WG 9: Strategies for funding and maintaining a paediatric research network

Mark Turner, Kalle Hoppu, Tim Lee, Stephen Greene, Carlo Giaquinto, Nicola Ruperto

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Aims

- To compare the network mediated paediatric research activity across Europe
- To develop a business case for paediatric research networks.
  - comparing resource inputs versus outcomes with a view to encourage governments to spend more on research infrastructure

Process

- Initial discussions with WG members
- Survey conducted by NIHR CRN: Children
- Results available this week

Questionnaire

- Questionnaires sent out to 21 networks
- Responses received from 16 networks to date
- Some responses require further discussion
- Work pending..

Summary of Responses

- Established between 1989 to 2010
- Age ranges of study participants
  - Preterm – 1 network (GNN)
  - Preterm to either up to/including 18yrs- 14 networks
  - Preterm to 21yrs- 1 network (Newcastle CCLG)
- Speciality
  - Multi speciality- 5 Networks
  - Disease specific- 9 Networks*
  - (*Vaccinology, HIV and Infectious Diseases, Oncology x 2, Hemato-oncology, Attention Deficit Disorder (ADHD), Neonatology , Cystic fibrosis (adults and children), Rheumatology & immunology)

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Completed trials

- based on 13 returns (unavailable for 3 networks)

number of completed trials	number of networks
0 to 10	5
11 to 50	6
50 to 100	0
100+	2

number of completed commercial trials	number of networks
0 to 10	9
11 to 50	2
50 to 100	0
100+	2

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Ongoing trials

Number of ongoing trials	Number of networks
0 to 10	5
11 to 50	5
50 to 100	1
100+	2

Number of ongoing commercial trials	Number of networks
0 to 10	6
11 to 50	5
50 to 100	0
100+	2

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

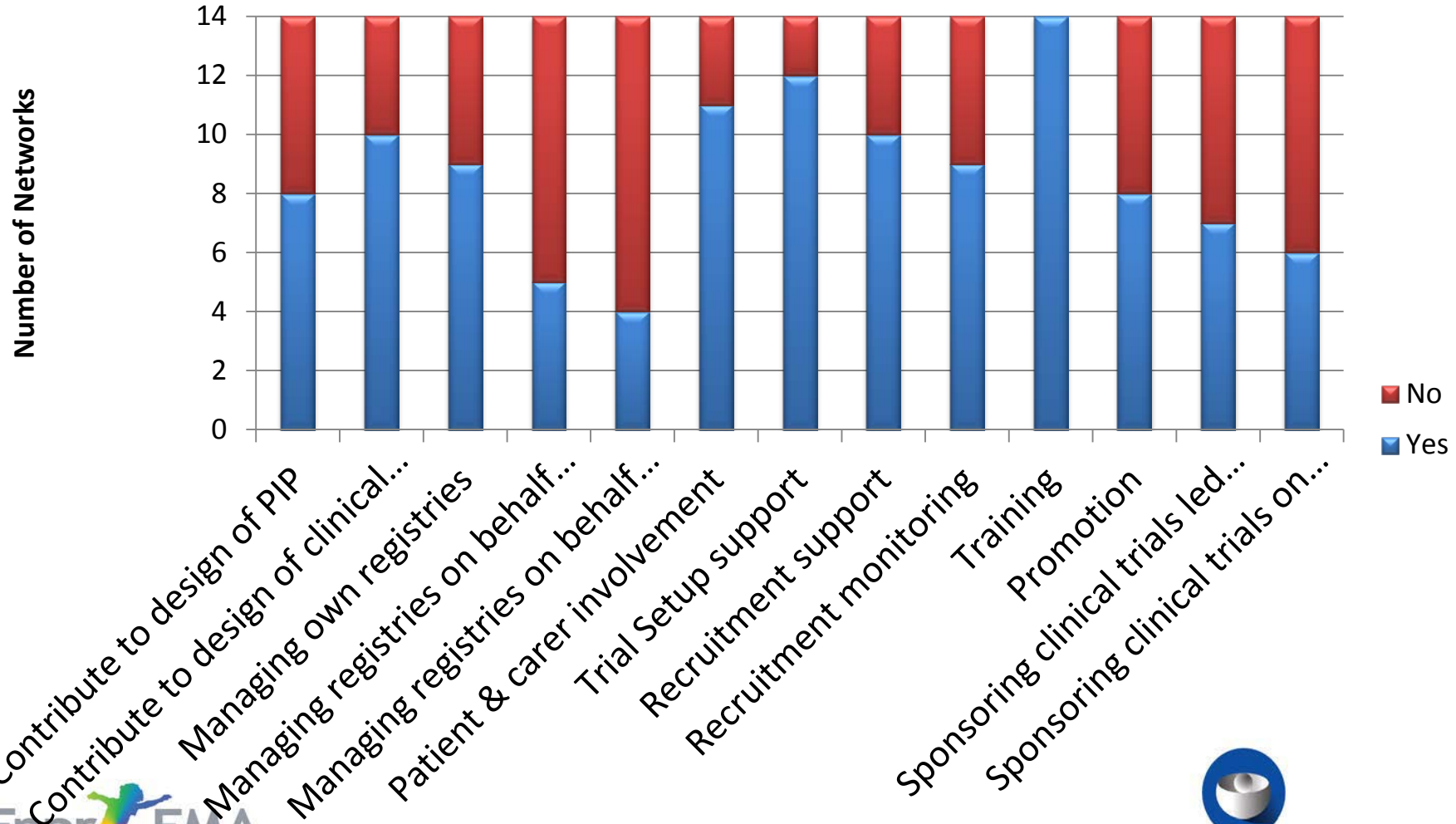
Total Recruitment to date:

Total number of participants recruited	number of networks
0 to 500	1
501 to 1000	2
1001 to 5000	1
5001 to 10000	5
10,000+	2

