



EFCCA

# What matters most to patients?

A European patient preference study



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**KU LEUVEN**

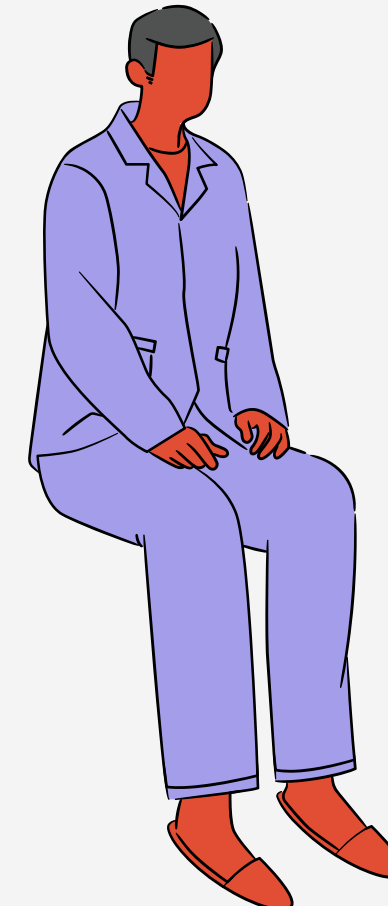
# Rationale: what matters to patients?

Increase in treatment response  
Novel administration form

Surgery  
Sleeping problems  
Fatigue  
Urgency



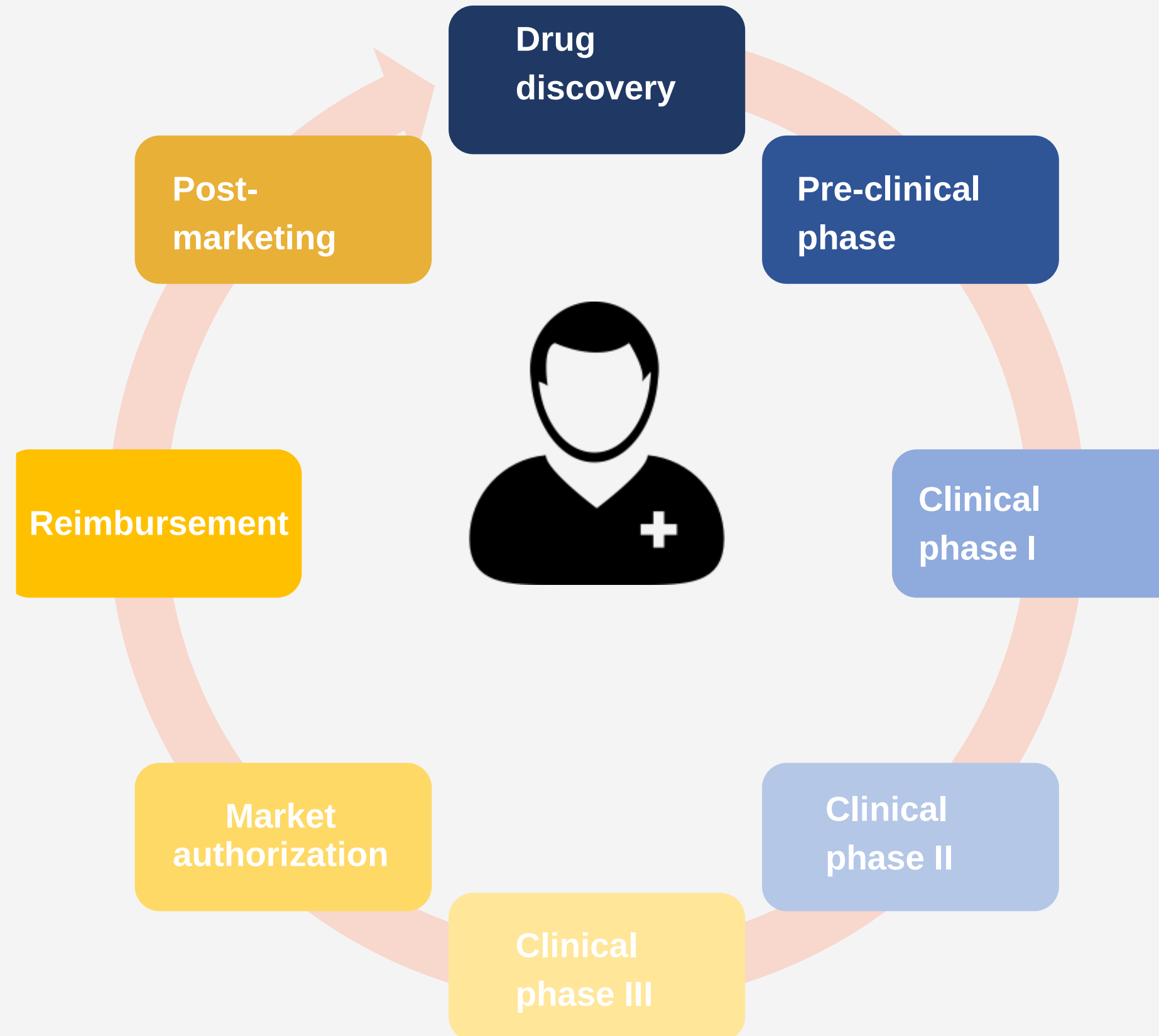
Other stakeholders: academics, drug developers, regulators, payers, clinicians ...



