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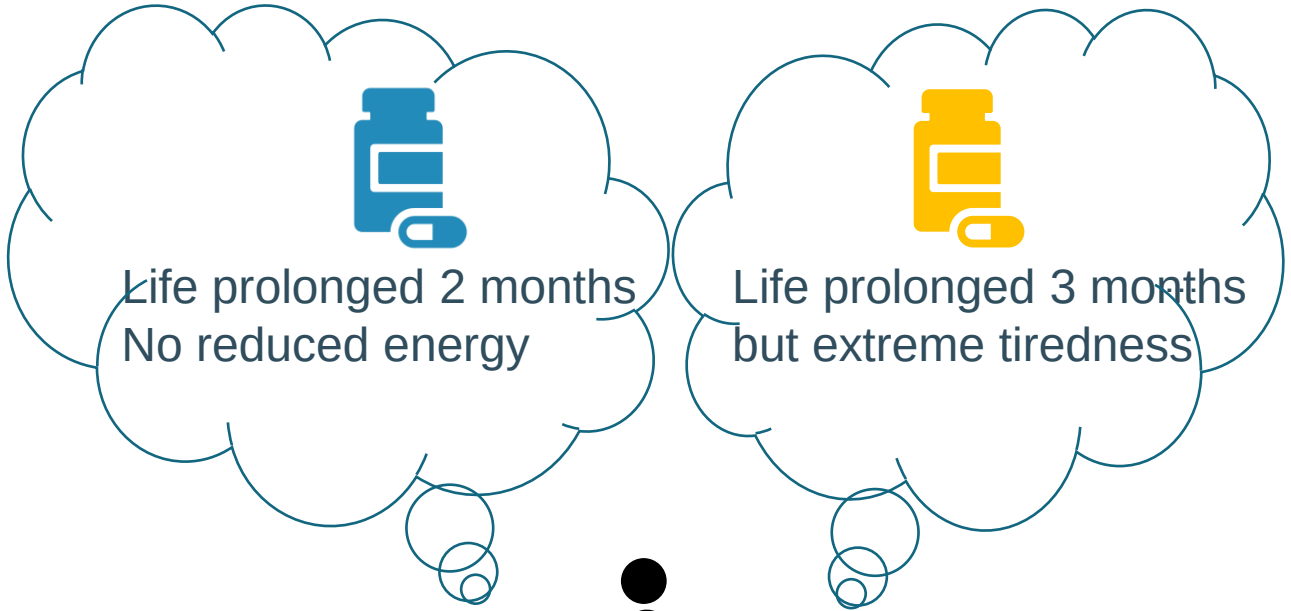
PREFER OUTCOMES
Patient preferences to inform medical decision-making

Prof. dr. Isabelle Huys

RSNN meeting Utrecht

5-09-2022

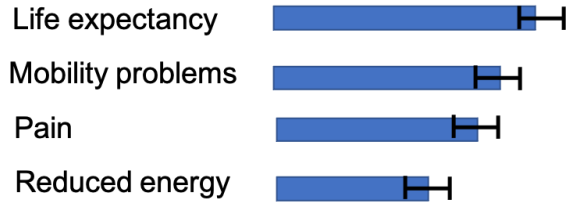
Patients

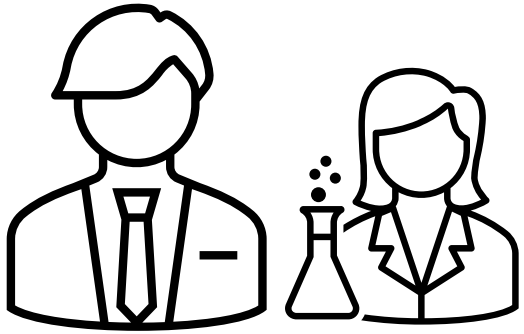


Patient's preferences

?

Drug developers, regulators, payers, academia,…





Qualitative and quantitative data

Patient preference studies

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Why PREFER?

What are the **preferences** of patients in diverse disease contexts?

What data are useful for decision-making?

Beyong nice-to-have data

What **methods** are reliable?

How to avoid bias and ensure robustness?

