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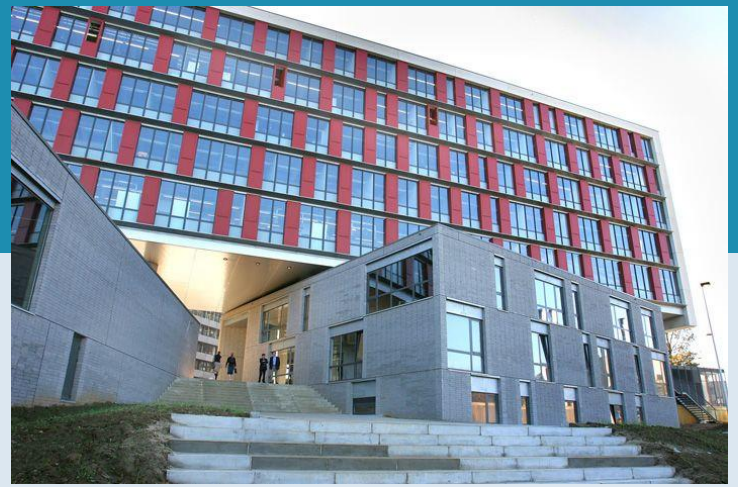
EMA/EUnetHTA qualification of the IMI PREFER Patient Preference Framework