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

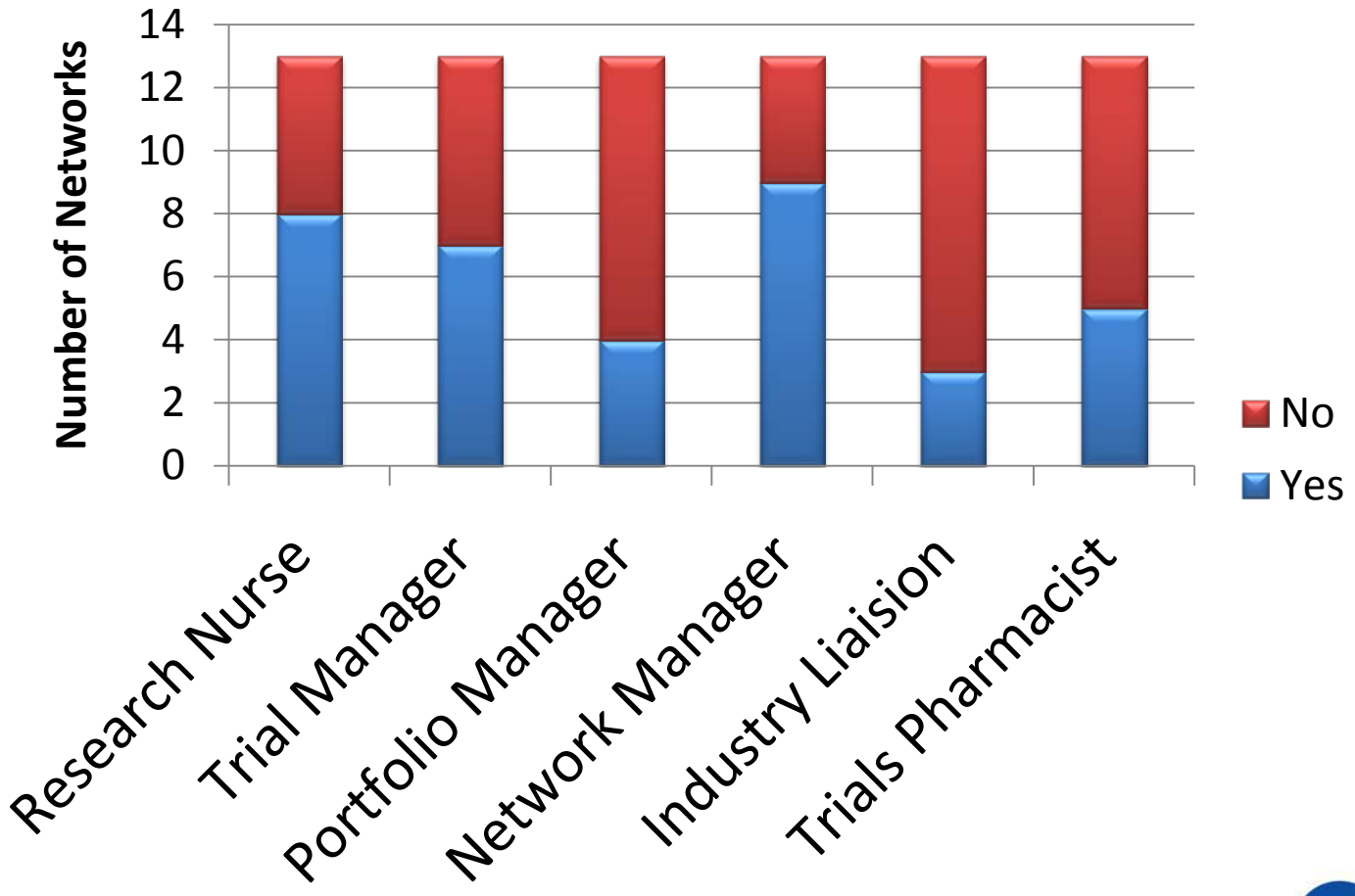
Main Activities



EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Funded staff



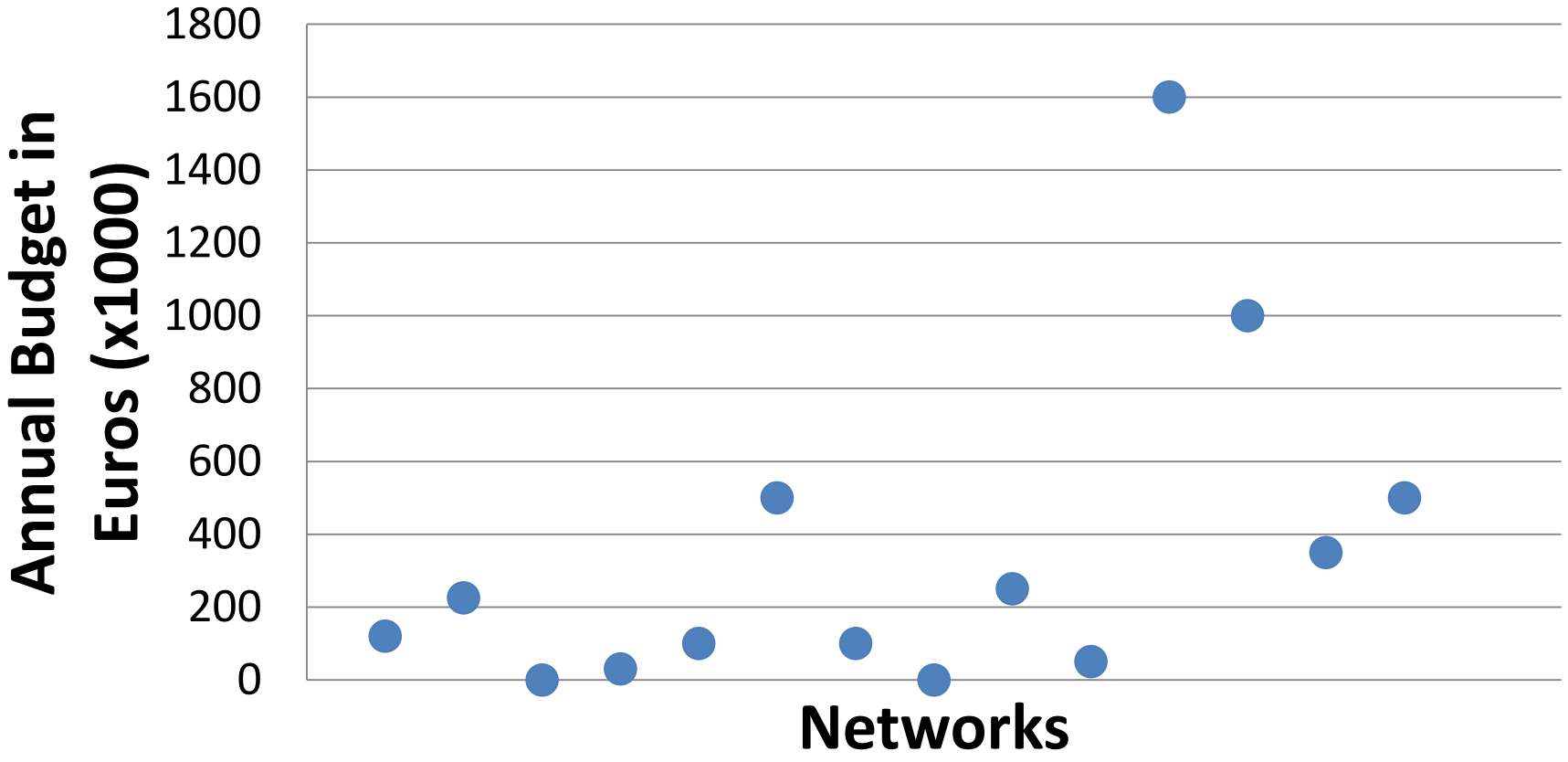
EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Organisation

