Review of Stakeholders Policies and Perspectives on Real-World Data (IMI-GetReal Deliverable)

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1. Introduction

- Although RCT’s provide valuable data on efficacy of medicines under ideal conditions, their results are not generalisable to routine clinical practice.
- Real-world data (RWD) (i.e. health data generated outside the context of RCT’s) offers many opportunities for better data on effectiveness in real-world conditions for drug developers and decision-makers.
- However, the incorporation of RWD in a pre-authorisation environment is fraught with ideological, political and procedural problems.

Aim
Conduct a review of different stakeholders’ policies and perspectives on using RWD for early drug development and clinical effectiveness.

2. Methods

- Qualitative methodology selected that combines semi-structured interviews with a literature review.
- 19 stakeholders from 8 different stakeholder groups were interviewed.
- Meanwhile, the review of academic and grey literature yielded 52 documents.
- Transcripts of interviews and data abstracted from review articles were subjected to coding analysis.

3. Results

- Current uses for RWD: drug development, regulatory activities and reimbursement activities.
- Definition of RWD: Consensus regarding the definition of RWD and types of RWD was lacking among stakeholders.
- Advantages vs. disadvantages: RWD has high external validity and can provide valuable data on long-term outcomes. Yet bias, poor data quality (incomplete, missing) and lack of standardisation entice scepticism for its use.
- Political considerations:
  - Governance: ambiguity on policies related to privacy & access
  - Cultural barrier against use in decision-making
  - More stakeholder collaboration needed on evidence needs
- Procedural Considerations:
  - Harmonisation & standardisation: tools, data types
  - Data linkage: combining RWD from different sources and different types
  - Infrastructural needs: ICT network and personnel training needs

4. Conclusion

- RWD is increasingly being used during drug development, registration activities and reimbursement activities. However, consensus on the definition of RWD is still lacking.
- Although RWD has several advantages (e.g. generalisability), disadvantages such as bias and poor quality entice scepticism for its use.
- Increased stakeholder collaboration is needed to address political and procedural considerations regarding implementation of RWD in drug development and relative effectiveness assessment.