RSNN Annual meeting

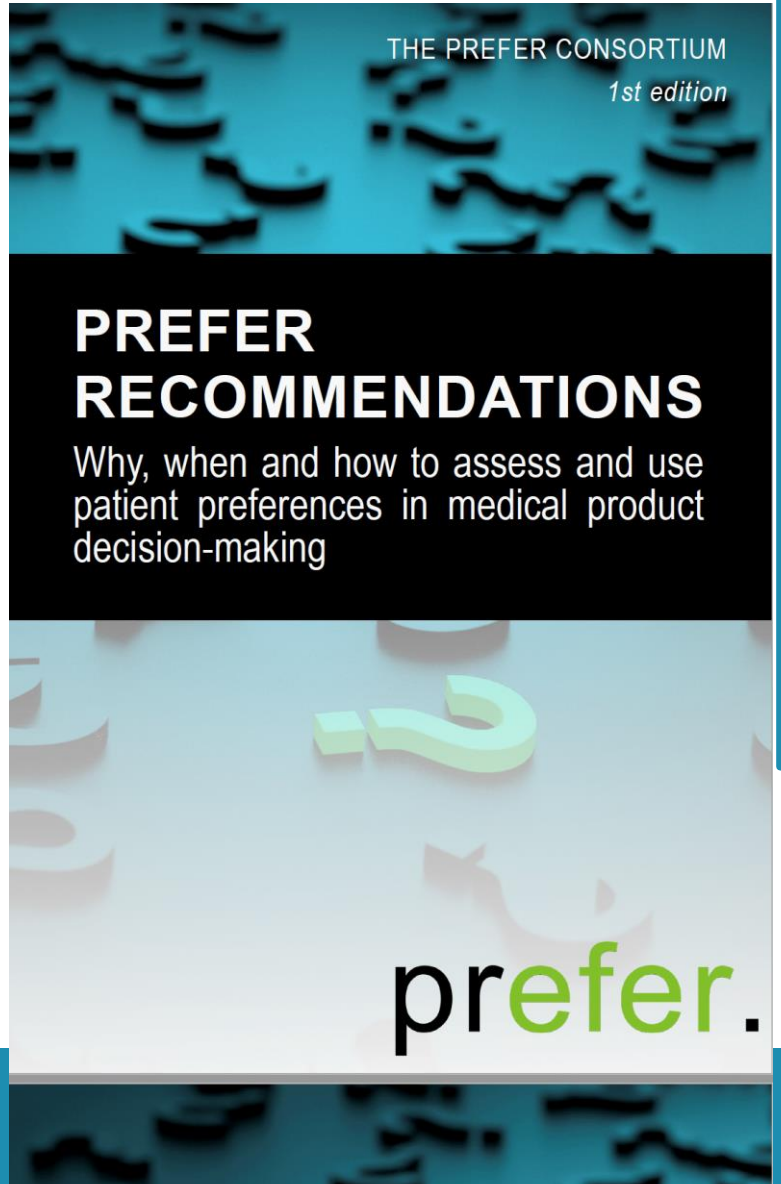


Dr. Liese Barbier

Postdoctoral researcher

Utrecht, 5 September 2022

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

May 2022

Link: Qualification Opinion of IMI PREFER

>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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What is EMA qualification?