- Number of collaborating centres
  - <10 centres – 2 Networks
  - 11 to 100 centres – 11 Networks
  - >500 centres – 2 Networks
- Annual budgets
  - From no funding to > 1 million Euros

Scatter chart of Annual Budgets



EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Sources

- Institutions
- National patient organizations
- Contribution of participating sites
- International conferences
- Industry
- EU grants for specific research projects
- University Hospitals
- Annual dues from Member institutions
- Charities
- Hospital Charity
- Government

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Limitations

- Missing data.
  - “difficult to provide precise information on institutional trials (and corresponding number of inclusions) within the network as we do not have dedicated coordination for this task and data will not be precise enough”

Limitations

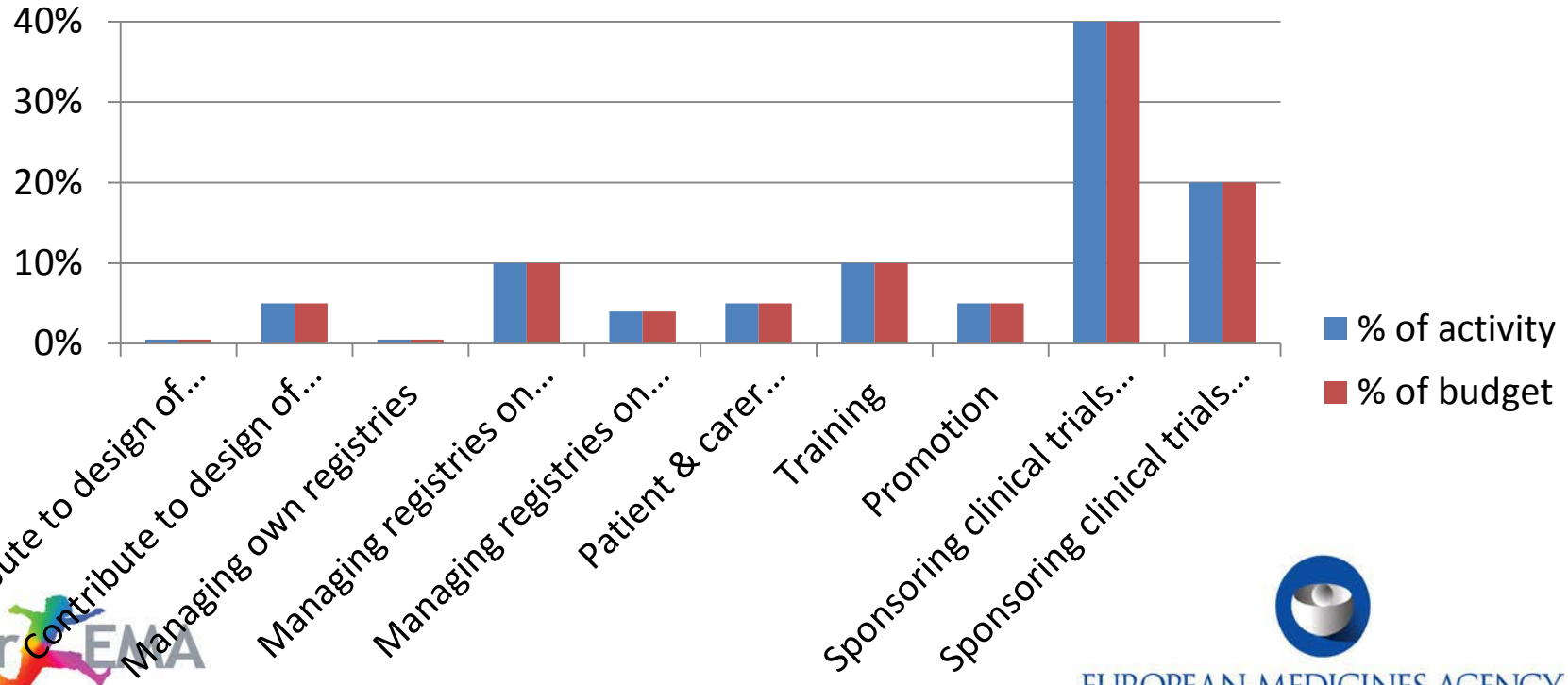
- Differentiating between activity done within the network to organise the network in comparison to trial support.
  - "Each centre has own research nurses. Research activities also supported by variable number of centre-specific research fellows and play therapists"

Limitations

- Lack of resources.
  - "large majority of organisational activity is done on a voluntary basis"
  - "supporting the activities of clinical trials is one of several functions-no staff funded by the network. Network activities are built upon the staff employed by the participating sites."

Case Study 1

- Disease Specific
- Annual Budget- 1,000,000 Euros (Industries, Charities and Public grants)
- 15 completed trials; 720 participants

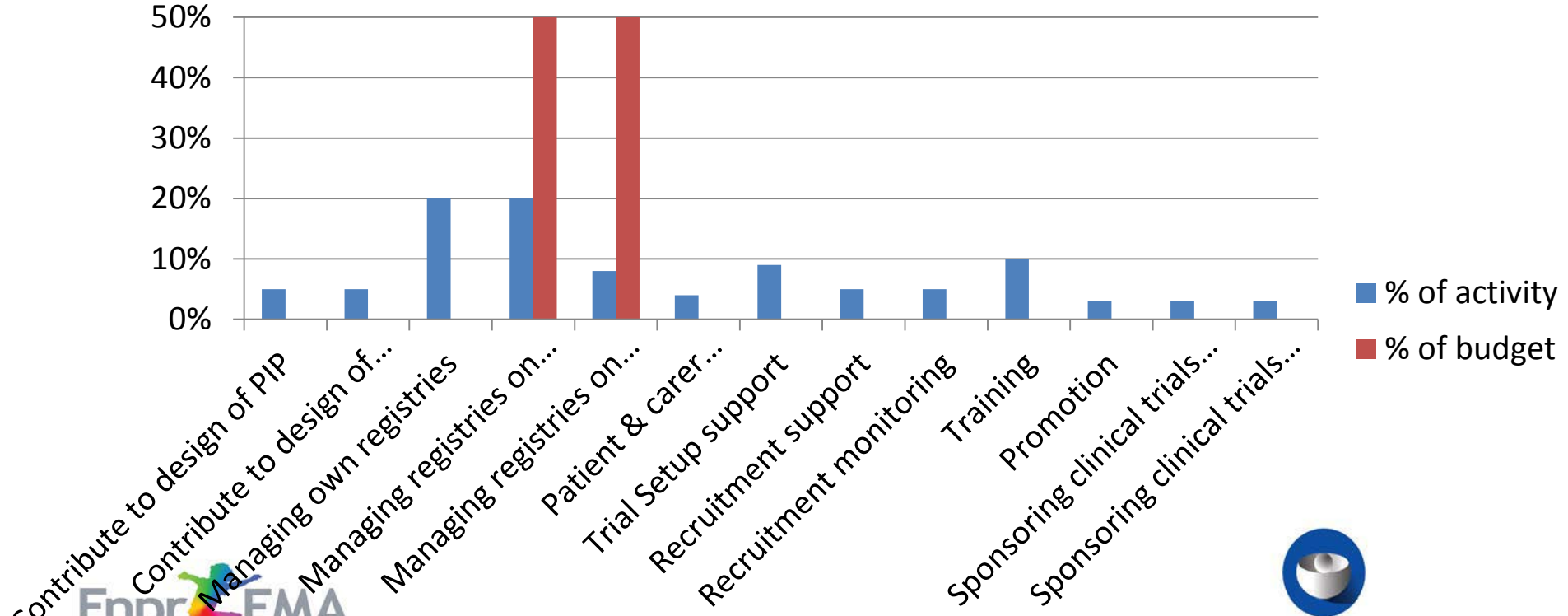


European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Case Study 2

- Disease Specific
- Annual Budget – 350,000 + Euros (Industry and public grants)
- 12 completed trials; 31,000 participants

