How Real World Data can support Clinical Drug Development

Alison Bourke, Scientific Director, IMS Health

March 2016
Real World Data in Clinical Drug Development

• What is RWD
• Uses in development
• Examples
Real-World Data (RWD)

RWD is PATIENT data

- Pharma data (observational)
- Lab/Biomarkers data
- Mortality data
- Social media data
- Pharmacy data
- Hospital data
- Claims data
- Disease registries
- Electronic medical and health records
- Consumer data
Real World Data in Clinical Drug Development

- What is RWD
- Uses in development
- Examples
Painting the Background

• Epidemiology
• Burden of disease
• Highlighting unmet needs

Blocking the colour

• Protocol optimisation

Adding detail

• Patient recruitment

Highlights

• Enriched studies

Plan

Build

Run

Enhance

Acknowledgement: Artist Cara Lockhart Smith: Fly-By-Night
Benefits & Issues

Benefits

• **Targeted** clinical trial program
• **Accuracy** of trial planning and data collected
• **Speed** of recruitment and data collection
• **Cost savings**

Issues

• Governance
• Interoperability of clinical systems
• Linking while preserving confidentiality
• Quality of data
• Analytics
• Patient consent models
Issues

Governance
  • Access model & permissions

Costs
  • Data fee

Interoperability of clinical systems
  • Multiple systems
  • How to put data together - common data models

Linking while preserving confidentiality
  • Trusted third parties
  • Inherent identifiability

Quality of data

Analytics
  • Costs
  • Expertise

Patient consent models
Real World Data in Clinical Drug Development

• What is RWD
• Uses in development
• Examples
Epi Example - Gestational Hypertension and Preeclampsia in Living Kidney Donors

BACKGROUND
What is the rate of hypertension & pre-eclampsia in living kidney donors?

METHODS
• Retrospective cohort study of living kidney donors - 85 women matched with 510 healthy non-donors from the general population
• Kidney donations between 1992 and 2009 in Ontario, Canada, with follow-up through linked health care databases until March 2013
• Primary outcome - a hospital diagnosis of gestational hypertension or preeclampsia

RESULTS
• Gestational hypertension/preeclampsia > among living kidney donors vs non-donors
• Odds ratio for donors, 2.4 (95% confidence interval, 1.2 to 5.0; P=0.01)
• No significant differences in rates of preterm birth (8% and 7%, respectively) or low birth weight (6% and 4%, respectively)

CONCLUSIONS
Gestational hypertension or preeclampsia was more likely to be diagnosed in kidney donors than in matched non-donors with similar indicators of baseline health.
Optimising Protocol Design

Conducting Feasibilities in Clinical Trials: An Investment to Ensure a Good Study

- Standard care (e.g., type of drugs, dosage)
- Actual study population vs the patient population treated or seen by the potential investigator
- Readiness & acceptance of therapy
- Anticipated subjects per week/month

“Conducting clinical trial feasibility is both an art and science ..... The challenge for clinical researchers is to ensure the highest level of quality while doing it. In a true sense, feasibility is an investment to ensure a good study.”

V Rajadhyaksha  Perspectives in Clinical Research 2010 Jul-Sep; 1(3): 106–109
Optimising Protocol Design Example.....or not

• Many organisations offer to use RWD for protocol optimisation

• Little scientific literature (apart from EHR4CR) to document this

• Ideal - Study where protocol is modelled and optimised, and the study carried out with several iterations to see if the optimisation has worked
To assess the symptom burden of AF in newly diagnosed patients identified within one week of symptom recording.

- Patients identified in THIN within 1 week of diagnosis
- 82% GP response rate and 50% patient response rate to questionnaires forwarded
- 516 case – control pairs

**Patient recruitment example - AFLOAT**

**Atrial Fibrillation Longitudinal Outcomes Assessment Study**

**To assess the symptom burden of AF in newly diagnosed patients identified within one week of symptom recording**

- Patients identified in THIN within 1 week of diagnosis
- 82% GP response rate and 50% patient response rate to questionnaires forwarded
- 516 case – control pairs
Enriched Example - Serevent nationwide surveillance study: comparison of salmeterol with salbutamol in asthmatic patients

Objective:
Compare safety of salmeterol & salbutamol in asthma

Method:
• Design-Double blind, randomised clinical trial in parallel groups over 16 weeks in the UK
• 25,180 patients with asthma requiring regular bronchodilators

Conclusion:
• The largest randomised, double blind clinical trial ever conducted in the United Kingdom up to that time
• Control of asthma better in patients allocated to salmeterol
• EMRs from a subset of patients who were already in the VAMP Research Bank (became GPRD and then CPRD) – were used to pre-populate e-CRFs
• Castle, Fuller, Hall, Palmer, Glaxo BMJ 1993;306:1034-7
IMS Research Platform in Respiratory

Background
• Clinical trials in asthma and COPD typically exclude up to 95% of the patient population
• Build sustainable RWE (Real World Evidence) research platform capable of providing insights on patient population that are not captured in clinical trials

Solution
• Inform CRF design by analysing capture rates of key variables in historical EMR data
• Assess recruitment potential by applying inclusion & exclusion criteria on historical EMR data
• Identifying high potential recruitment sites
• Use enriched RWD Studies to bring benefits of both prospective and retrospective data collection methods
Vision for the Future: The RWE Ecosystem

IMS Health Real-World Evidence Solutions

Drug utilization studies

Economic models

Trial optimization

Marketing effectiveness

Claims

LRx data

Hospitals

Social media

Survey

EMR

Enriched datasets

ePRO

pRCTs

EMR+ eCRF

Registrars

Economic models

HEOR/ Safety

R&D

Commercial
Any questions?

Thank you

abourke@uk.imshealth