Real-Life Data in Drug Development: Perspectives on R&D, market authorisation and HTA (IMI Get Real)

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NICE

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Two problems needing a joint solution

R & D decision
- What combination of possible studies will provide the most valuable information to HTA/payers?
- What is the feasibility of the study options pre-launch and what would be commitments post launch?
- How do options reconcile with the regulatory process?

HTA decision
- Would we predict an improvement in patient outcome or care pathway efficiency over and above current practice in my healthcare?
- Would we accept the uncertainty for a period of time while waiting for studies to complete?
Background and motivation

1. HTA / healthcare decision making require additional information to Regulatory to address the “efficacy – effectiveness” gap

2. Current initiatives on relative/comparative effectiveness research show how real world data can be harnessed post launch

3. There is an opportunity to adapt such techniques pre-launch... but there are practical issues
   – HTA <-> regulatory objectives
   – Ethical and legal obligations
   – Managing cost and operational feasibility

4. Integrating heterogeneous trial and observational data to inform predictive modelling of effectiveness requires new analytical techniques
GetReal: Project Vision

For pharmaceutical R&D and healthcare decision makers to better understand how real-world data and analytical techniques can be used to improve the relevance of knowledge generated during development, e.g., through innovation in clinical trial design.

Lasting impact of the project

To provide a methodological and analytical framework that informs policy and process evolution beyond the project and at an international level; and to provide tools, techniques and training that ensure that the potential of real world data can be exploited in drug development.
Innovative Medicines Initiative:
Joining Forces in the Healthcare Sector
€1,945,135,308

- €711,963,033 Infectious diseases
- €214,136,227 Drug discovery
- €182,980,698 Brain disorders
- €116,880,300 Metabolic disorders
- €116,287,312 Drug safety
- €78,225,417 Stem cells
- €69,739,527 Inflammatory disorders
- €72,710,786 Cancer
- €70,310,746 Data management
- €55,930,958 Biologics
- €49,310,000 Geriatrics
- €37,378,289 Education and training
- €37,966,496 Lung diseases
- €44,722,763 Vaccines
- €30,601,855 Sustainable chemistry
- €18,118,249 Drug kinetics
- €14,910,397 Relative effectiveness
- €14,910,397 IMI funding
- €14,910,397 Corporate contributions

Nat Med 2014;20:5.
Nat Med 2014;20:5.

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Public/SME partners

**Universities, research organisations, public bodies, non-profit groups**
- Universitair Medisch Centrum Utrecht, the Netherlands
- Academisch Ziekenhuis Groningen, the Netherlands
- College voor Zorgverzekeringen, 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 partners

- GlaxoSmithKline Research and Development
- Amgen
- AstraZeneca
- Bayer Pharma
- Boehringer Ingelheim
- Bristol Myers Squibb
- Eli Lilly
- F. Hoffmann-La Roche
- Janssen Pharmaceutica
- Merck KGaA
- Merck Sharp & Dohme Corp.
- Novartis Pharma AG
- Novo Nordisk
- Sanofi-Aventis
- Takeda Development Centre
Structure and topics of GetReal

WP1
- Choice of comparator
- Frameworks of relative effectiveness assessment

WP2
- Drivers of relative effectiveness
- Innovative Development options / study designs

WP3
- III a options / study designs
- Predictive power / residual uncertainty
- Acceptable uncertainty?
- Reg + HTA Process Simulations
- International Reg & HTA policy implications

WP4
- Full Evidence Integration
- Predictive Modelling
- Operational cost / feasibility / solutions
- Ethics & Regulations
WP 2: better understand the gap between efficacy & effectiveness

Clinical trials (efficacy)

Real world (effectiveness)
WP3: overcoming practical barriers in undertaking pre-launch RE research.

“In an increasingly complex world, sometimes old questions require new answers.”
WP4: Promote best practice in evidence synthesis and predictive modelling
WP1: The WP1 case study approach
Development strategy redesign workshops

**Information Sources**
- Publicly available documents (reg, HTA)
- Stakeholder interviews
- Company commentaries & presentations
- Original company source documents

**Workshop 1 Outputs**
- Discussion summary / minutes
- Key scientific questions (sources of bias and uncertainty in REff)
- Alternative development design options

**Workshop 2 Outputs**
- Discussion summary / minutes
- Stakeholder insight & reactions to potential options
- Scenario Summary
- Contribute to decision framework
- Publications

**Summaries**
**Simulations**
Project deliverables and benefits

- Frameworks developed jointly by Regulatory, HTA and Industry experts for use in:
  - Practical Solutions to enable implementation of studies with greater value of information for relative effectiveness assessment
  - Advances in methodology to reliably predict effectiveness from available data
  - Aligning innovation in evidence generation with evolution of regulatory and HTA processes
For more information:

• Visit the GetReal website: imi-getreal.eu