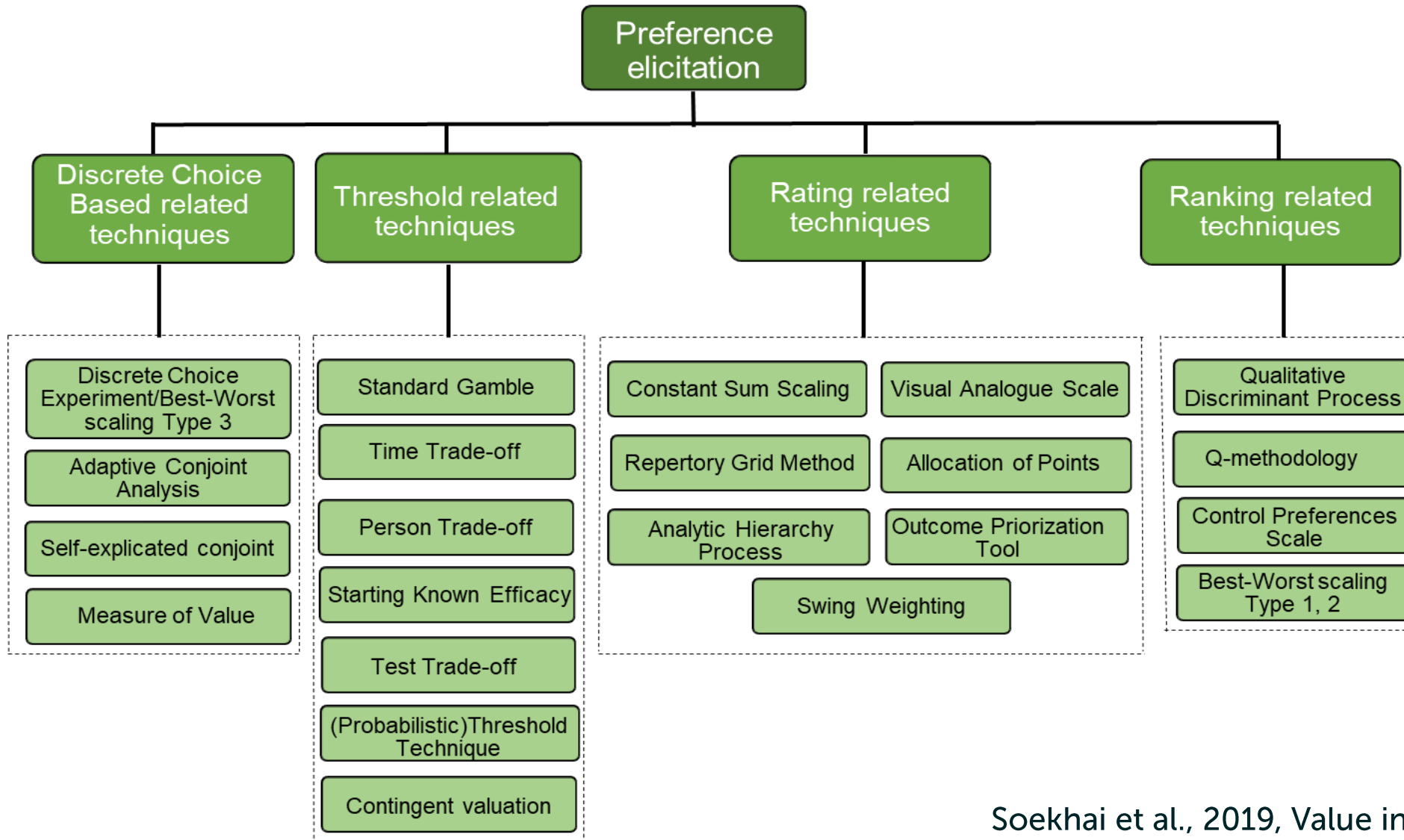|	Very important	Important	Neutral	Unimportant	Very unimportant
Price	✖	0	0	0	0
Waiting time	✖	0	0	0	0
Side-effects	✖	0	0	0	0
Effectiveness	✖	0	0	0	0

Patients' preferences

- Pairwise comparisons!