What about **patient heterogeneity**?

prefer.
PATIENT PREFERENCES

Patient preference studies

IMI PREFER is a public-private partnership

- **Coordinator:** Uppsala University
- **Academic co-lead**: KU Leuven
- **Project leader:** Novartis Pharma

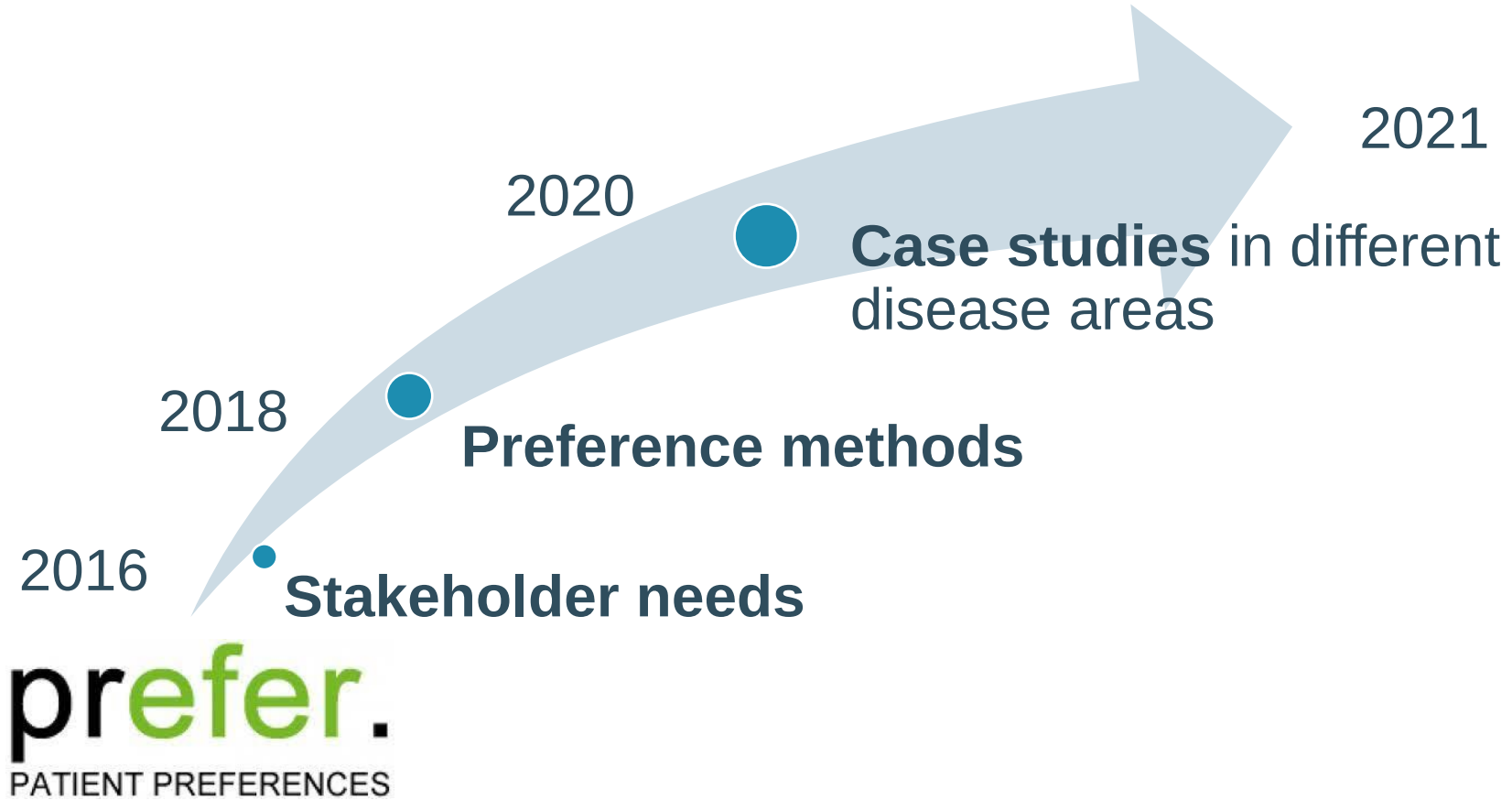
10 Academic research institutions
4 Patient organisations
1 HTA
2 SMEs
16 Pharmaceutical companies

prefer. PATIENT PREFERENCES

imi innovative medicines initiative

SME: small and medium sized enterprises; HTA: Health Technology Assessment body

IMI PREFER at a glance



>50 scientific papers, 5 PhDs, conference presentation, abstracts, workshops, Scientific advice, Youtube ….

