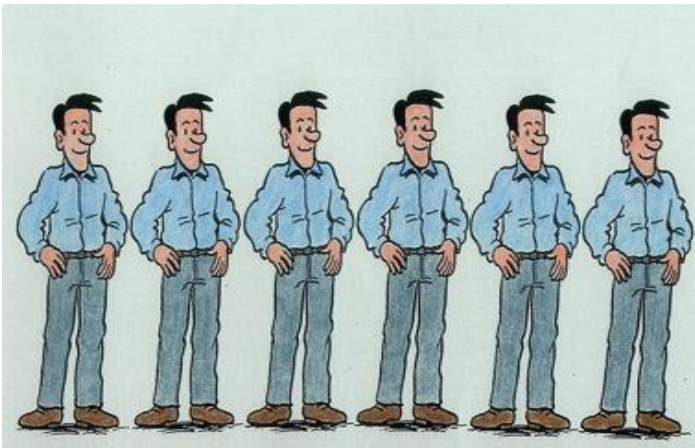


## Real- world data – moving beyond the hype

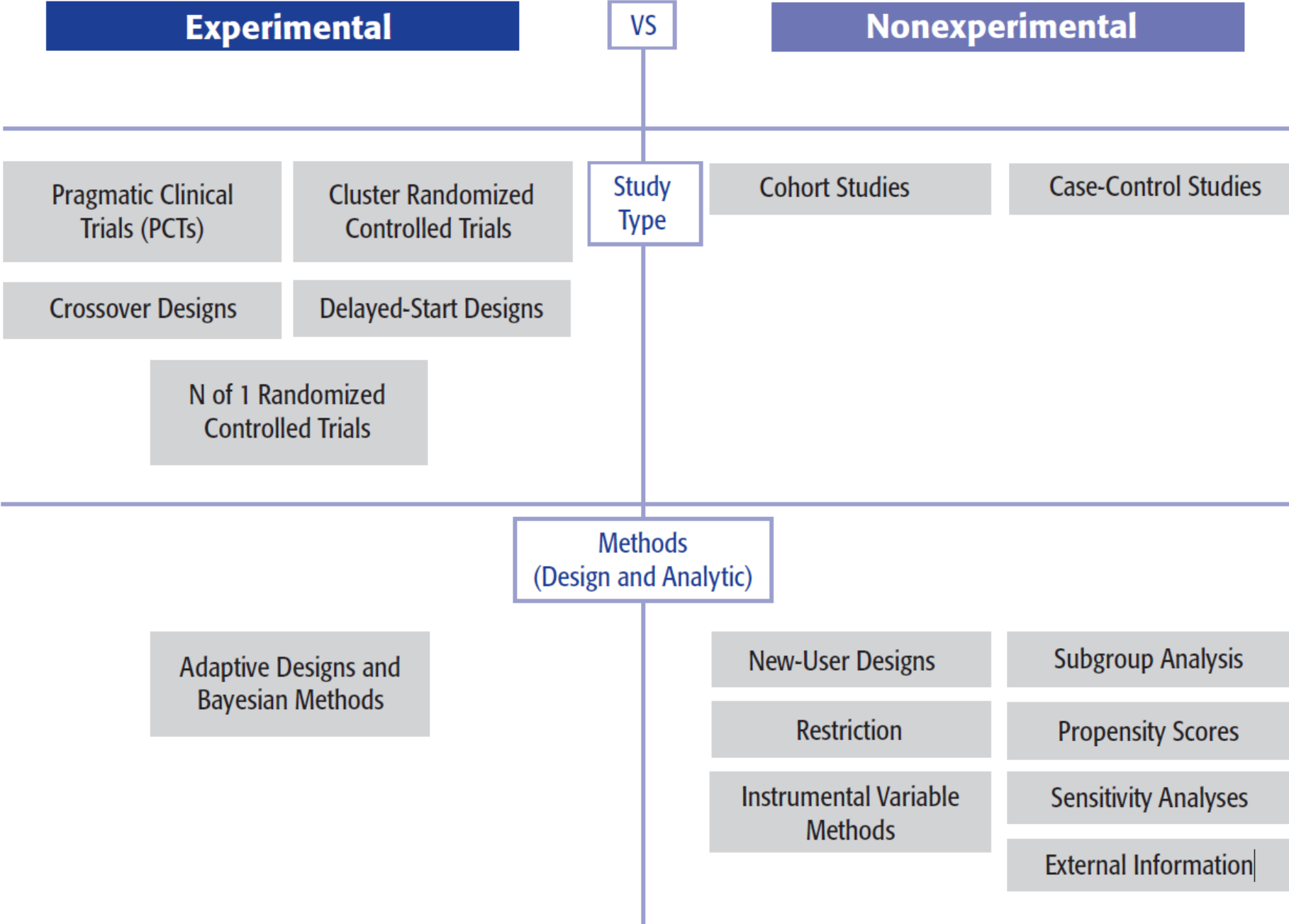
Professor Sarah Garner  
Associate Director NICE – Science Policy and Research  
[sarah.garner@nice.org.uk](mailto:sarah.garner@nice.org.uk)