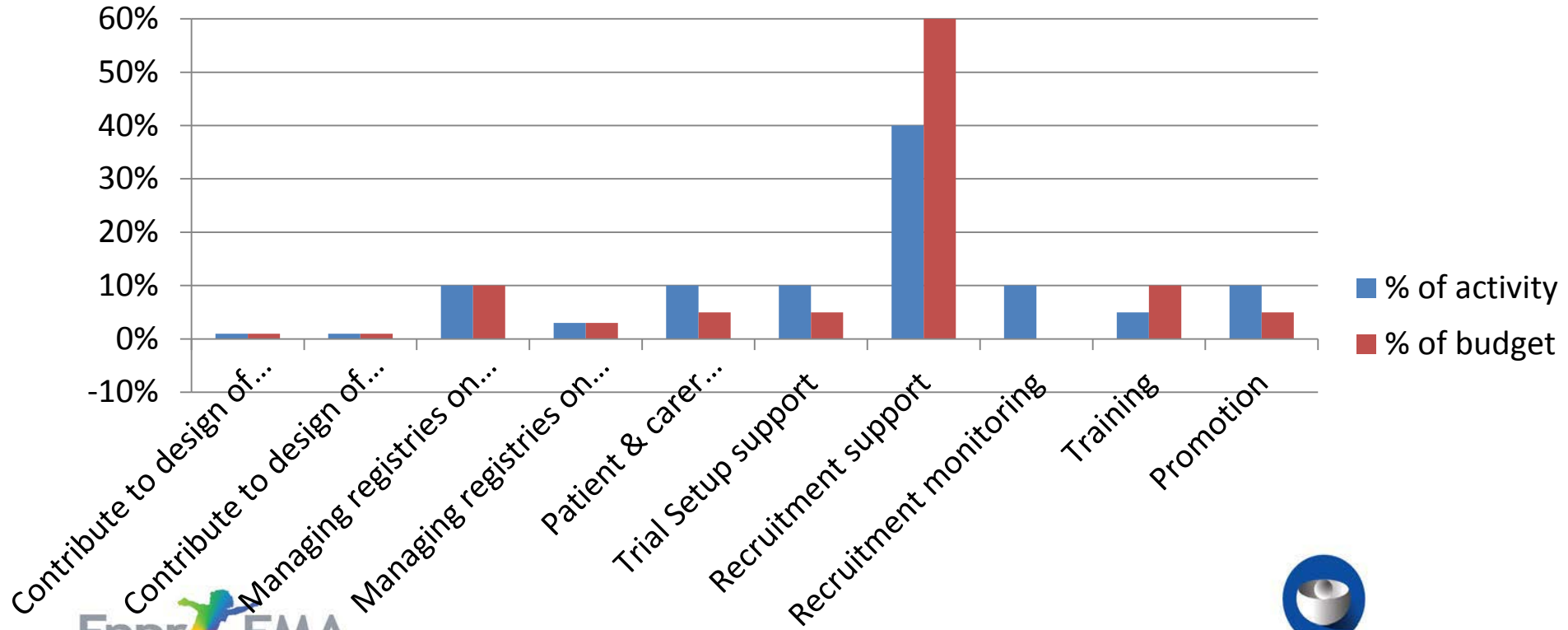


EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Case Study 3

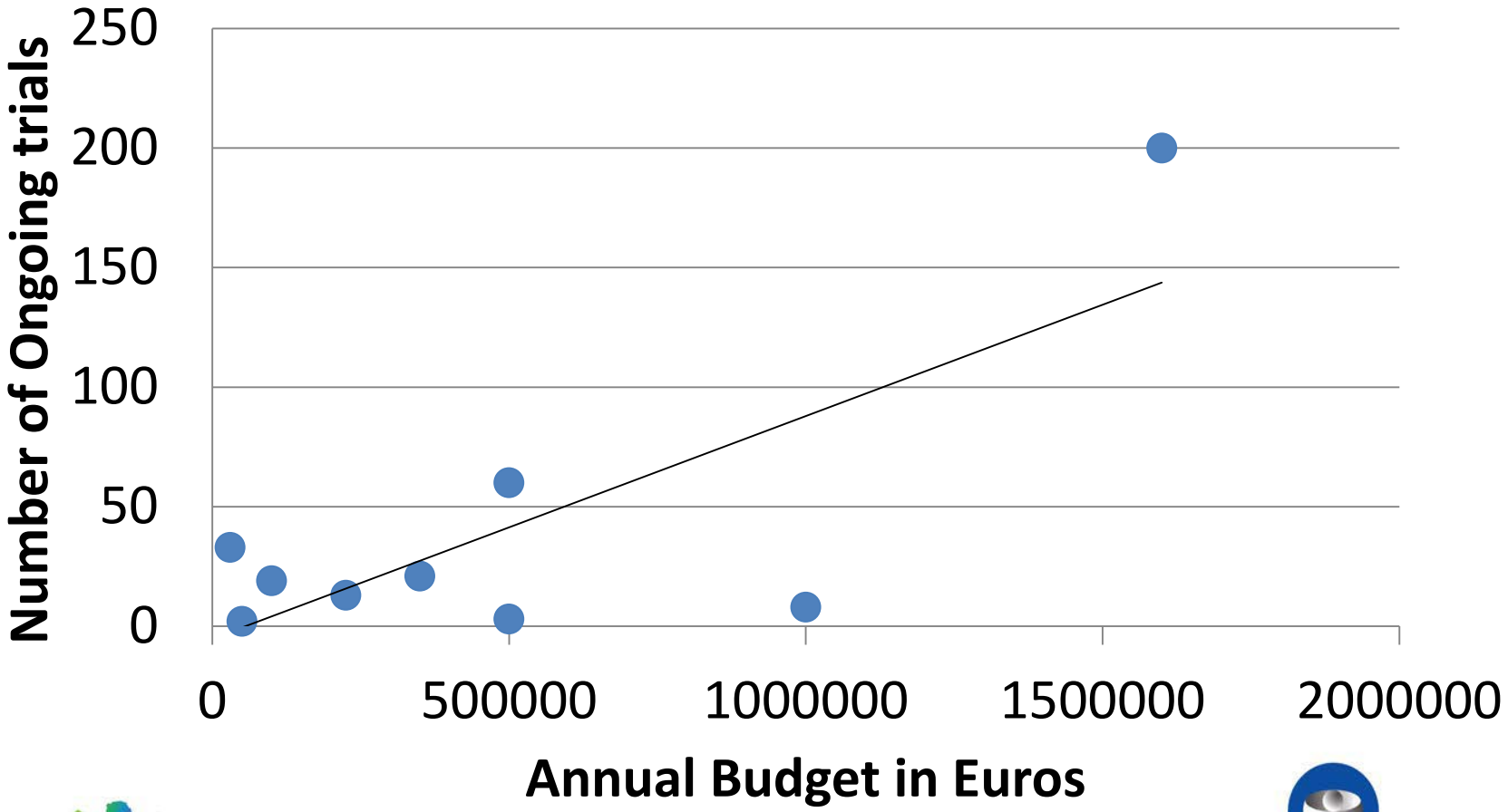
- Multi Speciality
- Annual Budget - 500,000 Euros (from Government)
- 47 completed trials; 6600 participants



EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Trials: Budget



EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

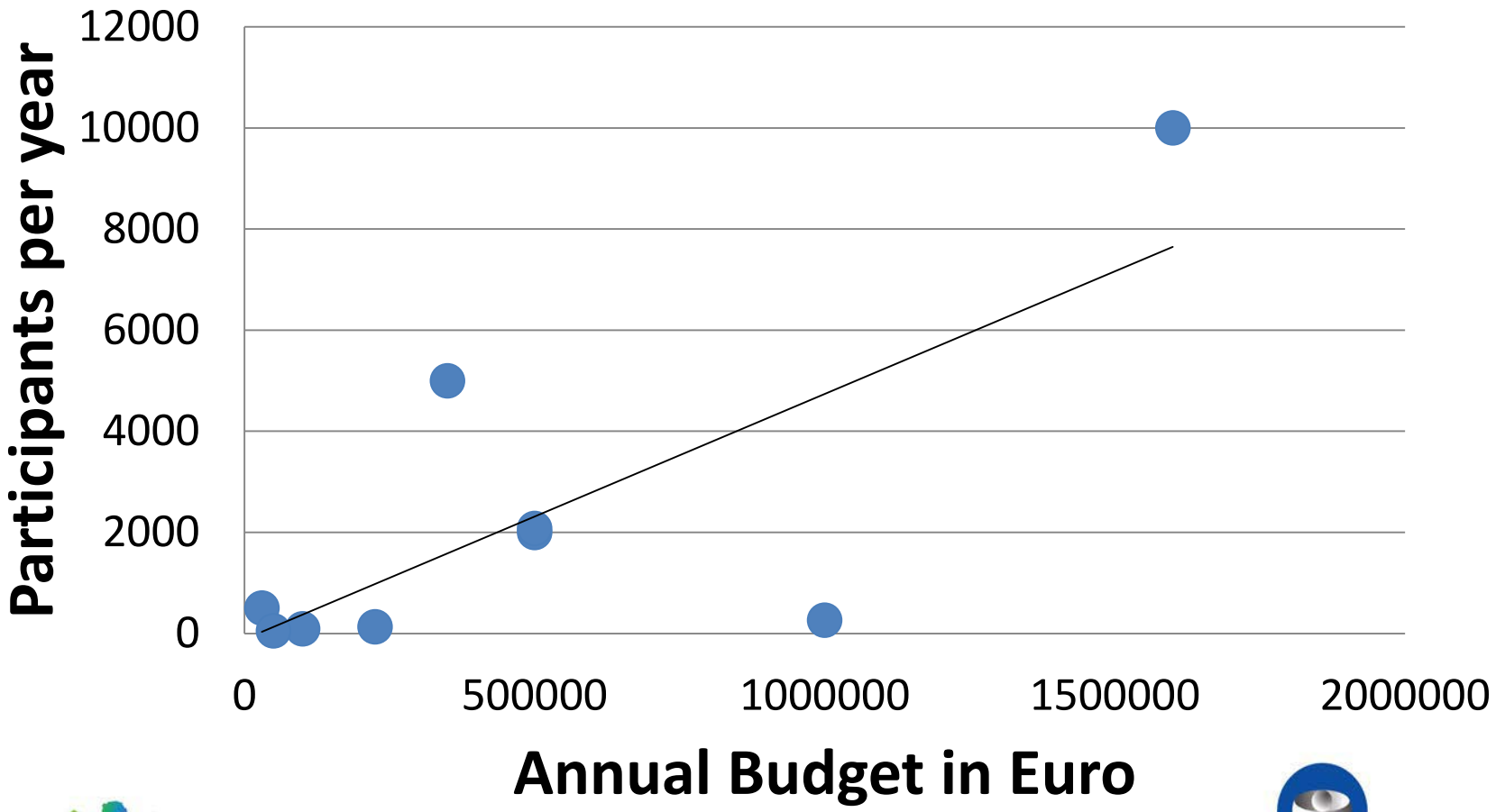
Trials to budget ratio

- Does not include network set-up cost
- Once network is established:
  - Disease specific- ranging from
    1 trial costs on average €50,000
    (median: €17,000; range €900 to €170,000)
  - Multi speciality
    1 trial costs on average €8000