= a voluntary scientific pathway to establish the regulatory acceptability of a specific use of a methodology for the development of medicinal products

Guidance to applicants: EMA/CHMP/SAWP/72894/2008

https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/qualification-novel-methodologies-drug-development-guidance-applicants_en.pdf

What is EMA qualificatior

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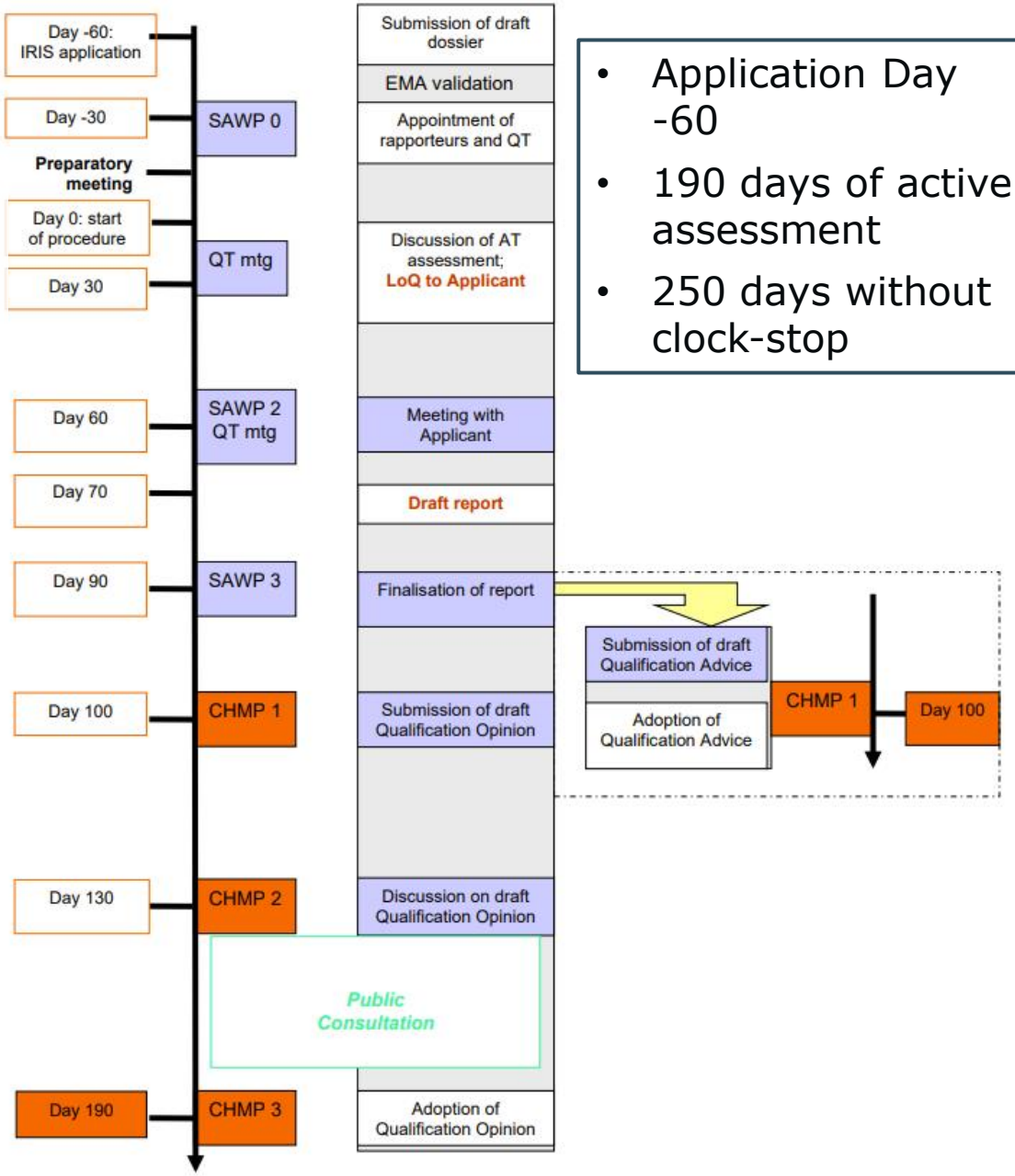
EMA qualification pathway

