A Regulator’s view on:

Could real world evidence data become the co-driver of regulatory and reimbursement decisions?

Panel discussion for: Can “Real World Evidence Data” advance equity of health care in Europe?

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The views expressed in this presentation are my personal views and may not be understood or quoted as being made on behalf of or reflecting the position of the European Medicines Agency or one of its committees or working parties.
What is real world data?

Real world evidence is defined as data that are collected outside the constraints of conventional randomised clinical trials.
Business Case – RWE in the product life cycle

**Development**
- Launch
- Understanding standard of care and NHD
- Patient recruitment
- Trial design
- Unmet need / disease burden
- PROs

**Growth phase**
- Conditional pricing review
- Adherence
- Budget impact
- Post marketing commitments (safety etc.)
- Utilization / prescribing patterns
- Effectiveness
- Target populations
- Differentiation in sub-populations
- Usage difference
- Effects of switching on outcomes
- Differentiate with or vs. protected galenics

**Mature phase**
- New competition
- New formulation / indication
- Competitive goes generic
- Utilization / prescribing patterns
- Adherence
- Head to head comparative effectiveness
- Effectiveness

**Evidence required**

- **Past**
- **Now**

*Source: IMI GetReal*
Past

Now

Evidence required

Development

Growth phase

Mature phase

RWE integrated into regulatory processes and decisions

Source: IMI GetReal
Use of real-world data in regulatory decision-making

What is the relevant patient population for gene therapy?

What is the risk of bleeding in new users of DOACs compared with warfarin?

What is compliance in the elderly?

What is the incidence and outcome of opportunistic infections with natlizumab?

What are the long term health benefits of a new treatment compared with standard treatment?

Genetic basis of responder/non responder status

What was the impact of the regulatory action following the Article 31 Referral on Combined hormonal contraceptives on prescribing and VTEs?

Patterns of codeine prescribing across Europe and the incidence of death

Infection spread following vaccination

Genetic susceptibility to adverse drug reactions

What is the extent of off label prescribing with SGLT2 inhibitors?
Benefits of access to and use of real world evidence

1. Improving medicines development
   - Enabling innovation
   - Understand the disease and target population
   - Understand treatment outcomes
   - Better evidence supplementing Clinical Trials

2. Post-authorisation
   - Utilisation and prescribing patterns
   - Faster identification and assessment of safety issues
   - Effectiveness data
   - Determining safety and efficacy in high risk groups

3. Overarching benefit
   - Improved EMA and HTA decision making
   - Optimising use of medicines through ongoing monitoring
   - Ability to define the impact of regulatory/HTA decisions

Overarching benefit

HTA

Enabling innovation

Understanding the disease and target population

Understanding treatment outcomes

Better evidence supplementing Clinical Trials

Utilisation and prescribing patterns

Faster identification and assessment of safety issues

Effectiveness data

Determining safety and efficacy in high risk groups

Improved EMA and HTA decision making

Optimising use of medicines through ongoing monitoring

Ability to define the impact of regulatory/HTA decisions

Medicines development

Post-authorisation

Overarching benefit
EMA, will undertake to improve stakeholder collaboration and make better use of registries through:

- mechanisms for regulators and marketing authorisation applicants to systematically consider the need for registries and interact with registry holders;
- sharing and disseminating information on disease registries;
- recommending governance principles and standards for stakeholder interactions;
- making recommendations on core data elements and quality standards acceptable for regulatory and HTA decision-making;
- identifying registry holders' needs for methodological and technical guidance;
- investigating what patient-reported outcomes registries should collect;
- exploring further measures to improve the sustainability of registries.
Thank you