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

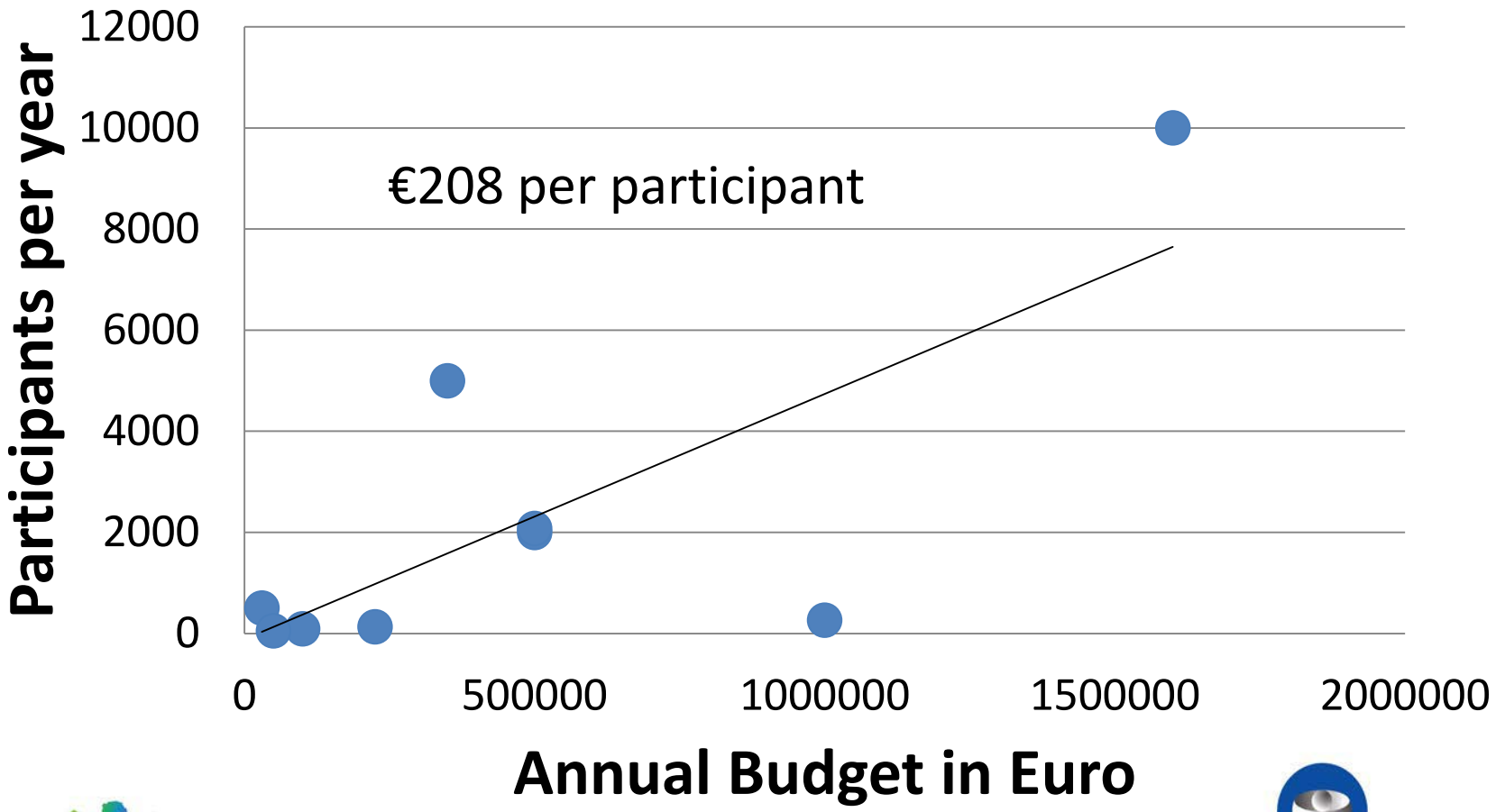
Participants to Budget ratio



EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

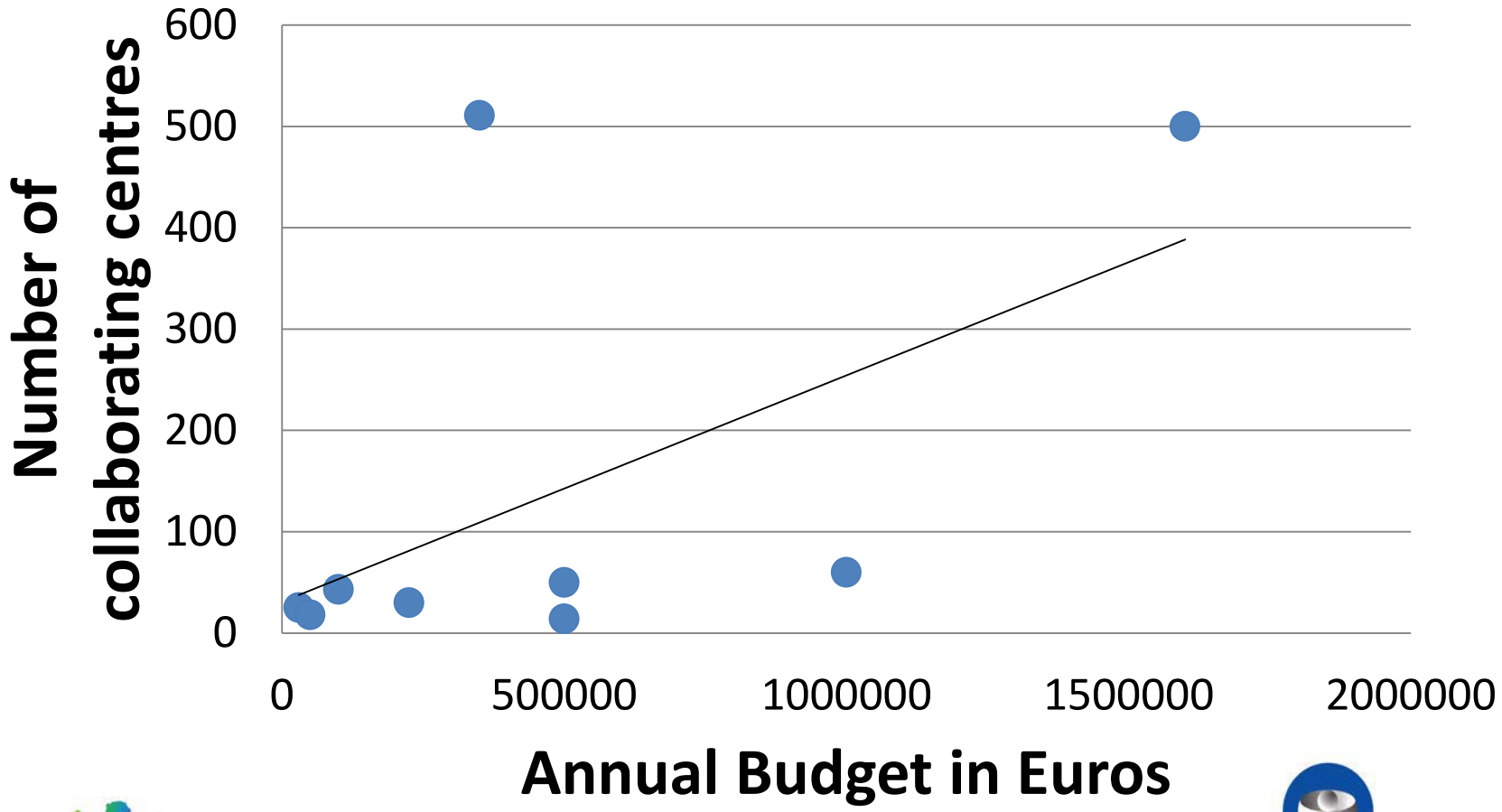
Participants to Budget ratio



EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Collaborating Centres to Budgets ratio



EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Interim analysis

- Significant trial activity
- Variable functions of networks
- Difficulty gathering metrics and costs
- Hidden costs
- This implies problems with performance management