Qualification advice

Qualification opinion

Guidance to applicants: EMA/CHMP/SAWP/728



- Application Day -60
- 190 days of active assessment
- 250 days without clock-stop

https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/q
development-guidance-applicants_en.pdf

How: the road to EMA qualification opinion

Refined objective

Qualification of method

- Qualification of Framework
- Points to consider for method selection

CHMP & EUnetHTA parallel

CHMP

Pre-consultation

Preparatory call EMA	**10/18**
EMA Premeeting	03/19
Draft briefing book	10/19

Qualification advice

Start	11/19
List of issues	02/20
Written response	03/20
Discussion meeting	03/20
CHMP advice	04/20

Qualification opinion (QO)

Preparatory call EMA	02/21
Briefing book	03/21
Written response	04/21
Discussion meeting	05/21
Draft opinion	09/21
Public consultation	10-11/21
Published final opinion	**05/22**

Iterative process!

Outcome:

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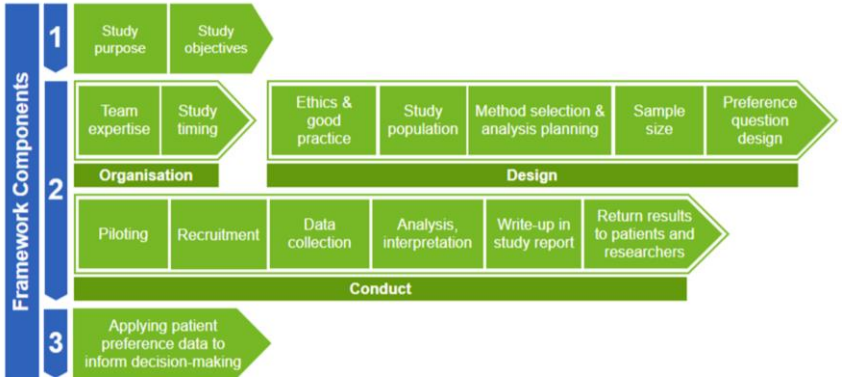
PREFER framework for PPS



Points to consider on method selection

what preference methods is most suitable to the research question

Outcome:
Highlights of qualification endorsements

- Platform for useful interactions between industry, regulators (and HTA bodies/payers, as well as patients) and could guide decision-makers
- The 'principal objectives of the framework with its components and sub-elements
- Information on technical approaches and examples for applying PPS are considered valuable
- A relevant spectrum of methods and points to consider for method selection presented

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Outcome:
Open topics within the Qualification Opinion

- How to define a preference sensitive situation
- How regulators will disclose their use of PPS to inform decision remains a matter of individual assessment
- Use cases/methods within the Points to Consider remain a selection and do not pre-empt regulatory decision