- Quantifying the relative importance of different aspects of a good or service

FDA guideline for the evaluation of devices for market approval

Measuring patient preferences

Outcomes of preference elicitation methods

- Preferences
  - Heterogeneity in preferences
  - Variables that explain heterogeneity in preferences

- Relative importance

- Trade-offs
  - Maximum acceptable risk (MAR)
  - Minimum acceptable benefit (MAB)
  - Willingness to pay (WTP)

- Predicted uptake / market share

Guidance needed on how to elicit preferences

- ISPOR task forces
- International Academy for Health Preference Research
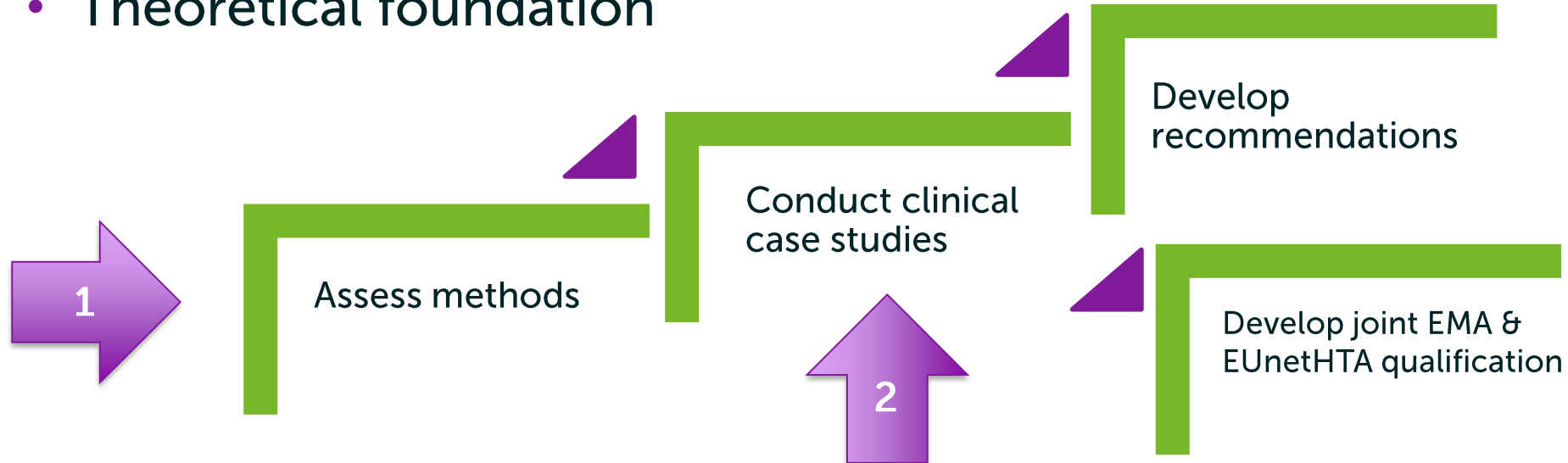- Erasmus Choice Modelling Centre

- PREFER Project
  - A five-year project that has received funding from the **Innovative Medicines Initiative** 2 Joint Undertaking which received support from the European Union's **Horizon 2020** research and innovation programme and the European Federation of Pharmaceutical Industries and Associations (**EFPIA**). A total of 31 partners from academia and industry working together.