Interim analysis

- Significant trial activity
- Variable functions of networks
- Difficulty gathering metrics and costs
- Hidden costs
- This implies problems with performance management

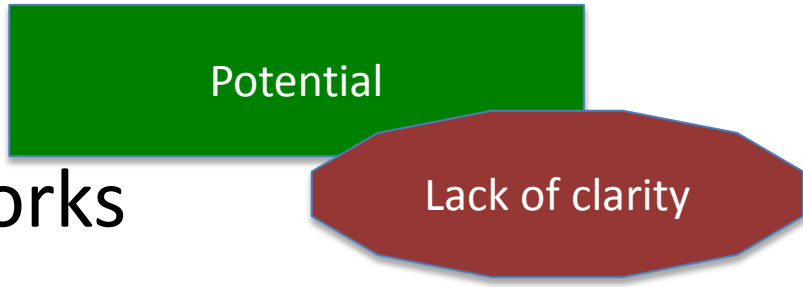
Potential

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

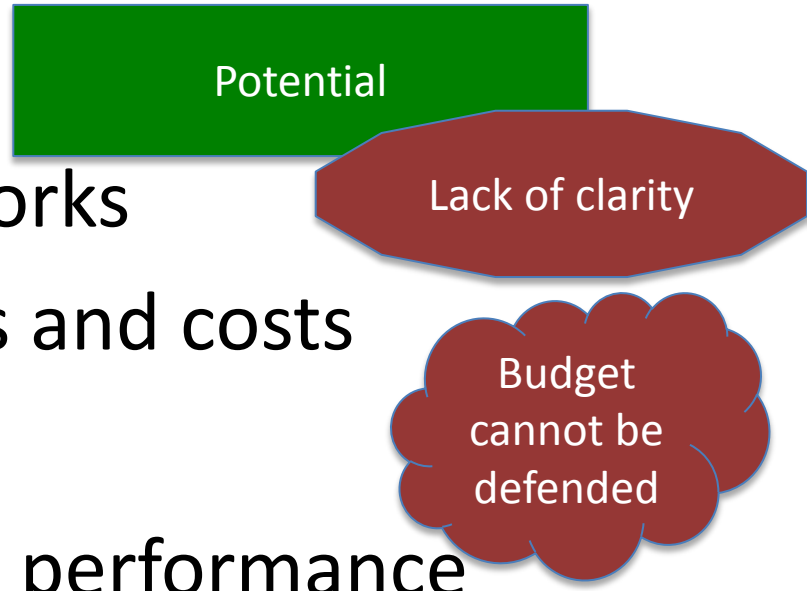
Interim analysis

- Significant trial activity
- Variable functions of networks
- Difficulty gathering metrics and costs
- Hidden costs
- This implies problems with performance management



Interim analysis

- Significant trial activity
- Variable functions of networks
- Difficulty gathering metrics and costs
- Hidden costs
- This implies problems with performance management



EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Interim analysis

- Significant trial activity
- Variable functions of networks
- Difficulty gathering metrics and costs
- Hidden costs
- This implies problems with performance management

Potential

Lack of clarity

Budget cannot be defended

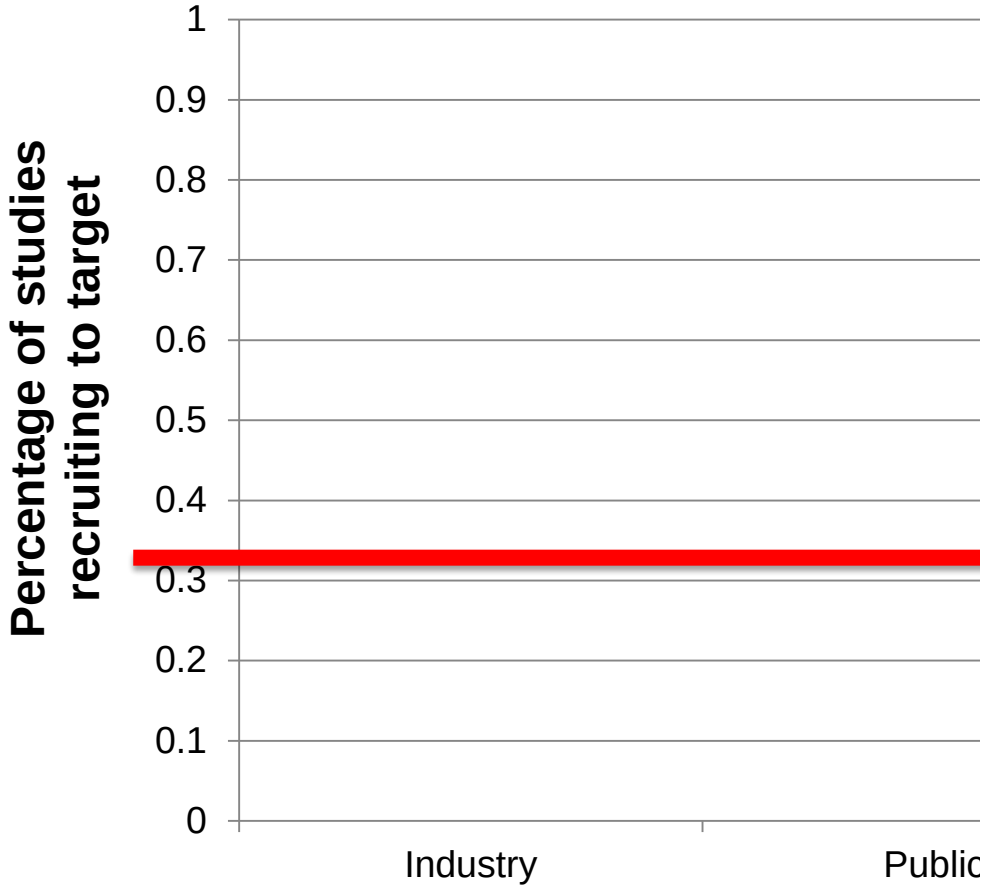
Goodwill goes at its own pace

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Evidence that networks work: Recruitment to target (2013/14)

