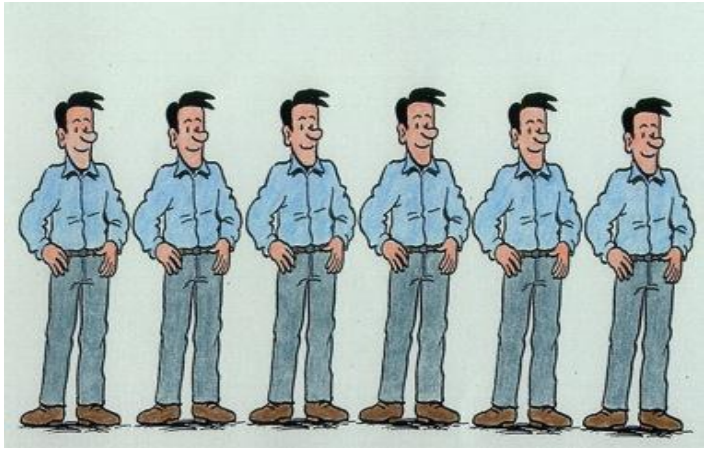
NICE National Institute for Health and Care Excellence

Real- world data – moving beyond the hype

Professor Sarah Garner
Associate Director NICE – Science Policy and Research
sarah.garner@nice.org.uk