EMA/EUnetHTA qualification

PREFER Recommendations

Organisational guidelines

Training materials/webinars

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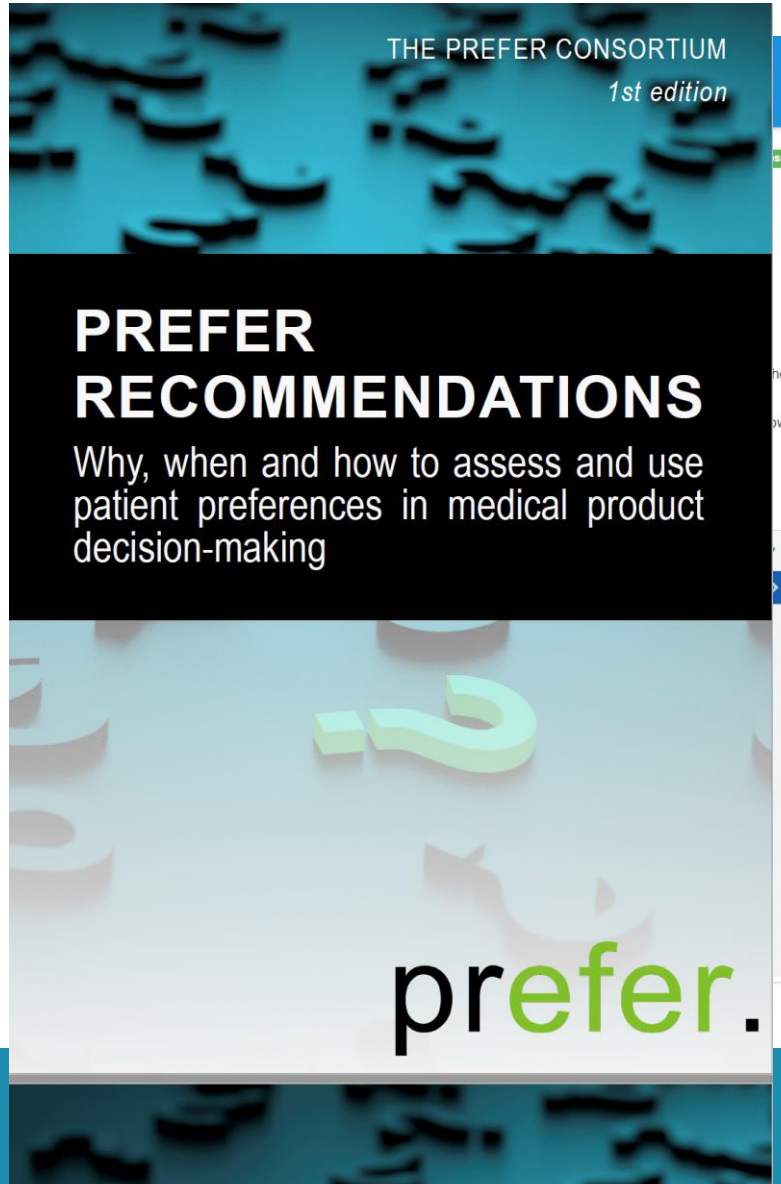
IMI PREFER at a glance



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

03 May 2022
EMADOC-1700519818-808373
Committee for Medicinal Products for Human Use (CHMP)

Qualification Opinion of IMI PREFER

Draft agreed by Scientific Advice Working Party (SAWP)	30 September 2021
Adopted by CHMP for release for consultation	14 October 2021[1]
Start of public consultation	15 October 2021 [2]
End of consultation (deadline for comments)	25 November 2021[3]
Adopted by CHMP	22 April 2022

Keywords	Qualification of Novel Methodologies, IMI PREFER, Patients Preference studies

making

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Versions

Version 3 Apr 19, 2022
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https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/qualification-opinion-imi-prefer_en.pdf