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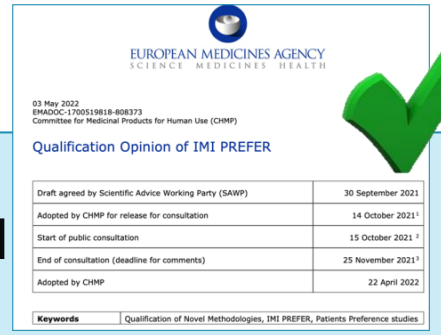
Why? What is the value of EMA qualification?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Direct – value of the outcome

- Regulatory assessment and opinion of application of novel method in medicine development
  - ➔ "endpoint selection" & "identify and value trade-offs for benefits and risks"
- Value for patients, regulators, researchers and developers

Barbier L, Muller M et al. (2022)

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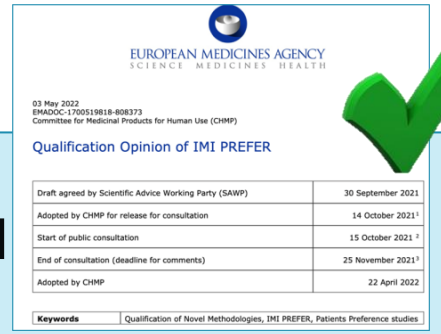
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Direct – value of the outcome

- Regulatory assessment and opinion of application of novel method in medicine development
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- Value for patients, regulators, researchers and developers

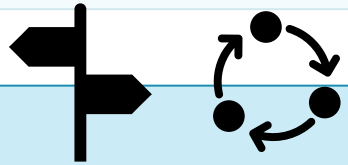
- Move from direct to structured patient input in regulatory/HTA decision-making
- Considerations on how to evaluate PPS and support decision-making; possible foundation to inform ICH guidelines (ICH PFDD)
- Set of tool to design, conduct and analyse PPS
- Advice on why, when and how to use PPS in medical product decision-making; increased certainty to the development and presentation of PPI in submissions

Barbier L, Muller M et al. (2022)
PPI: Patient preferences information; PPS: Patient preferences studies

Why? What is the value of EMA qualification?

Direct – value of the outcome

- Regulatory endorsement of application of novel method in medicine development:
  - ➔ "endpoint selection" & "identify and value trade-offs for benefits and risks"
- Value to patients, developers, researchers, regulators



Indirect – value of the process

- Structured exchange builds familiarity & understanding
- Informs scientific approach and methodological considerations; redirect research
- Helps refine thinking & crystalize understanding of value and regulatory applicability (context of use)
- Insight into different regulatory views & consensus building
- Public consultation & final opinion; raises awareness and interest

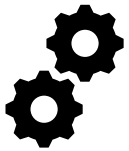
Barbier L, Muller M et al. (2022)

Key take-aways

An important step forward towards more structured conduct and evaluation of PPS in decision-making

Learnings from the qualification provide clarity into the value and applicability of PPS in medical product development

Process increased familiarity & awareness about PPS



Qualification Opinion serves as foundation for future research

Thank you

Contact:

