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

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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.
Unmet need PREFER addresses

Currently a lack of understanding of how patient perspectives on benefits and risks can best inform decision-making.

PREFER aims to identify, characterise, and apply preference elicitation methods in all stages of drug life cycle.
PREFER project goals

• Establish **recommendations to support development of guidelines** for
  – Industry
  – Regulatory Authorities
  – HTA bodies & payers

• **on how and when to include patient preferences** on benefits and risks of medical products.
PREFER approach

• Run patient preference studies in academic and industry settings.

• Provide a better understanding of the best-practice approach to patient-preference studies.

• Show how patient preference studies can support decision making for industry, regulators and HTA bodies.
Seeks methods for all stages

Pre-discovery
Upstream research

Drug Discovery

Preclinical

Clinical drug development
(Phase I, II, III)

Post approval
(Phase IV)
Public-private partnership

- **Coordinator**: Uppsala University (Mats Hansson)
- **Project leader**: Novartis Pharma (Conny Berlin)
  - 10 Academic research institutions
  - 4 Patient organisations
  - 1 Health Technology Assessment body
  - 2 SMEs (small and medium sized enterprises)
  - 16 Pharmaceutical companies
PREFER partners
Organisation of PREFER

- **Shared leadership at all levels**
  - From leadership, to work packages to tasks
- **Stakeholder partners & advisory groups**
  - Patient Advisory Group
  - HTA and Payers Advisory Group
  - Regulatory Advisory Group
- **Scientific & Ethics advisory boards**
Stakeholder advisory groups

PATIENTS
4 partners

HTA AND Payers
1 partner, 5 external advisors

REGULATORS
External advisors

Committee for Medicinal Products for Human Use (CHMP)
Scientific Advice Working Party (SAWP)
Committee for Orphan Medicinal Products (COMP)

EPF
European Patients Forum
International Alliance of Patients’ Organizations
Muscular Dystrophy UK

KCE
Kernkommissie voor de Racemativering
Centre National d’Expertise des Soins de Santé
Belgian Health Care Knowledge Centre

Ludwig Boltzmann Institut
Health Technology Assessment

Gemeinsamer Bundesausschuss

CADTH
Canadian Agency for Drugs and Technologies in Health

INAMI
Institut National des Affaires Sanitaires

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PREFER work packages

Work Package 1
Project management, communication, stakeholder coordination

Work Package 2
Identify stakeholder’s needs and methodologies

Work Package 3
Testing in clinical case studies

Work Package 4
Developing recommendations
Stepwise approach

- Assess methods
- Conduct clinical case studies
- Develop recommendations
1: Assessing methods

- Literature review
- Interviews and focus group meetings with
  - patient organisations & patients
  - physicians
  - regulatory authorities
  - health technology assessment bodies & payers
  - industry experts
  - & academics

on their key concerns, needs, expectations and desires on the assessment and use of patient preferences.
Patient preference studies to be conducted in three disease areas where patients and clinical research partners already provide expertise:

- Cancer
- Rheumatoid arthritis
- Neuromuscular disorders

Partners from the pharmaceutical industry will provide additional patient preference studies to cover disease areas from the companies’ portfolio.
3: Recommendations

- **Mid-2019**: draft recommendations to be available, testing in other disease areas and decision points by stakeholder advisory groups.
- **Mid-2021**: refined draft recommendations to be available
- **Autumn 2021**: Final recommendations to be presented.
In summary, PREFER

- Will develop evidence-based recommendations to guide industry, Regulatory Authorities, HTA bodies, reimbursement agencies
- Carried out by a diverse consortium that involves stakeholders: both as partners and advisors
More information

www.imi-prefer.eu