Patients' preferences



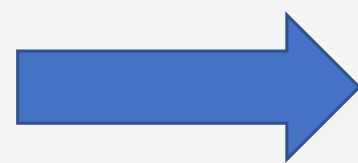
# Rationale: what matters to patients?





# What are patient preferences?

“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”



Benefits



Risks/inconveniences



Uncertainties

**PATIENT PREFERENCE STUDIES**

# When are patient preferences particularly valuable in decision-making?



In patient preference sensitive decisions, namely:

When it is unclear **what are the most important disease or medical product characteristics to patients**

When there are **multiple treatment options** and no option is clearly superior

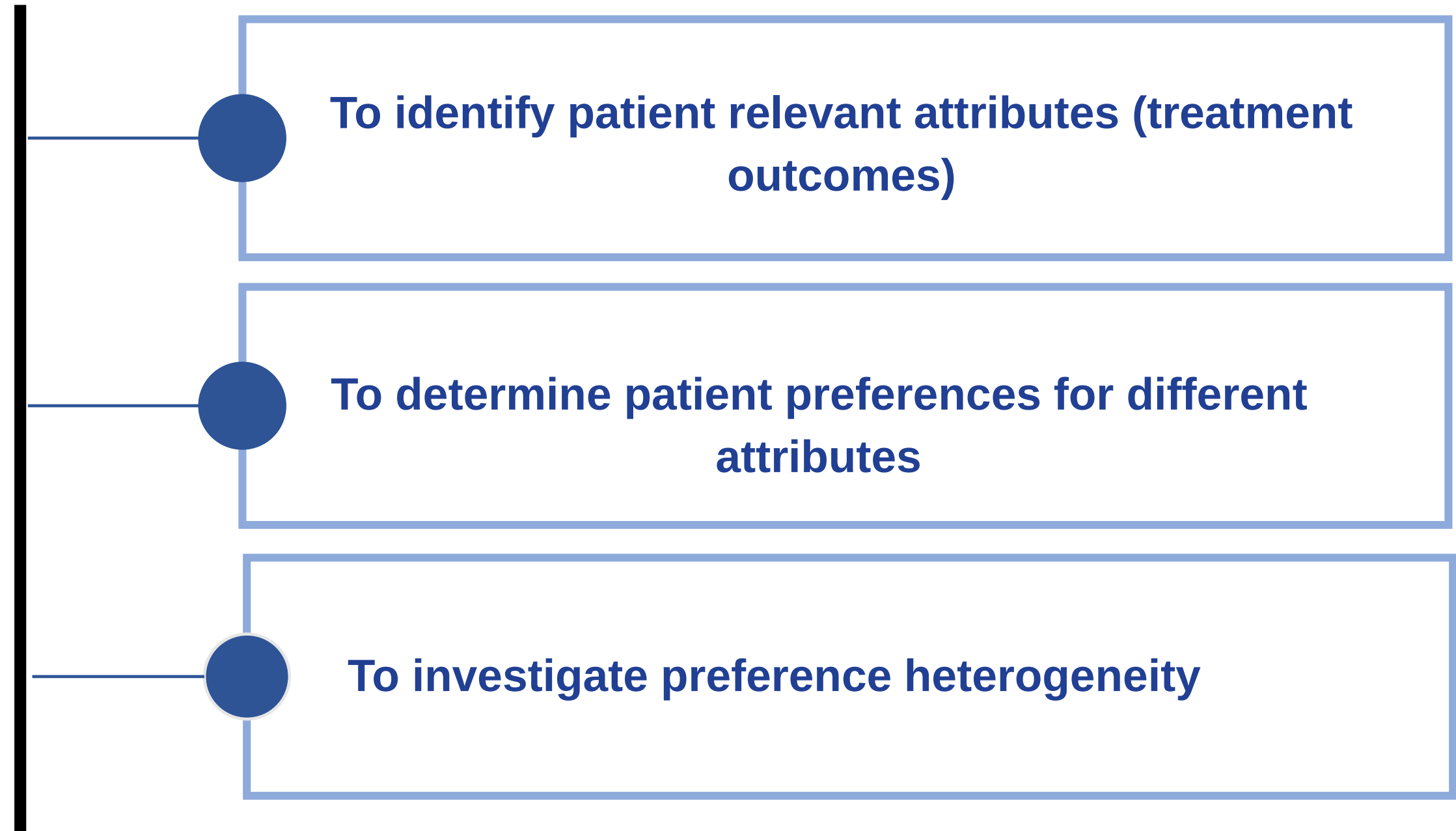
When the **evidence** supporting one option over another **is very uncertain or variable**