# Efficacy vs. effectiveness

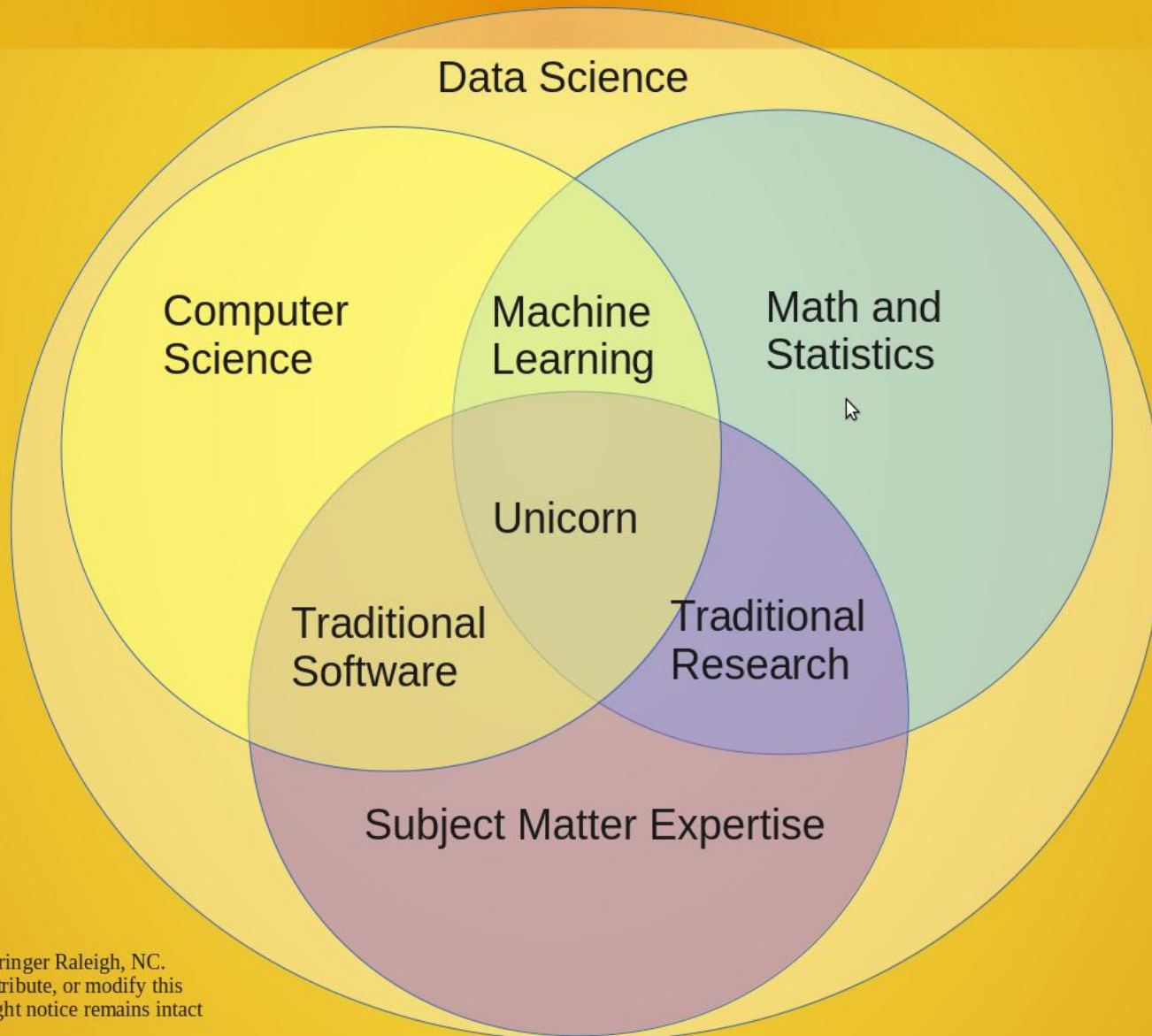
- patient benefit and harm in experimental and closely monitored research studies, normally RCTs.
  - Design minimises bias- high internal validity
  - generalisability questionable
    - restricted entry criteria
    - unrepresentative settings
- patient benefit and harm when the technology is actually applied in everyday practice.
    - pragmatic clinical trials
    - observational studies
    - synthesis
  - *“evidence used for decision-making that is not collected in conventional randomized controlled trials (RCTs)” \* ISPOR*
  - *“Dirty” with a lot of variability and biases*