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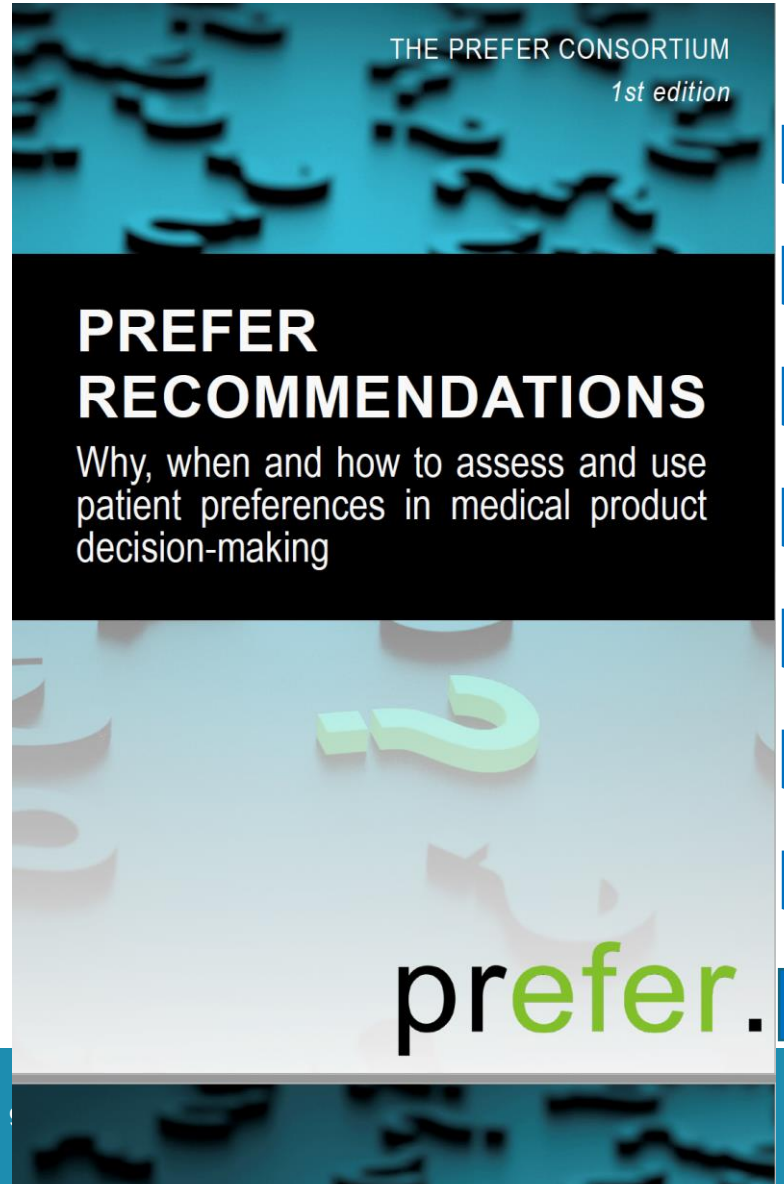
IMI PREFER at a glance



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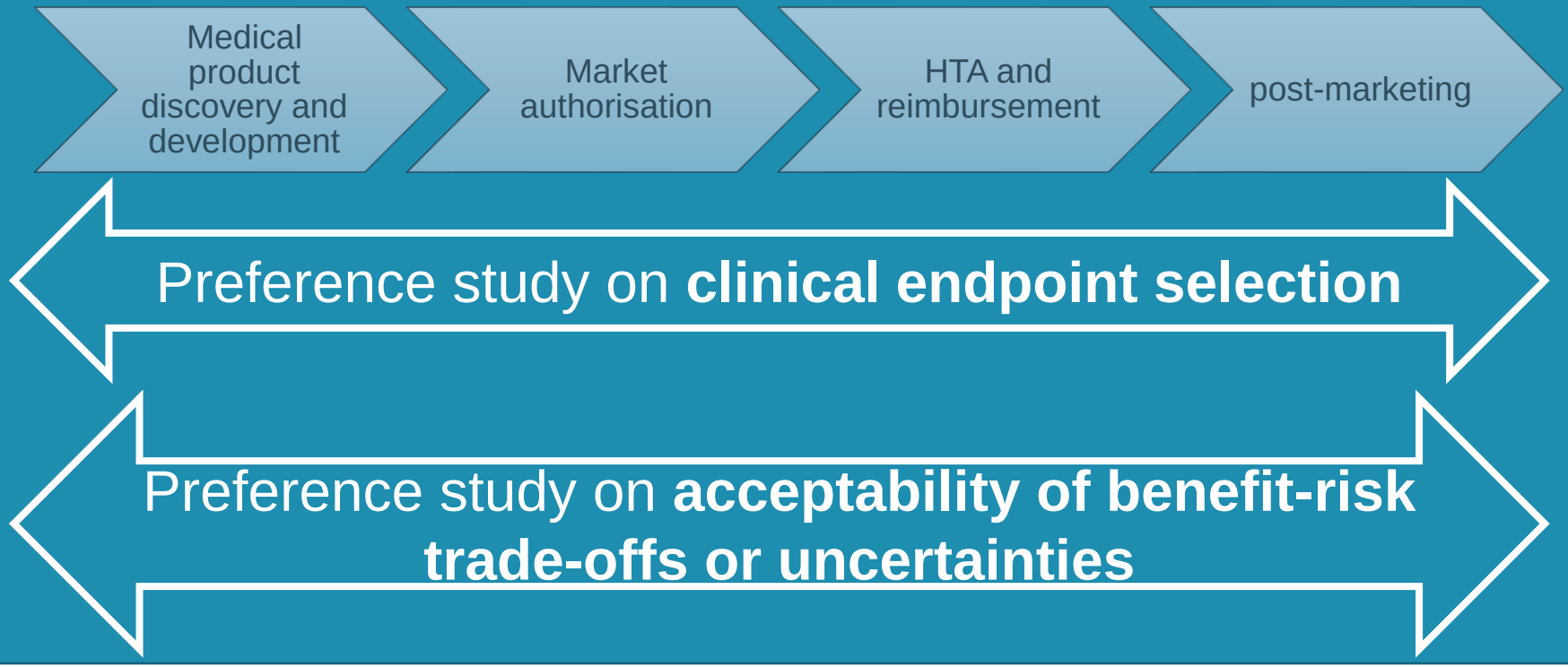
Key messages

Key message: **Value**

CHMP Qualification Opinion identifies “endpoint selection” and “identify and value trade-offs for benefits and risks” as potential applications of preference studies



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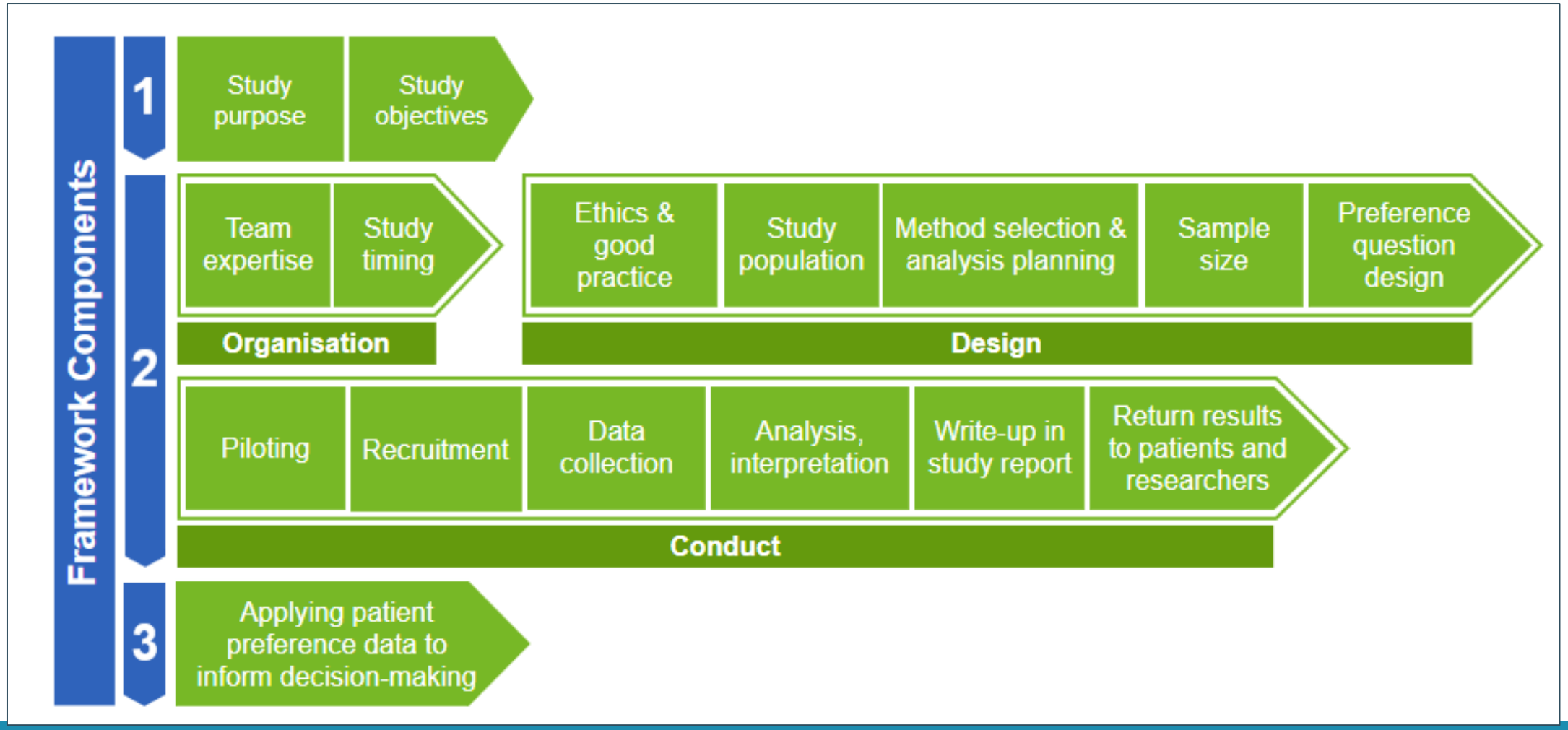
Key message: **PREFER Framework**