liese.barbier@kuleuven.be

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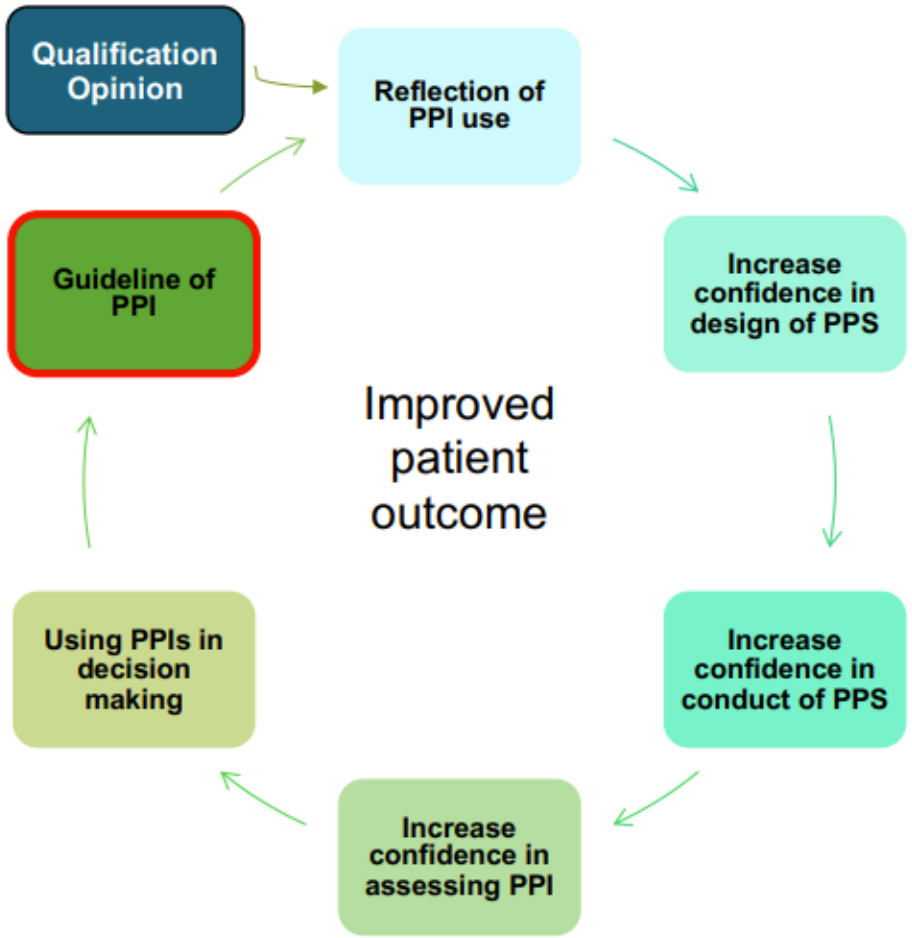
Back-up slides

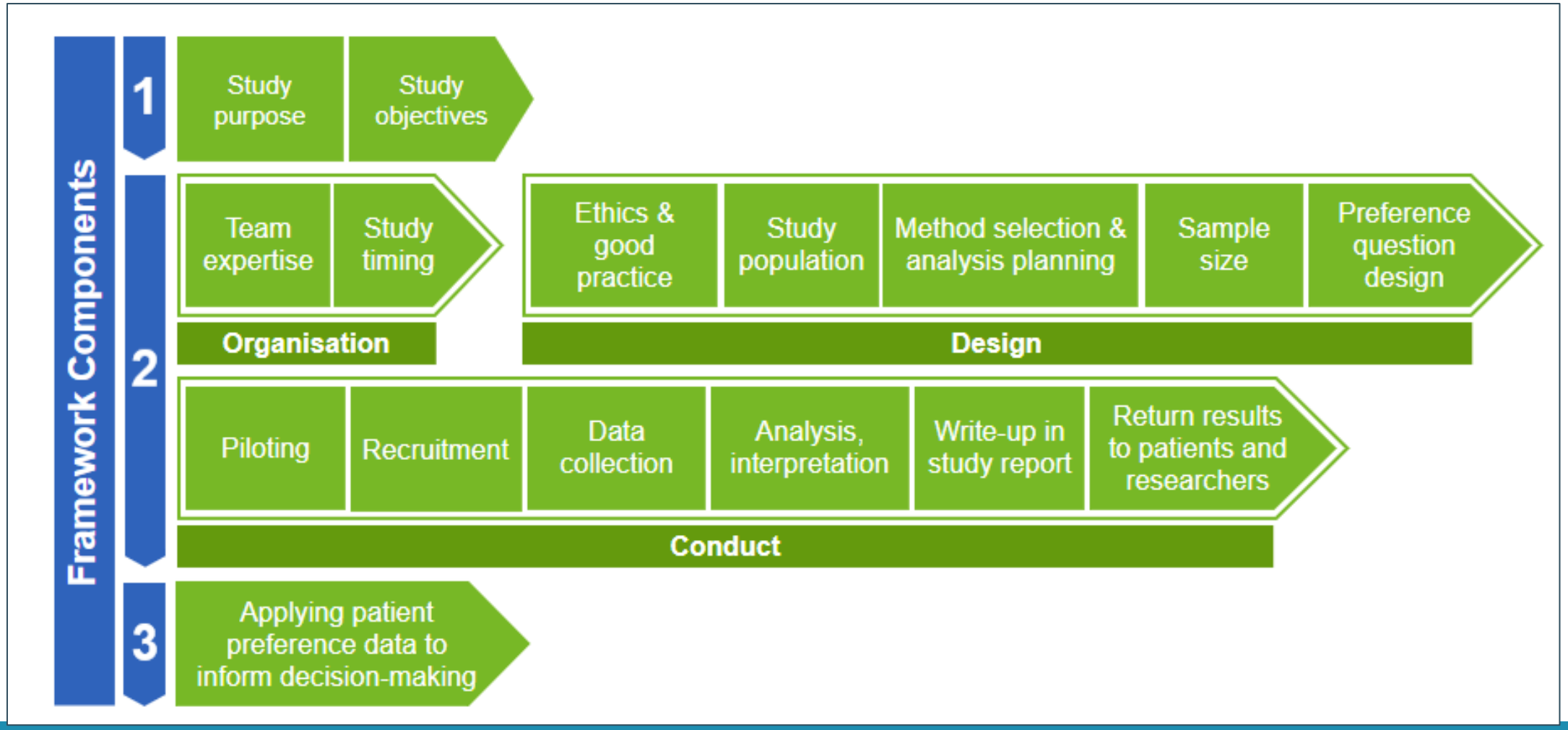
Possible future benefits of this QO?



