A FRAMEWORK TO GUIDE THE USE OF REAL-WORLD EVIDENCE TO SUPPORT EVALUATION OF RELATIVE EFFECTIVENESS OF NEW MEDICINES

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INTRODUCTION

- Payers and health technology assessment agencies are interested in evidence of effectiveness in real-world settings.
- Real-world evidence (RWE) is the evidence derived from the analysis and/or synthesis of real-world data (RWD). RWD is all data collected outside traditional randomised controlled trials (RCTs) on the effects of health interventions (for example benefits, risks, and resource use).
- There are operational and methodological issues in using RWD and RWE to estimate relative effectiveness before a medicine is licensed.
- The objectives of the framework are to guide the use and generation of RWD and RWE, and to help design strategies to provide better information about relative effectiveness of new medicines.
- The framework pulls together original academic research and insights from the whole IMI GetReal (www.imi-getreal.eu) consortium.

METHODS

- In Work Package 1 of GetReal, multiple stakeholders helped to develop the decision-making framework. These included patient organisations, clinicians, academic specialists, clinical trialists, pharmaceutical companies, European regulators, health technology assessment agencies and payers.
- Case study workshops in 5 disease areas examined different analytical methods and study designs using RWD to provide estimates of relative effectiveness, and to find out how useful and acceptable stakeholders thought each option was.
- Stakeholders within the GetReal project tested the framework.

RESULTS

1. Web-based decision-making tool
   - Core of the framework
   - Takes users (normally health technology assessment agencies and pharmaceutical companies) through a set of questions to consider and actions to carry out, to see if there are potential issues in demonstrating relative effectiveness
   - Takes users through the RWE options that might address these issues

2. Review of RWD sources
   - Comprehensive list of sources
   - Gives descriptions and examples of RWD, details of potential usefulness and limitations, and any related GetReal work (such as on social media)
   - Links to key authoritative resources

3. Catalogue of RWE study designs
   - An overview and description of study designs providing RWE, including potential usefulness and limitations
   - Tips on generating RWE, including links to a tool for designing pragmatic trials
   - Highlights authoritative resources, including GetReal publications and deliverables resources

4. Summarises analytical approaches
   - Directs users to GetReal case studies and other authoritative resources (including software and decision-making tools) on including RWE in evidence synthesis
   - Describes methods of predicting outcomes in the real world beyond the available RCT data (either for the population or for the prediction over time)

5. Summarises stakeholder perspectives
   - Summarises the case studies that looked at the views of key decision-makers, such as regulators, pharmaceutical companies, health technology assessment agencies, clinicians and patients
   - Highlights authoritative resources, including GetReal publications and deliverables resources

6. Alternative evidence development pathways
   - Describes the potential issues in demonstrating relative effectiveness using the PICOS (population, intervention, comparator, outcome, study design/setting) framework
   - Describes the evidence development pathways and how they may change with the use of RWE

7. Current policies and perspectives on RWE
   - Links to a review of current policies and perspectives
   - Describes and links to key relevant initiatives, including adaptive pathways

Conclusions

- The framework captures a wide range of stakeholder views on the value and acceptability of different RWD study designs and analyses.
- It will help pharmaceutical R&D to assess how real-world study designs can be integrated in medicine development programmes.
- It supports engagement between pharmaceutical R&D and decision-makers, and will help to improve the scientific and policy agenda in this area.