Real World Evidence in Europe

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BEFORE I BEGIN; DISCLAIMERS:

Dual perspective:

Pharmaceutical: I work for Lilly, but this presentation represents my own views and opinions.

NHS healthcare: I also work for IOW NHS Trust as a Non-Executive Director (NED, designate) on the Trust Board.

Views expressed here are my own
• What is RWE, what it can (and cannot) do?

• Compare and contrast perspectives of the healthcare provider and a company

• How Lilly is engaging in RWE
WHAT IS RWE?
WHAT IS RWE?

• **RWD** – is data collected in a non-interventional ‘real-world’ setting (excludes Randomized Controlled Trials, pooled Analysis of RCTs, Meta-analysis of RCTs, cost-effectiveness models)

**Primary sources of RWD**
- Prospective patient Registries
- Prospective observational or longitudinal cohort Studies
- Pragmatic Clinical Trials
- Patient and Caregiver Surveys
- Hybrid Studies (e.g., retrospective observational + survey)

**Secondary sources of RWD:**
- Retrospective databases/ patient registries
- Electronic Medical Records
- Administrative claims records
- personal health records
- genetic and biomarker databanks
- Patient-derived data (via web-based or smart technologies)
THE ‘EVIDENCE EGG’:
RWE REQUIREMENTS ARE ALREADY MANDATORY IN SOME COUNTRIES, BECOMING MORE IMPORTANT ACROSS EU

- **RWE** is essential to maintain access in France, Italy, Netherlands, Belgium, Nordics, and others.

### Stakeholder Need for Real World Evidence

- **Essential**
  - Critical for access and maintenance

- **Important**
  - Beneficial for product assessment and uptake

- **Low**
  - Never demanded or considered

### Availability of Real World Data

- **Low**: Low quality / patchy RWD sources
- **Medium**: Some good sources with intention to develop further
- **High**: Good quality integrated RWD sources

### Future trend?

- **Belgium**, **Germany**, **Netherlands**, **Nordics**
- **Australia**, **Spain**, **Italy**, **Switzerland, Austria**, **Canada**, **UK**
- **Portugal, Greece**
- **Eastern Europe**
- **Japan, Korea**
- **Eastern Europe**
WHAT RWE CAN DO
QUESTIONS RWE MAY BE ABLE TO ANSWER ACROSS LIFECYCLE

? Burden and cost of illness – what is the healthcare need in the patient population?
? Which patient population should be studied in a clinical trial programme?
? What is being used in real life practice, drugs and doses for comparison?
? What endpoints are measured in real life, how to link to surrogate endpoints?
? Post-registration risk benefit profile (safety AND effectiveness)
? Is post authorisation/reimbursement utilisation appropriate,
? What is clinical effectiveness and resource use in real life patients?
? What is the longer term effectiveness data beyond regulatory pivotal trials
? How is the medicine used in complex chronic conditions, with multiple therapeutic options and switches over time?
HEALTHCARE PROVIDER AND PHARMA PERSPECTIVES
LOCAL/REGIONAL HEALTHCARE CHALLENGES

- Large number of RWE data sources
- Limited information access across different care providers
- Poor data quality
- Lack of clarity on patient consent
- Diagnosis code needed for care pathways
- Lack of funding or resources to support projects
PHARMA CHALLENGES

• RWE cannot support changes or extensions to the regulatory marketing authorisation
• Comparative effectiveness is subject to selection bias
• RWE studies may have a slower speed of recruitment and be of less interest to clinical community (competition with RCTs)
• The number of patients in the real world lead to unbalanced arms in studies
• RWE retrospective data sources may lack detailed clinical information
• Peer reviewed publication potential maybe more limited with RWE research
# Comparing Perspectives Summary

## Themes

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<thead>
<tr>
<th>Healthcare System</th>
<th>Pharmaceutical</th>
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<tr>
<td><strong>Terminology</strong></td>
<td>Information / Patient healthcare records</td>
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<tr>
<td><strong>Objectives</strong></td>
<td>Integration of care and improved information across patient pathways</td>
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<td><strong>Resources</strong></td>
<td>Few dedicated resources, significant cost pressures within system</td>
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<td><strong>Perspective</strong></td>
<td>Local data, highly fragmented, little use of data outside care boundaries</td>
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<td><strong>Capability</strong></td>
<td>Lots of data, less evidence. Analytics focus on mandatory performance returns</td>
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<td><strong>Implementation</strong></td>
<td>Evidence shared is usually focused upon safety and quality metrics</td>
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RWE IN LILLY
1) Half studies prospective + retrospective studies to provide **primary sources** of evidence required across EU

2) 91% of **secondary data sources** conducted in OUS were sources not held within Lilly + required locally negotiated agreements

3) A small proportion of secondary data source analyses could be done internally – but only one data source in UK **CPRD**

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**n = 147 studies 2012-2013***
VARIED TYPES OF EXTERNAL RWE PARTNERSHIPS AND AGREEMENTS

1. **Data broker** vendor agreements – for example, work with IMS to identify external data source

2. Work with **approved** research group/vendor to **sub-contract** with local clinical or academic groups, supporting analysis and resource

3. Mutual **research collaboration/partnership** – a shared contract between Lilly and government or academic group on shared research interested, with shared level of resource and commitment

4. **Corporate ‘sponsorship’** – fees paid to be member of steering committee and receive reports on registry, no impact upon the data collected
PARTNERSHIP AND SHARED GOALS?
HOW CAN WE USE RWE TOGETHER TO MEET EU HEALTH SYSTEM NEEDS?

- Core interest is disease and treatment pathways, not medicines
- Extension of evidence beyond RCTs conducted in other countries to your country
- Early Access to Medicine Schemes (EAMS)/ Conditional or adaptive licensing
- Coverage with evidence development to support patient access
- Managed entry or risk sharing schemes to support reimbursement and budget
- Registry data to support guidelines and clinical practice
- Evidence to support reimbursement authorities on which treatments provide value to population and the healthcare system
- Identification of variety or inequity of treatment patterns across different regions
?QUESTIONS?