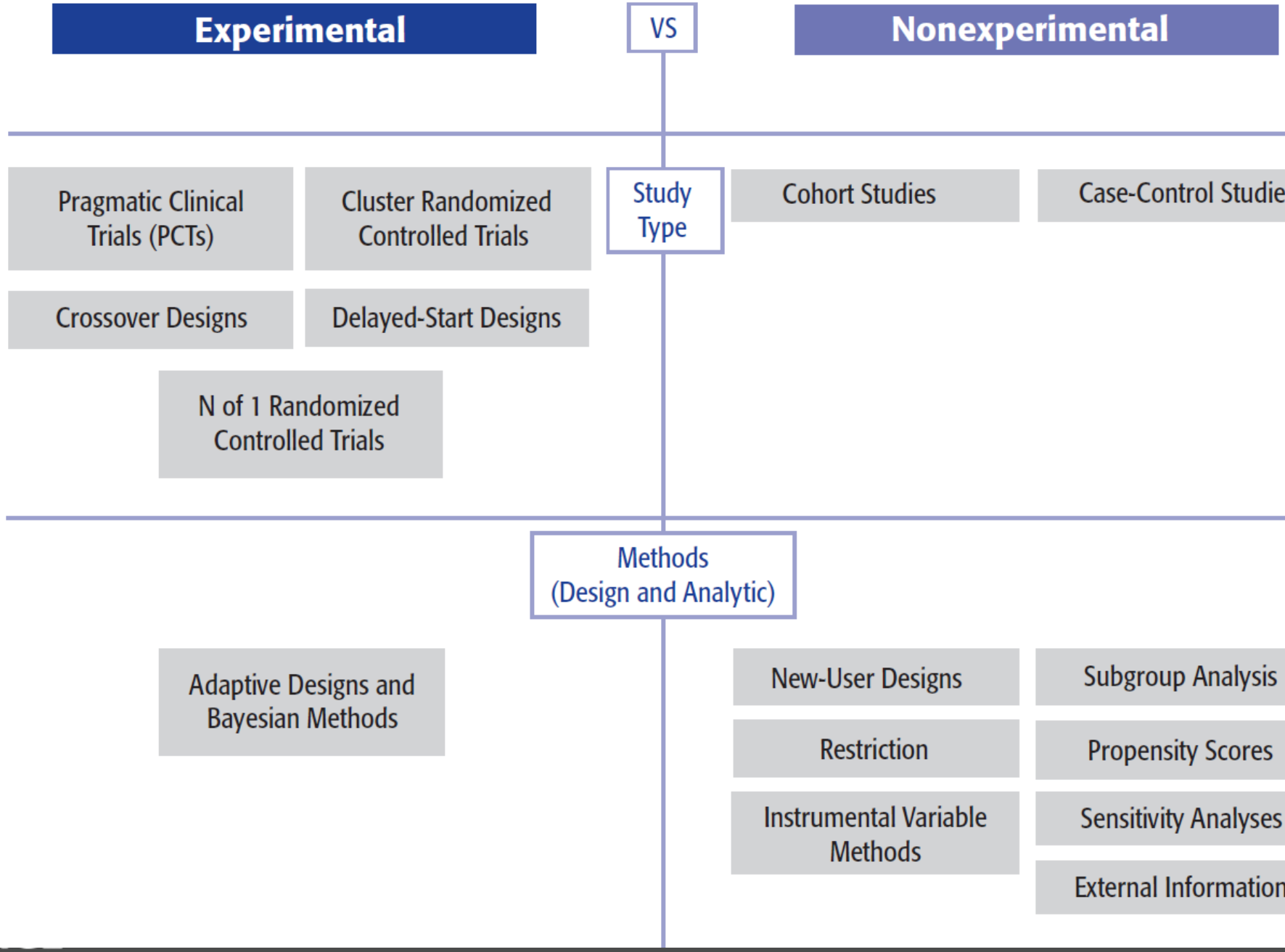
Efficacy vs. effectiveness

- 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

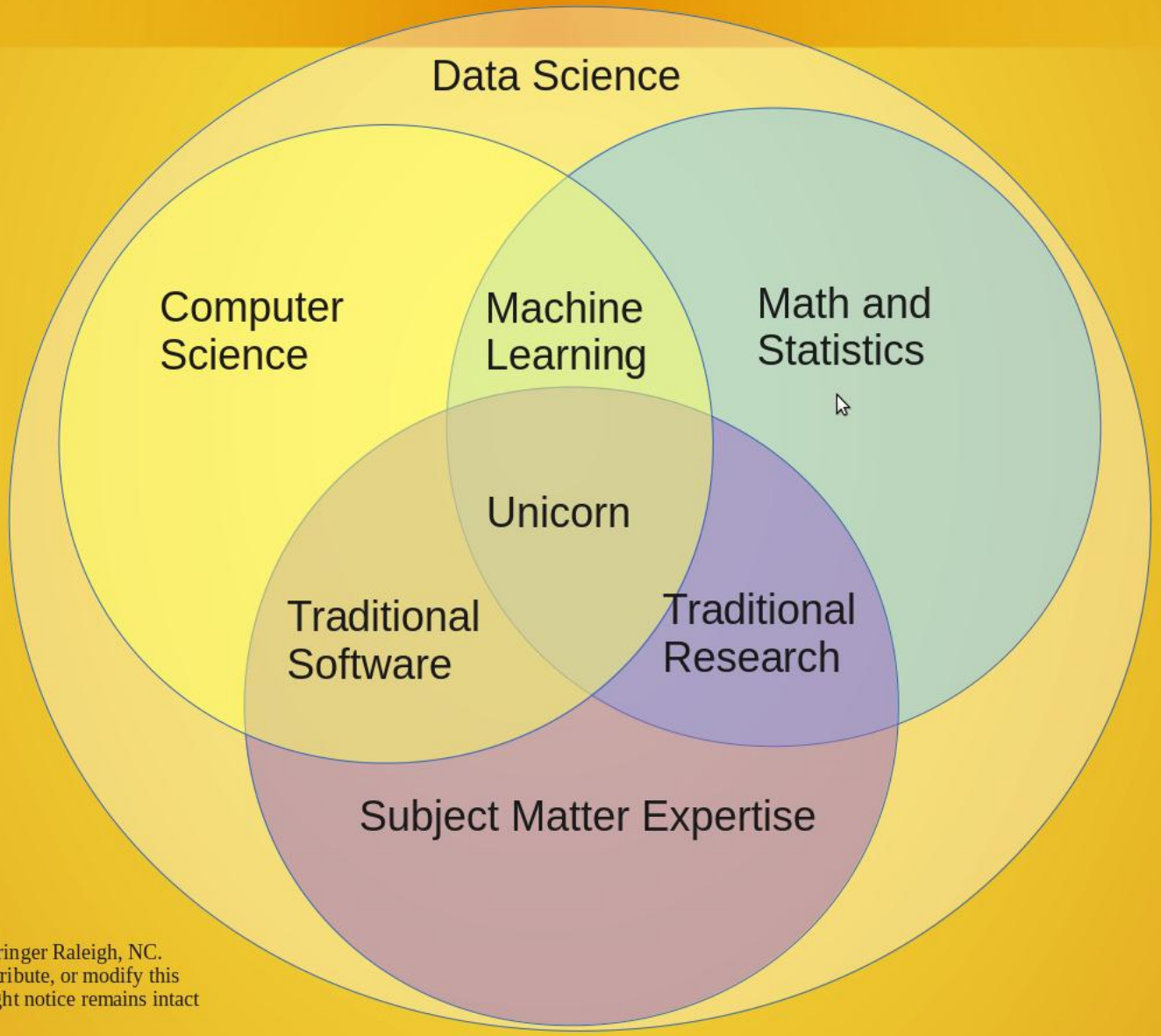


- patient benefit and harm when the technology is actually applied in everyday practice.
  - pragmatic clinical trials
  - observational studies
  - synthesis
- *"evidence used for decision-making that is not collected in conventional randomized controlled trials (RCTs)" * ISPOR*
- '"Dirty" with a lot of variability and biases