**Figure 1. Experimental and nonexperimental study types and methods**



# Data Science Venn Diagram v2.0





Accelerated Development of Appropriate Patient Therapies  
a Sustainable, Multi-stakeholder Approach from Research to Treatment-outcome

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ADAPT SMART is an enabling platform for the coordination of Medicines Adaptive Pathways to Patients (MAPPs) activities. MAPPs seeks to foster access to beneficial treatments for the right patient groups at the earliest appropriate time in the product life-span in a sustainable fashion.

ADAPT SMART will support IMI2 projects investigating MAPPs tools and methodologies, and engage in a dialogue with all relevant stakeholders to prove and develop workable MAPPs concepts.

The ADAPT SMART consortium aims to facilitate and accelerate the availability of MAPPs to all healthcare stakeholders. It will:

- Distribute findings, key discoveries and case studies from ongoing or completed MAPPs pilot projects, creating a MAPPs repository of knowledge and opportunities



ADDRESSING DRIVERS,  
OPPORTUNITIES AND  
OBSTACLES OF MAPPs

# Existing 'fixed menu' of drug development

Phase I/II

-----

Phase  
III/Regulation

-----

HTA/Reimbursement

-----

Post market

COMPARATIVE

**COST**



# The MAPPS menu

Using the same menu ingredients to create new combinations tailored to the drug and patient group

## Safe harbour

(Co-design the evidence development and access strategy)

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## Early evidence development

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## Patient access

Conditional license and reimbursement

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## Confirmatory evidence and review





## Welcome to the GetReal Website!


Launched in October 2013, GetReal is a three-year project of the Innovative Medicines Initiative (IMI), a EU public-private consortium consisting of pharmaceutical companies, academia, HTA agencies and regulators (e.g., NICE, HAS, EMA and ZIN), patient organisations and SMEs.

GetReal aims to show how robust new methods of RWE collection and synthesis could be adopted earlier in pharmaceutical R&D and the healthcare decision making process. The consortium is doing this by:


- Bringing together healthcare decision makers, academics, pharmaceutical companies, clinicians, and other societal stakeholders;
- Assessing existing processes, methodologies, and key research issues;
- Proposing innovative trial designs and assessing the value of information;
- Proposing and testing innovative analytical and predictive modelling approaches;
- Assessing operational challenges and proposing and testing the impact of solutions;
- Creating new decision making support, and building tools to allow for the evaluation of development programmes and use in the assessment of the value of introducing new treatments;
- Sharing and discussing deliverables with healthcare decision makers, academics, pharmaceutical companies, clinicians, and other societal stakeholders;
- Developing training for researchers, healthcare decision makers and societal stakeholders in the public and private sector in order to increase knowledge about various aspects of effectiveness.

## Latest News


### GetReal vacancy at NICE: Scientific Project Manager

Posted on August 25, 2015 12:11  
The National Institute for Health and Care Excellence... 

### Response from WP3 to article about PRECIS-2 tool published online by BMJ


Posted on August 04, 2015 14:33  
The rapid response from WP3 entitled "Pragma... 

### New WP1 co-lead

Posted on July 08, 2015 10:01  
WP1 will have a new project co-lead from July 2015... 

[All news...](#)

## Links

 Internet | Protected Mode: On

# Real world data challenges

- Culture change
- Understanding of potential impact
- Skill development and capacity
- Concerns over confidentiality
- Research or care?
- Ethical issues: coercion
- Terminology
- Methods
- How to define best practice
  - Critical appraisal tools