PREFER: Approach and case studies
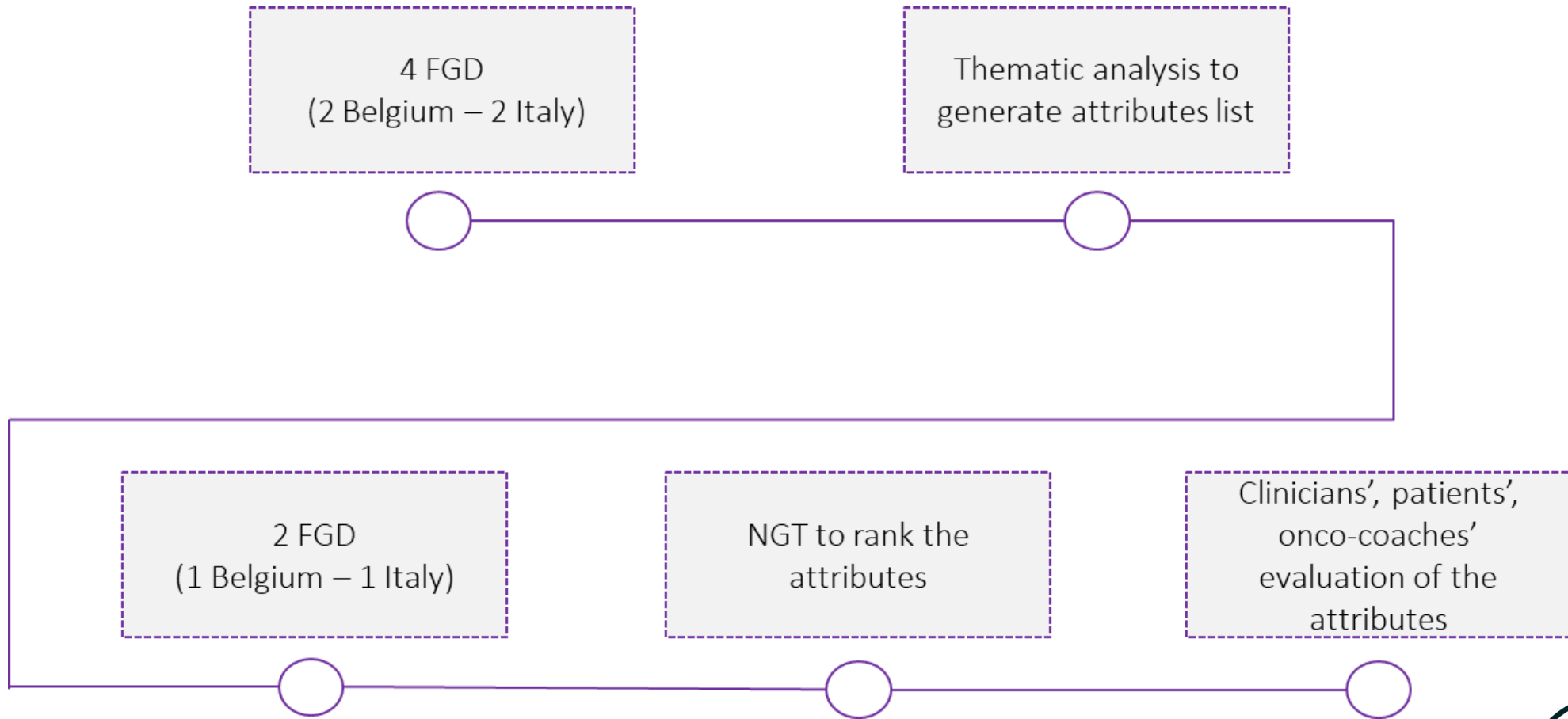
- Theoretical foundation

- 12 prospective case studies
- 10 different disease areas
- >10 different countries
- Including patients as research partners
- Comparing different preference elicitation methods

Lung cancer case study Background

- EMA approved a combination of immunotherapy & chemotherapy as first-line treatment of metastatic squamous NSCLC

- NSCLC patients with PD-L1 expression >50% can now choose between:
  - Immunotherapy alone
  - Immunotherapy & chemotherapy