When there is **potential for considerable heterogeneity** in views between patients

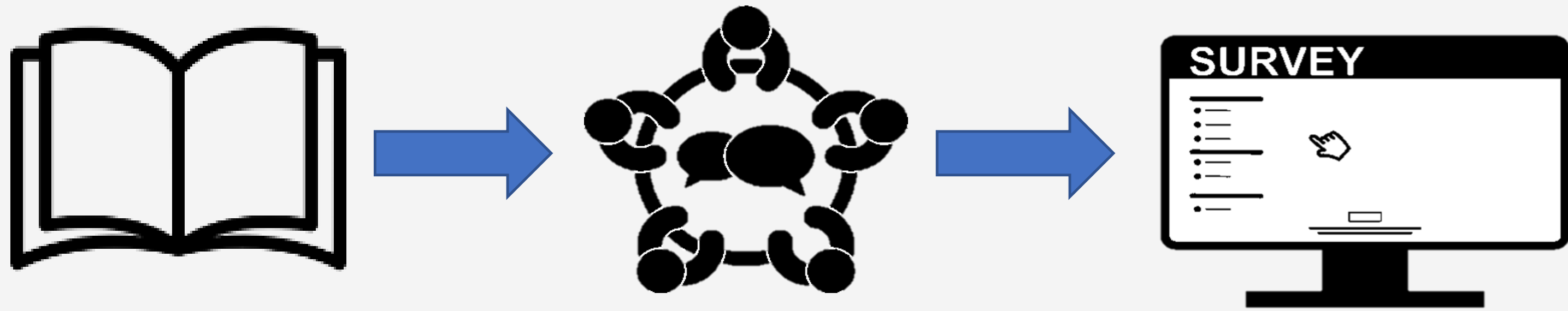
# Patient preference study in inflammatory bowel disease