- Strengthen drug development – design trials with endpoints that matter to patients
- Move from direct patient input to more systematic patient involvement
- Support decision-making across all stages of medical product life-cycle
- Increase the adoption of patient preferences (studies and information) through transparency

PREFER webinar on the EMA qualification opinion, 2022
PPI: Patient preferences information; PPS: Patient preferences studies

Key message: **PREFER Framework**



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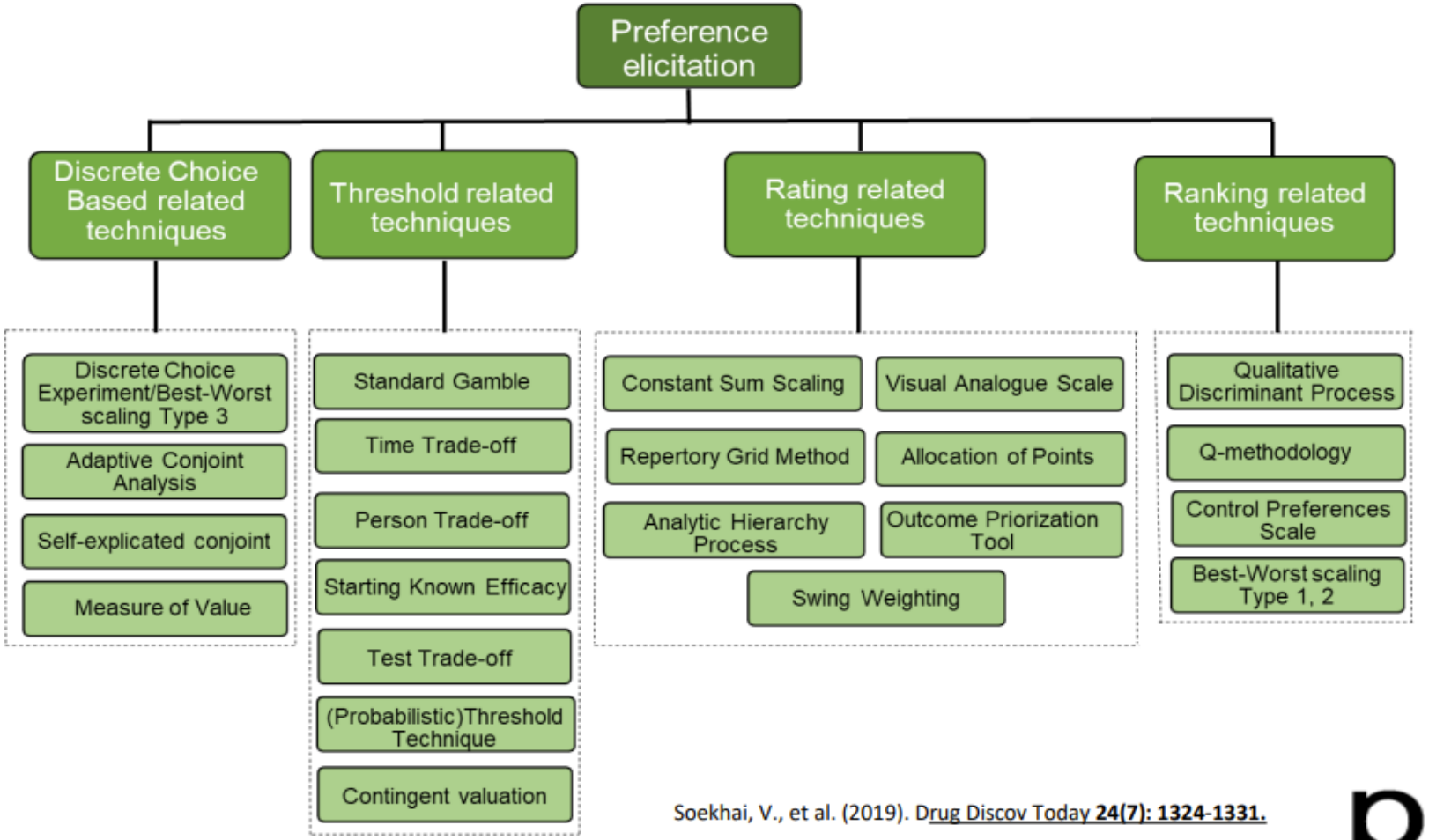
T2.4: Preference elicitation methods



