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Involving Patients in Oncology Drug Development: The Academic Perspective

CDDF MULTI-STAKEHOLDER WORKSHOP ON INVOLVING PATIENTS IN ONCOLOGY DRUG DEVELOPMENT
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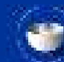

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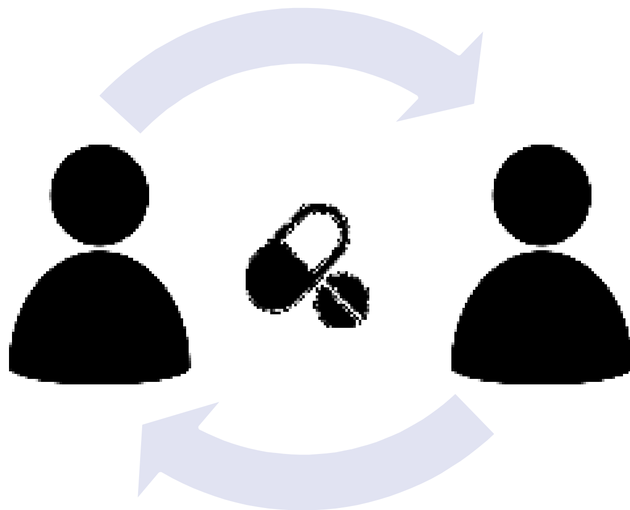
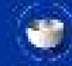
Why?

Drug development begins and ends with patients



1. **Unique insights of patients**
   - Experiential knowledge of disease and treatment
   - Patients` priorities, values and risk tolerances may differ from those of "traditional" decision-makers
2. **Unique position of patients**
   - End-consumers of healthcare
   - Directly affected by decisions concerning their health
3. **Impact of involving patients on the quality of drug development decisions**
   - Better alignment between drug development decisions and patient values and needs
   - Greater legitimacy of the decision by considering aspects that may not be considered by a professional panel of decision-makers



How?

Involve patients throughout the drug life cycle

Discovery and preclinical development

- **Disease understanding**
- **Medical need and focus of development**
- **Indication**
- **Route of administration**
- **Pharmaceutical form**

Clinical development

- **Clinical trial design**
  - Endpoint selection
  - PROs
  - Research protocol
  - Informed consent form
- **Feedback on:**
  - Frequency of administration
  - Dosage
  - Side effects

Marketing authorisation

- Relevance of clinical outcomes
- Benefit-risk trade-offs

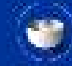
HTA/Reimbursement

- Unmet medical need
- Economic evaluations
- Relevance of clinical outcomes
- Cost-effectiveness assessment
- Predicting uptake rates

Commercialization

Patient feedback on:
- Administration
- Dosage
- Side effects
- PROs

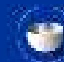
Janssens *et al.*, 2018, Frontiers in Medicine, https://doi.org/10.3389/fmed.2018.00285



Obstacles on different levels…

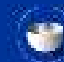
1. **Cultural and educational**
    - Different concepts: involvement, preferences, priorities, direct vs indirect involvement
    - Lack of broad consensus among all stakeholders in drug life cycle on patient involvement and preference methods
2. **Methodological**
    - Questions surrounding use of preference and involvement methods across life cycle:
        - Which methods to use
        - Bias
        - Ethics and compliance
3. **Procedural**
    - Lack of clarity on how to systematically integrate patient input and preference studies in drug decision-making across the drug life cycle
    - Lack of clear incentives for patient involvement and preference studies across life cycle:
        - Patient involvement requires time and financial resources
        - Patient involvement not mandatory

Janssens *et al.*, 2019, accepted, The Patient - Patient-Centered Outcomes Research



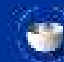
… require continued efforts on these levels

1. **Cultural and educational**
    - Increasing further the understanding of concepts (patient involvement, preferences, priorities) and methods for patient involvement
    - Wider consensus on use of involvement and preference methods
2. **Methodological**
    - Further developing quality criteria for involvement and preference methods across life cycle
        - Research into methodological challenges
        - Best practices
    - Towards more standardization via guidance on how to involve patients and conduct preference studies throughout lifecycle
3. **Procedural**
    - Increasing understanding on how to systematically integrate patient input and preference study results in decisions throughout life cycle

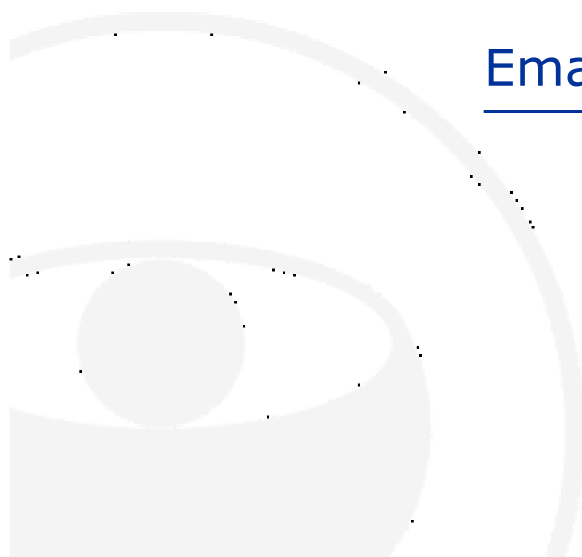


Concluding remarks

- Continued efforts by all stakeholders involved to translate obstacles into opportunities
- Already good experiences exist:
  - **EMA model of interaction** - progressively involved patients and worked to address these challenges: e.g. framework for interaction, agreed methodology, quality criteria for involvement, training and support, added value, etc.
  - Stakeholders to learn from each others` experiences to involve patients *systematically* in decisions *across* the life cycle
- Indirect involvement via preference studies:
  - Limited experience with preference studies in (regulatory) decisions
  - Further work necessary on how to both *measure and use* results from preference studies in decisions *across* the life cycle



Any questions, comments or ideas?



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