## Objectives



# Different steps of a patient preference study



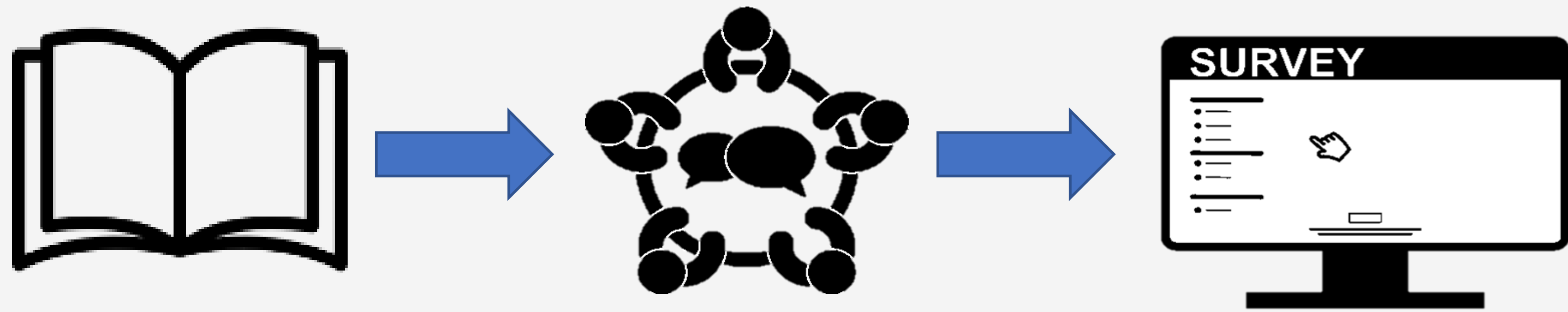
Literature review

Focus group discussions

Survey



# Different steps of a patient preference study



Literature review

Focus group discussions

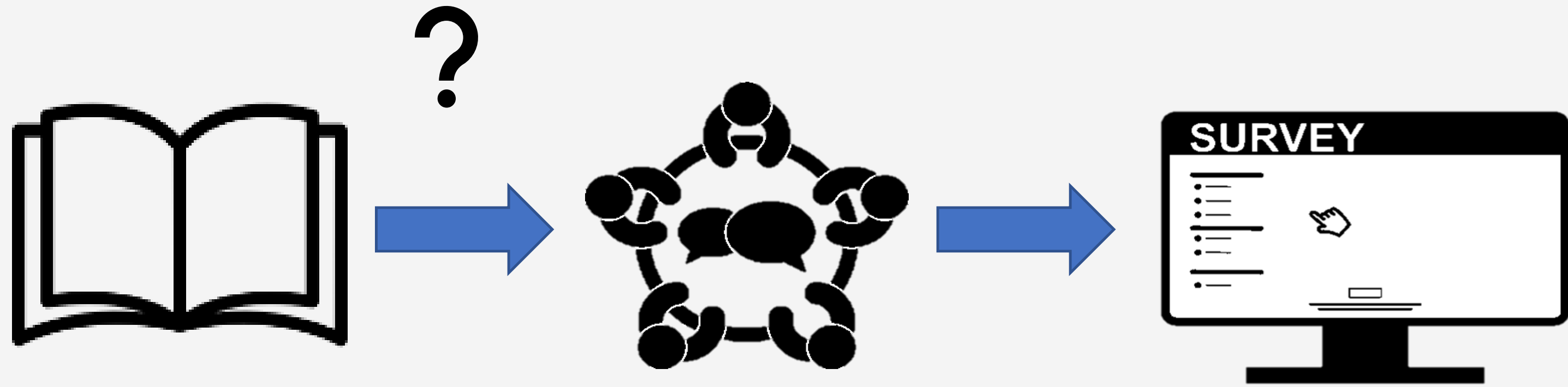
Survey

Partnership!





# Different steps of a patient preference study



Literature review

Focus group discussions

Survey

# Results literature review



## SCOPING LITERATURE REVIEW

- Previous patient preference studies
- Available IBD treatments
- IBD clinical trials



**List of characteristics  
possibly  
important to IBD patients**



**Further used in the focus  
group discussions**

Characteristic	Explanation	Grading
Characteristics related to the treatment efficacy		
Short-term clinical response	The treatment reduces symptoms of the disease in the short-term	
Long-term clinical remission	The treatment ensures that the disease is suppressed, disappears, and stays away for a long time	
Prevention of flare-ups	The treatment prevents disease flare-ups whereby the complaints get worse, or whereby new complaints arise	
Macroscopic healing of the intestinal mucosa	The treatment ensures an absence of fragility, blood, damage, and sores in the intestinal mucosa	
Microscopic healing of the intestinal mucosa	The treatment ensures an absence of abnormalities when the intestinal tissue (biopsies) is examined under a microscope	