Figure 1. Experimental and nonexperimental study types and methods



Data Science Venn Diagram v2.0



ADAPTSMART

Accelerated Development of Appropriate Patient Thera

a Sustainable, Multi-stakeholder Approach from Research to Treatment-outc

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

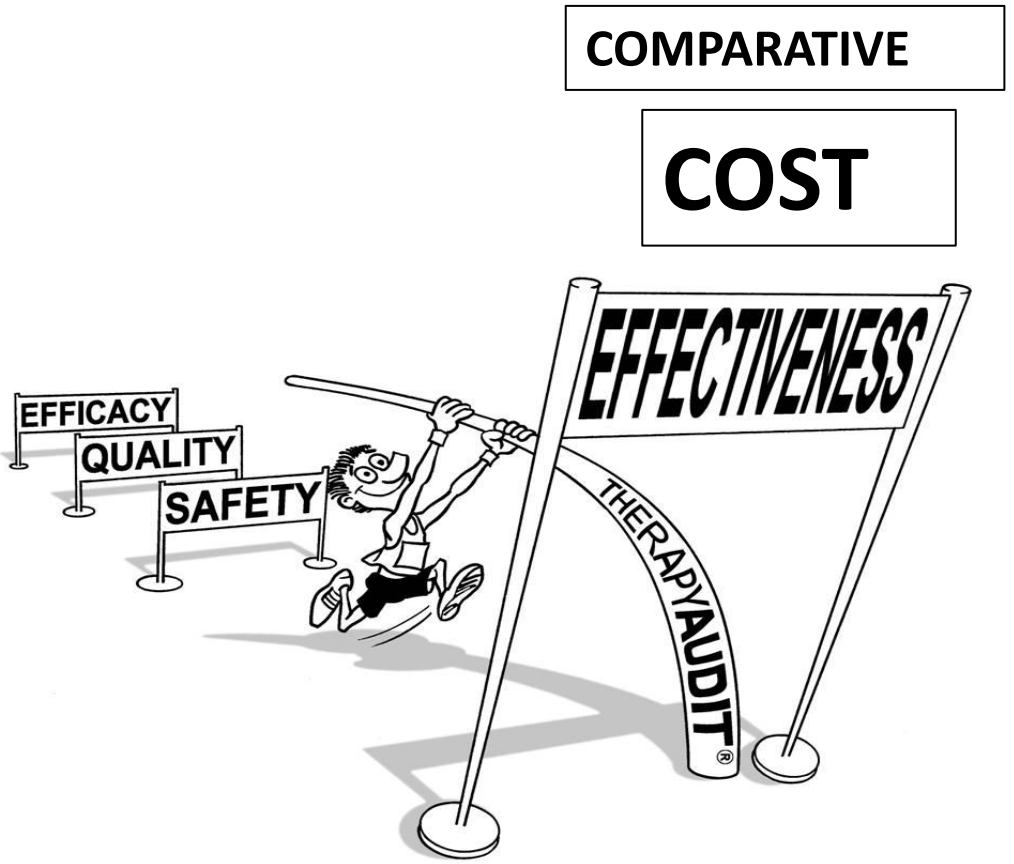
Existing ‘fixed menu’ of drug development

Phase I/II

Phase III/Regulation

HTA/Reimbursement

Post market



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)

