W19: Estimation and Prediction of Relative Effectiveness Using Real-World Evidence: Case Studies

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Innovative Medicines Initiative (IMI)

- Europe’s largest public-private initiative
- Joint undertaking between European Union and European pharmaceutical industry association EFPIA.

GetReal project
- Understanding how real-world data can contribute to decision-making
  - October 2013 to December 2016 (39 months)
  - 29 partners
  - Total budget: €18 million
    - 50% staff from the 15 participating pharma companies
    - 50% cash contribution from the EU to fund ‘public’ sector
Universities, research organisations, public bodies, non-profit groups
Universitair Medisch Centrum Utrecht, the Netherlands
Academisch Ziekenhuis Groningen, the Netherlands
Zorginstituut Nederland, the Netherlands
European Medicines Agency, UK
European Organisation for Research and Treatment of Cancer, Belgium
Haute Autorité de Santé, France
London School of Hygiene and Tropical Medicine, UK
National Institute for Health and Care Excellence, UK
Panepistimio Ioanninon, Greece
Universität Bern, Switzerland
University of Leicester, UK

Small and medium-sized enterprises (SMEs)
LA Santé Epidemiologie Evaluation et Recherche, France

Patients’ organisations
International Alliance of Patients’ Organizations, UK

EFPIA companies
GlaxoSmithKline Research and Development Ltd, UK
AstraZeneca AB, Sweden
Bayer Pharma AG, Germany
Boehringer Ingelheim International GmbH, Germany
Bristol Myers Squibb EMEA sarl, US
Eli Lilly, UK

F. Hoffmann-La Roche AG, Switzerland
Janssen Pharmaceutica NV, Belgium
Merck KGaA, Germany
Merck Sharp & Dohme Corp., US
Novartis Pharma AG, Switzerland
Novo Nordisk A/S, Denmark
Sanofi-Aventis Research and Development, France
Takeda Development Centre Europe Ltd, UK

• WP1 Frameworks
  • Processes
  • Policies

• WP2 Understanding the efficacy-effectiveness gap
  • Simulation of trials to improve design

• WP3 Overcoming practical barriers to the design of real-world studies

• WP4 Identifying best practice and creating new methods for evidence synthesis and predictive modelling

• 5 Case studies using drugs that had difficulty at regulation and HTA
    • 360 degree reviews
    • Re-designing development pathways to include real-world data
    • Simulation
    • Ascertaining impact on decision makers

• Standardising terminology
• Interviews to understand and the perspectives and policies of different stakeholders
• Designing a framework for decision-making during development
• Numerous methods are available for inclusion of RWE in R+D/regulatory/HTA process/pathway, BUT ...
  – They make various assumptions which my be untestable/subjective
  – Rely on availability of RWE(I)
  – There maybe differing levels of acceptance by different HTA bodies

• Therefore ...
  – Further case studies are required & elicitation of stakeholder views
  – More research on methods for inclusion and adjustment of RWE
    (especially in form of AD)
  – Greater use of NMA & simulation in design of both RCTs & RWE studies
  – Support for initiatives to share RWE IPD or at least relevant summary
    AD, e.g. Conditional covariate distributions, subgroup estimates, etc.

• Your views & experience of using/reviewing use of RWE in R&D/HTA?