Integrating patient preferences in the drug life cycle
The basic concepts and why it is important

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Disclaimer: This presentation and its contents reflects the view of the presenter and not the view of PREFER, IMI, the European Union or EFPIA.
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.
The objective of PREFER

To develop recommendations for measuring and using patient preferences in industry, regulatory, and health technology assessment body/reimbursement agency decision-making across the drug life cycle

- Patient preferences?
- The drug life cycle?
- Decision-making?
Overview of today’s presentation

1. What are "patient preferences"?
2. The drug life cycle
3. The main decisions of the drug life cycle
4. How can patient preferences contribute in these decisions
5. PREFER’s approach
What are “patient preferences”?

• Difficult question…
• Defined by the Food and Drug Administration (US):

“the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions” (1)

What are patient preferences?

• Or in plain language:

“Patient preferences reflect why patients choose a particular health intervention over other available options. This health treatment can be a drug or a medical device. A preference can be stated for a health intervention as a whole or for the advantages and disadvantages of one intervention. In order to make a choice or state a preference, patients need to weigh up the advantages and disadvantages and compare them to those of other health intervention.”
The main **decisions** in the drug life cycle

- Pre-discovery, Discovery and pre-clinical
- Clinical drug development (Phase I, II, III)
- Post approval (Phase IV)
The main **decisions** in the drug life cycle

1. **Industry decisions:** e.g. “Which product will we develop?”

2. **Regulatory decision:** “*Do we allow the drug to come on the market?*”
   
   Decision mainly based on **benefits** (=does the drug work) relative to **risks** (=side effects)

3. **Reimbursement decision:** “*What will the healthcare payer and patient have to pay for this drug?*”

   Decision based on **more** than benefits and risks: e.g. cost of the treatment, impact on national health budget, improvement in outcomes compared to existing treatments
Patient preferences in these decisions

1. Industry decisions: e.g. “Which product will we develop?”
   - e.g. Patient preference studies to help industry define areas of unmet medical needs

2. Regulatory decision: “Do we allow the drug to come on the market?”
   - e.g. Patient preference studies to provide regulators better understanding of how patients value benefits and risks

3. Reimbursement decision: “What will the healthcare payer and patient have to pay for this drug?”
   - e.g. Patient preference studies are performed to provide payers with a better understanding of how valuable and important the better outcomes of the drug are to patients
Many questions still remain

• What are the decisions where patient preferences can be used?
• What do stakeholders need in order to integrate patient preferences in their decisions?
• What methods are best suited to inform their decisions?
• ...

How does PREFER addresses these questions?

1. By identifying **decision-making processes** where patient preferences could be used and identifying **stakeholders’** desires, expectations, requirements and concerns (WP2)

2. By identifying available **methods** for measuring patient preferences and quality criteria for these methods (WP2)

3. By conducting **patient preference studies** (WP3)

4. By developing **recommendations** to guide industry, regulatory authorities and HTA/reimbursement bodies (WP4)

All of the above will be done with **intensive communication with all stakeholders and in particular with patient representatives**
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
Public-private partnership

• **Coordinator:** Uppsala University
• **Project leader:** Novartis Pharma
  – 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

- **European Cancer Patient Coalition**
- **European Patients Forum**
- **International Alliance of Patients’ Organizations**
- **Muscular Dystrophy UK**

## HTA AND PAYERS
1 partner, 6 external advisors

- **KCE** (Koninklijk Centrum voor de Gezondheidszorg)
- **Ludwig Boltzmann Institut** (Health Technology Assessment)
- **Gemeinsamer Bundesausschuss**
- **CADTH**
- **SBU**
- **INAMI**
- **Eunetha**

## REGULATORS
External advisors

- **Committee for Medicinal Products for Human Use (CHMP)**
- **Scientific Advice Working Party (SAWP)**
- **Committee for Orphan Medicinal Products (COMP)**
1: Assessing methods

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

  on their key concerns, needs, expectations and desires on the assessment and use of patient preferences.
2: Clinical case studies

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