Making informed decisions about patient registries

DR MARK LARKIN, FOUNDER, VITACCESS
PATIENT LEADERSHIP SUMMIT MARCH 7TH 2015
Agenda

• Introduction to Vitaccess
• Introduction to registries
• Considerations for starting a registry for a genetic disorder
• Useful resources
Introduction to Vitaccess
Introduction to Vitaccess

- Patient centred outcomes (PCO) research and consulting company
- Technical expertise
  - PCOs
  - Clinical
  - Pharmacoeconomics and reimbursement
- Simple and reliable electronic data capture platform for patients
  - Any device, any language, any country
  - Industry-standard data security
  - Flexibility of smartphone, tablet, mobile web, SMS

Source: Vitaccess;
Introduction to registries
What is a patient registry?

• “an organized system that uses observational study methods to collect uniform data (clinical or other) to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure, and that serves a predetermined scientific, clinical, or policy purpose(s)”
Product vs. disease registries

**Product registry**
- All patients in the registry have received a specific product
  - Pharmaceutical/biotech/device
  - Pregnancy registries (exposed population = foetus)
  - Does not allow for comparative analyses

**Disease registry**
- All patients in the registry have been diagnosed with a particular condition
  - Usually includes patients that receive any type of treatment for that disease
  - Allows for comparative analyses

Source: Vitaccess;
Why use a registry to collect Real World Data (RWD)?

- **Reality**
  - Data from clinical trials do not always reflect real-world practice and outcomes

- **Generalisability**
  - Data from clinical trials cannot necessarily be assumed to apply to subpopulations of patients not studied in those clinical trials

- **Applicability**
  - Data from clinical trials do not answer questions of physician practice behaviour and outcomes of that behaviour

- **Availability**
  - There are a limited number of clinical trials relative to the number of decisions that must be made

Source: Vitaccess;
# RWD differ from clinical trial data

<table>
<thead>
<tr>
<th></th>
<th>Clinical trial data</th>
<th>Real world data</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Objective</strong></td>
<td>Does a drug work under ideal circumstances?</td>
<td>What happens under usual circumstances?</td>
</tr>
<tr>
<td><strong>Purpose</strong></td>
<td>Regulatory approval (FDA/EMA)</td>
<td>Understand drug or patients in the real world</td>
</tr>
<tr>
<td><strong>Intervention or treatment</strong></td>
<td>Fixed regimen</td>
<td>Flexible regimens</td>
</tr>
<tr>
<td><strong>Subjects</strong></td>
<td>Stringent inclusion/exclusion (homogeneous)</td>
<td>Any (heterogeneous)</td>
</tr>
<tr>
<td><strong>Compliance</strong></td>
<td>High</td>
<td>Varies</td>
</tr>
<tr>
<td><strong>Outcomes</strong></td>
<td>Clinical endpoints</td>
<td>Adverse events, treatment patterns, resource utilization, adherence</td>
</tr>
</tbody>
</table>

From White, 2008 Real World Data: Moving Beyond Clinical Trials
Considerations for starting a registry for a genetic disorder

Source: Vitaccess;
Why start a registry for a genetic disorder?

• To improve understanding of patients and disease
  • Current treatment patterns
  • Natural history of disease
  • Cost/ burden of illness (patient and caregiver)

• Identify patients and prescribers worldwide

• To improve existing treatment paradigms

• To encourage access to new treatments
  • Help manufacturers understand the “business opportunity”
  • Reduce unknowns for payers when evaluating reimbursement

Source: Vitaccess;
Identify data gaps

• What is already available?
  • Data from registration work
  • Previous compounds?
  • Previous studies?
  • PARENT registry of registries (see later)

• Who are you trying to reach?
  • Patients are an increasingly strong decision maker in today’s care

Source: Vitaccess;
Study design considerations

- How many sites, and patients per site?
  - Size, enrollment timeframe, level of ‘engagement’ – site support

- Budget
  - Sites generally not paid – only reimbursed for time
  - Investigators, scientific advisory boards

- Duration of the registry
  - Time needed to assess outcomes?
  - Plan for evolution? For exit?

- Regions/countries?
  - Differing medical practices and cultures, differing regulations
  - Translations

- Data ownership
  - In general, who pays owns the data

Source: Vitaccess
Can technology be used to help data entry?

• Patient vs. caregiver vs. clinical observer

• If feasible, electronic data capture offers benefits
  • More convenient for patients
  • More intuitive and easier for patients to complete
  • Minimises missing data and illogical/uninterpretable answers
  • Real-time edit checks
  • Improves compliance (alarms, data monitoring)
  • Data integrity (minimises back-filling or forward-filling of data).
  • Privacy and discreetness
  • Avoidance of secondary data-entry errors

Source: Vitaccess
Registry key success factors

• Simple and feasible study design
• Scientifically interesting
• Rapid and simplified site recruitment
• Appropriate site management and monitoring
• Ease of data capture

Source: Vitaccess
Useful resources
## Guidelines for observational research

<table>
<thead>
<tr>
<th>Organisation</th>
<th>Guideline</th>
<th>Document Link</th>
</tr>
</thead>
<tbody>
<tr>
<td>grace</td>
<td>Good Research for Comparative Effectiveness: Guidelines to enhance the quality of observational comparative effectiveness (CER), and to facilitate its use for decision making about therapeutic alternatives</td>
<td>GRACE Principles</td>
</tr>
</tbody>
</table>


| International Society for Pharmacoeconomics and Outcomes Research | Data Privacy, Medical Record Confidentiality, and Research in the Interest of Public Health | [http://www.pharmacoepi.org/resources/privacy.cfm](http://www.pharmacoepi.org/resources/privacy.cfm) |

PARENT Framework (www.patientregistries.eu)

- Knowledge (policy documents, best practices)
- Registry of registries
  - Structured data source discovery based on metadata
  - Process support of data request handling and data exchange
  - Registry assessment tool
- Reusable IT components, models, processes for registry creation and improvement, data querying and retrieval
- Centralised services for registry holders (IT, support, counselling)

Source: Vitaccess
Thank you