Update on KCE Trials and available sponsor capacity

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KCE Trials Team

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KCE, Belgian Health Care Knowledge Centre

www.kce.fgov.be

• Semi-governmental institution

• Operational 2004

• 50 researchers
  • medicine
  • economics
  • statistics, sociology, law

• Studies (n>250)
  • Health technology assessment (HTA)
  • Good clinical practice
  • Health services research
Need for non-commercial trials

- Important research questions of interest to society that will never be answered by industry (no commercial interest)

- Examples
  - Pragmatic comparative effectiveness
  - Areas not owned by industry (surgical techniques, life style, diet, psychotherapy, ...)
  - Drugs in paediatrics and orphan diseases
  - Medical devices including diagnostics
  - Repurposing of older drugs, including early clinical development
The healthcare payers

- Aim to maximise health within the available budget
- HTA desktop research: often no answer, comparative trials missing
- Selecting and funding clinical trials should be part of the R&D of healthcare payers
- The trials should answer questions of relevance for the healthcare payer
Comparative Effectiveness

Comparator

- best active
- active
- placebo
- none

pragmatic practice-oriented trial

Endpoints
- Quality of Life (EQ-5D)
- Survival

Endpoints
- narrow (efficacy)
- broad (effectiveness)

Study population
“In addition to patient benefit, publicly funded trials can provide a positive return on investment”

Publicly funded practice-oriented clinical trials: of importance for healthcare payers.
Impact of KCE Report June 2015

- Decision October 2015
  - KCE to set up a programme of practice-oriented clinical trials: “KCE Trials”

- Budget
  - 2016 and 2017: €5 million per year
  - From 2018 onwards: €10 million per year

- Return on investment is expected

- Challenge 2016: first patient in a trial
KCE Trials programme

- Immediately useful
  - to patients, clinical practice (effectiveness)
  - to policy or decision makers (efficiency)
- Extension of HTA programme, as at NIHR
- National and international trials
- Commissioned and investigator-led
- KCE is the funder
- Non-commercial sponsor
- Need for clinical trial units (CTU)
Key success factors for publicly funded trials
Key success factors for publicly funded trials

**SELECTION CRITERIA**
- Panels
- Trials Board

**IMPLEMENT RESULTS**
- Prioritisation Group
- KCE Board
- CTU

**PROFESSIONAL CONDUCT**
- Need for the evidence
- Value for money/ROI
- Scientific rigour

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Key success factors for publicly funded trials

SELECTION CRITERIA
- Need for the evidence
- Value for money/ROI
- Scientific rigour

WOULD YOU LIKE TO JOIN A PANEL?
trials@kce.fgov.be

IMPLEMENT RESULTS

PROFESSIONAL CONDUCT

Panels
Trials Board
CTU
KCE Board
Prioritisation Group

Would you like to join a panel?
trials@kce.fgov.be
Sponsor capacity - CTU

- Industry: dedicated department
- Hospital clinical trial unit:
  - Currently mainly legal, budget, ethics
  - Also needed for data management, monitoring, vigilance etc.
  - Out of scope of hospital accreditation
- Two day visits to all 7 university hospitals
  - Period May – September 2016
  - Including clinical departments conducting trials
  - Win-win situation
## Sponsor capacity - results

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Sponsor capacity - conclusions

- Few multicentre RCTs
- Expertise scattered, not shared
  - No central management of sponsored trials, procedures, quality management, recruitment, vigilance, vendors etc.
- Trial data management
  - Limited eCRF expertise
  - Study data in excel, not in a database
- KCE support (IT tools, training,...)
Status KCE Trials

- Commissioned workstream 2016
  - 165 Topic suggestions
    - proposed by clinicians, patients, payers etc.
  - 11 Clinical questions published
    - GP topics to high cost specialized care
  - Two step review process ongoing

- International collaboration
  - 3 trials funded by ZonMw (Netherlands)
Frequently asked questions I

- Why a pragmatic trial approach?
  - Most informative for payers
- Why non-commercial sponsors only?
  - Regulatory exemptions
- What about industry involvement?
  - Free product is possible, if no strings attached
- Why multicenter trials only?
  - Speed of recruitment
  - Broad support for implementation
Frequently asked questions II

- When are international trials indicated?
  - Speed of recruitment
  - Co-funding may be needed
- Why strict data management, data access?
  - Solid basis for public health decision making
- Why detailed budget with all activities specified?
  - Fairness, reassurance all activities are planned
  - Microscopic monitoring
- Why involve patients from the start?
  - Input on acceptance, feasibility, endpoints
Status – What is next

- Investigator-led workstream 2017
  - Focus on return on investment
  - Announcement Q1 2017
  - Any questions? trials@kce.fgov.be