- Case study aims:
  - Identifying patient-relevant benefit-risk attributes of LC treatments
  - Quantifying the risk tolerance for adverse events that patients are willing to accept for an increased probability of prolonged survival
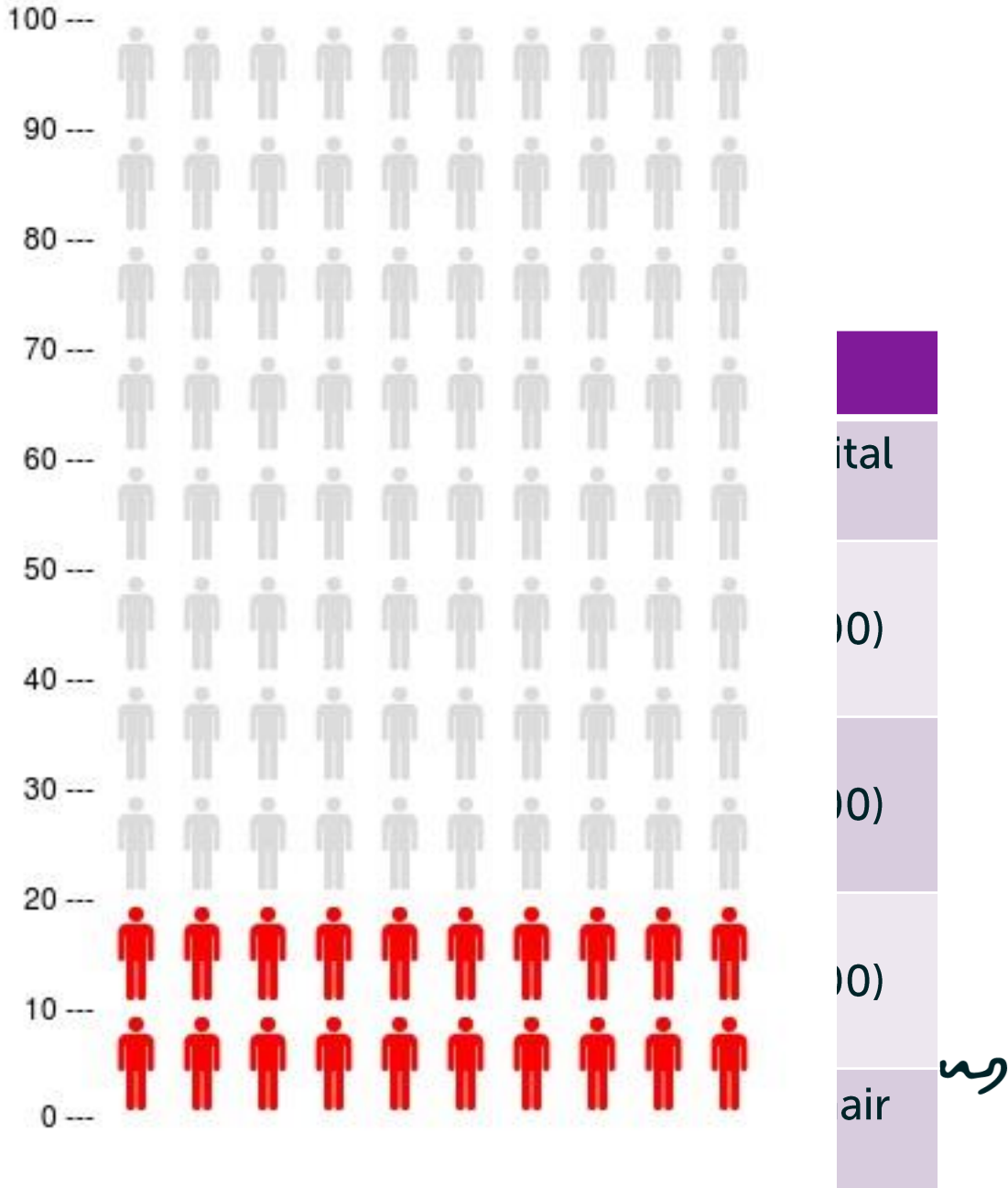
Lung cancer case study Qualitative part

Attributes and levels

Attribute	Level 1	Le
Treatment administration	Oral	In
Chance to survive 5 years	10% (10 out of every 100)	(2
Chance of long-lasting skin problems	10% (10 out of every 100)	(2
Chance of being extremely tired	10% (10 out of every 100)	(4
Severity of hair loss	No hair loss	W

Lung cancer case study Quantitative part

- Online survey including:
  - Discrete choice experiment
  - Swing weighting

    Randomized order

  - Demographics
  - Disease specific questions
  - Health literacy (objective and subjective)
  - Numeracy
  - Health locus of control

Patients: 336 Italy & 168 Belgium
Stage I, II, III, IV LC patients older than 18 years

Pre-selection by clinicians

Pre-testing and pilot testing!

- Video instructions about: disease context, attributes & levels, methods

Where to go from here

- Integration of patients' preferences in decision-making
of all stakeholders along the medical product lifecycle is crucial

- <u>PREFER to make a first important step:</u> Impactful evidence-based recommendations
  - A framework to guide accurate set up of preference studies
  - A description of commonly used and suitable methods
    - Based on science and responding to changing environment
    - In alignment with needs of and developments of all stakeholders

Thank you

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