Aligning Drug Development with Future Patient-Centric Market Access (PACEMA) requirements

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Abstract

Project background: Drugs often do not realize market access due to clinical trial data which do not meet health technology assessment bodies (HTAs) requirements. Societal changes have caused an increasing awareness of the importance to include the patient-centric perspective during drug development. Patients' desire to give input into the different phases of the drug life cycle and patient engagement makes the patient an important stakeholder to incorporate at diverse levels, requiring data generation on patient preferences, patient relevant outcomes and real-life data. In addition, HTAs increasingly expect that innovative drugs address patient preferences and that patient benefits and patient relevant outcomes (e.g. quality of life) are demonstrated. There is however a knowledge gap about patient preferences, and lack of structured methodologies and tools for eliciting patient preferences useful in demonstration of cost-effectiveness in real-life.

Aim: The patient-centric market access (PACEMA) project aims to develop a set of new and innovative tools that allow patient-centric drug development, meeting future HTA requirements to realize market access of new drugs or therapies: 1. A standardized scenario-based interactive tool to elicit patient preferences. 2. The data on patient level will subsequently be incorporated in a validated dynamic disease patient-centric cost-effectiveness model, able to assess value of medications and allowing to analyze the impact of policies on cost-effectiveness in complex healthcare environments. 3. To facilitate patient and caregiver interaction, an educational scenario-based tool, allowing patient participation in the decision process on a therapeutic approach.

Approach: A systematic literature search is conducted to assess current methodologies to generate, process and integrate patient preference data. Current HTA requirements and expectations for inclusion of patient preference data in HTA submissions are assessed to identify the patient-centric market access tool requirements. A gap analysis is subsequently conducted between the identified HTA needs for future patient-centric tools and the systematic assessment of current methodologies, in order to conceptualize the key features of PACEMA tool development. The developed methods and approaches are then incorporated into 3 prototype tools meeting the scientific methodological rigor and in line with stakeholders' quality standard. The final objective is to validate the 3 PACEMA prototype tools in a real world situation, allowing to substantiate their effectiveness in eliciting and incorporating patient preference data useful in developing and realizing market access of new drugs or therapies.

Rationale: Patient empowerment causes shift towards patient-centric drug development

Societal needs
- Correct decisions by HTA bodies
- Patient centered decisions
- Patient empowerment

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PhD mission
- Realizing market access for innovative drugs
- Demonstrating correct value of drugs
- Involving patients in healthcare

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Pharma needs[1]
- Meeting HTA and regulatory requirements
- Patient centered development

HTA / Regulatory needs[2,3]
- Transparent decisions for society
- Scientific based decisions

PACEMA research objective: Development of innovative tools to ensure drug development meets future patient-centric criteria

Approach:

Partnership translating scientific approach into validated market access tools

Research objectives

Baekeland mandate
- PhD: doctoral research
- Clear economic objectives
- Added value in Flanders

Mandate holder
- Helping patients by improving involvement in healthcare

Integrating know-how

Providing scientific foundations

KU LEUVEN

Patients

ISMS

mind bytes

Meeting HTA requirements
- Market access process
- Scientific communication
- Models & analytics
- Healthcare Compliance

Integrating patient perspective
- Innovative communication
- Serious games
- Policy tools & dashboards
- Gamification and infographics

Methodology: Evidence-based systematic approach

Research phase

Qualitative

Quantitative

Assessment HTA requirements

Identify current HTA requirements and expectations on patient preference data
- Patient preferences
- Patient related outcomes
- Real-life impact on disease

GAP analysis
Identify tool requirements to align drug development with future HTA requirements

Assessment + appraisal existing preference elicitation methods

Assessment current methodologies
- Literature search
- Patient preference elicitation tools in pharma
- Patient preference elicitation tools in consumer
- Gamified elements in patient preference elicitation
- Individual decision aids

Tool conceptualization

Development phase

Tool development

Patient preferences elicitation tool

Identification patient preferences
- Scenario-based tool to determine parameters

Quantification patient preferences
- Scenario incorporation to educate and inform
- DCE to capture patient preferences and trade-off data

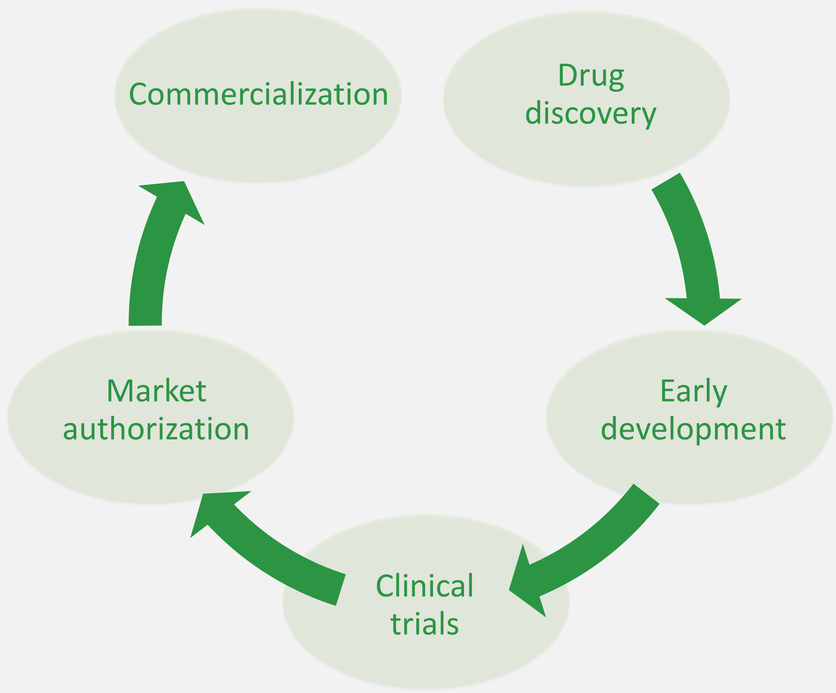
Patient-centric cost-effectiveness model
- Patient preferences
- Real-life long-term assessment
- Assessment societal impact

Treatment decision aid
- Educational scenario-based tool to facilitate patient – caregiver interaction

Valorization: Using patient preference data in the entire drug life cycle

Ensuring patient involvement in dialogue with healthcare providers
- Patient empowerment
- Improved health literacy
- Shared decision-making
- Facilitating communication between patients and their medical team

Translating patient-centric development into market access
- Improved value assessment of medicines
- Insight in impact on quality of life, patient relevant outcomes and utility
- Patient level data need to be integrated in body of evidence to substantiate patient value towards HTA and regulatory bodies



Using patient preferences to guide drug development
- Elicit clear and unbiased preferences
- Improved understanding relevant outcomes from payer perspective
- Develop clear recommendations to incorporate patient preferences in clinical trial design

References

1. M. Lowe. *Value in Health (19*), 2016 2. A. Mühlbacher. *Value in Health (19)*, 2016 3. FDA's Guidance on patient preference information, 2016