National Institute for Health Research
Clinical Research Network Children



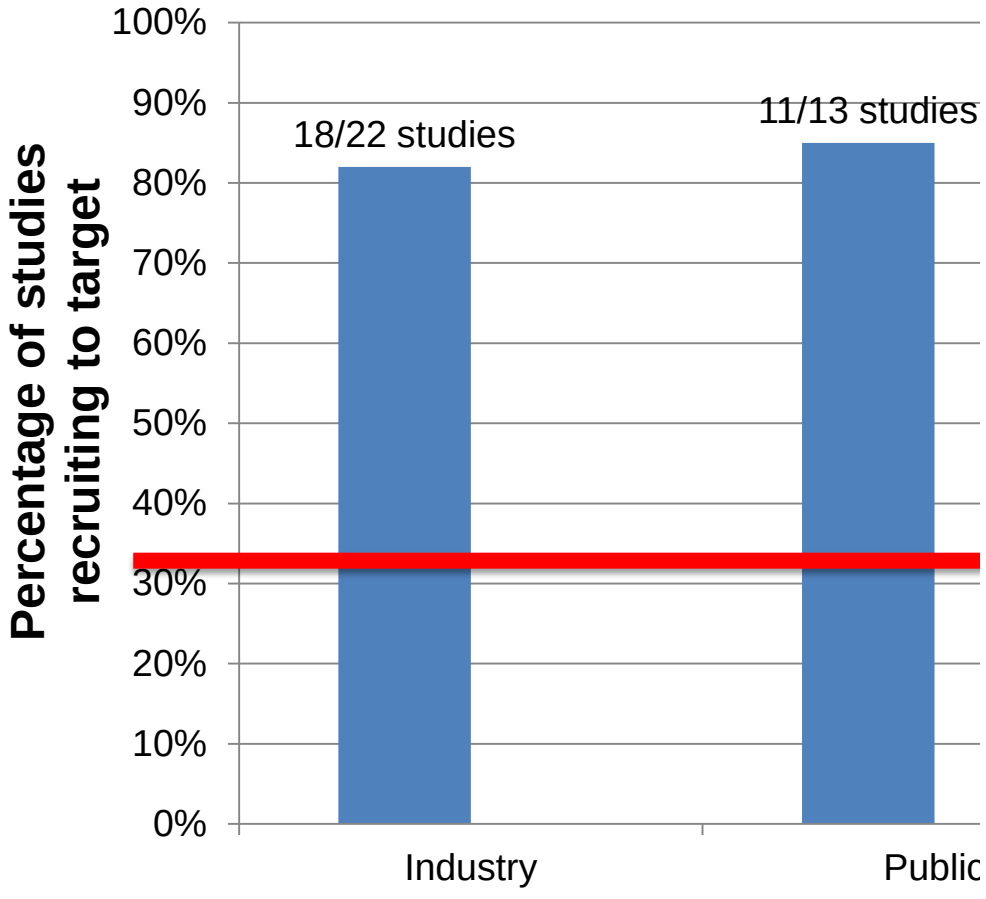
Recruitment to randomised trials: strategies for trial enrolment and participation study. The STEPS study Campbell et al. Health Technology Assessment 2007; Vol 11: No 4
http://www.hta.ac.uk/execsumm/summ1148.htm

Prior to 2007

Fewer than one-third of trials recruited their original target within the time originally specified, and around one-third had extensions.

Evidence that networks work: Recruitment to target (2013/14)

National Institute for Health Research
Clinical Research Network Children



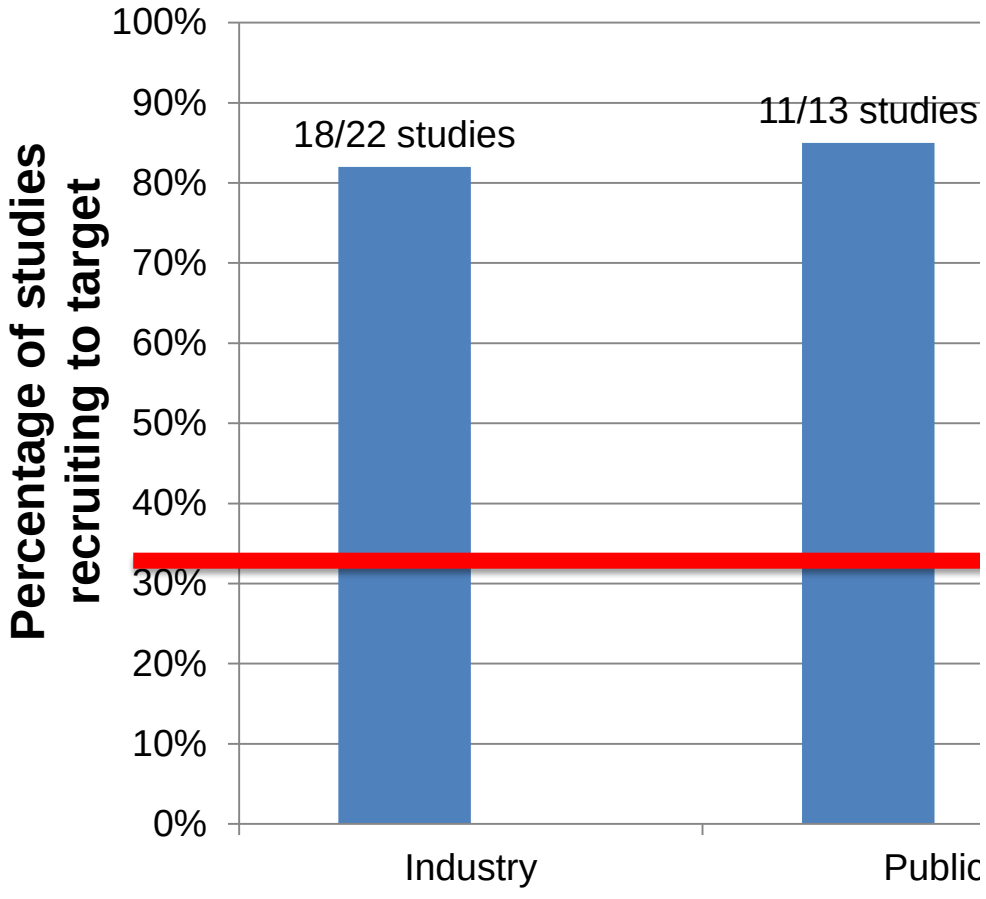
Recruitment to randomised trials: strategies for trial enrolment and participation study. The STEPS study Campbell et al. Health Technology Assessment 2007; Vol 11: No 4
http://www.hta.ac.uk/execsumm/summ1148.htm

Prior to 2007

Fewer than one-third of trials recruited their original target within the time originally specified, and around one-third had extensions.

Evidence that networks work: Recruitment to target (2013/14)

NHS National Institute for Health Research
Clinical Research Network Children



Recruitment to randomised trials: strategies for trial enrolment and participation study. The STEPS study Campbell et al. Health Technology Assessment 2007; Vol 11: No 4
http://www.hta.ac.uk/execsumm/summ1148.htm

Prior to 2007

Fewer than one-third of trials recruited their original target within the time originally specified, and around one-third had extensions.

Driven by consistent performance management based on explicit metrics

Strategies for funding and maintaining a paediatric research network

- Celebrate success
- Be clear about network function
  - Consistent descriptions of core functions
- Be clear about budgets
  - You can't bill for things you don't know about

- Branding
- Metrics

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Key questions

Funders

- What will I get for my money?

Investigators

- What will I get for my effort?

- What will happen without this investment?

Strategies for funding and maintaining a paediatric research network

- If we want to move beyond variation based on historic low budgets need to become more consistent and rigorous

EnprEMA
European network of paediatric research at the European Medicines Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH