

# Publicly funded Practice-oriented Clinical Trials

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# Background

- Important research questions of interest to society that will never be answered by industry (no commercial interest).
- Think about the following clinical trials
  - Pragmatic comparative effectiveness
  - Repurposing of older drugs
  - Drugs in pediatrics and orphan diseases
  - Medical devices including diagnostics
  - Areas not owned by industry (surgical techniques, life style, psychotherapy, population screening, ...)



	Commercial clinical trials	Practice-oriented non-commercial clinical trials	Other clinical trials
Funding	Company	Healthcare deptm. (+university, charity)	Scientific research (+university, charity, industry)
Aim	For profit, development cycle	Optimize practice, comparative effectiveness	Create knowledge; proof of concept, translational
Interventions	Medicines, medical devices	+ surgery, lifestyle, psychotherapy, screening,...	+ surgery, lifestyle, psychotherapy, screening,...
International	Confirmatory (phase 2b/3)	If appropriate	Rarely
Risk level	++/+++	+/++	++/+++

# Is this worth its money?

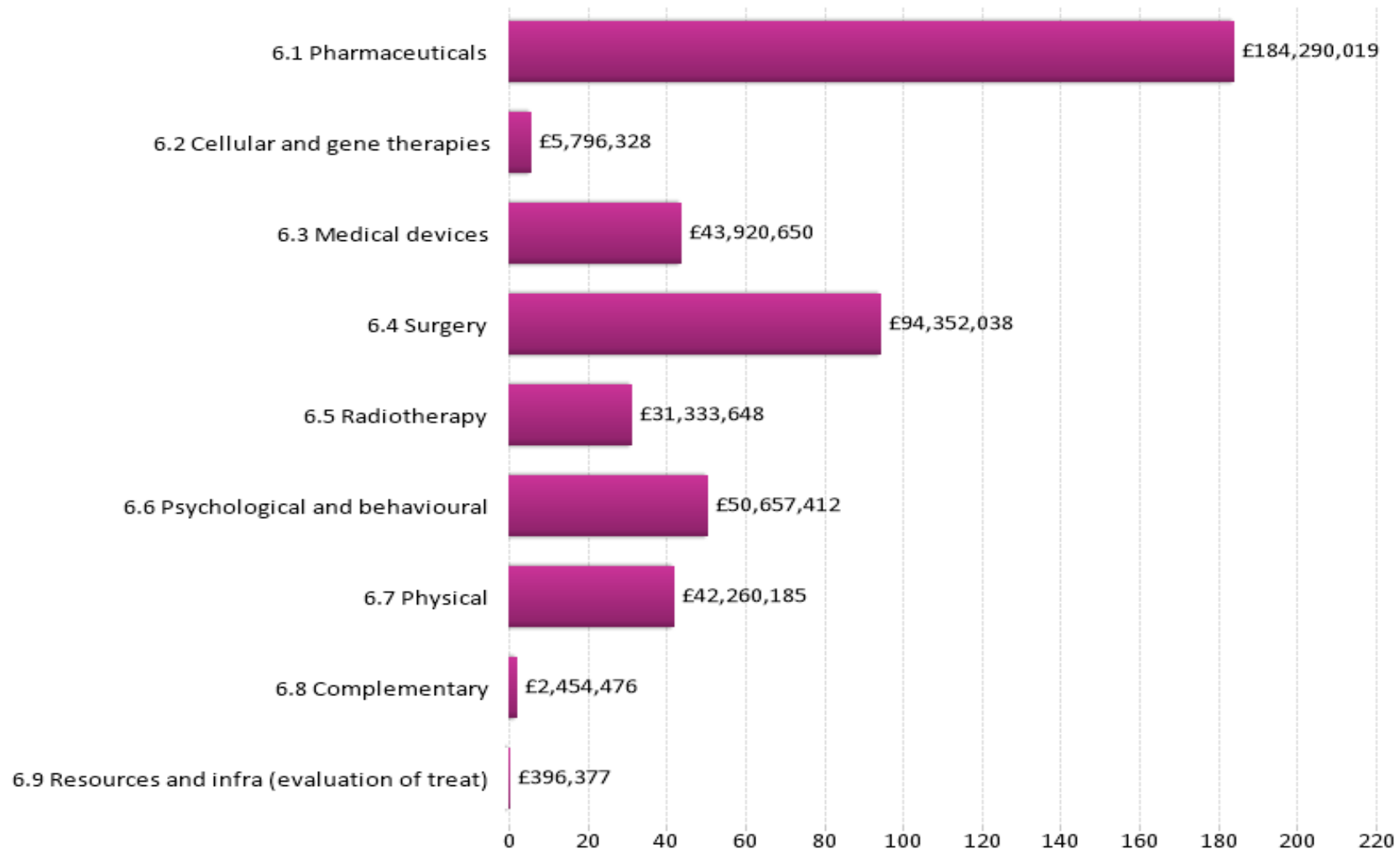
## Optimistic (biased) literature

- Individual cases (e.g. WHI, ALLHAT, ...)
  - High cost, long duration, huge benefits
- Trial program evaluations
  - YES: US, UK, Australia, Sweden, The Netherlands



# Publicly funded clinical trials in the UK, 2014

## Research funding by HRCS Research Activity Code Group 6



# Hurdles and benefits

## Financing

Trial participation

Competing trials

## Infrastructure/network

Free-rider

Time

Access comparator

## Health outcomes

Survival, QoL, ...

## Efficiency

Self supporting

Attracting industry

Stimulating EBM

# ECRIN: European Clinical Research Infrastructure Network

## ➤ Information and consultancy during the preparation of the trial

- Information on regulatory and ethical requirements
- Information on sites and participant recruitment
- Information on clinical trials units
- Information on insurance
- Information on cost and funding opportunities
- Information on contracting
- Adaptation to local context

*Full protocol*



Scientific evaluation

Logistical assessment

Contract with sponsor

## ➤ Services during the conduct of the trial

- Interaction with competent authorities and ethics committees
- Support with insurance contracting
- Adverse event reporting
- Monitoring
- Data management
- Investigational medicinal product management
- etc.

# Success factors

- **Topic selection**
  - **Pragmatic, practice-oriented, practice-changing**
    - Programme management team + independent experts
    - Top down and bottom up proposals
- **Infrastructure and network**
  - Local (to be developed)
  - International (ECRIN)
- **Implementation**
  - Publication
  - Implementation projects



# Policy recommendations (national)

- **Programme management team**
  - Topic selection
  - Standards and QA
  - Services: outsourcing, logistics, insurance, collaboration (local + ECRIN)
  - Payments, monitor progress
  - Publication and implementation.
- **Sufficient financing**
  - Health care budget
  - In part based on existing industry taxes
  - Management, network (~UK, Germany,...), trials

# Policy recommendations (international)

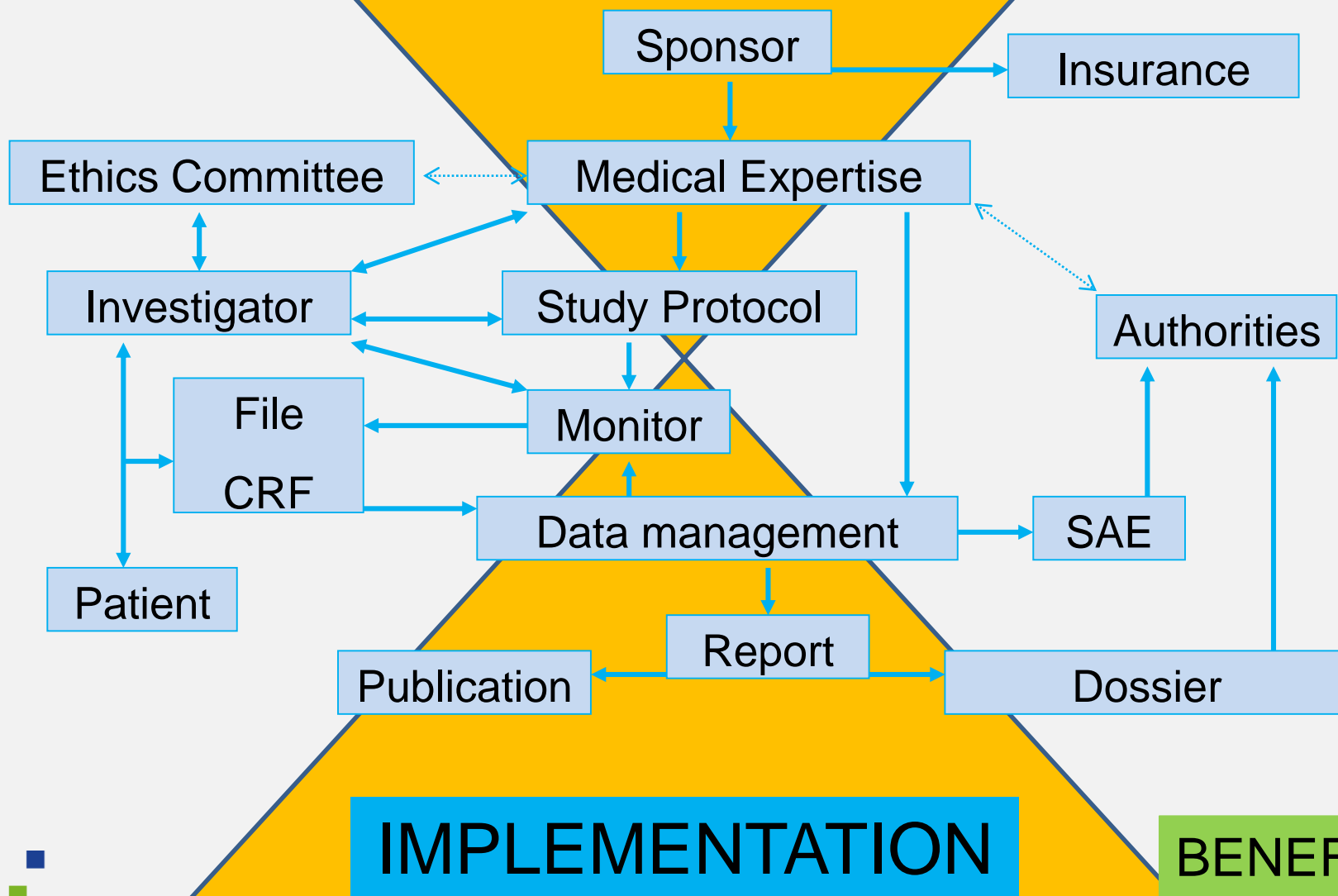
- **Database of planned publicly funded trials**
- **Uniform, coded, electronic patient records**
- **Trial data exchange standards**
- **EU**
  - **Balanced funding of PPPs versus publicly funded practice-oriented clinical trials**
- **EMA**
  - **More pragmatic trials**
  - **Review by independent researchers**
  - **Optimal treatment as a direct comparator**



# SELECTION

Top down + bottom up  
Immediate impact

Systematic search  
International or not



COST

BENEFIT

# Colophon

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