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Key message: **PREFER Framework**

Study purpose should include detailed information about the **intended decision**, decision-makers, **decision-context** and **whose** preferences are of interest

Team should be multidisciplinary, depending on the study objectives

Timing depends on many factors including feasibility. **Sufficient time to consult with key decision-makers, such as regulators and HTA bodies, is essential**

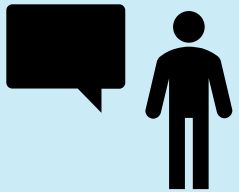
Study design includes qualitative and quantitative parts -> templates

Study conduct requires adequate piloting, analysis but also interpretation and return of results to the study population

Applications cover the use of preference data, eventually along clinical trial data

Key message: **Stakeholder interaction**

Patients



- Empower patients as **research partners**
- Involve patients as study team members or advisors throughout **entire** process
- Involve patients in the **communication plans**

Academics

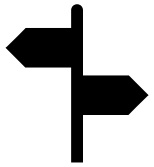
- **Scientific** robust research
- **Independent** (no commercial interest)
- **Access** to patients

Regulators, HTA, Payers

- **Early consultation** with regulator and/or HTA body
- **Awareness** of needs and expectations of regulators and HTA bodies
- **Checklist** to help study sponsors guide scientific advice discussions

Key message: **Methods**

Patient preference information can be obtained through **many different established preference exploration (qualitative) or elicitation (quantitative) methods**



Choosing the most appropriate method is a crucial step, and multiple factors should be considered

Detailed descriptions of the five most promising methods and points to consider for method selection

Gain familiarity with using the methods and their results in the medical decision making context

Future outlook

- **FDA: Draft benefit-risk guidance** with a section on Role of Patient Experience Data in FDA's Benefit-Risk Assessment
  - **PDUFA VII:** commitment letter
  - **FDA are writing 4 guidance documents** on Patient-Focused Drug Development
  - **CDRH already have a guidance in place** on the use of patient preference information in regulatory submissions
- **EMA:** Regulatory Science Research Needs Initiative 2021 and 2021 workplan highlights interest in optimal approaches for patient input into the benefit-risk assessment in decision making
- **EMA Qualification Opinion of IMI PREFER** endorsing the framework for patient preference studies and many aspects of the recommendations
- **ICH Reflection Paper:** Proposed ICH Guideline Work to Advance Patient Focused Drug Development. (Reference to IMI PREFER)

Future outlook

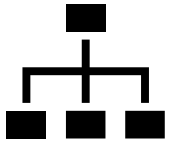
CIOMS (COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES): Working Group XI – Patient Involvement in Development and Safe Use of Medicines

HTA: EUnetHTA21; Sweden (Patient preferences to supplement QALYs)

Preference Research:

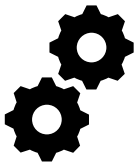
- HTA: ISPOR Patient Preferences Task Force and Quantitative Benefit-Risk (qBRA) Task Force
- HTA international (HTAi) Patient and Citizen Involvement Interest Group (PCIG)
- International Academy of Health Preference Research IAHPR

Areas for further research



Which method to use?

How to best to select and describe **attributes** (e.g. uncertainty) to increase validity?



Preference dynamics vs new clinical evidence

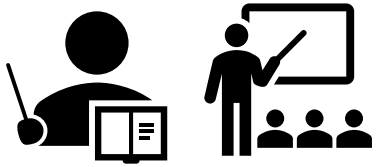
Implementation of preference study data results in decision-making context

How to get there?

Increase **familiarity** with patient preference studies

Raise **awareness** for interaction/early dialogue with decision-makers via EMA scientific advice or innovation task force

Draft fit for purpose operational **guidances** for the use of preference study data in decision-making



Organise **education** for sponsors and decision-makers to underscore unbiased methodological approaches

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Thank you

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