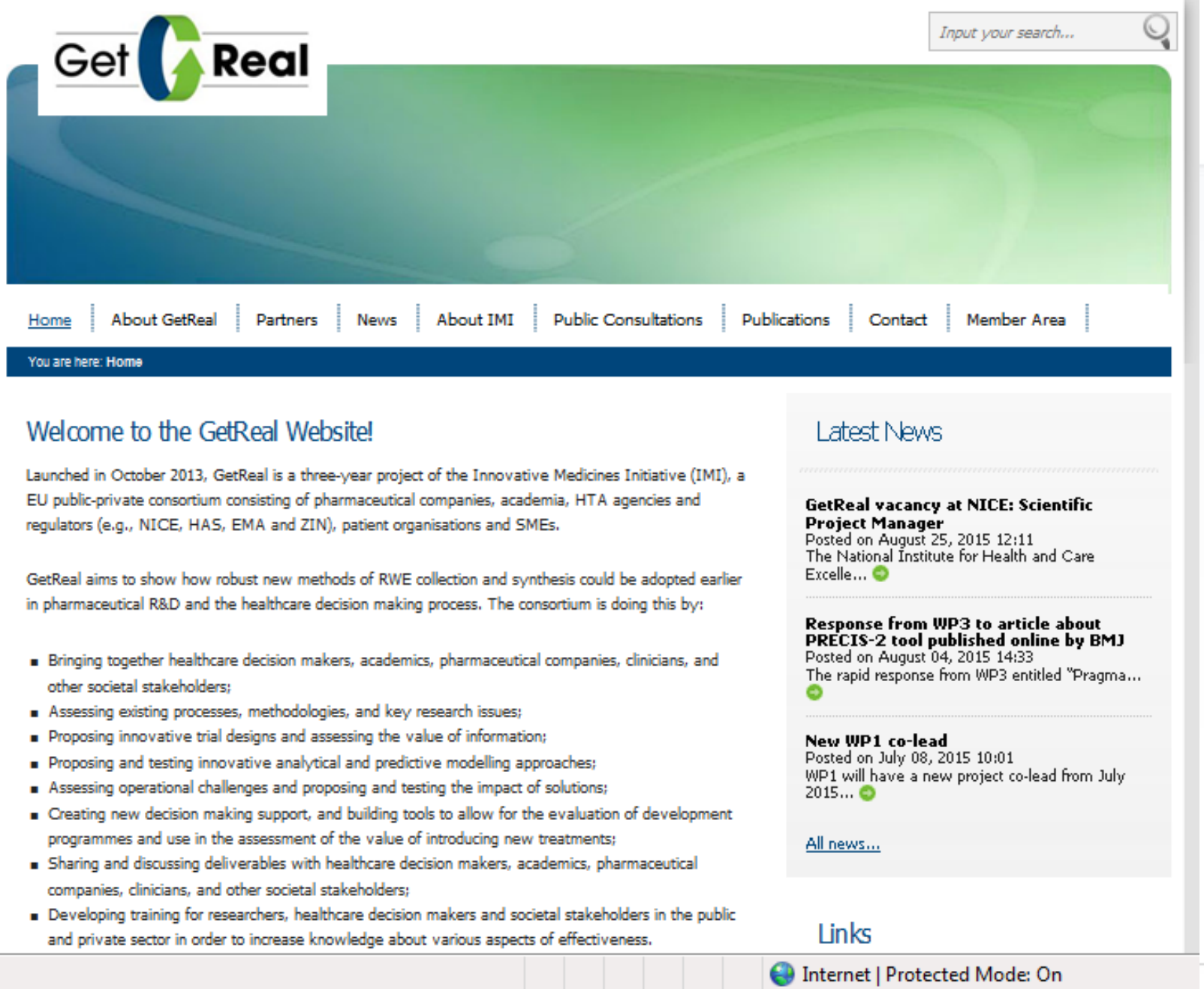
Early evidence development

Patient access

Conditional license and reimbursement

Confirmatory evidence and review

Get



The research leading to these results has received support from the Innovative Medicines Initiative Joint Undertaking under grant agreement no [115303], resources of which are composed of financial contribution from the European Union's Seventh Framework Programme (FP7/2007-2013) and EFPIA companies' in kind contribution.
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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

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