Integrating patient preferences in the drug life cycle

The basic concepts and why it is important

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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**.

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)

(1) Patient Preference Information - U.S. Department of Health and Human Services, Food and Drug Administration, Center for Devices and Radiological Health and Center for Biologics Evaluation and Research; 2016.

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

The main decisions in the drug life cycle

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)

1. Industry decisions: e.g. "Which product will we develop?"

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

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Patient preferences in these decisions

e.g. Patient preference studies to provide regulators better understanding of how patients value benefits and risks

2. Regulatory decision: *"Do we allow the drug to come on the market?"*

1. Industry decisions: e.g. "Which product will we develop?"

e.g. Patient preference studies to help industry define areas of unmet medical needs

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

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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)

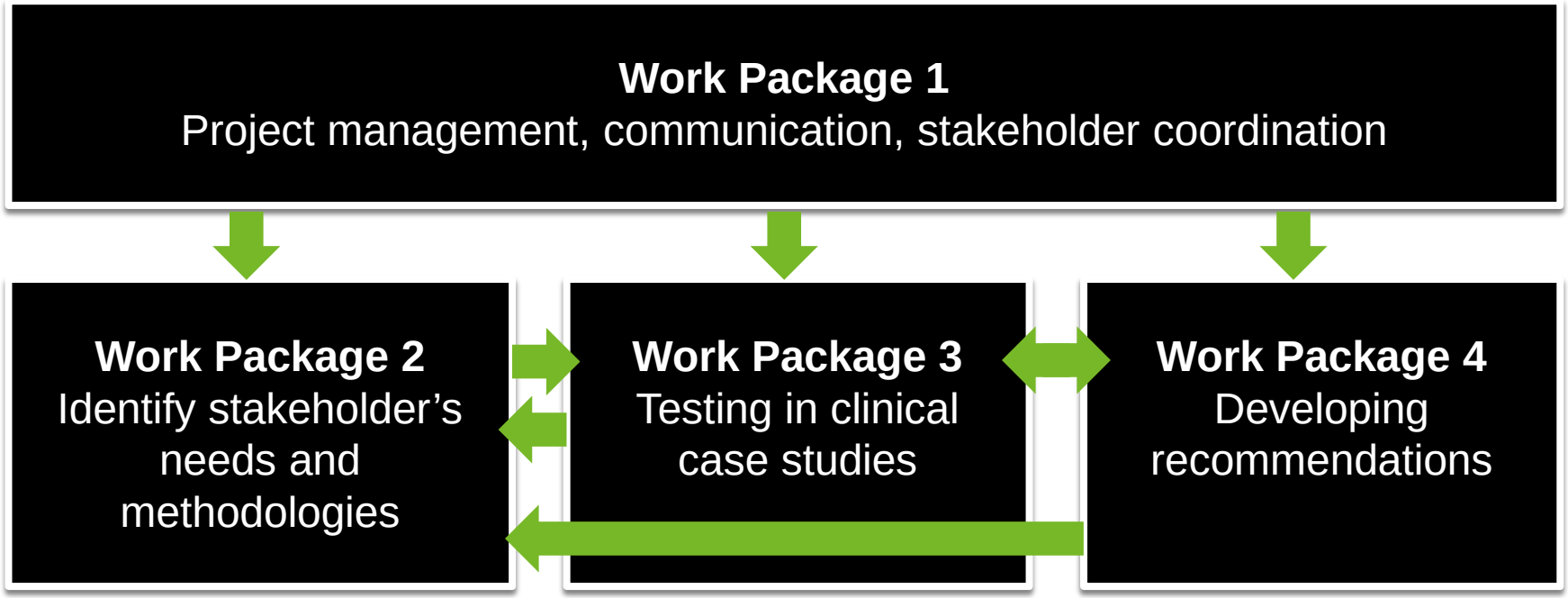
2. By identifying available **methods** for measuring patient preferences and quality criteria for these methods (WP2)

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



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

EPF European Patients Forum

International Alliance of Patients' Organizations

Muscular Dystrophy UK
Fighting muscle-wasting conditions

HTA AND PAYERS
1 partner, 6 external advisors

KCE
Federaal Kenniscentrum voor de Gezondheidszorg
Centre Fédéral d'Expertise des Soins de Santé
Belgian Health Care Knowledge Centre

Ludwig Boltzmann Institut
Health Technology Assessment

Gemeinsamer Bundesausschuss

CADTH

SBU

INAMI

eunethta

REGULATORS
External advisors

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Committee for Medicinal Products for Human Use (CHMP)

Scientific Advice Working Party (SAWP)

Committee for Orphan Medicinal Products (COMP)

FDA
Center for Biologics Evaluation and Research

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1: Assessing methods

WP 2

- 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

WP 3

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

WP 4

- **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