Soekhai, V., et al. (2019). Drug Discov Today **24(7): 1324-1331.**

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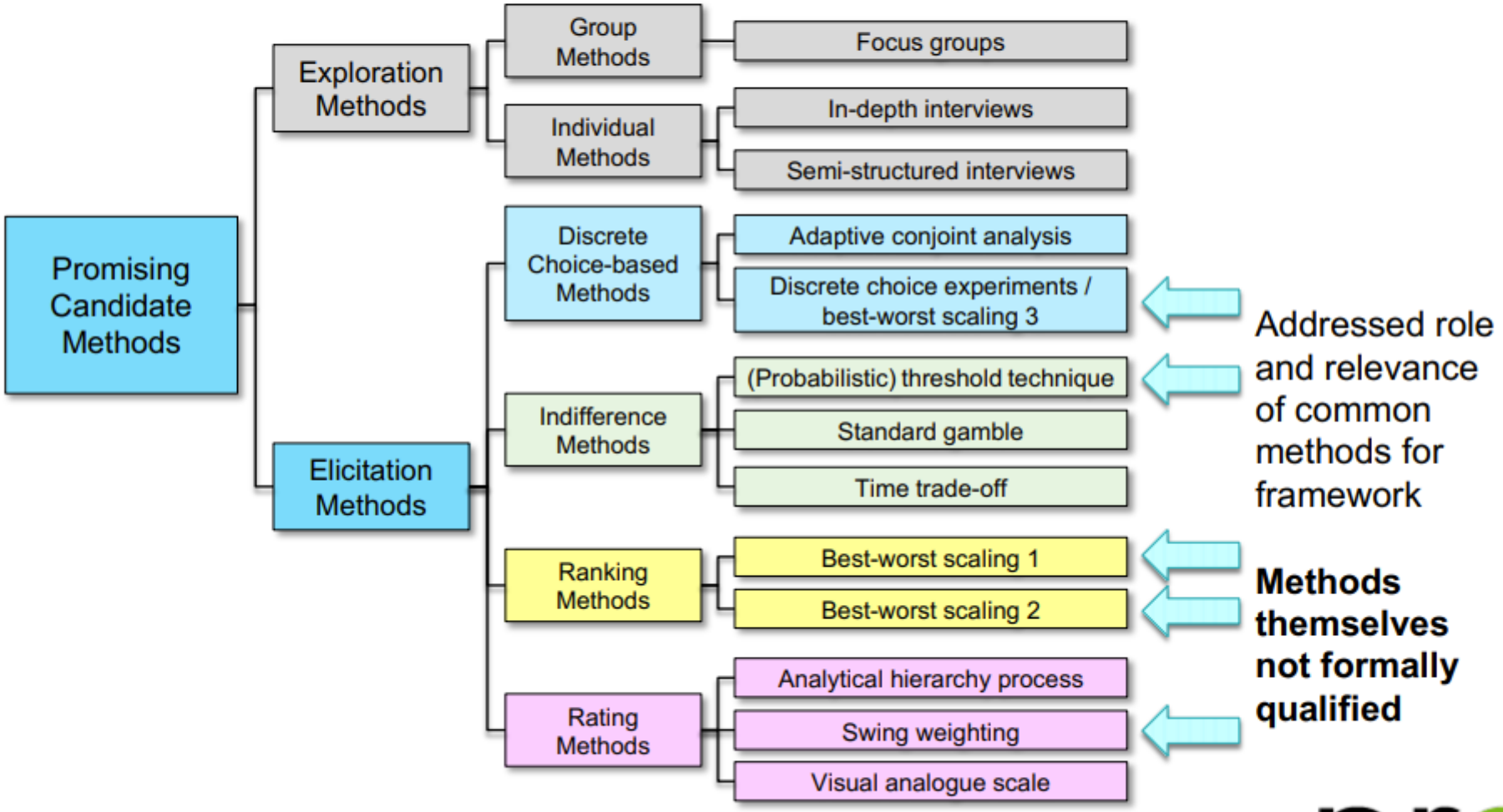
Most promising Preference Methods: 5 Methods chosen



Whichello C, Levitan B, Juhaeri J, Patadia V, DiSantostefano R, Pinto CA, et al.. BMC Med Inform Decis Mak. 2020;20(1):114.

Categories of Points to Consider

Category	Description
Methodological	Factors related to the specific requirements and capabilities of the method. Methodological factors primarily address the ability of the method to answer the research question and the extent to which the quality of the data can be assessed. Methodological factors can also influence the suitability of a method for different types of participants and the feasibility of implementing the method given resource constraints of the study sponsor.
Participant	Factors related to the characteristics of the patient population, the ability of individual patients to participate in the preference elicitation exercise, and the ability of the available patient population to provide the type and amount of information required by the method.
Feasibility	Factors related to the expertise required to implement the method, conduct the study, and analyze the data.

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Methodological Points to Consider

1. Does the method provide estimates at the level of the individual, the sample, or both?
2. Can the method account for and/or test for sources of preference heterogeneity?
3. What minimum sample size is required to implement the method?
4. Can the method elicit preferences for individual treatment features or attributes? If so, how many features or attributes can be evaluated?
5. Can the method elicit preferences over multiple levels of each feature or attribute?
6. Does the method directly elicit relative importance, trade-offs, or both?
7. If the method can be used to estimate trade-offs, can it directly estimate trade-offs among multiple attributes simultaneously (or are trade-offs estimated pairwise)?
8. Can the research method accommodate interactions between treatment features or attributes?

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