PREFER

Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle

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About the PREFER project

The Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle (PREFER) is a five year project that has received funding from the **Innovative Medicines Initiative** 2 Joint Undertaking under grant agreement No 115966. This Joint Undertaking receives support from the European Union's **Horizon 2020** research and innovation programme and **EFPIA**.

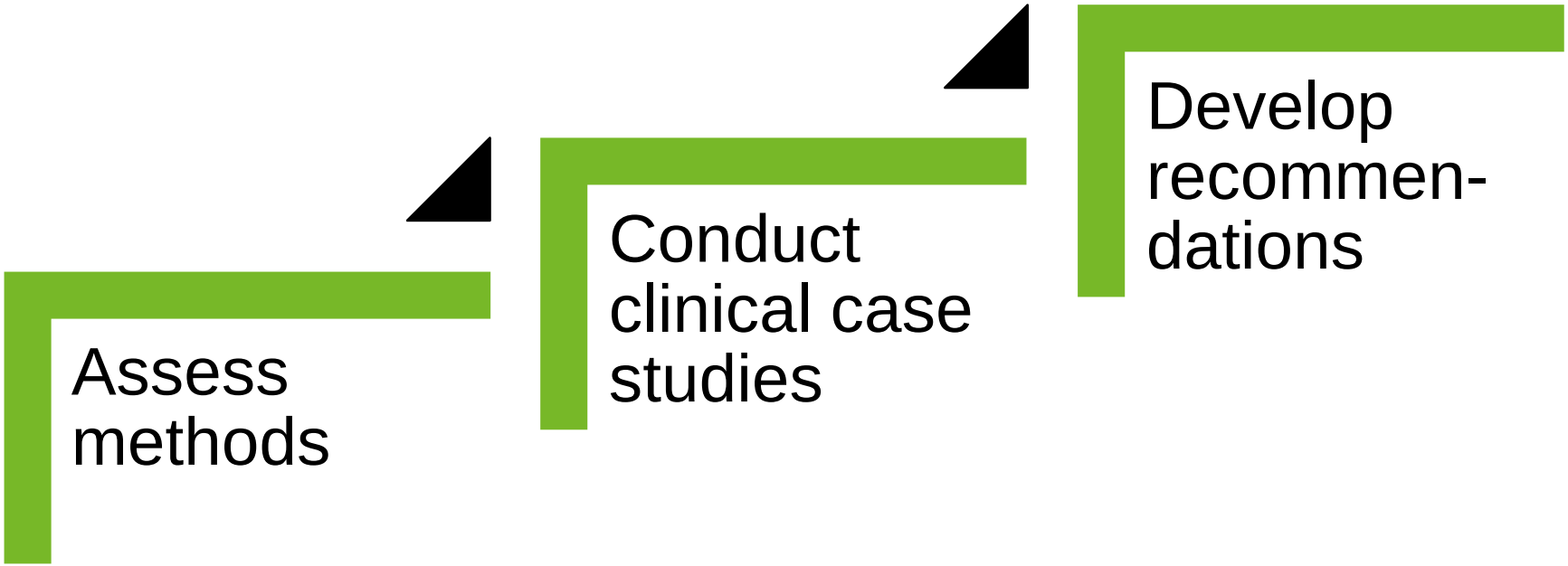
Health preference research

1. What matters to patients?
2. How much does it matter?
3. What matters most?

At different decision points in the medical product life cycle.

Well informed and reflected if used in regulatory decisions

Stepwise approach



Results

- Through literature review, 143 semi-structured interviews and focus groups with stake holders:
- lack of consensus:
- on definition and role of patient preferences
- on the study design, and conduct of patient preference studies
- Patients want to be well-informed and that heterogeneity is acknowledged
- 32 preference exploration and elicitation methods identified that have been applied to the development of medicinal products and medical devices.
- Although the use of patient preferences is desired by stakeholders, their concerns and requirements need to be addressed before patient preferences can be integrated throughout the medical product life cycle

Example Questions for Case Studies

	Concerns	Research question
Industry	Patient sample	To what degree does repeat application of a method on different samples from the same population give similar results ?
Regulator	Generalizability of the results	How generalizable are preferences from one specific population in a disease to different populations in that or related diseases?
HTA	Study design (e.g. risk of influencing PP research)	To what degree do small changes in the number, type and definitions of attributes impact results for a given method
Patient	Lack of patient knowledge and education	Does a serious game improve the understandability of questions in preference studies significantly without impacting the results?

Case Studies

12 high potential PP methods to use

At least 3 disease case study teams to fit PREFER methodological questions with clinical questions (lung cancer, reuma arthritis, and neuromuscular disorders)

Simulation studies

An RA (side) study (Karin Schölin Bywall)

- What do RA-patients prefer when stepping up RA treatment with (another) biologic or JAK-inhibitor?
- Will interactive information affect preferences?
- Literature and expert reviews, focus groups:
- Attributes: route of administration, frequency of administration, reduction of disease activity, improvement in functional capacity, seriousness of side effect, chance of getting a side-effect, monthly costs for society and number of sick days.
- Patient partners
- DCE based on Swedish RA-registry (N=5000)