# Focus group discussions



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**Nominal group technique**



**In-depth discussion**





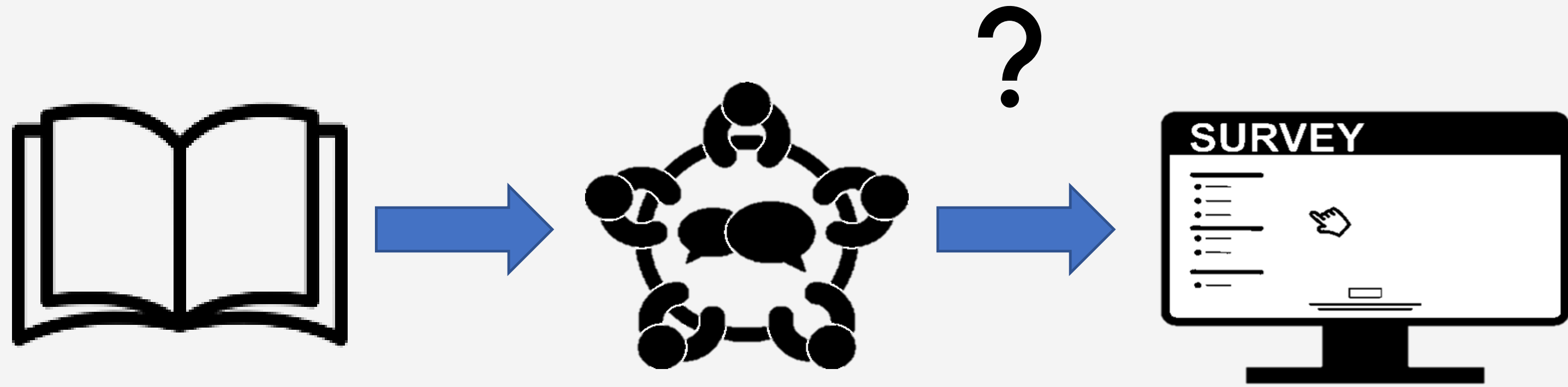
# Patient prioritized characteristics



Rank	Characteristic	Mean score
1	Prevent surgery	9,55
2	Long-term clinical remission	9,45
3	Improved quality of life	9,36
4	Occurrence of urgency	9,27
5	Improved labor rate	9,27
6	Occurrence of diarrhea	9,18
7	Occurrence of severe infections	9,18
8	Prevent hospitalization	9,09
9	Occurrence of joint pain	9,00
10	Prevent flare-ups	9,00

- Importance of **gastrointestinal problems, quality of life, and surgery** in line with qualitative results
- Characteristics related to **administration and frequency** less important

# Different steps of a patient preference study



Literature review

Focus group discussions

Survey

# Development of attributes and levels



## Multidisciplinary advisory board meetings and revision rounds

- Iterative process
- Final list of 14 attributes related to IBD patients treatment and/or disease



### **Risk of undergoing SURGERY**

This is the risk that you need to undergo surgery because:

- Medical therapy cannot adequately control your intestinal inflammation
- You have recurrent flare-ups
- There is a puncture in the wall of your bowel (perforation), a narrowing in a part of your bowel (stricture), or a pus-filled area in the wall of your bowel (abscess)
- There is a high risk of cancer in the bowel
- There is cancer in the bowel

Surgery may be accompanied by the construction of a (temporary) stoma.



### **FREQUENCY of having to go to the toilet**

This is the frequency that you have to go to the toilet.



### **URGENCY and PAIN of having to go to the toilet**

This is the urgency that you have to go to the toilet and the pain that you experience with it.



### **Severity of daily ABDOMINAL PAIN and CRAMPS**

This is the severity of abdominal pain and cramps you may daily experience.



### **Severity of FATIGUE**


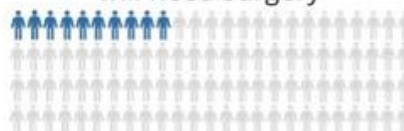










This is the severity of an overwhelming sense of tiredness, lack of energy, or feeling of exhaustion that is not relieved after rest or sleep.



