CDDF MULTI-STAKEHOLDER WORKSHOP

USE OF REAL WORLD DATA TO OPTIMISE ONCOLOGY DRUG DEVELOPMENT AND ACCESS

6-7 July 2016
London, United Kingdom

CDDF Workshop on Real World Data

Use of Real World Data to Optimise Oncology Drug Development and Access

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6th & 7th July 2016

CDDF
Cancer Drug Development Forum

Scientific/program committee

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Why observational data/RWD?

- Oncology drug development is difficult
  - randomised controlled trials take a long time
  - RCT conducted in a selected patient population
  - RCT are expensive
  - RCT often too small to study predictive (bio)markers
  - drug efficacy unfortunately usually marginal
  - MA often based on only one pivotal trial
- At the time of licensing there may be limited knowledge about the true benefit/risk balance
- With accelerated drug development that problem will not decrease

Observational data

- Sources of data
  - data quality
- Better estimates of efficacy and safety with use in the general patient population
  - sample size
  - bias
- Study predictive biomarkers
  - sample size
  - bias
- Handling of (unmeasured) confounding
  - confounding by indication

Program overview

Session 1 - Sources of Real World Data in oncology

Session 2 - Use of RWD to support efficacy and/or safety assessments in label expansions

Session 3 - Real world oncology clinical outcomes

Session 4 - Advances in methodology for observational research

Session 5 - Can RWD accelerate patient access to medicines?

Highlights/discussion

- Much going on in Europe
- Terminology
- Challenges of data access and harmonisation
- Role of (health)registries
- Opportunities – new tools/new end points?
- US *vs* Europe – sharing of data
- Generalisability of RCT results
- Advances in integrated data platforms
- Data capture

Highlights/discussion cont

- Methodological research - association *vs* causality
- Short vs long-term follow-up
- Practice *vs* research
- The patient perspective
- Addressing regulators' concerns
- Simplicity *vs* complexity
- Validation of biomarkers
- Excisting initiatives: IMI GETREAL MAPPS/AP/PAES/PASS

How can we utilize RWD to better assess benefit/risk?

Continue exchange of ideas and knowledge