# Online survey



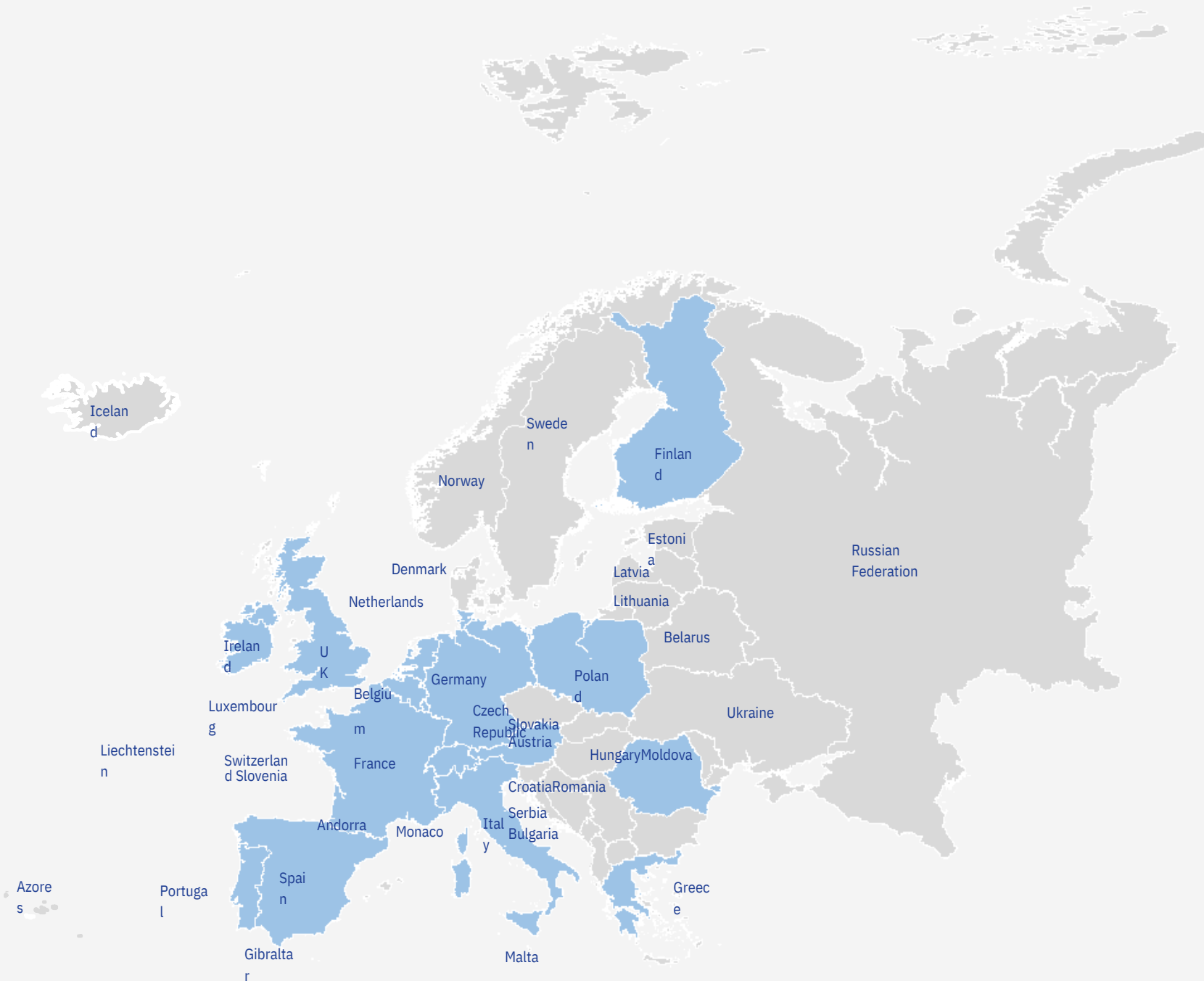
- **Discrete choice experiment (DCE) elicitation questions**
- **Sociodemographic and treatment related questions**

	Treatment A	Treatment B
<b>Risk of undergoing SURGERY</b> 	HIGH risk: 10 out of 100 people (10%) who take this treatment will need surgery 	HIGH risk: 10 out of 100 people (10%) who take this treatment will need surgery 
<b>FREQUENCY of having to go to the toilet</b> 	High frequency, more than before you had inflammatory bowel disease 	Normal frequency, as before you had inflammatory bowel disease 
<b>Severity of daily ABDOMINAL PAIN and CRAMPS</b> 	Severe pain and cramps 	No pain and cramps 
<b>How FAST the treatment will work</b> 	Fast reduction of symptoms (within first 2 weeks after starting the treatment) 	Slow reduction of symptoms (3 months after starting the treatment) 
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# Next steps: piloting, translation and dissemination of the survey



- European Scope
- Recruitment: patient organisations + clinicians



# Use of results



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 <sup>1</sup>
Start of public consultation	15 October 2021 <sup>2</sup>
End of consultation (deadline for comments)	25 November 2021 <sup>3</sup>
Adopted by CHMP	22 April 2022

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

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



Tool for  
EFCCA



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# Thank you!

Questions or contact, feel free to reach out to me:  
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